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2026
Global Stem Cell Industry Development Blue Book

September 2026

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Executive Summary

Stem cell therapy is an advanced form of cell therapy that harnesses the unique biological properties of stem cells, including self-renewal and multilineage differentiation, to repair and regenerate damaged cells, tissues, or organs through ex vivo preparation and transplantation. As clinical demand for treatments for degenerative diseases, autoimmune disorders, and organ damage continues to grow worldwide, regulatory frameworks for stem cells are being refined across countries. Meanwhile, advances in iPSC reprogramming, gene editing, standardized cell manufacturing, and organoid modeling are being translated into practice. The stem cell industry is entering a critical stage of transition from basic research to standardized clinical translation and commercialization.

Frost & Sullivan hereby publishes the 2026 Global Stem Cell Industry Development Blue Book, which provides an in-depth analysis of the global stem cell industry. The report comprehensively examines the fundamental definitions and classifications of stem cells, their clinical applications across multiple diseases, regulatory frameworks in major global markets, the current status of core stem cell therapy technologies, challenges in clinical translation, and future industry trends. It also outlines the evolution of the industry and its technologies, explores development potential, and analyzes the technological, clinical, and policy drivers underlying market growth.

Continued R&D Progress and an Improving Technology System

Stem cell therapy has undergone decades of scientific and clinical exploration. With their core properties of self-renewal and multilineage differentiation, stem cells have become a major focus of global regenerative medicine research. As understanding of cellular mechanisms has advanced, two principal development pathways have emerged: adult stem cells and pluripotent stem cells, with the latter comprising embryonic stem cells and induced pluripotent stem cells. The integration of gene editing, organoid, and exosome technologies can further expand indications and reduce transplantation risks.

Addressing Key Clinical Needs in Difficult-to-Treat Diseases

The global patient populations affected by graft-versus-host disease, heart failure, Parkinson's disease, knee osteoarthritis, stroke, liver cirrhosis and liver failure, idiopathic pulmonary fibrosis, thrombocytopenia, chronic obstructive pulmonary disease, and chronic periodontitis continue to grow. Conventional drugs and surgical treatments can generally alleviate symptoms but cannot repair damaged or nonfunctional tissues. Through mechanisms including cell differentiation and replacement, paracrine signaling, immunomodulation, and direct cell transfer, stem cell therapies repair diseased tissues and restore organ function at the cellular level. They can reduce inflammation, improve patients' quality of life, and reverse tissue degeneration, offering a new regenerative approach for difficult-to-treat chronic diseases that lack curative options.

Establishment of an Integrated Stem Cell Product Manufacturing System

Stem cell product manufacturing typically spans donor and starting-material qualification, where applicable, cell isolation or derivation, cell banking, scalable expansion in GMP-compliant closed and automated systems, cryopreservation, quality testing, and product release. As living products, stem cell therapies face donor and starting-material variability, process-dependent product quality, high manufacturing costs for autologous products, potential immunogenicity of allogeneic products, and complex supply-chain requirements. Key challenges include maintaining cell purity, functionality, and critical quality attributes during processing and scale-up; controlling residual undifferentiated cells in pluripotent stem cell-derived products; ensuring cryopreservation stability, predictive potency testing, and batch consistency. Industry solutions include xeno-free and feeder-free culture systems, closed and automated manufacturing systems, DMSO-reduced or DMSO-free cryopreservation technologies, real-time in-process monitoring, qualified and locally sourced critical materials, and digital traceability and cold-chain management. Long-term patient follow-up is managed separately as part of clinical and regulatory risk management.

Catalogue

Catalogue

Catalogue

Catalogue

Chapter 1 Overview of the Stem Cell Therapy Industry

01

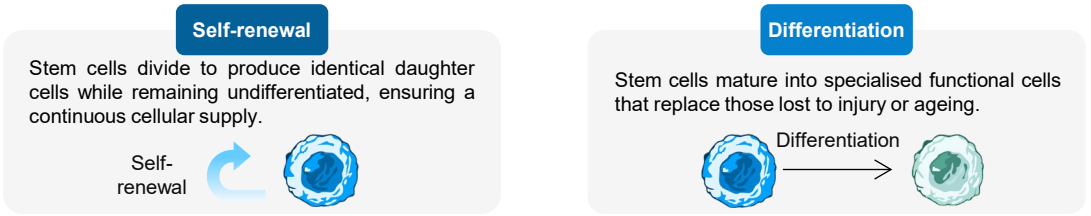
Overview of Stem Cells

Stem cells are characterized by self-renewal and multilineage differentiation potential. They hold significant value in disease treatment, disease modeling, and drug screening. Advancing stem cell research has thus become a critical foundation for driving progress in regenerative medicine.

Introduction to Stem Cells

Stem cells constitute a class of undifferentiated cells that possess the potential to differentiate into multiple cell types in vivo while retaining the capacity for repeated division in an undifferentiated state. Present in both embryos and adult tissues, they are defined by two core properties: self-renewal and differentiation. By continuously supplying new cells to replace those lost to physiological turnover, injury or disease, stem cells maintain tissue homeostasis—repairing damaged tissue, replenishing high-turnover compartments and replacing apoptotic cells.

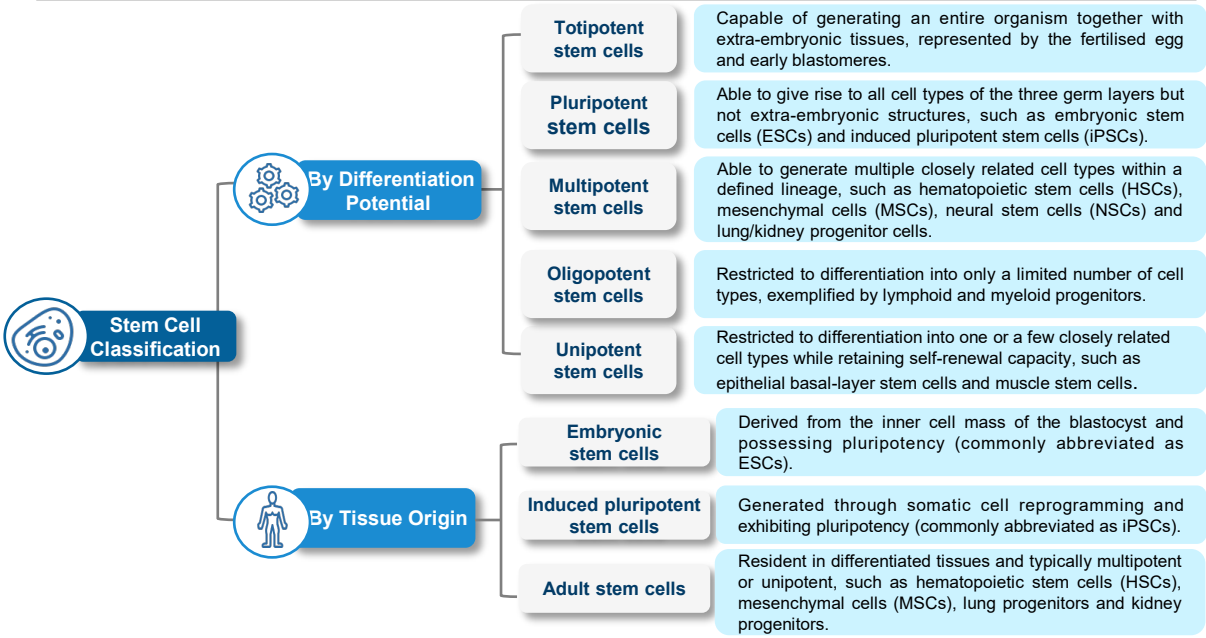
Figure. Defining Properties of Stem Cells



Classification of Stem Cells

Stem cells are classified according to differentiation potential or tissue origin. By potency, five categories are recognised: totipotent stem cells, which can form an entire organism and extra-embryonic tissues (exemplified by the fertilised egg); pluripotent stem cells, which give rise to all three germ layers but not extra-embryonic structures, such as embryonic stem cells (ESCs) and induced pluripotent stem cells (iPSCs); multipotent stem cells, which generate multiple closely related cell types within a lineage, including hematopoietic stem cells (HSCs), mesenchymal cells (MSCs) and neural stem cells (NSCs); oligopotent stem cells, restricted to a few cell types such as lymphoid or myeloid progenitors; and unipotent stem cells, which produce only one or a few closely related cell types while retaining self-renewal capacity, for example epithelial basal-layer or muscle stem cells. By tissue origin, they are divided into embryonic stem cells derived from the blastocyst inner cell mass and possessing pluripotency; adult stem cells resident in differentiated tissues and responsible for repair and regeneration, such as HSCs and MSCs; and induced pluripotent stem cells generated by somatic reprogramming and exhibiting pluripotency comparable to ESCs.

Figure. Classification of Stem Cells



Source: Literature Review, Public Information, Frost & Sullivan Analysis

Application Fields of Stem Cells

Stem cells hold significant value in disease treatment, disease modelling and drug screening. They enable tissue repair via functional differentiation, recapitulate human pathology through 3D organoids, and—combined with stably transfected cell lines and co-cultures—efficiently assess drug efficacy, regenerative potential in screening.

Applications in Disease Treatment

Owing to their unique functions and properties, stem cells offer broad clinical application prospects. Leveraging robust differentiation capacity, stem cells convert into specialised cells that directly replace damaged or dysfunctional tissue cells. Applications have expanded from hematological disorders to neurological, metabolic, autoimmune, genetic disorders, infectious, and respiratory and renal regenerative diseases.

Figure. Applications of Stem Cells in Disease Treatment

Hematological Disorders

- **Core approach:** HSCs
- **Applicable diseases:** leukaemia, aplastic anaemia, myelodysplastic syndromes and other hematological disorders.
- **Principle:** Transplantation of hematopoietic stem cells from healthy donors reconstitutes the patient's hematopoietic and immune systems, replacing diseased bone-marrow function.

Neurological Disorders

- **Core cells:** neural stem cells (NSCs), progenitors or functional neurons
- **Applicable diseases:** stroke, Parkinson's disease, Alzheimer's disease, ALS, traumatic brain injury, spinal cord injury, epilepsy, etc.
- **Principle:** Post-transplantation differentiation into functional neurons or direct replacement of missing neurons, together with paracrine support of neural-circuit repair.

Immune Disorders

- **Core cells:** MSCs
- **Applicable diseases:** systemic lupus erythematosus, rheumatoid arthritis, multiple sclerosis, GvHD, etc.
- **Principle:** Potent immunomodulation suppresses hyper-activated immune cells, restores tolerance and mitigates autoimmune tissue damage.

Metabolic and Cardiovascular Diseases

- **Core cells:** iPSCs, adipose-derived stem cells, MSCs
- **Applicable diseases:** obesity, type 2 diabetes, cardiovascular diseases (myocardial infarction, heart failure, etc.).
- **Principle:** Repair of damaged tissues (e.g. cardiomyocytes and pancreatic β-cells), metabolic regulation and paracrine improvement of the local microenvironment.

Genetic Deficiency Disorders

- **Core cells:** gene-edited HSCs or iPSC-derived cells
- **Applicable diseases:** monogenic disorders (e.g. thalassemia), ophthalmic diseases (e.g. retinitis pigmentosa), diabetes, etc.
- **Principle:** Gene editing restores function in autologous HSCs, or edited iPSCs are directed to differentiate into functional cells for tissue replacement or repair.

Infectious Diseases

- **Core cells:** MSCs from umbilical cord, adipose tissue, bone marrow, dental pulp
- **Applicable diseases:** chronic periodontitis, refractory pulmonary inflammation, persistent wound infection, sepsis, infected skin ulcers, etc.
- **Principle:** Secretion of antimicrobial peptides and anti-inflammatory factors suppresses inflammation, mitigates tissue damage and remodels the local microenvironment to reduce recurrence risk.

Renal Regenerative Diseases

- **Core cells:** autologous renal progenitor cells
- **Applicable diseases:** chronic kidney disease, diabetic nephropathy, etc.
- **Principle:** Directed differentiation repairs tubular injury and reconstitutes epithelial and filtration function.

Respiratory Regenerative Diseases

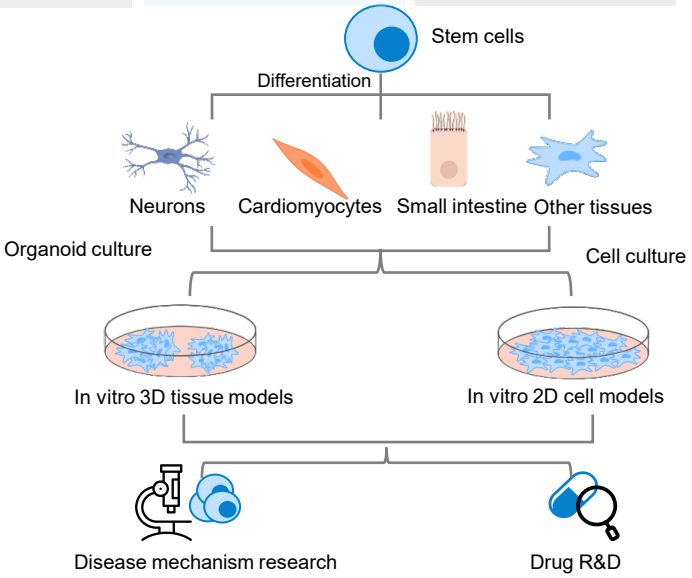
- **Core cells:** airway basal stem cells
- **Applicable diseases:** idiopathic pulmonary fibrosis, COPD, bronchiectasis, etc.
- **Principle:** Directed differentiation repairs alveolar injury and reconstitutes epithelial and gas-exchange function.

Applications of Stem Cells in Disease Modelling

Stem cells are induced to differentiate into target cells and form three-dimensional organoids that recapitulate human organ structure and function, enabling controlled simulation of disease pathophysiology and providing a robust platform for mechanistic research.

Applications of Stem Cells in Drug Screening

Stem cells are used to construct drug-responsive stably transfected lines for high-throughput qualitative and quantitative compound screening. Combined with directed differentiation and co-culture validation, this approach further evaluates tissue-repair potential and efficacy mechanisms in neurological, cardiovascular and pulmonary diseases, providing an efficient and reliable platform for drug discovery and mechanistic studies.



Source: Literature Review, Frost & Sullivan Analysis

Stem Cell Therapy

Stem cell therapy harnesses the self-renewal and differentiation potential of stem cells to achieve repair, replacement and regeneration of damaged cells, tissues or organs.

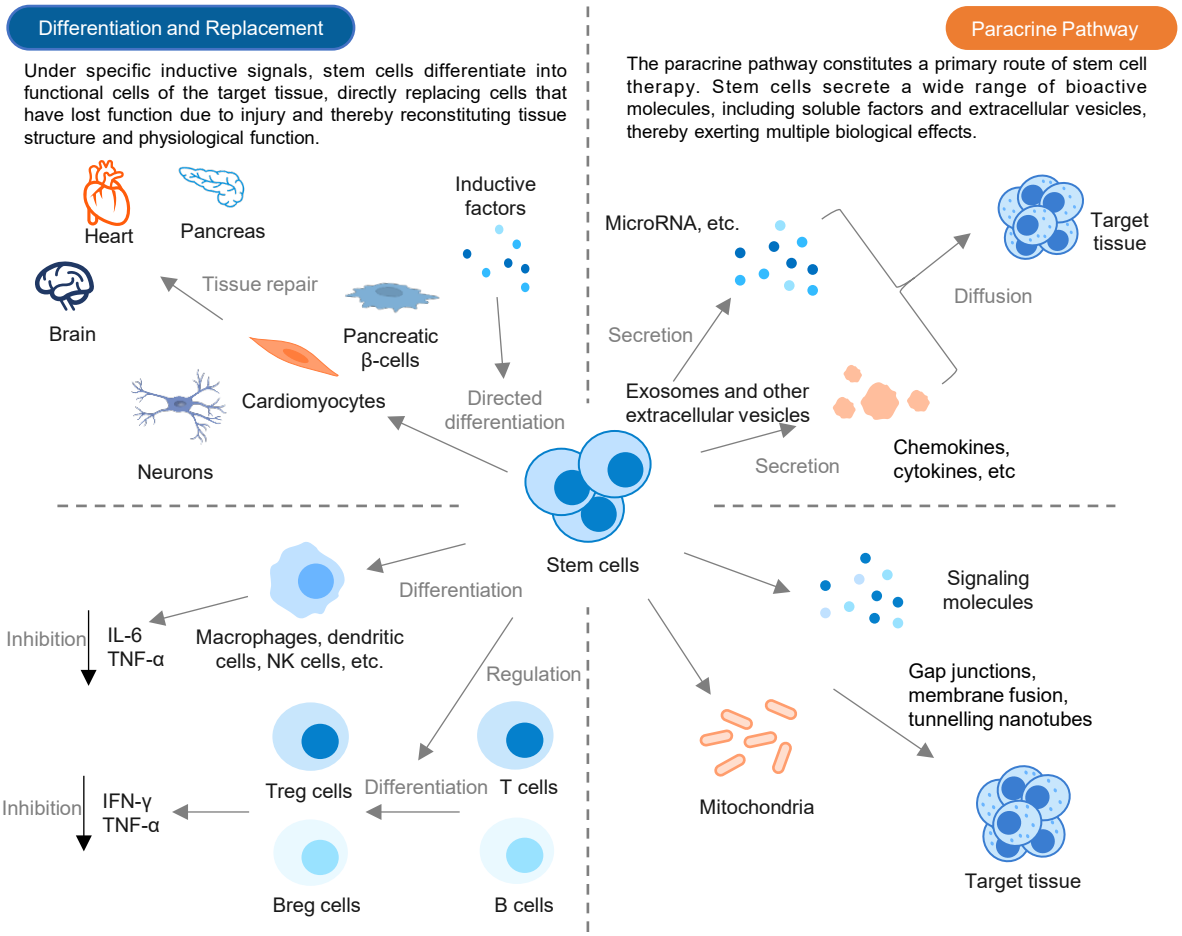
Concept of Stem Cell Therapy

Stem cell therapy utilises the unique self-renewal and differentiation potential of stem cells. Through in vitro culture, expansion, directed differentiation and transplantation, exogenous cells are introduced into the patient to regenerate damaged cells and tissues, or to replace them with healthy, fully functional cells, thereby achieving the therapeutic goals of repair, replacement or regeneration. Stem cells used in therapy are classified by source as autologous (derived from the patient) or allogeneic (derived from a healthy donor).

Mechanisms of Action of Stem Cell Therapy

Stem cell therapy exerts its effects through four principal mechanisms: differentiation and replacement, immunomodulation, paracrine signalling and direct intercellular transfer.

Figure. Mechanisms of Action of Stem Cell Therapy



Source: Literature Review, Frost & Sullivan Analysis

Pluripotent Stem Cell Therapy

Pluripotent stem cells have attracted considerable attention owing to their unlimited self-renewal capacity and potential to differentiate into nearly all mature somatic cell types. Pluripotent stem cells, represented by embryonic stem cells and induced pluripotent stem cells, have become a major research focus in the field of cell therapy.

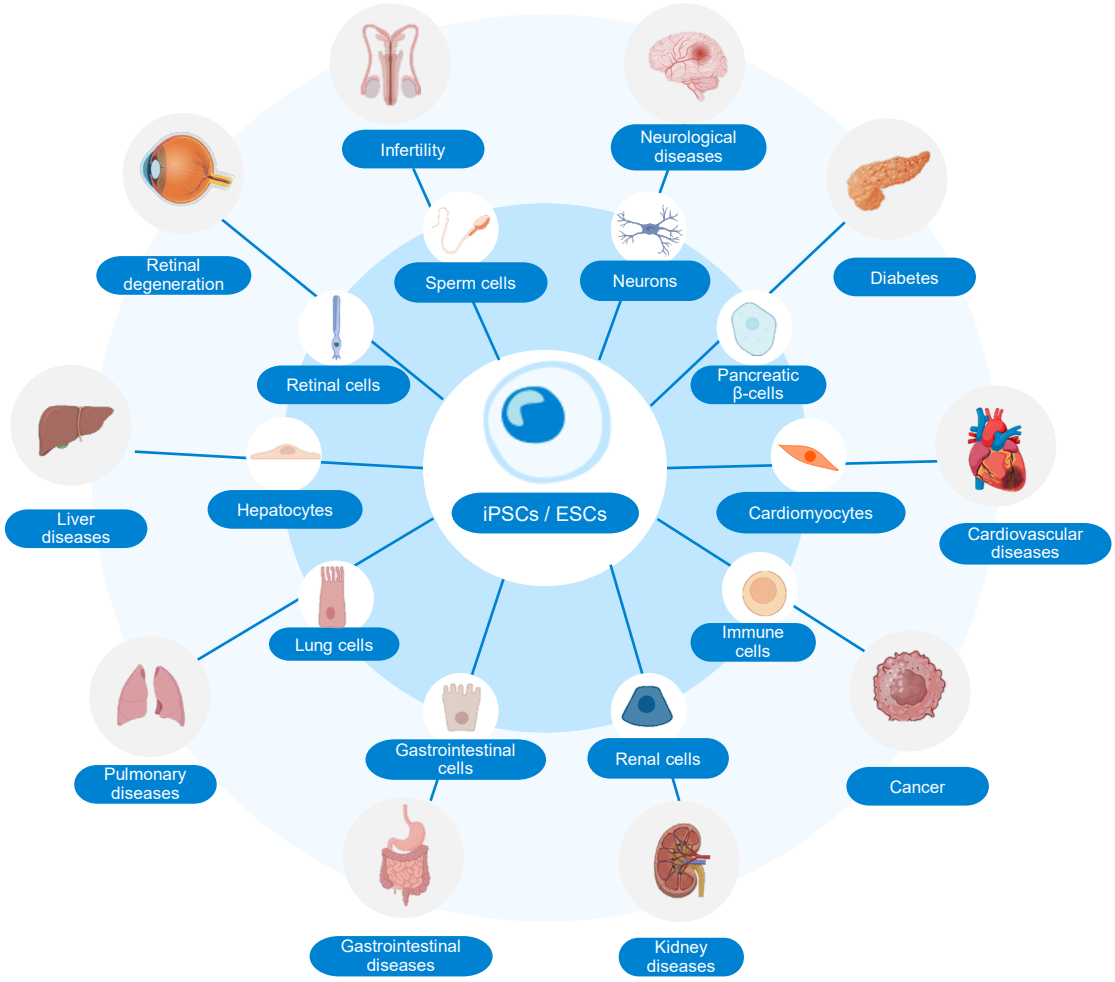
Pluripotent Stem Cells

Pluripotent stem cells (PSCs) are a class of cells possessing self-renewal capacity and multilineage differentiation potential. They can give rise to all cell types of the three germ layers—for example, neurons and skin from the ectoderm, cardiomyocytes and blood from the mesoderm, and liver and pancreas from the endoderm. PSCs primarily comprise embryonic stem cells (ESCs) and induced pluripotent stem cells (iPSCs). ESCs are derived from the inner cell mass of the early blastocyst, while iPSCs are generated through somatic cell reprogramming; both hold broad clinical application prospects.

Pluripotent Stem Cell-Derived Therapies

Pluripotent stem cell-derived therapy utilises the robust self-renewal and multilineage differentiation potential of PSCs. Cells are directed in vitro to differentiate into tissue-specific stem cells, terminally differentiated functional cells or tissues, which are then transplanted into the patient to replace damaged or lost cells, promote tissue repair or restore organ function. Key advantages include a stable and homogeneous cell source that enables scalable production of “off-the-shelf” cell or tissue products with reduced dependence on donor availability and fewer ethical concerns, and the ability to generate cell populations functionally equivalent to human tissues, thereby achieving genuine cell or tissue replacement beyond the regenerative limits of conventional drugs.

Figure. Differentiation Lineage of Pluripotent Stem Cells and Disease Treatment Landscape



Source: Literature Review, Frost & Sullivan Analysis

Adult Stem Cell Therapy

Adult stem cells have attracted widespread attention due to their limited self-renewal capacity and directed differentiation potential. Represented by mesenchymal cells (MSCs) and hematopoietic stem cells (HSCs), these cells are currently a major focus of research in stem cell therapy.

Adult Stem Cells

Adult stem cells reside in differentiated tissues (such as placenta/umbilical cord, bone marrow, adipose tissue and cord blood). They possess limited self-renewal capacity and multilineage differentiation potential, yet cannot give rise to a complete organism and lack the pluripotency of embryonic stem cells. They play important roles in tissue repair, immunomodulation and regenerative medicine, and primarily include hematopoietic stem cells (HSCs), mesenchymal cells (MSCs), neural stem cells (NSCs) and lung/kidney organ-specific progenitor cells.

Adult Stem Cell Therapy

Adult stem cell-derived therapy isolates, expands and, where needed, genetically modifies stem cells from adult tissues before administration to the patient. It aims to replace damaged or dysfunctional cells, promote tissue repair, modulate immune responses or improve organ function, showing favourable potential for repair and immunomodulation. Major modalities include mesenchymal cell therapy, hematopoietic cell therapy, neural stem cell therapy, osteoprogenitor cell therapy, endothelial progenitor cell therapy, epidermal stem cell therapy, limbal stem cell therapy and organ-specific progenitor cell therapy.

Table. Hematopoietic Cell Therapy

	Therapeutic principle	Technical advantages	Limitations	Application scenarios
Conventional HSC Transplantation	Myeloablative conditioning followed by infusion of donor or autologous HSCs to reconstitute the hematopoietic and immune systems	• Most mature technology with extensive clinical experience • curative for multiple high-risk hematological malignancies • graft-versus-leukaemia effect can eliminate chemotherapy-resistant residual tumor cells	• Scarcity of fully matched donors; alternative sources increase risk of severe GvHD or delayed engraftment • GvHD and infection remain major causes of mortality and morbidity, conditioning toxicity limits use in elderly or frail patients • risk of relapse and secondary complications	• Hematological malignancies • Non-malignant hematological disorders • Immunodeficiency diseases • Inborn errors of metabolism • Autoimmune diseases
Gene-edited HSCs	Precise genetic modification of the patient's autologous HSCs ex vivo (using lentiviral vectors or CRISPR technology), followed by administration of the edited cells	• Autologous cells eliminate rejection risk • Addresses the disease at its genetic root • Mature lentiviral vector technology and continually improving CRISPR editing precision	• Low long-term HSC editing efficiency and uncertain engraftment durability • Off-target mutations and process-related damage may impair cell viability • Lengthy and costly individualized workflow; difficult to apply in patients with exhausted stem cell pools	• Haemoglobinopathies • Primary Immunodeficiencies • Lysosomal storage disorders • Metachromatic leukodystrophy, etc

Figure. Other Stem Cell Therapies

Mesenchymal Cell Therapy

Mesenchymal cells have broad sources (placenta/umbilical cord, bone marrow, adipose tissue, dental pulp, cord blood, etc.) and low immunogenicity. They exert immunomodulatory and reparative effects via paracrine signalling and homing, applied in cardiovascular diseases, autoimmune disorders and tissue injury.

Neural Stem Cell Therapy

Differentiate into neurons and glial cells, secrete neurotrophic factors and modulate the immune microenvironment. Limited endogenous sources; often derived via iPSC induction or MSC transdifferentiation. Combined with drugs or gene editing for neurodegenerative diseases, demyelinating disorders and acute injury.

Osteoprogenitor Cell Therapy

Differentiate into osteoblasts /chondrocytes and synthesise matrix to repair osteochondral defects. May be autologous (bone marrow, adipose) or allogeneic (umbilical cord, placenta) and combined with scaffolds to form tissue-engineered constructs for osteoarthritis, joint reconstruction and bone-defect repair.

Organ-specific Progenitor Cell Therapy

Oligopotent adult stem cells from the patient's own lung or kidney can be directed to differentiate into mature alveolar or renal tubular epithelial cells, directly repairing parenchymal injury while secreting anti-inflammatory and anti-fibrotic factors to remodel the organ microenvironment.

Endothelial Progenitor Cell Therapy

Participate in neovascularisation and endothelial repair by differentiating into endothelial cells and secreting pro-angiogenic factors. Broad sources enable applications in stent endothelialisation, ischemic vascular disease, injury repair and soft-tissue regeneration.

Epidermal Stem Cell Therapy

Epidermal stem cells isolated from the basal layer or hair-follicle bulge are expanded in vitro to construct epidermal grafts or promote wound healing, applied in extensive burns, chronic ulcers and skin-defect repair.

Limbal Epithelial Stem Cell Therapy

Limbal epithelial stem cells are expanded in vitro and transplanted to reconstruct the ocular surface epithelium, thereby repairing corneal injury and treating limbal stem cell deficiency (LSCD) and associated corneal blindness.

Source: Literature Review, Frost & Sullivan Analysis

Mesenchymal Cell Therapy

MSCs are isolated from multiple human tissues. After preparation they can differentiate into various mesodermal functional cells and, through immunomodulation and tissue repair, are under clinical investigation for autoimmune, neurological, infectious, metabolic and endocrine diseases. A complete industrial chain has been established, making MSC therapy a widely applied cutting-edge cell-therapy technology.

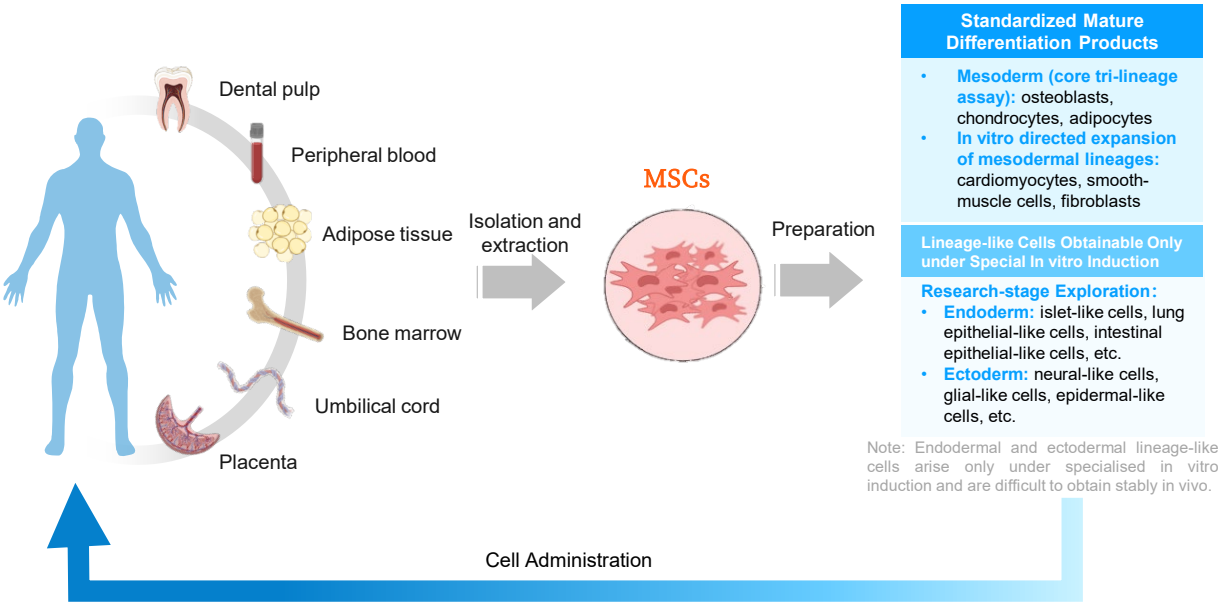
MSCs

MSCs originate from the mesoderm and are multipotent cells residing mainly in connective tissues and the interstitium of certain organs. They are widely distributed in bone marrow, umbilical cord, placenta, adipose tissue and other sites. MSCs possess self-renewal capacity and multilineage differentiation potential, enabling induction into osteoblasts, chondrocytes, adipocytes and other functional cells. They also exert potent immunomodulatory, anti-inflammatory and tissue-repair effects, and rank among the most widely used cell types in current cell therapy and clinical disease research.

MSC Therapy

MSC therapy is a mainstream cell-therapy approach. Relying on immunomodulation, tissue repair, anti-inflammatory activity and paracrine effects, it is under clinical investigation for osteoarthritis, metabolic disorders, neurodegenerative, cardiovascular and autoimmune diseases refractory to conventional treatments. The industry has established a complete value chain covering tissue procurement, GMP-compliant manufacturing, cold-chain logistics, automated cryopreservation, genetic engineering and the development of MSC-derived exosome products. Global trials and regulatory filings continue to advance, positioning MSC therapy as a clinically versatile and translationally leading modality.

Figure. Schematic of MSC Therapy



Source: Literature Review, Frost & Sullivan Analysis

Chapter 2 Regulatory Policy Analysis of Stem Cell Therapy

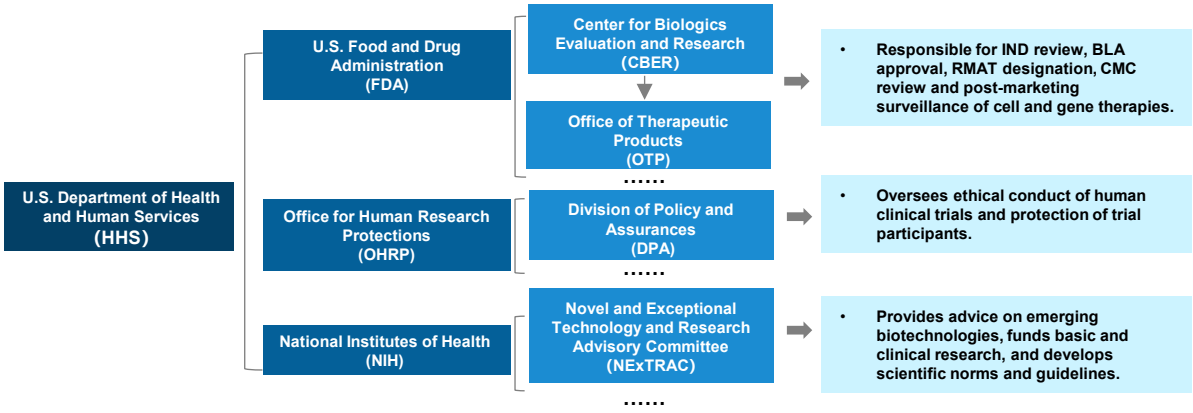
02

U.S. Stem Cell Therapy Regulatory Landscape (1)

U.S. Stem Cell Therapy Regulatory Framework

U.S. stem cell therapy is under the overall coordination of the U.S. Department of Health and Human Services (HHS). The U.S. Food and Drug Administration (FDA) serves as the primary regulatory authority, working in coordination with the Office for Human Research Protections (OHRP) and the National Institutes of Health (NIH). Within the FDA, the Center for Biologics Evaluation and Research (CBER) and its subordinate Office of Therapeutic Products (OTP; formerly the Office of Tissues and Advanced Therapies, OTAT) are responsible for the review of Investigational New Drug (IND) applications, Regenerative Medicine Advanced Therapy (RMAT) designations, and Biologics License Applications (BLA) for stem cell therapies.

Figure. Stem Cell Therapy Regulatory Framework in U.S.



U.S. Stem Cell Therapy Regulatory System

The United States has established a three-tier regulatory system for stem cell therapy comprising statutes, regulations, and guidance documents. At the statutory level, the primary legal bases are Sections 351 and 361 of the Public Health Service Act (PHS Act) and relevant provisions of the Federal Food, Drug, and Cosmetic Act (FD&C Act). At the regulatory level, the key provisions are found in Title 21 of the Code of Federal Regulations (21 CFR), particularly Part 1271 under Subchapter L, which specifically governs Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps).

The Public Health Service Act classifies human cells and tissues into low-risk and high-risk products:

- **Low-risk biological products:** Products that meet all the criteria listed in 21 CFR 1271.10(a) are regulated solely under Section 361 of the PHS Act. These products do not require submission of an IND or BLA and only need to be registered with the FDA.
- **High-risk biological products:** Products that do not meet all the criteria in 21 CFR 1271.10(a) are regulated under Section 351 of the PHS Act and must follow full drug regulatory requirements, including submission of an Investigational New Drug (IND) application and a Biologics License Application (BLA) to the FDA.

Expedited Review Mechanisms for Stem Cell Therapy in the United States

To accelerate the development and marketing of stem cell and regenerative medicine products, FDA's CBER provides a series of expedited programs for eligible products to address the urgent clinical needs of patients with serious diseases:

- **Regenerative Medicine Advanced Therapy (RMAT) Designation:** Established by the FDA under the 21st Century Cures Act specifically for regenerative medicine products such as cell therapies and tissue-engineered products. Products granted RMAT designation benefit from all the early consultation advantages of Breakthrough Therapy and Fast Track designations, enable earlier patient access for serious conditions, and allow more flexible approaches to meeting rigorous clinical evidence requirements before and after full approval.
- **Other standard expedited programs:** In addition to RMAT, stem cell therapies may also qualify for Fast Track, Breakthrough Therapy designation, Priority Review, and Accelerated Approval. These pathways shorten development and review timelines through more frequent FDA interactions, rolling review, priority review clocks, and the use of surrogate or intermediate endpoints.

Source: FDA, Government Official Websites, Frost & Sullivan Analysis

U.S. Stem Cell Therapy Regulatory Landscape (2)

Figure. Examples of the U.S. Stem Cell Therapy Regulatory System

Policy Name	Release Date	Issuing Body	Main Content
Points to Consider (PTC) in Human Somatic Cell and Gene Therapy	1991	FDA	First proposed the directions that should be considered and noted when using gene therapy.
Guidance for Human Somatic Cell Therapy and Gene Therapy	1998	FDA	Updated and replaced the 1991 PTC, aiming to provide manufacturers with the latest regulatory information on production, quality control testing, recombinant vectors for gene therapy, and preclinical testing.
Public Health Service Act (PHS Act)	2001	U.S. Government	Sections 361 and 351 of the Act form the legal foundation for U.S. cell therapy and product regulation: Section 361 applies to minimally manipulated, homologous-use autologous cells and tissue products; Section 351 applies to allogeneic, more-than-minimally manipulated, or non-homologous-use cell and tissue products.
Code of Federal Regulations (CFR), 21 CFR Part 1271	2005	U.S. Government	Detailed provisions on registration, donor eligibility, production process controls and operating standards for human cells, tissues, and cellular and tissue-based products (HCT/Ps); clarifies the definition of HCT/Ps and classifies them into two categories requiring premarket approval or not, based on risk level.
Long Term Follow-Up after Administration of Human Gene Therapy Products	2006	FDA	Strengthened recommendations for long-term health follow-up of participants after marketing, providing design recommendations for long-term follow-up (LTFU) observational studies to collect data on delayed adverse events of gene therapy products.
Content and Review of Chemistry, Manufacturing, and control (CMC) Information for Human Somatic Cell Therapy Investigation New Drug Application (INDs)	2008	FDA	Standardized CMC requirements for IND applications of all somatic cell therapy products (including stem cells), aiming to address the special complexity of the production process of somatic cell therapy products.
NIH Guidelines for Human Stem Cell Research	2009	NIH	Provided policy basis for NIH-funded stem cell research.
Potency Tests for Cellular and Gene Therapy Products	2011	FDA	This guidance provides recommendations on effective potency testing for manufacturers of cellular and gene therapy products to support IND or BLA applications.
Preclinical Assessment of Investigational Cellular and Gene Therapy Products	2013	FDA	Specified issues that need to be considered in preclinical research applicable to all gene therapy products, including preclinical study objectives, overall recommendations on preclinical study design, and selection of animal species for testing.
Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products	2015	FDA	The guidance provided recommendations for OCTGT on the design of clinical trials whose main purpose is the preliminary evaluation of safety, tolerability or feasibility of investigational products.

Source: FDA, Government Official Websites, Frost & Sullivan Analysis

U.S. Stem Cell Therapy Regulatory Landscape (3)

Figure. Examples of the U.S. Stem Cell Therapy Regulatory System

Policy Name	Release Date	Issuing Body	Main Content
21st century cures bill	2016	FDA	Introduced the Regenerative Medicine Advanced Therapy (RMAT) expedited pathway. For regenerative medicine therapies treating serious conditions, if preliminary clinical evidence suggests the potential to address unmet medical needs, RMAT designation may be granted and expedited review applied.
Regulatory Considerations for Human Cells, Tissues, and Cellular and Tissue-Based Products: Minimal Manipulation and Homologous Use	2017	FDA	Provided official interpretation of “minimal manipulation” and “homologous use” in 21 CFR Part 1271, serving as the core basis for determining whether an HCT/P falls under PHS Act Section 361 (low risk, registration only) or Section 351 (high risk, requiring IND/BLA). Applicable to all HCT/Ps, including hematopoietic stem cells, MSCs, cord blood stem cells, etc.
FDA Announcement on Stem Cell Products	2018	FDA	FDA issued warning letters to multiple enterprises selling unapproved stem cell products, clearly emphasising that all stem cell products must go through strict FDA approval procedures to ensure their safety and efficacy.
Expedited Programs for Regenerative Medicine Therapies for Serious Conditions	2019	FDA	Recommendations on accelerating the development of regenerative medicine therapies in the field of serious diseases, clarifying the meaning of regenerative medicine advanced therapy and information on expedited approval.
Studying Multiple Versions of a Cellular or Gene Therapy Product in an Early-Phase Clinical Trial	2022	FDA	Provided guidance on IND applications, information submission and adverse event reporting for sponsors studying multiple versions of a cellular or gene therapy product simultaneously in early-phase clinical trials for a single disease.
Manufacturing Changes and Comparability for Human Cellular and Gene Therapy Products Draft Guidance for Industry	2023	FDA	Provided recommendations on product comparability and manufacturing change management for IND sponsors and BLA applicants of gene therapy products. Provided risk-based approaches to managing manufacturing changes of gene therapy products based on the product lifecycle, as well as comparability studies to evaluate the impact of manufacturing changes on product quality.
Potency Assurance for Cellular and Gene Therapy Products	2023	FDA	FDA’s latest framework guidance on potency assurance for CGT products, upgrading traditional end-product potency testing to a risk-based full-lifecycle management system covering cell design, process control, in-process testing and release testing, applicable to all stem cell products.
Safety Testing of Human Allogeneic Cells Expanded for Use in Cell-Based Medical Products	2024	FDA	FDA’s first specialised guidance on safety testing of ex vivo expanded allogeneic cells, providing comprehensive requirements for risk-based grading of cell expansion potential, establishment of cell banks, exogenous factor testing, genetic stability assessment and tumorigenicity testing, applicable to all allogeneic stem cell products.
Recommendations for Determining Eligibility of Donors of Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps)	2025	FDA	Provided general recommendations for determining donor eligibility of HCT/Ps under the 21 CFR Part 1271 framework, including donor screening, testing and eligibility determination standards.

Source: FDA, Government Official Websites, Frost & Sullivan Analysis

EU Stem Cell Therapy Regulatory Landscape (1)

EU Stem Cell Therapy Regulatory Situation

The Committee for Advanced Therapies (CAT) under the European Medicines Agency (EMA) is the core review body for Advanced Therapy Medicinal Products (ATMPs), including stem cell therapies. It handles scientific recommendations on classification, evaluation of marketing authorisation applications, SME data certification and related guidance. Clinical trial approvals remain the responsibility of individual EU Member State authorities. The EU regulates stem cell therapies as ATMPs under the same standards as medicinal products, based on Directive 2001/83/EC, Regulation (EC) No 1394/2007, Regulation (EC) No 726/2004 and Directive 2009/120/EC. The procedure is clear: CAT prepares a draft opinion, CHMP issues the final opinion, the European Commission adopts the decision, and EMA grants the marketing authorisation, followed by ongoing pharmacovigilance and safety/efficacy monitoring.

EU Expedited Review Mechanisms for Stem Cell Therapy

To enable stem cell therapies for serious diseases to benefit patients as early as possible, the EMA has established multiple expedited approval pathways. The PRIME (Priority Medicines) scheme provides early dialogue and priority review support for stem cell products with outstanding clinical potential, thereby accelerating development and the approval process. Conditional Marketing Authorisation (CMA) allows marketing approval to be granted on the basis of early positive results before comprehensive clinical data are complete, with companies required to submit confirmatory study data subsequently.

Figure. Examples of the EU Stem Cell Therapy Regulatory System

Policy Name	Release Date	Issuing Body	Main Content
Tissues and Cells Directive, EUTCD	2006	EMA	Established a unified EU regulatory system for human tissues and cells. The parent directive was issued in 2004, with two technical directives supplemented in 2006, regulating the donation, procurement, testing, processing, preservation, storage and full-process traceability requirements for stem cells and other cell products, providing a technical basis for Member State legislation.
Regulation (EC) No 1394/2007	2007	EMA	Established a unified regulatory system for Advanced Therapy Medicinal Products (ATMPs) in the EU, fully incorporating gene therapy, cell therapy (including stem cells) and tissue-engineered products into the medicinal products regulatory framework, implementing centralized authorisation procedures with a standard review period of 210 days.
Guideline on Human Cell-based Medicinal Products	2008	EMA	Provided guidance on the development, manufacture and quality control as well as non-clinical and clinical development of cell therapy products, covering both somatic cell therapy medicinal products and tissue-engineered products.
Guideline on safety and efficacy follow-up and risk management of advanced therapy medicinal products	2008	EMA	Provided guidance on pharmacovigilance, risk management planning, safety and efficacy aspects of advanced therapy medicinal products, as well as follow-up of patients treated with such products.
Guidelines on Good Clinical Practice (GCP) specific for Advanced Therapy Medicinal Products (ATMP)	2009	EMA	Detailed the clinical trial design, non-clinical research, quality of investigational ATMPs, safe conduct of clinical trials, cell preparation and administration, drug traceability, retention, protection of participants, safety reporting and monitoring requirements for ATMPs.
Reflection Paper on Stem Cell-Based Medicinal Products	2011	EMA	Covered marketing authorisation applications for products using stem cells as starting material, focusing on explaining the pluripotency, differentiation control, tumorigenicity risks and clinical development requirements of stem cells.
Guideline on the risk-based approach according to annex I, part IV of Directive 2001/83/EC applied to Advanced therapy medicinal products	2013	EMA	Established a full-lifecycle risk classification and supervision system for ATMP products. Based on product risk levels, it sets differentiated clinical trial, CMC and post-marketing supervision requirements, allowing low-risk stem cell products to simplify R&D and approval processes.

Source: EMA, Government Official Websites, Frost & Sullivan Analysis

EU Stem Cell Therapy Regulatory Landscape (2)

Figure. Examples of the EU Stem Cell Therapy Regulatory System

Policy Name	Release Date	Issuing Body	Main Content
PRIME (Priority Medicines) scheme	2016	EMA	Products granted entry into the PRIME scheme receive major EMA support in clinical trials and medicinal product development, accelerating the approval of truly innovative drugs.
Guideline on potency testing of cell based immunotherapy medicinal products for the treatment of cancer	2016	EMA	Updated technical content based on the 3Rs principles relative to the 2007 version guideline, providing specific regulatory requirements for the development and validation of potency assay methods for genuine cell-based immunotherapy products (including hematopoietic stem cell and non-stem cell immune cells).
Guidelines on Good Manufacturing Practice (GMP) specific for Advanced Therapy Medicinal Products (ATMP)	2017	EMA	Adjusted the existing GMP framework specifically for ATMPs, elaborating on the new and complex production situations involved in such products, mainly adopting a risk-based approach to production inspection, allowing manufacturers certain flexibility in process control systems according to the risk level of the product.
Guideline on quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials	2019	EMA	Provided guidance on the structure and data requirements for exploratory and confirmatory clinical trial applications of advanced therapy medicinal products.
Medicinal Products Directive	2023	EMA	Reaffirmed that stem cell products belong to the special medicinal product category of ATMPs and are subject to the unified medicinal products regulatory system together with gene therapy and tissue-engineered products.
Medical Devices Regulation(MDR), (EU)	2023	EMA	Clarified that medical devices containing stem cell components must meet basic safety requirements and may be placed on the EU market only after passing conformity assessment and affixing the CE mark.

Source: EMA, Government Official Websites, Frost & Sullivan Analysis

Japan Stem Cell Therapy Regulatory Landscape (1)

Japan Implements a Dual-Track Regulatory System of Medical Technology and New Drugs for Stem Cell Products

Japan operates a dual-track regulatory system for stem cell products—"regenerative medical technology pathway + new drug pathway"—to balance the speed of innovation with clinical safety. In 2013–2014, Japan enacted the Act on the Promotion of Regenerative Medicine and the Act on the Safety of Regenerative Medicine, establishing the foundational legal framework for the field. Concurrently, the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (PMD Act) was revised to classify stem cell-related products into "regenerative medical technology" and "new drugs/biological products" for registration purposes.

- **Regenerative Medical Technology Pathway:** Supervised by the Ministry of Health, Labour and Welfare (MHLW). Applicable to clinical treatment technologies that primarily use autologous patient cells and are difficult to standardise. Through the "provision plan notification + medical institution certification" fast-track route, clinical application can begin at an early stage.
- **New Drug Pathway:** Supervised by the Pharmaceuticals and Medical Devices Agency (PMDA). Applicable to allogeneic stem cell products that can be standardized and mass-produced. These products undergo clinical trials and marketing authorisation under biological product standards, with accelerated pathways such as conditional and time-limited approval. This system has significantly shortened the timeline from clinical research to market application, positioning Japan as a key global benchmark for regenerative medicine translation.

Figure. Japan's Dual-Track Regulatory System for Stem Cells

Regulatory Authority	Governing Law	Applicable Category	Regulatory Features	Approval Process	Final Approval
Ministry of Health, Labour and Welfare (MHLW)	Act on the Safety of Regenerative Medicine	Registered medical institutions only	Risk-based classification; safety and efficacy assessed	Preclinical → Clinical research → Hospital application	Regenerative medical technology
Pharmaceuticals and Medical Devices Agency (PMDA)	PMD Act	Company applications for manufacture and marketing of regenerative medicine products	Conditional/time-limited marketing authorisation after safety and efficacy assessment	Preclinical → Clinical trial → Marketing approval → Phase 1 listing → Final marketing application	Regenerative medicine product

Stem Cell New Drugs Are Supervised by the Pharmaceuticals and Medical Devices Agency (PMDA)

Stem cell new drugs are supervised by the Pharmaceuticals and Medical Devices Agency (PMDA). Enterprise-developed stem cell products intended for marketing authorisation must undergo PMDA review and follow the registrational clinical trial and marketing authorisation procedures under the PMD Act. The Act uniformly defines cellular therapy products, ex vivo gene therapy products and in vivo gene therapy products as "regenerative medicine products", and introduces a conditional access mechanism on top of the conventional drug approval process. For products demonstrated to be safe and potentially efficacious in small-scale clinical trials, companies may apply for conditional and time-limited marketing authorisation; The target standard review time for regenerative medical products is nine months. Approved products may be commercialised and used to collect clinical data; after full verification of safety and efficacy within 7 years, an application may be submitted for conversion to full marketing authorisation.

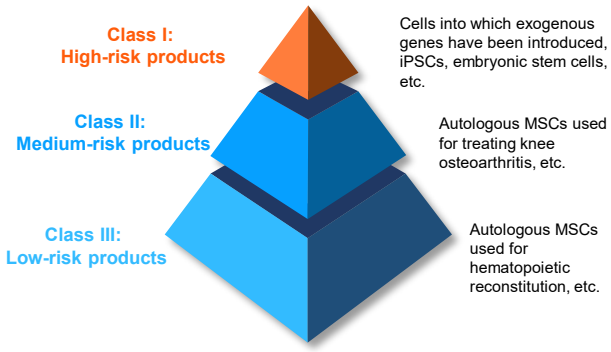
Stem Cell Medical Technologies Are Supervised by the Ministry of Health, Labour and Welfare (MHLW)

Stem cell medical technologies are regulated by the Ministry of Health, Labour and Welfare (MHLW) under the Act on the Safety of Regenerative Medicine. The system primarily covers clinical research and clinical applications outside of drug-registration clinical trials. It applies a three-tier risk classification based on cell source, ex vivo processing methods and clinical scope of use:

- Class I (High risk): Technologies involving iPS cells, etc.
- Class II (Medium risk): Autologous cells used for non-homologous purposes
- Class III (Low risk): Autologous cells used for homologous purposes

Medium- and low-risk projects require only notification of the provision plan. Class I high-risk projects, in addition to notification, must obtain approval from a Certified Committee for Regenerative Medicine and undergo a 90-day special review by the MHLW before they may proceed.

Figure. Three-Tier Risk Classification



Source: Government Official Websites, Frost & Sullivan Analysis

Japan Stem Cell Therapy Regulatory Landscape (2)

■ Japan's Favorable Policies for Stem Cells

Japan has established a full-chain accelerated approval system covering both drug marketing authorisation and medical technology application through multiple world-first institutional innovations, thereby shortening the clinical translation cycle of stem cell therapies:

- **World-first Conditional and Time-Limited Approval System:** Implemented in 2014. Provided that basic safety has been confirmed and a reasonable possibility of efficacy exists, a conditional marketing authorisation of up to 7 years may be granted based solely on small-scale clinical data, with confirmatory data to be supplemented after marketing.
- **SAKIGAKE (Pioneer) Designation:** This designation targets world-first or breakthrough innovative products, offering priority consultation throughout the process and priority review, shortening the review period from the standard 12 months to 6 months.
- **Tiered Fast-Track for Medical Technologies:** Medium- and low-risk stem cell medical technologies may proceed after notification to an institutional Certified Committee for Regenerative Medicine; high-risk technologies may be applied clinically directly after approval by the national Certified Committee for Regenerative Medicine, without undergoing the full drug registration process.

Figure. Examples of Japan's Stem Cell Regulatory System

Policy Name	Release Date	Issuing Body	Main Content
Regenerative Medicine Promotion Law	2013	National Diet of Japan	As a national strategy, it clearly promotes the development of regenerative medicine (including stem cells), establishes a "government-industry-academia-research" collaboration mechanism, sets up a national-level regenerative medicine research fund, and builds a regenerative medical technology certification system.
Act on Pharmaceuticals and Medical Devices (PMD Act)	2014	National Diet of Japan	Newly added the independent regulatory category of "regenerative medicine products," with review conducted by PMDA; introduced the world's first "conditional and time-limited approval system": maximum 7-year conditional marketing authorisation, with confirmatory data to be supplemented after marketing.
Act on the Safety of Regenerative Medicine (ASRM)	2014	National Diet of Japan	Standardises the medical technology pathway: stem cell clinical research and application conducted by medical institutions are regulated under this Act; implements three-tier risk classification management; medium- and low-risk projects require internal review and notification by the medical institution, while high-risk projects require approval by the national Certified Committee for Regenerative Medicine.
SAKIGAKE Designation System	2014	MHLW、PMDA	Targets regenerative medicine products that are world-first or superior in efficacy to existing therapies, providing full-process one-on-one priority consultation + 6-month ultra-fast review (standard 12 months), which can be used in combination with conditional approval.
Guidelines on Manufacturing Practice for Regenerative Medicine Products (GCTP)	2015	MHLW、PMDA	Targeting the personalised, small-batch and short-cycle production characteristics of regenerative medicine products (including stem cell products), it formulates the specialised Standards for Manufacturing Control and Quality Control of Regenerative Medical Products (GCTP), imposing strict quality management and risk control requirements on the entire process of cell collection, processing, culture, storage and transportation. It is a mandatory technical standard for the production quality management of regenerative medicine products.
Act on the Safety of Regenerative Medicine (2024 Amendment)	2024	National Diet of Japan	Expanded the regulatory scope to new technologies such as in vivo gene therapy; strengthened requirements for patient informed consent and information disclosure; increased penalties for violations; established a national regenerative medical technology application database.
Guidelines on Conditional and Time-Limited Approval of Regenerative Medicine Products	2024	MHLW、PMDA	Provides specific operational guidance for regenerative medicine products (including stem cell products) applying for "conditional and time-limited approval," clarifying applicable conditions, application data requirements, post-marketing confirmatory evaluation plans and quality reproducibility improvement plans, aiming to enhance the predictability of the system and support the safe and efficient early marketing of regenerative medicine products.
Special Evaluation Criteria for Conditional Approval of Human Mesenchymal Stem Cell Products	2024	MHLW、PMDA	The world's first specialised technical standard for conditional approval of human MSC products, clarifying the minimum clinical data requirements, efficacy endpoints standards and post-marketing verification requirements for MSC products applying for conditional approval, significantly improving the predictability of MSC product approval.
Pharmaceuticals and Medical Devices Act (2025 Amendment)	2025	National Diet of Japan	Comprehensively programss Japan's innovative medical product approval system, expands the conditional approval system exclusive to regenerative medicine to all innovative drugs, establishes an innovative drug R&D fund, and significantly shortens the time to market.

Source: Government Official Websites, Frost & Sullivan Analysis

China Stem Cell Therapy Regulatory Landscape (1)

Overview of the Development of Stem Cell Therapy Regulation in China

China's stem cell clinical practice began in 1964 (Asia's first bone marrow transplant), yet the early regulatory system remained largely blank for a long period. In 1993, the former Ministry of Health issued the first cell therapy regulatory document, Key Points for Quality Control of Clinical Research on Human Somatic Cell Therapy and Gene Therapy. In 2004, the first stem cell-related registered clinical trial was approved, and the industry gradually entered a phase of exploratory regulation. The year 2015 marked an important watershed: the former National Health and Family Planning Commission and the former China Food and Drug Administration jointly issued the Administrative Measures for Stem Cell Clinical Research (Trial), establishing a non-registered clinical research system of "institution filing + project filing." This ended the disordered development of the industry and formed a dual-track regulatory framework consisting of the drug registration pathway (led by NMPA) and the filing-based clinical research pathway (managed by the National Health Commission). In December 2017, the National Medical Products Administration (NMPA) released the Technical Guidelines for Research and Evaluation of Cell Therapy Products (Trial), formally regulating stem cell products as drugs and incorporating them into the biological products regulatory system, thereby fully launching the pharmaceutical-oriented R&D and application process for stem cell therapy products.

Figure. Development History of Stem Cell Therapy Regulation in China

Initial Regulatory Stage (up to 2004)

Core logic: From "no regulation" to "initial inclusion in the regulatory framework."

- 1993: The former Ministry of Health issued the Key Points for Quality Control of Clinical Research on Human Somatic Cell Therapy and Gene Therapy, bringing stem cell-related somatic cell therapy into drug regulation for the first time.
- 1999: The former State Drug Administration (SDA) issued supporting regulations, incorporating cell therapy products into the new biological products approval and clinical trial regulatory system.
- 2004: China's first stem cell IND application was approved, marking formal entry into the standardized registered clinical trial stage.

Comprehensive Adjustment Stage (2007–2015)

Core logic: From "decentralized management" to "establishment of ethical and technical norms"; 2015 was the watershed.

- 2007: The former Ministry of Health issued the Measures for the Ethical Review of Biomedical Research Involving Human Participants (Trial), establishing ethical review procedures.
- 2009: Stem cell therapy was classified as a Class III medical technology and began to be managed from the medical technology perspective.
- 2015: The former National Health and Family Planning Commission and the former CFDA jointly issued the Administrative Measures for Stem Cell Clinical Research (Trial), establishing the non-registered clinical research system of "institution filing + project filing" and forming the dual-track framework of the drug registration pathway and the filing-based clinical research pathway.

Comprehensive Regulatory Stage (2016–present)

Core logic: Formation of a dual-track regulatory system of "drug registration pathway + filing-based clinical research pathway," promoting industrial standardization and translation.

- 2017: NMPA released the Technical Guidelines for Research and Evaluation of Cell Therapy Products (Trial), formally regulating stem cell products as biological products and launching the pharmaceutical pathway.
- 2020: The new Provisions for Drug Registration was implemented, establishing accelerated pathways such as breakthrough therapy, conditional approval and priority review, providing routes for stem cell product marketing.
- 2023: The National Health Commission issued the Work Guidelines for Somatic Cell Clinical Research (Trial), extending the stem cell NRCR system to all somatic cells and further improving the management norms for non-registered clinical research.
- 2024-2025: Regulation further upgraded toward "dual-track coordination and full-lifecycle risk management." The NHC and NMPA strengthened the linkage between filing-based research and drug registration, encouraged high-quality clinical data interoperability, and issued special guidance for frontier products such as allogeneic stem cells and gene-edited stem cells, promoting parallel innovation and standardization.
- 2026: In May, the Regulations on the Administration of Clinical Research and Clinical Translational Application of New Biomedical Technologies (State Council Order No. 818) officially came into effect, establishing in the form of administrative regulations the systems for clinical research filing and clinical translational application approval by medical institutions for new biomedical technologies. Concurrently, CDE intensively issued supporting technical documents, including the formal Technical Guideline for the Study and Evaluation of CMC Changes to Cell Therapy Products (Trial) and the publicly consulted Technical Guidelines for Pharmaceutical Research and Evaluation of MSC Products. Combined with existing biological product change review requirements, these cover both the medical technology pathway and the cell drug registration pathway, jointly building a full-chain closed-loop regulatory system from source manufacturing and CMC quality control to clinical translation and post-approval changes.

