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Cell Therapies in Healthcare

Landscape Overview Q1 2023

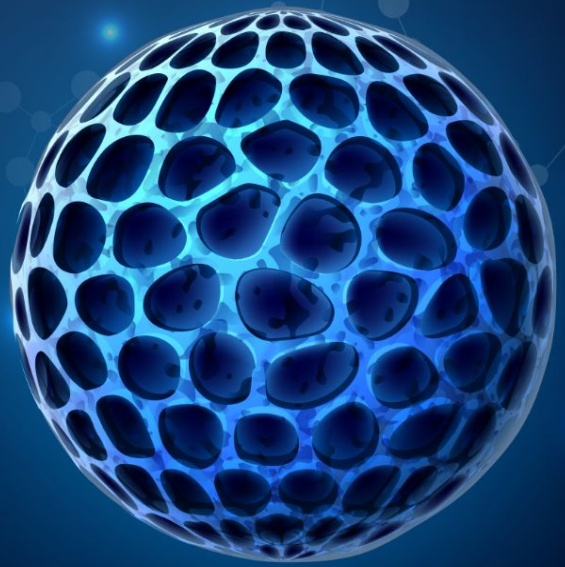

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Table of Contents

Introduction

This 62-page **"Cell Therapies in Healthcare Landscape Overview Q1 2023"** report represents the market analytics focused on the Cell Therapy Industry.

The primary goal of this report is to give a complete picture of the industry environment of Cell Therapy Industry, its clinical researches, and other elements of pharmaceutical research and development. This overview highlights recent trends and insights in the form of helpful mind maps and infographics and gauges the performance of prominent players who shape the industry's space and relationships. It can help the reader comprehend what is going on in the sector and potentially predict what will happen next.

Introduction to Cell Therapies

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Cell Therapies in Healthcare Landscape Overview Q1 2023

Companies - 400
Investors - 900

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Types of Cell Therapies

For purposes of this report, we define cell based classes as **Classical Cell Therapy** and **Gene-Based Cell Therapy**. Classical Cell Therapy can be divided into **Stem Cell-Based Therapy** and **Non Stem Cell-Based Therapy**. In this report we are going to discuss **CAR-T Cell Therapy** and **TCR-T Cell Therapy** as a basement of Gene-Based Cell Therapy. The third group will include any other Gene-Based Therapy, but the most relevant competitors are other immunotherapies such as **TILs/ NK Cells**.

Classical Cell Therapy

➔ **Stem Cell-Based Therapy**

Stem cells used or targeted by cell therapy can be grouped into three categories: Embryonic stem cells (ESCs), Adult stem cells (ASCs) and Induced pluripotent stem cells (iPSCs).

➔ **Non Stem Cell-Based Therapy**

The use of somatic cells that have been removed from a patient's body, propagated, grown, and then chosen for use in treatment, prevention, or diagnosis. Fibroblasts, chondrocytes, keratinocytes, hepatocytes, and immunological cells such T cells, dendritic cells (DCs) are examples of non-stem cell-based cell treatments.

Gene-Based Cell Therapy

➔ **CAR-T Cell Therapy**

T cell genetically modified to express a Chimeric Antigen Receptor (CAR) designed to bind a tumor-specific cell surface protein.

➔ **TCR-T Cell Therapy**

T cell genetically modified to express a T cell receptor (TCR) that binds tumor-specific antigens expressed anywhere in the cell.

➔ **Other (TILs, NK Cells)**

For example, TILs (expanded and activated naturally-occuring T cells), NK cells activated or modified to recognize a tumor specific antigen.

Cell Therapy Applications

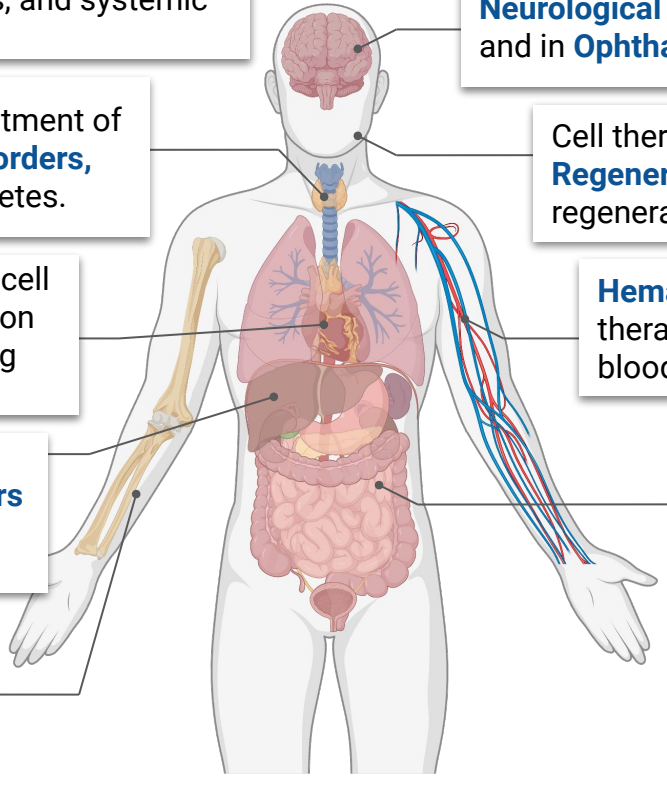
Mesenchymal stem cell therapy, has shown potential in the treatment of **Autoimmune diseases** such as rheumatoid arthritis, multiple sclerosis, and systemic lupus erythematosus.

Cell therapy holds promise for the treatment of **Metabolism and Endocrinological disorders,** including liver disease and type 1 diabetes.

Cardiovascular diseases benefit from cell therapies by promoting the regeneration of damaged heart tissue and improving heart function.

Cell therapy has shown promising results in treating **Hepatology disorders** by promoting liver regeneration and improving liver function.

Orthopedics and **Rheumatology** use cell therapy to repair damaged bones, cartilage, and joints



Cell therapy has been used in the treatment of **Neurological Disorders** such as Parkinson's disease, and in **Ophthalmology** to repair corneal damage.

Cell therapy is used in **Dermatology** and **Regenerative medicine** to promote skin regeneration and wound healing.

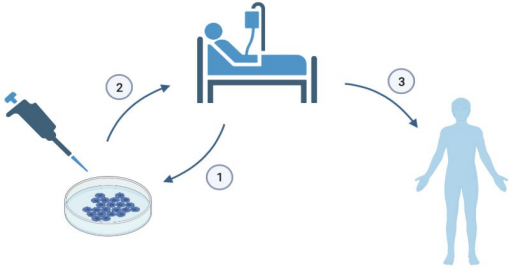
Hematology involves the use of stem cell therapy for bone marrow transplants to treat blood disorders.

In **Oncology,** stem cells in bone marrow transplants and CAR-T cells are used to target cancer cells.

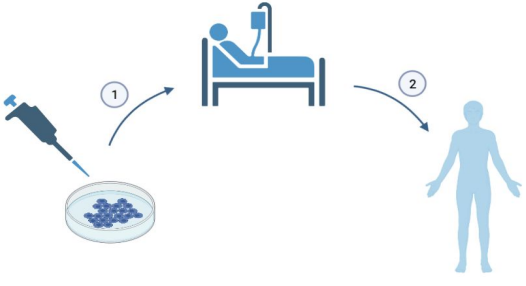
Cell therapy has shown potential in the treatment of **rare diseases,** including lysosomal storage disorders and inherited metabolic disorders.

Autologous and Allogeneic Cell Therapies

Autologous therapy: the patient's own cells are extracted (1) and edited/cultivated and returned (2) to the patient where they fight disease (3).



Allogenic therapy: donated cells, edited or unedited, (1) are delivered to a patient where they fight disease (2).



	Autologous	Allogenic
Low cost	✗	✓
Readily available	✗	✓
Long lived	✓	✗
Standard quality	✗	✓
Immune tolerance	✓	✗

The patient's own cells are transformed into treatments in **autologous cell therapies**. It is unlikely that these cells will be rejected because they originate from the patient.

Some of the problems with autologous therapies are avoided by **"allogeneic" cell therapies**. Allogeneic cells come from healthy donors rather than patients. These cells can be genetically modified ex vivo to both fight disease and reduce the likelihood of rejection.

Allogeneic cell therapies ought to be of a higher caliber as a result. Additionally, they ought to be simpler to obtain immediately or "off the shelf." Allogeneic cells that have been genetically modified are **being tested in clinical trials**.