Source: Government Official Websites, Frost & Sullivan Analysis

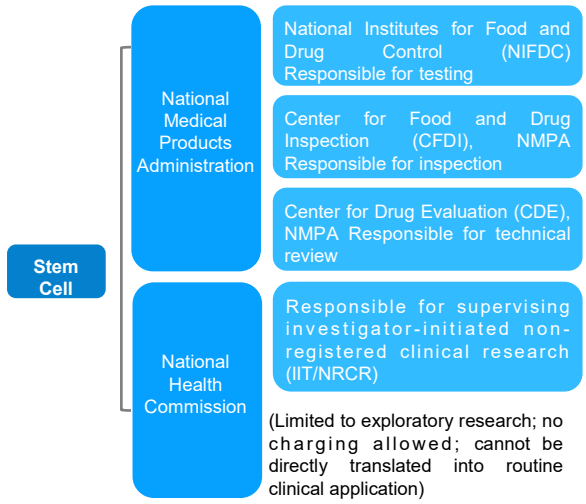
China Stem Cell Therapy Regulatory Landscape (2)

China's Stem Cell Therapy Regulatory System

China implements a dual-track regulatory system of "non-registered clinical research (NRCR) filing + drug registration and marketing authorisation." Routine clinical application of stem cell therapies must obtain drug approval from the NMPA; it is strictly prohibited to directly use filing-based research projects for charged treatment or clinical translation. Regulatory work is divided between the National Medical Products Administration (NMPA) and the National Health Commission (NHC):

- **National Medical Products Administration (NMPA):** Responsible for the full-lifecycle drug regulation of stem cell therapy products, including IND and BLA review, evaluation of drug safety and efficacy, GMP production quality management, post-marketing surveillance and re-evaluation, ensuring that products are developed and marketed according to drug standards.
- **National Health Commission (NHC):** Responsible for supervising investigator-initiated non-registered clinical research (IIT/NRCR), implementing the institution filing and project filing system, formulating norms for clinical research ethics, quality control and participants protection, and clearly requiring that such research shall not charge participants fees nor be directly conducted as routine medical services.

Figure. China's Stem Cell Therapy Regulatory System



China's Three Regulatory Pathways for Stem Cells

Hematopoietic Stem Cell Transplantation

Managed as a restricted medical technology, based on documents such as the Management Specifications for Hematopoietic Stem Cell Transplantation Technology (2017 Edition) issued by the General Office of the former National Health and Family Planning Commission in 2017.

Other Stem Cells

Other stem cells follow two pathways:

- One is the technology pathway, applicable under the Regulations on the Administration of Clinical Research and Clinical Translational Application of New Biomedical Technologies (State Council Order No. 818, effective from 1 May 2026);
- The other is the drug pathway, managed by the NMPA through IND/BLA review.

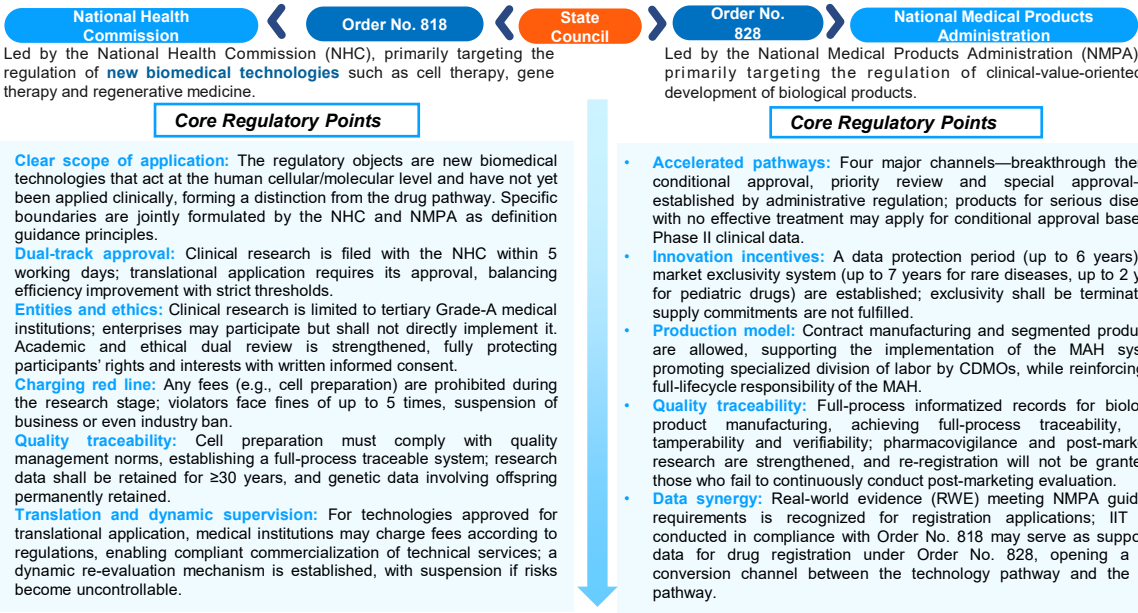
Source: Government Official Websites, Frost & Sullivan Analysis

China Stem Cell Therapy Regulatory Landscape (3)

■ Favorable Policies and Milestones for Stem Cell Therapy in China

China has continuously issued multiple targeted favorable policies to accelerate the progression of stem cell therapy from early research to clinical translation and industrial commercialization. Key milestones and open policies are as follows:

- **Milestone Event:** In January 2025, the first domestically developed human umbilical cord MSC injection received conditional marketing approval from the NMPA. This is China's first officially approved stem cell therapy product.
- **Open Policy:** In September 2024, relevant Chinese authorities jointly issued a policy allowing foreign-invested enterprises to develop and apply human stem cell and gene diagnosis and treatment technologies in the pilot free trade zones in Beijing, Shanghai, Guangdong, and Hainan Free Trade Port, thereby attracting global innovation resources and accelerating industry internationalization.
- **Decree No. 818:** In September 2025, the State Council promulgated *Regulations on Administration of Clinical Research and Clinical Translational Application of New Biomedical Technologies (Decree No. 818)*. Effective May 1, 2026 and primarily overseen by NHC, the Regulations govern non-standardized biomedical technologies through stringent access, ethical oversight and clearly defined clinical translation pathways.
- **Decree No. 828:** In January 2026, the State Council promulgated the revised *Regulations on the Implementation of the Drug Administration Law of the People's Republic of China (Decree No. 828)*. Effective May 15, 2026 and administered by NMPA, it governs standardized, scalable cell therapy products and emphasizes accelerated approval, pharmaceutical innovation, regulated contract manufacturing and stringent quality control.



Short-term Impact

The industry will experience compliance growing pains. Small and medium-sized institutions and those relying on grey-area business will be rapidly cleared out due to insufficient qualifications, costs and technical thresholds. Leading enterprises with advantages in compliance systems, clinical resources and data quality will increase market concentration, making standardization the baseline for survival.

Long-term Impact

The "dual-track" regulatory system constructed by Order No. 818 and Order No. 828 is driving a profound long-term paradigm shift in China's cell and gene therapy industry. On the R&D side, the shift from "laboratory-oriented" to dual-driven by "clinical value and data quality" makes the technology-to-drug pathway conversion of RWE and IIT data the new normal. On the production side, upgrades toward intelligent manufacturing, GMP standardization and specialized CDMO division of labor will raise compliance costs in the short term, but will achieve an optimal balance of cost and safety through scale in the long term.

In terms of industrial positioning, domestic standards are accelerating alignment with international ones, and leading enterprises have begun "going global" exploration. On the payment side, innovative products leverage commercial health insurance, such as Huiminbao, and other diversified payment channels to improve accessibility, yet inclusion in basic medical insurance still faces the dual bottlenecks of pricing and efficacy evaluation. On the regulatory front, closer coordination between the NHC-led technology pathway and the NMPA-led drug pathway will make the transition from technology-based clinical application to drug development an increasingly standard strategic option for companies.

For pilot regions, Order No. 818 and Order No. 828 have unified national regulatory standards, compressing the special policy space of pilot regions, whose development focus shifts from policy differentiation to comprehensive competition in compliance capability and industrial ecosystem.

Source: Government Official Websites, Frost & Sullivan Analysis

China's Policies Related to Stem Cell Therapies (1)

Figure. Examples of China's Stem Cell-Related Policies

	Related Content	Policy & Release Time	Issuing Body
Favorable Policies	Clearly proposed strengthening the development of new technologies and equipment such as gene therapy and cell therapy	Bio-industry Development Plan (2013)	State Council
	Listed "stem cells and regenerative medicine" as a major science and technology special project, elevating it to a national strategy	"Healthy China 2030" Planning Outline (2016)	State Council
	Proposed focusing on the development of gene therapy drugs, stem cell and immune cell therapy products	Guide for Pharmaceutical Industry Development Planning (2016)	MOST
	Clarified the pharmaceutical registration pathway for stem cell products and formally incorporated them into the regulatory scope of therapeutic biological products	Technical Guidelines for Research and Evaluation of Cell Therapy Products (Trial) (2017)	Former CFDA
	Established three major accelerated pathways: breakthrough therapy drugs, conditional approval and priority review	Provisions for Drug Registration (2020)	NMPA
	Focused on developing CAR-T, CAR-NK and other immune cell therapies, stem cell therapies, gene therapy products and specific immunoglobulins targeting new targets and new indications	"14th Five-Year" Plan for the Development of the Pharmaceutical Industry(2021)	MOST, NDRC and 9 departments
	Focused on accelerating innovative drug marketing approval and strengthening post-marketing supervision, building drug regulatory science research bases and quality & safety evaluation technology platforms for gene therapy products	"14th Five-Year" Plan for Bioeconomic Development (2021)	NDRC
	Allowed foreign-invested enterprises to conduct human stem cell technology development and application in the free trade zones of Beijing, Shanghai, Guangdong and Hainan	Notice on Carrying out Pilot Work of Expanding Opening-up in the Medical Field (2024)	MOFCOM, NHC, NMPA
	Optimized the overall review and approval mechanism for cell and gene therapy drugs, simplified communication procedures for stem cell new drugs, and shortened IND acceptance and clinical inspection cycles	Matters Concerning Optimising the Review and Approval of Cell and Gene Therapy Drugs (Draft for Comments) (2026)	NMPA

Source: Government Official Websites, Frost & Sullivan Analysis

China's Policies Related to Stem Cell Therapies (2)

Figure. Examples of China's Stem Cell-Related Policies

	Related Content	Policy & Release Time	Issuing Body
Regulatory Policies	Established the "institution + project" dual filing system for stem cell clinical research, no longer managed as Class III medical technology	Administrative Measures for Stem Cell Clinical Research (Trial) (2015)	Former NHFPC, Former CFDA
	Clarified quality control requirements for stem cell preparations and preclinical research evaluation methods	Guiding Principles for Quality Control and Preclinical Research of Stem Cell Preparations (Trial) (2015)	Former NHFPC, Former CFDA
	Proposed special considerations and requirements for non-clinical research and evaluation of gene-modified cell therapy products	Technical Guidelines for Non-clinical Research of Gene-Modified Cell Therapy Products (Trial) (2021)	CDE
	Unified release testing standards for stem cell preparations to reduce the risk of clinical adverse reactions	Specifications for Release Testing of Stem Cell Preparations (Trial) (2021)	The National Stem Cell Translational Resource Center (NSCTRC)
	Provided recommendations on data preparation for communication and considerations of clinical R&D elements during the clinical development of cell and gene therapy products	Technical Guidelines for Clinical-Related Communication of Cell and Gene Therapy Products (Draft for Comments) (2023)	CDE
	The first dedicated pharmaceutical research guideline for stem cells, standardising the full R&D process of stem cell products	Technical Guidelines for Pharmaceutical Research and Evaluation of Human Stem Cell Products (Trial) (2023)	CDE
	The first dedicated clinical trial guideline for stem cells, supporting the use of IIT data for drug registration	Technical Guidelines for Clinical Trials of Human Stem Cell and Derived Cell Therapy Products (Trial) (2023)	CDE
	The first dedicated non-clinical research guideline for stem cells, clarifying safety evaluation requirements	Technical Guidelines for Non-clinical Research of Human Stem Cell Products (2024)	CDE
	Clarified core rules for clinical research filing, translational approval and strict prohibition of charging for stem cells and other frontier technologies; one of the current top-level regulatory regulations	Regulation on the Administration of Clinical Research on and Clinical Translational Application of New Biomedical Technologies (Order No. 818 of the State Council) (2025)	State Council
	Strengthened accelerated approval pathways for stem cells and other innovative drugs, providing important legal guarantee for the pharmaceutical pathway	Regulations for the Implementation of the Drug Administration Law of the People's Republic of China (Order No. 828 of the State Council) (2026)	State Council
	Unified the standards for declaration and evaluation of changes in production process, cell banks and excipients of stem cell preparations, standardising post-marketing change management	Technical Guidelines for Pharmaceutical Change Research and Evaluation of Cell Therapy Drugs (Trial) (2026)	CDE
	Clarified the official definition of MSCs and the full set of pharmaceutical research requirements covering donor, banking, expansion, quality control and stability	Technical Guidelines for Pharmaceutical Research and Evaluation of Mesenchymal Stem Cell Products (Draft for Comments) (2026)	CDE
	Clarified the scope and classification of cell therapy drugs in China without affecting the current drug registration classification	Technical Guidelines on the Scope and Classification of Cell Therapy Drugs (2026 Edition) (2026)	CDE
	National health industry standard, unifying the full set of operating specifications for collection, separation, preparation, cryopreservation and quality testing of cord blood hematopoietic stem cells	Standard for Preparation and Cryopreservation of Cord Blood Hematopoietic Stem Cells WS/T 895—2026 (issued April 2026, effective 1 September 2026)	National Health Commission

Source: Government Official Websites, Frost & Sullivan Analysis

Chapter 3

Induced Pluripotent Stem Cell Technology Analysis

03

Overview of Induced Pluripotent Stem Cell Therapies (1)

iPSC therapy combines somatic cell reprogramming, pluripotent cell expansion, directed differentiation, and tissue replacement or immune remodeling. It shifts disease treatment from symptomatic relief toward cellular-level repair of underlying pathology.

Definition of iPSCs

Induced pluripotent stem cells (iPSCs) are generated by reprogramming somatic cells, such as skin fibroblasts and peripheral blood mononuclear cells (PBMCs), using integrating viral vectors, such as retroviral and lentiviral vectors, or non-integrating approaches based on episomal plasmids or messenger RNA (mRNA). Reprogramming reset's cellular identity and generates cells with pluripotency comparable to that of embryonic stem cells. Under appropriate culture conditions, iPSCs can self-renew extensively and generate derivatives of all three germ layers. Integration-free reprogramming, including mRNA-based delivery, has reduced integration-related risks, while CRISPR-mediated genome editing and organoid technologies have expanded the applications of iPSC technology. iPSCs are widely used in disease modeling and drug screening, while their regenerative medicine applications continue to advance through preclinical and clinical development.

Key challenges include tumorigenic risks from residual undifferentiated cells, genomic instability arising during reprogramming or prolonged culture, potential immunogenicity of autologous products, and immune rejection of allogeneic products. Other limitations include variable differentiation efficiency, incomplete cellular maturation, high manufacturing costs, lengthy production timelines, and residual epigenetic memory. These challenges continue to constrain large-scale clinical translation. Nevertheless, iPSCs remain an important platform for stem cell research and clinical development.

Figure. Biological Characteristics of iPSCs

Self-renewal Capacity

- Under appropriate culture conditions, iPSCs can overcome Hayflick-associated replicative senescence and undergo sustained expansion while maintaining an undifferentiated state and pluripotency through serial passaging.
- This property provides a renewable and scalable cell source for disease modeling, drug screening, toxicological testing, and cell-based therapies, helping alleviate supply constraints in clinical translation.

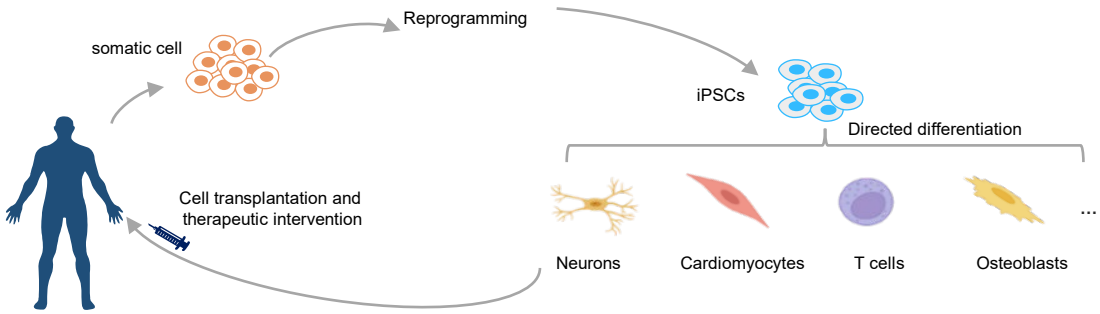
Pluripotency

- Under defined lineage-specific cues, iPSCs can differentiate into most somatic cell types derived from the three embryonic germ layers, including cardiomyocytes and endothelial cells (mesoderm), neurons and epidermal cells (ectoderm), and hepatocytes and pancreatic islet cells (endoderm).
- Like embryonic stem cells, iPSCs are pluripotent rather than totipotent. They cannot form a complete organism and do not normally generate extraembryonic tissues such as the placenta. This property supports the generation of functional cells for tissue repair and replacement, developmental research, and disease modeling.

iPSC Cell Therapy

iPSC cell therapy is an emerging therapeutic strategy rooted in regenerative medicine and cell engineering. It involves reprogramming somatic cells obtained from the patient (autologous) or a healthy donor (allogeneic) into iPSCs, directing their differentiation into the desired functional cell types, and administering or transplanting the resulting iPSC-derived cells into the patient to promote tissue repair, restore function, or enable immunotherapy.

Figure. Schematic Overview of iPSC Therapies



Source: Literature Review, Frost & Sullivan Analysis

Overview of Induced Pluripotent Stem Cell Therapies (2)

iPSC therapy integrates somatic cell reprogramming, expansion in the pluripotent state, directed differentiation, and tissue replacement and/or immune modulation. This regenerative strategy shifts treatment from symptom management toward addressing underlying disease processes through cellular-level repair.

■ Clinical Development of iPSC Therapies

Clinical research on iPSC therapies has made progress across multiple fields. Programs targeting Parkinson's disease, heart failure, corneal diseases and diabetes have established a solid clinical foundation, with some advancing to late-stage trials and demonstrating favorable safety and preliminary efficacy. Meanwhile, iPSC-derived immune cell therapies, organoid transplantation and treatments for autoimmune diseases remain largely at early clinical translation or experimental stages, with research focused on differentiation efficiency, scalable manufacturing and immune modulation.

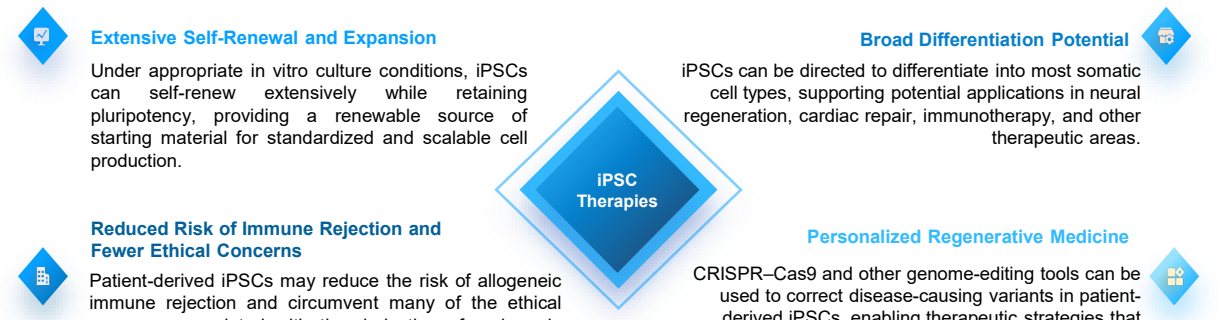
Figure. Overview of Clinical Research on iPSC Therapies

Neurological Disorders	• Patient-specific iPSC-derived midbrain dopaminergic progenitor cells were transplanted to treat Parkinson's disease. At 18–24 months after transplantation, clinical measures of Parkinsonian symptoms remained stable or improved.
Corneal Disorders	• iPSC-derived corneal epithelial cell sheets (iCEPS) were transplanted to reconstruct the damaged ocular surface, demonstrating a favorable safety profile in the treatment of limbal stem cell deficiency (LSCD).
Cardiovascular Diseases	• Allogeneic iPSC-derived cardiomyocyte sheets for ischemic cardiomyopathy have demonstrated safety in clinical trials and shown potential for the treatment of heart failure.
Diabetes	• Following transplantation of islet tissue differentiated from autologous endoderm stem cells, the patient achieved markedly improved glycemic control, providing preliminary evidence that stem cell-derived islet tissue may restore islet function in patients with late-stage type 2 diabetes.

■ Advantages of iPSC Therapies

iPSCs combine broad differentiation potential with long-term self-renewal, enabling extensive in vitro expansion and generation of functional cells for neurodegenerative diseases, corneal disorders, cardiovascular diseases, and metabolic disorders. As they do not require embryo-derived material, they reduce ethical concerns associated with embryonic stem cells. Autologous sourcing and hypoimmunogenic engineering may lessen immune rejection. Combined with genome editing, 3D organoid technologies, and precision medicine, iPSCs support personalized regenerative medicine and disease modeling. Key challenges remain genomic stability, tumorigenicity, manufacturing standardization, and cost.

Figure. Advantages of iPSC Therapies



Source: Literature Review, Frost & Sullivan Analysis

Analysis of Technological Advances in iPSC Therapies

Safer, scalable iPSC production uses non-integrating, mRNA, and small-molecule reprogramming to reduce genomic integration and exogenous residues. Improved differentiation, purification, 3D culture, and organoids enhance cell quality, supporting the shift from costly autologous therapies to off-the-shelf allogeneic products.

iPSC Reprogramming Methods

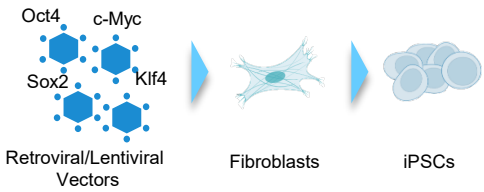
iPSC reprogramming methods are classified as integrating or non-integrating based on whether reprogramming sequences are stably integrated into the host genome. Integrating methods use retroviral or lentiviral vectors but may cause insertional mutagenesis and increase tumorigenic risk. Non-integrating methods—including episomal plasmids, Sendai virus vectors, mRNA, miRNA, recombinant proteins, and small molecules—avoid permanent genomic integration and are better suited to clinical translation. These platforms differ in efficiency, preparation, delivery, cost, and safety, with integration-free approaches generally preferred for clinical-grade iPSC generation.

Figure. Classification of iPSC Reprogramming Methods

First Generation: From Zero to One—Integrating Viral Reprogramming

Retroviral or lentiviral vectors integrate transgenes encoding OSKM factors (OCT4, SOX2, KLF4, and c-MYC) to reprogram somatic cells. In 2006, Takahashi and Yamanaka used retroviral vectors to generate mouse iPSCs, demonstrating pluripotency induction without embryo use. However, random integration may disrupt genes, activate oncogenes, or reactivate transgenes, raising tumorigenicity concerns. These methods remain primarily research tools, while clinical development favors non-integrating approaches and stringent vector safety assessment.

Figure. Integrating Viral Vector–Based Reprogramming



Second-Generation Approaches: Transitional Strategies Toward Improved Safety

To reduce integration-related risks, transitional methods adopted transient expression or excisable integration.

Non-Integrating Viral Reprogramming: Sendai virus or adenoviral vectors transiently deliver RNA or DNA without stable genomic integration. Sendai clearance may require prolonged passaging, and vector production can be costly. Integrating Non-Viral

Reprogramming: PiggyBac or loxP-based systems integrate reprogramming transgenes, which are removed after iPSC generation using transposase or Cre recombinase, respectively.

Third-Generation Approaches for Clinical Translation: Non-Viral, Non-Integrating, and Chemical Reprogramming

Non-viral, non-integrating methods use episomal plasmids, in vitro-transcribed mRNA, or recombinant proteins without permanent genomic integration. Episomal plasmids are lost during passaging, while mRNA and proteins are transient, supporting their use in IND-stage iPSC pipelines.

Chemical reprogramming uses defined small-molecule cocktails to generate CiPSCs without exogenous genes, avoiding integration-related risks and potentially supporting standardized GMP manufacturing. Hongkui Deng's team established fully chemical reprogramming of human somatic cells. From 2022 to 2025, the approach progressed to generating hCiPSCs in as few as 10 days, with more than 20-fold higher efficiency under the reported conditions. It represents a promising route toward scalable cell manufacturing.

Source: Literature Review, Frost & Sullivan Analysis

Overview of Marketed and Investigational iPSC Therapies

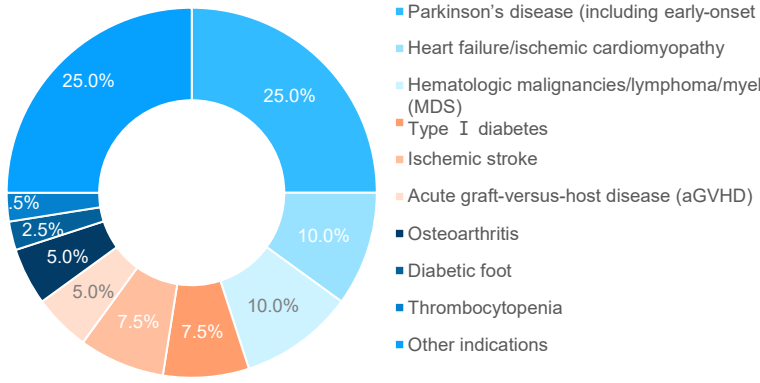
Conditionally Approved iPSC-Based Products

In March 2026, Japan granted conditional, time-limited marketing approval to AMCHEPRY® and RiHEART®, the first two iPSC-derived regenerative medicine products. AMCHEPRY (Sumitomo Pharma/RACTHERA) uses allogeneic dopaminergic neural progenitors to improve motor symptoms in Parkinson's disease. RiHEART® (Cuorips) comprises allogeneic iPSC-derived cardiomyocyte sheets applied to the heart surface, with the aim of improving cardiac function and symptoms in patients with severe heart failure caused by ischemic cardiomyopathy. Both approvals require post-marketing confirmation of safety and efficacy.

Investigational iPSC Therapy Pipeline by Indication

According to publicly available data, clinical-stage iPSC-based products worldwide and in China primarily target Parkinson's disease, heart failure, malignancies, type 1 diabetes, stroke, and bone and joint disorders. Parkinson's disease accounts for up to 25%, making it the most active development area. Cardiovascular diseases and hematologic malignancies also form distinct pipeline clusters. The range of indications is expected to expand as research progresses.

Figure. Distribution of Global Investigational iPSC-Based Products by Indication

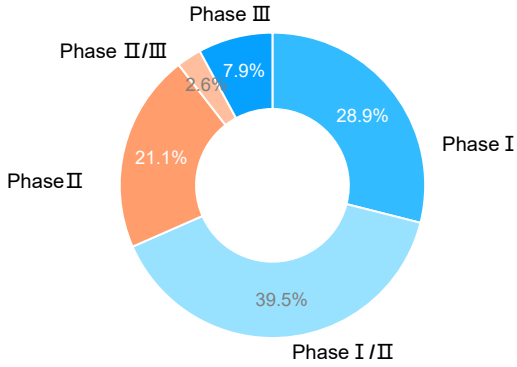


Note: The data include iPSC-based products that had entered clinical trials as of May 2026.

Investigational iPSC Therapy Pipeline by Clinical Phase

According to publicly available data, Phase I/II programs account for the largest share (39.5%) of investigational iPSC-based products in clinical trials worldwide, including China, followed by Phase I (28.9%) and Phase II (21.1%). Phase III and Phase II/III programs account for 7.9% and 2.6%, respectively. All trials included are industry-sponsored registrational studies ultimately intended to support New Drug Application (NDA) or Biologics License Application (BLA) submissions. Overall, current iPSC-based therapy development remains concentrated in early- to mid-stage clinical development.

Figure. Distribution of Global Investigational iPSC-Based Products by Clinical Phase



Note: The data include iPSC-based products that had entered clinical trials as of May 2026.

Source: Literature Review, Frost & Sullivan Analysis, ClinicalTrials, CDE

Chapter 4 Embryonic Stem Cell Technology Analysis

04

Overview of Embryonic Stem Cell Therapies (1)

ESCs are embryo-derived pluripotent stem cells obtained from the inner cell mass of preimplantation blastocysts. They are characterized by long-term self-renewal, differentiation into all three germ layers, and distinctive morphological and epigenetic features.

Definition of ESCs

Embryonic stem cells (ESCs) are isolated from the inner cell mass of preimplantation blastocysts and stably maintained in vitro. Since mouse ESCs were established in 1981 and human ESCs (hESCs) were first derived in 1998, they have become essential cellular platforms for studying early embryogenesis, disease modeling, and regenerative medicine. Their defining properties include self-renewal, pluripotency, distinct molecular and epigenetic profiles, and characteristic cell-cycle and metabolic programs. ESCs exist in two functionally distinct pluripotent states: naïve and primed.

Figure. Biological Characteristics of ESCs

ESCs

01 **Long-Term Self-Renewal**

- Unlike pluripotent epiblast cells, which exist only transiently during embryogenesis in vivo, embryonic stem cells (ESCs) can undergo indefinite self-renewal under appropriate in vitro culture conditions, with minimal replicative senescence, while retaining a normal diploid karyotype after prolonged passaging. This capacity is governed by a core pluripotency transcriptional circuitry comprising OCT4, SOX2 and NANOG. In mouse ESCs, the JAK/STAT3, Wnt and PI3K/AKT pathways act together to suppress differentiation, whereas human primed ESCs rely primarily on Wnt and PI3K/AKT signaling to regulate pluripotency. Wnt signaling exerts context-dependent effects: lower activity supports pluripotency, while higher activity promotes differentiation. Together, these pathways cooperate with Polycomb repressive complexes PRC1 and PRC2 to silence germ-layer lineage promoters through histone modifications, thereby maintaining the undifferentiated state.

02 **Multilineage Differentiation Potential**

- Classical functional assays include embryoid body formation in vitro and teratoma formation in immunodeficient mice. ESCs can generate derivatives of the ectoderm, mesoderm, and endoderm. Naïve mouse ESCs contribute to germline chimeras, whereas primed hESCs resemble the postimplantation epiblast and generally lack blastocyst integration and germline contribution. Experimentally induced naïve human pluripotent stem cells have shown limited chimeric potential in experimental systems. Transitions between pluripotent states are regulated partly by FGF/ERK signaling.

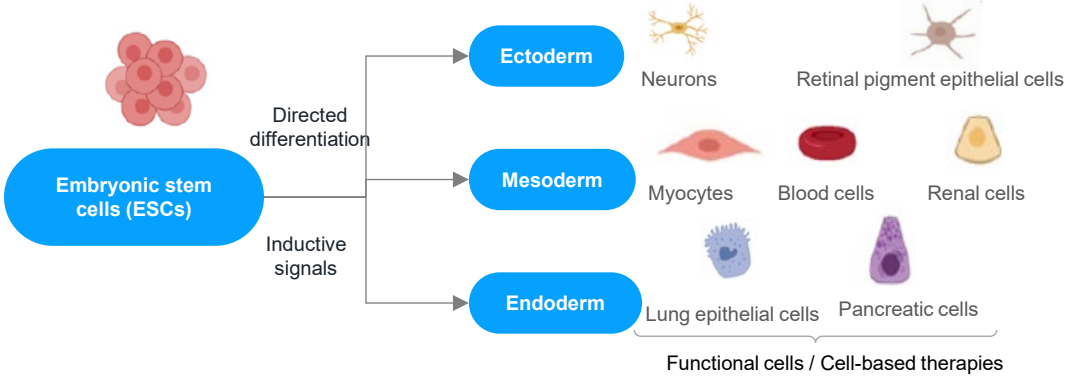
03 **Distinctive Morphology, Surface Markers, and Epigenetic Features**

- ESCs show a high nuclear-to-cytoplasmic ratio and compact colony morphology. Mouse ESCs typically express SSEA-1, whereas primed hESCs typically express SSEA-3, SSEA-4, TRA-1-60, and TRA-1-81. Many developmental genes carry bivalent H3K4me3/H3K27me3 marks and remain transcriptionally silent but poised for activation. Selective resolution of these marks during differentiation enables lineage-specific gene activation or repression. Global DNA hypomethylation is more characteristic of the naïve state.

ESC Therapies

ESC therapies generate functional cells, including cardiomyocytes, neural cells, and pancreatic islet cells, through directed differentiation in vitro. These cells are transplanted to replace or repair damaged tissues in degenerative diseases, organ injuries, and other conditions with limited treatment options.

Figure. Schematic of ESC Therapies



Source: Literature Review, Frost & Sullivan Analysis

Overview of Embryonic Stem Cell Therapies (2)

ESC therapies offer trilineage differentiation, batch consistency, an established clinical evidence base, and scalable supply. Advances in serum-free manufacturing, strategies to mitigate allogeneic immune rejection, cell purification, and quality control are reducing transplantation costs, rejection, and tumorigenicity from residual undifferentiated cells.

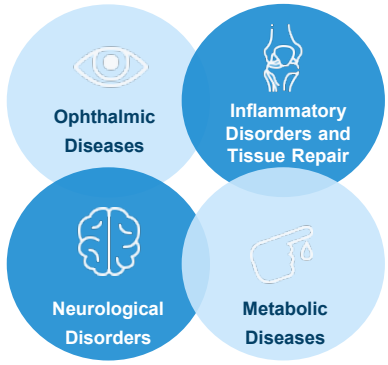
Clinical Research on ESC Therapies

ESC therapies have generated clinical data in ophthalmology, neurodegenerative diseases, inflammatory tissue repair and orthopedics, with standardized allogeneic off-the-shelf products demonstrating consistent safety and regenerative effects. hESC-derived cardiomyocyte, midbrain dopaminergic neuron and corneal endothelial cell programs remain at the preclinical or investigator-initiated trial stage, with optimization focused on scalable serum-free manufacturing, immune management and cell purity.

hESC research and clinical translation are subject to strict ethical and regulatory oversight. In many jurisdictions, hESC lines may be derived from donated surplus IVF blastocysts, while creating embryos solely for stem cell production is prohibited or restricted. Undifferentiated hESCs cannot be directly transplanted; only differentiated, purified and quality-controlled cells may enter authorized clinical studies.

Figure. Schematic Overview of Clinical Research on ESC Therapies

- Retinal pigment epithelial cells and corneal endothelial cells can be generated from hESCs through directed differentiation and are being investigated for dry age-related macular degeneration and corneal endothelial decompensation, respectively.
- Midbrain dopaminergic progenitor cells derived from human embryonic stem cells have been evaluated in several early-stage clinical trials overseas as a cell replacement therapy for Parkinson's disease.



- Immunomodulatory MSCs derived from hESCs remain under preclinical and early clinical evaluation. Intravenous or local administration is being explored for systemic hyperinflammatory conditions, organ injury, and cartilage degeneration.
- hESC-derived pancreatic islet β-cell products for type 1 diabetes are currently being evaluated primarily in large-animal efficacy studies. Cell maturation and graft immune rejection remain major technical challenges.

Analysis of the Advantages of ESC Therapies

ESC therapies offer four main advantages. First, broad pluripotency enables directed differentiation into functional derivatives of all three germ layers, supporting diverse indications. Second, hESC seed stocks maintained within defined passage limits provide stable properties and batch-to-batch consistency. Serum-free, xeno-free banking, scalable differentiation, and stringent control of residual undifferentiated cells enable allogeneic off-the-shelf products and reduce donor-related variability. Third, ESC therapies have a longer clinical development history, with accumulating evidence of safety and tissue-repair potential and increasingly standardized manufacturing, quality control, and administration. Fourth, a single clinical-grade hESC line can generate large numbers of allogeneic doses, shortening treatment waiting times, potentially reducing manufacturing and delivery costs, and supporting commercial-scale development.

Figure. Advantages of ESC Therapies

Intrinsic Pluripotent Differentiation Capacity	High Batch-to-Batch Consistency	Established Evidence Base	Scalable Manufacturing and Supply
ESCs can generate functional derivatives of all three germ layers, supporting applications in degenerative eye diseases, neurological injuries, inflammatory and organ injuries, cardiovascular diseases, and metabolic disorders. Their differentiation breadth exceeds that of tissue-specific adult stem cells.	ESCs support long-term expansion and self-renewal. GMP-compliant cell banks standardize manufacturing, minimize donor-related variability, and ensure consistent potency and batch quality for allogeneic off-the-shelf products.	ESC-derived therapies were among the earliest pluripotent stem cell-based therapies to enter human clinical trials. Phase I/II studies in Europe, the United States, and China have provided follow-up data in dry age-related macular degeneration, Parkinson's disease, and acute myocardial injury. Quality-control, ethical-review, and clinical procedures continue to evolve.	A limited number of donated blastocysts can establish clinical-grade ESC banks for long-term use within defined passage limits. These banks support large-scale standardized production, reducing costs and lead times compared with patient-specific autologous therapies.

Source: Literature Review, Frost & Sullivan Analysis

Evolution of ESC Culture Technologies and Overview of Clinical Pipelines in China and the United States

ESC culture has evolved through four generations into scalable, animal-origin-free GMP platforms. The United States has multiple clinical-stage hESC pipelines, while China's CAStem Cell Injection has completed Phase II trials.

In Vitro ESC Line Derivation and Scalable Culture Systems

ESC culture has evolved through four generations: mouse feeders and fetal bovine serum with xenogeneic risks; human feeders and serum replacements; feeder-free recombinant extracellular matrices maintaining pluripotency; and synthetic, chemically defined matrices with bioreactor suspension expansion. The latest platform enables xeno-free, GMP-compliant clinical-scale production with improved batch consistency and scalable supply.

First Generation

Early ESC derivation used inactivated mouse embryonic fibroblast (MEF) feeders and fetal bovine serum (FBS). Although feeder-secreted factors maintained pluripotency, xenogeneic proteins and adventitious-agent risks made these systems unsuitable for clinical manufacturing.

Second Generation

Human fibroblast feeders and serum replacements reduced xenogeneic contamination. However, feeder dependence caused batch variability and residual-cell contamination, while the incompletely defined system remained unsuitable for GMP manufacturing.

Third Generation

Early feeder-free systems used animal-derived Matrigel with serum-free defined media. Xenogeneic risks and batch variability persisted, limiting their suitability for GMP manufacturing.

Fourth Generation

Xeno-free recombinant human matrices, such as vitronectin and laminin, or synthetic peptide matrices are combined with defined media and microcarrier bioreactor expansion. These systems reduce xenogeneic risks, improve batch consistency, and enable scalable, GMP-compliant hESC manufacturing.

U.S. Investigational ESC Therapy Pipeline

The U.S. ESC clinical pipeline spans neurological, ophthalmic, and metabolic disorders, leveraging the broad differentiation potential of hESCs to target Parkinson's disease, epilepsy, macular degeneration, and type 1 diabetes. Clinical trials for these indications were first posted between 2015 and 2025.

Product	Company	Indication	Clinical Phase	First Posted Date
Bemdaneprocel	BlueRock Therapeutics	Parkinson's disease	Ⅲ	2025/04
VX-880	Vertex Pharmaceuticals, Semma Therapeutics, ViaCyte	Type 1 diabetes	Pivotal Study	2024/11
VX-264	Vertex Pharmaceuticals, Semma Therapeutics	Type 1 diabetes	Ⅰ/Ⅱ	2023/03
OpRegen	Lineage Cell Therapeutics, CellCure Neurosciences, Roche	Geographic atrophy secondary to age-related macular degeneration	Ⅱ	2022/11
NRTX-1001	Neurona Therapeutics	Drug-resistant mesial temporal lobe epilepsy	Ⅰ/Ⅱ	2021/11
ASP7317	Astellas Pharma, CHA Biotech	Geographic atrophy secondary to age-related macular degeneration	Ⅰ/Ⅱ	2017/05
PEC-Direct	ViaCyte	Type 1 diabetes	Ⅰ/Ⅱ	2017/05
CPCB-RPE1	Regenerative Patch Technologies	Dry age-related macular degeneration	Ⅰ/Ⅱ	2015/10

Investigational ESC Therapy Pipeline in China

Zephyrm Bioscience's hESC-derived CAStem Cell Injection is China's only candidate in Phase II trials, targeting inflammatory conditions and meniscal injury. Other hESC-derived products remain in preclinical development or investigator-initiated studies.

Product	Company	Indication	Clinical Phase	First Posted Date
ZH901	Zephyrm Bioscience	Meniscal injury	Ⅰ/Ⅱ	2022/02
ZH901	Zephyrm Bioscience	ARDS	Ⅱ	2022/06
ZH901	Zephyrm Bioscience	AE-ILD	Ⅱ	2023/07
ZH901	Zephyrm Bioscience	aGVHD	Ⅱ	2023/07

Zephyrm Bioscience — ZH901 for aGVHD

Product Overview

China's leading and only hESC-derived cell therapy to have completed Phase II trials

ZH901 targets multiple indications, including aGVHD. Its paracrine activity supports inflammation control and tissue repair, while a single hESC seed bank enables standardized off-the-shelf production with less donor variability and stronger immunomodulation than conventional MSCs.

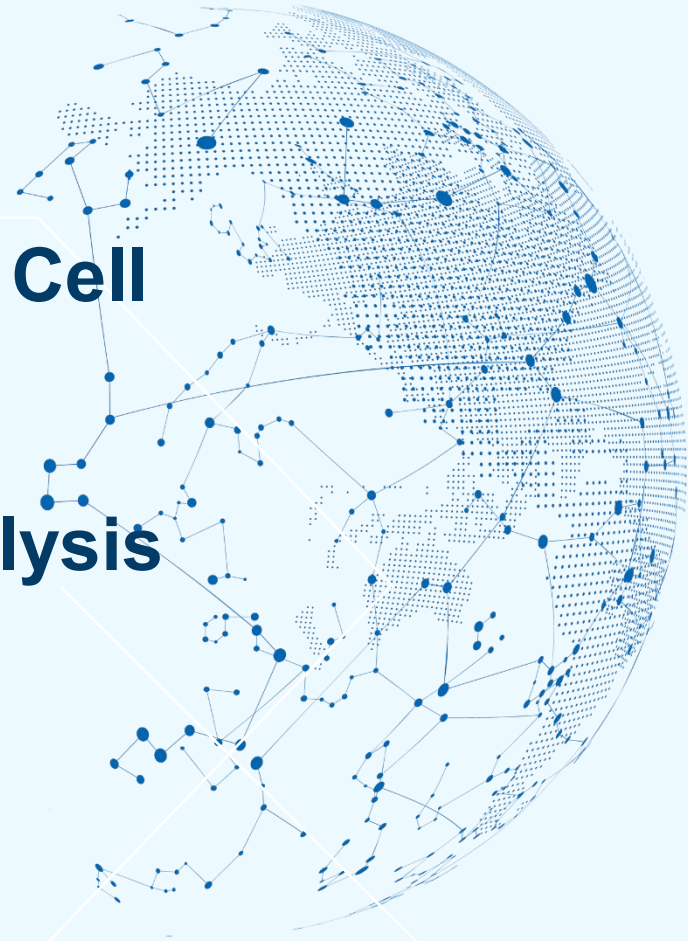
Clinical Data

- Approximately 100 participants have received ZH901 across indications. In the Phase II steroid-refractory aGVHD cohort, the overall response rate (ORR) was 60%.
- The overall safety profile was favorable, with no severe adverse events directly attributed to ZH901.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Chapter 5

Mesenchymal Cell Therapies Technical Analysis



05

Overview of Mesenchymal Cell Therapy

Mesenchymal cell therapy utilizes self-renewing and multipotent adult stem cells to promote tissue repair and exert immunomodulatory effects, offering therapeutic potential across a wide range of diseases, with advantages such as low immunogenicity and relatively limited ethical concerns.

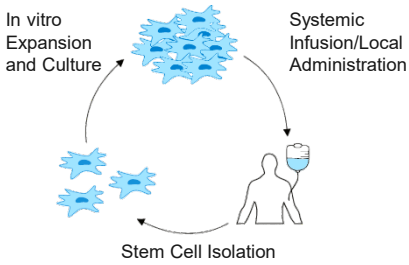
Concept of Mesenchymal Cells

Mesenchymal cells (MSCs) are adult stem cells widely distributed in tissues such as bone marrow, adipose tissue, placenta, and umbilical cord. They possess stable self-renewal capacity and multipotent differentiation potential, enabling differentiation into mesodermal cells including adipocytes, osteoblasts, and chondrocytes. MSCs also exhibit strong immunomodulatory, anti-inflammatory, and paracrine functions, making them one of the most clinically mature stem cell types.

Concept of Mesenchymal Cell Therapy

Figure. Concept of Mesenchymal Cell Therapy

MSC therapy is an advanced therapeutic strategy based on adult stem cells, with multiple MSC products approved for marketing worldwide. Leveraging their self-renewal capacity and multilineage differentiation potential, MSCs can be isolated ex vivo, expanded at large scale, and subjected to stringent quality control before administration. MSCs actively home to injured tissues and exert therapeutic effects mainly through paracrine mechanisms. By secreting bioactive factors such as TGF-β, PGE2, and IL-10, MSCs modulate the local immune microenvironment, suppress excessive inflammation, promote angiogenesis, and facilitate tissue repair and regeneration. This mechanism, integrating tissue repair, immunomodulation, and anti-inflammatory effects, supports the broad clinical potential of MSCs in osteoarthritis, autoimmune diseases, cardiovascular diseases, and inflammatory disorders.



In May 2025, the International Society for Cell & Gene Therapy (ISCT), based on single-cell sequencing evidence, clarified the molecular distinctions between MSCs and conventionally defined stem cells and officially recommended replacing "mesenchymal stem cells" with "mesenchymal stromal cells" to better reflect their therapeutic mechanisms involving paracrine effects, immunomodulation, and stromal support. This change addressed a long-standing terminological debate over three decades. In China, the regulatory framework continues to use the term "mesenchymal stem cells" to maintain industrial and regulatory continuity. The Technical Guidelines for Pharmaceutical Research and Evaluation of Mesenchymal Stem Cell Products (Draft for Public Consultation), issued in May 2026, retains this terminology and recognizes MSCs as the key active component of related products, emphasizing their therapeutic effects through paracrine activity, immune regulation, and microenvironmental interactions. Despite different terminology, "mesenchymal stromal cells" and "mesenchymal stem cells" refer to the same category of mesenchymal cell-based therapeutic products, with differences reflecting scientific and regulatory contexts rather than fundamental biological distinctions.

Advantages of Mesenchymal Cell Therapy

The major advantages of mesenchymal cell therapy include strong immunomodulatory effects, tissue repair and regeneration capacity, broad availability, and relatively low ethical concerns. MSCs can be readily obtained from tissues such as bone marrow, adipose tissue, placenta, and umbilical cord, and expanded ex vivo to obtain sufficient cell quantities. Through paracrine mechanisms, MSCs promote endogenous tissue repair and regeneration while exhibiting low immunogenicity and effective immune regulation, supporting their therapeutic potential in autoimmune diseases, inflammatory injuries, and degenerative diseases. MSCs also possess favorable homing ability, allowing migration toward damaged tissues and inflammatory microenvironments. Under China's classification-based regulatory framework, MSCs are generally considered medium-to-high-risk biomedical new technologies, whereas autologous MSC products are typically categorized as medium-to-low risk.

Figure. Advantages of Mesenchymal Cell Therapy

High Proliferative Capacity

MSCs can rapidly proliferate under in vitro culture conditions and maintain a stable karyotype and differentiation potential after multiple passages.

Low Immunogenicity

MSCs express low levels of MHC class I molecules and lack MHC class II and co-stimulatory molecules, resulting in low immunogenicity and reduced risk of allogeneic rejection, supporting their use in allogeneic transplantation.

Broad Sources

MSCs are usually derived from adult adipose tissue, bone marrow, dental pulp, and the placenta/umbilical cord of newborns, and they are relatively easy to obtain and raise fewer ethical concerns.

Favorable Safety Profile

MSCs have been widely investigated in preclinical and clinical studies across various diseases, with mostly mild adverse reactions and rare serious adverse events reported.

Source: Literature Review, Frost & Sullivan Analysis

Sources of Mesenchymal Cells (1)

Mesenchymal cell therapy utilizes self-renewing and multipotent adult stem cells to promote tissue repair and exert immunomodulatory effects, demonstrating therapeutic potential across various diseases with advantages including low immunogenicity and relatively limited ethical concerns.

Placenta-derived Mesenchymal Cells

Placental mesenchymal cells (PMSCs) are adult stem cells isolated from placental tissues. Studies have confirmed that MSCs are distributed throughout the placenta, with the middle layer beneath the umbilical cord attachment being an enriched region. Under appropriate induction conditions, PMSCs can differentiate into osteoblasts, chondrocytes, and adipocytes. Moreover, PMSCs exhibit strong immunomodulatory and paracrine functions, regulating the local microenvironment through cytokine secretion. They also possess strong proliferative capacity, low immunogenicity, and minimal ethical concerns, supporting their wide applications in tissue repair and regenerative medicine.

PMSCs have demonstrated promising clinical application potential. In orthopedic diseases, intra-articular injection of PMSCs can effectively relieve pain and improve joint function in patients with osteoarthritis. In wound healing, PMSCs promote angiogenesis and granulation tissue formation, accelerating the repair of difficult-to-heal wounds such as diabetic foot ulcers. Furthermore, their strong immunomodulatory ability supports applications in graft-versus-host disease, and clinical studies have demonstrated good safety and therapeutic potential in liver cirrhosis, bronchopulmonary dysplasia in preterm infants, and acute respiratory distress syndrome.

Umbilical Cord-derived Mesenchymal Cells

Umbilical Cord-derived Mesenchymal Cells (UC-MSCs), originating from newborn umbilical cord tissue, possess multilineage differentiation capacity toward bone, cartilage, and adipose tissues. They also exhibit paracrine functions and immunomodulatory properties, showing promising potential in tissue repair. UC-MSCs can be obtained safely and non-invasively, with readily available sources, strong expansion capacity, low immunogenicity, and limited ethical concerns, making them a highly promising MSC source. In regenerative medicine, UC-MSCs have attracted considerable attention due to their superior proliferation capacity and genetic stability compared with adult-derived MSCs.

Clinically, UC-MSCs have been applied in osteoarthritis, diabetic foot ulcers, immune diseases, and organ repair. In osteoarthritis treatment, intra-articular injection of UC-MSCs or exosomes improves joint function through chondrogenic differentiation and anti-inflammatory effects. For refractory wounds such as diabetic foot ulcers, UC-MSCs accelerate healing by promoting angiogenesis and immunomodulation. Additionally, clinical studies have demonstrated their therapeutic potential in liver cirrhosis, graft-versus-host disease, spinal cord injury, and COVID-19-related acute respiratory distress syndrome.

Adipose Tissue-derived Mesenchymal Cells

Adipose-derived Mesenchymal Cells (ADSCs) serve as an important alternative source to Bone Marrow Mesenchymal Cells (BMSCs). ADSCs can be isolated from adipose tissue and possess multilineage differentiation potential, including differentiation into adipocytes, osteoblasts, and chondrocytes, demonstrating promising prospects in tissue and organ repair. Due to the wide availability of adipose tissue, minimally invasive collection, and strong expansion capacity, ADSCs have become a highly promising MSC source. In orthopedic application research, approximately 27.9% of studies utilize adipose-derived MSCs, ranking second only to bone marrow-derived MSCs.

The clinical applications of ADSCs cover multiple fields, including osteoarthritis and cartilage repair, tissue injury and wound healing, immune-mediated diseases, and organ repair. In osteoarthritis treatment, ADSCs are primarily applied through their anti-inflammatory properties and chondrogenic differentiation ability; intra-articular injection of ADSCs or stromal vascular fraction improves joint function. For tissue injuries such as diabetic foot ulcers, complex perianal fistulas associated with Crohn's disease, and scleroderma, ADSCs promote angiogenesis and regulate the immune microenvironment to accelerate wound healing and tissue repair. Furthermore, their strong immunomodulatory capacity supports potential applications in clinical studies of type 1 diabetes, multiple sclerosis, Parkinson's disease, and myocardial ischemia.

Bone Marrow-derived Mesenchymal Cells

Bone Marrow Mesenchymal Cells (BMSCs) are the earliest identified source of MSCs. They exhibit plastic-adherent growth in vitro and exhibit fibroblast-like morphology, representing a heterogeneous cell population. Under appropriate induction conditions, BMSCs can differentiate into osteoblasts, chondrocytes, and adipocytes. They also possess strong immunomodulatory and paracrine functions, regulating the local microenvironment through cytokine secretion. Therefore, BMSCs are widely used in tissue repair and regenerative medicine research.

BMSCs have been widely applied in clinical settings. In neurological diseases, BMSC products for ischemic stroke have entered phase II clinical trials, demonstrating improvements in patients' neurological and motor functions. In bone and joint diseases, intra-articular injection of BMSCs can significantly alleviate osteoarthritis pain and improve joint function, and related therapies have entered clinical trials for post-menopausal osteoporosis. Additionally, BMSCs are used for graft-versus-host disease and have been investigated in liver cirrhosis and liver failure, showing promising clinical potential.

Source: Literature Review, Frost & Sullivan Analysis

Sources of Mesenchymal Cells (2)

Mesenchymal cell therapy utilizes self-renewing and multipotent adult stem cells to promote tissue repair and exert immunomodulatory effects, demonstrating therapeutic potential across various diseases with advantages including low immunogenicity and relatively limited ethical concerns.

Dental Pulp-derived Mesenchymal Cells

Dental Pulp Stem Cells (DPSCs) are primarily isolated from the dental pulp tissue of extracted permanent teeth, especially third molars, or deciduous teeth. They are readily accessible, and their collection involves minimal ethical concerns. Compared with MSCs from other sources, DPSCs exhibit several unique biological advantages. Derived from neural crest ectodermal mesenchyme, DPSCs have a stronger capacity to secrete neurotrophic factors, including BDNF, NGF, and GDNF, providing unique potential for neurological disease treatment. Compared with umbilical cord-derived MSCs (UC-MSCs) and adipose-derived MSCs (ADSCs), DPSCs show superior osteogenic differentiation capacity and pro-angiogenic activity, making them suitable for bone defect repair and reconstruction of ischemic microenvironments. Moreover, DPSCs inhibit T cell activation through secretion of IDO, PGE2, and HGF, exhibiting low immunogenicity and strong immunomodulatory properties that support potential allogeneic applications.

DPSCs have broad application potential in oral regenerative medicine and systemic disease treatment. In oral medicine, periodontal alveolar bone defect repair is the most valuable translational direction: local DPSC injection promotes reconstruction of the alveolar bone-periodontal ligament complex. Clinical data demonstrate sustained new bone formation and alveolar bone mineralization one year after injection, suggesting long-term paracrine regulation rather than transient filling effects. DPSCs can also support dental pulp regeneration by restoring vascular and neural functions of the pulp, providing an alternative to conventional root canal treatment. In systemic diseases, their neurotrophic and immunomodulatory properties have supported clinical exploration in neurodegenerative diseases, including spinal cord injury and Parkinson's disease, as well as autoimmune diseases such as systemic lupus erythematosus.

Peripheral Blood-derived Mesenchymal Cells

Peripheral blood-derived mesenchymal cells (PB-MSCs) are adult stem cells present in peripheral blood circulation. Compared with traditional sources such as bone marrow and adipose tissue, PB-MSCs have become an emerging research focus in regenerative medicine due to their advantages, including minimally invasive collection, repeatable sampling, and suitability for autologous transplantation. They show application potential in bone and cartilage repair, severe limb ischemia, and tissue engineering. PB-MSCs can differentiate into osteoblasts and chondrocytes to repair joint defects, while also promoting angiogenesis in ischemic limbs and reducing amputation rates. Furthermore, co-culture-based "rejuvenation" technology can delay cellular senescence and enhance immunomodulatory functions, potentially providing a new cell therapy strategy for refractory diseases.

■ Mesenchymal cells from different sources

The figure compares the advantages and disadvantages of MSCs from different sources, including bone marrow, adipose tissue, umbilical cord, placenta, dental pulp, and peripheral blood, across four dimensions: acquisition difficulty, expansion ability, homing ability, and immunogenicity, comprehensively illustrating the distinct characteristics of MSCs from various tissue sources.

Figure. Comparison of the advantages of mesenchymal cells from different sources

Source	Acquisition Method	Proliferative Capacity	Immunomodulatory Capacity
Bone marrow	Invasive; iliac bone/sternum marrow puncture under local anesthesia, with potential secondary trauma	Weak proliferation (the population doubling time is approximately 40 hours)	Earliest studied source with the most extensive clinical applications
Adipose tissue	Minimally invasive liposuction; risk of postoperative infection	Relatively high proliferation (population doubling time ~45 h; stable expansion up to 8 passages before senescence)	Moderate immunomodulatory capacity
Umbilical cord	Non-invasive; collection of the umbilical cord and Wharton's jelly during childbirth	Highest proliferation (population doubling time ~24 h; sustained up to 10 passages)	Robust immunosuppressive capacity and low immunogenicity
Placenta	Non-invasive; obtained after childbirth	Relatively high proliferation; varies by region, with decidual MSCs (D-MSCs) showing longer lifespan.	Possesses immunomodulatory capabilities; decidual subpopulation exhibits prominent immunosuppressive ability
Dental pulp	Non-invasive; exfoliated deciduous teeth, extracted wisdom teeth/orthodontic waste teeth, and gingival waste materials	Relatively high proliferation rates (with a population doubling time of approximately 25–30 hours)	Low immunogenicity; low MHC-I expression, negative MHC-II expression, and absence of rejection after autologous cryopreservation and reinfusion
Peripheral blood	Non-invasive and straightforward collection; low stem cell yield and difficulty obtaining sufficient cell numbers	Colony-forming efficiency: 1.2–13 clones/million mononuclear cells; lowest in vitro expansion capacity, limiting clinical applications	Immunomodulatory capacity with low secretion of anti-inflammatory factors

Source: Literature Review, Frost & Sullivan Analysis

Analysis of Technological Advances in MSC Therapies

Despite its therapeutic promise, MSC therapy is hindered by heterogeneity, culture-induced functional decline, and poor homing, yet emerging approaches in gene editing, delivery optimization, and tissue engineering are opening new pathways for clinical application.

Gene-edited MSC Technology

Naturally derived MSCs have demonstrated acceptable safety and stability in long-term clinical development, with several products approved worldwide, including Ryoncil, approved by the U.S. FDA, and Amimestrocel Injection, conditionally approved by China's NMPA. The technological pathway is highly mature, and these marketed products have established comprehensive systems for MSC isolation and culture, quality control, large-scale production, and clinical translation, providing a solid foundation for next-generation technologies.

Building on this foundation, gene-edited MSC technology addresses key limitations in allogeneic transplantation, including immune responses, insufficient functional persistence, low homing efficiency, and product heterogeneity, through precise genetic modification. This approach enhances MSCs with improved immune tolerance, homing capacity, and stable secretion of therapeutic factors. Early strategies mainly relied on transient transgene expression or random integration, whereas advances in technology have led to the era of precise gene editing represented by CRISPR/Cas9. Current strategies primarily employ viral or non-viral delivery systems to achieve targeted gene knockout, knock-in, or endogenous gene regulation while maintaining cell viability, thereby enhancing MSCs properties related to stemness maintenance, migration, engraftment, anti-apoptosis, and immune-inflammatory regulation, and improving therapeutic efficacy and clinical translation potential.