Cell Therapy Industry at a Glance

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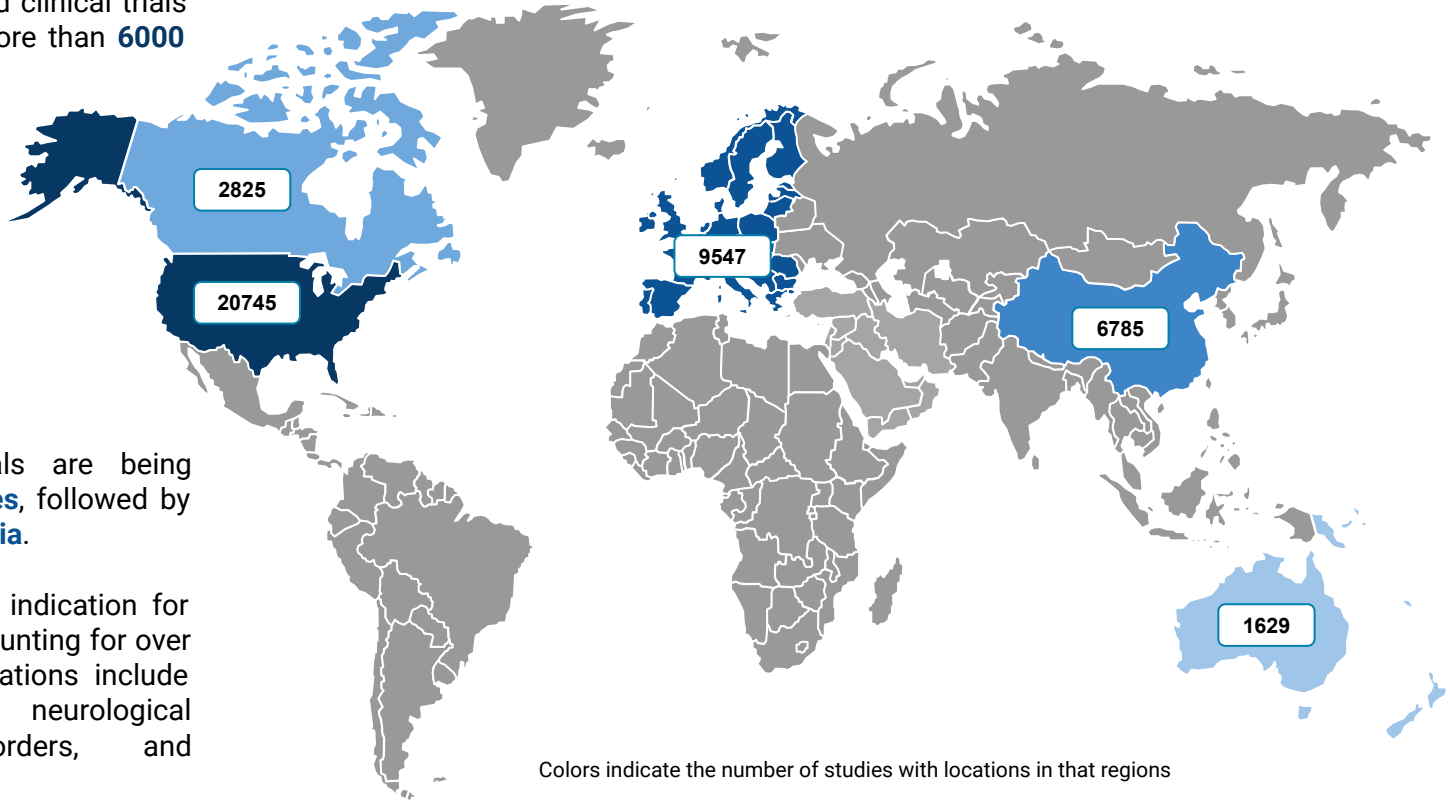
Global Cell Therapy Clinical Trials Activity: Top 5 Regions

According to ***ClinicalTrials.gov,*** there are currently over **39000** registered clinical trials related to cell therapy, with more than **6000** currently recruiting patients.

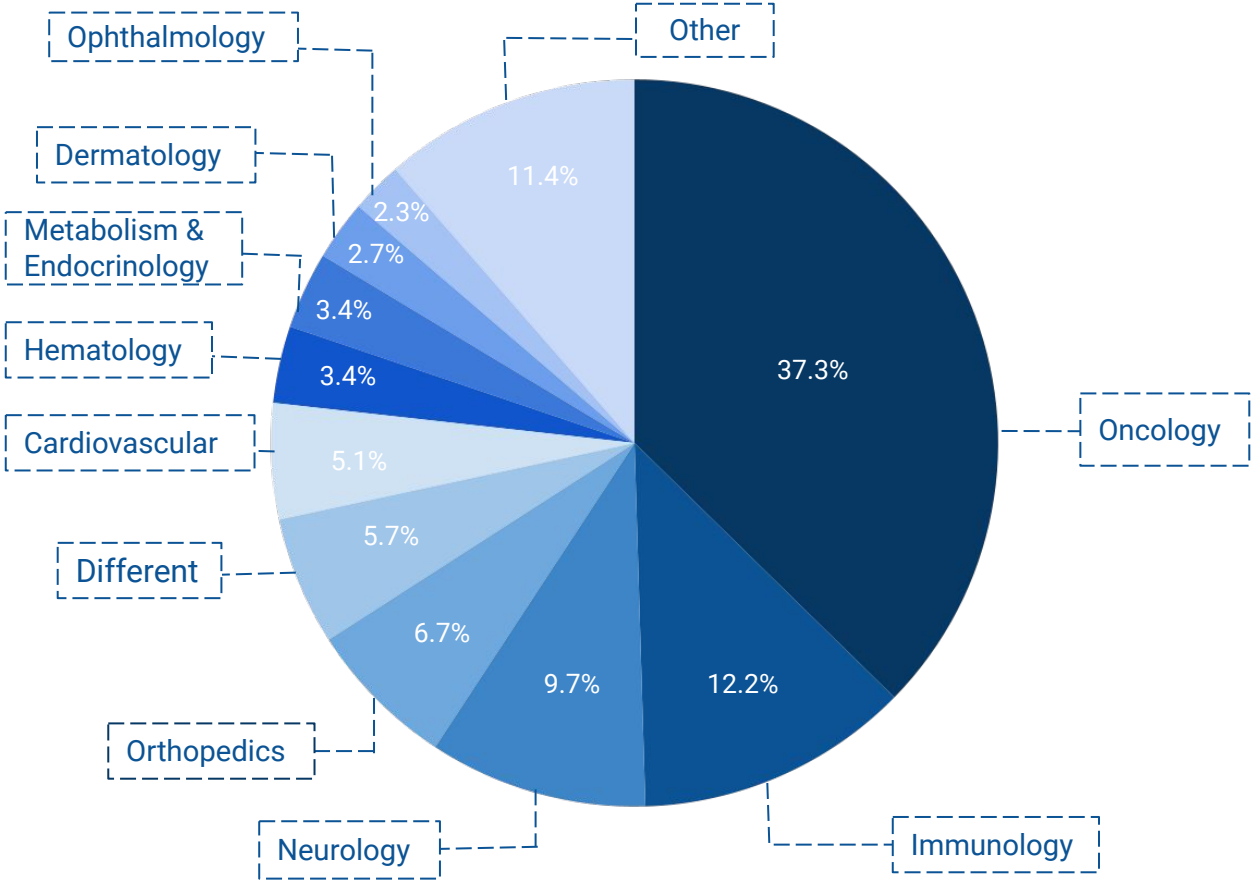


The majority of these trials are being conducted in the **United States**, followed by **EU**, **China**, **Canada**, and **Australia**.

Cancer is the most common indication for cell therapy clinical trials, accounting for over **50% of all trials**. Other indications include cardiovascular disease, neurological disorders, genetic disorders, and autoimmune diseases.

Source: https://clinicaltrials.gov/

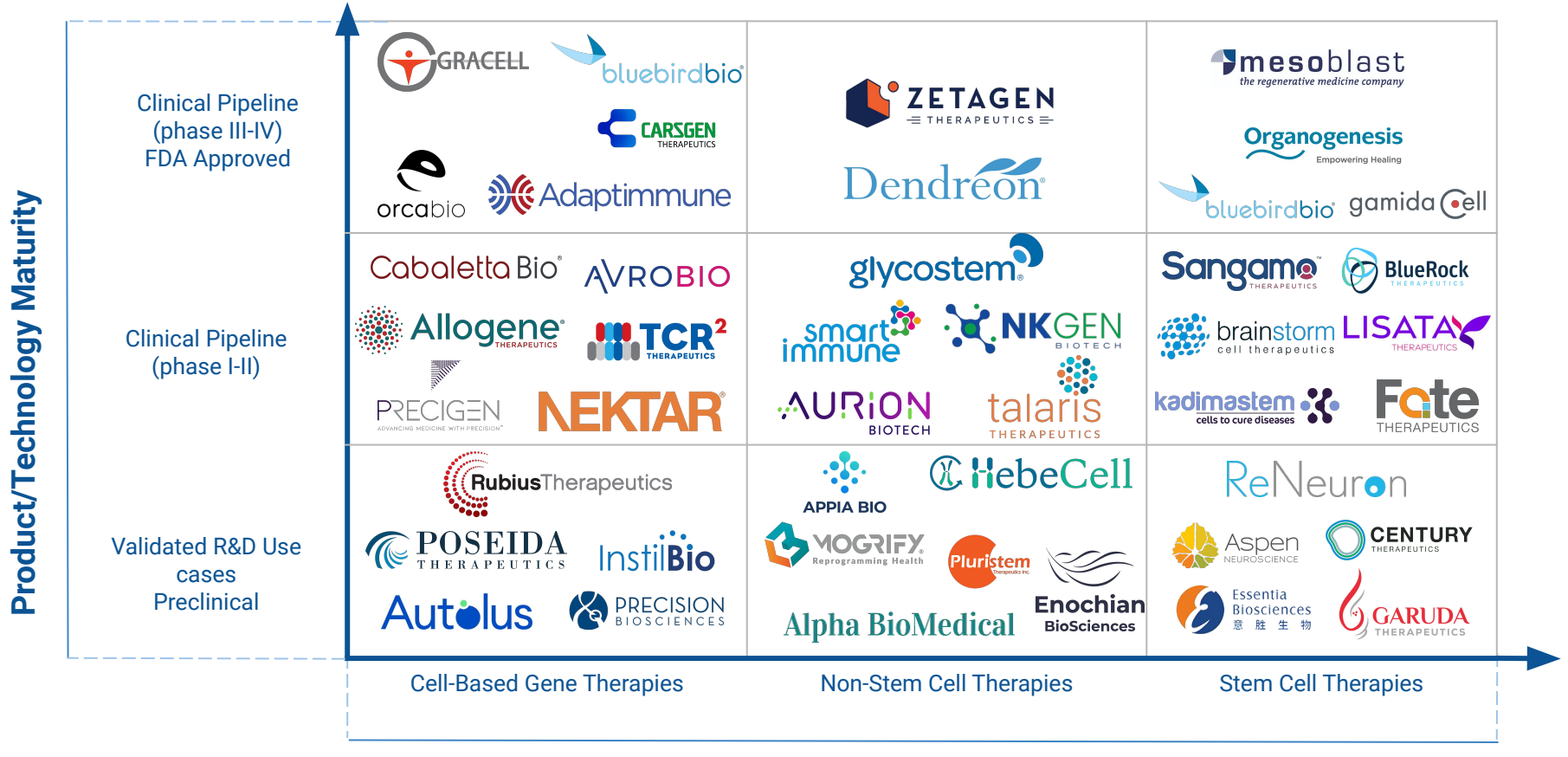
Global Cell Therapy Companies Activity: Therapeutic area



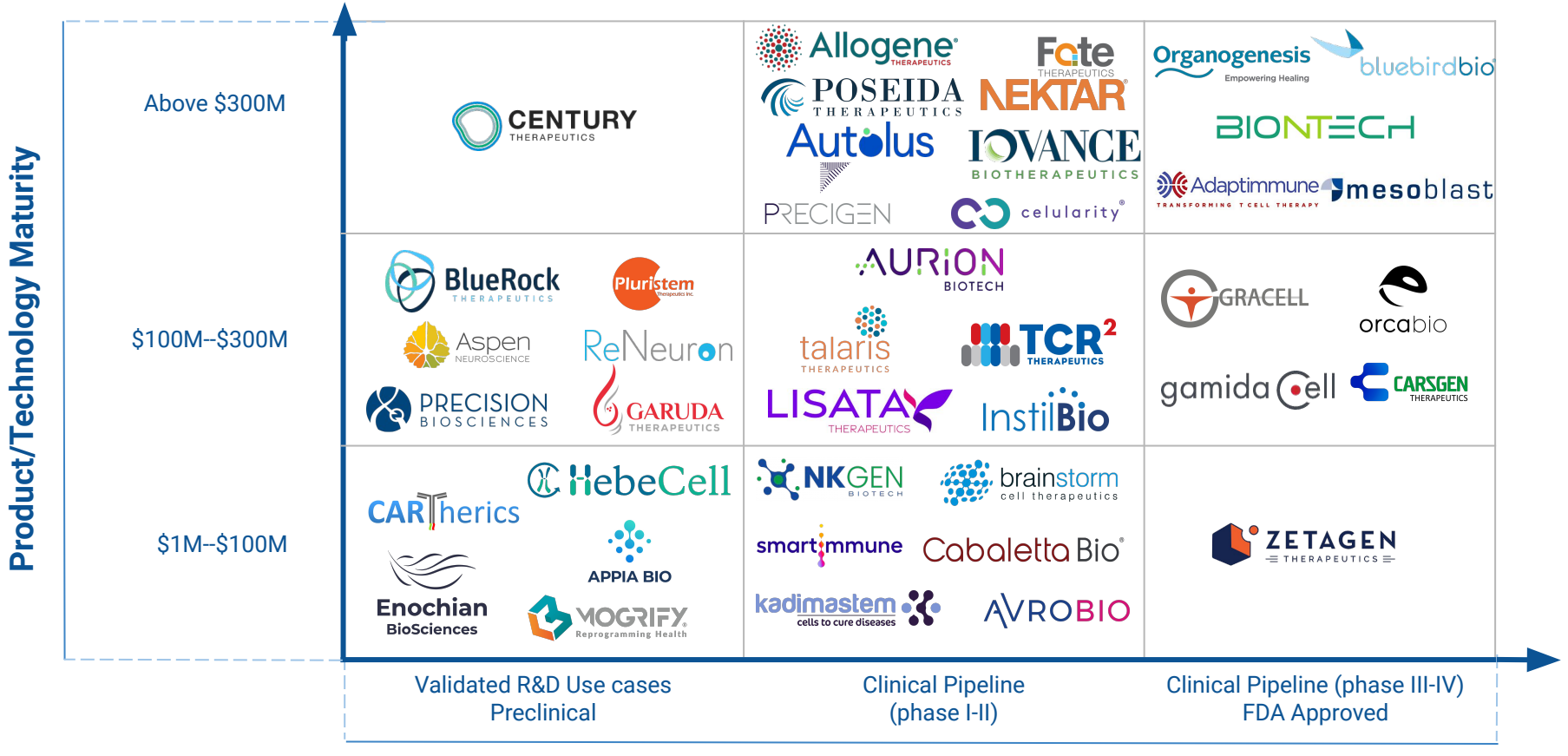
This pie chart represents a distribution of Cell Therapy Industry companies by the **therapeutic area.** The highest percentage of focus is on **Oncology,** accounting for **37.3%** of the total. This is followed by **Immunology** at **12.2%**, **Neurology** at **9.7%**, and **Orthopedics** at **6.7%**. Other areas such as **Cardiovascular, Hematology, Metabolism & Endocrinology, Dermatology**, and **Ophthalmology** are also being explored to a lesser extent, with percentages ranging from **2.3%** to **5.1%**. Additionally, **5.7%** of the companies are focusing on more than 5 different therapeutic areas simultaneously and were classified as **Different. 11.4%** of the researched companies specify on **Other** therapeutic aries such as **Hepatology, Infection diseases, Gastroenterology, Inflammation Rare diseases,** and **Rheumatology**.

Overall, the data suggests a significant emphasis on using cell therapy for cancer treatment, with a growing interest in other therapeutic areas as well.

Analysis of Top 45 Cell Therapy Companies: R&D Maturity vs Application Focus



Analysis of Top 40 Cell Therapy Companies: R&D Maturity vs Funding Level



Stem Cell Therapy

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Stem Cells Overview

ESCs

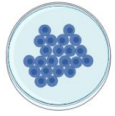
ASCs

iPSCs

Source	Derived from embryos	Found in adult tissues	Reprogrammed from adult cells
Pluripotency	Pluripotent	Multipotent	Pluripotent
Differentiation potential	Can differentiate into any cell type	Can differentiate into a limited number of cell types	Can differentiate into any cell type
Self-renewal	High	Limited	High
Ethical concerns	Controversial	None	None, but derived from adult cells
Clinical use	Limited by ethical concerns	Limited by differentiation potential and supply	Widely used, but still under study

Embryonic stem cells (**ESCs**) are derived from **early-stage embryos**, while adult stem cells (**ASCs**) are found in **various tissues** of the body. Induced pluripotent stem cells (**iPSCs**) are generated by **reprogramming** adult cells to an embryonic-like state. ESCs are pluripotent and can differentiate into any cell type, while ASCs are multipotent and can differentiate into a limited number of cell types. iPSCs have pluripotent potential similar to ESCs and can be derived from adult cells. Stem cells have potential therapeutic applications in regenerative medicine, but their use is still under study, and there are scientific challenges to overcome.

Stem Cell-Based Cell Therapies



iPSCs

Induced pluripotent stem cell (iPSC) therapies are still in the early stages of development, but they are being studied as a potential treatment for conditions such as **Parkinson's disease**, **heart disease**, and **macular degeneration.**

AcuraStem, AGEX THERAPEUTICS, Ambys MEDICINES, elpis BIOPHARMACEUTICALS, CENTURY THERAPEUTICS, Fate THERAPEUTICS, Plasticell, ReNeuron

ESCs

Embryonic stem cell (ESC) therapies are being studied as a potential treatment for conditions such as **spinal cord injuries**, **heart disease**, and **diabetes.**

AGEX THERAPEUTICS, imstem BIOTECHNOLOGY, kadimastem cells to cure diseases, LifeCell, LINEAGE CELL THERAPEUTICS, NEURONA THERAPEUTICS

ASCs

Adult stem cell (ASC) therapies include: Mesenchymal stem cell (MSC) therapy for conditions such as osteoarthritis, inflammatory bowel disease, and graft-versus-host disease;
Neural stem cell (NSC) therapy for conditions such as stroke and spinal cord injuries and many others.

NOVADIP, Orchard therapeutics, Prolacta BIOSCIENCE, Sernova Corp, NOVARTIS, regeneus living regenerative medicine, TreeFrog therapeutics

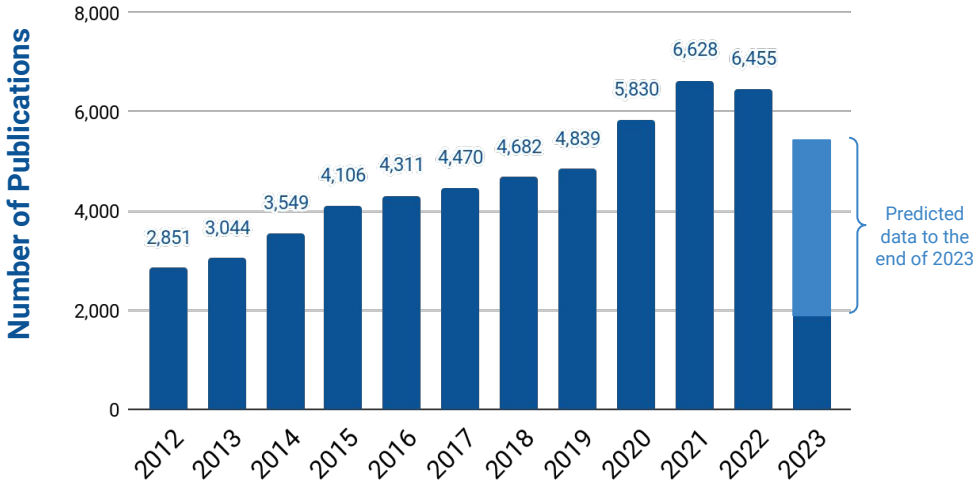
FDA Approved Stem Cell Therapies

Company	Cell Therapy Name	Disease	Year of Initial US Approval
Gamida-Cell	OMISIRGE	Hematopoietic Disorders	2023
bluebird bio	SKYSONA	Cerebral Adrenoleukodystrophy	2022
bluebird bio	ZYNTEGLO	ß-Thalassemia	2022
MD Anderson Cord Blood Bank	HPC, Cord Blood - MD Anderson Cord Blood Bank	Hematopoietic Disorders	2018
Cleveland Cord Blood Center	CLEVECORD	Hematopoietic Disorders	2016
LifeSouth Community Blood Centers	HPC, Cord Blood - LifeSouth	Hematopoietic Disorders	2016
Bloodworks	HPC, Cord Blood - Bloodworks	Hematopoietic Disorders	2016
SSM Cardinal Glennon Children's Medical Center	ALLOCORD	Hematopoietic Disorders	2013
Duke University School of Medicine	DUCORD	Hematopoietic Disorders	2012
Organogenesis	GINTUIT	Mucogingival conditions	2012
Clinimmune Labs	HPC, Cord Blood	Hematopoietic Disorders	2012
New York Blood Center	HEMACORD	Hematopoietic Disorders	2011