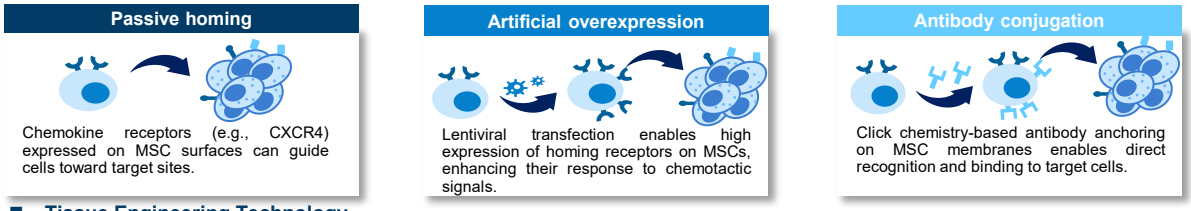
Figure. Types of Gene-edited Mesenchymal Cell Technology

Type	Definition
Zinc finger nucleases (ZFNs) technology	Zinc finger proteins (ZFPs), derived from DNA transcription factors, are fused with FokI nuclease. ZFNs recognize specific DNA sequences through zinc finger domains and induce double-strand DNA breaks (DSBs), enabling gene modification via endogenous repair mechanisms.
TALENs gene editing technology	TALENs consist of TALE repeats derived from AvrBs3 and FokI nuclease. Individual TALE repeats recognize specific DNA bases, enabling TALENs to bind the target sequence and induce site-specific DSBs.
CRISPR/Cas9 gene editing technology	Based on prokaryotic immune mechanisms, CRISPR/Cas9 uses single-guide RNA (sgRNA) to direct Cas9 cleavage of target DNA. Following DSB formation, gene editing is achieved through NHEJ-mediated knockout or HDR-mediated knock-in and repair.
Single-base editing (BE) technology	Developed from CRISPR/Cas9, base editing combines inactivated Cas9/nCas9 with deaminases to achieve precise single-base conversion without DSBs. CBE enables C→T conversion, while ABE enables A→G conversion.
Prime editing (PE) technology	CRISPR-derived prime editors use pegRNA for target recognition and template delivery. Without exogenous donor DNA or DSBs, PE enables precise base substitutions and small insertions or deletions.
PASTE technology	Based on the PASTE system, a Cas9 nickase generates a single-strand nick, while serine integrase mediates site-specific insertion of large DNA fragments (~36 kb), enabling stable and long-term target gene expression.

Targeted Delivery of Mesenchymal Cell Technology

The major bottleneck of traditional intravenous MSCs delivery is low homing efficiency: most cells are trapped in non-target organs such as the lungs and spleen, with only a small proportion reaching injured sites. Early strategies attempted to achieve passive homing by utilizing cellular chemokine receptors; however, their expression decreases during in vitro expansion, resulting in limited targeting efficiency. Lentiviral transfection was subsequently used to induce stable and high expression of chemokine receptors, improving migration capacity but introducing risks associated with viral integration, complex preparation, and high costs. Recently, antibody-conjugated targeting strategies have emerged as a mainstream approach. By covalently anchoring disease-specific antibodies onto MSC surfaces through methods such as click chemistry, this technology enables "plug-and-play" targeted modification with flexible target switching, while avoiding genetic manipulation and offering advantages in safety and scalability.

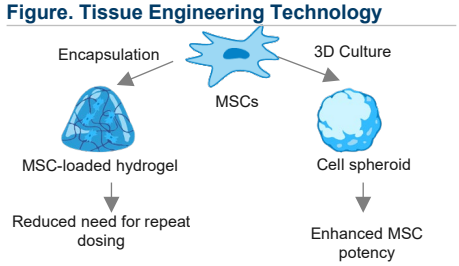
Figure. Targeted MSC Delivery Strategies



Tissue Engineering Technology

Tissue engineering technology provides an important approach to address key bottlenecks in mesenchymal cell therapy. Directly infused MSCs are prone to rapid loss in vivo, often requiring repeated administration. Hydrogel technology creates a protective microenvironment with tunable physicochemical properties, enhancing cell retention, survival, and functional maintenance, thereby enabling precise and controllable cell delivery and improving post-transplantation cell survival. Additionally, MSC homing and implantation efficiency are affected by long-term culture and passaging. Compared with traditional adherent culture, 3D culture-derived cell spheroids exhibit enhanced stemness, biological functions, and therapeutic efficacy, providing a feasible strategy to optimize MSC therapy and improve clinical outcomes.

Figure. Tissue Engineering Technology



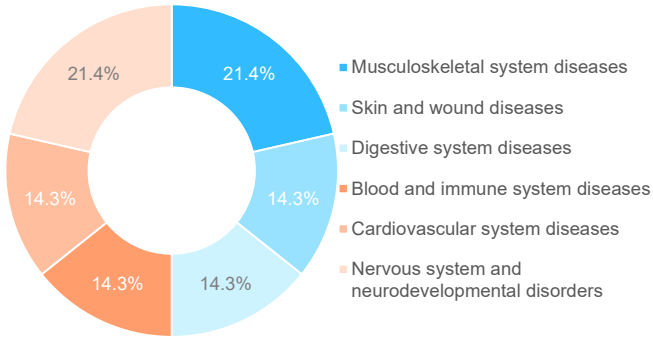
Source: Literature Review, Frost & Sullivan Analysis

Overview of Marketed and Investigational MSC Therapies

■ Approved MSC Therapies Classified by Indications

Approximately 13 MSC products have been approved for marketing worldwide, covering six major disease areas. Musculoskeletal diseases and nervous system and neurodevelopmental disorders account for the highest and equal proportions (21.4% each), representing the two most commercially mature therapeutic areas. Digestive system diseases, skin wounds, blood and immune disorders, and cardiovascular diseases each account for 14.3%, demonstrating commercial breakthroughs across multiple systems. Overall, MSC products have progressed from early exploration of single indications to broader clinical application across multiple disease areas, with further potential to expand into refractory diseases and broaden the range of clinically addressable indications.

Figure. Global Approved MSC Therapies by Therapeutic Indications

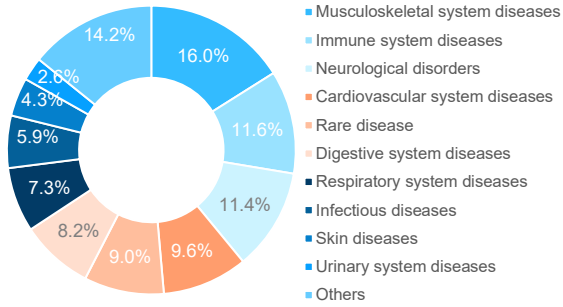


Note: The data cover MSC products approved for marketing worldwide as of May 2026.

■ MSC Therapies in Clinical Development, by Indication

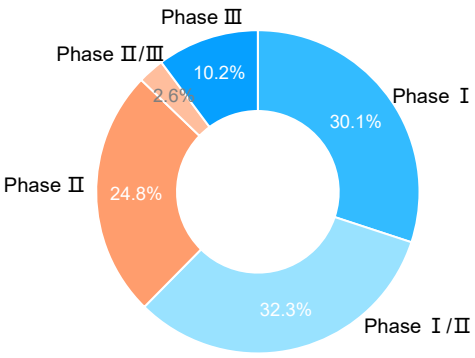
According to data from ClinicalTrials.gov, global MSC products in clinical development show broad indication distribution, mainly concentrated in musculoskeletal diseases (16.0%), immune system diseases (11.6%), nervous system diseases (9.6%), and cardiovascular diseases (9.0%). Rare diseases (8.2%) and digestive system diseases (7.3%) also represent important areas. Overall, musculoskeletal and immune system diseases are the two leading fields for MSC therapy development, with emerging R&D clusters in nervous system, cardiovascular, and rare diseases. With deeper mechanistic understanding and continued technological advancement, MSC therapy is expected to expand into broader indications and benefit more patient populations.

Figure. Global MSC Therapy Pipeline by Indications



Note: The data cover MSC products that have entered the clinical trial stage, with the time period ending in May 2026.

Figure. Global MSC Therapy Pipeline by Clinical Stage



■ MSC Therapies in Clinical Development, by Clinical Stage

According to ClinicalTrials data, among MSC products in clinical development worldwide, including those in China, Phase I/II pipelines account for the largest proportion (32.3%), followed by Phase I (30.1%) and Phase II (24.8%). Phase III and Phase II/III pipelines account for 10.2% and 2.6%, respectively. All included clinical trials are pharmaceutical company-led registration studies aiming for marketing approval (NDA/BLA). Overall, MSC therapy development remains concentrated in early-to-mid clinical stages.

Note: The data cover MSC products that have entered the clinical trial stage, with the time period ending in May 2026.

Source: ClinicalTrials, Frost & Sullivan Analysis

Chapter 6
Clinical Applications of Stem Cells

06

Global Landscape of Approved Stem Cell Products

More than 30 stem cell products have received marketing authorization worldwide, including two iPSC-derived products. Major indications include degenerative diseases, immune disorders, blood and lymphatic system disorders, and cardiac diseases.

Approved Stem Cell Therapies Worldwide

To date, approved stem cell products worldwide are derived primarily from MSCs, HSCs, iPSCs and limbal stem cells. More than 30 products have been approved, including 13 MSC-based products, three HSC-based products, two iPSC-based products and one limbal stem cell product. Their indications span multiple therapeutic areas, including degenerative, immune, hematological and lymphatic, and cardiovascular diseases.

Table. Representative Stem Cell Products Approved Worldwide

	Product	Company	Indication	Cell Source	Date (Region)
HSCs	RegeneCyte	StemCyte	Disorders affecting the hematopoietic system	Allogeneic umbilical cord blood	2024 (United States)
	Zemcelpro	ExCellThera	Hematologic malignancies requiring allogeneic hematopoietic stem cell transplantation	Allogeneic cord blood	2025 (European Union)
	Waskyra	Fondazione Telethon ETS	Wiskott–Aldrich syndrome	Autologous HSCs	2025 (European Union、United States)
MSCs	MPC	Mesoblast	Bone defects and fractures requiring repair	Autologous/allogeneic bone marrow	2010 (Australia)
	Queencell	Anterogen	Subcutaneous tissue defects	Autologous adipose tissue	2010 (South Korea)
	Hearticellgram	FCB-Pharmicell	Acute myocardial infarction	Autologous bone marrow	2011 (South Korea)
	Cupistem	Anterogen	Complex perianal fistulas associated with Crohn's disease	Autologous adipose tissue	2012 (South Korea)
	Cartistem	Medi-post	Knee articular cartilage defects caused by degenerative osteoarthritis or repeated trauma	Allogeneic cord blood	2012 (South Korea)
	NeuroNata-R	Corestem	Amyotrophic lateral sclerosis	Autologous bone marrow	2014 (South Korea)
	Remestemcel	Mesoblast	Acute graft-versus-host disease following allogeneic hematopoietic stem cell transplantation	Allogeneic bone marrow	2016 (Japan)
	Alofisel	TiGenix	Complex perianal fistulas associated with Crohn's disease	Allogeneic adipose tissue	2018 (European Union)
	Stemirac	Nipro Corp	Traumatic spinal cord injury	Autologous bone marrow	2018 (Japan)
	Stempeucel	Stempeutics	Critical limb ischemia due to Buerger's disease	Allogeneic bone marrow	2020 (India)
	AMESANAR	RHEACELL	Chronic venous ulcers	Skin	2021 (Germany)
	Akuugo	SanBio	Chronic motor deficits following traumatic brain injury	Allogeneic bone marrow	2024 (Japan)
	Amimestrocel Injection	Platinumlife Biotechnology	aGvHD	Allogeneic umbilical cord	2025 (China)
Limbal stem cells	Holoclar	Chiesi Farmaceutici	Limbal stem cell deficiency caused by physical or chemical ocular burns	Autologous limbal tissue	2015 (European Union)
Neural progenitor cells	Raguneprocel	Sumitomo Pharma	Parkinson's disease	iPSCs	2026 (Japan)
Cardiomyocyte patches	ReHeart	Cuorips	Severe heart failure	iPSCs	2026 (Japan)

Source: Company Information, Public Information, Frost & Sullivan Analysis

Clinical Translation and Application at Boao Lecheng

Thanks to its pioneering policies, Boao Lecheng has successfully facilitated the clinical translation and implementation of 18 stem cell therapy technologies, covered a wide range of disease areas including respiratory conditions, chronic diseases and rare diseases, and achieved compliant commercial application.

Overview of the Implementation Catalogue for the Translation and Application of Stem Cell Technologies

As of June 2026, 18 stem cell technologies have been authorized for clinical translational application at Boao Lecheng, including 13 MSC-based technologies, 3 airway basal stem cell technologies, 1 vascular endothelial progenitor cell technology, and 1 iPSC-based technology. The catalogue specifies the implementing hospital and fee schedule for each technology, with indications covering osteoarticular, respiratory, cardiovascular, hepatic, endocrine, and neurodegenerative diseases, among others.

Table. Implementation Catalogue of Stem Cell Translation and Application at Boao Lecheng

No.	Full Project Title	Stem Cell Type	Implementing Hospital	Filing Price
1	Umbilical Cord-Derived Mesenchymal Stem Cell Injection for Knee Osteoarthritis	UC-MSC	Ruijin Hainan Hospital	RMB 36,350 / session (per knee)
2	Autologous Pulmonary Airway Basal Stem Cell Therapy for Chronic Obstructive Pulmonary Disease	Airway basal stem cells	Ruijin Hainan Hospital	RMB 150,000 / session
3	Human Umbilical Cord-Derived MSC Therapy for Heart Failure with Reduced Ejection Fraction (HFrEF)	UC-MSC	West China Lecheng Hospital	RMB 60,000 / session (3 sessions / course)
4	Mesenchymal Stem Cell Therapy for Complications Following Bone Marrow Transplantation	Universal allogeneic MSCs	West China Lecheng Hospital	RMB 80,000 / session (1–3 sessions)
5	Airway Basal Stem Cell Therapy for Interstitial Lung Disease	Airway basal stem cells	Ruijin Hainan Hospital	RMB 150,000 / session
6	Airway Basal Stem Cell Therapy for Bronchiectasis	Airway basal stem cells	Ruijin Hainan Hospital	RMB 150,000 / session
7	Menstrual Blood-Derived Mesenchymal Stem Cell Therapy for Pulmonary Fibrosis	Menstrual blood (endometrial)-derived MSCs	Shulan (Boao) Hospital	RMB 140,000 / course (3 sessions)
8	Umbilical Cord Mesenchymal Stem Cell Therapy for Primary Ovarian Insufficiency	UC-MSC	Ciming Boao International Hospital	RMB 80,000 / bilateral
9	Mesenchymal Stem Cell Therapy for Rheumatoid Arthritis	UC-MSC	West China Lecheng Hospital	RMB 50,000 / session (4 sessions)
10	Human Umbilical Cord-Derived Mesenchymal Stem Cells (VUM02 Injection) for Liver Cirrhosis	UC-MSC	Hainan Mellsser Hospital	RMB 150,000 / session; RMB 450,000 / full course
11	Dental Pulp Mesenchymal Stem Cell Therapy for Periodontal Disease	Dental pulp-derived MSCs	Hainan Yiling Hospital	RMB 59,800 / per tooth
12	Human Umbilical Cord Mesenchymal Stem Cell Therapy for Type 2 Diabetes Mellitus	UC-MSC	Ciming Boao International Hospital	RMB 59,800 / session (3 sessions / course)
13	Menstrual Blood-Derived Mesenchymal Stem Cell Therapy for Decompensated Liver Cirrhosis	Menstrual blood-derived MSCs	Shulan (Boao) Hospital	RMB 150,000 / course (3 sessions)
14	Umbilical Cord-Derived Mesenchymal Stem Cell Injection for Knee Osteoarthritis	UC-MSC	Hainan Divine Hospital	RMB 36,350 / injection
15	Allogeneic Endothelial Progenitor Cells (EPCs) for Critical Limb Ischemia	Vascular endothelial progenitor cells	West China Lecheng Hospital	RMB 100,000 / case
16	Human GLP-1- and FGF21-Overexpressing Adipose-Derived MSC Injection for Type 2 Diabetes Mellitus	Adipose-derived MSCs	Ruijin Hainan Hospital	RMB 360,000 / course
17	M-021001 Cell Therapy for Knee Joint Injury	Pluripotent stem cell-derived MSCs	Boao Super Hospital	RMB 80,000 / per knee
18	Human iPSC-Derived Motor Neuron Progenitor Cells for Amyotrophic Lateral Sclerosis	iPSC	Ruijin Hainan Hospital	To be determined

Source: Public Information, Frost & Sullivan Analysis

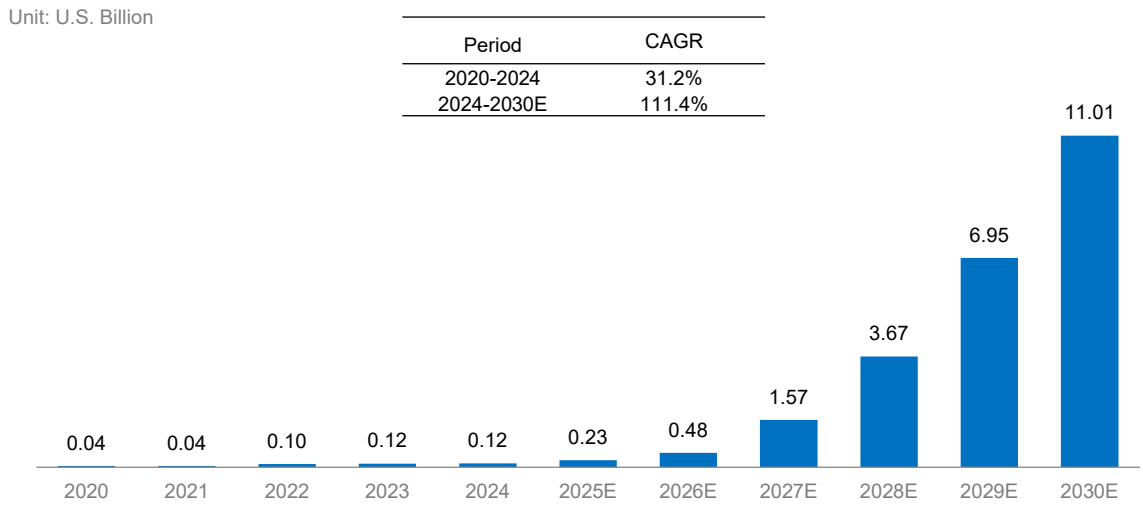
The Global and Chinese Stem Cell Therapeutics Market

The global stem cell therapy market is set to experience rapid growth; coupled with the commercial launch of China's first stem cell therapy in 2025, the Chinese market is expected to see even more significant growth.

Global Stem Cell Drug Market Size

The global stem cell therapeutics market was valued at U.S.0.12 billion in 2024 and is projected to reach U.S.11.01 billion by 2030, representing a compound annual growth rate of 111.4 per cent between 2024 and 2030. The market is expected to grow further in the future.

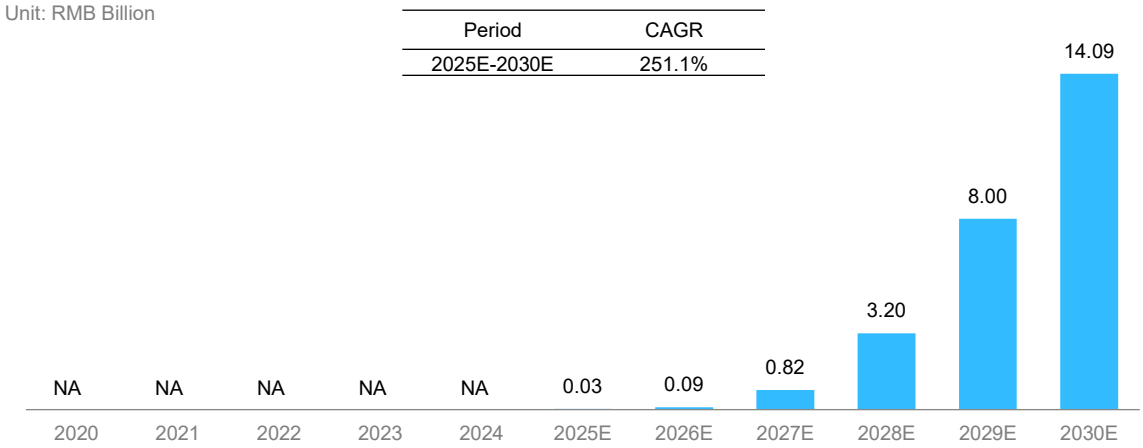
Figure. Global Stem Cell Drug Market Size



China Stem Cell Drug Market Size

In January 2025, the National Medical Products Administration (NMPA) granted conditional approval for China's first stem cell drug, Amimestrocel Injection (brand name: Ruibosheng; human umbilical cord-derived MSCs; approved indication: steroid-refractory acute graft-versus-host disease (aGVHD) with predominant gastrointestinal involvement in patients aged 14 years and older). Prior to this approval, no stem cell drug had been approved for marketing in China; therefore, the China stem cell drug market size was zero between 2020 and 2024. With the commercial ramp-up of this product, the market entered a substantive growth phase starting from 2025. China's stem cell drug market size is projected to reach RMB 0.03 billion in 2025 and RMB 14.09 billion in 2030, representing a CAGR of 251.1% from 2025 to 2030. The market is expected to grow further in the future.

Figure. China Stem Cell Drug Market Size



Source: Company Information, Public Information, Frost & Sullivan Analysis

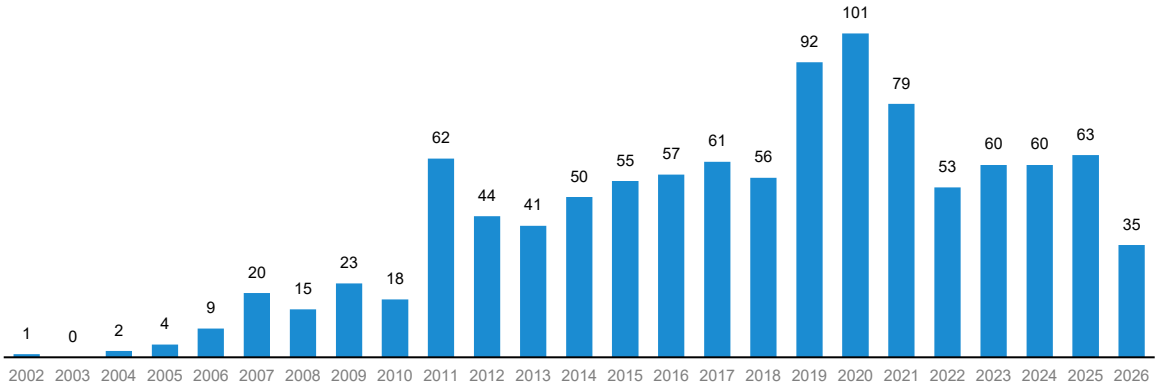
Analysis of the Global Stem Cell Therapy Pipeline (1)

Overview of Registered Clinical Trials for Stem Cell Therapies

According to ClinicalTrials.gov data, as of May 2026, counted by registered clinical trial identifiers, this analysis includes only registrational trials sponsored by pharmaceutical companies with the ultimate goal of marketing authorization (NDA/BLA submission). The cumulative number of registered stem cell therapy–related clinical trials worldwide has exceeded 1,060. Prior to 2010, the annual number of newly registered stem cell therapy trials remained at a low level, ranging from single digits to fewer than 20 per year. From 2011 to 2018, annual registrations entered a phased growth period, fluctuating between approximately 40 and over 60 per year. Starting in 2019, clinical advancement in the stem cell therapy space accelerated, with annual registrations surpassing 90 and reaching an all-time peak of 101 trials in 2020. From 2021 to the present, R&D activity in this space has remained robust, with annual new registrations stabilizing at 50 to 80 trials per year. Among these, MSC therapies account for approximately 61% of all stem cell therapies.

Figure. Global Registrations for Clinical Trials of Stem Cell Therapies – Analysis by Year

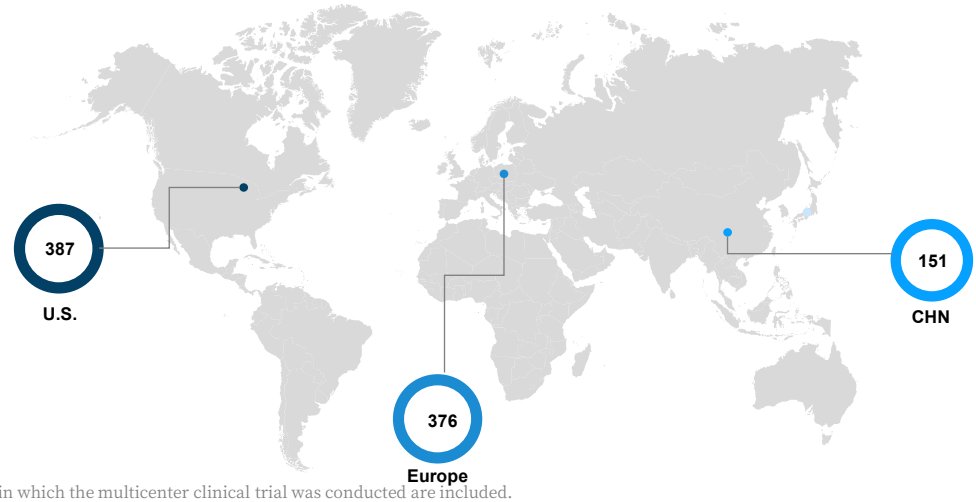
Unit: Trials



Global Stem Cell Therapy Pipeline Overview — by Trial Region/Country

According to search results from the ClinicalTrials.gov database, stem cell therapy clinical trials are primarily concentrated in the United States, Europe, China (including Hong Kong, Macao, and Taiwan), and Japan. The United States has the largest number of stem cell therapy clinical trials, with 387 trials registered as of May 2026; Europe follows closely with 376 trials. Meanwhile, according to the CDE (Center for Drug Evaluation) database, China has 151 clinical trials.

Figure. Clinical trials of stem cell therapies in major countries and regions



Note: All regions in which the multicenter clinical trial was conducted are included.

Source: ClinicalTrials, CDE, Frost & Sullivan Analysis

Analysis of the Global Stem Cell Therapy Pipeline (2)

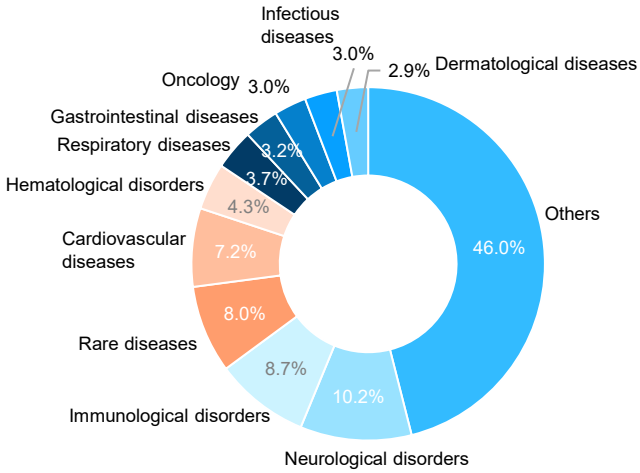
Global Stem Cell Therapy Pipeline Analysis by Therapeutic Area

As of May 2026, the global pipeline of therapies in this field spans a broad range of therapeutic areas while remaining concentrated in several key indications. Neurological diseases represent the largest clearly defined category, accounting for 10.2% of the pipeline.

Immune disorders, rare diseases and cardiovascular diseases are also major areas of development due to substantial unmet medical needs, representing 8.7%, 8.0% and 7.2% of the pipeline, respectively.

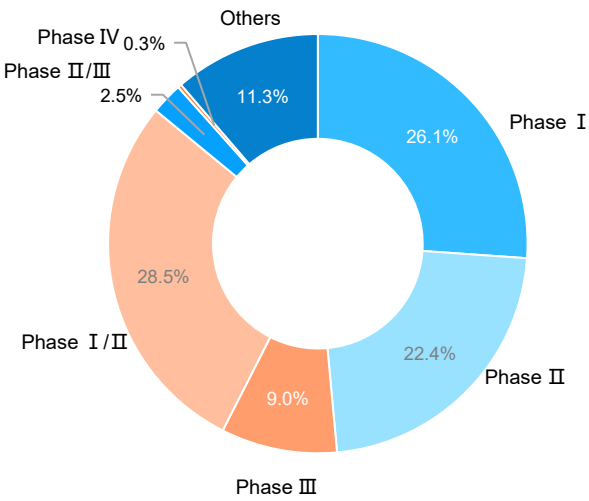
As enabling technologies and clinical development continue to advance, pipelines are also progressing across hematological (4.3%), respiratory (3.7%), gastrointestinal diseases (3.2%), oncological (3.0%), infectious (3.0%) and dermatological diseases (2.9%), highlighting broad clinical potential and diverse commercialization opportunities.

Figure. Global Stem Cell Therapy Pipeline by Therapeutic Area



Note: Products with multiple therapeutic areas were included in each relevant category.

Figure. Global Stem Cell Therapy Pipeline by Clinical Phase



Global Investigational Stem Cell Therapy Pipeline Analysis by Clinical Phase

As of May 2026, global investigational stem cell therapy pipelines are primarily concentrated in early- and mid-stage development. The included clinical trials are pharmaceutical company-sponsored registrational studies aiming to support NDA/BLA submissions. Phase I, Phase I/II, and Phase II programs represent the majority of pipelines, accounting for 77.0% in total. Phase I/II programs represent the largest proportion (28.5%), followed by Phase I (26.1%) and Phase II (22.4%), indicating that many candidate programs are undergoing intensive evaluation of early-stage safety and preliminary efficacy.

Late-stage confirmatory programs remain relatively limited, and the number of programs generally decreases as development advances to later clinical phases. Phase III programs account for 9.0%, while Phase II/III programs account for 2.5%, representing 11.5% in total.

Only 0.3% of programs have progressed to Phase IV post-marketing surveillance, while approximately 11.3% remain in other or undisclosed stages, completing the overall landscape of global stem cell therapy pipelines by clinical phase.

Source: ClinicalTrials, Frost & Sullivan Analysis

Analysis of China's Stem Cell Therapy Pipeline

Investigational Stem Cell Therapy Landscape Analysis

According to CDE data, as of May 2026, 151 stem cell therapy clinical trial registrations had been identified in China. Counted by registration number, all included trials were industry-sponsored registrational trials intended to support eventual NDA/BLA submissions.

China Investigational Stem Cell Therapy Pipeline Analysis by Therapeutic Area

Based on CDE database statistics, as of May 2026, China's investigational stem cell therapy pipelines cover a broad range of therapeutic areas. The included clinical trials are pharmaceutical company-sponsored registrational studies aiming to support NDA/BLA submissions. Among clearly classified indications, knee osteoarthritis represents the largest segment, accounting for approximately 12.1%, and remains the primary focus of domestic R&D investment. Pipelines for graft-versus-host disease (GVHD), ischemic stroke, idiopathic pulmonary fibrosis (IPF), and β-thalassemia account for 7.6%, 7.6%, 5.7%, and 5.1%, respectively. Acute respiratory distress syndrome (ARDS), Parkinson's disease, and acute-on-chronic liver failure (ACLF) each account for approximately 4.5%, forming a secondary group of well-established indications. Crohn's disease-associated perianal fistula accounts for 3.8% and represents a promising development area. Overall, China's stem cell therapy pipelines demonstrate broad coverage across difficult-to-treat diseases affecting multiple organ systems.

Figure. China Stem Cell Therapy Pipeline by Therapeutic Area

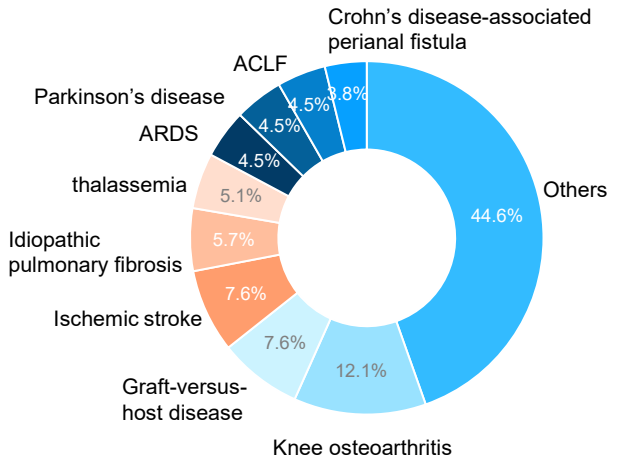
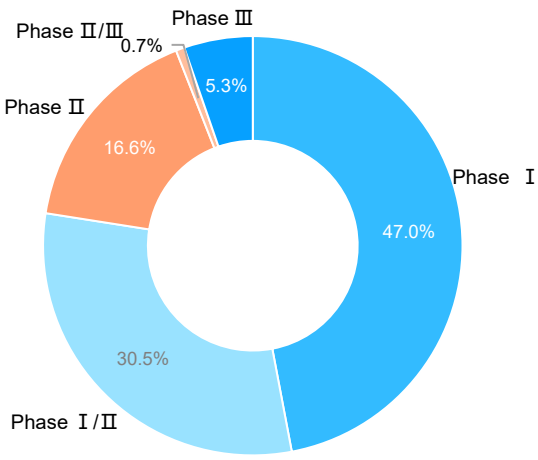


Figure. China Stem Cell Therapy Pipeline by Clinical Phase



China Investigational Stem Cell Therapy Pipeline Analysis by Clinical Phase

Based on CDE database statistics, as of May 2026, China's investigational stem cell therapy pipelines show a clear concentration in early clinical development stages. The included clinical trials are pharmaceutical company-sponsored registrational studies aiming to support NDA/BLA submissions.

Among these pipelines, Phase I programs account for the largest proportion (47.0%), representing the largest development stage in China's stem cell therapy landscape. Phase I/II programs rank second, accounting for 30.5%, and together with Phase I programs constitute the core of early-stage clinical development, with a combined share exceeding 75%. This highlights that early clinical exploration remains the dominant focus of domestic stem cell therapy development.

Phase II programs account for 16.6%, followed by Phase III programs (5.3%) and Phase II/III programs (0.7%), indicating a relatively limited proportion of late-stage clinical programs. Overall, China's stem cell therapy pipeline has established a development landscape centered on early clinical research with steady advancement toward late-stage trials, broadly consistent with global stem cell therapy development trends.

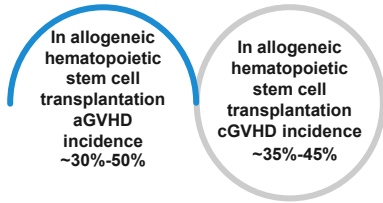
Source: CDE, Frost & Sullivan Analysis

Clinical Applications of Stem Cells — Graft-versus-host Disease

MSCs are used to treat aGVHD through immunomodulation and tissue repair, with multiple products approved globally. iPSC-derived MSCs are advancing in clinical development as a standardized next-generation therapy.

GVHD Definition and Mechanism

Graft-versus-host disease (GVHD) is a common complication following allogeneic hematopoietic stem cell transplantation (allo-HSCT), with a relatively high incidence. The core pathogenesis of GVHD involves multiple steps. Host tissue injury caused by pre-transplant conditioning activates host antigen-presenting cells (APCs), which subsequently activate donor T lymphocytes. The activated donor T cells undergo extensive proliferation and differentiate into effector T cells, such as Th1 and Th17 cells, releasing large amounts of pro-inflammatory cytokines, including TNF-α and IFN-γ. This results in a "cytokine storm" that directly attacks target organs such as the skin, gastrointestinal tract, and liver. Meanwhile, impaired regulatory T-cell function prevents the establishment of immune tolerance, further exacerbating tissue injury.

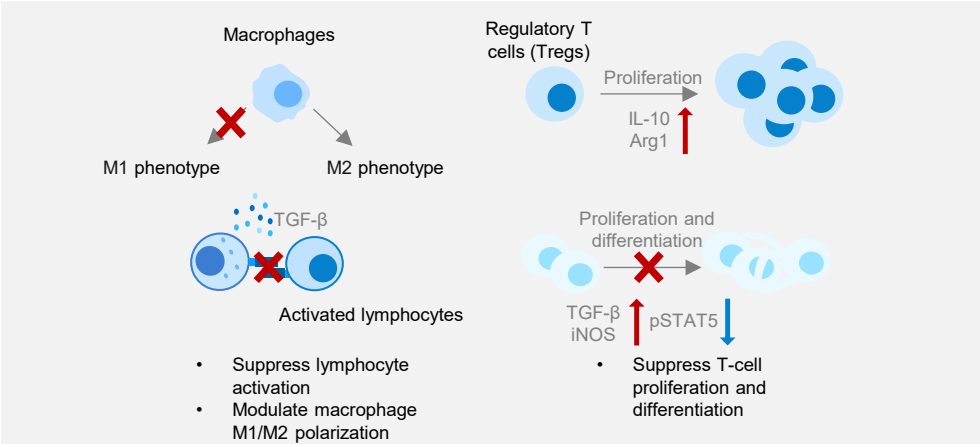


Note: Incidence rates vary depending on transplant type, conditioning regimen, and risk factors.

GVHD Management and Unmet Clinical Needs

Despite significant advances in GVHD management, substantial unmet clinical needs remain. Approximately half of allogeneic hematopoietic stem cell transplant recipients are at risk of GVHD, which impairs quality of life and contributes to non-relapse mortality. Corticosteroids remain the first-line treatment for acute GVHD, but response rates are below 50%, only one-third of responders maintain remission, and approximately 30%–50% develop steroid-refractory disease. At least one-third of patients with chronic GVHD require subsequent treatment. Although ruxolitinib is the standard second-line treatment for steroid-refractory acute GVHD, resistance and intolerance remain major challenges, and no consensus exists for third-line or later treatment, particularly for fibrotic manifestations such as skin sclerosis and pulmonary involvement. Evidence for many emerging therapies remains limited and inconsistent. Separating GVHD from the graft-versus-leukemia (GVL) effect also remains a key challenge, requiring effective GVHD control without compromising GVL activity.

Figure. Mechanism of MSC Therapy for GVHD



Mechanism of Stem Cell Therapy for GVHD

Mesenchymal cell (MSC) therapy uses ex vivo expanded bone marrow- or umbilical cord-derived MSCs administered to patients by intravenous infusion. MSCs have dual immunomodulatory and tissue-repair functions. They suppress excessive activation of donor T cells, reduce the release of pro-inflammatory cytokines such as TNF-α and IFN-γ, and promote the generation of regulatory T cells (Tregs), thereby effectively controlling the progression of steroid-refractory acute graft-versus-host disease (SR-aGVHD) and repairing damaged tissues in the gastrointestinal tract, skin, and liver.

In addition, iMSCs exert immunomodulatory effects through the same mechanisms described above. Their derivation from a single cell line enables standardized, large-scale manufacturing, providing a next-generation MSC product option for SR-aGVHD treatment with greater quality consistency and a more stable supply.

Source: Literature Review, Frost & Sullivan Analysis

Clinical Applications of Stem Cells — Graft-versus-host Disease

■ Approved and Representative MSC Products for GVHD

Three allogeneic MSC therapies have been approved globally for steroid-refractory acute graft-versus-host disease (SR-aGVHD): Mesoblast's RYONCIL® in the United States (bone marrow-derived MSCs; approved in 2024), JCR Pharmaceuticals' TEMCELL® HS Inj. in Japan (bone marrow-derived MSCs; approved in 2015), and Amimestrocel Injection (trade name: Ruibosheng) in China (umbilical cord-derived MSCs; conditionally approved by the NMPA in January 2025). RYONCIL® and TEMCELL® HS Inj. are both bone marrow-derived products approved in different markets. Against this backdrop, Nuwacell's NCR102, an investigational iMSC therapy developed with a next-generation technology, may offer a novel option with greater product consistency and a more stable supply for SR-aGVHD.

Mesoblast — RYONCIL®

RYONCIL® is indicated for pediatric patients aged ≥2 months with steroid-refractory acute graft-versus-host disease (SR-aGVHD). Derived from healthy adult donor bone marrow MSCs, RYONCIL® exerts immunomodulatory effects by suppressing donor T-cell activation and proliferation, reducing pro-inflammatory cytokines (TNF-α and IFN-γ), enhancing anti-inflammatory cytokine secretion, and recruiting anti-inflammatory cells to affected tissues.

RYONCIL is Mesoblast's brand name in the U.S. market, while Temcell is the brand name used in Japan.

The World's First Approved MSC Therapy for Pediatric Patients Under 12 Years of Age with SR-aGVHD

Pricing

Approximately U.S.$194,000 per infusion
Equivalent to approximately RMB1.4 million

Approval Status

Approved for marketing by the U.S. FDA in December 2024

Clinical Data

The approval was primarily based on a multicenter, prospective, single-arm Phase III clinical trial involving 54 pediatric patients with steroid-refractory acute graft-versus-host disease (SR-aGVHD). The results showed that:

- The primary efficacy outcome measures were the overall response rate (ORR) at Day 28 and the duration of overall response. ORR included complete response (CR) and partial response (PR). The ORR at Day 28 was 70%, including a CR rate of 30% and a PR rate of 41%.
- The most common non-laboratory adverse reactions, with an incidence of ≥20%, included viral infections, bacterial infections, infections with an unspecified pathogen, pyrexia, hemorrhage, edema, abdominal pain, and hypertension.
- The recommended dose is 2×10^6 MSCs/kg body weight per intravenous infusion, administered twice weekly for four consecutive weeks, for a total of eight infusions. Infusions should be administered at least three days apart.

Platinumlife Biotech — Ruibosheng

Ruibosheng (Amimestrocel Injection) is indicated for patients aged ≥14 years with steroid-refractory acute graft-versus-host disease predominantly involving the gastrointestinal tract. Its primary mechanism is immunomodulation mediated by human umbilical cord-derived MSCs. Through cell-to-cell contact and paracrine signaling, MSCs secrete anti-inflammatory mediators such as PGE_2 and IDO, enhance regulatory immune cells including Tregs, and suppress pro-inflammatory immune cells. MSCs can also home to sites of inflammatory injury, enabling targeted tissue repair.

In November 2025, the product was officially added to the specialty drug list of Beijing Inclusive Health Insurance, with reimbursement of up to 65%.

China's First Approved MSC Therapy

Pricing

Approx. RMB 19,800 per dose

Approval Status

Conditionally approved by China's NMPA in January 2025

Clinical Data

The approval was primarily based on the results of a Phase II clinical trial. The clinical data showed that:

- The overall response rate (ORR) at Day 28 was 63.0% (95% CI: 48.74%–75.71%), including a complete response (CR) rate of 55.6% (95% CI: 41.4%–69.08%).
- The ORR at Day 56 was 51.9% (95% CI: 37.8%–65.7%), and the CR rate was 50.0% (95% CI: 36.1%–63.9%).
- The overall survival (OS) rates were 94.4% at Day 28, 88.9% at Day 56, 79.6% at Day 100, 72.2% at Day 180, and 65.8% at Day 360.
- Regarding safety, no treatment-related adverse events (TRAEs) or infusion toxicity were reported.

Nuwacell — NCR102

Product Overview

NCR102 Injection is an iPSC-derived MSC (iMSC) product independently developed by Nuwacell. It is generated by directed differentiation of induced pluripotent stem cells (iPSCs) from healthy donors into iMSCs, all derived from a single seed iPSC clone, enabling standardized large-scale manufacturing with stable and uniform quality. Compared with conventional MSCs, iMSCs more closely resemble fetal MSCs, exhibiting the strongest proliferative capacity and full differentiation potential—making them the "youngest" MSCs available. Through immunomodulation, they suppress T-cell activation and inflammatory responses, promote tissue repair, and help control graft-versus-host disease (GVHD) progression.

Development Status

Indication

Steroid-Refractory Acute Graft-versus-Host Disease

Clinical Progress

Phase II Clinical Trial in China First Posted in September 2025

Clinical Data

NCR102 has demonstrated favorable safety and efficacy in clinical studies. As the first iPSC-derived MSC product in China to receive clinical trial authorization for the treatment of SR-aGVHD, NCR102 has progressed smoothly through clinical development and is about to enter Phase III clinical trials, and its clinical value in the target patient population continues to be evaluated.

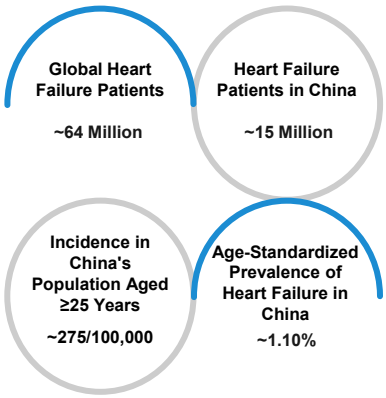
Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Heart Failure

Current treatments for heart failure alleviate symptoms but cannot repair damaged myocardium. iPSC-derived cardiomyocyte therapies may restore cardiac function through myocardial regeneration and paracrine effects. With an approved product overseas and a Chinese candidate entering Phase III development, they represent a new regenerative approach to heart failure.

Overview and Epidemiology of Heart Failure

Heart failure (HF) is a complex clinical syndrome caused by structural or functional cardiac abnormalities that impair ventricular filling or ejection, resulting in insufficient cardiac output to meet the metabolic demands of the body. Rather than a distinct disease, HF represents the terminal and most severe stage in the progression of most cardiovascular diseases, such as coronary artery disease and hypertension. It commonly manifests as persistent dyspnea, severe fatigue, and fluid retention, including pulmonary congestion and peripheral edema. With population aging and improved survival among patients with severe cardiovascular diseases, the global number of people living with HF continues to increase.



Etiology and Pathogenesis of Heart Failure

Ischemic heart disease is the leading cause of HF globally and in China and a major trigger of acute decompensation and hospitalization. HF progression involves ventricular remodeling driven by cardiomyocyte loss, extracellular matrix alterations, and chronic activation of the RAAS and sympathetic nervous system, leading to hypertrophy and irreversible fibrosis. Major cardiovascular guidelines classify HF into four stages: Stage A (at risk), Stage B (pre-HF), Stage C (symptomatic HF), and Stage D (advanced HF).

Current Landscape of Heart Failure Treatment

Current standard treatments for heart failure, including angiotensin-converting enzyme inhibitors (ACEIs), angiotensin receptor blockers (ARBs), β-blockers, and mineralocorticoid receptor antagonists (MRAs), primarily reduce cardiac workload and delay disease progression through neurohormonal modulation. Although these treatments alleviate symptoms, they cannot address the underlying loss of cardiomyocytes or reverse structural myocardial damage.

For patients with advanced heart failure, heart transplantation remains the only curative treatment, but its application is severely limited by the shortage of donor hearts. Ventricular assist devices (VADs) may be used as a bridge to transplantation or as destination therapy, but they carry risks of infection, thrombosis, bleeding, and device-related complications and cannot achieve true myocardial repair. Therefore, regenerative therapies capable of promoting myocardial revascularization and restoring cardiac contractile function are urgently needed.

Unmet Clinical Needs in Heart Failure

Although current standard treatments for heart failure—including the four foundational classes of guideline-directed medical therapy (GDMT), as well as device-based and surgical interventions—can effectively reduce cardiac workload and improve symptoms and prognosis, lost cardiomyocytes cannot be replaced or repaired because adult cardiomyocytes have limited endogenous regenerative capacity. Consequently, the five-year mortality rate for advanced heart failure remains high, highlighting an urgent clinical need for transformative therapies that can fundamentally repair the myocardium.

Figure. Current Therapeutic Options and Unmet Clinical Needs in Heart Failure

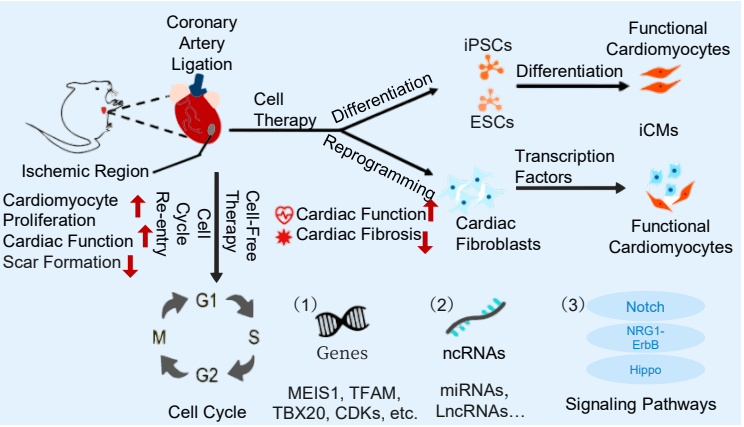
	Current Standard of Care (Foundational Four-Drug Regimen)	*Key Unmet Needs*
For heart failure with reduced ejection fraction (HFrEF), guideline-directed medical therapy (GDMT) includes an angiotensin receptor–neprilysin inhibitor (ARNI) (e.g., sacubitril/valsartan, or an ACEI/ARB), a β-blocker, a mineralocorticoid receptor antagonist (MRA), and a sodium-glucose cotransporter-2 (SGLT2) inhibitor (e.g., dapagliflozin and empagliflozin).		Adult cardiomyocytes are terminally differentiated cells with minimal endogenous regenerative capacity. The four foundational classes of GDMT and device-based interventions can provide compensatory benefits and reduce cardiac workload but cannot restore lost cardiomyocytes.
For HF patients with ventricular dyssynchrony or malignant arrhythmia risks, CRT or ICD is used to prevent sudden cardiac death; for Stage D advanced HF patients, LVAD or heart transplantation remains the definitive intervention.	*Device- and Surgical Interventions*	*Persistent Mortality Challenge*	Despite significant leaps in compensatory pharmacological therapies for heart failure over the past decade, the 5-year mortality rate for advanced heart failure remains persistently high, potentially exceeding 50%.

Source: Literature Review, Frost & Sullivan Analysis

Clinical Applications of Stem Cells — Heart Failure

Mechanisms of Stem Cell Therapy for Heart Failure

iPSC therapies promote myocardial regeneration by transplanting iPSC-derived cardiomyocytes into damaged myocardium through cardiac patches or intramyocardial injections. These cells may improve cardiac function by replenishing lost cardiomyocytes and through paracrine effects that inhibit apoptosis, promote angiogenesis, and reduce fibrosis and ventricular remodeling. iPSC technology also enables the large-scale production of off-the-shelf cardiomyocytes for sustained functional improvement.



Approved iPSC Therapy for Heart Failure

The scalable production of highly purified cardiomyocytes from induced pluripotent stem cells (iPSCs) provides a promising solution for achieving true myocardial regeneration in heart failure. The world's first approved iPSC-derived cardiac regenerative therapy, RiHEART developed by Cuorips, uses cardiomyocyte patches generated from healthy donor iPSCs to treat severe heart failure caused by ischemic cardiomyopathy, demonstrating the clinical feasibility of iPSC-based cardiac regeneration.

Japan Cuorips — RiHEART®

Product Overview

The world's first approved iPSC-derived cardiac regenerative therapy

RiHEART® was developed by Cuorips Inc., a startup spun out of Osaka University. Healthy donor-derived iPS cells are differentiated in vitro into functional cardiomyocytes and then cultured into a coin-sized tissue sheet containing approximately 100 million cells, known as a cardiomyocyte sheet. This "living patch" is surgically applied directly to the damaged surface of the heart rather than injected into the myocardium. The transplanted cardiomyocyte sheet acts primarily through paracrine effects, secreting growth factors that promote angiogenesis and inhibit myocardial fibrosis and ventricular remodeling, thereby improving cardiac function.

Approval Status

Pricing

Predicted >JPY 10 Million

Approval Date

Received conditional and time-limited marketing approval from Japan's MHLW in March 2026.

Indication

Severe Heart Failure

(Particularly Ischemic Cardiomyopathy)

Clinical Data

RiHEART® was approved based on a single-arm, open-label Phase I trial in eight patients with heart failure caused by severe ischemic cardiomyopathy.

- Safety: No serious adverse events, immune rejection, or tumor formation were observed, indicating an acceptable safety profile.
- Efficacy: All patients showed relief of fatigue, palpitations, and dyspnea; more than half improved in cardiac function (e.g., LVEF) and exercise capacity (6-minute walk distance).

Key Limitations: No placebo or control group, a very small sample size (n = 8), and limited follow-up time.

Representative iPSC Therapies for Heart Failure

HiCM-188 is the world's first iPSC-derived cell therapy for heart failure to enter Phase III clinical trials.

HELP Therapeutics—— HiCM-188

Product Overview

HiCM-188 is the world's first iPSC-derived cell therapy for heart failure to enter Phase III clinical trials.

HiCM-188 Injection, developed by HELP Therapeutics, is an allogeneic, off-the-shelf iPSC-derived cardiomyocyte therapy. Healthy donor-derived iPSCs are differentiated into functional cardiomyocytes and delivered to infarcted or damaged myocardium through grid-pattern intramyocardial injections guided by radionuclide imaging and cardiac MRI. The cells act by replenishing lost cardiomyocytes and through paracrine effects that promote angiogenesis and inhibit fibrosis.

Development Status

Latest Update

A pivotal clinical trial was published in May 2026, with a planned enrollment of 80 patients.

Indication

Severe Chronic Ischemic Heart Failure

Clinical Data

Clinical studies of HiCM-188 have generated several key findings.

- The world's first investigator-initiated trial (IIT) of an iPSC-based therapy for heart failure, conducted in collaboration with Professor Dongjin Wang's team at Nanjing Drum Tower Hospital, showed improvements in cardiac function sustained for four years, with no transplantation-related serious adverse events.
- Interim results presented at the 19th Qianjiang International Cardiovascular Conference in November 2025 showed that the Phase I dose-escalation and Phase II effective-dose expansion stages had been completed, supporting the therapy's feasibility and safety.

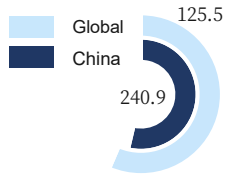
Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Parkinson's Disease

Parkinson's disease is a highly prevalent neurodegenerative disorder affecting middle-aged and older adults. Current pharmacological and surgical treatments can only alleviate symptoms and cannot prevent progressive neuronal loss, whereas iPSC-derived dopaminergic neural cells may restore neurological function through cell replacement.

Overview and Epidemiology of Parkinson's Disease

Parkinson's disease (PD) is a common neurodegenerative disease that progressively worsens with age. It is characterized by resting tremor, muscular rigidity, bradykinesia, and postural and gait abnormalities, often accompanied by mood disorders, sleep disorders, cognitive impairment, and autonomic dysfunction. PD results from the combined effects of genetic and environmental factors, with approximately 10% of patients having a family history. Multiple autosomal dominant and autosomal recessive mutations have been linked to familial Parkinson's disease. Its core pathogenic mechanism is the progressive degeneration and loss of dopaminergic neurons in the substantia nigra, causing dopamine depletion, striatal pathway dysfunction, and ultimately tremor and rigidity.



Prevalence of Parkinson's Disease: ~/100,000 Population

Unmet Clinical Needs in PD

Current therapies for Parkinson's disease (PD) cannot modify disease progression, provide inadequate control of motor complications and non-motor symptoms, are associated with significant adverse effects, and lack specific biomarkers for disease progression assessment.

Management of Motor Complications

Levodopa remains the standard therapy for Parkinson's disease (PD). However, up to 50% of patients develop motor fluctuations after three years or more of levodopa treatment. Levodopa may also induce abnormal gait patterns, freezing of gait (FOG), and dyskinesia, which is difficult to reverse once established.

Management of Non-Motor Symptoms

Non-motor symptoms (NMS), including pain, constipation, and sleep disorders, occur throughout Parkinson's disease (PD), involving both dopaminergic and non-dopaminergic systems and reducing patients' quality of life. Research on NMS pathophysiology, diagnosis, and treatment remains limited, with insufficient R&D investment.

Neuroprotection and Disease-Modifying Therapy

Currently, no therapy can halt or slow the progression of Parkinson's disease (PD). The development of disease-modifying therapies (DMTs) depends on specific biomarkers for disease progression, without which the disease-modifying efficacy of new therapies cannot be effectively evaluated.

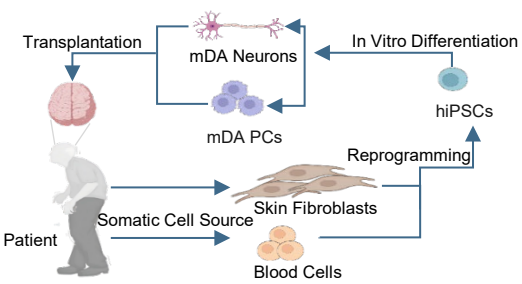
Novel Therapies with Fewer Adverse Effects

Reducing the adverse effects of conventional drugs is a key need in Parkinson's disease (PD) drug development. Conventional dopamine receptor agonists commonly cause nausea, orthostatic hypotension, hallucinations, somnolence, and impulse control disorders. Novel drugs with fewer adverse effects will be more competitive.

Mechanism of Stem Cell Therapy for PD

Somatic cells (e.g., skin fibroblasts or blood cells) from healthy donors or patients are reprogrammed into iPSCs, which are then differentiated into dopaminergic neural progenitor cells or mature dopaminergic neurons via directed differentiation. These cells are transplanted into the putamen of Parkinson's patients using minimally invasive stereotactic surgery. Once engrafted, the progenitors mature into functional dopaminergic neurons, replacing lost nigral neurons, restoring putaminal dopamine synthesis and secretion, and thus alleviating motor symptoms.

Figure. Mechanism of iPSC Therapy for Parkinson's Disease



Source: Literature Review, Frost & Sullivan Analysis

Sumitomo Pharma — AMCHEPRY® (raguneprocel)

AMCHEPRY® (raguneprocel) is indicated for improving motor symptoms in patients with Parkinson's disease who respond inadequately to existing therapies, including levodopa. The product consists of allogeneic iPSC-derived dopaminergic neural progenitor cells, which are transplanted into the putamen via stereotactic surgery. Following transplantation, the cells mature into functional dopaminergic neurons, replace lost dopaminergic neurons, and restore dopamine production.

The world's first approved iPSC-derived regenerative medicine product

Pricing

Approximately JPY 55.3 million (approximately RMB 2.38 million)

Approval Status

Received conditional and time-limited marketing approval from Japan's MHLW in March 2026.

Clinical Data

- Approval was primarily based on an investigator-initiated, open-label, dose-escalation Phase I/II clinical trial conducted at Kyoto University Hospital. Seven patients aged 50–69 years with advanced Parkinson's disease received bilateral putaminal transplantation of iPSC-derived dopaminergic neural progenitor cells.
- At 24 months, motor function improved in 4 of 6 evaluable patients. Dopamine synthesis (assessed by PET imaging) increased by 63.5% in the high-dose group versus 7.0% in the low-dose group. No serious tumor formation or other severe adverse events were observed during the study, although long-term safety requires further post-marketing evaluation.

Clinical Applications of Stem Cells — Parkinson's Disease

■ Approved and Representative Investigational iPSC Therapies for PD

With the conditionally-approved commercialization of Sumitomo Pharma's AMCHEPRY® (raguneprocel) in Japan and encouraging clinical follow-up data reported by iRegene Therapeutics, XellSmart, and Nuwacell, iPSC therapies are advancing the treatment of PD from symptomatic management toward the commercial application of disease-modifying therapies.

Figure. Representative iPSC Therapies for PD

iRegene Therapeutics — NouvNeu001 Injection

Product Overview

NouvNeu001 Injection is an allogeneic, off-the-shelf iPSC-derived cell therapy independently developed by iRegene Therapeutics using its proprietary "AI + chemical induction" platform. Through chemical induction, iPSCs are differentiated into functionally enhanced dopaminergic progenitor cells of A9 subtype. These cells are transplanted into the putamen through single trajectory per hemisphere, and are intended to treat PD and other disorders.

Technology Platform

The proprietary "AI + chemical induction" platform enables targeted modification and optimization of cellular functions without gene editing.

Development Status

- Clinical trial clearances obtained in both China and the United States; multiple FDA special exemptions granted
- FDA RMAT Designation granted
- FDA Fast Track Designation received

PD: The world's first off-the-shelf cell therapy to receive a special exemption from the FDA and the only iPSC product to receive both FDA Fast Track Designation (FTD) and Regenerative Medicine Advanced Therapy (RMAT) Designation. Phase II dosing has been completed in China, and a Phase II trial is ongoing in the United States. Multiple System Atrophy (NouvNeu004): A Phase I/III registrational trial is ongoing in China, and a Phase I trial is ongoing in the United States. The product has received a special exemption from the FDA. Early-Onset PD (NouvNeu003): A Phase I registrational trial has been completed in China.

Clinical Data

At 12 months after a single administration, the mean Movement Disorder Society–Unified PD Rating Scale Part III (MDS-UPDRS Part III) score improved by 30.6 points in the OFF state, representing a 52.82% improvement, and by 12.9 points in the ON state, representing a 54.67% improvement, outperforming existing therapies. The treatment demonstrated a favorable safety profile and successfully passed multiple reviews by safety committees in China and the U.S..

XellSmart — XS411 Injection

Product Overview

XS411 Injection is an allogeneic, off-the-shelf iPSC-derived dopaminergic neural progenitor cell therapy independently developed by XellSmart. Healthy donor-derived iPSCs are differentiated into dopaminergic neural progenitor cells, which are transplanted into the putamen via minimally invasive stereotactic surgery. The indications for which the product has entered clinical development include idiopathic parkinson's disease (primary PD), early-onset parkinson's disease(EOPD), and multiple system atrophy–parkinsonian type (MSA-P).

Trial Design

For primary PD, a nationwide, multicenter Phase II registrational trial led by the National Center for Neurological Disorders is conducting. The trial uses an internationally recognized randomized controlled PROBE design, which provides a higher level of evidence than a single-arm study. Enrollment in the Phase II trial has been completed.

Development Status

- FDA Fast Track Designation received
- Clinical trial clearances obtained in both China and the United States; multiple FDA special exemptions granted
- Blinded randomized controlled trial design providing a high level of clinical evidence

Primary PD: Clinical trial clearances obtained in China and the U.S., with an FDA exemption and Fast Track designation granted; a nationwide multicenter Phase II registrational trial is ongoing. MSA-P: Clinical trial clearances obtained in China and the U.S.; a Phase I/II registrational trial is ongoing. Early-Onset PD: A Phase II registrational trial is ongoing in China.

Clinical Data

Primary PD: XellSmart is leading an Innovative Drug Research and Development National Science and Technology Major Project. Before formal registrational trials, it completed GCP- and GMP-compliant cell therapy in multiple patients, including the first such case in China. Follow-up of more than 40 XS411-treated participants showed significant improvements from baseline in OFF-state MDS-UPDRS Part III motor scores, ON time without troublesome dyskinesia, and quality of life, with no adverse events related to the transplanted cells.

Parkinson's Disease iPSC Therapy

Nuwacell — NCR201 Injection

Product Overview

NCR201 Injection is an off-the-shelf, high-efficiency iPSC-derived dopaminergic neural progenitor cell (iDAP) product independently developed by Nuwacell for the treatment of PD. Healthy donor-derived iPSCs are directionally differentiated into iDAPs using an iteratively optimized technology. The cells are then transplanted into the putamen via minimally invasive stereotactic surgery using one needle tract per hemisphere, improving procedural safety. Following transplantation, the cells mature in vivo into functional dopaminergic neurons, fundamentally replacing lost neurons and restoring dopamine synthesis and secretion.

Development Status

- Low Immunogenicity
- High In Vivo Conversion Rate Following Transplantation

The product has demonstrated excellent Phase I data, and its Phase II clinical trial was publicly registered in December 2025.

Clinical Data

Clinical trials are underway at several leading research hospitals across China, and multiple patients have received treatment. The results showed a favorable safety profile, with improvements in OFF time and disease stage in multiple patients, substantially enhanced cerebral dopaminergic PET–CT signals, and reduced doses of oral dopaminergic medications, demonstrating a trend toward disease reversal.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Knee Osteoarthritis

Current treatments for KOA can relieve pain but cannot reverse cartilage degeneration. MSCs and iMSCs may improve joint structure through anti-inflammatory effects and cartilage repair.

Overview and Epidemiology of Knee Osteoarthritis

Knee osteoarthritis (KOA) is a heterogeneous chronic degenerative joint disease characterized primarily by progressive articular cartilage degeneration. The pathological process also affects the synovium, causing synovitis, as well as the ligaments, menisci and subchondral bone. Clinical manifestations include joint pain, stiffness, swelling and restricted mobility. Advanced disease is often accompanied by anxiety and depression, substantially impairing physical function and quality of life. The knee is the most commonly affected joint in OA. With population ageing and rising obesity rates, the prevalence of KOA is expected to increase.

Global prevalence
~4,500/100,000 population

Current Treatments and Unmet Clinical Needs in KOA

Current KOA treatment mainly follows a stepwise, individualized, symptom-oriented approach aimed at relieving pain, delaying disease progression and restoring function. However, effective therapies that fundamentally modify the disease course remain lacking, resulting in substantial unmet clinical needs.

Core Treatment: Core treatment is a first-line intervention applied throughout the course of KOA and across all disease stages. Patient education promotes joint protection, while weight reduction decreases mechanical stress on the knee. Physical therapies, such as heat therapy and ultrasound, together with low-intensity aerobic exercise, improve joint circulation and maintain muscle strength. These measures can alleviate mild symptoms.

Pharmacological Treatment: Treatment may progress from topical non-steroidal anti-inflammatory drugs (NSAIDs) to oral NSAIDs or acetaminophen, followed by intra-articular injections such as corticosteroids or hyaluronic acid. These treatments may provide short-term pain relief and reduce synovial inflammation.

Surgical Treatment: Surgery is generally used for patients with moderate-to-advanced KOA who have severe structural joint damage and have not responded to conservative treatment. Stepwise surgical options include minimally invasive arthroscopic debridement, corrective osteotomy, and unicompartmental or total knee arthroplasty for end-stage disease. Arthroscopy and osteotomy may temporarily improve the joint environment and delay joint replacement, while total knee arthroplasty is a highly invasive procedure associated with risks such as infection and prosthetic wear.

Lack of disease-modifying treatment	• Existing medicines cannot repair damaged cartilage, prevent cartilage-matrix degradation or inhibit abnormal osteophyte formation. They may slow disease progression but cannot halt or reverse continuing structural joint damage.
Initial "treatment gap"	• A transition period of 10–20 years may occur between the initial diagnosis of KOA following the onset of joint pain and the development of joint damage requiring surgery. During this period, patients may repeatedly experience pain while walking, sharp pain when using stairs and pain at rest during the night, with persistent limitations in daily activities and physical mobility.
Inadequate pain control	• NSAIDs are the principal first-line analgesics used for KOA. Long-term oral use may irritate the gastrointestinal mucosa and increase the risk of gastrointestinal adverse events. Prolonged treatment may also affect vascular endothelial function, blood pressure and coagulation, increasing the risk of cardiovascular events such as hypertension, myocardial infarction and ischemic stroke.

Representative iPSC Product for KOA

iMSCs represent a potentially disease-modifying biological therapy for KOA through multiple mechanisms. In addition to retaining strong regenerative potential, iMSCs can be continuously generated as highly homogeneous and biologically active cells by leveraging the extensive expansion capacity of iPSCs, thereby overcoming donor dependence and cellular senescence associated with conventional adult-tissue-derived MSCs.

Figure. Analysis of Clinical Data for a Representative Investigational Product

Investigational iPSC Pipeline
Nuwacell — NCR100

Product Overview

NCR100 Injection is the first iPSC-derived MSC-like cell product cleared for clinical trials in China and is administered by intra-articular injection directly to the affected knee joint. It is derived from a single monoclonal iPSC line, supporting consistent and controllable product quality. Through immunomodulatory and paracrine effects, NCR100 is intended to reduce inflammation and relieve pain, providing a new pathway for applying stem cell technology to osteoarticular diseases.

Development Status

The Phase II clinical trial of NCR100 in China was posted in May 2025, and enrolment was completed in November 2025.

Clinical Data

The Phase I clinical trial of NCR100 has been completed, with the following results:

- **Safety:** NCR100 demonstrated a favourable safety profile, with no serious adverse events related to the cell product reported.
- **Efficacy:** Positive efficacy signals were observed, with consistent improvements across clinically relevant measures. NCR100 showed the potential to alleviate clinical symptoms and provide clinical benefit.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Knee Osteoarthritis

Representative MSC Products for Knee Osteoarthritis

The therapeutic mechanism of MSC therapy for knee osteoarthritis (KOA) is based on immunomodulation and paracrine effects, which reshape an anti-inflammatory and pro-regenerative joint microenvironment. This approach is intended to slow joint degeneration and may have disease-modifying potential, rather than providing symptomatic pain relief alone.

Anti-Inflammation and Pain Relief — Suppressing Inflammation and Restoring Immune Homeostasis	Cartilage Protection and Structural Repair — Preventing Degeneration and Promoting Regeneration	Microenvironment Remodeling and Long-Term Benefits — Achieving Disease Modification
MSCs suppress key pro-inflammatory cytokines (TNF-α, IL-1β, IL-6, and IL-17), reducing synovial inflammation and cartilage degradation. They promote macrophage polarization from M1 to reparative M2, decrease local nociceptive sensitivity, and rapidly relieve joint pain, swelling, and stiffness while improving physical function.	Through paracrine secretion of growth factors (e.g., TGF-β, IGF-1, and FGF) and extracellular vesicles, MSCs promote chondrocyte proliferation and type II collagen and proteoglycan synthesis, while inhibiting MMPs and other degradative enzymes, thereby reducing cartilage matrix loss and improving cartilage structure and thickness.	Unlike conventional analgesic and viscosupplement therapies that primarily relieve symptoms, MSCs improve the hypoxic, inflammatory, and fibrotic joint microenvironment, reduce subchondral bone lesions and synovitis progression, delay or avoid joint replacement, and achieve disease modification through structural improvement.

Figure. Clinical Data Analysis of Approved and Representative Pipeline Products

MEDIPOST —— CARTISTEM®

Product Overview

- CARTISTEM® is the world's first approved allogeneic hUCB-MSC therapy, developed by MEDIPOST for osteoarthritis patients with ICRS Grade IV knee cartilage defects.
- Manufactured using MEDIPOST's proprietary cord blood stem cell platform, CARTISTEM® comprises hUCB-MSCs expanded from umbilical cord blood mononuclear cells and formulated with a hyaluronic acid hydrogel carrier. After implantation into cartilage defects, the MSCs exert immunomodulatory and paracrine effects by suppressing intra-articular inflammation, relieving pain, and promoting cartilage regeneration. As a disease-modifying osteoarthritis drug, it improves clinical symptoms while slowing or preventing irreversible cartilage degeneration.

Approval Status

- Approved by the Korean Ministry of Food and Drug Safety (MFDS) in January 2012 as the world's first approved umbilical cord blood stem cell therapy.

Pricing

- KRW 8–10 million per vial

Clinical Data

- Since its launch in South Korea in 2012, CARTISTEM® has treated over 35,000 patients with knee osteoarthritis. More than a decade of follow-up has shown no severe immune rejection or tumor formation, supporting the long-term safety of the allogeneic hUCB-MSC platform.
- Multiple 2-year follow-up studies showed durable hyaline cartilage regeneration, sustained pain reduction, delayed total knee arthroplasty, and long-term structural repair.

Representative MSC Products
ElpasBio — AlloJoin®

Product Overview

AlloJoin ® (lotazadromcel) is an investigational allogeneic human adipose-derived mesenchymal progenitor cell (haMPC) therapy for knee osteoarthritis. Through the paracrine and immunomodulatory properties of MSCs, it suppresses inflammation and promotes cartilage repair, providing sustained pain relief and functional improvement beyond conventional symptomatic therapies.

Development Status *China's First Stem Cell Therapy for KOA to Enter Phase III Clinical Development*

In June 2026, ElpasBio announced completion of patient enrollment for the pivotal Phase III trial of AlloJoin® in May.

Clinical Data

AlloJoin® demonstrated positive results in Phase I and Phase II clinical trials, with the following findings:

- **Safety:** Both Phase I and Phase II clinical trials demonstrated favorable safety and tolerability.
- **Efficacy:** The Phase II clinical trial showed a statistically significant improvement in the primary WOMAC endpoint and evidence of cartilage repair.

Representative MSC Products
Boyalife — Wharton's Jelly MSC Injection

Product Overview

Developed by Boyalife Pharma, this pipeline is an allogeneic Wharton's jelly-derived MSC (WJ-MSC) therapy. Using Boyalife's proprietary GMP-compliant perinatal stem cell manufacturing platform, WJ-MSCs are isolated from discarded umbilical cord Wharton's jelly and expanded using a standardized process to produce an off-the-shelf MSC therapy. It does not require patient-specific cell matching and can be administered by intra-articular injection.

Development Status

In December 2025, the company announced that the product had entered Phase II clinical development.

Clinical Data

The Phase I clinical trial demonstrated positive results, showing that:

- **Safety:** At 48 weeks, the therapy demonstrated favorable safety and tolerability. Only a few participants experienced transient mild joint soreness or local swelling that resolved spontaneously.
- **Preliminary efficacy:** Significant reductions in VAS pain scores, improved WOMAC scores, and imaging evidence of slowed cartilage degeneration were observed across all dose groups.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Stroke

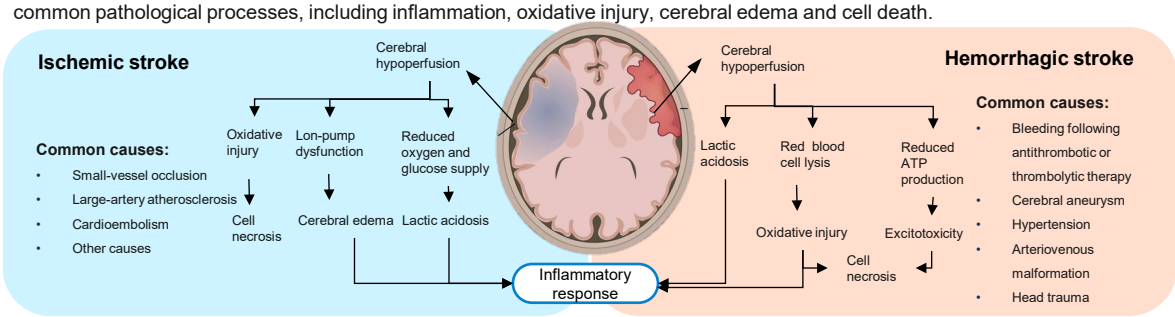
Stroke can be broadly categorized as ischemic or hemorrhagic. Significant unmet needs remain in both acute treatment and post-stroke recovery, while stem cell therapies offer a new approach to stroke treatment and rehabilitation.

Overview of Stroke

Stroke, commonly known as a cerebrovascular accident, is a medical emergency caused by the sudden interruption of blood supply to part of the brain. Its two major types are ischemic stroke, caused by vascular occlusion, and hemorrhagic stroke, caused by vascular rupture. Common risk factors include hypertension, atrial fibrillation, smoking, diabetes, and hyperlipidemia. Typical acute symptoms include facial drooping or deviation of the mouth, arm weakness, speech difficulties, impaired balance, and visual disturbances. Timely treatment is critical to preventing permanent brain damage and disability.

Global prevalence: ~1.24%

Ischemic stroke is primarily caused by arterial occlusion, which reduces cerebral blood flow and deprives brain cells of oxygen and glucose, leading to energy-metabolism failure. Hemorrhagic stroke is most commonly caused by rupture of a hypertensive vessel, cerebral aneurysm or arteriovenous malformation. The resulting bleeding compresses brain tissue, increases intracranial pressure and releases neurotoxic blood products. Although the two subtypes have different pathogenic pathways, they ultimately converge on common pathological processes, including inflammation, oxidative injury, cerebral edema and cell death.



Current Diagnosis and Treatment of Stroke

Diagnosis combines clinical symptoms with neuroimaging to distinguish ischemic stroke from hemorrhagic stroke.

	Stage	Core Treatment Objective	Main Treatment Modalities
Ischemic stroke	Acute (≤2 weeks)	Rapid recanalization; salvage ischemic penumbra	IV thrombolysis ≤4.5 h (up to 24 h in selected patients); mechanical thrombectomy for large-vessel occlusion
	Recovery (weeks–1 year)	Promote neurological remodeling; reduce sequelae	Rehabilitation, neuromodulation; cell therapy in clinical development
	Stable (≥1 year)	Restore and maintain neurological function	Rehabilitation, neuromodulation; cell therapy in clinical development
Hemorrhagic stroke	Acute (≤2 weeks)	Hemostasis; reduce ICP; prevent rebleeding	BP/ICP control; hematoma evacuation when indicated
	Recovery (weeks–1 year)	Prevent rebleeding; promote neurological recovery; control BP	Rehabilitation, neuromodulation, chronic-disease management; cell therapy in clinical development
	Stable (≥1 year)	Preserve function; delay decline; reduce sequelae	Maintenance rehabilitation and chronic-disease management

Based on disease course, stroke is divided into acute, recovery, and stable phases. The acute phase, within two weeks of onset, focuses on lifesaving treatment and stabilization. Ischemic stroke requires urgent intravenous thrombolysis or mechanical thrombectomy, while hemorrhagic stroke requires blood pressure control, reduction of intracranial pressure, or surgical hematoma removal. The recovery phase focuses on rehabilitation, complication prevention, and secondary prevention to restore motor, speech, and cognitive functions. Pharmacological or interventional treatment may improve cerebral circulation after ischemic stroke, while risk-factor control supports hematoma resorption and tissue repair after hemorrhagic stroke. The stable phase, from one to several years after onset, focuses on restoring or maintaining neurological function and delaying functional decline through rehabilitation, neuromodulation, and cell therapy in clinical development.

Source: Literature Review, Frost & Sullivan Analysis

Clinical Applications of Stem Cells — Stroke

Unmet Clinical Needs in Stroke

Current standard treatments for acute ischemic stroke are dependent on a narrow therapeutic time window. A major unmet need is that many patients miss the optimal treatment opportunity because they present too late. Significant gaps also remain during rehabilitation. Rehabilitation resources are scarce and unevenly distributed, particularly in developing countries and primary-care settings. Financial constraints, transportation difficulties, and shortages of rehabilitation professionals frequently lead to treatment interruption. In addition, conventional rehabilitation approaches are limited by uniform treatment models, slow progress, and heavy reliance on patient engagement, with limited effectiveness in patients with severe or long-standing disease.

Mechanisms of Stem Cell Therapy for Stroke

iPSC Therapy: Ischemic stroke causes neurological deficits through the loss of neurons and glial cells. In animal models, transplanted iPSC-derived neural progenitor cells (NPCs) promote recovery through cell replacement, neuroprotection, angiogenesis, synapse formation, and endogenous neurogenesis. NPCs can differentiate into neurons, astrocytes, and oligodendrocytes, replace lost cells, and integrate into neural circuits. Transplanted cells also secrete neurotrophic factors such as BDNF, supporting neuronal survival and synaptic plasticity. They reduce neuronal apoptosis, microglial activation, infarct volume, and pro-inflammatory cytokines—including IL-1β, TNF-α, and IL-6—while improving cerebral metabolism, white matter integrity, and perfusion and alleviating blood–brain barrier disruption and immune-cell infiltration. By secreting VEGF, transplanted cells promote angiogenesis. They can also extend axons into ischemic regions, form synapses with host neurons, and stimulate the proliferation and migration of endogenous NPCs, thereby promoting synaptic remodeling and endogenous neurogenesis.

MSC Therapy: MSCs treat stroke primarily through paracrine effects rather than direct differentiation into neural cells. They secrete exosomes and soluble bioactive factors that promote neural repair through multiple pathways.

MSCs induce anti-inflammatory M2 macrophage polarization, suppress excessive T-cell activation, and regulate cytokine networks, reducing post-stroke secondary brain injury. MSC-secreted TGF-β1, IL-1Ra, miR-124, and miR-145 inhibit neuronal apoptosis and pyroptosis and preserve blood–brain barrier integrity by maintaining ZO-1, occludin, and claudin-5 expression.

MSCs also release VEGF, HGF, and SDF-1α to promote angiogenesis, endogenous neural stem cell proliferation and differentiation, and synaptic plasticity. They may also transfer mitochondria to damaged neurons through tunneling nanotubes, providing energy support and promoting neural repair.

Figure. Representative Stem Cell Therapy Products for Stroke

Huode —— hNPC01

Product Overview

hNPC01 is an allogeneic, off-the-shelf iPSC-derived forebrain neural progenitor cell product developed by Huode for stable-phase post-ischemic stroke hemiplegia. Using second-generation RONA technology, healthy donor-derived iPSCs are differentiated into forebrain neural progenitor cells and stereotactically transplanted near brain lesions. The cells then differentiate into functional cortical neurons and glial cells, promoting neural circuit reconstruction through cell replacement and paracrine trophic support.

Development Status *FDA Fast Track and RMAT designations Granted*

The Phase I clinical trial has been completed. The FDA has agreed that, based on the Chinese Phase I data, the program may proceed seamlessly into a U.S. Phase III clinical trial following bridging and dose-confirmation study.

Clinical Data

hNPC01 demonstrated a manageable safety profile in Phase I studies through 12 months endpoint observation of 20 patients with ischemic stroke sequelae, extending to 18–24 months. Transplanted neural cells formed new tissue within the infarcted areas and remained viable at 24 months. In the target subgroup, the mean FMMS score improved by 16 points at 12 months, with continued improvement through 18–24 months and response rate exceeded 92%. More than half of the participants achieved a lower disability grade, and most participants improved in language. Some also gained substantial improvements in daily functioning, enabling independent living and even return to work.

Cellgenes —— CG-BM1

Product Overview

CG-BM1 Injection is an allogeneic human bone marrow-derived MSC product independently developed by Guangzhou Cellgenes Biotechnology Co., Ltd. MSCs isolated from healthy adult donors are expanded in vitro and administered intravenously. Through immunomodulatory and paracrine effects, they suppress inflammation, regulate the local immune microenvironment, promote vascular repair and angiogenesis, inhibit neural cell apoptosis, and may support neurological repair and functional reconstruction by activating endogenous neural stem cells.

Development Status

In June 2025, a Phase IIa clinical trial of CG-BM1 was publicly registered, with a planned enrollment of 18 patients. CG-BM1 is the first domestically developed bone marrow MSC product to enter clinical development in China for this indication.

Clinical Data

The Phase I clinical trial has been completed, with results showing that:

- CG-BM1 Injection demonstrated a favorable safety profile across different dose levels, with no serious adverse events reported, and showed preliminary positive effects on neurological recovery in patients with stroke.

Preclinical studies also showed that:

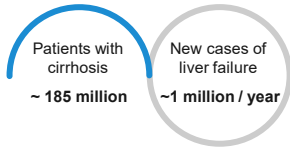
- CG-BM1 improved neurological dysfunction, reduced cerebral infarct size, and promoted vascular repair in damaged regions.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

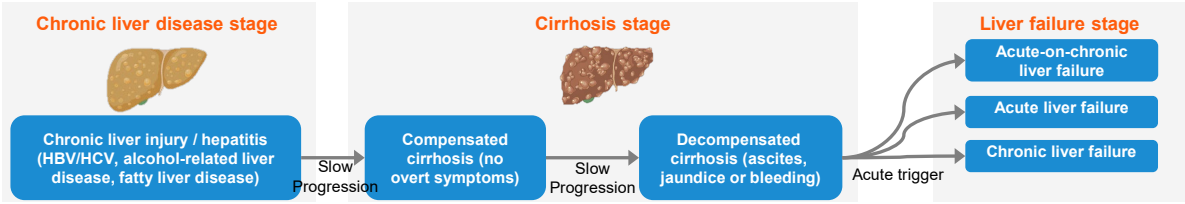
Clinical Applications of Stem Cells — Liver Cirrhosis and Liver Failure

Cirrhosis can progress from chronic liver disease to liver failure. Current therapies have limited efficacy, while liver transplantation is constrained by severe donor shortages. Stem cell and exosome therapies offer new approaches to liver repair and functional restoration.

Overview and Pathogenesis of Cirrhosis and Liver Failure



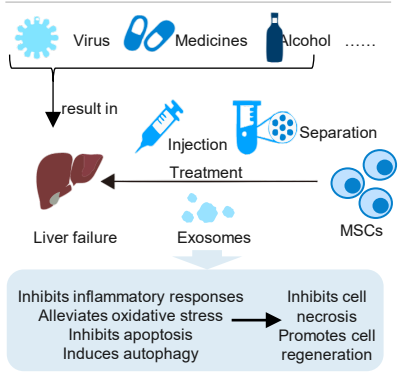
Liver cirrhosis is the end stage of chronic liver injury, characterized by hepatocyte necrosis, fibrosis, and pseudo lobule formation. Persistent hepatic stellate cell activation drives myofibroblast transformation and excessive extracellular matrix production, causing structural damage. Liver failure is severe hepatic dysfunction characterized by jaundice, coagulopathy, hepatorenal syndrome, hepatic encephalopathy, and ascites. Gut–liver axis dysfunction can trigger systemic inflammation and accelerate organ deterioration. Acute-on-chronic liver failure (ACLF) is a critical subtype triggered by acute insults in patients with cirrhosis, leading to multiorgan failure and high short-term mortality.



Treatment Landscape & Unmet Needs

Current cirrhosis management is largely etiologic, including long-term antivirals for HBV, alcohol abstinence and intensive metabolic control for MASH, supplemented by stage-specific management of complications. While these measures may slow disease progression, they cannot reverse established fibrosis or pseudo lobules or restore lost hepatic reserve. Liver failure lacks effective curative medical therapy, while liver transplantation remains the only definitive treatment but is constrained by severe donor shortages and high costs. Regenerative therapies capable of repairing liver tissue and restoring functional reserve are therefore urgently needed.

Figure. Mechanism of Stem Cell-Derived Exosome Therapy for Liver Failure



Emerging Stem Cell Paracrine-Based Technologies for Liver Injury

MSC-derived exosomes are nanoscale extracellular vesicles that carry bioactive molecules and regulate the hepatic immune microenvironment and cell fate through two coordinated mechanisms. First, exosomal miRNAs such as miR-223 and miR-146a enter macrophages and target pathways including NLRP3 and TRAF6/IRAK1, promoting polarization from pro-inflammatory M1 to anti-inflammatory M2 macrophages. Together with surface anti-inflammatory factors such as IL-10, they suppress excessive immune-cell activation and reduce hepatocyte injury. Second, exosomes deliver molecules such as miR-423-5p into damaged hepatocytes. miR-423-5p suppresses ZBP1 expression, thereby blocking ZBP1-mediated hepatocyte pyroptosis through the caspase-1/GSDMD pathway. Together, these mechanisms suppress inflammation, promote cell survival, and support liver repair and regeneration. Clinical studies involving blood purification and IV administration are underway in China.

Clinical-Stage Products Based on Stem Cell Paracrine Mechanisms

Unisum Biotech —— MSCs for Blood Purification & PT-MSCs-EV Injection

Product Overview

The world's first stem cell drug suitable for hybrid bioartificial liver systems: "MSCs for Blood Purification" ; The world's first stem cell-derived exosome product approved for exploratory clinical research in liver failure

Both products were independently developed by Unisum. Building on the concept of functional replacement, the company introduced inflammation control and proposed a new rescue approach based on the paracrine mechanisms of stem cells. Clinical exploration has been conducted through artificial liver support or intravenous infusion. Exosomes are nanoscale vesicles measuring 30–150 nm in diameter and carrying bioactive molecules such as proteins, mRNAs, and miRNAs. Following administration through artificial liver support or intravenous infusion, the exosomes act primarily through paracrine effects to repair impaired liver function.

Development Status

Latest Progress

MSCs for Blood Purification: A Phase I registrational clinical trial has been completed.

PT-MSCs-EV Injection: An exploratory clinical study in humans has been completed.

Indications

Acute-on-chronic liver failure (ACLF)

Clinical data

MSCs for Blood Purification: A Phase I registrational clinical trial has been completed. Fifteen patients with liver failure were enrolled, with a mean baseline Model for End-Stage Liver Disease (MELD) score of 26.667. The 90-day survival rate was 100%, and the transplant-free survival rate was 94.7%.

PT-MSCs-EV Injection: Preliminary results were reported from an exploratory clinical study completed at Zhujiang Hospital of Southern Medical University. Among 20 participants with liver failure, the survival rate was 85% at Week 4 and 70% at Week 12. No drug-related serious adverse events occurred during the study. This was the first completed human clinical study worldwide to report results of stem cell-derived exosome therapy for liver failure.

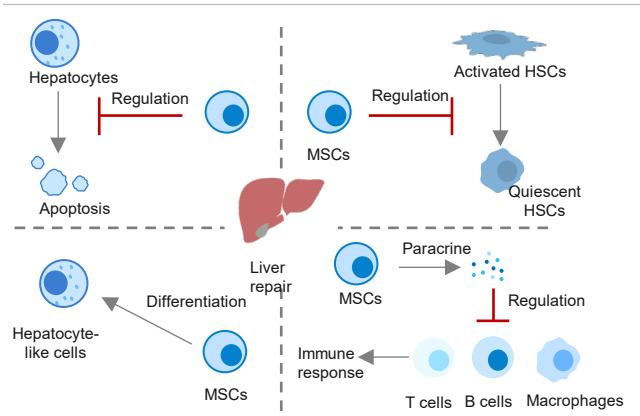
Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Liver Cirrhosis and Liver Failure

■ Mechanisms of Stem Cell Therapy for Cirrhosis

The core mechanisms of MSC therapy for liver cirrhosis are its potent immunomodulatory and antifibrotic effects. Through paracrine signaling, MSCs secrete soluble factors—including hepatocyte growth factor (HGF), bone morphogenetic protein 7 (BMP-7), and prostaglandin E2 (PGE2)—and extracellular vesicles containing bioactive molecules such as microRNAs. These factors directly inhibit hepatic stellate cell activation and their transformation into myofibroblasts, thereby reducing the excessive deposition of collagen and other extracellular matrix components. MSCs also induce the polarization of pro-inflammatory M1 macrophages toward the anti-inflammatory M2 phenotype and regulate T cells, dendritic cells, and other immune cells, thereby remodeling the hepatic immune microenvironment and limiting chronic inflammatory injury. Through these coordinated mechanisms, MSCs can alleviate or even reverse the progression of liver fibrosis and cirrhosis and improve liver function.

Figure. Mechanisms of Stem Cell Therapy for Cirrhosis



In liver failure, MSCs act primarily by rapidly suppressing excessive immune-inflammatory responses and promoting hepatocyte survival and regeneration following acute injury. Paracrine factors such as PGE2, IL-10, and IDO suppress the excessive activation of infiltrating T cells, natural killer T cells, and macrophages, as well as their production of cytotoxic factors such as TNF-α and IFN-γ, thereby reducing extensive hepatocyte necrosis caused by the "inflammatory storm." MSC-secreted growth factors, including HGF and VEGF, also inhibit hepatocyte apoptosis and promote hepatocyte proliferation and survival, creating favorable conditions for liver repair.

■ MSC Products Cleared for Clinical Trials in Liver Cirrhosis

MSCs can be manufactured at scale in vitro, possess strong immunomodulatory activity, and do not require donor–recipient matching, providing a potential breakthrough toward achieving true liver regeneration in liver failure.

Cellgenes —— CG-BM1

Product Overview

China's first bone marrow MSC drug cleared for clinical trials in ACLF; the Phase II clinical trial was completed in January 2026.

CG-BM1 Injection, an allogeneic human bone marrow-derived MSC injection, is a core product independently developed by Guangzhou Cellgenes Biotechnology Co., Ltd. MSCs isolated from the bone marrow of healthy adult donors are expanded at scale in vitro and administered intravenously. The transplanted MSCs act primarily through immunomodulation by suppressing pro-inflammatory cytokine secretion and increasing anti-inflammatory cytokine levels, thereby reducing excessive hepatic inflammation. CG-BM1 can also improve liver function and reverse liver fibrosis in ACLF animal models.

Development Status

Latest update

The Phase II clinical trial was completed in January 2026

Indications

ACLF
Ischemic stroke
Sarcopenia, etc.

Clinical Data

Early clinical studies conducted by the research team showed that:

- Bone marrow MSC therapy increased the survival rate of patients with ACLF **from 55.6% to 73.2%.**
- The completed Phase I clinical trial provided preliminary evidence of the safety and efficacy of MSC therapy for ACLF, **with a 90-day survival rate of 90%.**

Vcanbio —— VUM02

Product Overview

China's first umbilical cord-derived mesenchymal cell product to enter a Phase Ib/II clinical trial for decompensated liver cirrhosis

VUM02 Injection is an off-the-shelf cell therapy product independently developed by Vcanbio. It consists of human umbilical cord-derived mesenchymal cells and is cryopreserved in liquid nitrogen for long-term storage. Following thawing, it can be administered directly by intravenous infusion. Its core mechanisms involve immunomodulation, antifibrotic effects, and tissue repair.

To date, VUM02 Injection has received IND clearances for 10 indications, the highest number among MSC products in China. Its liver disease indications include decompensated liver cirrhosis and acute-on-chronic liver failure. Four nationally registered stem cell clinical research projects evaluating VUM02 in liver cirrhosis were previously conducted.

Clinical Data

Previous registered clinical studies of VUM02 Injection have generated several key findings. The results showed that the product was well tolerated and demonstrated a favorable safety profile in patients with decompensated liver cirrhosis. An optimal dose was explored, and a preliminary efficacy trend was observed.

No drug-related serious adverse events occurred in any participant, and the overall safety profile was acceptable. Improvements were observed in MELD scores and liver function parameters, including albumin, bilirubin, and prothrombin time. The findings were published in Signal Transduction and Targeted Therapy (STTT).

Latest Update

On April 21, 2026, the first participant was enrolled and dosed in the Phase Ib/II clinical trial of VUM02 Injection for decompensated liver cirrhosis, marking the product's entry into the key clinical validation stage. It is currently the most advanced product of its type for this indication.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Idiopathic Pulmonary Fibrosis

Idiopathic pulmonary fibrosis is a progressive and fatal interstitial lung disease with a poor prognosis. Existing drugs can only slow disease progression and are associated with substantial adverse effects, while lung transplantation is subject to significant practical limitations. Accordingly, major unmet clinical needs remain.

Overview of Idiopathic Pulmonary Fibrosis

Idiopathic pulmonary fibrosis (IPF) is a chronic, progressive, and fatal fibrotic interstitial lung disease of unknown cause that is confined to the lungs. It predominantly affects men over 50 years of age and long-term smokers, affecting approximately 3 million people worldwide, with incidence increasing with age. The median survival after diagnosis is only 2.5–3.5 years, and mortality during acute exacerbations is extremely high. Its fatality and survival outcomes are worse than those of most intermediate- and advanced-stage malignancies. Major clinical manifestations include progressively worsening exertional dyspnea, persistent dry cough, characteristic crackles over the lower lung fields, and digital clubbing. As ventilation and gas exchange progressively and irreversibly deteriorate, most patients eventually die from respiratory failure or cor pulmonale caused by severe hypoxemia.

Global prevalence:

~ 35.1/100,000 population

Current Diagnosis and Treatment of IPF and Unmet Clinical Needs

Only three oral small-molecule drugs are currently approved worldwide to slow IPF progression: nintedanib, pirfenidone, and nerandomilast. However, long-term multicenter follow-up has shown that these agents only partially slow the decline in vital capacity and can neither halt disease progression nor reverse established fibrotic scarring. Patients may also require dose reduction or treatment discontinuation because of adverse effects such as severe diarrhea, liver dysfunction, nausea, and photosensitivity. Lung transplantation remains the only curative option, but its use is severely limited by high costs, donor shortages, and poor tolerability among elderly patients, leaving most patients with very limited treatment options.

Applications and Representative Products of MSC Therapy for IPF

MSCs act in IPF mainly through paracrine-mediated immunomodulatory and antifibrotic effects. They secrete soluble factors—including IL-1Ra, PGE2, and IDO-1—and extracellular vesicles that regulate macrophage polarization, inhibit T-cell maturation and dendritic cell activation, and reduce B-cell proliferation.

Figure. Mechanisms of MSC Therapy for IPF

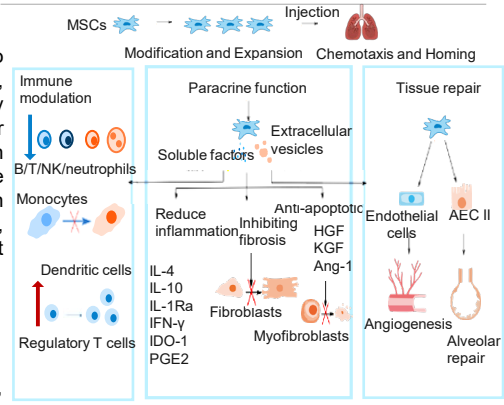


Image source: Therapeutic Applications of Mesenchymal Cells in Idiopathic Pulmonary Fibrosis

MSCs also regulate the MMP/TIMP ratio to reduce collagen deposition and secrete growth factors such as HGF and KGF to promote alveolar epithelial repair and vascular regeneration. However, only early-stage clinical trials have evaluated their safety and preliminary efficacy, and their therapeutic effects require further validation.

Sino Biotech —— HYstem-001

Product Overview

HYstem-001 Injection is a human umbilical cord-derived MSC product independently developed by Sino Biotech for the treatment of IPF. Through immunomodulatory and paracrine effects, the MSCs home to injured lung regions, secrete anti-inflammatory cytokines to suppress inflammation, and release growth factors to promote tissue repair.

Development Status

The Phase I clinical trial was first publicly registered on November 26, 2025. The study is being conducted at Beijing Chao-Yang Hospital, Capital Medical University, using a single-arm, open-label, dose-escalation design.

Cell-Batch Screening

To identify the optimal stem cell batch, Sino Biotech demonstrated in an in vitro model that the selected umbilical cord-derived MSCs inhibited TGF-β1-induced epithelial–mesenchymal transition by upregulating MMP-7 and CDH1 and downregulating COL1A1 and vimentin. The cells also inhibited bleomycin-induced bronchial epithelial cell apoptosis. In a bleomycin-induced rat model of pulmonary fibrosis, different MSC doses ranging from 1×10^6 to 1×10^7 cells/kg improved lung function, increased survival, and reduced pulmonary lesions in a dose-dependent manner. These in vitro and in vivo studies provided a scientific basis for selecting the optimal stem cell source and supporting subsequent clinical trials.

Vcanbio —— VUM02

Product Overview

VUM02 Injection is an allogeneic, off-the-shelf human umbilical cord-derived MSC product independently developed by Vcanbio. The MSCs are isolated, selected, and expanded from healthy neonatal umbilical cord tissue. The product contains 5×10^7 cells/10 mL per bag, is cryopreserved in liquid nitrogen, and is administered intravenously after thawing. Its therapeutic effects in pulmonary fibrosis involve the coordinated actions of immunomodulation, anti-inflammatory and antifibrotic effects, and tissue repair and regeneration.

Development Status *Granted FDA Orphan Drug Designation*

Nonclinical studies supported the safety and efficacy of MSC therapy for IPF. A **Phase I trial began in Nov 2023**, led by The First Affiliated Hospital of Guangzhou Medical University with multiple centers participating. All participants have completed treatment and **6-month follow-up**; long-term follow-up is ongoing, while the company is pursuing regulatory clearance for a subsequent pivotal trial.

Clinical data

Available results from the Phase I clinical trial of VUM02 Injection in IPF showed that human umbilical cord-derived MSCs demonstrated favorable safety and tolerability, slowed the decline in lung function, improved exercise capacity, and improved pulmonary imaging findings and respiratory symptoms.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Idiopathic Pulmonary Fibrosis

■ Application of Lung Progenitor Cell Therapy in Idiopathic Pulmonary Fibrosis

The core mechanism of lung progenitor cell therapy for IPF lies in a "structural repair" strategy that addresses the root cause of pathology, rather than merely slowing the decline of pulmonary function. The scientific rationale is as follows: the essence of IPF is the persistent loss of alveolar epithelial structure and irreversible deterioration of gas exchange function, which existing antifibrotic drugs cannot reverse. This therapy utilizes the patient's own healthy $P63^+$ airway basal stem cells — the key stem cell population responsible for epithelial repair in the adult lung. A small number of cells are collected via bronchoscopic brushing from the patient's relatively healthy lung lobes, expanded ex vivo using proprietary technology, and then re-administered into the patient's lungs. The transplanted $P63^+$ airway basal stem cells exert their effects through two synergistic pathways: reconstitution of the airway epithelial barrier and regeneration of gas exchange structures. The cells migrate and engraft in damaged alveolar tissue, where they differentiate to form a continuous epithelial barrier — like a bandage covering a wound — suppressing inflammation and fibrogenesis on one hand; on the other hand, they differentiate into mature epithelial cells with high expression of tight junction proteins, forming vesicle-like structures capable of gas exchange.

Regend —— REGEND001

Product Overview

REGEND001 is a first-in-class autologous lung progenitor cell therapy. The product utilizes the patient's own $P63^+$ airway basal stem cells. After obtaining a small sample of healthy airway epithelium via bronchoscopic brushing, cells are expanded and cultured ex vivo under feeder-free conditions using the proprietary R-Clone technology platform, then re-administered into the patient's lungs via bronchoscopy. The transplanted cells can engraft in areas of lung injury and exert therapeutic effects through two core mechanisms — reconstitution of the airway epithelial barrier and regeneration of alveolar gas exchange structures — directly repairing damaged alveolar architecture and thereby restoring pulmonary gas exchange function.

Development Status

The Phase II clinical trial of this product for IPF has been completed.

Clinical Data

According to data presented by Professor Wei Zuo, Chief Scientist of Regend, in an oral presentation at the International Society for Stem Cell Research (ISSCR) Annual Meeting in June 2025:

- **Efficacy:** Pulmonary diffusing capacity was significantly improved from baseline, demonstrating a statistically significant difference compared with the placebo control group. In addition, pulmonary fibrosis scores decreased significantly from baseline, also showing a statistically significant difference versus placebo.
- **Safety:** No treatment-related serious adverse events (SAEs) were observed, and the incidence of adverse events in the treatment group was comparable to that in the placebo group.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Chemotherapy-induced Thrombocytopenia

With their strong self-renewal and multilineage differentiation potential, hematopoietic stem cells can effectively reconstruct the damaged hematopoietic system and have been successfully used clinically to treat chemotherapy-induced thrombocytopenia.

Definition and Pathogenesis of Chemotherapy-induced Thrombocytopenia

Chemotherapy-induced thrombocytopenia(CTIT) is a common complication of anticancer treatment, characterized by a peripheral platelet count below the normal range. Its incidence varies by tumor type and treatment regimen, affecting approximately 10%–21% of patients with solid tumors, with the highest risk associated with gemcitabine- and platinum-based chemotherapy. Published studies have reported thrombocytopenia in up to 21.8% of patients receiving anticancer therapy.

Overall incidence among patients receiving anticancer therapy: ~21.8%

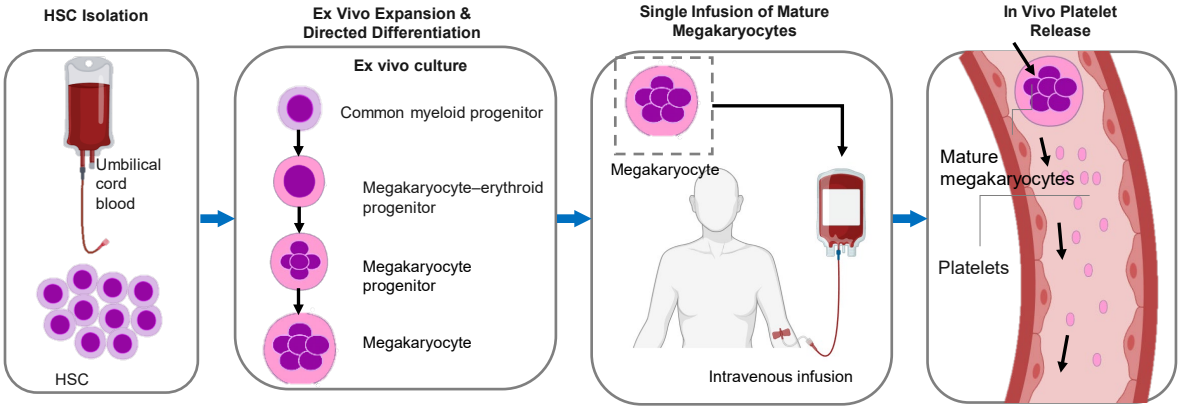
CTIT primarily results from reduced platelet production, increased platelet destruction and abnormal platelet distribution. Reduced production is the predominant mechanism: chemotherapy and certain targeted therapies can directly suppress HSC and megakaryocyte progenitor proliferation or damage the hematopoietic microenvironment, resulting in ineffective thrombopoiesis. Increased destruction may result from drug-induced antiplatelet antibodies or immune checkpoint inhibitor (ICI)-activated T-cell attacks on platelets. Distribution abnormalities are mainly associated with treatment-related hepatosplenic injury and portal hypertension, leading to increased splenic platelet sequestration and destruction.

Current Diagnosis and Treatment Landscape and Unmet Clinical Needs in CTIT

Current CTIT management relies mainly on platelet transfusion and thrombopoietic agents, such as recombinant human thrombopoietin (rhTPO) and IL-11, but substantial unmet needs remain. First, there is a lack of biomarkers that can accurately predict the risk of severe thrombocytopenia, meaning intervention often occurs only after platelet counts have declined. Second, approximately 30% of patients with ICI-induced immune thrombocytopenia relapse after retreatment, while existing thrombopoietic agents have limited efficacy against immune-mediated platelet destruction and lack targeted management strategies. Third, mechanisms of thrombocytopenia vary across chemotherapy, targeted therapy and immunotherapy, whereas current treatment approaches remain relatively homogeneous, highlighting the need for mechanism-based precision therapies.

Platelet Production through Ex Vivo Directed Differentiation of Cord Blood-Derived Hematopoietic Stem Cells

The technology uses cord blood-derived HSCs as the starting material, followed by stepwise directed differentiation and scalable ex vivo expansion into common myeloid progenitors, megakaryocyte–erythroid progenitors, megakaryocyte progenitors and, ultimately, mature megakaryocytes. The resulting megakaryocytes are administered intravenously and continuously release functional platelets in the circulation, effectively increasing peripheral platelet counts for the treatment of thrombocytopenia.



Representative Megakaryocyte Therapy for CTIT

HemaCell —— XJ-MK-002

HemaCell Biotechnology Inc. has established a differentiated competitive position in the CTIT treatment landscape through its megakaryocyte injection products. **In November 2025**, the company first publicly disclosed **the Phase I clinical trial of XJ-MK-002**, its allogeneic megakaryocyte injection, making it **the world's first megakaryocyte cell therapy product to enter clinical development**. XJ-MK-002 is administered as a single intravenous infusion of umbilical cord blood-derived megakaryocytes, the precursor cells of platelets. These cells use shear forces in the bloodstream to naturally fragment into functional platelets, enabling a rapid and sustained increase in platelet counts while avoiding the delayed onset of conventional agents and IL-11-related cardiotoxicity. To date, XJ-MK-002 has received two FDA Rare Pediatric Disease Designations (RPDDs) and three FDA Orphan Drug Designations (ODDs) for adult indications. For CTIT, HemaCell is also developing **XJ-MK-001, an autologous megakaryocyte injection** whose **investigational new drug application was cleared by the FDA in August 2025**.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Chronic Obstructive Pulmonary Disease

With their strong self-renewal and multilineage differentiation capacities, autologous lung progenitor cells can effectively repair damaged airway and alveolar epithelial barriers and have been successfully evaluated clinically for the treatment of chronic obstructive pulmonary disease.

Definition and Pathogenesis of COPD

Chronic obstructive pulmonary disease (COPD) is a progressive lung disease caused by long-term exposure to harmful particles or gases, such as cigarette smoke, air pollution, and occupational dust. It is a common, preventable, and treatable chronic airway disease characterized by persistent airflow limitation and respiratory symptoms associated with airway and/or alveolar abnormalities, primarily chronic bronchitis and emphysema.

COPD incidence: ~197 per 100,000

The pathogenesis of COPD remains incompletely understood. Oxidative stress, inflammation, and protease–antiprotease imbalance contribute to tissue remodeling, chronic respiratory symptoms, and airflow limitation. Recent studies suggest that autoimmune regulation, genetic risk factors, and impaired lung development may also contribute to the development and progression of COPD.

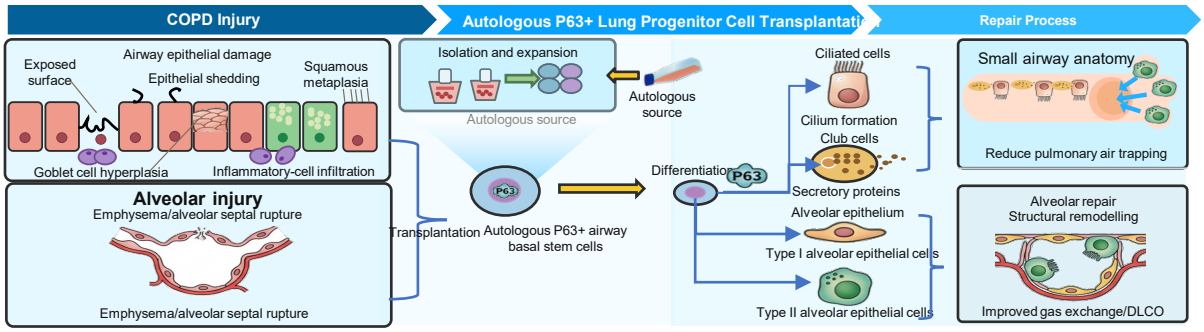
Current Diagnosis and Treatment Landscape and Unmet Clinical Needs in COPD

Current COPD management relies mainly on long-acting bronchodilators (LABA/LAMA), inhaled corticosteroids (ICS) and triple inhaled therapy, supplemented by pulmonary rehabilitation, long-term oxygen therapy and other supportive measures. However, substantial unmet needs remain. First, reliable biomarkers for predicting disease progression and severe exacerbations are lacking, and many patients already have irreversible damage at diagnosis. Second, current therapies mainly relieve symptoms and reduce exacerbations but cannot reverse airway remodeling or alveolar destruction, while regenerative approaches for severe disease remain limited. Third, disease mechanisms vary across COPD phenotypes, including emphysema and chronic bronchitis, whereas treatment remains relatively homogeneous. In particular, emphysema-predominant COPD lacks therapies capable of repairing damaged small airways and alveoli, highlighting the need for regenerative treatments targeting structural lung repair.

Autologous Lung Progenitor Cell Therapy

Autologous lung progenitor cell therapy using P63⁺ airway basal stem cells aims to repair and reconstruct damaged small airways and alveolar structures. P63⁺ lung progenitor cells are collected from the patient's bronchial basal layer by bronchoscopic brushing, expanded ex vivo under feeder-free GMP conditions, and precisely delivered to damaged lung regions via bronchoscopy. Following transplantation, the cells engraft at damaged sites and differentiate into functional epithelial cells, including ciliated epithelial cells and Club cells, thereby reconstructing small-airway and alveolar structures, reducing pulmonary air trapping, and improving gas exchange.

Figure. Mechanism of Autologous Lung Progenitor Cell Therapy for COPD



Representative Stem Cell Therapy for COPD

Regend—— REGEND001

Product Overview

The world's first autologous lung progenitor cell therapy

REGEND001 is the world's first autologous lung progenitor cell therapy. P63⁺ lung progenitor cells are collected from the patient's bronchial basal layer by bronchoscopic brushing, expanded ex vivo under GMP conditions, and delivered back to damaged lung regions. The transplanted cells can differentiate into airway and alveolar epithelial cells, reconstruct the epithelial barrier, repair small-airway and alveolar damage, reduce pulmonary air trapping, and improve gas exchange.

R&D Status

Latest Progress

The Phase II clinical trials in IPF and COPD completed follow-up for all patients in June and October 2025, respectively, and reported positive results. Based on these findings, the launch meeting for the Phase III confirmatory trial in COPD was held in May 2026, marking a key step toward potential commercialization.

Clinical Data

Safety: Overall adverse-event rates were comparable between the treatment and control groups, with no serious adverse events related to the cell therapy observed.

Efficacy: DLCO improved significantly from Week 4, peaked at Week 24, and remained stable through Week 52. Alveolar volume also increased, while 3D CT reconstruction showed increased functional lung volume and partial repair of emphysematous regions. Quality-of-life improvement was significantly greater than in the control group.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Clinical Applications of Stem Cells — Chronic Periodontitis

Chronic periodontitis causes alveolar bone defects and tooth loss through persistent periodontal inflammation. Current therapies face challenges including recurrence and limited bone regeneration, while stem cell therapy offers a novel strategy for periodontal tissue repair and functional restoration.

Overview and Mechanism of Chronic Periodontitis

Chronic periodontitis is a chronic inflammatory disease of periodontal supporting tissues driven by local stimuli. Persistent inflammation damages the periodontal ligament, alveolar bone, and cementum, leading to tooth mobility and tooth loss, and is associated with systemic diseases such as diabetes, cardiovascular diseases, and malignancies. Dysbiosis of the oral microbiota, host immune responses, genetic factors, and environmental factors contribute to disease development. Pathogens such as *Porphyromonas gingivalis* form biofilms and release virulence factors, inducing TNF-α and IL-1β production, amplifying inflammation, and accelerating alveolar bone resorption.

Global Prevalence: ~15 /100 Population

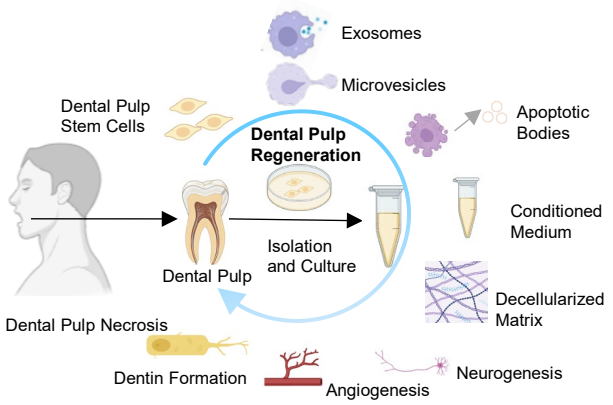
Current Treatment Landscape and Unmet Needs in Chronic Periodontitis

Current management of chronic periodontitis mainly relies on mechanical debridement and periodontal surgery. Initial treatments include scaling and root planing (SRP) to control plaque-induced inflammation. Severe cases may require flap surgery, regenerative procedures such as guided bone regeneration (GBR), guided tissue regeneration (GTR), and enamel matrix derivatives (EMD) to delay periodontal tissue defect progression. However, conventional SRP has limited efficacy for periodontal pockets ≥6 mm and severe bone defects, while regenerative procedures have limited success rates. Current treatments are invasive, costly, and associated with variable outcomes due to individual bone conditions, oral hygiene, and reduced endogenous stem cell activity in middle-aged and elderly patients. Significant unmet needs remain for minimally invasive, convenient, and safe therapies capable of complete regeneration of periodontal supporting tissues.

Dental Pulp Stem Cell (DPSC)-Based Therapy

Dental pulp stem cells (DPSCs) are dental tissue-derived MSCs with self-renewal and multilineage differentiation potential. They can differentiate into osteogenic, periodontal ligament, and endothelial cells, making them suitable seed cells for periodontal regeneration. In addition to directed differentiation, DPSCs contribute to repair mainly through paracrine effects mediated by extracellular vesicles, including exosomes and microvesicles, which deliver miRNAs and cytokines to regulate the local inflammatory microenvironment. Combining extracellular vesicle-mediated signals with decellularized extracellular matrix (dECM) scaffolds provides biological cues and structural support, with the potential to promote odontogenic, angiogenic, and neural differentiation. With low immunogenicity, DPSCs can suppress inflammation and repair alveolar bone and periodontal ligament, enabling minimally invasive periodontal regeneration.

Figure. Schematic Diagram of Paracrine Effects and Differentiation of Dental MSCs



Beijing SH Biotechnology — hDP-MSC Injection

Product Overview

This product is a self-developed innovative dental-derived stem cell drug isolated from healthy dental pulp tissue. It is formulated at 1 × 10^7 cells/0.6 mL per vial for moderate-to-severe periodontitis.

It is administered by local minimally invasive injection and acts through both immunomodulation and tissue regeneration. Compared with bone marrow- and periodontal ligament-derived stem cells, DPSCs are abundant, highly proliferative, easy to isolate, and suitable for drug development. With low immunogenicity in allogeneic use, they can simultaneously suppress periodontal inflammation and repair alveolar bone defects, addressing the inability of conventional treatments to regenerate periodontal tissues and the invasiveness of surgical regenerative procedures.

Development Status

The lead program has the potential to become China's first dental-derived MSC drug.

The first-generation DPSC program has completed its Phase II clinical trial, with follow-up completed for all 204 participants. Comprehensive clinical data analysis is currently underway, and a marketing application is expected to be submitted within two to three years.

The second-generation gene-modified EMSC program has completed animal efficacy studies across multiple indications. PCT patent applications are being pursued in China, the United States, and Europe to further strengthen the program's long-term technological barriers.

Clinical Data

Phase I Clinical Trial and IIT Study:
Among patients with Stage III severe periodontitis and AL ≥5 mm, mean periodontal pocket depth improved by 1.81 mm at 180 days, and bone repair indicators were significantly better than those in the control group.

Phase II Clinical Trial:
Among patients with bone defects ≥3 mm, bone defects improved by 1.132 mm at 360 days after a single administration, with sustained promotion of bone regeneration and delayed tooth loss.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Chapter 7

Stem Cell Product Development and Manufacturing Technology

07

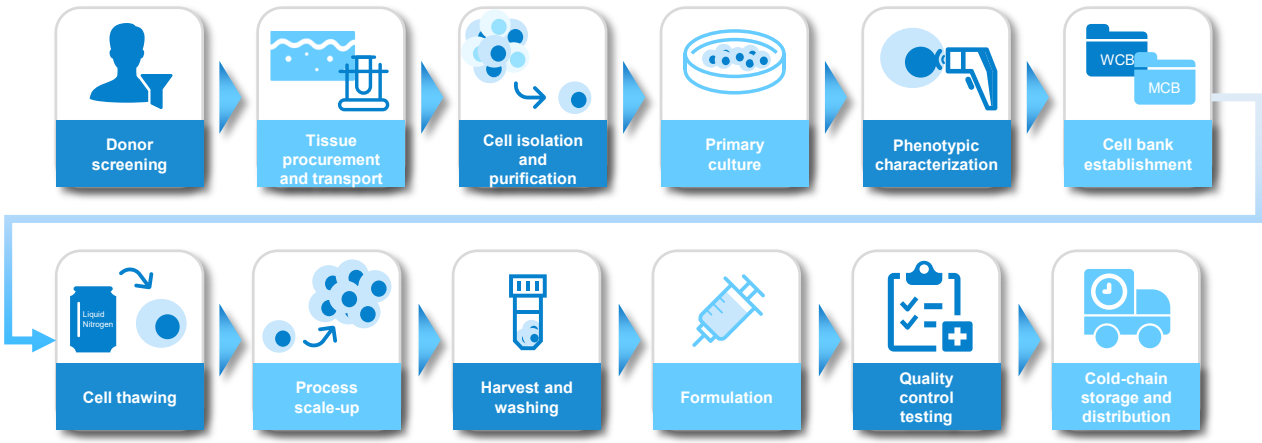
Stem Cell Product Manufacturing Process

The manufacturing of stem cell products entails donor screening, tissue procurement, cell isolation, expansion, quality control, and cryopreservation. Distinct from conventional biopharmaceuticals, these living biologics feature profound donor heterogeneity, process-dependent efficacy, and intricate supply chain logistics.