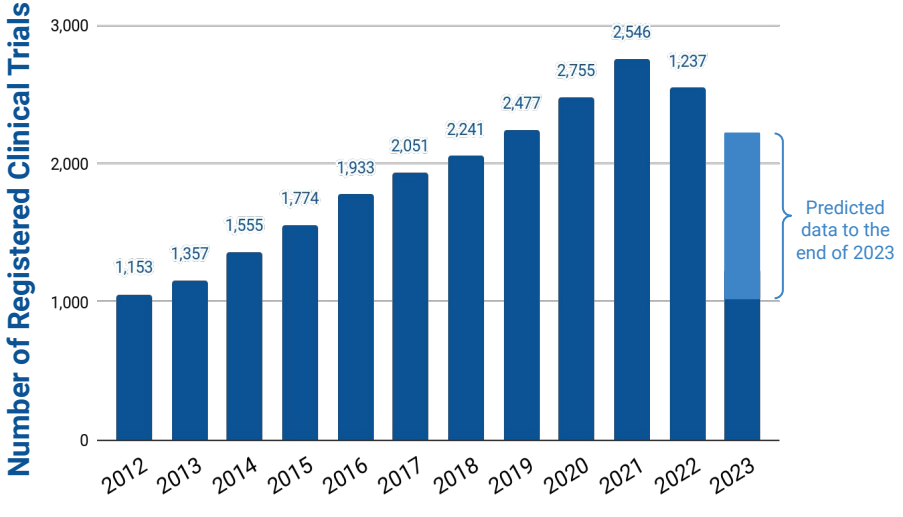
Rise of Stem Cell Therapy Development

The increasing prevalence of **chronic diseases** such as cancer, diabetes, and cardiovascular diseases has led to a rise in demand for regenerative therapies. Stem cell therapies have shown promise in treating conditions such as **osteoarthritis**, **spinal cord injuries**, and **liver disease**, among others. In addition to the medical benefits, the stem cell therapy industry has also attracted significant investment from pharmaceutical companies, research organizations, and venture capital firms. This has led to the development of **new therapies** and the expansion of **clinical trials**, further driving the growth of the industry.

Total Scientific Interest in Stem Cell Therapy



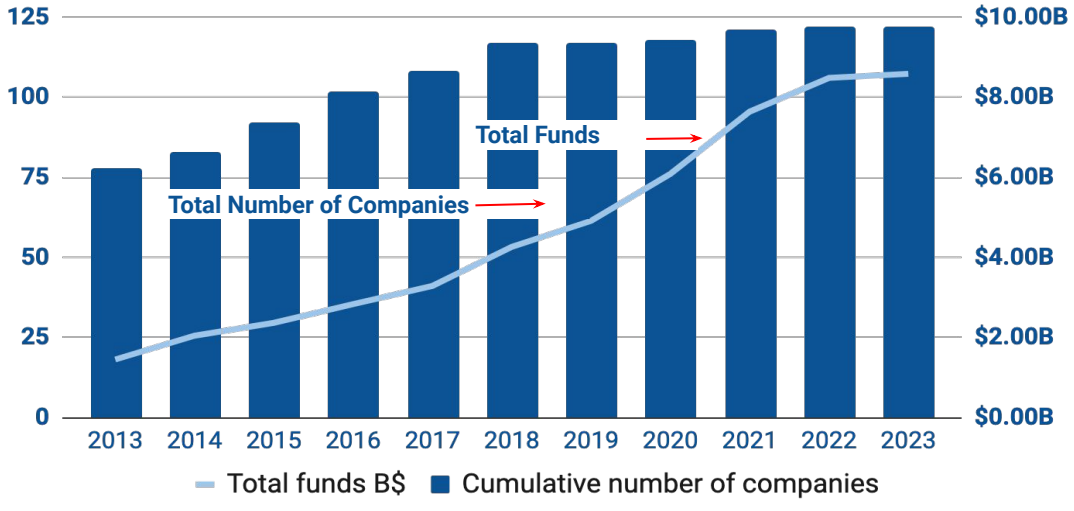
Total Clinical Interest in Stem Cell Therapy



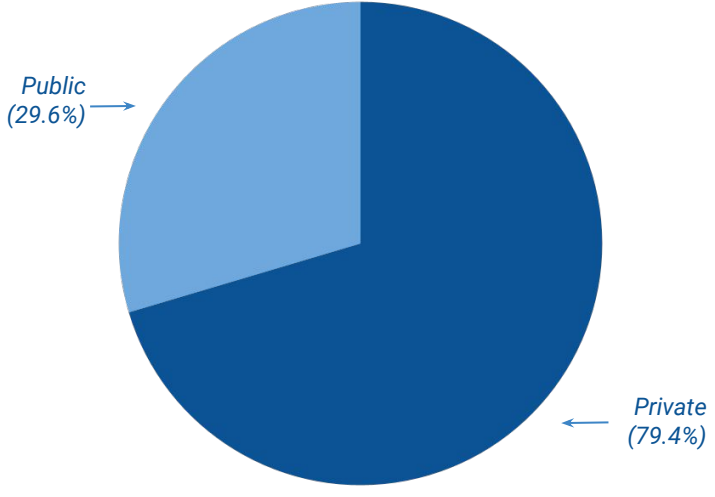
Source: https://www.ncbi.nlm.nih.gov/, https://clinicaltrials.gov/

Growth of Stem Cell Therapy Industry

Cumulative Number of Companies and Total Value of Their Funds, 2013-2023



Distribution of Companies by IPO Status

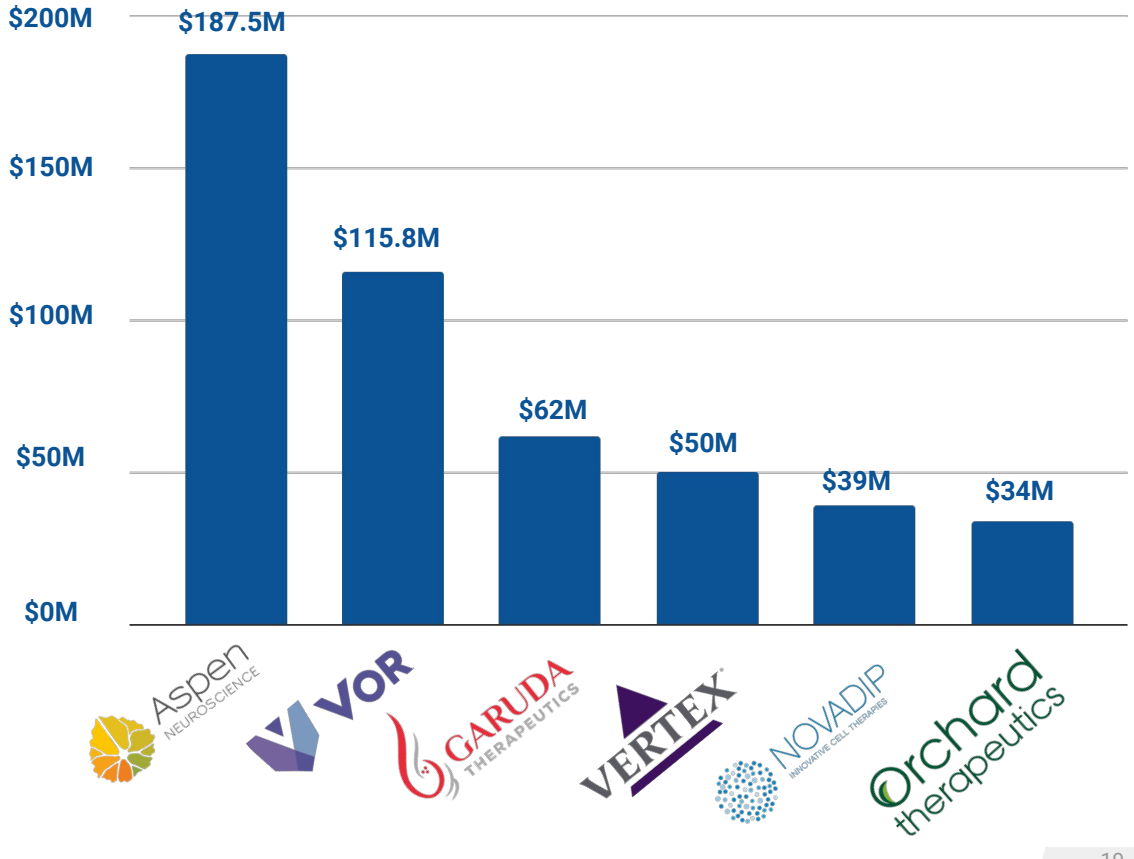


The global **stem cell therapy market** size growth significantly over the last 10 years. The share of new companies from the 2010 to 2020 period is **64%**. Stem Cell development market is valued at approximately **$8.58 billion** in Q1 2023. The industry's growth is being driven by the increasing prevalence of chronic diseases, such as **cancer** and **cardiovascular diseases**, which create a demand for stem cell therapies. Technological advancements, such as genetic engineering and gene editing, have also contributed to the development of new and innovative stem cell therapies.

Stem Cell Therapies Investment Landscape

Some of the major deals in 2022-2023 included:

- **Aspen Neuroscience** received a funding **$187.5 million** over 2 rounds, Series A and Debt Financing in 2022.
- **Vor Biopharma** raised **$115.8 million** on IPO in the United States in December 2022
- **Garuda Therapeutics** received a funding of **$62 million** over one Series B round in February 2023.
- **Vertex Pharmaceuticals** raised on IPO **$50 million** in May 2022.
- **Novadip Biosciences** has raised a total of **$50 million** during two rounds in November 2022.
- **Orchard Therapeutics** obtained on IPO **$50 million** in the United Kingdom in March 2023.



Cell Secretome Therapies

Stem cells have revolutionized **regenerative medicine,** becoming a widely used tool for tissue and organ reconstruction. However, while stem cells offer great potential for healing, their therapeutic use is limited by several challenges. The primary issue with allogeneic therapies is **immune rejection**, while the **tumorigenicity** of cell transplants and the **risk of infection** transmission also pose significant obstacles. Recent research has revealed that the therapeutic effects of stem cells are primarily due to their **secretome**, which has led to the development of **cell-free therapies**. The secretome typically consists of **soluble factors** (growth factors, cytokines, chemokines, and enzymes) and **extracellular vesicles** that transport lipids, proteins, RNA, and DNA.