Stem Cell Manufacturing Workflow

The manufacturing process commences with rigorous donor screening and tissue procurement in order to ensure biosafety. Following cell isolation, purification, and primary culture, cell identification and characterization is performed to establish either a two-tiered master cell bank (MCB) and working cell bank (WCB) system or a single-tiered MCB-only system, thereby providing uniform and stable starting materials. During full-scale production, banked cells are thawed and subsequently expanded using GMP-compliant, closed automated platforms. Upon harvest, cell washing and formulation are completed prior to comprehensive lot release testing for viability, phenotype, potency, differentiation potential, and sterility, before final delivery through validated clinical cold-chain distribution.

Figure. Stem Cell Product Manufacturing Workflow



Differences Between Stem Cell Products and Conventional Biopharmaceuticals

Stem cell products exhibit four major differences from conventional biopharmaceuticals: 1) Heterogeneity inherent to living biological materials; 2) "The process is the product," presenting significant challenges for straightforward process scale-up; 3) High costs associated with autologous therapies, alongside potential risks of immunogenicity and infusion-related adverse reactions in allogeneic products; 4) Difficulty in the long-term preservation of living cells, resulting in a complex supply chain.

Figure. Comparison Between Stem Cell Products and Conventional Biopharmaceuticals

Dimension	Stem Cell Products	Conventional Biopharmaceuticals
Raw Materials	Cellular raw materials from diverse tissues, such as bone marrow, umbilical cord, and adipose tissue, exhibit profound differences in gene expression and functionality, while individual donor factors, including age, sex, BMI, and health status, directly impact cell quality, morphology, and differentiation potential.	Chemically defined or highly purifiable molecules are manufactured via standardized processes, achieving high batch-to-batch consistency.
Manufacturing Process	Subtle variations in medium composition, growth factors, and culture techniques alter final therapeutic efficacy, presenting significant challenges for straightforward process scale-up.	Mature and comprehensively validated processes exhibit well-defined correlations with final product structure and function, wherein process scale-up engineering challenges are effectively resolved.
Manufacturing Scale	Autologous therapies require individualized patient production, resulting in exorbitant costs, protracted cycles, and no economies of scale; while off-the-shelf allogeneic therapies permit large-scale production, they face clinical obstacles including immune rejection.	Standardized manufacturing leverages large-scale centralized production, enabling large batches for extensive populations post-quality control, whereby costs scale inversely with production volume.
Shelf Life	Products require ultra-low temperature storage, such as liquid nitrogen at -196°C, to maintain long-term viability; formulation utilizes specialized cryoprotectant solutions and controlled-rate freezing protocols, while distribution mandates dedicated liquid nitrogen shippers with continuous temperature monitoring, resulting in intricate supply chain logistics.	Molecular structures remain relatively stable, typically formulated as lyophilized powders or liquid injections that achieve long-term stability during storage and transport at 2–8°C or lower temperatures.

Source: Literature Review, Public Information, Frost & Sullivan Analysis

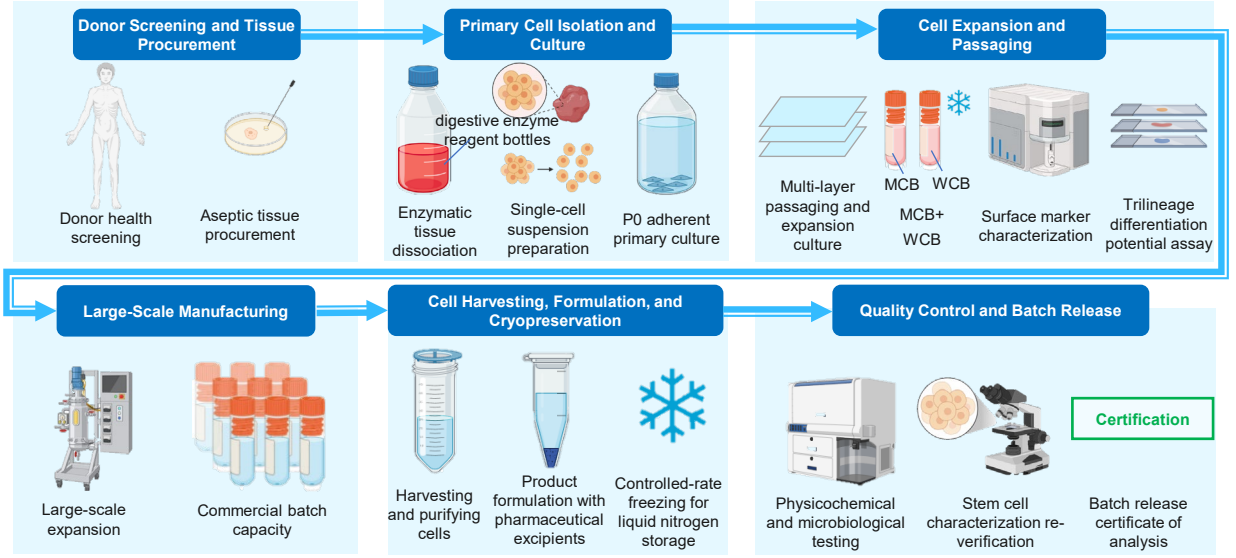
Large-Scale Stem Cell Manufacturing

Stem Cell Preparation

Stem cell preparation refers to the comprehensive process of isolating and purifying target stem cells from specific human tissues or cellular sources, followed by rigorous *in vitro* expansion and manipulation, ultimately culminating in the formulation of stem cell products that meet clinical or research standards.

Stem Cell Preparation Workflow

Core Steps in Stem Cell Preparation: (1) Donor Screening and Tissue Procurement: Comprehensive pathogen, infectious disease, and genetic history screening excludes high-risk donors with cancer or autoimmune disorders to ensure biosafety at the source, followed by procuring target tissues such as umbilical cord, adipose tissue, and bone marrow. (2) Primary Cell Isolation and Culture: Target tissues undergo collagenase digestion or density gradient centrifugation for stem cell isolation, followed by adherent primary culture where unpassaged cells are designated as P0. (3) Cell Expansion, Passaging, and Cell Bank Establishment: Serial passaging utilizes culture flasks and multi-layer cell factories; An MCB is commonly established at P1, followed by a WCB at P2–P3. At the cell banking stage, appropriate quality testing should be performed according to the cell type. (4) Large-Scale Manufacturing: Production of allogeneic, off-the-shelf products requires substantial capacity, wherein three-dimensional suspension expansion via stirred-tank bioreactors and microcarriers represents a widely adopted industrial route. (5) Cell Harvesting, Formulation, and Cryopreservation: Qualified cells are harvested and formulated using pharmaceutical excipients including human serum albumin and buffered salines; products are administered fresh or undergo controlled-rate cryopreservation with DMSO-based formulations for long-term liquid nitrogen storage. (6) Full-Process Quality Control and Batch Release: Quality control spans donors, cell banks, intermediate cells, and final products; release testing evaluates cell identification, viability, purity, microbiological limits, functional characteristics, and tumorigenicity to ensure batch-to-batch consistency and clinical safety.



CytoNiche: An Integrated Platform for Large-Scale Intelligent Cell Therapy Manufacturing

- **End-to-End Smart Manufacturing:** Centered on its proprietary 3D cell manufacturing platform, CytoNiche brings upstream expansion and downstream processing into a unified, automated, and scalable manufacturing architecture for both MSC and exosome production. Powered by 3D TableTrix™ degradable microcarriers and 3D FloTrix™ bioprocessing systems, the platform integrates cell expansion, harvesting, washing, formulation, and fill-finish within a streamlined, closed workflow.
- **Industrial-Scale Cell Manufacturing Platform — Scaling from Tens to Hundreds of Billions:** Supporting the development of China's first approved stem cell drug and China's first exosome therapeutic IND, the platform enables high-density MSC expansion at clinically and commercially relevant scales, with single-batch harvests reaching tens to hundreds of billions of cells while maintaining high cell viability.
- **Advanced 3D Microcarrier Technology:** At the core of the platform is an advanced, degradable 3D microcarrier architecture engineered for high-density cell expansion within a biomimetic microenvironment. Offered conveniently in both tablet and powder formats, 3D TableTrix™ rapidly disperses into tens of thousands of resilient microcarriers featuring >90% porosity. Expanding on this technology, 3D RecomTrix™ recombinant collagen microcarriers, introduced in late 2025, scale production to tens of billions while offering an animal-component-free (ACF) alternative for scalable biomanufacturing. Both products have Drug Master File (DMF) filings with the U.S. FDA and CDE registrations with China's NMPA, establishing a regulatory foundation for broader global adoption.
- **Gentle, Controlled Cell Harvesting:** The degradable microcarrier architecture enables cell recovery through controlled, specific degradation, providing a gentle alternative to conventional enzymatic detachment. Following culture, the microcarrier matrix is completely dissolved into soluble components, facilitating efficient cell–microcarrier separation while minimizing mechanical and enzymatic stress during harvest and washing. This approach supports high post-harvest viability, recovery efficiency, and preservation of key cellular quality attributes, creating a seamless transition from upstream expansion to downstream cell processing.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

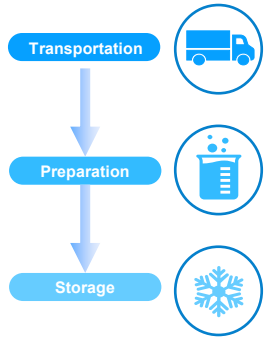
Stem Cell Banking

Stem Cell Banking

Stem cell banking refers to the process of isolating specific stem cell populations from blood or other tissue specimens via specialized separation technologies, followed by long-term cryopreservation using ultra-low temperature techniques to maintain cells in a dormant yet viable state for future thawing and utilization in downstream research and clinical applications. In lay terms, stem cell banking can be conceptualized as a "biological insurance policy," wherein autologous stem cells or those derived from neonates are banked during youth and optimal health to meet potential therapeutic demands for future disease management.

Stem Cell Banking Workflow

Core Stages of Stem Cell Banking: Stem cell banking generally encompasses the following stages: (1)Health Screening and Contracting: Clients provide tertiary hospital medical and pathogen screenings to ensure source biosafety; upon verification, a storage agreement is executed and procurement scheduled. (2)Aseptic Procurement and Cold-Chain Transportation: Specimens are transported to the cell bank within 24 hours in specialized 0–4°C containers via uninterrupted cold-chain logistics to maintain viability. (3)Cell Preparation (Isolation and Purification): Received specimens undergo secondary verification, followed by stem cell isolation and purification from tissues via density gradient centrifugation per standard operating procedures. (4)Safety Testing: Cells undergo testing for nucleated cell counts, blood typing, aerobic/anaerobic bacteria, and pathogens including HIV, hepatitis, and syphilis. Contamination triggers independent reexamination; wherein non-compliant specimens are disqualified. (5)Ultra-Low Temperature Cryopreservation: Cells are formulated with DMSO cryoprotectant, subjected to controlled-rate freezing, and stored in -196°C liquid nitrogen. Qualified batches are archived and released into the repository upon quality management approval. (6)Report Generation and Routine Monitoring: Following banking, clients receive an initial cell banking report alongside periodic quality monitoring updates.



Types of Stored Stem Cells

Currently available stem cell types for banking include the following:

MSCs

Isolated from tissues including umbilical cord, placenta, adipose tissue, and dental pulp, these cells differentiate into bone, cartilage, adipose, neural, and myocardial lineages under specific conditions. They are primarily applied in tissue repair and immune regulation, covering nervous, immune, urogenital, endocrine, respiratory, cardiovascular, digestive, and musculoskeletal systems.

HSCs

Clinically utilized to treat diverse hematological disorders, including acute leukemia, chronic leukemia, aplastic anemia, thalassemia, and multiple myeloma.

iPSCs

Capable of directed differentiation into somatic lineages such as cardiomyocytes, neurons, and pancreatic islet cells, with applications spanning nervous, cardiovascular, endocrine, and digestive systems.

Sino Biotech: An Integrated One-Stop Service Platform for Cell Therapy

- **Cell Resource Banking:** Sino Biotech operates 16 GMP-compliant cell storage facilities spanning over 100,000 square meters that conform to human genetic resource management regulations. Its proprietary SinoLife immune cell banking system relies on three core competencies: an automated PBMC cryopreservation system, a high-purity immune cell preparation system, and a cell manufacturing system adhering to NIFDC standards. Supporting a comprehensive automated cryopreservation workflow, the company banks diverse cell types, including PBMCs from oncology patients and healthy adults, neonatal perinatal tissues, veterinary PBMCs, and hematopoietic stem cells for hematological disorders.
- **CDMO:** Supported by six core capabilities, Sino Biotech's CDMO platform offers full-lifecycle cell therapy development services. Combining specialized R&D, compliant manufacturing facilities, standardized quality management systems, validated regulatory filing experience, and an extensive market partnership network, the platform provides end-to-end solutions covering early process development, clinical manufacturing, and regulatory submissions.
- **Cell Culture Technical Services:** Sino Biotech conducts functional cell-source screening, establishes iPSC manufacturing processes from peripheral blood and fibroblasts, and advances exosome production and translational applications.
- **Stem Cell Therapeutic IND Filings:** Supported by extensive cell therapy R&D and regulatory submission experience alongside robust clinical resources.

Boyalife: Full-Industry-Chain Services in Cell Technology

- **Full-Chain Quality Management System Enabling End-to-End Data Traceability:** Boyalife holds international accreditations from the AABB, NRL, and CAP, alongside certifications including ISO 9001 and the ICCBBA. The company has established a GMP-compliant identification and traceability system across the manufacturing lifecycle, achieving bidirectional traceability from cell sourcing to terminal application.
- **Cell Banking:** Boyalife operates automated, clinical-grade cell banks and manufacturing centers, ranking among China's leading providers with a 32% market share. It has five facilities, over 10,000 m² of GMP laboratories and 14 consecutive years of AABB accreditation. Its services cover perinatal and adult stem cell banking, immune cell banking, cell processing, long-term storage and personalized family cell asset and health management.
- **Automated Cellular Medical Devices:** Provides automated equipment for cell isolation, processing, and cryopreservation alongside clinical-grade automated medical devices for the cell therapy market, thereby accelerating clinical translation.
- **Cell Therapy Product Development and Industrial Application:** Advances multi-pipeline drug R&D and clinical development targeting placenta- and umbilical cord-derived stem cells, exosomes, and immune cells. Pipeline activities cover clinical studies on diverse degenerative and autoimmune disorders, as well as collaborative research on autologous bone marrow concentrate for sports injuries, anti-aging interventions in women, vascular diseases, and organic lesions including liver cirrhosis.
- **Stem Cell Exosome Technology Platform:** Its exosome raw material has completed a U.S. FDA DMF filing and obtained an INCI listing.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Automated Stem Cell Manufacturing

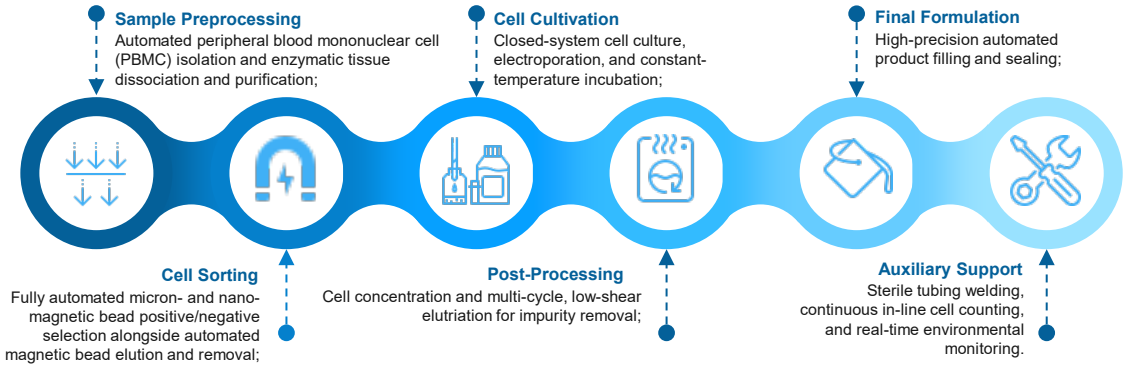
Automated Stem Cell Manufacturing

Automated manufacturing uses integrated cell-processing equipment, single-use closed consumables and programmable software to perform key steps from raw-material processing to final formulation, including fluid transfer, incubation, washing, concentration and filling. Modular closed systems reduce manual handling and enable standardized, traceable production with limited operator involvement.

As stem cell therapies move towards commercialization, conventional manual or semi-open production may face high labor costs, contamination risks, limited capacity and batch-to-batch variability. Automated closed systems can reduce these risks, improve efficiency and consistency, and enhance data traceability, supporting GMP-compliant manufacturing and the industry's transition towards scalable commercial production.

Automated Process Stages Across the Entire Stem Cell Manufacturing Workflow

Current automation technology covers the entire industrial stem cell preparation pipeline, enabling machine substitution for manual operations across all key process nodes:



Key Advantages of Automated Stem Cell Manufacturing

Automated cell processing systems are a widely adopted standardized approach to compliant commercial-scale stem cell manufacturing, offering five key advantages: (1) Reduced reliance on manual operations: Automation reduces operator workload and operator-related variability, thereby lowering the risks of batch rejection and process failure. (2) Improved product consistency: Programmable processes enable precise control of key parameters, including fill volume and cell concentration, while minimizing viability loss and improving batch-to-batch consistency. (3) Lower facility and operating costs: Fully closed, integrated systems may support operation in Grade C cleanroom environments, subject to process design and regulatory requirements, potentially reducing cleanroom space and long-term facility costs. (4) Improved contamination control: Single-use closed sterile consumables minimize exposure to the external environment and reduce microbial contamination risks. (5) Flexible scalability: Modular systems can support both small-batch, patient-specific autologous manufacturing and large-scale production of allogeneic stem cell products.

CellBri: Automated Bioprocessing Equipment for Cell Therapies

- **Cell Preparation and Processing:** CellBri offers three core platforms: the Gentle Flex system, a versatile closed platform for PBMC isolation, bead incubation, viral transduction, harvest, and formulation across varied cell types; Gentle Flex Pro, a high-throughput variant designed for solid tumor and allogeneic therapies, capable of large-volume washing, concentration, and formulation for up to 4E10 T cells per run; and CellFAB One, an all-in-one automated manufacturing workstation that integrates PBMC isolation, activation, transduction, expansion, harvest, formulation, and magnetic bead selection and removal into a single seamless workflow.
- **Cell Sorting:** The sorting portfolio includes Gentle n-MagS, a fully automated platform using single-use closed disposables for nano-magnetic bead cell selection, and Gentle m-MagS, designed for micro-magnetic bead selection and depletion. Featuring continuous-flow operation without volume limits, Gentle m-MagS achieves ≥80% sorting purity, completing either selection or bead removal within 10–15 minutes per sample.
- **Cell Expansion:** The Gentle Exp automated closed expansion system utilizes a specialized rocking platform with precise parameter controls over speed, tilt angle, gas flow, pH, dissolved oxygen, temperature, and CO_2/O_2 levels. Supporting interchangeable trays (10L, 20L, 50L) with flexible working volumes from 400 mL to 25 L, it accommodates everything from early R&D to pilot- and commercial-scale manufacturing.
- **Formulation Filling and Auxiliary Equipment:** For cell formulation, the Gentle P-Pac system offers high-precision flow sensing for continuous 200-bag filling while maintaining low temperatures to keep viability loss under 5%. The Gentle P-Pac Pro workstation scales up to 1,000 bags with AI-driven filling accuracy within ±0.2mL and T-cell dispensing uniformity ≤5%. Together, they cover small-batch precision and high-throughput commercial needs, complemented by essential accessories including handheld tube sealers, automated fluid transfer systems, and sterile tubing welders.

Source: Literature Review, Company Information, Frost & Sullivan Analysis.

Quality Control

Quality Control of Stem Cell Therapies

Given the diverse origins, process complexity, and volatile *in vitro* functional viability characteristic of live-cell therapeutics, stem cell products require comprehensive lifecycle quality management built upon three functional pillars: cellular biological identity, overall biosafety, and clinical biological potency. The primary regulatory and technical barriers in quality control rely on a robust compliance paradigm integrating personnel, equipment, materials, methodology, and environmental controls. Translating live-cell therapies into compliant manufacturing requires highly qualified R&D and QA/QC personnel, calibrated cell-processing and analytical instrumentation, and fully traceable supply chains for donor tissues and processing consumables. Furthermore, implementing standardized, auditable isolation and expansion protocols under strict GMP-classified environments is imperative to maintain inter-batch reproducibility and minimize the safety risks inherent to living cell products.

Regulatory Requirements for Quality Control

On January 13, 2025, NMPA's CFDI released the Guidelines for Inspection of Cell Therapy Product Manufacturing, establishing strict GMP standards requiring end-to-end quality management and full batch traceability across cell collection, processing, storage, transport, and release to mitigate production risks. In 2026, CDE issued two key directives to standardize post-approval and lifecycle modifications: the Technical Guidelines for Pharmaceutical Changes Research and Evaluation of Cell Therapy Products (Trial) on January 30, detailing technical comparability and archival traceability requirements for CMC changes across all development phases; and the general Guidelines for Acceptance Review of Biological Product Changes (Trial) on May 15, unifying formal review standards for supplementary application filings.

Key Certifications and Quality Management Systems

AABB System

- **The Association for the Advancement of Blood & Biotherapies (AABB) accreditation** represents an internationally recognized gold standard in cell therapy, covering the entire pipeline from cell collection, manufacturing, testing, and cryopreservation to distribution and clinical application. Standards are updated biennially, accompanied by mandatory on-site re-evaluations every two years to ensure sustained product quality and safety.

NRL/CAP

- **NRL:** Validates laboratory proficiency in viral diagnostics and testing, ensuring banked cell repositories remain free of bloodborne pathogens as an essential safeguard for bio sample safety.
- **CAP:** Offered by the College of American Pathologists, CAP accreditation serves as a premier global benchmark for clinical laboratories, signifying that testing systems, standard operating procedures, and personnel competency meet international standards for worldwide trust and mutual recognition.

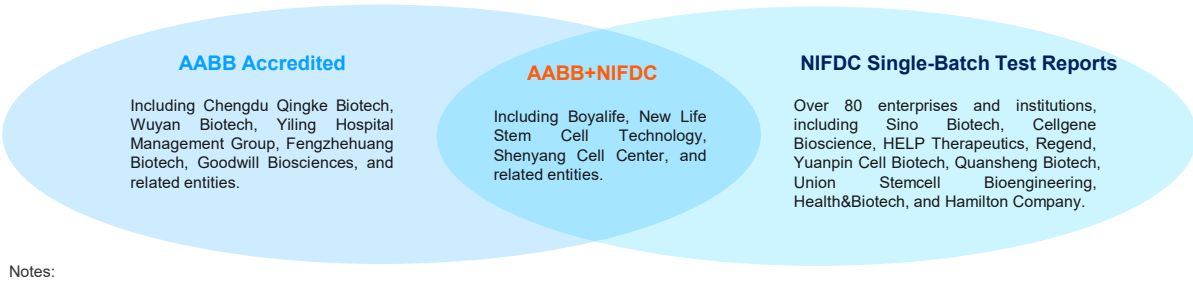
ISO9001 Quality Management System

- Formulated by the International Organization for Standardization (ISO), ISO 9001 provides a globally recognized framework establishing requirements for creating, implementing, maintaining, and continually improving quality management systems.
- **System Accreditation Status:** It stands as the most widely adopted quality management standard worldwide.

NIFDC Cell Quality Verification

- As a statutory testing authority and supreme technical arbitration body directly affiliated with the NMPA, the National Institutes for Food and Drug Control (NIFDC) oversees the quality supervision and testing of pharmaceuticals, biologics, and cell products across China.
- **Inspection Report Status:** Test reports issued by the National Institutes for Food and Drug Control (NIFDC) provide evidence of the quality of the tested samples, providing authoritative validation of process robustness, system compliance, and clinical-grade quality.

Figure. Representative Enterprises for AABB Accreditation and NIFDC Single-Batch Cell Quality Verification Records



Notes:
1. The list of AABB-accredited entities is retrieved from the official AABB website.
2. Lacking a centralized NIFDC enterprise registry, this list of over 80 entities was compiled exclusively from public submission records. Note that single-batch inspection reports apply solely to the tested samples and do not imply full-process regulatory compliance for the enterprise.

Source: Literature Review, Public Information, Frost & Sullivan Analysis

Core Technical Challenges (1)

Key challenges in stem cell product manufacturing include donor variability, starting material control, balancing purity and functionality, loss of stemness during expansion, gene-editing risks, cryopreservation safety, limited in vivo validation of potency assays, and production timelines and batch consistency during scale-up.

Raw Material Sourcing

Key challenges in raw material sourcing are increasingly being addressed through standardization. Donor variability related to age, genetics and health status requires strict donor screening and material-release criteria. Multiplex nucleic acid testing can reduce the risk of missed infections during window periods, while standardized collection media, cold-chain transport and controlled processing windows help preserve tissue viability. Because donor-derived materials are limited, expansion processes must balance cell yield with the preservation of stemness and potency during passaging. Different stem cell sources also have distinct advantages and limitations, requiring continued optimization for specific product applications.

Figure. Comparative Analysis of Stem Cell Sources and Their Industrial Applicability

Source	Core Advantages	Primary Challenges	Solutions & Optimization Directions	Typical Cell Types / Target Applications
Bone Marrow	Long history of clinical use; mature technology; contains both HSCs and MSCs	Invasive extraction; high donor discomfort; yield/quality decline with age; high inter-individual variability	Enforce strict donor screening; optimize collection protocols	HSC transplantation (hematological diseases); bone marrow MSCs (bone/cartilage repair, immunomodulation)
Peripheral Blood	Convenient collection with high donor acceptance; yields abundant HSCs post-mobilization	Requires mobilization agents with potential side effects; variable mobilization efficiency	Optimize mobilization regimens; establish predictive evaluation frameworks; standardize collection	HSC transplantation (dominant source)
Umbilical Cord Blood	Non-invasive postpartum collection; lower immunogenicity than BM/PB HSCs; higher HLA mismatch tolerance; low GVHD risk	Limited $CD34^+$ cell yield per unit (insufficient for heavy adults); requires HLA matching; risk of rejection upon severe mismatch	Implement *in vitro* expansion; utilize double-unit cord blood transplantation; expand public and autologous biobanks	HSC transplantation (pediatric/low-weight adults); cord blood MSCs (exploratory); autologous use unsuitable for genetic blood disorders or congenital leukemia
Umbilical Cord Tissue	Abundant non-invasive source; high MSC yield and strong proliferative capacity; low immunogenicity	Inconsistent tissue standardization; lack of unified quality standards	Establish standardized tissue collection/processing; optimize MSC isolation and expansion protocols	Allogeneic off-the-shelf MSCs (immunomodulation, tissue repair)
Adipose Tissue	Abundant source; minimally invasive retrieval (liposuction); high MSC yield and strong proliferation	Significant individual variability; high heterogeneity among adipose-derived MSCs	Establish donor screening criteria; refine isolation and culture processing	Autologous/allogeneic adipose MSCs (soft tissue repair, anti-inflammation, aesthetics/regenerative medicine)
Placental Tissue	Broad availability; non-invasive collection; rich MSC content; low immunogenicity; strong proliferation	Variable tissue standardization; risk of maternal-fetal cell cross-contamination	Standardize tissue acquisition and processing; optimize isolation and purification protocols	Allogeneic off-the-shelf MSCs (immunomodulation, tissue repair)
iPSCs	Unlimited supply; pluripotency to differentiate into virtually all cell types; autologous compatibility; easy gene editing	Tumorigenicity risks; high differentiation cost with variable efficiency; genomic instability	Optimize differentiation protocols; refine quality control systems; establish universal off-the-shelf iPSC lines	Autologous/universal iPSC-derived cellular products (neural, cardiac, immune cells); high-throughput drug screening

Cell Isolation and Purification

Cell isolation requires balancing purity with functional preservation. Liquid samples, such as cord blood and bone marrow, are commonly processed using density-gradient centrifugation, MACS, FACS or microfluidics. Solid or semi-solid tissues, including umbilical cord, adipose tissue and placenta, require tissue dissection and enzymatic dissociation, followed by differential adhesion or selective culture. However, increasing purity may impair cell function or remove rare functional subsets. For example, long-term hematopoietic stem cells (LT-HSCs) represent only around 1% of $CD34^+$ cells and remain difficult to distinguish precisely while maintaining adequate purity and function.

Figure. Comparative Analysis of Mainstream Isolation and Purification Technologies for Liquid Cell Suspensions

Density Gradient Centrifugation

- **Principle:** Separates cells into distinct layers under centrifugal force based on differences in cell density and size.
- **Advantage:** Straightforward operation; highly cost-effective.
- **Disadvantages:** Low purity; induces mechanical stress and potential cell damage.

Magnetic-Activated Cell Sorting (MACS)

- **Principle:** Labels cells via magnetic bead-conjugated anti-CD34 antibodies, isolating $CD34^+$ cells within a magnetic field.
- **Advantage:** High purity (reaching 90%); lower operational costs than FACS; widely used clinical approach.
- **Disadvantages:** Potential alteration of cell surface marker expression.

Fluorescence-Activated Cell Sorting (FACS)

- **Principle:** Identifies single cells via fluorescently labeled antibodies, applying electrostatic charge deflection for multi-parameter, high-precision sorting.
- **Advantage:** Highest achievable purity.
- **Disadvantages:** Slow throughput speed; exposes cells to shear stress damage.

Microfluidics

- **Principle:** Manages precise cell manipulation within microscale channels by exploiting physical or biological differences via external forces such as electromagnetic fields.
- **Advantage:** High throughput capacity; minimal cell damage or shear stress.
- **Disadvantages:** Technical bottlenecks in large-scale industrial manufacturing.

Source: Literature Review, Public Information, Frost & Sullivan Analysis

Core Technical Challenges (2)

In Vitro Culture and Expansion

Clinical-grade stem cell manufacturing requires a balance between cell yield, product quality and cGMP compliance. Conventional two-dimensional culture is suitable for research and early process development but is difficult to scale while maintaining batch-to-batch consistency. The industry is therefore adopting scalable systems such as stirred-tank, fixed-bed and hollow-fiber bioreactors. Stirred-tank systems offer high scalability and precise process control but require shear optimization; fixed-bed systems support low-shear perfusion culture but may develop nutrient gradients at high cell densities; and hollow-fiber systems enable automated, closed expansion. Cytokines, small molecules and matrix signals may also be used to support proliferation and stemness.

A key challenge is that extensive expansion may reduce stemness, homing capacity, marker expression, secretory function and biological potency. Culture conditions must therefore control oxygen levels, shear stress and nutrient distribution. Physiological hypoxia, often around 5% oxygen depending on cell type, may help preserve an undifferentiated state, whereas atmospheric oxygen can increase oxidative stress and differentiation. Spatial gradients and mechanical stress during scale-up may also compromise cell quality and batch consistency.

Figure. Primary Supplements in Cell Expansion

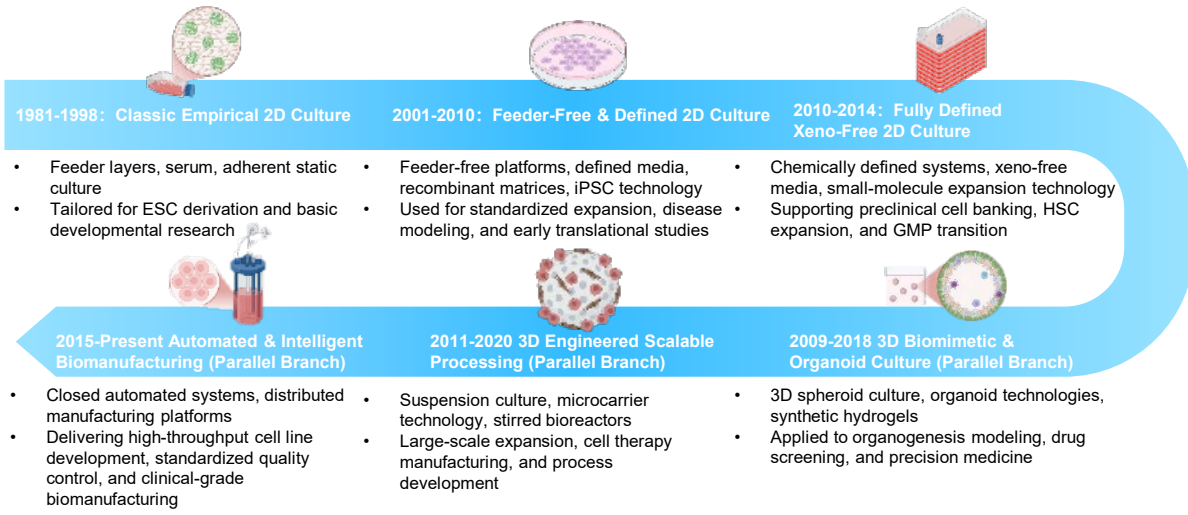
Name	Mechanism
Cytokine Cocktails	Deliver essential growth signals required for stem cell survival, proliferation, and differentiation, serving as the foundational support across diverse *in vitro* culture systems.
Small-Molecule Compounds	Modulate specific signaling cascades (such as PI3K/AKT) to precisely regulate the equilibrium between stem cell self-renewal and lineage differentiation, effectively replacing certain traditional cytokines.
Small-Molecule Additives	Function as auxiliary components across various stem cell systems (including ESCs, MSCs, and HSCs) to preserve undifferentiated states and suppress spontaneous differentiation.

In Vitro Culture Technologies

Cell culture has evolved from empirical benchtop protocols into standardized, intelligent biomanufacturing. Early efforts focused on reducing reliance on animal serum and xenogeneic feeder layers, with culture systems evolving from conventional 2D adherent culture toward feeder-free, chemically defined and xeno-free platforms.

Driven by translational demands, modern bioprocessing has advanced along complementary trajectories: synthetic hydrogels and biomimetic organoid cultures rectify 2D physiological inaccuracies during exploratory R&D; microcarrier suspension cultures and stirred bioreactors overcome mass transport limits in static systems to enable large scale industrial expansion; and fully closed, robotic fluidic platforms eliminate manual contamination and batch variability, establishing a high throughput, standardized biomanufacturing paradigm.

Figure. Timeline and Multi-Branch Evolution of *In Vitro* Cell Culture Technologies



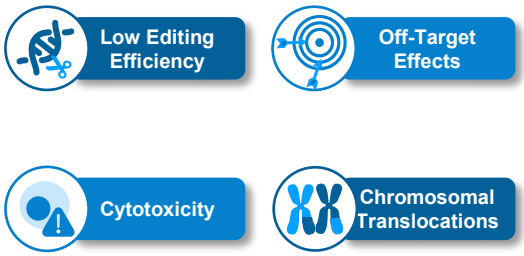
Source: Literature Review, Public Information, Frost & Sullivan Analysis

Core Technical Challenges (3)

Gene Modification and Editing

Gene modification and editing represent critical bioprocessing steps for introducing therapeutic genetic modifications into stem cell products, with delivery strategies broadly categorized into viral vectors and non-viral platforms. Lentiviral vectors are widely used in clinical applications because they can efficiently transduce quiescent hematopoietic stem cells and support stable genomic integration, which has underpinned multiple FDA approved ex vivo gene therapies. Conversely, retroviruses require active cell division and carry insertion oncogenesis risks, leading to declining clinical utility. Among non-viral options, electroporation is the most widely adopted physical method for directly transfecting large macromolecules like CRISPR ribonucleoprotein complexes, though it frequently causes marked cellular damage and diminished viability. Meanwhile, emerging platforms such as lipid nanoparticles (LNPs) and virus-like particles (VLPs) are advancing rapidly to deliver gene editing machinery with higher precision and lower cytotoxicity, establishing essential technical foundations for next-generation engineered stem cell therapeutics.

Figure. Key Challenges in Gene Editing



Formulation and Cryopreservation

In the development and application of stem cell therapeutics, formulation and cryopreservation technologies serve as pivotal links to safeguard cellular viability, enable product standardization, and ensure long term supply chain availability. Within this framework, formulation formats and cryoprotectant selections represent the two primary determinants.

Formulations are broadly classified into fresh and cryopreserved preparations: fresh preparations offer optimal cellular viability but suffer from a narrow stability window that limits them to immediate clinical point of care settings, whereas cryopreserved preparations allow long term ultralow temperature storage and facilitate large scale manufacturing and distribution, representing the dominant commercial format in the stem cell industry.

Cryoprotective agents comprise three distinct classes: traditional high concentration DMSO formulations demonstrate robust cryoprotection and widespread adoption but exhibit marked cytotoxicity; low concentration DMSO combination regimens, co formulated with trehalose and protective proteins, effectively attenuate cytotoxic effects; and novel DMSO free formulations completely exclude dimethyl sulfoxide to achieve superior clinical safety, representing the strategic focus of ongoing industrial refinement.

Quality Control

As living cellular therapeutics, stem cell products require comprehensive evaluation across identity, purity, potency, safety, and genomic stability. Manufacturing must follow end-to-end quality management and risk control principles to ensure safety, efficacy, and inter-batch consistency from donor procurement through release. Currently, potency assessment remains challenging: *in vitro* CFU assays show variable correlation with *in vivo* long-term engraftment owing to inter-laboratory standardization limits, and final products cannot undergo destructive *in vivo* validation. To establish standardized potency testing, emerging approaches leverage single-cell sequencing to predict stemness and AI models to integrate multi-parametric evaluations.

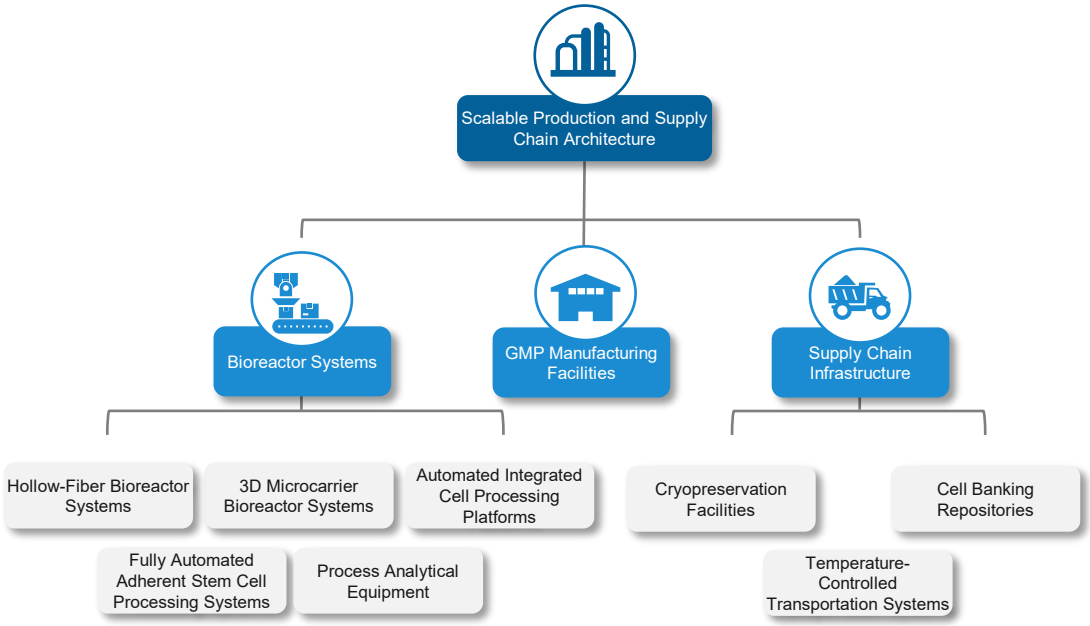
Quality Attribute	Objective	Key Analytical Methods
Identity	Confirm cell origin, lineage type, and genetic background	STR profiling (against the donor reference sample), cell morphology, surface marker profiling
Purity	Quantify target cell proportion and control process-related impurities/residues	Cell viability, surface marker analysis, residual vector/plasmid testing, toxic/hazardous residue assays
Potency	Evaluate biological activity underlying intended therapeutic efficacy	Differentiation potential (tri-lineage differentiation), functional validation (immunomodulatory capacity), biomarker-based bioassays
Safety	Ensure freedom from contamination, tumorigenic/oncogenic risks, and adverse immune responses	Microbiological testing (bacterial/fungal culture, mycoplasma, endotoxin), viral screening (HIV, HBV, HCV), residual undifferentiated iPSC detection, tumorigenicity testing (teratoma formation in immunodeficient mice), oncogenicity assessment (mutational expansion risk), cell viability
Genomic Stability	Verify normal chromosomal structure and number without mutagenic risk	Karyotyping, telomerase activity assay, whole-genome sequencing (as necessary)

Source: Literature Review, Public Information, Frost & Sullivan Analysis

Core Technical Challenges (4)

Large-Scale Production and Supply Chain

The large-scale manufacturing and supply chain architecture for stem cell products has evolved from traditional manual operations toward fully closed, automated, and intelligent "cell factory" paradigms. The stem cell supply chain exhibits distinct peculiarities: raw biological materials are sourced directly from medical institutions; autologous (patient-specific) products require bi-directional logistics (collection → manufacturing → reinfusion) under constrained sample processing time windows, whereas off-the-shelf allogeneic products necessitate long-term ultralow-temperature inventory control, comprehensive batch management, and strict execution of first-in, first-out principles. Maintaining this end-to-end chain requires close communication and collaboration between manufacturing enterprises and clinical institutions across both process technology and on-site operational dimensions.



Life-Cycle Risk Management

Life-cycle risk management for stem cell products must focus on three core risks: tumorigenicity, immunogenicity, and batch-to-batch consistency. Tumorigenicity stems from residual undifferentiated cells and gene mutations induced during *in vitro* expansion, requiring mitigation through *in vitro* and *in vivo* tumorigenicity testing alongside long-term clinical follow-up. Immunogenicity primarily arises from allogeneic HLA mismatching and xenogeneic antigens introduced during culture, where gene editing technologies (such as CRISPR-Cas9-mediated MHC knockout) serve as key strategies for reduction. Batch-to-batch consistency must be safeguarded via cGMP compliance, closed automated manufacturing systems, and multi-parametric release testing covering phenotype, function, and genomic stability.

Source: Literature Review, Frost & Sullivan Analysis

Unmet Needs and Future Technological Directions

While stem cell therapies have reached early clinical and commercial stages, critical bottlenecks remain: scalable expansion, standardized QC, DMSO-free cryopreservation, automated biomanufacturing, cost containment, and long-term safety monitoring.

Six Core Technological Directions in Stem Cell Industrialization

To enhance clinical accessibility and affordability, the global industry is driving systemic breakthroughs across six core technological directions: 1) developing feeder-free, xeno-free scalable expansion systems to preserve stemness; 2) establishing non-invasive real-time quality control across the bioprocess to guarantee batch-to-batch consistency; 3) advancing DMSO-free, serum-free cryopreservation protocols to minimize clinical adverse risks; 4) adopting closed automated bioreactors to achieve fully closed and highly automated manufacturing; 5) optimizing bioprocessing and substituting critical raw materials with domestic alternatives to lower per-treatment-course costs and increase clinical availability; and 6) implementing lifelong monitoring architectures leveraging single-cell sequencing and molecular imaging for precise tracking of *in vivo* cellular behavior and long-term safety.

Figure. Network Interrelationships of the Six Core Technological Directions in Stem Cell Industrialization

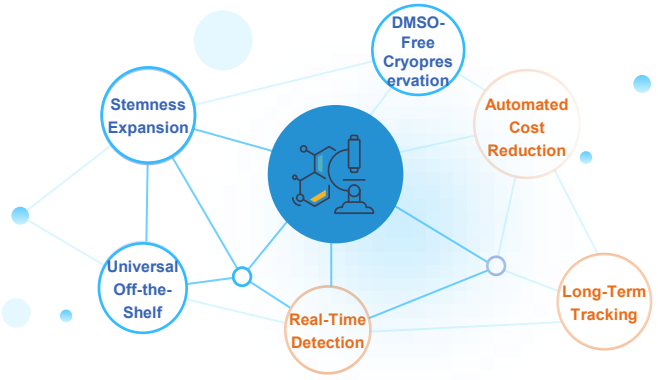


Figure. Core Pain Points and Cutting-Edge Solutions in Stem Cell Therapy

Stemness-Preserving Expansion

Traditional 2D cultures trigger differentiation drift and loss of stemness while relying on manual open processing, restricting single-batch yields and large-scale clinical supply.

Solutions

Type I collagen hydrogels mimic bone marrow niches to induce nestin-expressing perivascular stroma and stabilize HIF-1α, maintaining HSC stemness and reconstitution without exogenous cytokines; degradable 3D porous microcarriers enable scalable, automated MSC expansion that preserves multipotency better than 2D culture.

Off-the-Shelf Products

Allogeneic transplants carry immune rejection risks, whereas patient-specific autologous therapies involve prolonged manufacturing, high costs, and poor standardization, impeding immediate clinical accessibility.

Solutions

CRISPR knockout of HLA-I (B2M) and HLA-II (CIITA) combined with CD47/MIF overexpression creates immune-evasive universal iPSC banks for off-the-shelf allogeneic therapies that do not require conventional HLA matching.

DMSO-Free Cryopreservation and Thawing

Standard 10% DMSO formulations pose cytotoxic, neurotoxic, and hypersensitivity risks, necessitating strict dosage control, formulation optimization, or pre-infusion cell washing.

Solutions

Three experimentally evaluated DMSO-free or low-DMSO approaches—sucrose/glycerol/isoleucine formulation, ultrasound microbubble-delivered intracellular trehalose with dextran systems, and recombinant human albumin (Optibumin 25) with 1%–5% low DMSO—markedly lower toxicity while maintaining viability, immunophenotype, and function.

Cost Control and Automation

High manual operation ratios, pronounced batch-to-batch variation, and heavy reliance on imported media, consumables, and bioreactors inflate single-course treatment costs (hundreds of thousands to over RMB 1 million per treatment course), severely restricting clinical accessibility and commercial adoption of stem cell therapies.

Solutions

Optimizing critical raw materials and consumables, including clinical-grade serum-free media, nanoscale immunomagnetic beads, and single-use bioreactors, combined with closed automated bioreactors enables fully closed and highly automated manufacturing across cell enrichment, expansion, and formulation, thereby reducing labor, facility, and supply costs.

Real-Time Potency Testing

Conventional quality testing relies on offline, destructive, endpoint assays that fail to monitor functional dynamics and cellular heterogeneity in real-time during bioprocessing, hindering batch consistency control, timely detection of functional drift, and release risk management.

Solutions

Non-invasive online Process Analytical Technology (PAT) combining Raman spectroscopy with deep learning captures lipid and protein biomarkers in real time to assess functional state and potency-related attributes. This label-free, non-destructive approach detects cellular abnormalities 1 hour prior to morphological changes, supporting real-time bioprocess quality control.

Long-Term Safety Monitoring

In vivo distribution, homing, persistence, and differentiation of reinfused stem cells remain difficult to predict, posing long-term safety risks like tumorigenicity, aberrant differentiation, and immunogenicity, while lacking non-invasive, precise, life-cycle monitoring platforms and systematic evaluation frameworks.

Solutions

Integrating CRISPR barcoding single-cell lineage tracing, single-cell sequencing, and multimodal molecular imaging, such as PET and MRI, constructs an *in vivo* long-term tracking framework. This enables dynamic tracking, differentiation trajectory mapping, and safety profiling of transplanted stem cells, providing visual evidence for tumorigenicity, immunogenicity, and functional persistence monitoring.

Source: Literature Review, Frost & Sullivan Analysis

Frontier Technology Analysis (1)

To overcome industrialization bottlenecks in stem cell product universality, scalable manufacturing, and intelligent process control, next-generation technologies are rapidly evolving. In gene editing, platforms have advanced from early CRISPR/Cas9 systems to base editing and prime editing, significantly enhancing editing precision and safety to provide core toolkits for universal off-the-shelf cell product development. Regarding manufacturing bioprocesses, traditional batch cultures are progressively upgrading to closed perfusion and fed-batch strategies. Because live stem cell products are subject to strict quality-control limits on *in vitro* passage numbers and population doubling levels, intact live cell therapeutics cannot adopt end-to-end continuous manufacturing; continuous production models are currently best suited for cell-free derivatives such as stem cell-derived exosomes. By integrating bioreactors with online waste separation, these process systems enable automated high-density culture within effective expansion windows, efficiently reducing batch-to-batch variability and manufacturing costs. Concurrently, the deep integration of Industry 4.0 principles with Process Analytical Technology (PAT), artificial intelligence, and end-to-end automation is driving stem cell manufacturing toward intelligent, digitalized, highly efficient, and cost-effective paradigms.

Base Editing (BE) Technology

BE technology is a novel gene editing approach derived from CRISPR/Cas systems, classified into Cytosine Base Editors (CBE) and Adenine Base Editors (ABE). Taking CBE as an example, the system comprises a catalytically inactive dCas9 or nickase nCas9 fused to a cytosine deaminase, guided by sgRNA to target DNA loci and form an R-loop complex. Cas9 unwinds local DNA, allowing cytosine deaminase to bind unpaired single-stranded DNA and deaminate cytosines (C) to uracil (U) within a specific editing window. Subsequent DNA repair or replication replaces U with thymine (T), accomplishing precise C-to-T and G-to-A base transitions. Base editing repairs single-nucleotide mutations without generating DNA double-strand breaks.

Figure. Base Editing (BE) Technology

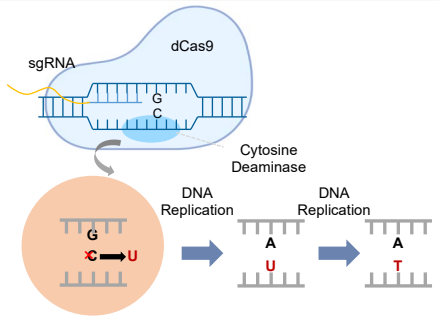
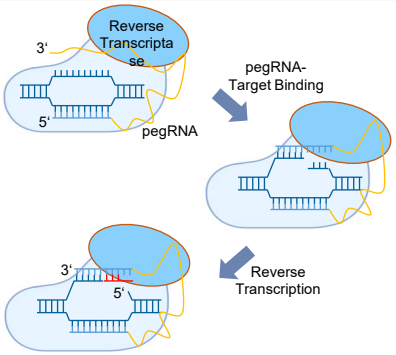


Figure. Prime Editing (PE) Technology



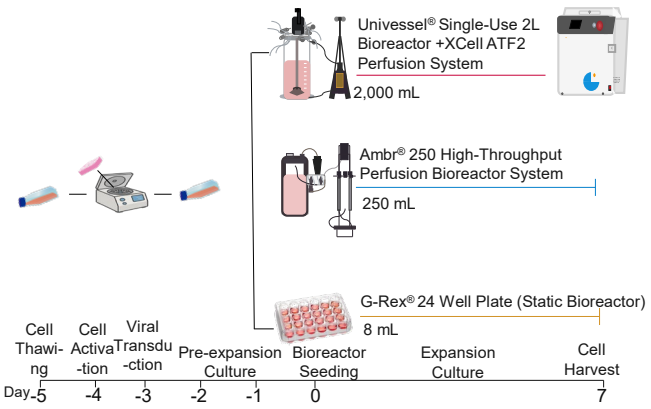
Prime Editing (PE) Technology

PE technology builds upon CRISPR/Cas9 utilizing an engineered prime editing guide RNA (pegRNA) to template desired editing sequences. The PE-pegRNA complex binds complementary genomic targets to form an R-loop. PE nicks one strand of target duplex DNA, releasing a 3' DNA end that hybridizes with the pegRNA extension to prime reverse transcription off the pegRNA template. Primer extension drives DNA polymerization to generate a 3' DNA flap containing the edit and homologies to downstream genomic sequences. Flap equilibrium displaces adjacent genomic strands, followed by 5' flap cleavage and ligation of the remaining nick, forming a heteroduplex with the edit on one genomic strand. DNA repair or replication incorporates the edit into the complementary strand, permanently fixing the modification. Beyond point mutations, PE mediates small insertions and deletions.

Perfusion Bioreactor Continuous Expansion Technology

Perfusion bioreactor continuous expansion builds on conventional batch culture by integrating cell retention devices with perfusion systems to establish closed-loop continuous or semi-continuous manufacturing paradigms. Fresh medium is continuously supplied while target cells are retained inside the reactor, with metabolic waste and spent media continuously depleted to maintain microenvironmental homeostasis, eliminating nutrient depletion and toxic metabolite accumulation seen in batch cultures. This technology increases cell density and overall yield, extends culture duration, reduces batch-to-batch variation, and minimizes manual intervention and manufacturing costs, serving as a core expansion module for scalable commercial production of cell therapies such as stem cells and CAR-T products.

Figure. Perfusion Bioreactor Continuous Expansion Technology



Source: Literature Review, Frost & Sullivan Analysis

Frontier Technology Analysis (2)

Alternating Tangential Flow (ATF) Continuous Separation and Purification

Replacing traditional batch centrifugation, ATF technology leverages an alternating tangential flow membrane filtration mechanism to enable inline, continuous harvest and purification of stem cells. Bioreactor fluid driven by a diaphragm pump flows back and forth across membrane modules; this alternating cross-flow minimizes membrane fouling while efficiently separating viable stem cells from dead cells and cellular debris. High-viability cells are continuously harvested as waste streams are purged, enabling ongoing product recovery without process termination. Beyond improving harvesting efficiency, ATF maintains cell viability, stemness, and functional activity, serving as a core purification module for continuous stem cell biomanufacturing.

Figure. ATF Continuous Separation and Purification

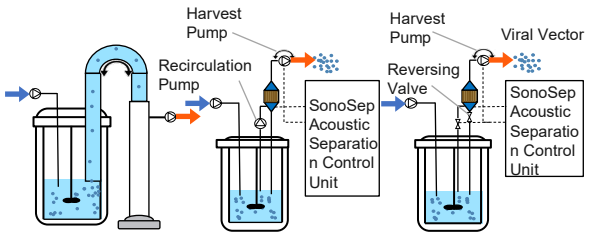
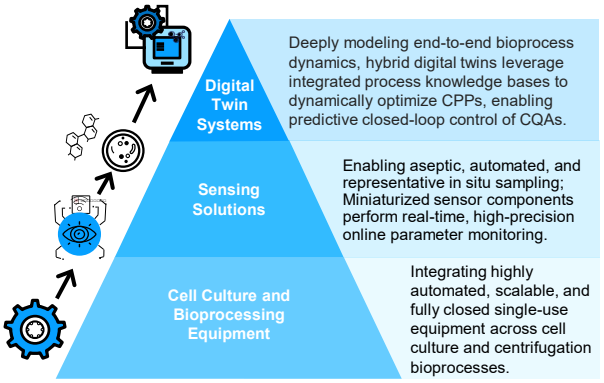


Figure. CGT 4.0 Hierarchy in PAT4CGT Framework



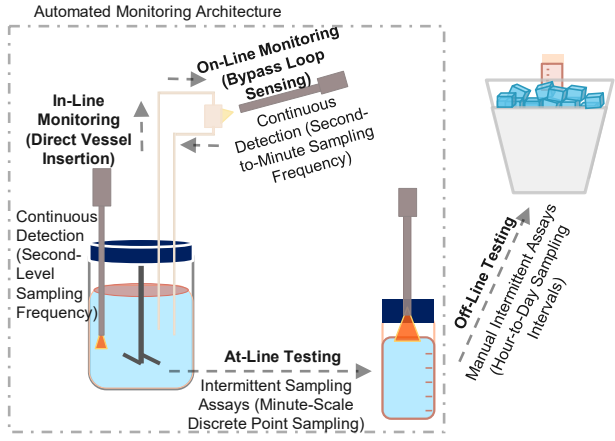
Intelligent Manufacturing

Upgrading from manual, operator-dependent workflows, intelligent biomanufacturing establishes a closed-loop platform by integrating Process Analytical Technology (PAT), machine learning algorithms, and fully automated systems. At the perception layer, non-invasive PAT tools monitor multidimensional cell parameters in real time. At the algorithmic layer, digital twins predict cell states and process deviations, dynamically adjusting critical process parameters (CPPs) to ensure target critical quality attributes (CQAs). Automated hardware at the execution layer implements closed-loop control, while automated data logging ensures regulatory compliance and full traceability under applicable GMP requirements. This architecture enhances process robustness and batch consistency, drives bioprocess self-optimization, and facilitates multi-site standardization for global commercialization.

In-Line/On-Line Non-Invasive Raman Spectroscopy

Eliminating offline sampling, in-line and on-line Raman spectroscopy uses optical sensing for real-time quality control and closed-loop process control across cell therapy manufacturing. In-line probes and on-line bypass loops capture spectral signatures of cellular lipids and proteins without destructive sampling, thereby reducing the risk of sample loss and contamination and enabling monitoring at intervals of seconds to minutes. Real-time telemetry of CPPs and CQAs provides early warnings for anomalous process shifts, ensuring dynamic end-to-end quality control without disrupting culture operations—a vital QC component for Industry 4.0 cell manufacturing.

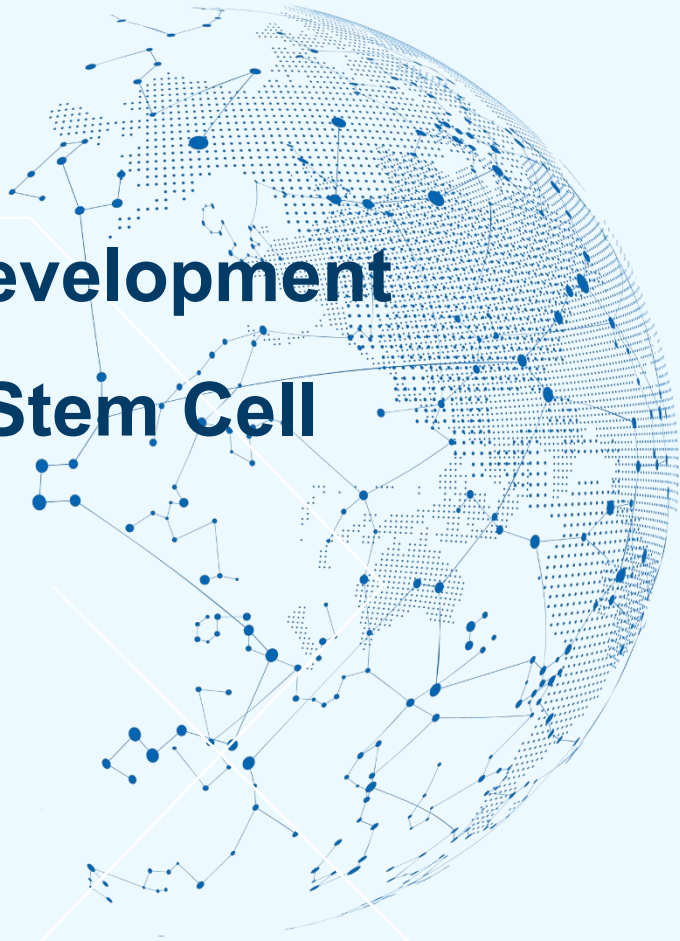
Figure. In-Line/On-Line Non-Invasive Raman Spectroscopy



Source: Literature Review, Frost & Sullivan Analysis

Chapter 8

Drivers and Development Trends in the Stem Cell Industry



08

Analysis of Driving Factors in the Stem Cell Industry

1 Clinical Demand

- In the field of major diseases such as cancer, neurodegenerative diseases, cardiovascular diseases and autoimmune disorders, traditional medicines and existing treatments have limited efficacy, and there is a lack of methods capable of reversing the course of the disease or curing it completely. At the same time, the global population ageing continues to accelerate, with the incidence and prevalence of degenerative and chronic diseases rising year on year, creating an increasingly urgent need for novel therapies capable of achieving tissue repair and regeneration. Furthermore, approximately 80 per cent of rare diseases worldwide lack effective treatments, leaving patients in a long-term predicament where 'no treatment is available'. Stem cell therapies, however, with their unique advantages—including self-renewal, multi-lineage differentiation and immunomodulation—offer hope for a fundamental cure for genetic disorders (such as thalassemia and severe combined immunodeficiency), and are expected to fill this significant clinical gap.