Examples of companies using cell-free therapies:

Theratome Bio

Theratome Bio develops cell-free therapeutics for the treatment of **neurological disorders**, such as traumatic brain injury and stroke. Their technology involves the identification and characterization of specific **secreted factors**, including **extracellular vesicles** (EVs).

NEOBIOSIS
Regenerative. Science.

Neobiosis develops cell-based and cell-free therapies for the treatment of **chronic kidney disease**. Their main focus is to exploit the potential of perinatal birth tissues including **cells, EVs** and **matrix**.

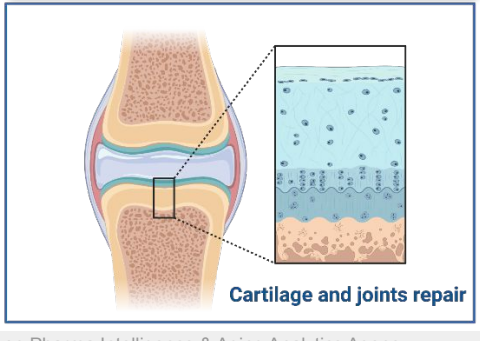
Secretome
THERAPEUTICS

Secretome Therapeutics is the world leader in the research and development of neonatal mesenchymal stem cells **(nMSCs)** for the treatment and prevention of disease. They generate a pipeline of novel secretomes that can address a wide range of diseases with a focus on **onconephrology**.

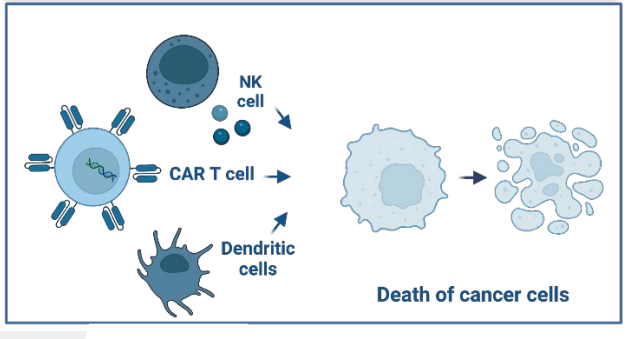
Non–Stem Cell-Based Cell Therapies

Traditionally, **Stem Cells** have been the main focus of cell therapy research, but there are also **Non-Stem Cell-Based Cell Therapies** that are promising in treating a range of conditions, including cancer, autoimmune diseases, and degenerative disorders. Unlike stem cell-based therapies, non-stem cell-based therapies use other types of cells, such as **immune cells** or **genetically modified cells**, to treat disease.One of the main benefits of non-stem cell-based therapies is that they do not require the use of embryonic stem cells, which have ethical concerns and are difficult to obtain. Non-Stem Cell-Based Therapies also have a lower risk of tumorigenesis, which is the formation of tumors, a potential risk associated with the use of stem cells.

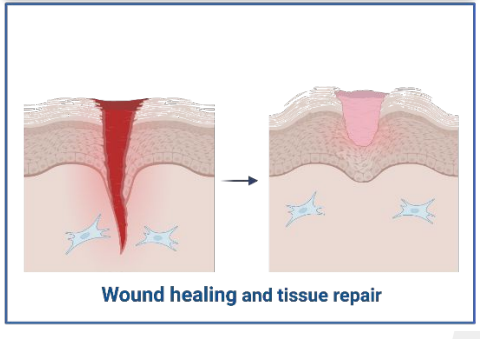
Chondrocyte Transplantation: This therapy involves transplanting healthy chondrocytes (cartilage producing cells) into a damaged joint to promote cartilage repair. It has shown promise in treating osteoarthritis, a common condition that causes joint pain and stiffness.



Immune cells therapy: This therapy uses tumor-infiltrating lymphocytes, T-cells with chimeric antigen receptor (CAR-T cells), dendritic cells, and natural killer cells to fight cancer and other conditions. These therapies involve collecting immune cells, genetically modifying them in a lab to enhance their cancer-fighting ability, and re-infusing them into the patient to target and destroy cancer cells.



Fibroblast Therapy: Fibroblasts are cells that produce connective tissue, including collagen and elastin. In fibroblast therapy, fibroblasts are harvested from a patient's own skin and cultured in a lab to increase their numbers. Afterwards, they are re-injected into the patient to promote tissue repair.



Non–Stem Cell-Based Cell Therapies MindMap

Natural Killer Cells

ImmuneBridge, PROTARA THERAPEUTICS, 鑫品生醫 Vectorite Biomedical, Alpha BioMedical, ZETAGEN THERAPEUTICS, ImmunityBio, glycostem, NEKTAR, CELLENKOS, NKGEN BIOTECH, beroni, NantKwest, CARTherics, Kyverna THERAPEUTICS, artiva, Acepodia, HebeCell, IMMUNOVATIVE Therapies Ltd., SHORELINE biosciences, MiNK Therapeutics, APPIA BIO

Bone marrow cells

BioCardia®, t2cure, Regen BioPharma, Inc.

T cells

HEMOSTEMIX, CELLENKOS®, chimera bioengineering, Acepodia, InstilBio, HEXAELL biotech, smart immune, Sana Biotechnology®

Skin cells

avita medical, LCT, TeVido BioDevices, Organogenesis Empowering Healing

Macrophages

SHORELINE biosciences, cellis®

Dendritic cells

IMMUNOVATIVE Therapies Ltd., ImmunoCellular Immunocellular Therapeutics Ltd., PDC*line pharma, REGENICN, Enochian BioSciences

Others

T, NK and B cells
CLADE THERAPEUTICS

T-reg cells
talaris THERAPEUTICS

Insulin Producing Cells
minutia

Human Auditory Sensory Cells
Rinri Therapeutics

Tumor Cells
ERC Epitopoietic Research Corporation

Endothelial Corneal Cells
AURION BIOTECH

Reprogrammed Cells
MOGRIFY Reprogramming Health

Primary Cells
KOLON TISSUEGENE

Placental Cells
Pluristem Therapeutics Inc.

Chondrocytes
ortho-cell

Note: This slide represents the main developers on the market of non-stem cell-based cell therapies. It is evident that NK cell therapy is the most popular type of non-stem cell therapies due to its demand and prevalence. Other fields are actively growing for now.

Gene-based Cell Therapy

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Gene-Based Cell Therapy

Cell-based gene therapy is an exciting area of research that involves using **genetically modified cells** to treat a variety of diseases, including cancer, genetic disorders, and autoimmune diseases. Here are some of the types of cell-based gene therapy and their main therapeutic areas:

TCR Therapy: T cell receptor (TCR) therapy involves modifying T cells to recognize and attack cancer cells. This is achieved by engineering T cells to express a specific TCR that recognizes a tumor antigen, which triggers an immune response against the cancer. TCR therapy has shown promise in treating melanoma, synovial sarcoma, and other solid tumors.

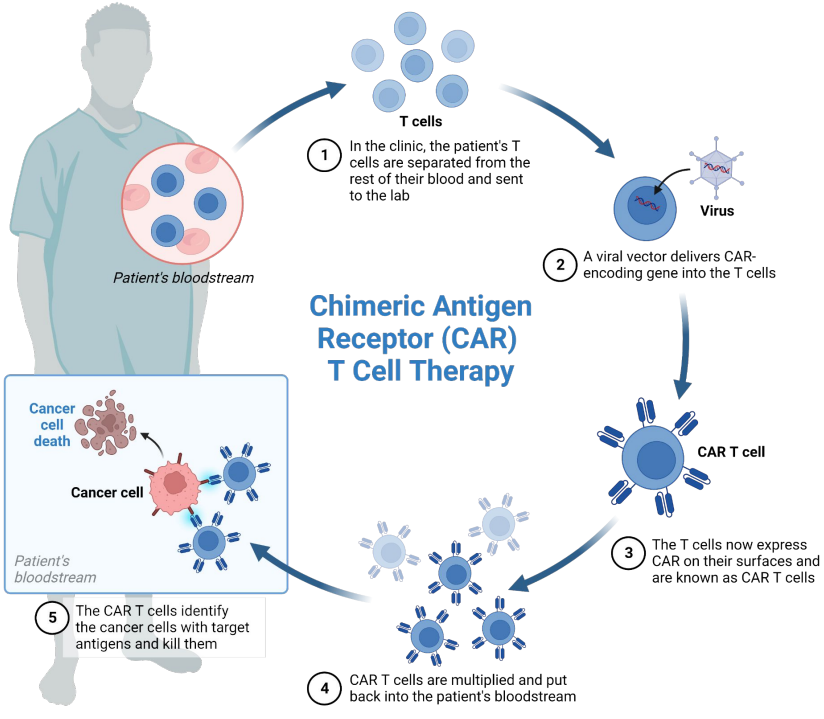
CAR-T Therapy: Chimeric antigen receptor (CAR) T cell therapy involves modifying T cells to express a CAR that recognizes a specific antigen on cancer cells. CAR-T cells are then infused back into the patient, where they can recognize and kill cancer cells. CAR-T therapy has shown remarkable success in treating certain blood cancers, such as acute lymphoblastic leukemia and non-Hodgkin lymphoma.

CAR-NK Therapy: Similar to CAR-T therapy, CAR-natural killer (NK) cell therapy involves modifying NK cells to express a CAR that recognizes a specific antigen on cancer cells. CAR-NK cells have several advantages over CAR-T cells, including their ability to recognize and kill cancer cells without the need for prior activation, making them a promising alternative for solid tumors.

Gene-Modified Hematopoietic Stem Cells: Hematopoietic stem cells (HSCs) are immature cells that can give rise to all blood cell types. By modifying HSCs to express a specific gene, researchers hope to use these cells to treat genetic disorders such as sickle cell anemia and beta-thalassemia.

CAR-T Cell Therapy

CAR-T (**Chimeric Antigen Receptor T-cell**) therapy is a type of cancer treatment that involves modifying a patient's T-cells, which are a type of immune system cell, to better recognize and attack cancer cells.



The general steps involved in CAR-T therapy are as follows:

Collection of T-cells: First, a patient's T-cells are collected through a process called leukapheresis, in which blood is drawn from the patient and the T-cells are separated from other blood cells using a machine.

Modification of T-cells: The collected T-cells are then genetically modified in a laboratory using a viral vector to introduce a new gene that codes for a chimeric antigen receptor (CAR) on the surface of the T-cell. The CAR is designed to recognize and bind to a specific antigen, or protein, on the surface of cancer cells.

Expansion of modified T-cells: The modified T-cells are then grown and multiplied in the laboratory over a period of several weeks to produce a large number of CAR-T cells.

Infusion of CAR-T cells: Once a sufficient number of CAR-T cells have been produced, they are infused back into the patient's bloodstream through a vein, where they can circulate and attack cancer cells throughout the body.

Monitoring and follow-up: After the CAR-T cell infusion, patients are closely monitored for potential side effects and their response to treatment. Follow-up visits and imaging scans are typically done to assess the effectiveness of the therapy and detect any potential recurrence of cancer.

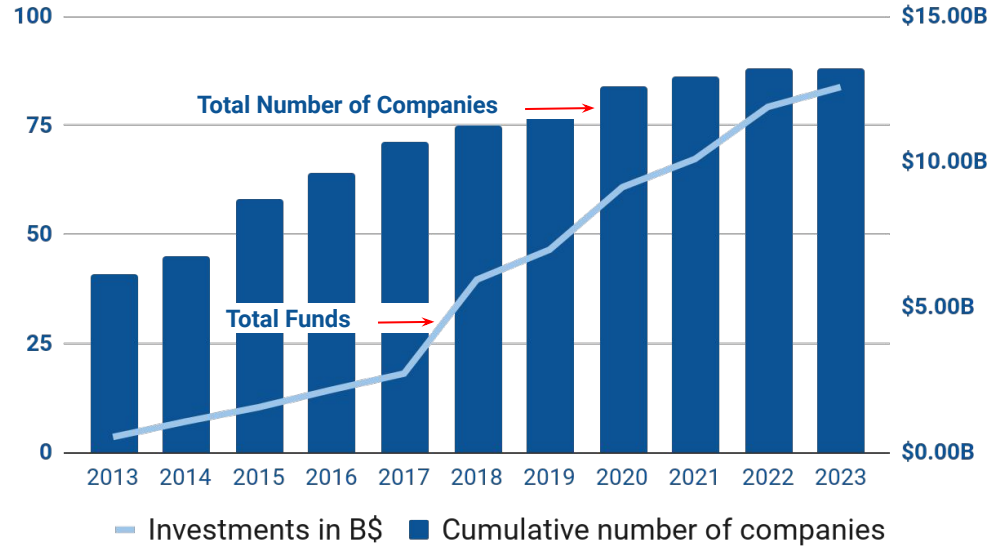
FDA Approved CAR-T Therapies

CAR T-cell therapies are approved by the US Food and Drug Administration (FDA) to treat some kinds of **lymphomas** and **leukemias,** as well as **multiple myeloma**. CAR T-cell therapy is typically used after other types of treatment have been tried. CAR-T cell therapy was approved by FDA in **2017** and since then **6 types** of CAR-T are used in medicine. Many other CAR T-cell therapies (and similar types of treatment) are now being studied in clinical trials, in the hope of treating other types of cancer as well.

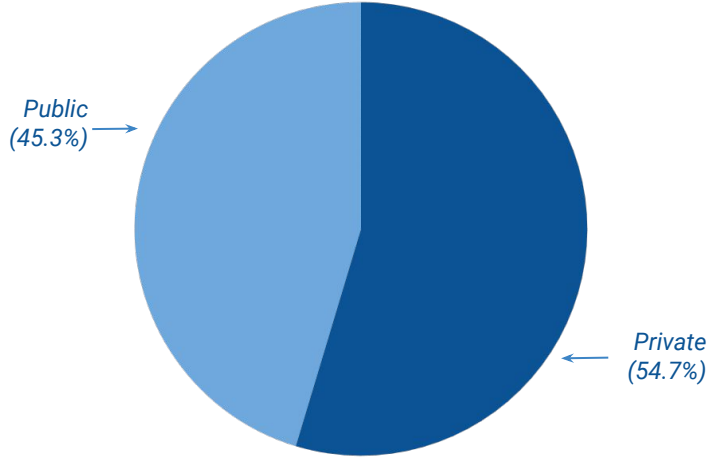
Company	Cell Therapy Name	Disease	Year of Initial US Approval
Janssen Biotech	CARVYKTI	Refractory Multiple Myeloma	2022
Celgene Corporation	ABECMA	Multiple Myeloma	2021
Bristol Myers Squibb	BREYANZI	B Cell Lymphoma	2021
Kite Pharma	TECARTUS	Mantle Cell Lymphoma	2020
Novartis	KYMRIAH	Refractory Follicular Lymphoma	2017
Kite Pharma	YESCARTA	B Cell Lymphoma	2017

Growth of CAR-T Therapy Industry

Cumulative Number of Companies and Total Value of Their Funds, 2013-2023



Distribution of Companies by IPO Status

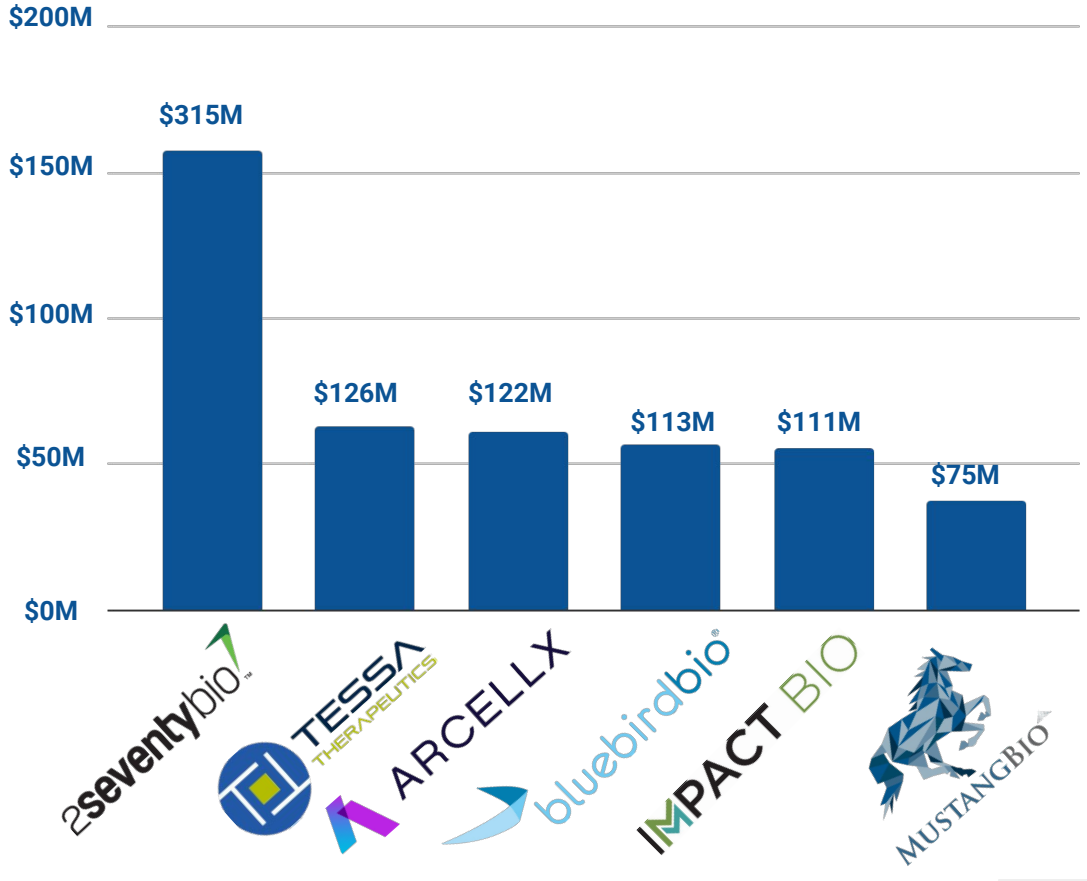


The **CAR-T therapy industry** was developed equally during the last 10 years. The share of new companies from 2010 to 2020 is **47%**.The market is valued at approximately **$12.56 billion** in Q1 2023. However, the high cost of CAR-T therapy and regulatory challenges remain significant obstacles for the industry's growth. The **cost of treatment** can be upwards of **USD 500,000 per patient**, making it inaccessible to many patients who could benefit from it. Despite these challenges, the CAR-T therapy industry is expected to continue its **growth trajectory**, offering new and improved treatment options for cancer patients.

CAR-T Therapy Investment Landscape

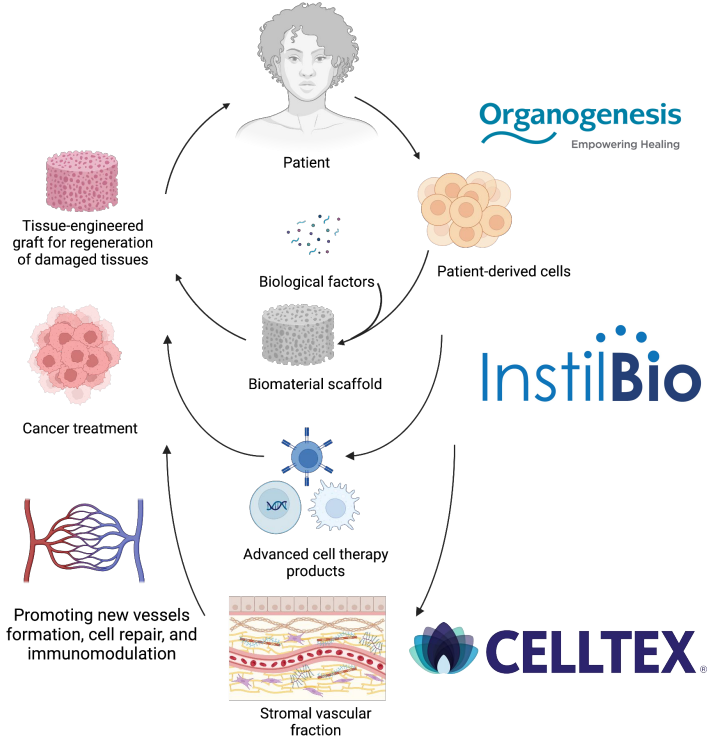
Some of the major deals in 2022-2023 included:

- **2seventhy bio** raised on IPO **$315 million** during the period February 2022 - March 2023.
- **Tessa Therapeutics** attracted 5 investors and received a funding of **$126 million** ove one Series A round in June 2022
- **Arcellx** raised on IPO **$122 million** in the United States in 2022.
- **Bluebird Bio** raised on IPO **$113 million** in January 2022.
- **ImPACT Bio** has raised **$111 million** during Series B round in January 2022.
- **Mustang Bio** obtained on IPO **$75 million** in the United States in March 2022.