2 Technological Breakthrough

- The rapid development of gene-editing technologies—in particular, the optimization of the CRISPR/Cas9 system and the emergence of new editing tools such as base editing and prime editing—has reduced the risk of off-target effects and enhanced the precision and safety of gene modification, thereby laying the technical foundation for the creation of engineered stem cells and the correction of disease-causing gene mutations. Continuous advances in reprogramming technology have significantly improved the differentiation efficiency of iPSCs, leading to breakthroughs in their directed differentiation into functional cells (such as hematopoietic stem cells, cardiomyocytes, dopaminergic neurons and islet cells). The application of three-dimensional culture and advanced biomaterials technology has made organoid culture, the construction of organ-on-a-chip systems and large-scale expansion in bioreactors possible, thereby driving the standardization and scaling-up of cell manufacturing. The deep integration of artificial intelligence and automation technologies has not only enabled the intelligent optimization of cell culture conditions but has also significantly reduced human error and the risk of contamination through fully enclosed automated production systems, thereby enhancing the consistency of cell product quality.

3 Policy & Capital

- The increasingly robust policy environment and the continuous inflow of capital have provided strong external support for the development of the stem cell industry. In recent years, major global regulatory bodies such as the FDA, EMA and NMPA have successively issued or updated specific technical guidance documents and review criteria for stem cell products, charting a course for the industry's standardized development. At the same time, various accelerated approval mechanisms established by regulatory bodies—such as Regenerative Medicine Advanced Therapy (RMAT) Designation and Breakthrough Therapy Designation—have provided a faster route to market for stem cell products with relative clinical advantages. On the reimbursement front, public healthcare systems and commercial insurers are actively exploring innovative payment models, such as pay-for-performance and instalment plans, with the aim of reducing the financial burden of high one-off payments for advanced therapies on patients and improving access to treatment. On the capital front, substantial amounts of venture capital, private equity and industrial capital from around the world continue to flow into the cell therapy sector. Major multinational pharmaceutical companies are accelerating their expansion through in-house R&D, collaborative development or mergers and acquisitions, thereby injecting strong momentum into industry innovation.

4 Industrial Ecosystem

- An increasingly sophisticated industrial ecosystem provides a solid foundation for the large-scale development of the stem cell industry. The Contract Development and Manufacturing Organization (CDMO) system is maturing, offering start-ups and biotechnology companies professional services in cell process development, quality control and large-scale production, thereby lowering the barriers to entry and reducing initial capital expenditure. The ultra-low-temperature cold chain logistics system has undergone a comprehensive upgrade, with a -150°C liquid nitrogen dry transport network covering major global markets. This effectively safeguards the viability and stability of stem cell products throughout the entire storage and transport process, thereby removing logistical bottlenecks for commercialization. Furthermore, the number of accredited stem cell clinical research centers in China has been growing steadily. Thanks to its vast patient base, abundant clinical resources and vibrant innovation ecosystem, China has joined the global forefront of stem cell clinical research, with the number of new clinical trials launched annually ranking among the highest worldwide. This has fostered a complete translational chain from laboratory research to clinical application, providing strong support for the industry's continued expansion.

5 Cost-Effectiveness

- Cost control and efficiency improvements are the key drivers propelling stem cell therapies from personalised treatment towards widespread adoption. In the cell manufacturing stage, the adoption of large-scale production technologies—such as three-dimensional bioreactors, microcarrier culture and continuous manufacturing processes—has effectively reduced the manufacturing cost per cell. The development of universal 'off-the-shelf' products, particularly standardized cell products derived from iPSCs, enables a 'produce once, use for many' model. This reduces the time and expense required for individualized preparation and avoids the cost burden associated with preparing treatments separately for each patient. In terms of technological approaches, cutting-edge techniques such as direct in vivo editing are being actively explored. These aim to bypass the complex in vitro steps of cell isolation, editing, expansion and reinfusion, instead performing gene repair or cell reprogramming directly within the patient's body. This holds the promise of fundamentally simplifying the treatment process and reducing treatment costs, thereby truly realising the long-term goal of making advanced therapies accessible to a wider patient population.

Source: Literature Review, Frost & Sullivan Analysis

Analysis of Development Trends in the Stem Cell Industry

1 Product Form Trends

The product forms within the stem cell industry are evolving towards greater diversity. With the shift from live cells to cell-free products, 'cell-free' products such as MSC-derived exosomes and cell lysates—which contain no live cells—can effectively circumvent the stringent requirements for storage, transport and cold-chain management associated with traditional live-cell products, and are expected to become more convenient and stable next-generation therapeutic products. There is also an upgrade from single-cell therapies to combination products; the combined use of stem cells with biomaterial scaffolds, gene-editing technologies and targeted drugs can synergistically enhance therapeutic outcomes and broaden clinical applications. Furthermore, the sector is extending from disease treatment to preventive applications; stem cell storage services for healthy individuals, alongside products targeting consumer healthcare areas such as anti-ageing interventions and functional maintenance, are developing rapidly, driving the penetration of stem cell technology from serious medical care into the broader health sector focused on 'preventing illness before it occurs'.

2 Indication Expansion

The range of indications for stem cell therapies is rapidly expanding from traditional areas of strength to cover the full spectrum of diseases. In the field of oncology, cell therapy as a whole is making significant in-depth advances from hematological malignancies to solid tumors. New immunotherapy technologies, such as tumor-infiltrating lymphocytes and CAR macrophages, are continuously overcoming therapeutic challenges posed by the microenvironment of solid tumors, forming a complementary approach alongside stem cell therapies. The non-oncology sector is emerging as a key direction for the expansion of stem cell therapy indications and industry growth. In addition to areas with established clinical or commercial foundations—such as graft-versus-host disease (GvHD), ophthalmic repair, and skin/wound repair—new applications are primarily concentrated in musculoskeletal and age-related conditions, such as sarcopenia and postmenopausal osteoporosis; neurodegenerative diseases, such as Parkinson's disease and Alzheimer's disease; metabolic and liver diseases, such as diabetes and liver failure; and cardiovascular and cerebrovascular diseases, such as stroke, heart failure and myocardial infarction, driving the industry's in-depth development from cancer immunotherapy towards the full spectrum of regenerative medicine. At the same time, the scope of cell therapy applications continues to broaden, extending from the treatment of specific diseases to the fields of functional restoration and health intervention. Standardized consumer-oriented regenerative medicine sectors, such as aesthetic anti-ageing and organ function restoration, are rapidly emerging, opening up vast opportunities for industry growth.

3 Business Model Trends

The business model of the stem cell industry is undergoing a profound transformation, shifting from traditional medical services towards a platform-based, service-oriented and globalized approach. It is transitioning from a hospital-centerd, decentralized model to a platform-based model comprising 'centralized laboratory preparation combined with a network of regional infusion centers', ensuring the consistency and accessibility of product quality through a centralized, standardized cell preparation system. The industry is expanding from the sale of individual products to a service subscription model, with the emergence of annual cell storage fees, efficacy guarantee contracts and comprehensive health management services covering the entire course of treatment. By providing continuous services throughout the entire life cycle, companies are building long-term relationships of trust with patients. Moving beyond the confines of a single national market towards a global footprint, the widespread conduct of multinational, multi-center clinical trials has facilitated the simultaneous registration and market launch of standardized cell products across multiple global markets, thereby accelerating global access to advanced therapies.

4 Regulatory & Ethical

The ongoing refinement of regulatory science and ethical frameworks provides institutional safeguards for the orderly development of the stem cell industry. At the regulatory level, the promotion of innovative regulatory tools—such as Regenerative Medicine Advanced Therapy (RMAT) Designation, the designation of breakthrough therapies, and the design of adaptive clinical trials—has accelerated the review and approval process for stem cell products with clinical advantages. Ethical guidelines for gene-editing technologies are being refined, and clear red lines have been established regarding the application of gene editing in germline cells, thereby delineating clear ethical boundaries for technological innovation within the sector. At the international coordination level, the advancement of relevant guidelines by the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH) and the strengthening of global regulatory mutual recognition mechanisms have significantly facilitated the synchronized research, development and market launch of stem cell products across multiple countries, thereby reducing the regulatory costs for companies submitting applications across different regions.

5 Competitive Landscape

The competitive landscape of the stem cell industry is undergoing a profound transformation. Large multinational pharmaceutical companies are rapidly gaining control of core technologies and product pipelines through large-scale mergers and acquisitions and strategic partnerships, thereby becoming the dominant forces in the industry. At the same time, small and medium-sized biotechnology companies, by adopting a differentiated positioning and specializing in specific technological platforms, are establishing unique competitive advantages in cutting-edge fields such as off-the-shelf cell therapies, in vivo direct editing, and the directed differentiation of induced pluripotent stem cells (iPSCs) and embryonic stem cells (ESCs), thereby becoming a vital source of innovation for the industry. It is worth noting that Chinese domestic enterprises are accelerating their transition from early-stage imitative innovation to original innovation. In several sub-sectors—including targeted stem cell delivery technology, gene-edited hematopoietic stem cells and haploidentical transplantation—they have already joined the ranks of international leaders, demonstrating strong global competitiveness and are poised to become a key player in the future global stem cell industry landscape.

Source: Literature Review, Frost & Sullivan Analysis

Chapter 9

Capital Market Performance Analysis of Stem Cell Therapy Companies

09

Financing of Domestic Stem Cell Therapy Developers (1)

Figure. Examples of Funding in the Stem Cell Therapy Sector in China

Company Name	2023	2024	2025	2026*	Latest Financing
HELP Therapeutics			Series D		Series D; investors: JOLMO CAPITAL and Sinowisdom.
Darwin Biotech	Series B		Series D Series C		Series D; investors: Optics Valley Sci-Tech Venture Capital and Tibet Godson Investment.
uBriGene	Series C+	Pre-Series D			Nearly RMB 200m; investors: Beijing Changping Industrial Development Investment Fund and Beijing Medicine Health Industry Investment Fund.
XellSmart	Series A+	Series B	Series B+	Series C+ Series C	Completed six consecutive years of market-based financing, raising over RMB 1 billion across multiple rounds from top-tier investors and national funds, including FreeS Fund, Qiming Venture Partners, LAV, Sequoia China, 3SBio, the National Venture Capital Fund, and Tigermed etc. This includes the completion of Series C and C+ financing rounds totaling more than RMB 500 million.
iRegene Therapeutics		Series B	Series B++ Series B+	Series C+ Series C	completed Series C and C+ financing rounds totaling more than RMB500 million. Its investors include Luminous Ventures, Fortune VC, Apricot Capital, Northern Light VC, Vertex Ventures, Yuanxi Haihe, Ceyuan Ventures, Chengdu Sci-tech Investment Group, CSC Capital, Wuxi Capital Group, Tigermed, Dintaixing Capital and Celgene.
Zephyrm	Series B+ Series B	Series B++	Series C		Series C; investors: CAS Star and H&G.
Regend	Series B		Series B+	Series C	RMB 340m Series C; 7 new investors: Yuze Capital, Hefei High-Tech Investment, Hongtai Fund, Hefei Industry Investment and Gongqingcheng Fuhui; 5 existing investors, including FIRHealth Capital and Tasly, also made follow-on investments.
Nuwacell			Series B	Series C	Nearly RMB 2bn raised across multiple financing rounds; shareholders: state-owned institutions such as CM Health, Guangzhou Industrial, CRHC, Hefei Industrial; professional market-oriented investors such as Legend, GF Xinde, BioTrack, DCP, U-Shine; and industry partners such as Huadong Medicine and CSPC Guofang.
CytoNiche			Series B+	Series C	Series C: RMB 100 million+; Investors: Beijing Medical and Health Industry Investment Fund, Beijing New Materials Industry Investment Fund, ICBC Investment, BSCOMC, CRHC, Gaorong Capital, CICC Capital, and 3E Bioventures Capital.
Beijing Sanyouli Heze			Series A	Series C	Jingtai Lifeng invested in Series A.
Huode				Series C	Nearly RMB 200m Series C first close; new investors: Tasly Capital, Haibang Capital and Guangzhou Healthcare Industry Fund; existing investor: Longmen Capital.
Cellgenes	Series B+			Pre-Series C	>RMB 100m Pre-Series C, co-led by Taiping Healthcare Fund and BOC&GFIH Private Equity Fund Management; Green Pine Capital participated.
CellBri		Series B	Series B+		Series B+; investors: Fosun Health Capital and Changzhou Hi-tech Group.
Huateng Biotech	Series A+	Series B		Series B+	Series B+, tens of millions of RMB; investor: Pingyun Capital.
HemaCell	Series A		Series B Series A++ Series A+	Series B+	>RMB 100m Series B2; Series B extension nearly RMB 300m; investors: SCGC, Hebei Industrial Investment Fund, CDH Investments and Weidu Capital.

Note: The financing information shown is based on public disclosures as of July 2026. Companies are ranked by financing round from highest to lowest and, within the same round, by completion date.

Source: Public Information, Frost & Sullivan Analysis

Financing of Domestic Stem Cell Therapy Developers (2)

Figure. Examples of Funding in the Stem Cell Therapy Sector in China

Company Name	2023	2024	2025	2026*	Latest Financing
IxCell Biopharmaceutical			Series B		Series B led by Gansu State-Owned Capital Investment Group; Shenghui Capital and Qianhai Zhibo Capital participated.
UniXell		Series A+ Series A	Series A++	Series A+++	>RMB 50m Series A+++; investors: Panlin Capital, Village-wide Venture Capital and Waigaoqiao Private Equity Fund.
iCamuno Biotherapeutics	Series A++			Series A+++	>RMB 100m Series A+++; investors: Voyagers Capital, InnoVision Capital and Lianxin Capital.
Unisum			Series A+	Series A++	>RMB 100m fifth round, the sector's first with strategic/market investors: Haier Capital, CAS Investment, National VC Fund, China Broadcasting Capital and Rongyi VC.
Ruiyuan Biotechnology		Series A	Series A+		Series A+; exclusive investor: New Film Private Equity Fund Management.
Tuohua Biotechnology	Series A		Series A+		Series A+; exclusive investor: Changxing Fund.
Xiatong Biotechnology		Series A	Series A+		Series A+; exclusive investor: Xiahui Tiantong Technology.
Reprogenix		Series A	Series A+		RMB 500m Series A+; investors: China Reform Fund, Yuanbio Venture Capital, FuZhe Capital, Sinowisdom, Orient Renaissance Capital, Lotus Lake Capital, Yuhang State-Owned Capital Investment, GAGE Capital and Beicheng Fund.
Wiseheart MedVal		Pre-Series A		Series A+ Series A	Series A+, tens of millions of RMB; exclusive investor: Beijing Medical and Health Care Industry Investment Fund.
Yuanpin Biotechnology		Series A			Series A; investors: Beijing Huichenglin Ecological Restoration and Guangdong Juncheng.
Tianjin Hechuang Biotechnology		Series A			Series A, tens of millions of RMB, led by the Macau University of Science and Technology Foundation.
ElpasBio			Seed	Series A	>RMB 100m seed round led by TF Capital; new and existing investors participated.

Note: The financing information shown is based on public disclosures as of July 2026. Companies are ranked by financing round from highest to lowest and, within the same round, by completion date.

Source: Public Information, Frost & Sullivan Analysis

Global Stem Cell Licensing Transactions and M&A (1)

■ Licensing Agreements and M&A Cases in Stem Cell Therapy

Multinational pharmaceutical giants collaborate with stem cell therapy developers to co-develop pipelines, absorbing advanced technologies and platform expertise from target companies to establish a global presence in the regenerative medicine industry and accelerate the R&D progress of clinical-stage pipelines. Numerous pharmaceutical companies such as Novo Nordisk, Astellas Pharma, Nippon Shinyaku, and Santen Pharmaceutical have secured core stem cell technologies and mature clinical pipelines through exclusive licensing, asset acquisitions, and joint ventures, thereby strengthening their regenerative medicine business layouts and accelerating commercial expansion.

Figure. Licensing Transactions and M&A in Stem Cell Therapy

Date	Parties Involved	Deal Value	Transaction Details
2026.05	novo nordisk® / CELLULAR INTELLIGENCE	Undisclosed	The parties entered into an exclusive global collaboration to jointly advance the clinical development and commercialization of STEM-PD, a clinical-stage allogeneic pluripotent stem cell-derived dopaminergic progenitor cell therapy for Parkinson's disease.
2026.02	Ayrmid / ACA Pharma	Undisclosed	Ayrmid appointed ACA Pharma as the exclusive distributor for OMISIRGE® and granted it registration and distribution rights across Greater China and parts of Southeast Asia.
2026.02	Ayrmid / ariti® next to human	Undisclosed	Ayrmid entered into a distribution agreement with Ariti S.A. for Greece and Cyprus (Omisirge® is a stem cell / regenerative medicine product).
2026.01	ElpasBio / FOSUN KAIROS 复星凯瑞	RMB 450 million	ElpasBio granted Fosun Kairos exclusive commercialization rights for AlloJoin® (lotazadromcel, allogeneic adipose-derived MSCs) in mainland China, Hong Kong, and Macau.
2026.01	CYTORA / ZEOSCIENTIFIX	Undisclosed	The parties entered into a collaboration under which ZEO ScientifiX obtained exclusive U.S. commercialization rights for Cytora's hOMSC300 (allogeneic human oral mucosa-derived MSCs).
2026.01	CYTORA / MADE SCIENTIFIC	Undisclosed	The parties entered into a collaboration under which Made Scientific will handle U.S. domestic manufacturing.
2025.12	MEDIPOST / 帝国制药株式会社	RMB 127 million	MEDIPOST granted Teikoku Seiyaku exclusive commercialization rights for CARTISTEM® (umbilical cord blood-derived MSCs) in Japan.
2025.12	细胞产品国家工程研究中心 天津昂赛细胞基因工程有限公司 / HKRM	Undisclosed	Tianjin AmcellGene Engineering granted Hong Kong Regen Medtech Limited exclusive rights to its autologous umbilical cord-derived MSCs (Amcell) in Hong Kong.
2025.10	MRC 江苏亿珂再生医学 / GVisionary	Undisclosed	The parties established a joint venture, Visionary Yike Stemcell Technologies Inc., to jointly advance the global development and commercialization of Yike's stem cell therapy for diabetes.
2025.06	Help 艾尔普再生医学 / 華潤三九 CR SANJIU	Undisclosed	HELP Therapeutics and China Resources Sanjiu signed a joint development agreement for HiCM-188 (iPSC-derived cardiomyocytes) in mainland China.
2025.06	株式会社ツーセル / KAKEN 科研製薬株式会社	RMB 348 million	TWOCELLS granted Kaken Pharmaceutical exclusive development and commercialization rights for gMSC®1 (allogeneic synovial membrane-derived MSCs) in Japan.
2025.04	InfiniClone / SHINECO	RMB 143 million	Shineco Life Sciences acquired all assets of Singapore-based stem cell company InfiniClone, including its stem cell technology platform and intellectual property, for a total consideration of RMB 143 million.
2024.09	ENCell / Lucy Biotech 露曦生物科技	RMB 138 million	ENCell granted Lucy Biotech exclusive development and commercialization rights for EN001 (umbilical cord-derived MSCs) in six Asian countries and regions: Hong Kong, Macau, Taiwan, Vietnam, Thailand, and Singapore.
2024.07	CARTherics / UNIQUEST Delivering Innovative Solutions / THE UNIVERSITY OF SYDNEY	Undisclosed	Cartherics entered into dual agreements with The University of Queensland (through its commercialization subsidiary UniQuest) and The University of Sydney, licensing its iPSC cell lines for the joint development of iPSC-derived cardiomyocytes (iPSC-CMs) for the treatment of end-stage heart failure.
2024.03	Aderans / STEMSON Therapeutics	Undisclosed	Aderans granted Stemson Therapeutics exclusive worldwide development and commercialization rights to its hair regenerative stem cell technology.

Source: Public Information, Frost & Sullivan Analysis

Global Stem Cell Licensing Transactions and M&A (2)

Figure. Licensing Transactions and M&A in Stem Cell Therapy

Date	Parties Involved	Deal Value	Transaction Details
2024.01	Athersys, Healios	RMB 14 million	Following the Phase III trial failure of its lead product MultiStem for ischemic stroke, Athersys filed for Chapter 11 bankruptcy protection in the U.S. Healios acquired all of Athersys' assets via credit bid for RMB 14 million.
2023.09	Ixaka, Alaya.bio	Undisclosed	Alaya.bio acquired all core assets of Ixaka, including its *in vivo* CAR-T platform and adipose-derived MSC pipeline.
2023.03	Human Life CORD JAPAN, 持田製薬株式会社	Undisclosed	The parties will jointly develop and commercialize HLC-001, an umbilical cord-derived MSC-based cell therapy, for refractory indications including non-infectious pulmonary complications following hematopoietic stem cell transplantation. Mochida Pharmaceutical will handle distribution in Japan and pay an upfront fee, milestone payments, and sales royalties.
2023.02	EverXa Therapeutics, LINEAGE CELL THERAPEUTICS	Undisclosed	Eterna granted Lineage an exclusive option and license to its hypoimmunogenic iPSC cell lines in the field of neurological disorders.
2023.02	Capricor Therapeutics, 日本新薬	Undisclosed	The parties entered into a collaboration under which Capricor granted Nippon Shinyaku exclusive commercialization rights for CAP-1002 in Japan.
2022.12	biorestorative therapies, Regenexx Las Vegas	Undisclosed	The parties entered into a collaboration under which Regenexx granted BioRestorative an exclusive worldwide license to its stem cell technology for chronic lumbar disc disease.
2022.08	curi bio, NEXEL	Undisclosed	Curi Bio granted NEXEL exclusive distribution and service rights for its iPSC drug discovery platform in South Korea.
2022.04	JRM, METCELA	Undisclosed	Japan Regenerative Medicine transferred all rights to JRM-001 (cardiac-derived MSCs) to Metcela.
2022.01	Capricor Therapeutics, 日本新薬	Undisclosed	Capricor granted Nippon Shinyaku exclusive commercialization rights for CAP-1002 in the United States.
2021.07	EXCELLTHERA, astellas	Undisclosed	ExCellThera granted Astellas Pharma exclusive rights to its UM171 molecule in the field of pluripotent stem cells.
2021.05	STEMEDICA, pulthera	Undisclosed	Stemedica granted Pulthera exclusive development and commercialization rights to its bone marrow-derived MSC technology for all respiratory indications worldwide (excluding certain markets).
2020.12	pluri, INNOVARE r&d	Undisclosed	Pluristem granted Innovare exclusive commercialization rights for its PLX cells (placenta-derived MSCs) in Mexico.
2020.10	Steminent, SCM Lifescience	Undisclosed	Steminent Biotherapeutics granted SCM exclusive development and commercialization rights for Stemchymal® in South Korea.
2020.10	Personalized Stem Cells, sorrento THERAPEUTICS	Undisclosed	Sorrento obtained exclusive worldwide development and commercialization rights for COVI-MSC (adipose-derived MSCs).
2020.10	Bone Therapeutics, PREGENE, HG 领晟医药	RMB 435 million	Bone Therapeutics granted Link Health and Pregene exclusive development and commercialization rights for ALLOB in Greater China, Taiwan, Singapore, South Korea, and Thailand.
2020.05	jCyte, Santen	RMB 1,789 million	jCyte granted Santen Pharmaceutical exclusive development and commercialization rights for jCell (human retinal progenitor cells) outside the United States.
2020.04	SanBio, OcuMension	RMB 544 million	The parties entered into a strategic collaboration to jointly develop two of SanBio's ophthalmic stem cell products, SB623 and MSC2 (bone marrow-derived MSCs).

Source: Public Information, Frost & Sullivan Analysis

Chapter 10 Profiles of Stem Cell Industry Companies

Note: Companies are listed alphabetically by their names.

10

Stem Cell Industry Company – Boyalife

Company Profile

Boyalife is a life sciences company with end-to-end capabilities across the development and application of cell therapy technologies. It houses three core business brand segments: Boyalife Bio-banking, Boyalife Biopharma and Boyalife Devices, spanning multiple business pipelines including clinical R&D of cell therapies, automated cell equipment and medical devices, stem cell and immune cell banking, and cell drug R&D. Boyalife's long-term goal is to establish itself as a globally competitive, leading enterprise in the life and health industry, striving for enhanced well-being.

- **Boyalife Bio-banking:** Focuses on cell banking and health management. It operates high-standard, automated, clinical-grade cell banks and manufacturing centers that provide a comprehensive range of cell banking and preparation services. It also offers health consultation and management services throughout the family lifecycle, using cell technologies to support family health.
- **Boyalife Devices:** Focuses on automated cell-processing equipment, medical devices and related technical services. Its portfolio includes automated tissue and blood cell separation systems, automated 3D cell culture systems and automated cell cryopreservation systems. It also provides patented, automated operating-room point-of-care systems to support the clinical application of cell-based technologies in China.
- **Boyalife Biopharma:** Focuses on cell therapy clinical research and product development, including stem cell therapies, immune cell therapies, cell-based medical technologies and cell-derived products. Boyalife has established clinical partnerships with more than 40 Grade A tertiary hospitals in China.

Development History

2009-2010
2009 : INCOSC was jointly established in July 2009 by seven leading stem cell research institutions from China and overseas. It was recognized by the Ministry of Science and Technology of the People's Republic of China (MOST) as a Major International Cooperation Project and was included in Jiangsu Province's Scientific and Technological Achievement Transformation Program.
2010 : Boyalife Bio-banking was established.

2011
Boyalife's Beijing facility was established and accredited as a provincial-level public technology service center.

2016
Jointly conducted the world's first clinical study of stem cell therapy for progeria with Peking University Third Hospital.

2020
ThermoGenesis, a Boyalife subsidiary, rapidly advanced a COVID-19 antibody therapy into clinical development, while its rapid COVID-19 antibody test kit received U.S. FDA marketing approval.

2022
Following a comprehensive reorganization, Boyalife consolidated its domestic operations under three business segments: Boyalife Bio-banking, Boyalife Devices and Boyalife Biopharma.

2023
Boyalife's independently developed Wharton's Jelly MSC Injection received IND clearance in China, advancing precision medicine for knee osteoarthritis.

2024
Boyalife entered into a formal collaboration with the Women and Children's Center of Beijing Children's Hospital to launch a clinical study of MSC therapy for intrauterine adhesions and begin patient recruitment.

2025
Boyalife completed both DMF and INCI filings for its human MSC-derived exosome ingredient. The company also published research in international journals identifying potential new targets for innovative CAR-T therapy.

2026
Boyalife was named an "Industry Competitiveness Leading Brand" and jointly recognized by Forbes and Frost & Sullivan as a "China Industry Development Leading Enterprise". It also passed its AABB re-audit, marking 14 consecutive years of AABB accreditation.

Core Advantages

Production and Quality Management System

Aligned with International Standards
- Three international certifications underpinning industry-leading cell manufacturing and quality management standards
- GMP-compliant laboratories and manufacturing facilities aligned with Chinese, U.S. and EU standards
- Clinical-grade cells tested by the National Institutes for Food and Drug Control
- Core patents covering the full value chain

R&D Capacity

Globally competitive full cell industrial chain
- 10,000 m² clinical-grade GMP laboratory
- 5 core laboratory sites across China
- Clinical partnerships with more than 40 Class A tertiary hospitals in China

Automation Platform

Automated cell therapy medical device platform
- Cell therapy medical devices with multiple NMPA and FDA 510(k) clearances
- Automated operating-room point-of-care application system
- Integrated solutions for automated clinical-grade cell manufacturing and preparation
- Applied across clinical programs covering more than 20 diseases

Core Patents

Possesses core patents covering the entire industrial chain
- Nearly 100 core cell technology patents
- Nearly 3,000 clinical applications involving perinatal stem cells
- Patented peripheral blood / bone marrow cell therapy platform
- A comprehensive quality control and quality management system for stem cells, immune cells and exosomes

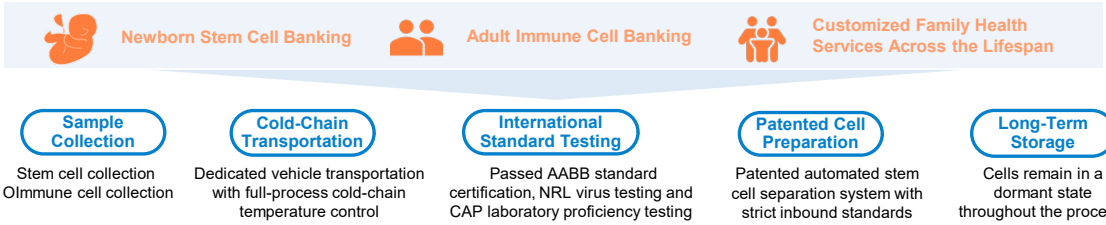
Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Boyalife

Core Business

Boyalife Bio-banking, a core business of Boyalife, has operated for 17 years and specializes in banking perinatal stem cells from newborns, as well as stem cells and immune cells from adults. As a leading player in the cell banking industry, it has obtained three international certifications and passed relevant proficiency testing under the standards of AABB, a WHO-designated NRL and CAP. It has also passed testing by China's National Institutes for Food and Drug Control, demonstrating compliance with clinical-grade cell quality standards.

Boyalife 博雅生命



- **Boyalife Devices** supports standardized, cost-efficient, large-scale cell manufacturing while maintaining high levels of cell recovery, viability and purity. It integrates three specialized systems: the AXP® rapid separation system for efficient sample processing and high cell recovery, the BACCS™ cold-chain system for precise temperature control and full traceability, and the BioArchive automated liquid-nitrogen storage system for controlled cryopreservation and high post-thaw cell viability. Together, these systems form an integrated, high-quality cell-processing workflow.

Original target cell recovery rate > 90%

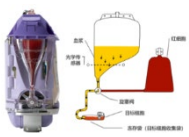
Original target cell viability > 95%

Cultured and harvested cell viability > 95%

24-hour viability maintained above 90%

Cell product purity reaches 99%

AXP® system



- ✓ Mononuclear cell recovery rate > 97%
- ✓ CD34+ stem cell recovery rate > 98%
- ✓ Red blood cell depletion rate > 88%
- ✓ 30-minute rapid separation, capable of processing six samples simultaneously

BACCS™ system

- ✓ Integrates traceability, positioning and real-time temperature monitoring
- ✓ Precisely senses temperature changes of 0.1°C
- ✓ Effectively ensures sample temperature stability during transportation

BioArchive® system

- ✓ Individual sample storage and retrieval to minimize thermal disturbance
- ✓ Post-thaw cell viability reaches 94% after sample retrieval
- ✓ Patient postoperative survival rate increased by 10% compared with traditional MVE

- **Boyalife Biopharma** focuses on cell therapy product development and is advancing multiple development programs involving placenta-derived stem cells, umbilical cord-derived stem cells, exosomes and immune cells. Its portfolio includes clinical research in degenerative conditions such as progeria, premature ovarian failure and osteoarthritis, as well as immune disorders including lupus erythematosus and scleroderma. It also conducts collaborative studies of autologous bone marrow concentrate for sports injuries, women's anti-aging applications, vascular diseases, and organ and tissue damage, including liver cirrhosis.

Core Technology Platforms

	PlacentaPro® Perinatal Stem Cell Process Development and Translational Platform	INCOCELL® Cell Therapy Development Platform	VitaEXO® Exosome R&D and Translation Platform
Technology	Perinatal MSCs（MSCs）	Induced pluripotent stem cell (iPSC) reprogramming	Engineered exosome delivery
Focus	Osteoarthritis, progeria, chronic diseases	Universal, off-the-shelf cell drugs	Neurodegenerative diseases, macular degeneration
Positioning	Cell resources: neonatal cell banking and MSC clinical translation focused on personalized cell therapies	Technical innovation: addressing cell-source standardization and scalable manufacturing	Precision medicine: high-throughput manufacturing and the development of cell-free therapies
Features	Extensive experience in isolating, manufacturing and clinically applying placenta- and umbilical cord-derived perinatal MSCs, supported by mature technologies and substantial clinical experience in stem cell therapies for age-related and degenerative diseases, including osteoarthritis, premature ovarian failure and diabetes.	Building a portfolio of stem cell R&D programs and a GMP-compliant iPSC induction, differentiation and manufacturing system, while advancing related preclinical research and clinical translation.	Extensive capabilities in high-throughput exosome production and clinical translation, with a focus on neurological and lower-limb degenerative diseases. The platform is also developing endogenous and exogenous cargo-loading technologies to optimize the targeted delivery of proteins and nucleic acids and enhance translational and therapeutic outcomes.

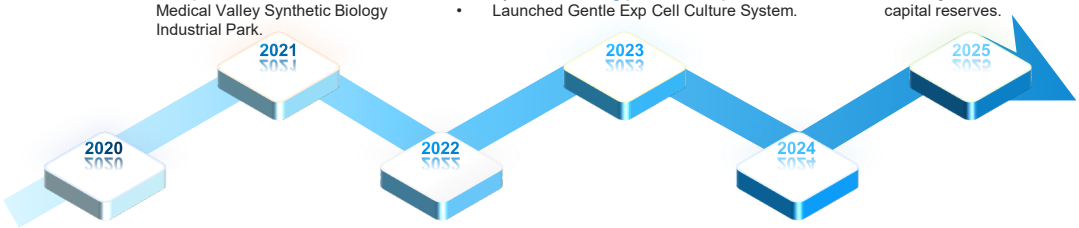
Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – CellBri

■ Company Profile

Founded in March 2020, CellBri is an industrialization capability builder dedicated to the CGT field. Centered on integrated bioprocess-equipment innovation, the company empowers CRO/CDMO platform construction to accelerate the standardized translation of cell therapies from early research and clinical trials to commercialization. CellBri continuously bridges enterprises, hospitals, research institutions, and international resources to advance the CGT industrial ecosystem and expand its global footprint. To date, CellBri has served over 200 enterprises and 100+ cell therapy pipelines, with operations spanning more than 10 countries worldwide.

■ Development History



2020
- CellBri was officially founded.
- 10 months post-founding, launched **China's first integrated cell processing system.**

2021
- Completed Pre-Series A and Series A financing rounds **totaling tens of millions of RMB.**
- Selected among the first cohort of enterprises to settle in South China Medical Valley Synthetic Biology Industrial Park.

2022
- Completed Pre-Series B financing round **exceeding RMB 100 million**.
- Recognized as **a National High-Tech Enterprise.**
- Launched first commercial product line: Gentle Flex Series Enclosed Automated Cell Processing System.

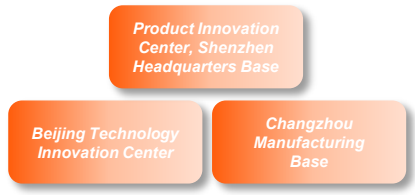
2023
- Launched Gentle P-Pac High-Precision Automated Fill & Finish System, filling a critical domestic market gap.
- Launched Gentle MagS Magnetic Bead Sorting System**, breaking patent monopolies.**
- Launched Gentle Exp Cell Culture System.

2024
- Completed a financing round of **nearly RMB 200 million**.
- Established U.S. subsidiary.
- Recognized as a **national-level "Little Giant" enterprise**.
- Constructed an 11,000 m² Global R&D and Industrialization Headquarters and Global Technology Innovation Center.

2025
- Achieved **over 70% YoY revenue growth** and reached financial break-even for the first time.
- Initiated Series C financing series, securing RMB hundreds of millions in capital reserves.

■ Core Platforms

CellBri has established a three-part framework comprising its Shenzhen Headquarters Base, Beijing Research Institute and Changzhou Manufacturing Base. Serving as the company's global R&D and industrialization center, the Shenzhen Headquarters Base drives frontier R&D, large-scale testing, and commercial translation to accelerate innovations from bench to market. The Beijing Research Institute focuses on foundational core technologies such as microfluidic chips, bolstering upstream innovation and industry-academia-research collaboration to break key automation bottlenecks in cell therapy. The Changzhou Manufacturing Base manages commercial manufacturing and supply chain management, featuring cGMP-compliant facilities, full-process self-reliance, and robust quality systems to ensure reliable global delivery. Through integrated synergy across R&D, validation, manufacturing, and quality control, CellBri continuously expands its global-grade cell therapy automation platforms and industrialization capabilities.



Full Lifecycle Commercialization Empowerment

CellBri provides comprehensive solutions across the entire lifecycle of cell therapies—from early research and clinical translation to commercial manufacturing—powered by automated processing technologies, process adaptation capabilities, and industrialization systems. Deeply covering technical pathways including MSCs, CAR-T, NK, and TCR-T, the company has supported over 40 leading cell drug candidates across China and the U.S. through critical developmental milestones. Delivering nearly 100 process solutions, CellBri helps biopharmaceutical companies, hospital platforms, and CDMO partners shorten development timelines, enhance manufacturing stability, and advance pipelines from concept design to scalable, standardized, and commercial realization.

Comprehensive Proprietary Intellectual Property

Committed to original upstream innovation, CellBri has built an advanced, full-spectrum product portfolio backed by over 400 patents to fulfill diverse process demands across all product classes and lifecycles. Setting high-performance industry benchmarks across key operational metrics, CellBri has completed the commercial loop for high-end synthetic biology equipment—from technological invention to large-scale deployment—enabling high-efficiency, cost-effective, and large-scale manufacturing for cell and gene therapies.

Manufacturing Capacity Expansion

CellBri is accelerating the construction of a dual-base synergistic framework combining its "Shenzhen Headquarters Smart Manufacturing Hub + Changzhou Consumables Supply Chain Base." The Shenzhen Headquarters drives automation equipment R&D, engineering validation, system integration, and smart manufacturing capacity; the Changzhou Base focuses on the large-scale, reliable supply of critical consumables, with a total planned cGMP-compliant production footprint of 20,000 m². Achieving full self-reliance across both equipment and consumables, CellBri enhances product consistency, delivery stability, and supply chain security—continuously empowering the industrialization efforts of pharma companies, CRO/CDMO platforms, and global partners.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – CellBri

■ Core Products

Focusing on bioprocessing equipment innovation for cell therapies, CellBri has constructed an automated product matrix spanning the end-to-end workflow: Isolation-Sorting-Transfection-Expansion-Counting-Harvesting-Formulation-Release. Through over 10 iterated series of standalone automated devices, CellBri delivers full-lifecycle coverage from early R&D to commercial manufacturing. Integrating bioprocesses with hardware tools, CellBri supports 100+ pipeline scenarios, enhancing manufacturing consistency and driving the industry's shift from customized R&D to scalable production with precise, efficient, and compliant solutions. The core product ecosystem is anchored by the CellFAB One All-in-One, Gentle Flex, and Gentle P-Pac series, alongside the Gentle Exp, Gentle n-MagS/m-MagS, and CB-LeakGuard lines.

All-in-One Automation: The CellFAB One Series

Redefining Automated Cell Processing Paradigms

The CellFAB One All-in-One System is CellBri's flagship automated platform, engineered to deliver high consistency, robust stability, and minimal operator dependency across the cell therapy continuum from R&D to commercial manufacturing. By integrating key unit operations—cell processing, expansion, media exchange, harvesting, washing, and formulation—within a unified closed system, it mitigates contamination risks while while improving batch-to-batch consistency and throughput and throughput. Supporting diverse cell types, bioprocesses, and application scenarios, it provides a foundational solution for biopharma, clinical centers, academia, and CRO/CDMO platforms to rapidly establish standardized cell manufacturing capabilities.

Automated & Enclosed

CellFAB One E
For bead-free processes

Flexible & Open

CellFAB One M
For micron-sized bead processes

Out-of-the-Box Manufacturing

CellFAB One N
For nano-sized bead processes

CellFAB One MN
For nano-sorting + micro-activation processes

CellFAB One I
For bead-free processing + incubator viral transduction

Modular Automation — Gentle Series:

Customized Automation for Targeted Unit Operations

The Gentle Series comprises standalone automated systems designed to streamline critical unit operations within cell therapy workflows, spanning PBMC isolation, magnetic bead processing, and final filling/finish processing. This modular design enables stepwise workflow construction, optimizing one unit operation at a time.

Gentle Flex Pro

Multi-Step Processing Capabilities: PBMC isolation, magnetic bead incubation, viral transduction, washing, and aliquoting.
Supports sample volumes up to 50 L, processes up to 1×10^{11} T cells.
High flow rates up to 220 mL/min, achieving over 90% recovery rates.
FaSe™ Rapid Separation Technology, low-shear separation preserving high cell viability.

Closed Automated Cell Processing: Engineered for solid tumor applications and high-volume allogeneic workflows.

Gentle n-MagS

Nanoparticle Magnetic Separation System Optimized for $CD3^+$ T cells and other target cell populations.

Fully Automated Enclosed Magnetic Separation
Delivers ≥ 95% purity, ≥ 95% viability, and ≥ 60% recovery rates
Integrated flow sensors and real-time gravimetric weighing functions
Connects smoothly with Gentle Flex Pro to enable end-to-end workflow automation

Gentle P-Pac

Flexible filling volumes of 2–150 mL per bag, with total batch capacity up to 3 L (up to 20 bags per cycle)
Volumetric precision within ± 0.3-2% with concentration coefficient of variation (CV) ≤ 5%
Maintains a temperature of 2–8 °C throughout filling to safeguard cell viability
10× faster than manual processing, ensuring consistent container integrity
Enables simultaneous multi-volume filling across separate channels

Purpose-built for both viral vector and cell therapy processing

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Cellgenes

Company Profile

Cellgenes Biotech Co., Ltd. was founded in 2016 and is a high-tech enterprise focusing on the R&D of innovative stem cell drugs. Based on a profound understanding of MSC mechanisms in maintaining body homeostasis and injury repair, the company has built a full-chain independent core technology cluster covering "source discovery – process scale-up – precise delivery – new drug R&D", including clinical-grade MSC large-scale expansion, targeted delivery systems for efficient homing to lesion tissues, directed sorting of MSC functional subpopulations along distinct development pathways, and in situ activation and regeneration regulation of endogenous MSCs. This system effectively solves the bottleneck problems of functional heterogeneity and efficacy stability in MSC therapy, providing new strategies for critical and severe diseases, neuropsychiatric diseases and ageing-related diseases.

In its clinical pipeline, Cellgenes has secured five IND approvals for stem cell new drugs. The lead candidate for acute-on-chronic liver failure (ACLF) has completed Phase II and is preparing to initiate a registrational Phase III trial—the first MSC therapy in China to reach this stage for the indication, with globally leading progress. The chronic ischemic stroke programme has advanced steadily into Phase II and ranks among the domestic first tier.

In 2026, the world's first MSC new drug for sarcopenia received IND approval and entered Phase I, establishing an early strategic foothold in ageing-related diseases. Under State Council Decree No. 818, the company has also obtained approvals to conduct clinical research on stem cell biomedical technologies for Alzheimer's disease, cerebral small vessel disease in older adults and ankylosing spondylitis, underscoring its sustained innovation and forward-looking pipeline strategy.

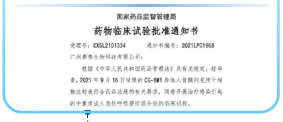
Guided by the belief that "life can be repaired," Cellgenes has built a full-chain new-drug platform compliant with CNAS and ISO 9001 standards, spanning early target discovery, process development, compliant manufacturing and clinical translation. With solid clinical data and an international outlook, the company is steadily advancing toward becoming a globally competitive leader in innovative biopharmaceuticals.

Development History

2016 — Company registered and established

2016-2019 — Released multiple scientific research achievements and accumulation

2020 — Obtained the first IND approval



2020 — Successively completed multiple rounds of market-oriented financing

松禾资本 中国太平 CHINA TAIPING 达安基因 DA AN GENE 粤财中报 YUECAI ZHONGYIN PRIVATE EQUITY

2024 — Obtained support from two National Key R&D Programs

2025 — Nansha pilot first: national first case

原创 羊城晚报·羊城派 张华
2025-06-28 08:30
2025年6月，广州市第一人民医院南沙医院（下称"南沙医院"）传来令人振奋的消息：全国首例异体骨髓间充质干细胞治疗慢加急性肝功能衰竭临床应用在广州南沙成功实施。这一里程碑式突

2026 — 2 ageing-related INDs & completed ACLF Phase II

2026 — Initiated sarcopenia Phase I/IIa; advanced targeted MSC and pluripotent stem cell R&D

Founder Introduction

Andy Peng Xiang, Professor of Sun Yat-sen University, Founder of Cellgenes

Director of the National-Local Joint Engineering Research Centre for Stem Cells and Regenerative Medicine

National-level Innovative Leading Talent

Chief Scientist of Key R&D Programme

Led in *more than 20* national and provincial/ministerial major projects

Published *more than 100* SCI papers as corresponding author in journals such as Nature

13 authorized invention patents and *6* international patents

Led the formulation of *2* group standards including "Human MSCs"

Achievements and Honours

- National-Local Joint Engineering Research Centre
- Guangdong Stem Cell and Regenerative Medicine Innovation Platform
- Dual certification of CNAS and ISO quality systems
- Member of Guangdong Stem (Somatic) Cell Clinical Research Quality Control Centre
- Sun Yat-sen University Doctoral Training Base and Postdoctoral Research Workstation
- National, provincial, municipal and district major science and technology special projects
- Led the National Key R&D Programme on "Stem Cell Research and Organ Repair"

- National High-Tech Enterprise
- Provincial Specialized, Refined, Differential and Innovative SME
- First Prize of 2025 China Innovation and Entrepreneurship Competition (Guangdong Division)
- 2025 KPMG China 3rd "Biotech Innovation Leading 50 Enterprises"
- 2024 Awarded "Guangzhou High-Tech Innovation Growth Top 20 and Rising Stars"
- 2017 "Development Zone Entrepreneurship Leading Talent Plan"

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Cellgenes

Technology Platforms and R&D Pipeline

Cellgenes' independently developed bone marrow MSC injection CG-BM1 has cumulatively obtained 5 NMPA IND approvals, with indications covering acute-on-chronic liver failure, ischemic stroke, acute respiratory distress syndrome, sarcopenia and postmenopausal osteoporosis. Among them, the CG-BM1 pipeline for acute-on-chronic liver failure has completed Phase II, the chronic ischemic stroke pipeline has advanced to Phase II, and senile sarcopenia has initiated Phase I.

On the basis of the first-generation MSC large-scale expansion platform, the company has further laid out the second-generation precise targeted MSC platform and the third-generation pluripotent stem cell induction and differentiation platform, reserving subsequent pipelines around acute ischemic stroke, inflammatory bowel disease, sepsis and Alzheimer's disease, forming an R&D pipeline layout of "first-generation platform clinical breakthrough, second- and third-generation platforms indication expansion".

	First Generation: Bone Marrow MSC CG-BM1 MSC Large-Scale Expansion Platform	Second Generation: Precise Targeted MSC CG-BM2 MSC Targeted Product Construction Platform	Third Generation: Pluripotent Stem Cell Induction and Differentiation Platform CG-X Directed Induction and Differentiation Platform
Leading Advantages	Stable and compliant bone marrow collection channels Unique serum-free culture system Mature and efficient production quality control process	Pioneering targeted delivery technology Optimized GMP-grade transfection technology	Stable clinical-grade PSC resources Unique cell differentiation pathways Innovative treatment strategies
Pipeline Selection Logic	Inflammatory diseases, regulation of paracrine function, mainly systemic critical and severe diseases, senile degenerative diseases and neuropsychiatric diseases, in target organs where MSCs can spontaneously enrich.	Focus on the construction of targeted lesion tissue capabilities, expanding to target organs where MSCs are difficult to spontaneously enrich.	Further expand to functional degenerative diseases, restoring body function by exogenous supplementation of functional cells
R&D Pipeline	• Acute-on-chronic liver failure • Postmenopausal osteoporosis • Acute respiratory distress syndrome • Chronic ischemic stroke • Senile sarcopenia • Autism	• Acute ischemic stroke • Inflammatory bowel disease • Sepsis • Steatohepatitis • Atopic dermatitis	• Alzheimer's disease • Hypogonadism

Acute-on-Chronic Liver Failure

The pathogenesis of acute-on-chronic liver failure (ACLF) is complex, with poor efficacy of single-target drugs and high mortality, urgently requiring new treatment strategies. Professor Andy Peng Xiang's team previously found that MSCs can regulate immune balance, reduce secondary infections and protect damaged hepatocytes. In collaboration with Professor Bingliang Lin's team at the Third Affiliated Hospital of Sun Yat-sen University, they initiated the world's first large-sample RCT study of allogeneic bone marrow MSC for ACLF, increasing patient survival rate from 55.6% to 73.2%. On this basis, the Cellgenes team, led by Dr. Sunxing Yan, carried out industrial development, obtained the implied approval to initiate a clinical trial for allogeneic bone marrow MSC injection (CG-BM1) for ACLF, and rapidly completed Phase I clinical trial, confirming safety and preliminary efficacy. Currently, the double-blind, randomised, placebo-controlled Phase II clinical trial has been completed, and Phase III clinical trial is about to be initiated.

Core Team

Strong Team Strength

Technical translation professional lineup composed of **top R&D team + top venture capital**

Cellgenes' core team possesses full-chain expertise spanning stem cell drug process development, pharmacological efficacy evaluation and clinical R&D. R&D staff account for over 80% of employees, with more than 80% holding master's or doctoral degrees, reflecting a strong scientific research capability.

- The company's management team brings extensive experience in stem cell industrialisation and government-enterprise investment and financing collaboration, overseeing daily operations and external business development. Four specialised teams support key commercialization functions: the R&D scientist team drives technology breakthroughs and IP strategy; the quality management team ensures full-process compliance; the medical affairs team liaises with clinical institutions and coordinates trial-related medical communications; and the regulatory affairs team advances IND applications and drug registration project management—collectively covering the critical links required for commercialising stem cell new drugs.

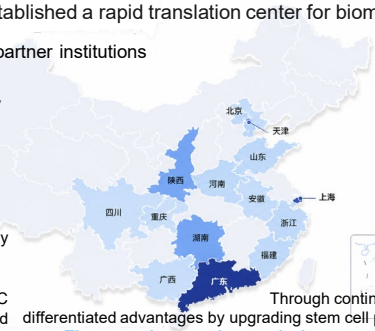
Core Advantages

Cellgenes possesses a mature stem cell large-scale production system and preclinical project operation management capabilities, with complete preclinical research experience for stem cell products, successful IND application experience and clinical trial operation management experience, and has established a rapid translation center for biomedical new technologies.

Clinical Resources

40+ Phase I/II/III clinical partner institutions

- The First Affiliated Hospital of Sun Yat-sen University
- The Third Affiliated Hospital of Sun Yat-sen University
- Peking Union Medical College Hospital
- Ruijin Hospital Affiliated to Shanghai Jiao Tong University School of Medicine
- West China Hospital of Sichuan University
- The First Affiliated Hospital of Zhejiang University School of Medicine
- The First Affiliated Hospital of Xi'an Jiaotong University



Production Capacity Advantages

Cellgenes operates a ***3,000 m²*** GMP-compliant clinical translation base (CNAS L17624 & ISO 9001 dual-certified) that ensures R&D compliance and consistent quality. An additional ***6,000 m²*** of industrial space is under construction, securing production capacity for Phase III trials and future commercial manufacturing and enabling a seamless transition from R&D to industrialisation.

Technical Advantages

Lead Candidate (allogeneic human bone marrow MSC injection) has received ***5*** IND clearances from NMPA; led the "14th Five-Year" National Key R&D Programme key special project on "Stem Cell Research and Organ Repair"; cumulatively applied for ***15*** patents with ***11*** authorised, building solid technical barriers.

Strategic layout

Through continuous technology iteration, Cellgenes has established differentiated advantages by upgrading stem cell products from universal to precision-targeted formats. ***The second-generation precisely targeted MSCs*** address the industry challenges of low lesion enrichment and short retention time associated with conventional stem cells; ***the third-generation pluripotent stem cell induction and differentiation platform*** enables blood-brain barrier penetration; and ***the in situ activation and regeneration regulation platform targets endogenous MSCs.***

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – CytoNiche

Company Profile

CytoNiche is a biotechnology company dedicated to advancing the industrialization of cell and gene therapy (CGT) through scalable, intelligent 3D cell manufacturing technologies. Its 3D FloTrix™ platform delivers an integrated ecosystem of GMP-grade dissolvable microcarriers, serum-free media, and GMP-compliant equipment and consumables, enabling seamless manufacturing across the entire cell production workflow—from expansion and harvesting to downstream processing and filling. By integrating advanced 3D culture technologies with intelligent, closed-system manufacturing, the platform enables the efficient production of billions of high-quality cells while optimizing production time, costs, facility footprint, and contamination control. Supported by FDA Master Files and adoption in registered clinical trials and a commercialized cell therapy product, CytoNiche is advancing global access to cell technologies through scalable manufacturing innovations.

Development History

2018

2018: Beijing CytoNiche Biotech Co., Ltd. established

2019-2020

- 2019: Tianjin CytoNiche Biotech Co., Ltd. was established
- 2020: Original 3D TableTrix™ Microcarriers and 3D FloTrix™ Pilot-Scale Bioreactors were launched

2021-2022

- 2021: 3D microcarriers obtained 2 China's CDE and 1 U.S. FDA pharmaceutical excipient registrations; Tianjin CytoNiche Intelligent Technology Co., Ltd. established
- 2022: "3D Microcarrier-Based Large-Scale Intelligent Cell Manufacturing Technology" won **Geneva Invention Gold Medal**; the 3D FloTrix™ cell technology platform was launched

2023-2024

- 2023: Shanghai CytoNiche Biotech Co., Ltd. established; secured additional FDA qualifications for 3D Microcarriers and MSC serum-free medium
- 2024: One-Stop and Single-Use Cell Intelligent Manufacturing Solutions were launched

2025

- Supported the manufacturing and commercialization of China's first approved stem cell therapy, at approximately one-seventieth the price of comparable U.S. products
- Established its international headquarters, CytoNiche Biotech Pte. Ltd., in Singapore

2026

- Supported the IND approval of the China's first exosome-based drug
- 3D RecomTrix™ Microcarriers obtained 3 U.S. FDA & 1 China's CDE pharmaceutical excipient registrations

Full Product Line

Core Consumables

3D TableTrix™ Microcarriers & 3D RecomTrix™ Recombinant Collagen Microcarriers

3D FloTrix™ Digest

3D FloTrix™ Serum Free Medium for MSCs

3D FloTrix™ MSC Chemically Defined Serum-Free Medium

3D FloTrix™ NK Cell Serum Free Medium Kit

Cell Culture

3D FloTrix™ microSPIN Multiplex System

3D FloTrix™ miniSPIN FLEX System

3D FloTrix™ vivaROCK Cell Culture System

3D FloTrix™ vivaSPIN Cell Culture System

3D FloTrix™ vivaSPIN-SU Single-Use Cell Culture System

3D FloTrix™ megaSPIN Single-Use Cell Culture System

Cell/Exosome Harvesting

3D FloTrix™ vivaPREP Cell Processing System

3D FloTrix™ vivaPREP ULTRA Cell Processing System

3D FloTrix™ vivaEXO Exosome Harvesting System

3D FloTrix™ megaEXO Exosome Harvesting System

Formulation & Filling

3D FloTrix™ vivaFILL Cryovial Filling System

3D FloTrix™ vivaPACK PRO Cryobag Filling System

3D FloTrix™ vivaVIAL Filling System

Supporting Reagents and Consumables

3D FloTrix™ Lysis Buffer Residual Detection Kit

3D FloTrix™ Microcarrier Residual Detection Kit

3D Stirred Cell Culture Plate

Disposable Spinner Flasks (125/500 mL)

3D FloTrix™ vivaSPIN Culture Processing Kit

3D FloTrix™ vivaSPIN-SU Cell Culture Bag

3D FloTrix™ vivaROCK Cell Culture Bag

3D FloTrix™ megaSPIN Cell Culture Bag

vivaPREP Disposable Processing Kit

vivaPREP ULTRA Disposable Cell Processing Kit

3D FloTrix™ vivaEXO Processing Kit

3D FloTrix™ vivaFILL Cryovial Filling Tubing Kit

3D FloTrix™ vivaPACK PRO Cryobag Filling Kit

3D FloTrix™ vivaVIAL Filling Tubing Kit

Auxiliary Equipment

- 3D FloTrix™ vivaWELD TPE Sterile Tube Welder
- 3D FloTrix™ vivaSEALER Tube Sealer
- 3D FloTrix™ vivaWELD PVC Sterile Tube Welder
- 3D FloTrix™ vivaSEALER-PVC Handheld Heat Sealer

The one-stop "consumables + equipment + services" solution enables large-scale, automated, intelligent, and closed-system manufacturing of cell therapy and cell-derived products, supporting production at the hundred-billion-cell scale.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – CytoNiche

■ Core Products

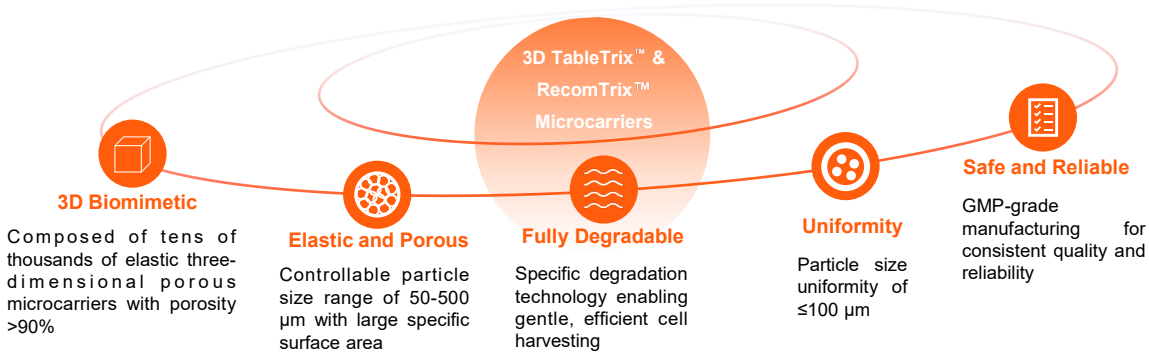
Leveraging its proprietary 3D FloTrix™ technology, CytoNiche has independently developed the 3D TableTrix™ Microcarriers and 3D RecomTrix™ Recombinant Collagen Microcarriers (xeno-free) series.

Technical Principle: These products create a three-dimensional, dynamic biomimetic microenvironment that supports the large-scale expansion of adherent cells. Combined with proprietary dissolvable microcarrier technology, they enable efficient and gentle cell harvesting while minimising cellular stress.

Application Scenarios: These products are widely used in upstream process development for stem cell therapies, extracellular vesicles, vaccines, and protein therapeutics, as well as regenerative medicine, organoid research, and emerging applications such as cultivated meat and food technologies.

Qualifications: The core product 3D TableTrix™ microcarrier series has obtained 3 U.S. FDA Master File registrations and 2 China CDE pharmaceutical excipient registrations, and has passed qualification and safety evaluations by the National Institutes for Food and Drug Control (NIFDC).

Built on its proprietary 3D microcarrier technology, CytoNiche delivers an integrated 3D cell manufacturing platform with customised, end-to-end solutions spanning the full production workflow—from cell culture and harvesting to formulation and filling. The platform enables scalable, automated, intelligent, and closed-system manufacturing of cell therapy and cell-derived products at the hundred-billion-cell scale.



■ Core Advantages

Tsinghua Original Innovation

Founded by ***Professor Yanan Du´ s team from Tsinghua University***, with Tsinghua as a co-founding shareholder. The platform is rooted in Tsinghua's scientific achievements and backed by ***with over ten years*** of R&D, establishing a robust technology foundation for large-scale cell manufacturing and intelligent production.

Clinical Validation & Industry Empowerment

Serving ***more than 2,000*** research institutions, biopharmaceutical enterprises and medical institutions worldwide, the platform has been validated across diverse stem cell and mammalian cell manufacturing applications. It ***has also supported the industrialized production of China´ s first stem cell drug***, demonstrating strong technological maturity and industry recognition.

Globally Leading Technology Platform

Independently developed 3D porous, degradable microcarriers and supporting process platforms, holding over 100 intellectual property rights and nearly 20 international patents, with dozens of high-level publications. Enables high-density cell expansion, high-viability harvesting, and large-scale production, providing key technical support for cell drug industrialisation.