Multicellular Therapies

Multicellular therapies are composed of at least two stem cell and/or non-stem cell types that are selectively expanded, rather than purified or enriched, and can mirror the composition of normal tissues. Examples include **Advanced cell therapy products(ACT), scaffold-based or -free cellular products, stromal vascular fraction, stem cell transplant, and bone marrow aspirate-derived therapies.**



Scaffold-based cellular products are 3D biocompatible tissue analogs seeded with different cell types and employ biodegradable natural or synthetic polymers. An example of commercially available, FDA-approved scaffold-free cellular products is **Epicel®**, composed of sheets of autologous keratinocytes and proliferation-arrested murine fibroblasts and indicated for deep/full burns.

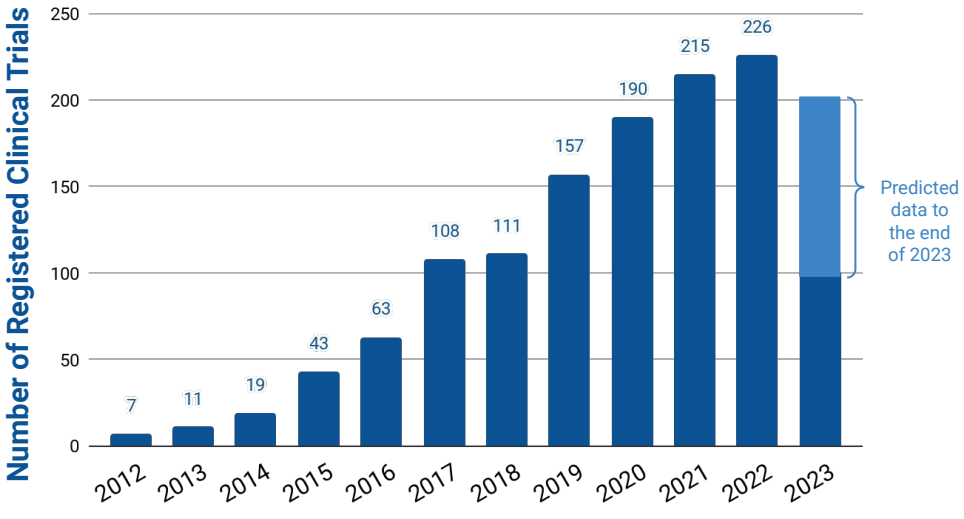
Advanced cell therapy products(ACT) include 3 types of therapy. **Tumor-infiltrating lymphocytes (TIL) treatment** is a type of therapy that consists of T and B cells that can be genetically modified to resist tumor suppression or improve their ability to target tumors. **Lymphokine-activated killer cells** are peripheral blood mononuclear cells that are activated with IL-2, primarily made up of NK cells, NKT cells, and T cells with non-specific cytotoxicity. And **cytokine-induced killer cells** are T lymphocytes that activate the immune system to recognize and eliminate tumor cells.

SVF is a mixture of stromal and vascular cells obtained from processing adipose tissue. It includes ASCs, granulocytes, monocytes, lymphocytes, pericytes, and EPCs. SVF functions through paracrine signaling and cell-cell interactions, promoting neovascularization, cell repair, and immunomodulation.

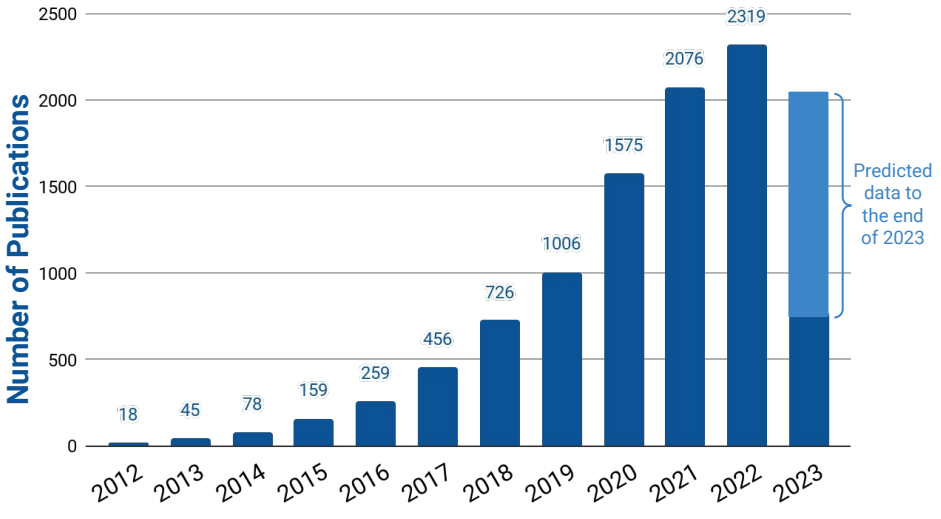
Rise of CAR-T Therapy Development

CAR-T therapy development has seen a significant rise in both **publications** and **clinical trials** in recent years. Since the approval of the first CAR-T therapy in 2017, the number of clinical trials for CAR-T has increased rapidly, with over **800 clinical trials** registered on ClinicalTrials.gov as of **2022**. This surge in clinical trials is also reflected in the number of research articles on CAR-T therapy, with over **2,000 articles** published **only in 2022**.
Overall, the rise of CAR-T therapy development reflects a growing recognition of the potential of cell-based therapies to revolutionize cancer treatment, and underscores the importance of continued investment in research in this area.

Total Clinical Interest in CAR-T Therapy



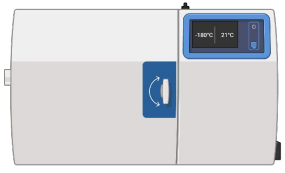
Total Scientific Interest in CAR-T Therapy



Source: https://www.ncbi.nlm.nih.gov/, https://clinicaltrials.gov/

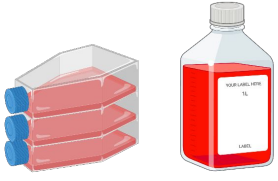
Cell Therapy Services

In addition to the **Types of Cell Therapies** mentioned previously, there are also various ancillary services that support the development and delivery of cell-based therapies. These services include:



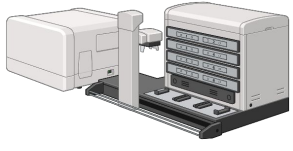
CryoCell INTERNATIONAL

goodcell®



Vitro BIOPHARMA

STEMCELL™ TECHNOLOGIES



Cell Line GENETICS

Ncardia Stem cell experts

atelerix

Biobanking: This involves the collection and storage of human tissue and cells for future use in research or therapy. Biobanks can be used to provide a source of autologous or allogeneic cells for cell-based therapies.

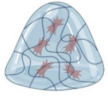
Cell processing and cultivation: This involves the isolation, expansion, and differentiation of cells for therapeutic use. This can include the use of specialized culture media, growth factors, and other technologies to promote cell growth and differentiation.

Quality control and testing: This involves the testing and characterization of cells to ensure safety, efficacy, and consistency of the final cell product. This can include assays to test for cell viability, purity, identity, and function.

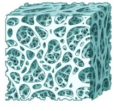
Cell storage and transportation: This involves the preservation and transportation of cells for use in therapy. This can include the use of cryopreservation technologies and specialized shipping and handling procedures to maintain cell viability and functionality.

Bioengineering

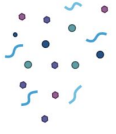
Tissue engineering is a fast-growing field in **regenerative medicine** and transplantation that is based on cultivating **stem cells**. However, the approach is not limited to cell expansion and also involves the use of **biomaterials, scaffolds, growth factors** and **bioreactors** for growing tissues, among other components. Such approach provides solutions to a wide range of health challenges, including **organ failure, tissue damage**, and **congenital defects**, by creating replacement tissues that can restore or enhance tissue function.



Biomaterials: used to provide structural support and guide the growth and development of cells. Biomaterials can be synthetic or naturally derived, and may include polymers, ceramics, or metals.



Scaffolds: These are three-dimensional structures that provide a framework for cells to grow and organize into tissue-like structures. Scaffolds can be made from a variety of materials and can be designed to mimic the mechanical and structural properties of native tissues.



Growth factors: These are signaling molecules that regulate cell growth and differentiation. Growth factors can be used to stimulate the growth and differentiation of cells into specific tissue types.

Bioreactors: These are devices that provide a controlled environment for the growth and development of cells and tissues. Bioreactors can be used to optimize the culture conditions for cells and tissues, and to promote their organization and development.