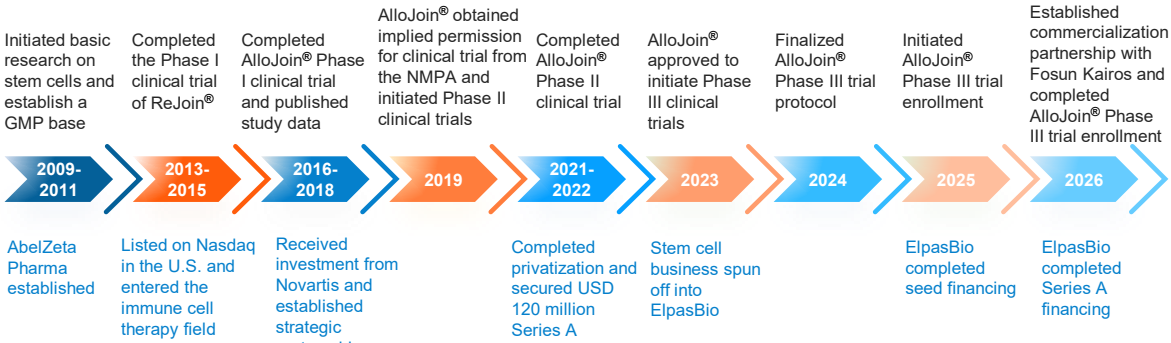
Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – ElpasBio

Company Profile

ElpasBio Co., Ltd. is a clinical-stage biotechnology company specializing in regenerative medicine. It was spun off from AbelZeta Pharma in 2023 and completed a seed financing round in the tens of millions of U.S. dollars in 2025. With 15 years of experience in stem cell development, ElpasBio has developed and explored multiple autologous, allogeneic, and multi-source stem cell products, supported by production and R&D facilities compliant with Good Manufacturing Practice (GMP) in Wuxi and Shanghai, China. Its core product, AlloJoin®, is the first stem cell drug for knee osteoarthritis approved to enter Phase III clinical trials in China.

Development History

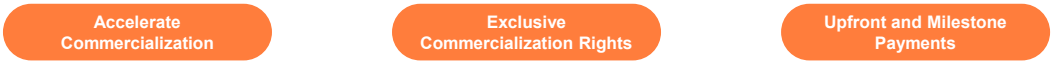


Core Products

AlloJoin® is a proprietary product developed by ElpasBio based on its advanced allogeneic MSCs technology platform. By inhibiting inflammation and repairing damaged cartilage, the product aims to provide safe and long-lasting symptom relief for osteoarthritis patients and has the potential to become the world's first approved regenerative medicine product with the potential to modify the disease course of knee osteoarthritis. AlloJoin® has demonstrated favorable safety and clinical efficacy in Phase II clinical trials, featuring what the company describes as the world's first dual-primary-endpoint design for a pivotal Phase III trial of a KOA stem cell therapy.

AlloJoin®	Iotazadromcel
Product Type	Allogeneic human adipose-derived MSCs injection
Target Indication	Knee Osteoarthritis, KOA
Mechanism of Action	By inhibiting inflammation and repairing damaged cartilage, AlloJoin® aims to provide safe and long-lasting pain relief and functional improvement for osteoarthritis patients. It not only addresses clinical symptom improvement but also focuses on structural cartilage repair.
Key Clinical Milestones	• AlloJoin® is the first independently developed stem cell product in China to receive implied permission from the CDE to proceed directly into Phase II clinical development, and the first stem cell drug for knee osteoarthritis in China approved to enter Phase III clinical trials. • The pivotal Phase III clinical trial initiated in April 2025 adopts the world's first "dual endpoint" design, with both symptom improvement and structural cartilage repair as primary evaluation endpoints.
Commercialization Progress	In January 2026, ElpasBio entered into a strategic partnership with Fosun Kairos, granting the latter exclusive commercialization rights for AlloJoin® in mainland China, Hong Kong, and Macau.

AlloJoin®, a proprietary product developed by ElpasBio based on its advanced allogeneic MSCs technology platform, demonstrated favorable safety and preliminary efficacy in a Phase II clinical trial, including improvements in knee pain and stiffness symptoms and potential slowing of cartilage degradation in KOA patients. Early trends of cartilage regeneration were observed in the high-dose group, while long-term follow-up showed reduced cartilage loss compared with the control group. Leveraging Fosun Kairos's commercialization capabilities and insights in cell therapy, ElpasBio granted Fosun Kairos exclusive commercialization rights for AlloJoin® in mainland China, Hong Kong, and Macau.



Source: Company Information; Frost & Sullivan Analysis

Stem Cell Industry Company – ElpasBio

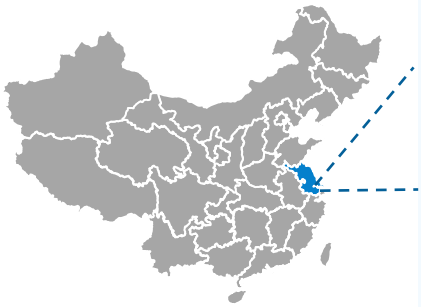
■ Pipeline

As an innovative biotechnology company independently operated after its spin-off from AbelZeta Pharma, ElpasBio focuses its pipeline on regenerative medicine, with AlloJoin® as the core program and broader development of promising stem cell products and stem cell-derived exosome products.

Product	Technology Platform	Indication	Preclinical	IND	Clinical Phase I	Clinical Phase II	Clinical Phase III
AlloJoin® Allogeneic Adipose-derived MSCs Injection	Stem cells	Knee osteoarthritis			NMPA		
				FDA			
Allogeneic Adipose-derived MSCs Injection	Stem cells	Knee osteoarthritis (with 3D-printed scaffold application)					
Allogeneic Adipose-derived MSCs Injection	Stem cells	Tendon disease					
Allogeneic Adipose-derived MSCs Injection	Stem cells	Nonunion fracture					
Allogeneic Adipose-derived MSCs Injection	Stem cells	Osteoporosis					
Stem Cell Derivatives	Stem cell derivatives	Skin injury					
Stem Cell Derivatives	Stem cell derivatives	Diabetic foot ulcer					

■ Core Advantages

Capabilities Supporting Product Commercialization



Stem Cell R&D and Manufacturing Base

Exosome R&D Laboratory

- Planned production capacity: 30,000 doses/year
- Advancement of existing and automated manufacturing processes toward BLA submission
- Compliance with AABB accreditation requirements and ISO 9001 certification standards
- Over 1,000 standardized operating procedures (SOPs) established

- ElpasBio currently operates an approximately 5,300 m² clinical manufacturing facility in Wuxi Huishan
- An approximately 1,800 m² exosome product R&D and raw material manufacturing base in Shanghai Pudong New Area

End-to-End Intellectual Property Protection

Granted Patents	Patent Applications
19	*17*

Leveraging over 30 core patents covering the entire value chain from sample collection, isolation and expansion, storage and transportation to final product formulation, ElpasBio has established an end-to-end intellectual property protection system. This comprehensive IP portfolio provides a strong technological moat, supporting its leading position in domestic Phase III clinical development of core pipelines and successful execution of major commercialization licensing transactions.

Leadership by Experienced Pharmaceutical Executives and Leading Scientists

ElpasBio's core team is led by experienced pharmaceutical executives and leading scientists, bringing extensive industry expertise and over 15 years of dedicated experience in stem cell development.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – HELP Therapeutics

Company Profile

Founded in 2016, HELP Therapeutics is a global leader in developing innovative regenerative therapies for aging-related intractable diseases. Dedicated to its mission, "Live Better & Longer," the company advances novel therapeutics for cardiovascular and other age-related conditions to help humanity live healthily to 120. Leveraging proprietary patented cell culture solutions to boost differentiation efficiency and reduce upstream manufacturing costs, combined with its HELP Cell-foundry industrial platform, HELP Therapeutics delivers safe, effective, quality-controlled, and affordable innovative therapies to patients worldwide.

艾尔普再生医学
HELP THERAPEUTICS

Development History

2016
- HELP Therapeutics was founded.

2019
- Initiated the world's first clinical study of iPSC-derived cardiomyocytes for severe heart failure.

2020
- 1-year follow-up results of the world's first iPSC-derived cardiomyocyte clinical trial for severe heart failure were published in *Nature*.
- "Human iPSC-derived Cardiomyocyte Injection" passed quality verification by the NIFDC.

2021
- Domestic pioneer: China's first clinical study of iPSC-derived cardiomyocytes for heart failure completed official filing with the NHC.
- Selected for National 13th Five-Year Plan Science and Technology Innovation Achievement Exhibition (the first enterprise in the cell therapy field).

2023
- "Human iPSC-derived Cardiomyocyte Injection" received NMPA clearance of its IND application.

2024
- "Human iPSC-derived Cardiomyocyte Injection" secured IND approval from the U.S. FDA.
- China's first MSC-based investigational drug for osteoporosis granted IND implied clearance by the NMPA.

2025
- 48-month long-term follow-up data for iPSC-derived cardiomyocyte therapy in severe heart failure patients published in *JACC: BTS*.
- Entered into a co-development agreement for "Human iPSC-derived Cardiomyocyte Injection" (HiCM-188) in Mainland China with China Resources Sanjiu Pharmaceutical Company.
- Recognized as the sole Chinese enterprise named an "Emerging Leader" in global stem cell therapy by authoritative international reports.

2026
- *Nature Medicine* has published HiCM-188 clinical trial data for advanced ischemic heart failure.
- Initiated the world's first pivotal clinical trial for iPSC-derived cardiomyocyte therapy.

Core Platforms

As China's pioneer in leveraging iPSC technology for cell therapy R&D and commercialization, HELP Therapeutics maintains a core technological moat anchored in integrating fundamental stem cell biology with automated, large-scale manufacturing.

HELP Cell-foundry Automated Mass Production Platform

As the world's first AI-driven, fully automated cell manufacturing line, this platform was featured in China's National 13th Five-Year Plan Science and Technology Innovation Achievement Exhibition. Overcoming traditional manual culture bottlenecks—such as long turnaround times, high batch-to-batch variation, and low yields—it enables scalable, standardized cell "smart manufacturing." A single production line has an annual capacity sufficient for approximately 100,000 patient treatments, dramatically reducing cost of goods sold (COGS) while enhancing product stability, batch consistency, and biomanufacturing success rates.

Automated Manufacturing

HELP Cell-foundry achieves end-to-end automation. From cell expansion to isolation and sorting, each unit operation is precisely automated to enable high-throughput production, reducing operator-related errors, minimizing batch variance, and improving production efficiency and product consistency.

Real-Time Monitoring & Feedback

Integrated with intelligent sensors and inline monitoring systems, the platform tracks CPPs such as temperature, pH, and dissolved oxygen in real time. Leveraging machine learning analytics, it dynamically adjusts culture conditions to maintain an optimal microenvironment, ensuring robust cell viability and consistent CQAs.

Flexible Adaptation

HELP Cell-foundry offers versatile adaptability across diverse cell lineages and therapeutic applications. Whether culturing specific cell lines or executing targeted screening and sorting, the platform can be customized to operational requirements to deliver optimal yields and quality.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – HELP Therapeutics

■ R&D Pipeline

Advancing off-the-shelf allogeneic cell therapies for age-related degenerative intractable diseases, HELP Therapeutics is committed to delivering safe, reliable, and affordable cell products. Currently, the company's iPSC-based off-the-shelf pipeline targets cardiovascular, orthopedic, and ophthalmic indications. In cardiovascular disease, its lead candidate, HiCM-188 (human iPSC-derived cardiomyocyte injection for severe heart failure), has entered a pivotal study intended to support a potential future marketing application.

艾尔普再生医学 HELP THERAPEUTICS

Field	Products	Indications	Proof of Concept	Pre-clinical	Phase Ⅰ	Phase Ⅱ	Phase Ⅲ
Cardiovascular Diseases	HiCM-188 (China)	Advanced Heart Failure					
	HiCM-188 (U.S.)	Advanced Heart Failure					
	HiCM-388 (China)	Acute Myocardial Infarction					
Bone Metabolic Diseases	HMM-910 (China)	Postmenopausal Osteoporosis					
Ocular Disease	HiO-630 (China)	Dry Age-related Macular Degeneration					

HiCM-188 Clinical Progress

As an innovative cell therapy for severe ischemic heart failure, HiCM-188 utilizes a novel "precision localization + targeted implantation" strategy. Combining radionuclide imaging and cardiac magnetic resonance (CMR) to visualize infarcted and injured myocardium, the therapy delivers iPSC-derived cardiomyocytes directly into lesions via a grid-pattern intramyocardial injection technique. This approach is intended to promote myocardial regeneration and functional cardiac recovery, representing a potential investigational treatment for patients with advanced heart failure who have limited treatment options.

The product is currently being evaluated in a multicenter, randomized, controlled, confirmatory Phase III clinical trial in China (REVIVE-HEART), with an expected enrollment of 80 patients with advanced heart failure (NYHA Class III–IV). Interim results from completed Phase I and Phase II trials demonstrated initial feasibility and safety profiles.

■ R&D Laboratories

CRIC | Cardiac Regeneration Innovation Center

Dedicated to developing and translating cardiac regenerative therapies, HELP Therapeutics leads the global development of HiCM-188 for heart failure. The candidate secured NMPA IND clearance and completed the first NHC-filed clinical research project based on iPSC technology, having finished Phase I dose-escalation and Phase II dose-expansion studies.

OD LAB | Ophthalmic Disease Joint Research Laboratory

Established in collaboration with Jiangsu Province Hospital, this lab advances iPSC-derived retinal pigment epithelial (RPE) cells for age-related macular degeneration (AMD). The program was selected for the 2022 Jiangsu Key Research and Development Program (Social Development Special Project) and the Nanjing Life and Health Science and Technology Special Program, aiming to address critical unmet needs in AMD.

MR LAB | Musculoskeletal Regeneration Laboratory

Utilizing robust and efficient cell manufacturing platforms, the lab optimizes isolation protocols and culture parameters to preserve cell viability, yield, and functionality, meeting clinical requirements for bone and joint disorders.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – HemaCell

Company Profile

HemaCell is a stem cell biotechnology company founded by overseas experts, dedicated to developing cell therapies through targeted hematopoietic regeneration. The company has proprietary technologies in stem cell reprogramming, editing, and differentiation, with in vitro platelet regeneration from megakaryocytes as its core development focus. By advancing cell therapies based on adult stem cells and induced pluripotent stem cells (iPSCs), HemaCell aims to address unmet needs in platelet supply for cancer, liver disease, acute and critical illnesses, and hematological disorders, while developing innovative therapies for platelet abnormalities and cancer-related diseases.

血霁生物 HemaCell

Development History

Since its establishment, HemaCell has attracted significant investor attention through its clear positioning, differentiated technology, stable operations, and experienced team. The company completed three financing rounds in its first year and subsequently secured multiple rounds of investment, including Series A+ and Series B financing, from institutions such as Sainuo Investment, Oriza Holdings, Shenzhen Capital Group, and CDH Investments. Meanwhile, HemaCell has achieved continuous progress in technology development, international clinical trial applications, and qualification approvals. In 2026, the company completed the second closing of its Series B financing, bringing the total Series B funding to nearly RMB300 million.

Company Established — **June 2021:** HemaCell Biotechnology Co., Ltd. was officially established in Suzhou, becoming one of the few companies globally and the first in China focusing on in vitro platelet regeneration from megakaryocytes.

Angel Financing — **October 2021:** Secured tens of millions of RMB in investments from Wedo Capital, Oriza Holdings, and Zhongli Fund.

Pre-Series A Financing — **2022:** Completed RMB 100 million Pre-Series A financing and subsequently secured Pre-Series A+ financing in the same year.

Series A Financing — **2023:** Completed Series A1 financing from Zhongbo Juli Investment, Guoxin Innovation, and other investors, and obtained tens of millions of RMB in debt financing to support IND applications and preclinical research for platelet-related pipelines.

Clinical Milestone — **2025:** In August 2025, HemaCell submitted XJ-MK-001 for FDA IND review; in November 2025, the Phase I clinical trial of XJ-MK-002 was first publicly disclosed; in December 2025, the company completed over RMB 100 million Series B1 equity financing, led by Hainan Health Medical Group with participation from Yonghui Venture Capital.

Series B1 Financing — **2026:** Received over RMB 100 million in investment and completed a nearly RMB 300 million Series B financing round, with investments from new shareholders including Shenzhen Capital Group and Hebei Industrial Investment, as well as follow-on investments from existing shareholders such as CDH Investments and Wedo Capital.

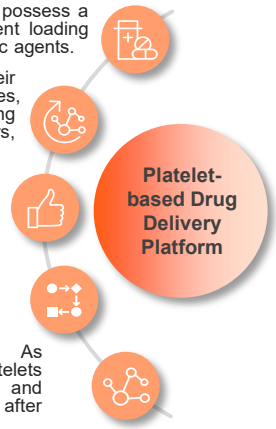
Core Technology Platforms

Leveraging three core technologies—the in vitro hematopoietic lineage cell regeneration platform, iPSC establishment and gene editing platform, and platelet-based drug delivery technology—HemaCell enables efficient in vitro production of functional cells such as platelets through biomimetic reconstruction of hematopoiesis and precise gene editing. By harnessing the natural targeting capability and high biocompatibility of platelets, the company further develops innovative drug delivery systems for major diseases, including cancer.

Core Technology

1. **In Vitro Hematopoietic Cell Generation Platform**
   Recapitulating human hematopoiesis, the platform enables the directed differentiation and scalable in vitro production of functional blood cells, including platelets, megakaryocytes, erythrocytes, and immune cells, from stem cells.
2. **iPSC Establishment and Gene Editing Platform**
   iPSC reprogramming: Utilizes non-viral and non-integrating approaches to reprogram somatic cells into iPSCs, minimizing genomic integration risks and improving safety. Serum-free iPSC culture: Employs proprietary serum-free culture media to support stable passaging, high viability, and reduced differentiation bias. Precision gene editing: Incorporates CRISPR-Cas9-based editing technologies for reducing immunogenicity, disease modeling, and functional gene regulation.
3. **Platelet-based Drug Delivery Technology**
   Leveraging platelets' natural targeting ability, long circulation half-life, and low immunogenicity, the platform develops platelet-based drug delivery systems for the treatment of major diseases, including cancer and autoimmune disorders.

- **High Loading Capacity:** Platelets possess a large surface area, enabling efficient loading of substantial amounts of therapeutic agents.
- **Targeting Capability:** Beyond their natural accumulation at bleeding sites, platelets exhibit preferential targeting toward inflammatory sites, tumors, and plaques.
- **Good safety:** As anucleate cells, platelets may reduce certain safety risks associated with nuclear genomic material.
- **High Drug Delivery Efficiency:** Platelets circulate directly in the bloodstream, minimizing drug loss during delivery.
- **Good Biocompatibility:** As endogenous cellular carriers, platelets are less likely to be recognized and cleared by the immune system after administration.



Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – HemaCell

■ R&D Pipeline

HemaCell is the first company in China and the third company globally, following Japan and the United States, to achieve in vitro platelet regeneration. The company's leading pipeline focuses on in vitro platelet regeneration, aiming to deliver a "second transfusion revolution" in China without reliance on blood donation. Leveraging its expertise in iPSC reprogramming, gene editing, and differentiation technologies, HemaCell has successfully filed patents covering iPSC establishment and the differentiation of stem cells into hematopoietic lineage cells, including NK cells and macrophages, providing broad development potential for future expansion.

血霁生物 HemaCell

Drug Code	Indication / Application	Preclinical	IND	Phase Ⅰ	Phase Ⅱ	Phase Ⅲ	Marketed	Region
Platelet-enhancing drugs								
XJ-MK-002	Chemotherapy-induced thrombocytopenia				CDE IND Clearance (Aug 28, 2025)			China
	Thrombocytopenia caused by aplastic anemia				CDE IND Clearance (Mar 3, 2026)			China
	Thrombocytopenia				FDA IND Clearance (Mar 13, 2026)			U.S.
XJ-MK-001	Chemotherapy-induced thrombocytopenia				FDA IND Clearance (Aug 22, 2025)			U.S.
PLT-001	Unpublished							China
Aging-related pipeline								
cPLT-001	Unpublished							U.S.
cPLT-002	Unpublished							China & U.S.
Platelet-derived products								
Platelet drug delivery platform	Applications: drug delivery for oncology and autoimmune diseases							NA

Drug Code	Rare Disease Category	Number	Region
Rare Disease Pipeline (Approved)			
XJ-MK-002	ODD	3	U.S.
	RPDD	2	U.S.
XJ-PLT-001	ODD	2	U.S.
XJ-PLT-002	ODD	1	U.S.

■ Core Advantages

Focusing on the critical unmet need for platelets, HemaCell leverages a world-class academic founding team, comprehensive intellectual property portfolio, and sustained multi-round financing from leading institutions, including Sequoia China, CDH Investments, and Shenzhen Capital Group, demonstrating strong advantages in both technological innovation and commercialization capabilities.

Clinical Need Alignment

Focused on the critical unmet need for platelet shortages, HemaCell addresses global annual demand exceeding one million units, aiming to reduce reliance on blood donation and improve supply stability, with significant market potential.

Capital & Team Advantages

Composed of experts from leading institutions including Stanford University, Harvard University, and Peking University, with extensive international research experience and commercialization capabilities.

Financing Capability

Completed multiple financing rounds within several years, attracting investments from leading institutions including Sequoia China, CDH Investments, Luxin Venture Capital, Northern Light Venture Capital, and others. The latest round (June 2026) secured investments from Shenzhen Capital Group and Hebei Industrial Investment Fund, demonstrating strong capital recognition.

Intellectual Property Portfolio

Obtained 71 registered trademar and 5 granted patents, establishi a comprehensive intellectual property portfolio.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Huode

Company Profile

Zhejiang Huode Bioengineering Co., Ltd., founded in January 2017, is a global innovative biotechnology company focused on the development of iPSC-derived off-the-shelf allogeneic cell therapy products. The company operates cell therapy R&D centers, GMP manufacturing facilities, quality control centers, and office sites in Hangzhou and Shanghai, with a total area of approximately 6,400 m². Huode has established globally leading patented technologies and innovative platforms in iPSC reprogramming, neural differentiation of pluripotent stem cells, and cell engineering, and has developed an iPSC product CMC platform, closed automated manufacturing processes, and multiple innovative analytical methods. The company has established GMP iPSC cell banks for commercial authorization in China and overseas, with multiple products under development. Among them, hNPC01, an iPSC-derived neural progenitor cell injection, was the first domestically developed product in its category to obtain China-U.S. IND approval, and has completed Phase I clinical trials with FDA Fast Track designation and RMAT recognition. It is currently one of the fastest-developing iPSC cell therapy products globally, targeting major unmet clinical needs including stroke, traumatic brain injury sequelae, cerebral palsy, and epilepsy.

Development History

2017
- Huode was established.
- Completed construction and operation of Hangzhou A+B+C-grade cell manufacturing and R&D facility.

2018
- Developed initial products and second-generation differentiation technologies.
- Submitted two core patents covering functional cerebral cortex cell induction differentiation and 3D brain organoid manufacturing methods.

2020
- Core patents entered the U.S., European, and Japanese markets through PCT applications.
- Established FDA-compliant GMP iPSC cell banks.

2021
- Completed hNPC01 drug research.
- Partnered with Neurophth Therapeutics for iPSC cell line and patent licensing collaboration.
- Completed GMP production facility construction at Hangzhou Medical Port.
- Received support from the National "14th Five-Year Plan" National Key R&D Program on Stem Cells and Organoids for hNPC01 clinical and mechanism studies.

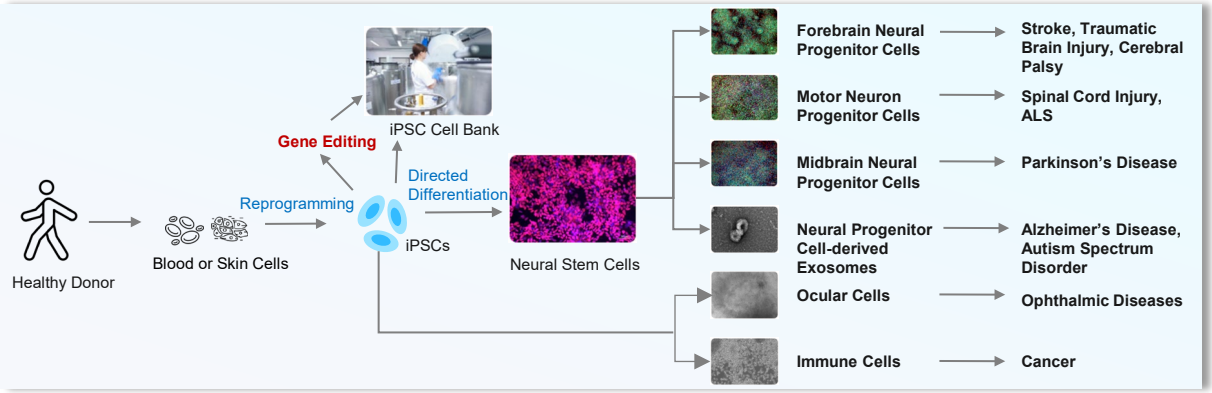
2023
- Received implied IND clearance in China for hNPC01 in chronic motor dysfunction after ischemic stroke and completed first participant dosing in the Phase I clinical trial.
- Obtained U.S. FDA IND clearance for hNPC01. Demonstrated positive results in the Phase I clinical trial of hNPC01 for chronic motor dysfunction after ischemic stroke in China.
- Granted two core patents in Europe and the United States.

2024
- hNPC01 for chronic motor dysfunction after ischemic stroke completed 12-month follow-up in both the Phase I clinical trial and the National Major Project study, entering long-term follow-up with sustained functional improvements.
- Received FDA Fast Track designation for hNPC01 injection.
- Established Shanghai Huode in Shanghai Lingang Special Area to expand human stem cell R&D services.

2025
- hNPC01 for motor dysfunction after ischemic stroke sequelae received FDA agreement to enter Phase II/III adaptive confirmatory clinical trials and obtained FDA Regenerative Medicine Advanced Therapy (RMAT) designation.
- hNPC01 for motor dysfunction after hemorrhagic stroke sequelae and traumatic brain injury obtained U.S. FDA IND approval.

2026

Core Technologies and Platforms



iPSC Reprogramming, Gene Editing & Multi-lineage Cell Differentiation Platforms

The company has established a GMP- and FDA-compliant iPSC seed cell bank potentially supporting up to five billion treatment doses. Leveraging its proprietary RONA2.0 neural differentiation technology, the company enables scalable manufacturing of high-quality clinical-grade hNPCs, with optimized processes and quality control systems supporting stable production of 600 doses per batch and reducing manufacturing costs to several thousand RMB per dose. FOXG1-positive neural progenitor cells derived from pluripotent stem cells demonstrate advantages in high yield, purity, and stability. These cells can differentiate into mature functional neural cells in vivo, while enabling observation of progressive integration into neural networks in animal models.

GMP Manufacturing Center

Huode has established an R&D center in Shanghai and a GMP manufacturing center of over 6,000 m² in Hangzhou, forming a global operational network integrating technology development, clinical translation, and international collaboration. Its R&D and manufacturing facilities include three independent cell therapy production lines, a 2,000 m² GMP manufacturing suite, and a 4,200 m² production and R&D headquarters facility.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Huode

R&D Pipeline

Huode has established multiple development pipelines centered around its core product, hNPC01 (human forebrain neural progenitor cells). The most advanced program targets ischemic stroke, which has received IND clearance in both China and the United States. The program has completed a Phase I clinical trial in China, obtained FDA Fast Track designation, and received FDA agreement to directly advance into Phase II/III confirmatory clinical trials. In June 2026, hNPC01 was granted Regenerative Medicine Advanced Therapy designation by the FDA. In June 2026, IND applications for hNPC01 targeting motor dysfunction after hemorrhagic stroke and motor dysfunction after traumatic brain injury were cleared by the FDA. Additional indications, including cerebral palsy and epilepsy, are currently under preclinical development.

霍德生物 HOPSTEM BIOTECH

Candidate Drug	Indication	R&D	CMC	Preclinical	IND	Phase Ⅰ	Phase Ⅱ & Ⅲ
Human Forebrain Neural Progenitor Cells (hNPC01)	Ischemic Stroke						
	Hemorrhagic Stroke						
	Traumatic Brain Injury						
	Cerebral Palsy						
	Epilepsy						
Neural Progenitor Cell-derived Exosomes (hNPCEX)	Autism Spectrum Disorder						
	Alzheimer's Disease						

Core product

Huode is developing hNPC01, the first off-the-shelf cell therapy product based on human forebrain neural progenitor cells, for cell replacement therapy in stroke and traumatic brain injury (TBI). Generated from GMP-manufactured iPSCs using Huode's second-generation neural differentiation technology, hNPC01 is a clinical-grade human forebrain neural progenitor cell product with >90% FOXG1-positive cells. It demonstrates long-term survival in vitro and after intracranial transplantation in rodent and non-human primate pMCAO models, and can differentiate into various functional neurons and glial cells with cellular composition and maturation characteristics comparable to the human cerebral cortex. Preclinical studies showed that neurons differentiated from transplanted human neural progenitor cells can form synaptic connections with host neurons, exhibit electrophysiological activity, and integrate into neural circuits. The cells may also release neurotrophic factors and other beneficial factors to support local neural function and connectivity. Compared with control groups, non-human primate stroke models receiving cell transplantation demonstrated significant functional improvement. In the Phase I clinical study of hNPC01 for chronic motor dysfunction after ischemic stroke in China, the longest ***follow-up has reached 2.5 years***. No product-related adverse events other than immune rejection were observed, and no functional deterioration compared with baseline was reported.

- At the ***12-month endpoint***, the target subgroup achieved a mean improvement of ***16 points*** on the Fugl-Meyer Motor Scale (FMMS), with nearly ***80%*** of patients achieving clinically meaningful improvement (≥***10-point*** FMMS improvement).
- At ***18 months***, more than ***92%*** of patients in the target subgroup achieved clinically meaningful improvement, and ***54%*** achieved a one-point improvement in the modified Rankin Scale (mRS) score.
- The ***2-year*** follow-up demonstrated that therapeutic effects reached a stable plateau, with no observed decline in efficacy.

Following ***FDA Fast Track designation*** and ***Regenerative Medicine Advanced Therapy (RMAT) designation***, the successful FDA clearance of the IND for the TBI indication makes hNPC01 ***the first forebrain neural progenitor cell product worldwide cleared by the FDA for clinical development across three major neurological injury indications: ischemic stroke, hemorrhagic stroke, and traumatic brain injury.***

Core advantages

IP Portfolio

Huode has established a leading global patent portfolio covering iPSC reprogramming, multi-lineage neural differentiation, and cell engineering technologies, with approximately 50 patents worldwide. Key patents, including "Methods for Preparing 3D Brain Organoids" and "Methods for Inducing the Differentiation of Functional Cortical Cells," have been granted in Europe, the United States, and other jurisdictions.

Manufacturing Capability

Huode operates R&D and manufacturing facilities exceeding 6,000 m². Its GMP manufacturing center includes three fully independent cell production suites with segregated cleanroom and isolator configurations, supporting the independent manufacturing and testing of three different cell products.

Team Expertise

The Huode team comprises experienced researchers from leading domestic and international universities, PhDs in cell biology, and senior executives from multinational pharmaceutical companies. The team covers expertise across process development, manufacturing, quality, regulatory affairs, commercialization, and clinical development, with members possessing 10–25 years of industry experience.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – iRegene Therapeutics

Company Profile

Founded in 2017, iRegene Therapeutics is a clinical-stage biotech company specializing in the development of iPSC-derived cell therapies using small-molecule-mediated chemical induction. The company's lead candidates primarily target Parkinson's disease (PD), multiple system atrophy (MSA-P), and ophthalmic disorders.

As the world's pioneer in leveraging an AI-driven chemical induction platform for cell therapy discovery, iRegene Therapeutics was founded by scientists returning from the University of Cambridge and the University of Edinburgh. Since inception, the company has accumulated numerous granted Chinese invention patents and PCT international patent filings, while undertaking multiple national key R&D projects. Between June and July 2026, iRegene Therapeutics successfully completed Series C and Series C+ financing rounds totaling over RMB 500 million.

Development History

2017
iRegene Therapeutics was founded.

August 2023
Secured NMPA IND clearance for NouvNeu001 injection to initiate clinical trials.

December 2023
Obtained NMPA IND approval for NouvNeu003 injection to launch Phase I clinical trials targeting early-onset PD.

March 2024
Breakthrough ophthalmic candidate NouvSight001 granted Orphan Drug Designation (ODD) by the U.S. FDA.

June 2024
Received FDA IND clearance for NouvNeu001 to conduct overseas clinical trials.

April 2025
Officially launched Phase II clinical trials for NouvNeu001 in China.

August 2025
NouvNeu001 granted Fast Track Designation (FTD) by the U.S. FDA, alongside clearance for expanded clinical indications.

September 2025
Completed a Series B financing round exceeding RMB 300 million amidst market headwinds.

December 2025
NouvNeu001 awarded Regenerative Medicine Advanced Therapy (RMAT) designation by the U.S. FDA.

February–March 2026
Completed first-participant dosing in U.S. Phase II clinical trials for NouvNeu001 in February, followed by full patient dosing completion across China Phase II trials in March.

June–July 2026
Completed Series C and Series C+ financing rounds totaling over RMB 500 million.

Core Technologies

iRegene Therapeutics commands a next-generation R&D ecosystem backed by proprietary intellectual property, centered on its distinct "AI + Chemical Induction" platform. This platform comprises five core technical modules: iReMeta (AI & Big Data-driven Metadata Integration Platform for Model Organisms), iReChem (Developmental Biology-focused Compound Discovery Platform), iReDita (Highly Efficient Genome Editing Platform), iReXam (Compound validation Platform), and iReCena (Chemically Induced Cell-derived Extracellular Vesicles Platform for Complex Drug Development), constructing a fully integrated R&D system spanning target discovery to drug delivery. The platform operates through a three-step core workflow: first, leveraging the proprietary computational biology platform iReMeta to mine core driver genes governing cellular transformation; second, utilizing AI high-throughput screening to identify precise small-molecule compound combinations that induce lineage-specific cell differentiation; and finally, executing precise reprogramming of cell fate and function via purely chemical induction.

The safety and efficacy of NouvNeu001, an allogeneic iPSC-derived cell therapy developed using this platform, have been preliminarily demonstrated. Compared with alternative cell therapies, chemically induced off-the-shelf cell products deliver lower manufacturing costs, superior patient accessibility, and higher commercial value.

iReMeta
AI Multi-Omics Data Mining
Integrating proprietary and public multi-omics datasets to perform advanced data mining and predictive analytics

iReDita
Gene Editing & Reporter Cell Lines
Utilizing CRISPR, HR, and TALEN technologies for precise genomic editing to construct stable reporter cell lines

iReChem
Deep Learning-Assisted Virtual Screening
Predicting drug activity leveraging structural biology and proprietary compound databases

iReXam
Functional Compound Validation
Providing phenotype-driven, real-time multi-omics validation data

iReCena
Drug Delivery-Oriented Extracellular Vesicle Engineering
Constructing engineered extracellular vesicles through modular assembly to serve as next-generation drug delivery systems

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – iRegene Therapeutics

Core Pipeline

iRegene Therapeutics strategically targets optimal directions in regenerative medicine, leveraging its expertise in developmental biology to focus on degenerative diseases lacking effective therapeutic interventions. Its current development portfolio primarily centers on central nervous system (CNS) and ophthalmic disorders. The lead candidate, NouvNeu001 (targeting PD), has secured dual IND approvals from both the U.S. FDA and China's NMPA, alongside dual FDA FTD and RMAT designation. Key pipeline candidates—including NouvNeu003 for early-onset Parkinson's disease, NouvNeu004 for MSA-P, and NouvSight001 for ophthalmic indications—likewise maintain industry-leading development positions.

Pipeline	Indication	IND	Phase Ⅰ	Phase Ⅱ	Phase Ⅲ
NouvNeu001	Parkinson's Disease	• First dual NMPA/FDA IND-approved iPSC therapy for PD, granted world-first FDA Special Exemption • Completed China Phase II dosing; dosed first U.S. Phase II cohort, holding S.E., FTD, and RMAT status			
NouvNeu003	Early-onset Parkinson's Disease	• Completed China Phase I registered trial.			
NouvNeu004	MSA-P	• World's first cell therapy for Multiple System Atrophy with dual NMPA/FDA IND approvals. • NMPA approval to conduct Phase I–III clinical trials; granted S.E. designation by the FDA; first participants dosed in the Phase I clinical trial in China.			
NouvSight001	Retinal Degenerative Diseases	• Granted U.S. FDA ODD; China IND clearance obtained; U.S. IND filing planned for Q3 2026.			
NouvNeu002	Ischemic Stroke	• Dual China/U.S. IND filings planned for H2 2026.			

Latest Financing Progress

In Q2 2026, iRegene Therapeutics completed Series C and C+ financing rounds exceeding RMB 500 million. Securing two consecutive rounds in short order reflects sustained investor confidence, marking a key transition from "platform validation" to "clinical value realization" and "accelerated capitalization." The Series C was co-led by Luminous Ventures and Apricot Capital; Series C+ was led by Chengdu Science and Technology Venture Capital Group, joined by China Capital Management, Wuxi Capital Group, Dingtai Xing, Xinjiye Investment Holdings, and Hony Capital, with follow-on investments from existing shareholders including Yuanxi Haihe and Jinshajiang Lianhe Runpu Yuanfeng Venture Capital Partnership. Previous backers include Fortune Capital, Ceyuan Ventures, Northern Light Venture Capital, Vertex Ventures, and Tigermed.

This syndicate unites local state-owned capital, securities firms, and industrial partners. Proceeds will fund global clinical advancement of core pipelines, "AI + Chemical Induction" platform iteration, cGMP industrial-scale manufacturing expansion, multi-regional clinical trials, and global commercial partnerships. Financial backers strengthen the company's capital market connectivity and potential IPO pathways, while strategic industry investors reinforce confidence in its pipeline value.

Core Advantages

As the world's pioneer in end-to-end, "AI + Chemical Induction" cell therapy R&D platforms, iRegene Therapeutics was founded by scientists returning from top-tier institutions such as Cambridge and Edinburgh. The company has established a comprehensive proprietary IP portfolio spanning compounds, culture media, and bioprocesses, alongside a GMP manufacturing facility and a 10,000+ m² R&D base—commanding full-stack capabilities from early discovery to low-cost, industrial-scale allogeneic cell manufacturing.

Technological Edge

iRegene Therapeutics pioneers the world's first "AI + Chemical Induction" end-to-end cell therapy platform, reshaping traditional cell therapy paradigms. Bypassing gene editing, this platform achieves highly efficient, low-cost, and large-scale manufacturing of functionalized, off-the-shelf cell therapeutics simply through precise combinations of small-molecule compounds and basal media.

Leadership Team

Founded by scientists returning from the University of Cambridge, the University of Edinburgh, and the Roslin Institute, iRegene Therapeutics has assembled an international team. Executive leadership brings over 15 years of domestic and international expertise spanning biomedical innovation, translational and clinical medicine, global regulatory affairs, Big Pharma management, and capital operations.

Intellectual Property Protection

iRegene Therapeutics maintains a robust IP moat covering novel-structure induction compounds, proprietary basal media, bioprocesses, and quality systems, anchored by its proprietary chemical induction platform and global patent estate.

Manufacturing & Logistics Capabilities

iRegene Therapeutics operates a GMP manufacturing facility with Grade A clean zones in a Grade B background within a 10,000+ m² industrial manufacturing and R&D complex, supporting allogeneic cell therapy supply for global commercial markets alongside cold-chain logistics capabilities serving China, the U.S., and Australia.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Nuwacell

Company Profile

Nuwacell Biotech Co., Ltd. was founded in 2016 and is dedicated to developing widely accessible iPSC-derived cell therapy products. The company has established a product and service system covering the entire value chain, including upstream iPSC technologies (clinical-grade culture reagents), midstream (one-stop iPSC-derived product CDMO services), and downstream (iPSC-derived cell therapy products). It operates internationally leading, high-standard advanced R&D and manufacturing facilities for research-grade and clinical-grade stem cells. The company has also established a systematic patent portfolio of invention patents centered around iPSC technology applications, covering areas such as iPSC reprogramming, directed differentiation of multiple functional cells, production processes, and key raw materials.

Development History

1 2016-2020

- 2016: Nuwacell was founded
- GMP cell laboratory officially put into operation
- The company undertook a National Key R&D Program of the Ministry of Science and Technology for the first time
- Accelerated the initiation of R&D for various cell drug pipelines, with CMC development commencing for each pipeline

2 2021-2024

- Undertook two additional National Key R&D Programs of the Ministry of Science and Technology
- Participated in the formulation of more than 10 national/group standards for cell products
- Six registered pipelines entered IND application and registrational clinical trials

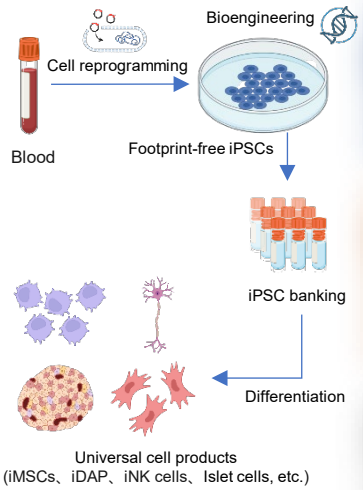
3 2025-2026

- Multiple pipelines of the company entered the pivotal clinical trial stage

Core Platform

Nuwacell's iPSC technology platform was established under the leadership of Dr. Junying Yu, one of the global pioneers of hiPSC technology. The core of the iPSC technology platform is to "reprogram" somatic cells (such as blood cells) into stem cells with multi-lineage differentiation potential, which can then differentiate into almost any type of human functional cell.

Based on its mature iPSC technology platform, Nuwacell directs the differentiation of iPSCs into target cells with specific therapeutic functions and applies them in three major medical fields: anti-inflammatory applications and tissue repair, regenerative medicine, and immunotherapy. Empowered by iPSC technology, the company is committed to achieving "off-the-shelf" and scalable supply of cell therapy products, fundamentally addressing the industrialisation challenges of traditional cell therapies, such as limited cell sources, large batch-to-batch variation and high costs.



Anti-inflammation and Tissue Repair

- MSCs: roles in injury repair, immune regulation, promotion of angiogenesis, anti-apoptosis, anti-oxidation, etc.;
- Applicable to the treatment of various diseases including immune diseases, pulmonary diseases and tissue injury diseases;
- iPSC-derived MSC cell drugs offer advantages such as large batch yield, uniform quality, stable efficacy and ease of genetic modification.

Regenerative Medicine

- For Parkinson's disease: implantation of healthy dopaminergic neural progenitor cells into the brain to replace or compensate for necrotic dopaminergic neurons may treat or even cure Parkinson's disease;
- For diabetes: islet cell replacement therapy is expected to become the most promising treatment method to "end" insulin injection;
- Through innovative iPSC technology, Nuwacell can obtain low-immunogenicity, universal and highly efficient iPSC-derived iDAP and islet cell preparations.

Immunotherapy

- Natural killer (NK) cells can exert direct (release of lytic granules, expression of apoptosis-inducing ligands, ADCC effect) or indirect (secretion of various cytokines, chemokines and growth factors to activate other immune cells) tumor-killing effects, with potential for treating various hematological malignancies and solid tumors;
- iPSC-derived NK cell products offer advantages such as superior tumor-killing performance, tolerance to cryopreservation and recovery, and ease of multi-element genetic modification.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Nuwacell

R&D Pipeline

Nuwacell focuses on its core iPSC technology and has established pipelines across three major therapeutic areas: anti-inflammation and tissue repair, regenerative medicine, and immunotherapy. Multiple product pipelines based on three iPSC-derived functional cell types (iMSC, iDAP, iNK) have been advanced to the stage of registered clinical trials. Among them, NCR100 and NCR300 are the first iMSC and iNK cell products approved for clinical trials in China, and NCR101 is the world's first gene-modified iMSC cell product approved for clinical trials. The company will continue to explore the clinical value of iPSC-derived cell therapy products.

Medical field	Products	Indication	IND-enabling studies	Phase I	Phase II	Phase III
Anti-inflammation and Tissue Repair	iMSC	Knee Osteoarthritis				
	iMSC	Steroid-refractory aGvHD				
	iMSC*plus*	Interstitial Lung Disease				
Regenerative Medicine	Universal iDAP	Parkinson's Disease				
	Universal Islet	Diabetes Mellitus				
Immunotherapy	iNK	Myelodysplastic Syndrome				
	iNK	Prevention of AML Relapse after allo-HSCT				

Core Advantages

Nuwacell is led by Dr. Junying Yu, one of the global pioneers of hiPSC technology. Since its establishment, it has grown into the company with the largest number of pipelines that have entered the clinical stage in China's iPSC sector. The company has successively been awarded the national-level Specialized, Sophisticated, Distinctive and Innovative "Little Giant" enterprise and selected for the first group of key cultivation pilot platforms by the Ministry of Industry and Information Technology, among other national-level honours. Relying on its proprietary iPSC technology, it has built industry-leading technical barriers. Through the dual-drive strategy of continuous innovation and industrialisation, Nuwacell has established a fully integrated in-house value chain covering upstream (clinical-grade culture reagents), midstream (one-stop iPSC-derived product CDMO services) and downstream (iPSC-derived cell therapy products).

As the only iPSC company in the world with pipeline presence in all four directions of iMSC, iDAP, islet and iNK, the company has successfully advanced 6 pipelines into the registered clinical trial stage. With comprehensive layouts in the three major fields of anti-inflammation and tissue repair, regenerative medicine and immunotherapy, it has advanced the world's first gene-modified iMSC product cleared for clinical trials, NCR101, as well as China's first iMSC and iNK products cleared for clinical trials, NCR100 and NCR300, respectively, establishing Nuwacell as a leading and first-mover in the field of off-the-shelf universal cell products.

Led by a Global iPSC Pioneer Team

Led by Dr. Junying Yu, one of the global pioneers of hiPSC technology, it is among the first batch of enterprises in China to carry out R&D of iPSC-derived cell therapy products, with industry-leading core R&D strength.

Global iPSC Core Patent Barriers Established

The company fully and independently masters the IP of iPSC reprogramming and directed differentiation, and has completed a comprehensive patent portfolio for functional cell differentiation in China, multiple overseas jurisdictions and through Patent Cooperation Treaty filings, forming a solid intellectual property protection system.

Diversified Product Pipeline

The pipeline is diversified and rich, with comprehensive layouts completed around multiple directions of iPSC-derived iMSC, iDAP, islet and iNK, covering the three major fields of anti-inflammation and tissue repair, regenerative medicine and immunotherapy. Currently, 6 pipelines have entered the registered clinical trial stage.

Fully Integrated In-house Value Chain

Established a fully integrated in-house value chain covering raw material reagents, one-stop iPSC-derived product CDMO services and iPSC-derived cell therapy products.

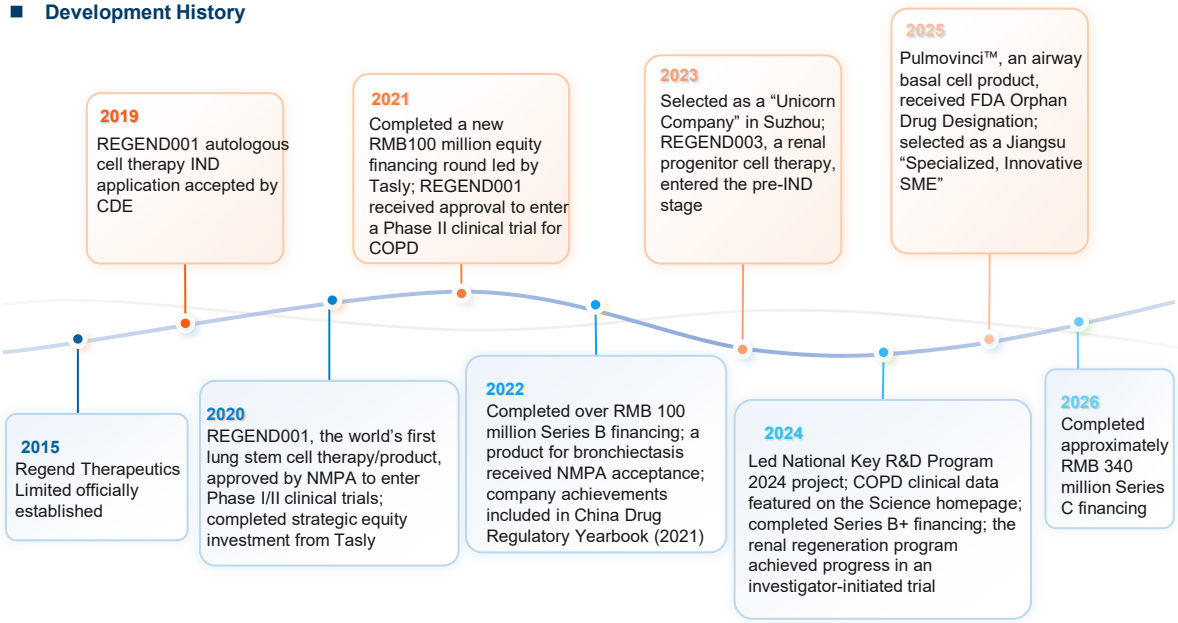
Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Regend

Company Profile

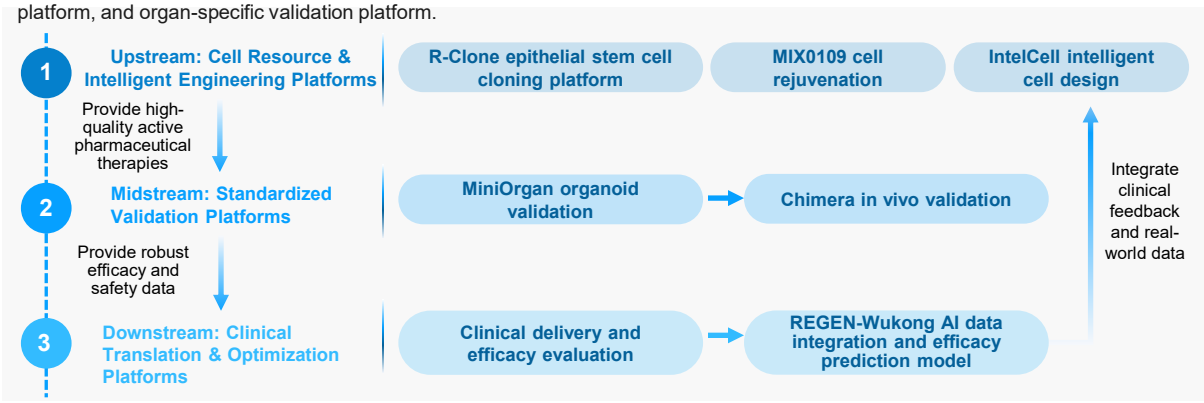
Regend Therapeutics Limited was established in 2015 as a biotechnology company transitioning from clinical-stage development toward early commercialization. The company is dedicated to the discovery, development, and commercialization of cell therapy products, aiming to repair, regenerate, and enhance the structure and function of human tissues and organs. Through innovative regenerative medicine solutions, Regend seeks to address irreversible organ damage, extend healthy lifespan, and improve patients' quality of life.

Development History



Core Technology Platforms

Led by Regend, the National Key R&D Program "Stem Cell Research and Organ Repair" project has established a comprehensive regenerative medicine technology platform, covering the full development cycle from cell isolation and expansion, functional enhancement, and intelligent design to in vitro/in vivo validation, targeted delivery, and AI-driven efficacy prediction and clinical feedback optimization. With strong scalability, this integrated platform can be rapidly extended to therapeutic applications across multiple organ systems, including the lung, kidney, brain, liver, uterus, pancreas, stomach, intestine, and skin. Regend has established multiple proprietary technology platforms, including the R-Clone epithelial stem cell cloning platform, IntelCell intelligent cell platform, MIX0109 tissue and cell regeneration technology, Chimera chimeric animal model platform, REGEN-Wukong AI platform, and organ-specific validation platform.



Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Regend

R&D Pipeline

Leveraging multiple proprietary technology platforms, Regend has established a globally innovative regenerative medicine pipeline. REGEND001 is an innovative autologous lung progenitor cell therapy. In 2025, the company completed Phase II clinical trials for chronic obstructive pulmonary disease (COPD) and idiopathic pulmonary fibrosis (IPF). Phase I/II clinical data demonstrated its potential to reverse disease progression in COPD and IPF patients, with evidence of airway and alveolar regeneration and multidimensional improvements in pulmonary function. Data from the first lung regeneration clinical trial were published in Science Translational Medicine and featured on the Science homepage. Regend's lung progenitor cell therapy was highlighted by Nature Reviews Drug Discovery as a promising drug development approach for COPD. REGEND001 has received FDA Orphan Drug Designation, supporting its future global development. In May 2025, REGEND003, a renal progenitor cell therapy for chronic kidney disease (CKD), received IND approval from China's National Medical Products Administration (NMPA) and subsequently entered Phase I clinical trials. The candidate therapy aims to regenerate nephrons and provide a potentially disease-modifying regenerative treatment for kidney failure. In March 2026, the first participant enrolled in the Phase I trial successfully completed autologous renal progenitor cell intrarenal transplantation, marking a milestone in regenerative medicine.

Product	Product Type	Indication	Preclinical	Researcher-Initiated Trial	IND	Phase I	Phase II	Phase III
REGEND001 (Aerioautotemcel)	Autologous lung progenitor cells (airway basal cells)	COPD (emphysema phenotype)					Phase II Completed	
		Idiopathic pulmonary fibrosis (IPF)					Phase II Completed	
						FDA ODD Granted		
		Interstitial lung disease			IIT Completed			
		Bronchiectasis			IIT Completed			
		Obliterative bronchiolitis						
REGEND002	Autologous lung progenitor cells(rejuvenated)	COPD, interstitial lung disease, bronchiectasis, etc.						
REGEND007	Universal lung progenitor cells	COPD, interstitial lung disease, etc						
REGEND003	Autologous renal progenitor cells	Chronic kidney disease (CKD)						
REGEND008	Universal renal progenitor cells	Chronic kidney disease (CKD)						
REJUVE101	Engineered stem cell exosomes	Age-related multi-organ diseases						
REJUVE109	Epigenetic reprogramming agents	Age-related multi-organ diseases (including cancer)						

R&D Capacity

GMP Manufacturing System — **Shanghai Super Organ R&D Center**

Current	Facility Area ***1,000 m²***	Annual Production Capacity ***90 billion cells***	Capacity Utilization (2025) ***28.2%***
Future	Facility Area ***10,000 m²***	Annual Production Capacity ***270 billion cells***	

Quality Management — A comprehensive Quality Management System (QMS) covering the entire process lifecycle enables precise quality control throughout manufacturing.

Automation — Based on the R-Clone epithelial stem cell cloning platform, Regend has co-developed an intelligent automated cell manufacturing platform integrating automation, intelligent systems, digitalization, artificial intelligence, and image processing technologies.

Safety & Reliability | **Quality Control** | **Efficiency & Intelligence**

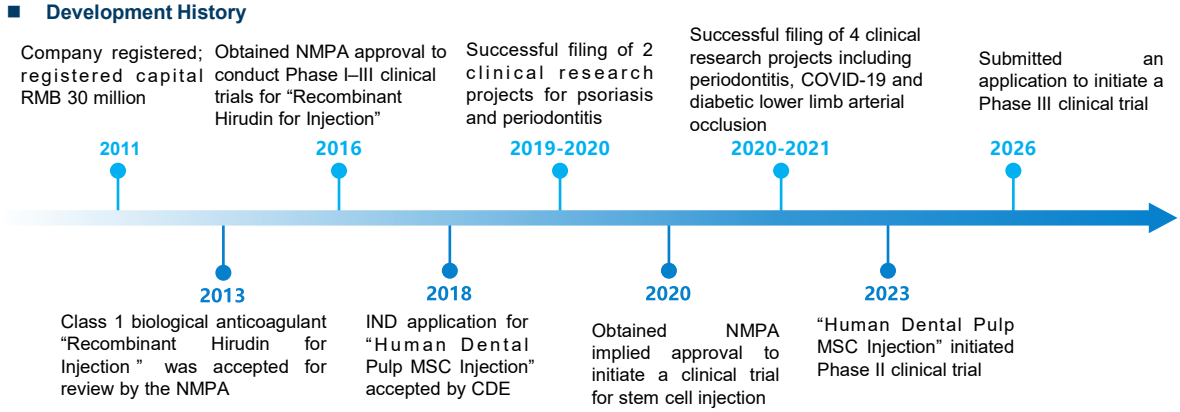
Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Sanyouli Heze

Company Profile

Beijing Sanyouli Heze Biological Technology Co., Ltd. (SHbio) was founded in 2011, relying on the academic teams and research platforms of Academicians Wu Zuze and Wang Songling. It is a benchmark enterprise in periodontal regenerative stem cells in China. Centred on cell and gene therapy (CGT), the company focuses on innovative drugs for chronic diseases. Leveraging China's first dental resource bank, it covers dental-source cell storage, GMP manufacturing, CDMO services and gene-modified stem cell patent technologies, and has built a complete technology chain of "dental pulp MSCs – gene-modified MSCs – engineered extracellular vesicles", forming a comprehensive CGT R&D pipeline. The enterprise possesses integrated capabilities in process development, pilot-scale manufacturing, clinical filing and IND application. Core products include its self-developed Class 1 dental pulp stem cell new drug and recombinant hirudin under the National Major Project for New Drug Creation.

Development History



Core Business Portfolio

The company focuses on cell biotechnology and innovative new drugs. It holds core patents for HGF gene-modified MSCs, has established a standardized dental pulp biological resource bank, and operates a cell therapy CDMO platform covering cell preparation, storage and quality control. Innovative drugs include the Class 1 allogeneic dental pulp stem cell new drug, with multiple pipelines targeting various chronic diseases.

Innovative Drug R&D (Long-term)

- **Cell Therapy Pipeline:** Allogeneic human dental pulp MSC injection (Class 1 innovative drug candidate in China) with tissue regeneration and repair capability. Relying on its technology platform, it has laid out more than ten differentiated pipelines under development, covering high-incidence chronic diseases such as oral repair, skin immunity and pulmonary fibrosis, forming a complete chronic disease treatment product matrix.

Technical R&D Services (Mid-term)

- **Biological Sample Storage Service:** Established China's first standardized dental pulp biological resource storage bank, covering dental tissue sample collection, stem cell isolation and expansion, and cell cryopreservation, forming a complete closed loop of cell preparation – cryogenic storage – GMP quality control.
- **CDMO Platform:** Opens mature process platforms externally, providing one-stop cell therapy CDMO technical output services to empower partners in advancing cell drug R&D and translation.
- **Technical Barriers:** Independently masters the core patent technology of HGF gene-modified MSCs (EMSCs).

Core Advantages

World's First Allogeneic Dental Pulp Stem Cell Therapy

Originality: Developed the world's first allogeneic human dental pulp stem cell (hDP-MSCs) injection for periodontitis, breaking through the limitations of traditional periodontal treatment that "treats symptoms but not the root cause" and relies on surgery, achieving a leap in "tissue regeneration and functional reconstruction".

Significant Clinical Advantages

Minimally invasive local injection with no surgical incision; operable in dental clinics, requiring only 5–10 minutes per session; long-term promotion of alveolar bone regeneration, delaying tooth loss by 2–3 years, with better overall cost-effectiveness than traditional surgery. Cells are derived from healthy discarded teeth, with painless collection, no ethical risks and abundant resources.

Team Advantages

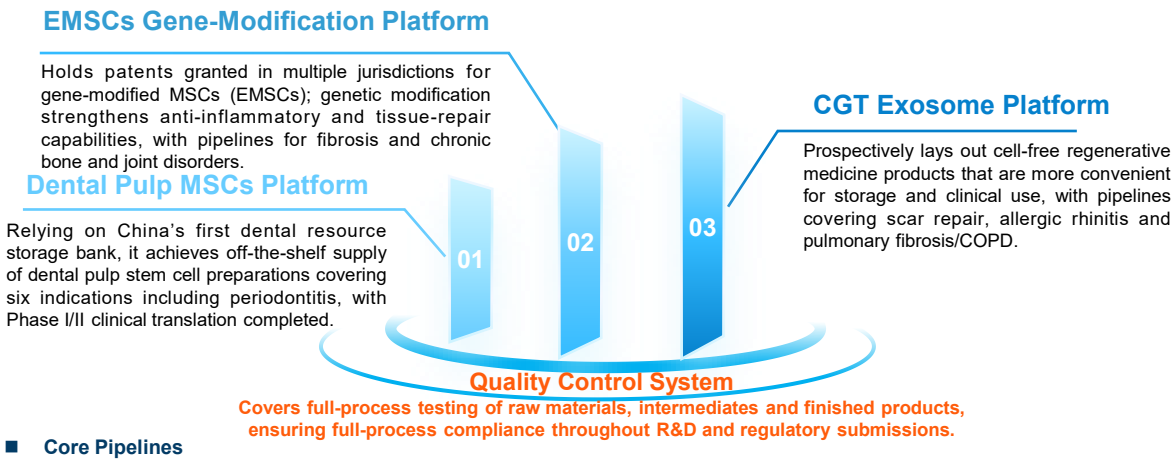
Backed by the research team led by two academicians, it has established China's first dental resource storage bank and GMP manufacturing and quality control platforms, connecting the full chain of R&D, manufacturing and clinical translation, with mature capabilities in rug development and clinical translation and technology output.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Sanyouli Heze

Technology Platforms

The company has built three core platforms—dental pulp MSCs, gene-modified EMSCs and CGT exosomes—supported by a GMP quality system, forming a complete chain from cells to engineered cells to cell-free exosomes. It provides end-to-end services covering process development, pilot manufacturing, clinical filing and IND application. China's first dental resource bank enables large-scale production of off-the-shelf dental pulp stem cell preparations for six indications, including periodontitis, with Phase I/II clinical progress completed. The patented EMSCs platform enhances anti-inflammatory and repair functions for fibrosis and chronic bone/joint diseases, while the CGT exosome platform develops convenient cell-free products for scar repair, allergic rhinitis and pulmonary fibrosis/COPD. Full-process quality control ensures compliance across R&D and application.



Core Pipelines

Based on dental pulp MSCs, the company develops along two differentiated technical routes: Dental pulp MSCs—without gene editing, relying on native cellular activity for immune regulation and tissue regeneration and repair, focusing on chronic diseases such as oral mucosal disorders, bone defects, skin immunity and peripheral vascular ischaemia. Gene-modified MSCs—introducing growth factor genes to strengthen anti-fibrosis, pro-angiogenesis, anti-inflammatory and lesion-targeted homing capabilities, targeting severe refractory chronic diseases such as pulmonary fibrosis, hepatic fibrosis, allergic airway diseases, limb ischaemia and nerve injury. The two platforms share the upstream manufacturing system. Dental pulp MSCs focus on mild-to-moderate tissue defect repair, while gene-modified MSCs focus on severe fibrosis and ischemic injury, forming a hierarchical and complementary therapeutic pipeline portfolio.

Product	Indication / Use	Preclinical	IIT	IND	Phase I	Phase II
MSCs Products	Periodontitis	→	→	→	→	→
	Lower limb arterial occlusion	→	→			
	Psoriasis	→				
	Apical periodontitis	→				
	Reduced saliva secretion due to Sjögren's syndrome	→				
	Oral submucous fibrosis (OSF)	→				
EMSCs Products	Pulmonary fibrosis	→	→	→		
	Lower limb arterial occlusion	→	→	→		
	Asthma	→	→	→		
	Allergic rhinitis	→	→			
	Knee osteoarthritis	→	→			
	Sequelae of stroke	→	→			
	Hepatic fibrosis	→	→			

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Sino Biotech

Company Profile

Sino Biotech is a state-accredited High-Tech Enterprise headquartered in the Tianjin Binhai High-tech Zone (THT), holding prestigious honors including National Technology-based SME, Tianjin Specialized, Sophisticated, Distinctive and Innovative SME. Committed to innovation and quality, and guided by the ethos of 'Achieving oneself by helping others succeed', Sino Biotech pioneered China's first international-standard CDMO + CQO + CRO platform for cell therapeutics. Leveraging 16 bio-cell banking bases compliant with cGMP standards and the Regulations on the Management of Human Genetic Resources, the company builds a robust translation ecosystem across cell drugs, biomedical technologies, and bio-resources—standardizing the cell industry to advance global human health.

华域生物 SINO BIO

Development History

2019
- Sino Biotech Co., Ltd. was established.

2020
- Construction of the Tianjin laboratory was completed, initiating pre-production.
- The first cell batch was successfully produced in the Grade B+A laboratory.
- The first invention patent was granted.

2021
- Signed a Stem Cell Quality Verification Contract with the NIFDC, establishing a national precedent by integrating critical anti-inflammatory factors into NIFDC quality review parameters.
- Tianjin Sino Pharmaceutical Co., Ltd. was established.
- Accredited as a National High-Tech Enterprise.

2022
- The core product hUC-MSCs passed NIFDC quality verification testing.
- Granted the *Medical Device Business License*.
- Sino Life Technology (Tianjin) Co., Ltd. was established.
- Achieved three ISO management system certifications
- The Zhejiang Regional Manufacturing Center commenced operations.

2023
- Co-authored the group standard General Requirements for the Preparation of hUC-MSC for Clinical Use, which was officially promulgated and implemented.
- Approved by the Administrative Examination and Approval Bureau of THT to establish the "Sino Biotech Tianjin High-Tech Zone Bio-Resource Storage and Manufacturing Base."
- Regional manufacturing centers in Fujian, Inner Mongolia, and Ningxia commenced operations.

2024
- Co-drafted standards including Specifications for Preparation and Inspection of Human MSC Exosomes and Clinical Application Guidelines for hUC-MSC in the Treatment of KOA, which were officially approved and published.
- Elected as a council member of the CMBA
- The Northeast Center, ASEAN Center, and Shanghai Center commenced operations.

2025
- Obtained registration for a Class II medical device.
- Obtained IND approval for the proprietary hUC-MSC Injection targeting Idiopathic Pulmonary Fibrosis (IPF).
- The Suzhou Center, Hubei Center, North China Center, and Ningbo City Center commenced operations.

2026
- Jointly registered a clinical study project on hUC-MSCs for COPD with Shanghai Pulmonary Hospital via the NHC new regulatory system. It became the first batch of biomedical technology projects—and the first in the respiratory system—to achieve official filing clearance under China's updated regulatory scheme (MR-N-31-2026-000007).

Business Segments

Anchored in cell biology and regenerative medicine, Sino Biotech focuses on advancing innovation across the cell technology industry. Its core business portfolio encompasses cell biobanking, CDMO services, biomedical technology translation, cell drug IND filings, raw and auxiliary material development, cutting-edge technology R&D, health management, and bio-skincare—building a fully integrated, closed-loop ecosystem for regenerative medicine.

Figure. Core Business Operations

Cell Biobanking	Providing perinatal stem cell, adult immune cell, and adipose-derived stem cell biobanking services. Utilizing advanced extraction and isolation technologies, the platform minimizes sample collection volumes, reduces seed-cell damage risks, and shortens processing times. Backed by proprietary control systems, automated AGV operations, medical LIMS, real-time GPS tracking for logistics, and remote cloud connectivity, the biobank delivers intelligent, fully digitalized, and customized storage solutions.
CDMO Platform	Delivering turnkey solutions spanning process development, quality assessment, and scalable cGMP biomanufacturing for cell therapy and biomedical tech enterprises. Supported by clinical network partnerships, the platform offers end-to-end expertise across product development, manufacturing, testing, and regulatory filings to accelerate commercialization.
Biomedical Technology Translation	Leveraging Boao Lecheng's pilot regulatory policies, Sino Biotech has established multiple government-approved, customized fee-for-service clinical projects. The company constructed a compliant pathway—"Hospital/Patient Direct Referral + Outreach → Boao Pilot Zone Intake → Standardized Clinical Application"—ensuring cell therapies operate strictly within regulatory frameworks.
Frontier Technology R&D	Advancing next-generation cell and gene therapy (CGT) platforms, including functional stem cell strain screening, iPSCs, gene-modified stem cells, CAR-NK, in vivo CAR-T, and exosome isolation. Conducting preclinical CMC, efficacy, and toxicology studies for neurological disorders, respiratory diseases, and solid tumors.
Cell Therapeutics Pipeline	R&D pipelines focus on stem and immune cell candidates across indications such as systemic lupus erythematosus, GvHD, fibrotic diseases, and solid tumors. The lead hUC-MSC candidate for IPF has progressed into Phase I clinical trials, while the hUC-MSC candidate for COPD has secured regulatory filing clearance for biomedical technologies under the NHC.
Raw & Auxiliary Material Development	Developing critical raw materials for cell therapy, headlined by a feeder-free, serum-free, xeno-free, user-friendly NK cell expansion kit and high-performance cell cryopreservation media.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Sino Biotech

■ Core Pipeline

Sino Biotech has established a diversified R&D pipeline anchored by UC-MSCs, spanning stem cell and immune cell therapies. Focusing on major disease areas such as IPF, GvHD, KOA, and solid tumors, the company has mapped a full-lifecycle development pathway from early-stage discovery to Phase III clinical trials, demonstrating end-to-end capabilities in cell drug innovation and clinical translation.

华域生物 SINO BIO

Figure. Core Application Pipeline for Cell Therapies and Biomedical Innovations

Source	Indication	Early Discovery	CMC Development	Nonclinical Research	IND	Phase Ⅰ	Phase Ⅱ	Phase Ⅲ
Cellular Products								
UC-MSCs	IPF	Clinical Trial Approval Number 2025LP01967						
UC-MSCs	GvHD							
Pipeline Projects								
MSCs	Ischemic wound healing of the lower limbs, Osteoporosis, Erectile Dysfunction (ED), Frailty Syndrome, Sarcopenia							
NK	Solid Tumors, Alzheimer's Disease (AD)							
Genetically Modified MSC	Pulmonary Fibrosis							
CAR-NK	Solid Tumors							
iPSC	Neurological Disorders, Injury Repair							

Source	Indication	Early Discovery	CMC Development	Nonclinical Research	Regulatory Filing	Clinical Research	Translational Approval	In-hospital Application
Biomedical New Technologies								
UC-MSCs	COPD	MR-N-31-2026-000007						
IPFP	KOA	Has Advanced to the Record-Filing and Approval Phase						
UC-MSCs	Acute Kidney Injury	Has Obtained IEC Approval						
UC-MSCs	New-Onset T1D	Has Obtained IEC Approval						

■ Manufacturing Footprint

Figure. Sino Biotech's Manufacturing Footprint

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – uBriGene Biosciences

Company Profile

uBriGene Biosciences, founded in 2015, is a global company dedicated to the development and application of advanced therapy medicinal products (ATMP) technologies, providing integrated CRDMO solutions. The company has established GMP manufacturing facilities in Maryland, United States, and in Beijing, Suzhou, Jinan and Guangzhou, China, with a total area exceeding 30,000 m² and over 50 production lines covering clinical and commercial manufacturing of plasmids, viral vectors, cell therapy products, and RNA therapeutics. uBriGene has also established global R&D centers focused on innovative technology development and applications in Vancouver and Nanjing. The company has supported the production and delivery of more than 100 batches of clinical-grade products (IIT/IND/Phase I–II), assisting clients in obtaining IND approvals for more than 30 innovative drug candidates.

30,000+ m² China-U.S. GMP Manufacturing Facilities	30+ IND Filings Supported in China and U.S.	50+ CDMO Projects	100+ On-site Customer QA Audits	100+ CGT Clients Served

Development History

2015 — uBriGene Biosciences was established

2017 — Completed first CAR-T project with GMP manufacturing and clinical application; initiated the local implementation of clinical-grade AAV manufacturing processes

2019 — Jinan GMP facility launched, becoming Shandong's first gene/cell therapy manufacturing facility

2020 — Completed RMB 120 million Series A financing to advance vector technology and GMP viral vector production

2021 — Completed nearly RMB 200 million Series B financing to enhance viral vector capabilities and expand commercial manufacturing capacity

2022 — Suzhou GMP facility launched, expanding capacity and supporting overseas business

2023 — US Maryland manufacturing facility launched

2024 — Completed nearly RMB 200 million Pre-Series D financing; launched Guangzhou GMP facility and expanded commercial footprint. Recognized as a Suzhou "Potential Unicorn" and Jiangsu "Specialized and Innovative" SME

2025 — Beijing commercial manufacturing facility launched; introduced iPSC reprogramming reagent, iPSC Cell Therapy Platform, Ubri-LVV Turbo, and fully automated, closed-system TIL manufacturing platform

2026 — Launched new platforms including uVivo™ LVV Platform, circRNA Manufacturing Platform, and iPSC Reprogramming & Differentiation Platforms

Business Overview

uBriGene has established an end-to-end ATMP CRDMO service platform integrating four core manufacturing platforms with full-lifecycle capabilities spanning process and analytical development, quality control testing, regulatory affairs support, and clinical and commercial manufacturing.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – uBriGene Biosciences

■ Core Technologies in Stem Cell Business

Based on international R&D capabilities and integrated manufacturing platforms, the company has developed GMP-grade multi-lineage cell manufacturing technologies, including circRNA-LNP-based iPSC reprogramming, directed iPSC differentiation, large-scale iPSC expansion (1×10^7 cells/mL via microcarrier suspension culture), and stem cell-derived exosome production (5-fold yield improvement). These platforms enable parallel development of off-the-shelf and personalized cell therapies. uBriGene has completed FDA DMF filings for nearly 20 core technology modules and facilities, building an end-to-end ATMP lifecycle service network.

circRNA-LNP Reprogramming Technology

PBMC expansion → circRNA-LNP reprogramming → single-cell clone selection → expansion culture → stemness characterization → cryopreservation

- Non-viral, non-integrating, safe and efficient
- CircRNA: longer-lasting than mRNA. Proprietary scarless circularization technology : >50% efficiency, no exogenous residues.
- Short cycle time, stable efficiency, and durable expression
- Preserves disease phenotypes
- Enables direct and efficient PBMC reprogramming

Multi-Tiered iPSC Cell Banking Technology

Integration of diverse iPSC differentiation and scalable cell bank establishment

- Establishes diverse cell banks covering healthy donors and specific diseases
- Enables directed differentiation of iPSCs into neurons, glial cells, hepatocytes/hepatic organoids, pancreatic islet cells/islet organoids, hematopoietic stem cells, chondrocytes, and other cell types
- Supports MSC sourcing from multiple tissues, including umbilical cord and adipose tissue, with mature large-scale expansion technologies
- Facilitates clinical translation and disease research applications

- Further optimize and improve iPSC reprogramming efficiency and stability
- Develop Naïve iPSC reprogramming technologies
- Expand the range of cell types generated through iPSC-directed differentiation
- Enable efficient functional molecule loading into stem cell-derived exosomes

Large-Scale Exosome Manufacturing Technology

Diverse culture systems and purification process innovations

- Utilizes iPSC microcarrier adherent suspension culture and suspension culture systems
- Integrates tangential flow filtration and multimodal chromatography, improving purification efficiency by >30%
- Enables high-yield production (2-fold increase in iPSC density through suspension culture)
- Achieves high purity (removes >99% impurities through multimodal chromatography), supporting clinical-scale exosome manufacturing

Cell Quality Control Technology

100% in-house development and validation to ensure product quality, meet clinical requirements, and reduce testing costs

- Establishes quality control methods for various cell products, including CAR-T, iPSCs, MSCs, and NK cells
- Develops flow cytometry-based methods for cell-based potency assays

■ Core Advantages

Powered by proprietary RNA-LNP iPSC reprogramming technology, a global expert team, comprehensive IP portfolio, and worldwide GMP manufacturing capacity, uBriGene delivers end-to-end stem cell CRDMO solutions spanning from iPSC establishment to commercial production.

Leveraging proprietary Ubri-iPSC® reprogramming platform and Ubri-Amrita® RNA-LNP reprogramming kit, uBriGene has established independent intellectual property rights and a commercially viable iPSC platform that enables safe, non-viral, and direct PBMC reprogramming into iPSCs.	**Technology Platform**	**IP Portfolio**	Built around RNA-LNP delivery, iPSC reprogramming, circular RNA expression, and large-scale cell culture, uBriGene has established comprehensive proprietary IP covering reprogramming vectors, formulations, manufacturing processes, detection platforms, and serum-free iPSC culture systems, reducing reliance on overseas technology patents.
The core founding and R&D team comprises scientists with multidisciplinary expertise in stem cells, gene delivery, and GMP cell manufacturing, including PhDs from leading global institutions. Key members bring extensive experience in CGT commercialization and deep expertise in iPSC reprogramming and stem cell quality standards.	**Team Advantages**	**Manufacturing Capacity**	With coordinated capabilities across its Nanjing R&D center and GMP manufacturing facilities in Beijing, Guangzhou, and Jinan, uBriGene operates fully closed stem cell production lines incorporating Grade A isolators.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Unisum

Company Profile

Unisum Biotechnology Co., Ltd. is a leading Chinese high-tech enterprise focused on R&D of combined bioartificial liver and organoid technologies and products. Addressing the unmet clinical need in liver failure, it offers full-dimensional solutions and a robust pipeline spanning Class III active medical devices and Class I therapeutic biologics. Its regenerative medicine technologies are expanding to additional indications, with the potential to tackle real medical challenges through proprietary innovations and benefit more patients.

Development History

1996
- Professor Gao Yi initiated basic research on bioartificial liver

2006
- Led major project under the National "11th Five-Year" 863 Science and Technology Key Programme

2011
- Guangdong Unisum Biotechnology Co., Ltd. formally established;
- Continuously received major project funding under the National "12th Five-Year" 863 Programme for bioartificial liver

2019
- Participated in the "13th Five-Year" National Key R&D Programme;
- Completed Pre-A round financing

2022
- Won First Prize for Technological Invention of Guangdong Science and Technology Progress Award;
- Won highest award of National Disruptive Technology Innovation Competition of MOST;
- Completed Series A financing

2023
- World's first stem cell drug for combined bioartificial liver obtained clinical trial application approval from CDE of NMPA;
- Recognised as "Specialized, Refined, Differential and Innovative SME";
- Participated in the "14th Five-Year" National Key R&D Programme exosome project

2025
- Initiated world's first clinical study of stem cell exosomes for systemic treatment of liver failure;
- Initiated world's first clinical study of stem cell exosomes for refractory sudden deafness;
- Completed Series A+ financing

2026
- World's first "Combined Bioartificial Liver Therapeutic Device" successfully completed two registered clinical trials and entered innovation application;
- World's first stem cell drug for combined bioartificial liver completed Phase I clinical trial and plans to initiate confirmatory clinical trial;
- Participated in the "15th Five-Year" National Major Science and Technology Special Project on Emerging and Sudden Infectious Disease Prevention and Control;
- Completed Series A++ financing

Core Technology

Based on clinical needs and a staged rescue strategy for liver failure, the Unisum team introduced inflammation control alongside functional replacement and pioneered a stem-cell paracrine-based rescue approach. Multiple combined bioartificial liver technologies and products are matched to different stages of disease progression. The world's first stem-cell drug designed for combined bioartificial liver use—"MSCs for Blood Purification"—leverages the paracrine mechanism within an artificial-liver blood-purification mode, shifting the paradigm from mechanical toxin clearance to biological blockade of inflammatory storms, homeostasis regulation and regeneration support. Related products have completed fully automated process iteration. The Combined Bioartificial Liver Therapeutic Device, a major scientific achievement, was selected for the large-scale exhibition marking the 100th anniversary of the founding of the Communist Party of China in 2021 and is permanently displayed at the Museum of the Communist Party of China. The current third-generation device (Model: QH-1A) features the following unique attributes:

Integrated Design, Minimally Invasive and Safe

The device integrates biological functions with non-biological blood purification in an innovative manner, significantly reducing extracorporeal circulation blood volume and markedly lowering the impact and burden on the patient's blood system during treatment.

Broad Compatibility, Precise Personalisation

The platform has strong compatibility, can flexibly adapt to multiple cells and bioreactors, supports free switching of multiple modes, and fully meets the personalised and precise treatment needs of different patients at different disease stages.

Intelligent Centralized Control, Convenient and Reliable

Relying on a highly intelligent and programmed system, it realises centralized and unified monitoring of equipment operation and data security management. While ensuring high safety, it greatly simplifies the clinical operation process.

Invention Patents	51
Utility Model Patents	21

Technical Standards

Participated in the drafting of 3 artificial liver-related technical standards

Source: Company information, Frost & Sullivan analysis.

Stem Cell Industry Company – Unisum

R&D Pipeline

Unisum has deeply cultivated innovative therapies for liver diseases and formed multiple core product pipelines, committed to providing full-dimensional product solutions. It has achieved multiple global first milestones: the world's first "Combined Bioartificial Liver Therapeutic Device" (Model: QH-1A) has completed two registered clinical trials and entered preparation for innovation application. The world's first stem cell drug suitable for combined bioartificial liver has completed Phase I registered clinical trial, with 15 liver failure participants enrolled (baseline average MELD score 26.667), 90-day survival rate 100% and survival rate without liver transplantation 94.7%. The world's first exploratory human clinical study of "stem cell exosomes" (PT-MSCs-EV Injection) for liver failure has been completed. Among 20 participants, the 4-week survival rate reached 85% and the 12-week survival rate reached 70%, with no drug-related serious adverse events, preliminarily verifying safety and potential efficacy signals. In addition to liver failure, human umbilical cord MSC-derived exosomes have actively expanded to other indications, including refractory sudden deafness.

Product	Product Type	Application Scope	Preclinical	IND	Phase I	Phase II	Phase III
Stem cell-based bioartificial liver drug-device combination product QH-863-3 + QH-1A	Drug-device combination product	Acute-on-chronic liver failure					
PT-MSCs-EV Injection	Stem cell-derived exosome preparation	Liver failure					
		Refractory sudden deafness					

Stem Cell-Based Bioartificial Liver Drug-Device Combination Product

The QH-863-3 + QH-1A drug-device combination product has recently made breakthrough progress. It consists of the stem cell-based bioartificial liver (Class I therapeutic biological product, QH-863-3) combined with the Combined Bioartificial Liver Therapeutic Device (Class III active medical device, QH-1A). Currently, the "Combined Bioartificial Liver Therapeutic Device" has completed registration testing for a Class III active medical device, and the supporting "MSCs for Blood Purification" received IND clearance as a Class 1 therapeutic biological product. This combination product, for the first time on the basis of functional replacement, added the concept of inflammation control and proposed a new rescue technology based on the stem cell paracrine mechanism, specifically providing a new rescue solution for severe liver failure patients in the acute phase. In the latest multi-center clinical research and registered clinical trials, it has demonstrated excellent efficacy and safety: 19 patients with severe liver failure were enrolled with baseline average MELD score greater than 25. After completing 29 treatments in three dose groups, there were no related serious adverse reactions, and the 90-day survival rate reached 100%, with survival rate without liver transplantation of 94.7%, compared with an approximately 50% survival rate reported for conventional treatment.

Core Advantages

Precise Entry into a High-Mortality Therapeutic Area

China is a country with high incidence of liver diseases. Liver failure, as the terminal stage of various liver diseases, is critical with extremely high mortality. Although liver transplantation is currently the only treatment with curative potential, it is limited by extreme scarcity of donors, and most patients cannot receive timely and effective treatment, resulting in huge unmet clinical needs. Unisum precisely enters this blank field with huge supply-demand gap and extreme lack of effective therapies, with extremely broad market potential and clinical application prospects.

Core Technology of "Combined Bioartificial Liver"

The company pioneered the internationally first "cell therapy + high-end medical device" combined bioartificial liver solution. This solution not only has the potential to significantly increase the expected cure rate of patients to 80%, but also compresses the treatment cost to within RMB 100,000 through underlying technological breakthroughs. Currently, two core products have entered the clinical IND application stage. At the same time, the mature cell bank it first established has built a "catch-up barrier" of at least 5 years for competitors. Combined with a full-dimensional patent portfolio of 72 patents including 51 high-value invention patents, covering core components such as contact bioreactors, it has established an extremely deep technological and temporal moat.

Interdisciplinary Top-Tier Industry-University-Research Translation Team

Drug-device combination innovation places extremely high requirements on the team's composite capabilities. Unisum was founded under the leadership of the Director of Hepatobiliary Surgery of Southern Medical University, who combines profound theory with rich frontline clinical experience. The R&D team further gathers 2 national "863" project leaders and 5 experts with Chinese Academy of Sciences system backgrounds. This top-tier "full-stack" team that integrates cutting-edge biological science, engineering devices and clinical medicine fundamentally opens up an efficient translation path for cutting-edge scientific research results from the laboratory to the operating table.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Vcanbio

Company Profile

Vcanbio Cell & Gene Engineering Corp., Ltd. (600645.SH), a leading publicly listed stem cell company in China, is guided by its vision of "Precision Medicine for the Benefit of Humanity." Its business spans Precision Prevention, Precision Diagnostics, and Cell Therapy, providing life-cycle health management services. In cell therapy, Vcanbio partners with leading institutions, including the Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences, to build an integrated industry–academia–research–clinical platform and advance cell therapy and healthcare in China.

Development History

1993 – Listed on the Shanghai Stock Exchange Main Board

2000 – Entered the life sciences sector

2002 – Tianjin Cord Blood Hematopoietic Stem Cell Tianjin Cord Blood Bank received one of China's first Ministry of Health licenses (No. [2002] 002); the National Stem Cell Engineering Research Center was approved.

2004 – Phase I of the National Stem Cell Product Industrialization Base completed

2012 – Established a nationwide cell-banking network

2014 – Entered the in vitro diagnostics sector

2015 – Signed a strategic agreement with the Institute of Hematology & Blood Diseases Hospital, CAMS; Phase II of the National Stem Cell Product Industrialization Base opened.

2016 – Acquired a CAR-T patent from the CAMS hematology hospital; launched global expansion through the Vcanbio Center for Translational Biotechnology in Boston; established the Vcanbio Award for Biosciences and Medicine.

2018 – The hospital's Huayuan Campus became operational, forming an integrated industry–academia–research–clinical ecosystem for the cell industry

2021 – Recognized by China's Ministry of Science and Technology for contributions to emergency COVID-19 research; contributed to the world's first expert consensus on MSC therapy for COVID-19

2023 – VUM02 Injection received its first NMPA IND approval; the Vcanbio Award marked eight editions and 104 recipients; Juventas Cell Therapy's Inaticabtagene Autoleucel Injection (CNCT19; 源瑞达®) was launched as China's first CAR-T therapy approved for leukemia

2025 – "The clinical translation project of human umbilical cord-derived MSCs (VUM02 Injection) for liver cirrhosis was launched in Boao Lecheng and implemented at Hainan Mellsser Hospital

2026 – Union Stem Cell & Gene Engineering's ViPS iPSC line was filed under a U.S. FDA drug-substance DMF and tested by NIFDC; VUM02/VUM03 Injections secured 11 IND approvals, alongside 11 registered stem cell clinical studies.

Core Advantages

With 26 years of experience in China's cell industry, Vcanbio leverages three national-level platforms—the National Stem Cell Product Industrialization Base, National Stem Cell Engineering Research Center, and Tianjin Cord Blood Hematopoietic Stem Cell Bank—along with multiple provincial- and ministerial-level platforms. It collaborates with leading medical and research institutions to advance stem cell R&D and translation. Over the past five years, the R&D team, led by Co-President Dr. Yu Zhang, has participated in major projects including the National Key R&D Program of China, secured 11 IND approvals and 11 registered stem cell clinical research projects, published over 30 SCI-indexed papers, and obtained or published more than 20 invention patents, laying a solid foundation for technology translation and application.

- **3** — National-Level Stem Cell Platforms
- **11** — Indications Have Received IND Approval
- **26 Years** — Safe Operations
- **20** — Provinces and Municipalities with Integrated Cell Banks
- **4,000+** — Cord Blood Units Provided for Clinical Use by Tianjin Cord Blood Bank
- **104** — Chinese and International Scientists Honored with the Vcanbio Award

Vcanbio has established R&D and manufacturing bases and translational centers across multiple countries and regions worldwide.

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Vcanbio

■ Stem Cell R&D Pipeline

To date, VUM02 and VUM03 Injections—proprietary cryopreserved cell products comprising human umbilical cord-derived MSCs developed by Vcanbio's wholly owned subsidiary, Wuhan Optics Valley Vcanbiopharma Co., Ltd.—have received clinical trial approvals across 11 indications. Human Dental Pulp MSC Injection, jointly filed by Vcanbio's investee Beijing SH Biotechnology Co., Ltd. and Capital Medical University for chronic periodontitis, completed enrollment in its Phase II trial in 2024 and is undergoing scheduled follow-up. Vcanbio collaborates with leading Grade III Class A hospitals, including the Institute of Hematology & Blood Diseases Hospital, CAMS, and has secured registrations for 11 stem cell clinical research projects involving VUM02 and VUM03 with the NHC/NMPA and the CMC Logistic Support Department. According to CDE data, Vcanbio ranks first in China for MSC drug clinical trial approvals. The clinical translation of VUM02 Injection for liver cirrhosis has also been implemented in Boao Lecheng.

Product	Indication/Use	Preclinical	Preclinical Filing	IND	Phase I	Phase II	Phase III	Marketed
VUM02	Severe/Critical COVID-19							
	Severe/Critical Pneumonia							
	Decompensated Cirrhosis							
	Idiopathic Pulmonary Fibrosis						FDA Orphan Drug Designation	
	aGvHD					FDA Orphan Drug Designation		
	Progressive Pulmonary Fibrosis Following Pneumonia							
	ARDS							
	Acute-on-Chronic (Subacute) Liver Failure							
	Systemic Sclerosis							
	Active Moderate-to-Severe Ulcerative Colitis							
VUM03	Complex Perianal Fistulas in Crohn's Disease							
Human Dental Pulp MSC Injection	Chronic Periodontitis							

■ Three Clinical-Grade Stem Cell R&D and Translation Platforms

Cord Blood HSCs (2000–Present)

- Tianjin Key Laboratory for Blood Cell Therapy Technology and Enterprise Technology Center
- 20+ years of collaboration with the Institute of Hematology & Blood Diseases Hospital, CAMS & PUMC
- Over 4,000 cord blood units supplied for transplantation by December 31, 2025
- Developing cord blood-derived NK cells, expanded HSCs and CBMCs
- Supported by the National Key R&D Program for Frontier Biotechnology

Umbilical Cord MSCs (2010–Present)

- Tianjin Key Laboratory of Stem Cell and Regenerative Medicine Translation
- 11 CDE-cleared INDs, two FDA Orphan Drug Designations and 11 registered clinical research projects
- NIFDC testing, CMBA recognition, CNAS accreditation, FDA DMF filing, INCI registration and three ISO certifications
- Supported by National Key R&D Programs for infectious disease control, stem cells and organ repair

Cord Blood-Derived iPSCs (2020–Present)

- Research- and clinical-grade HLA-homozygous iPSC library
- Safer non-integrating electroporation system
- Derived from ethically approved, donor-consented banked CBMCs
- NIFDC testing, FDA DMF filing, hPSCreg registration and three ISO certifications
- Supported by national and Tianjin key R&D programs

■ Yuanqi ViPS Cell Bank

Vcanbio's CBMC-Derived HLA-Homozygous iPSC Bank

Leveraging over two decades of cell-industry experience and a strong track record in cell banking and clinical translation, Vcanbio (600645.SH) has built a competitive platform for clinical-grade iPSC banking and services. It is discussing HLA-homozygous iPSC line co-development with over 10 domestic and international companies and provides CDMO services for iPSC generation and banking, advancing cell therapy translation and commercialization.

Core Advantages:

- Efficient and safe reprogramming technologies for multiple cell sources, including HLA-homozygous CBMCs and PBMCs
- Certification under ISO 9001 for quality management, ISO 14001 for environmental management, and ISO 45001 for occupational health and safety management
- Testing standards accredited by the CNAS)
- Quality review and testing reports issued by the NIFDC
- Two U.S. FDA Type II Drug Master File (DMF) filings for drug substances
- Registration with the Human Pluripotent Stem Cell Registry (hPSCreg)

Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – XellSmart

Company Profile

XellSmart was founded in 2021 by Dr. Xiang Li. The company develops clinical-grade, allogeneic, off-the-shelf iPSC-derived neural cell therapies for central nervous system diseases with significant unmet medical needs, particularly Parkinson's disease. XellSmart has developed a portfolio of internationally leading proprietary technologies and platforms and has established a global intellectual property portfolio around them. Through sustained in-house R&D and innovation in its core business areas, the company has independently developed clinical-grade iPSC-derived neural cell therapy candidates for serious or life-threatening neurological diseases with clear unmet medical needs, including parkinson's disease, spinal cord injury and amyotrophic lateral sclerosis. Several of these candidates are either the first of their kind in China or the first worldwide. XellSmart is currently conducting registrational clinical trials in both China and the United States and has established itself as a global leader in this field.

Development History

2021

XellSmart was officially founded.

2023

XellSmart's clinical-grade iPSC therapy for Amyotrophic Lateral Sclerosis (ALS) has been granted Orphan Drug Designation (ODD) by the U.S. FDA, making it the world's first iPSC product to receive this recognition in the ALS field.

2024

Successfully executed China's first clinical-grade iPSC-derived cell transplantation for PD.

Secured China's first national clinical research filing approval for iPSC-derived cell therapy targeting PD.

2025

Off-the-shelf iPSC therapy for PD received FDA IND clearance to initiate a registrational clinical trial, alongside a special exemption reported by the company.

Off-the-shelf iPSC therapy for ALS secured U.S. FDA IND clearance for registrational clinical trials—the world's first for ALS.

2025

Partnered with The Third Affiliated Hospital of Sun Yat-sen University to complete first-participant dosing in a global-first registered clinical trial of off-the-shelf iPSC-derived neural cells for Spinal Cord Injury (SCI).

2025

Beijing Tiantan Hospital led first participant dosing in a registered clinical trial of XellSmart's off-the-shelf iPSC therapy for primary PD.

Achieved world-first clinical improvement in SCI using iPSC-derived neural cell therapy with The Third Affiliated Hospital of Sun Yat-sen University.

2025

Completed first participant dosing in a global-first registered clinical trial of iPSC-derived neural cells for ALS in partnership with Peking University Third Hospital.

2025

Completed first-participant dosing of clinical-grade iPSC-derived dopaminergic progenitor cells for early-onset PD in collaboration with Huashan Hospital, National Center for Neurological Disorders.

2026

Announced RMB 400 million in market-based Series B/B+ and C1 financing rounds.

Series B/B+ co-led by 3SBio, Tigermed/Taikun Capital, and Asymchem; Series C1 led by China Reform Fund, with follow-on backing from Yuyao Industry Investment.

2026

Announced RMB 500 million in Series C/C+ market-based financing rounds, co-led by China's State-Owned Capital Venture Capital Fund and Jiangsu Social Security Science and Technology Venture Capital Fund.

2026

Initiated a nationwide multicenter Phase II clinical trial for off-the-shelf iPSC-derived cell therapy in primary PD, led by Beijing Tiantan Hospital, National Center for Neurological Disorders, and have now completed Phase II enrollment.

2026

Beijing Tiantan Hospital led first participant dosing in a registered clinical trial of off-the-shelf iPSC therapy for MSA-P.

Launched a National Science and Technology Major Project as project lead for its off-the-shelf dopaminergic candidate (XS411 Injection) in PD.

Core Platforms

XellSmart's core technology is anchored by its proprietary XellSmart Reprogramming & Lineage-Specific Differentiation Platform, which efficiently converts somatic cells into clinical-grade iPSCs at high safety standards with zero genomic integration—enabling the scalable manufacturing of off-the-shelf, allogeneic neural cell therapeutics to tackle major neurological disorders.

XellSmart Reprogramming Platform	Rapidly, efficiently, and stably converts somatic cells into clinical-grade iPSCs completely free of genomic integration. The platform integrates a single-cell clone tracking and traceability system, ensuring fully traceable cell origins and stringent quality control for every cell line.
XellSmart Gene Editing Platform	Enables single or multiplex gene knockout, knock-in, knockdown, overexpression, and locus-specific mutations at the iPSC stage in a single step, establishing a precise, stable, and scalable source for off-the-shelf allogeneic cell therapies.
XellSmart Pluripotent Stem Cell Induction & Differentiation Platform	Rapidly and efficiently directs the differentiation of human pluripotent stem cells (hPSCs) into functional neurons, as well as region-specific human glial cells and other specialized cell types, with high efficiency and stability *in vitro*.
XellSmart Preclinical Animal Disease Models Platform	Constructs high-quality, disease-relevant animal models paired with pathological phenotype mapping and multi-dimensional evaluation metrics for core neurological indications, providing robust support for mechanistic study, drug screening, and therapeutic validation.

Source: Company information, Frost & Sullivan analysis.

Stem Cell Industry Company – XellSmart

R&D Pipeline

XellSmart has received nine clinical trial clearances from the NMPA and the U.S. FDA. Multiple off-the-shelf, iPSC-derived neural cell therapeutics are currently advancing through registrational clinical trials across China and the U.S, targeting primary Parkinson's disease (China Phase II and U.S. Phase I), early-onset Parkinson's disease (China Phase II), MSA-P (China Phase I/II and U.S. Phase I), and Spinal Cord Injury (SCI; China and U.S. Phase I). Additionally, its candidate for ALS (China Phase I/II and U.S. Phase I) stands as China's first proprietary iPSC-derived cell therapeutic to receive FDA IND clearance and Orphan Drug Designation (ODD).

Products	Indication	Drug Discovery	Pre-clinical	IND-Enabling	FIH Research	Phase I	Phase II	Phase III	Region
XS411 Injection	Primary PD	FDA Special Exemption, Innovative Drug Research and Development; National Science and Technology Major Project, FDA FTD							China/ U.S.
	EOPD	National Science and Technology Major Project for Brain Science and Brain-Inspired Research							China
XS228 Injection	MSA-P								China/ U.S.
	Spinal Cord Injury	First-in-class							China/ U.S.
	Amyotrophic Lateral Sclerosis	First-in-class, FDA ODD							China/ U.S.

Abbreviations: primary PD – Primary Parkinson's disease; EOPD – Early-onset Parkinson's disease; MSA-P – Multiple system atrophy, parkinsonian type

R&D Capabilities

XellSmart has long focused on developing clinical-grade, off-the-shelf allogeneic iPSC-derived cell therapies to address central nervous system disorders with major unmet medical needs. Building upon the founding team's 16 years of technological expertise, its early-stage research milestones and clinical translation pathways across three core therapeutic areas are as follows:

Parkinson's Disease—The second most common neurodegenerative disorder globally

Prior to formal registrational clinical trials, XellSmart completed multiple GCP/GMP-compliant cell therapy transplantations in primary Parkinson's disease patients, including China's first case. Key efficacy data from registrational clinical trials (>40 patients with mid-to-late-stage PD) showed significant post-transplantation improvements in OFF-state MDS-UPDRS Part III motor scores, ON-time without troublesome dyskinesia, and daily quality-of-life scores compared to baseline. Regarding safety, no adverse events related to the transplanted cells were reported. Enrollment has been completed in a nationwide multicenter Phase II registrational trial using an internationally recognized PROBE-design RCT, providing substantially stronger evidence than a single-arm study.

Amyotrophic Lateral Sclerosis—A fatal neurodegenerative motor neuron disease

XS228 Injection is China's the first and only proprietary iPSC-derived cell candidate to receive U.S. FDA clearance and global ODD for the treatment of ALS. Led by Peking University Third Hospital, with Professor Dongsheng Fan serving as the principal investigator, more than 10 participants received XS228 via lumbar puncture. The procedures were completed successfully, with no procedure-related or periprocedural complications reported. Phase I follow-up data indicated a favorable safety profile and preliminary signs of efficacy.

Spinal Cord Injury—A severe physical injury to the central nervous system

Following clinical trial clearances in China and the U.S., registrational clinical trials are underway at the Third Affiliated Hospital of Sun Yat-sen University and the First Affiliated Hospital of Dalian Medical University. More than 10 participants have received treatment, including the first participant enrolled globally. At 360 days after treatment, this patient showed sustained improvements in motor and neurological function and regained the ability to stand. These findings represent an important clinical milestone in regenerative medicine research for spinal cord injury.

Core Advantages

Patents & Intellectual Property

XellSmart maintains a robust global IP portfolio covering lineage-specific subtype differentiation and hypoimmunogenic stem cell engineering platforms. With ***dozens of patents*** filed and granted ***across major pharmaceutical markets***—including the US, EU, Japan, and China—XellSmart strategically protects the commercial value of its innovations.

Leadership & Team

Led by Dr. Xiang Li, XellSmart's 100+ member team spans all key stages of iPSC-derived therapy R&D and commercialization. R&D personnel comprise ***over 70%*** of total staff, ***with 30+ members holding PhD/Postdoc degrees or 10+ years of industry experience***. Recruited from top universities and global cell therapy leaders, the team brings deep expertise in stem cell innovation and clinical translation.

Industrialization Platforms & Facilities

Operating ***>14,000m²*** R&D center, B+A-grade GMP manufacturing facilities and dedicated QC laboratory, XellSmart has completed end-to-end CMC development and established ***clinical-grade bioprocessing and QC systems***. As an integrated platform, it manufactures formal registration and clinical batches of diverse GMP-grade neural progenitor candidates to support multiple national clinical trial filings and registrational clinical trials.

Source: Company information, Frost & Sullivan analysis.

Stem Cell Industry Company – Yuanpin Biotech

Company Profile

Founded in 2015, Yuanpin Biotech is a national high-tech enterprise specializing in stem cell and immune cell banking, cell therapy R&D, biomanufacturing, clinical applications, and life-cycle health management. As the anchor enterprise of Hunan Province's whole-cell industry chain, Yuanpin operates an end-to-end business pipeline across stem cell science and regenerative medicine.

YUANPIN BIOTECH 源品生物

Since opening its Central South Yuanpin Stem Cell Science Park in 2019, Yuanpin has driven major breakthroughs in core cell technologies and drug pipelines, while setting industry benchmarks in cell biobanking, cGMP biomanufacturing, quality control, and standardized operations—solidifying its position among China's top-tier cell therapy platforms. Yuanpin is dedicated to building a domestically leading and internationally competitive regenerative medicine industrial platform that leverages cutting-edge cell technologies to advance human health and protect tens of thousands of families.

Development History

2015
Founded in Changsha, Hunan, focusing on stem cells, immune cells, and regenerative medicine.

2018
Invested in China's first Stem Cell Science Park at Changsha Economic and Technological Development Zone (CETZ), building an end-to-end platform encompassing a biobank, biomanufacturing center, quality control center, R&D center, and clinical translation center.

2019
The Science Park officially opened. Authorized by the Hunan Provincial Development and Reform Commission, Department of Science and Technology, Medical Products Administration, and Health Commission to operate the Hunan Cell and Tissue Bank and Hunan Provincial Cell Preparation Center.

2020
Earned dual quality management certifications for cell banking and cell preparation from the China Medicinal Biotechnology Association (CMBA), becoming China's first enterprise to achieve this distinction.

2021
Partnered with Tongji University, the Changsha Municipal People's Government, and the CETZ Management Committee to establish Changsha Institute of Stem Cell and Regenerative Medicine Industrial Technology. The hUC-MSC therapy project for diabetic foot ulcers registered with the National Health Commission (NHC).

2022
Approved by the Hunan Provincial Department of Science and Technology, Hunan Engineering Research Center of Stem Cells and Translational Medicine was founded. The cell bank was built with the official approval from the Office of Chinese Human Genetic Resources Administration of the Ministry of Science and Technology.

2024
Commissioned a pilot cGMP biomanufacturing facility, completing its end-to-end industrialization platform. Obtained IND approval for a world-first hAMSC drug candidate targeting ARDS, filling a key gap in Hunan Province's Class 1 stem cell drug clinical trials.

2025
Secured a cumulative total of four IND approvals for hAMSC and hUC-MSC drug candidates, initiating Phase I clinical trials for indications including ARDS and COPD.

Core Technological Advantages

Single-Cell Sequencing and Bioinformatics-Driven Platform for Stem Cell Source Selection

Building on single-cell sequencing findings published in Nature, Yuanpin established a stem cell multi-omics database. Integrating single-cell sequencing, cell subpopulation analysis, and cytokine arrays, the company elucidates functional links between clinical efficacy and MSCs derived from distinct sources, including umbilical cord and amniotic membrane. Concurrently, optimized bioprocessing addresses bottlenecks associated with the low isolation purity and limited proliferative capacity of amniotic MSCs, forging exclusive technical advantages for cell banking and clinical translation.

Universal iPSC Engineering Platform for Enhanced Efficacy and Clinical Accessibility

Leveraging non-viral reprogramming, gene editing, and directed iPSC differentiation technologies, Yuanpin resolves industry challenges concerning iPSC genomic stability, tumorigenicity, allogeneic immunogenicity, and lineage-specific differentiation efficiency. The platform manufactures functional cells, including islet, neural, and NK cells, building cutting-edge technical barriers to address major intractable conditions such as diabetes, neurological disorders, and oncology while driving the commercialization of cell therapies.

Cell types	Source of cells	Indication	Basic research	Preclinical	Phase I	Phase II
MSCs	Human amniotic membrane	Chronic Obstructive Pulmonary Disease (COPD)				
	Human amniotic membrane	Menopausal Syndrome (MS)				
	Human amniotic membrane	Acute Respiratory Distress Syndrome (ARDS)				
	Umbilical cord	Knee Osteoarthritis (KOA)				
Islet cells	Allogeneic iPSCs	Type 1 Diabetes Mellitus (T1 DM)				
	Allogeneic iPSCs	Type 2 Diabetes Mellitus (T2 DM)				
Neural Progenitor Cells	Allogeneic iPSCs	Neurological Disorders				

Source: Company information, Frost & Sullivan analysis.

Stem Cell Industry Company – Yuanpin Biotech

Core Business

Yuanpin Biotech executes a "One Body, Two Wings" strategic roadmap, aligning with global technological frontiers and clinical market demand. With cell drug R&D, clinical translation, and industrialization serving as the primary body, two cash-flow-generating businesses—cell biobanking and cell-derived products—form the supporting wings. This strategy secures industry leadership, opens blue-ocean markets in cell therapy, and establishes a fully integrated, closed-loop cell industry platform.

YUANPIN BIOTECH 源品生物

Cellular drugs, Technology R&D, and Clinical Applications

Leveraging single-cell sequencing and multi-omics databases, the R&D team elucidates functional links between tissue-specific MSCs (umbilical cord, amniotic, chorionic, and adipose tissue) and clinical indications via subpopulation profiling and cytokine arrays. Innovative bioprocess optimization overcomes historical bottlenecks, resolving low separation purity and weak proliferation in human amniotic MSCs.

- **Proprietary Drug R&D:**

Human amniotic MSCs for acute respiratory distress syndrome (ARDS), human amniotic MSCs for COPD, human amniotic MSCs for menopausal syndrome (MS), and human umbilical cord MSCs for knee osteoarthritis (KOA).

- **Proprietary Technical Research:**

Human umbilical cord MSCs for non-healing diabetic foot ulcers, human amniotic MSCs for premature ovarian insufficiency (POI), human umbilical cord MSCs for pediatric autism spectrum disorder, and scalable manufacturing of stem cell-derived exosomes for tissue repair.

Neonatal Stem Cell Banking

- **Stored cell types:** Umbilical cord MSCs, placental amniotic MSCs, and placental sub-totipotent stem cells.
- **Key concept: "Neonatal Restoration"**—birth represents the optimal window for stem cell preservation.
- Operations span multiple provinces nationwide, covering 14 cities and prefectures across Hunan Province with nearly 100 partner hospitals, leading the regional market.

Adult Immune Cell Banking

- Cryopreserving adult peripheral blood immune cells for long-term storage to boost immunity and support interventions for oncology and immune-related disorders.
- Backed by the **Hunan Cell and Tissue Bank** and **Hunan Provincial Cell Preparation Center**, providing full-cycle health management services paired with immune health monitoring and clinical-grade preparation.
- Successfully developed professional service networks across 12 provinces and regions, including Macao and Malaysia, with over 60 service partners established in Hunan alone.

iPSC Biobanking

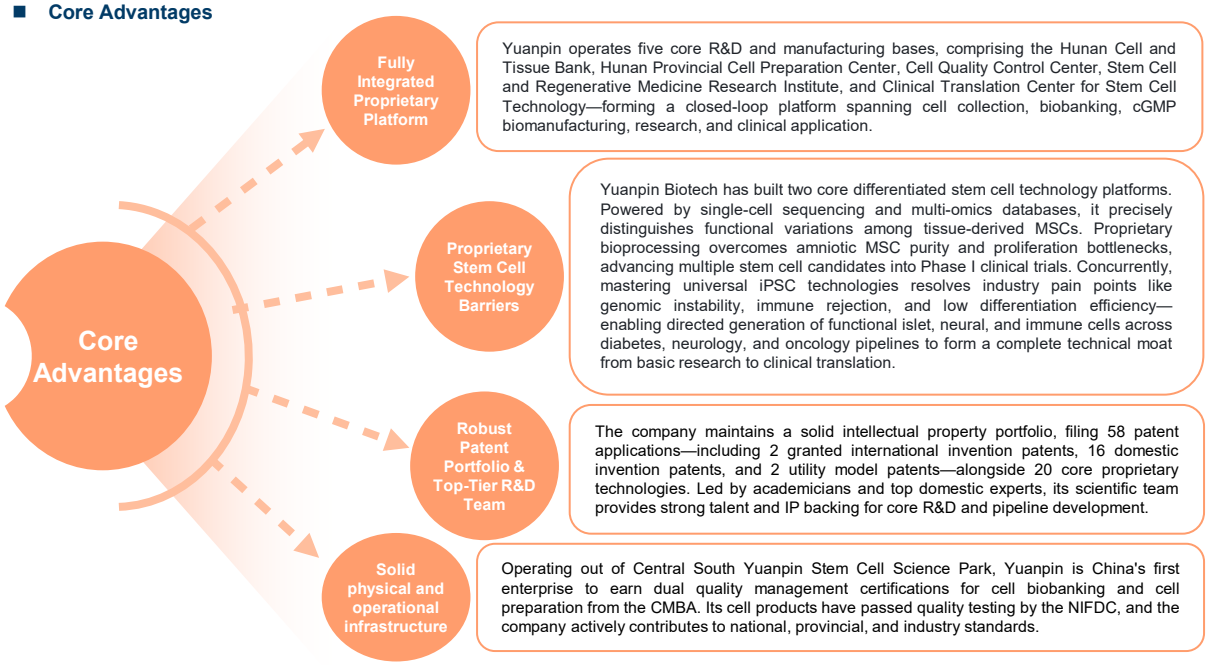
Induced pluripotent stem cell (iPSC) banking collects healthy somatic cells (e.g., peripheral blood), reprograms them into pluripotency, and cryopreserves them in liquid nitrogen (-196°C).

Technical Support: Backed by proprietary platforms spanning iPSC reprogramming, gene editing, culture, preparation, differentiation, identification and quality testing to target diabetes, neuropsychiatric disorders, neurological disorders, and oncology.

Cell-Derived Products

- Developing cell-derived skincare products, including liquid formulations, creams and lotions alongside novel bioactive ingredients (e.g., cell peptides) for functional skincare.
- Brands: CERIN, CERIN Cellab
- Regulatory Registrations and Filings: 2 Class II Medical Devices, 13 Cosmetic Registrations (China), 10 U.S. FDA Registrations

Core Advantages



Source: Company information, Frost & Sullivan analysis.

Stem Cell Industry Company – Zephyrm

Company Profile

Zephyrm Bioscience is dedicated to developing innovative cell therapy products derived from pluripotent stem cells ("PSC") for the treatment of multiple diseases. The company was recognised as a National High-Tech Enterprise in 2021 and as a Beijing "Specialized, Refined, Differential and Innovative" SME in January 2022. As one of the earliest companies in China and globally to develop PSC-derived cell therapy products, Zephyrm is among the first batch in China to obtain IND approval for PSC-derived cell therapies and the only company in China with an hESC-derived cell therapy product that has entered clinical trials.

Development History

2017
- Company founded

2019
- R&D and GMP production base completed;
- Strategic cooperation agreement signed with the Institute of Stem Cell and Regenerative Medicine, Chinese Academy of Sciences;
- Recognised as Zhongguancun High-Tech Enterprise

2020
- Received the Ministry of Science and Technology "Science and Technology Boosting the Economy 2020" key special project

2021
- Recognised as "2020 China Potential Unicorn Enterprise", National High-Tech Enterprise and "Top 50 Most Innovative Biopharmaceutical Enterprises in China 2021";
- CAStem cell injection obtained implied clinical trial approval for the treatment of meniscus injury

2022
- CAStem cell injection obtained implied approval to initiate a Phase II clinical trial for acute respiratory distress syndrome;
- Participated in and contributed to the publication of ISO 24603:2022, the first international standard for pluripotent stem cell biobanking

2023
- Testing laboratory obtained CNAS accreditation;
- CAStem cell injection approved for Phase II trials in acute exacerbation of interstitial lung disease and acute graft-versus-host disease

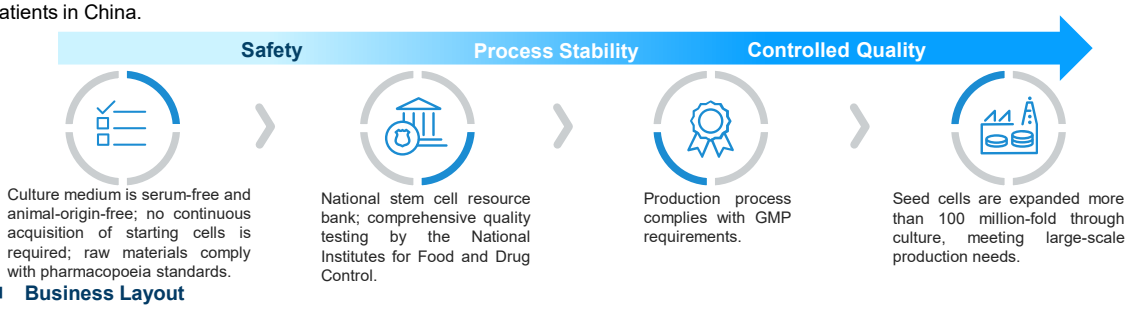
2024
- Completed Series B++ financing

2025
- Completed Series C financing

2026
- aGVHD indication preparations are underway for a Phase III clinical trial; AE-ILD indication in discussions with the regulatory authority regarding a Phase III clinical trial

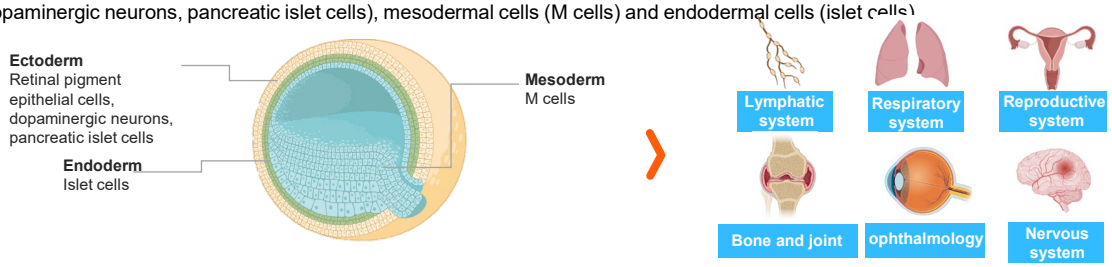
Production Base

Zephyrm has a 2,400 m² R&D and production base in the East Zone of Zhongguancun Science Park, Changping, Beijing, featuring a 500 m² "B+A" cleanroom workshop and a CNAS-accredited QC laboratory. It has established an international first-class GMP stem cell drug production facility equipped with comprehensive cell culture and quality-testing equipment, capable of preparing clinical trial materials meeting cGMP requirements. Multiple batches of investigational cell therapy products have been used in local and systemic administration clinical trials, fully meeting the current demand for providing safe, reliable and high-quality stem cell drugs to patients in China.



Business Layout

The recent focus of Zephyrm's development work is on hESC-derived therapeutic products, which are human embryo-derived PSCs capable of differentiating into all cell types of the human body. The company possesses multiple unique core technologies for differentiating PSCs (pluripotent stem cells) into different functional cells, covering ectodermal cells (retinal pigment epithelial cells, dopaminergic neurons, pancreatic islet cells), mesodermal cells (M cells) and endodermal cells (islet cells).



Source: Company Information, Frost & Sullivan Analysis.

Stem Cell Industry Company – Zephyrm

Core Products

Zephyrm leverages its proprietary PROF integrated pluripotent stem cell platform to develop hESC-derived therapies, building a differentiated pipeline of four core candidates. The lead asset ZH901 (CAStem cell injection), an immune and matrix regulatory cell derived from human embryonic stem cells, is among the few PSC-derived products in China with multiple indications advancing in parallel. It has received NMPA clearance to conduct clinical trials in four indications—acute respiratory distress syndrome, acute exacerbation of interstitial lung disease, acute graft-versus-host disease (aGVHD, Phase II completed) and meniscus injury—while ZH902, ZH903 and ZH906 remain in preclinical development for dry age-related macular degeneration, Parkinson's disease and corneal endothelial decompensation. The pipeline addresses degenerative, inflammatory and tissue-injury diseases across respiratory, immune, musculoskeletal, neurological and ophthalmic systems that currently lack effective treatments. All products are manufactured from a standardized seed cell bank derived from a common source, enabling GMP-compliant large-scale production.

ZEPHYRM 泽辉生物

Clinical Pipeline

	Product	Indication	Regulator	Preclinical	IND	Phase I	Phase II	Phase III
M cells	ZH901	Acute exacerbation of interstitial lung disease	NMPA					
		Acute graft-versus-host disease	NMPA					
		Meniscus injury	NMPA					
		All-cause acute respiratory distress syndrome	NMPA					

Preclinical Pipeline

	Product	Indication	Regulator	Preclinical	IND	Phase I	Phase II	Phase III
mDAP cells	ZH903	Parkinson's disease	NMPA					
RPE cells	ZH902	Dry AMD	NMPA					
CenC cells	ZH906	Corneal endothelial decompensation	NMPA					

Core Advantages

Supported by the fully integrated PROF platform, exclusive patent collaboration with the Chinese Academy of Sciences, a standardized GMP manufacturing base and stable funding, Zephyrm has established one of the few multi-indication PSC pipelines in China. Its combination of batch consistency, industrial scalability, differentiated disease coverage and a comprehensive proprietary intellectual property portfolio creates clear competitive advantages in the China's pluripotent stem cell therapy sector, with a focused strategy on advancing clinical development of cell therapy products for refractory diseases with substantial unmet medical needs.

R&D Innovation

Zephyrm has established a pluripotent stem cell drug R&D system to develop different cell therapy products for diseases with currently unmet medical needs, enabling future industrial large-scale production of cell drugs.
Based on the characteristics of its own products and key proprietary technologies in the field of cell therapy development, Zephyrm has developed multiple functional cells covering various indications including pulmonary diseases, bone and joint diseases, hematological diseases, central nervous system diseases and ophthalmic diseases.

National Standard Setting

The company participated in the formulation of the first national standard for human stem cells GB/T 42466, *Technical Specification for the Management of Pluripotent Stem Cells in Biobanks* and five group standards, including T/CSCB 0009-2022 Ethical Guidelines for Human Stem Cell Research, T/CSCB 0011-2022 Human Midbrain Dopaminergic Progenitor Cells, T/CSCB 0012-2022 Human Neural Stem Cells, T/CSCB 0015-2022 General Requirements for Extracellular Vesicles Derived from Human Stem Cells and T/CSCB 0010-2022 Human Natural Killer Cells.

Production Capacity

Since its establishment, the company has developed rapidly. In May 2019, it established an R&D and production base in the East Zone of Zhongguancun Science Park, Changping, Beijing. The production facilities are designed and operated in accordance with GMP standards, equipped with complete cell culture and testing facilities. A stem cell drug quality management system has been established, and process research and quality research on stem cell products have been successfully carried out, fully meeting the current demand for providing safe, reliable and high-quality stem cell drugs to domestic patients.

Source: Company Information, Frost & Sullivan Analysis.

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