PolTiss

cormatrix
HEALING THE HEART

ACERA SURGICAL

VITRO3D

TISSUE REPAIR

AGOMAB THERAPEUTICS

APTUS BIOREACTORS
Regenerating Tissues, for Life

CELLEC BIOTEK AG

Market Size Overview

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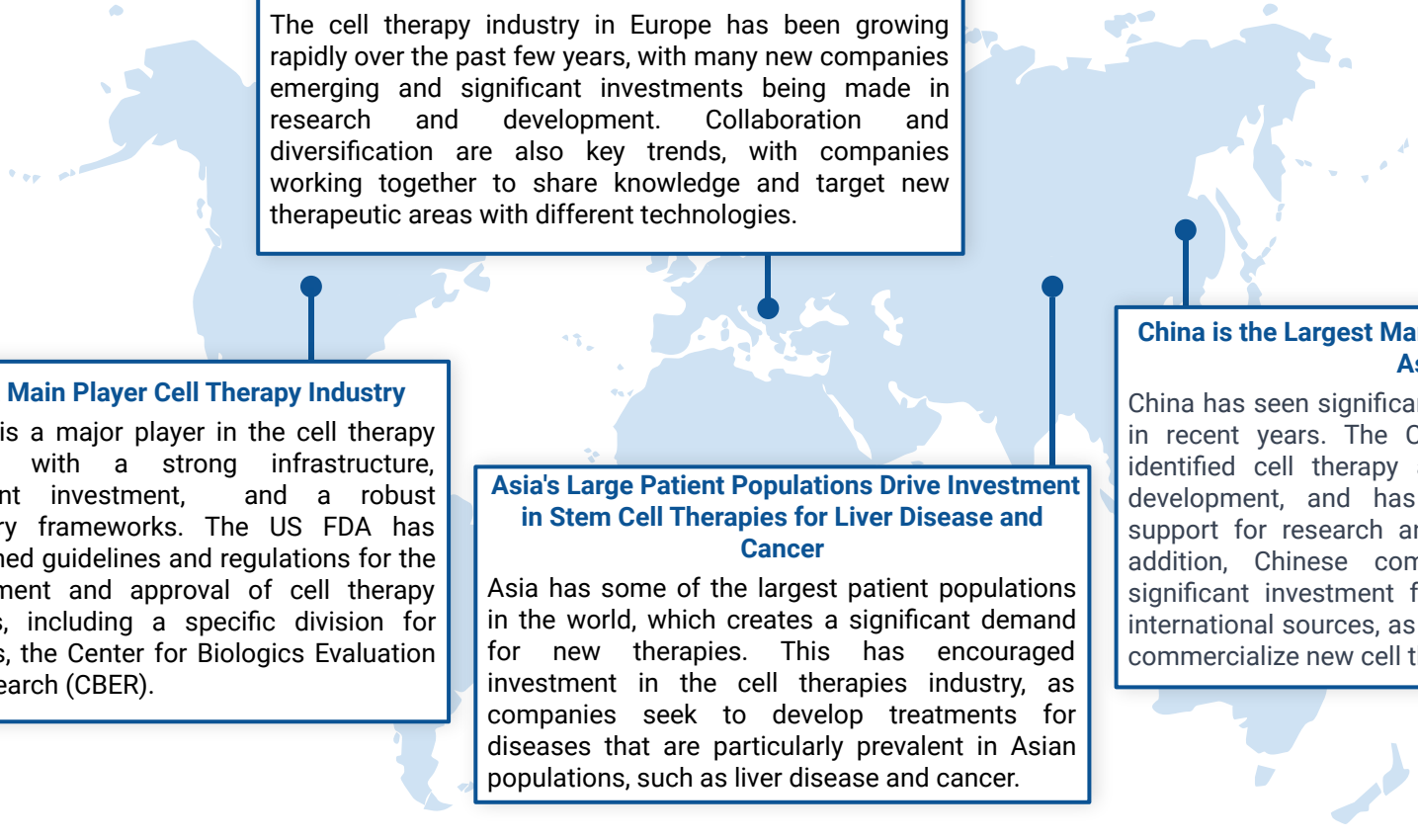
Cell Therapy Industry in the Global Context



The Cell Therapy Industry in Europe is Rapidly Growing

The cell therapy industry in Europe has been growing rapidly over the past few years, with many new companies emerging and significant investments being made in research and development. Collaboration and diversification are also key trends, with companies working together to share knowledge and target new therapeutic areas with different technologies.

US is a Main Player Cell Therapy Industry

The US is a major player in the cell therapy industry, with a strong infrastructure, significant investment, and a robust regulatory frameworks. The US FDA has established guidelines and regulations for the development and approval of cell therapy products, including a specific division for biologics, the Center for Biologics Evaluation and Research (CBER).

Asia's Large Patient Populations Drive Investment in Stem Cell Therapies for Liver Disease and Cancer

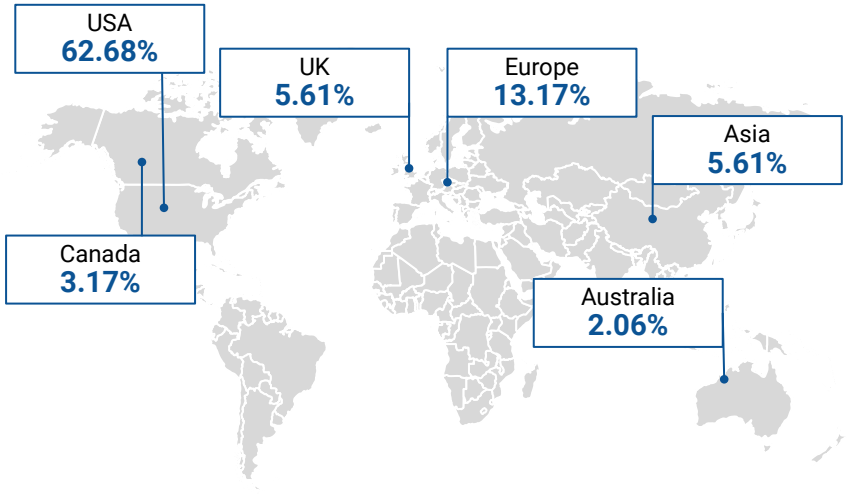
Asia has some of the largest patient populations in the world, which creates a significant demand for new therapies. This has encouraged investment in the cell therapies industry, as companies seek to develop treatments for diseases that are particularly prevalent in Asian populations, such as liver disease and cancer.

China is the Largest Market for Cell Therapies in Asia

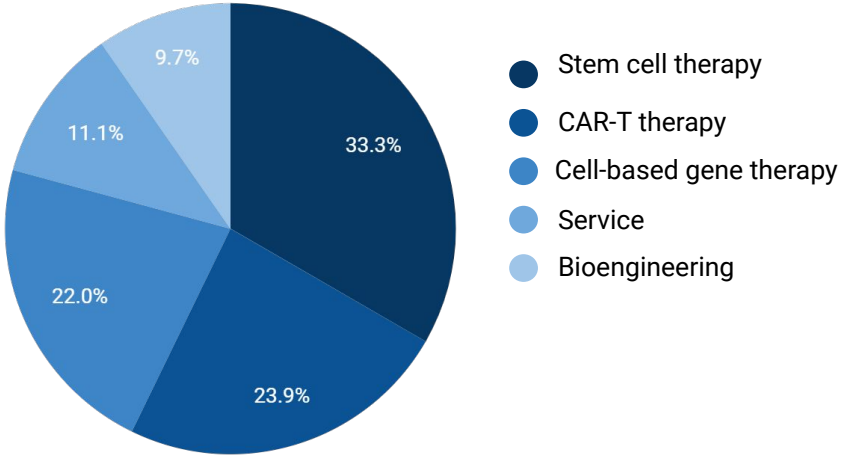
China has seen significant investment in this area in recent years. The Chinese government has identified cell therapy as a strategic area for development, and has provided funding and support for research and commercialization. In addition, Chinese companies have attracted significant investment from both domestic and international sources, as they seek to develop and commercialize new cell therapies.

Market at a Glance: Companies

Distribution of Companies by Country, %



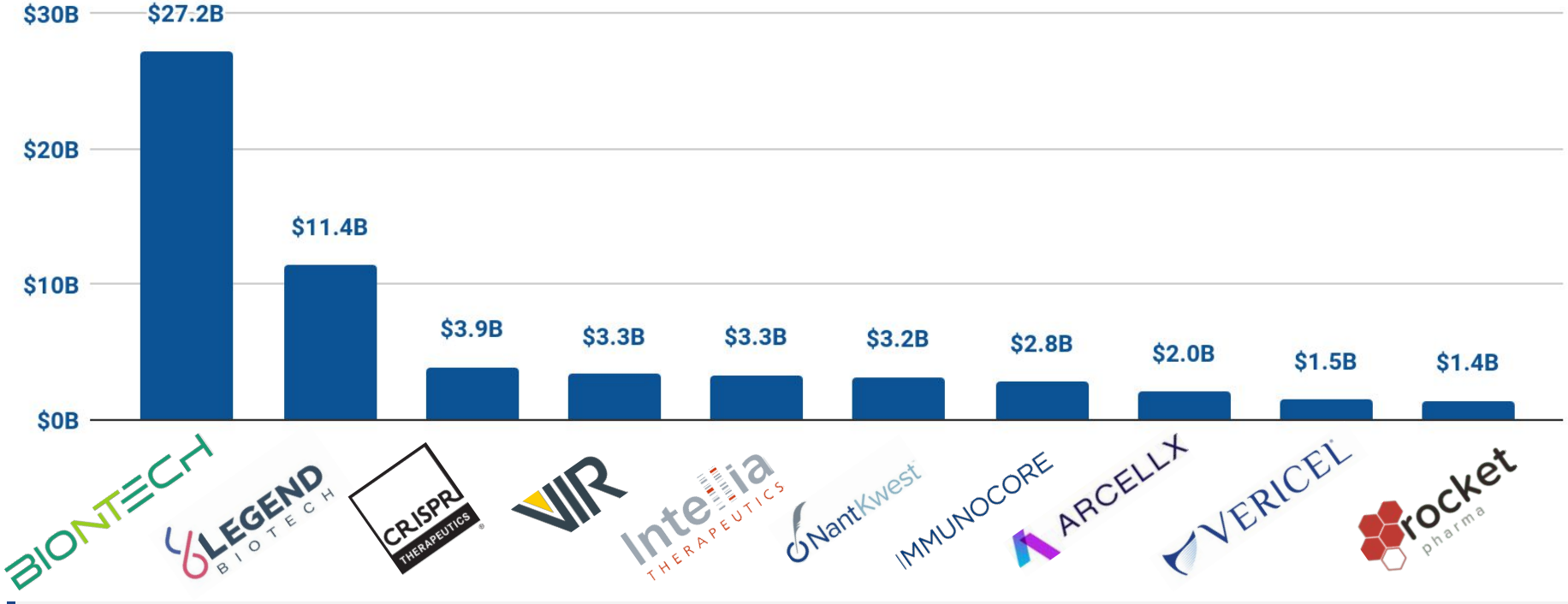
Distribution of Companies by Category, %



The **vast majority** of companies of Cell Therapies Industry is located in the **United States** and accounts for **62.68%** of the whole range of analysed companies. The United States, region is followed by Europe with the total companies amounting to **13.17%** of all companies in both regions.

The main domains in which companies are being conducted are **Stem cell therapy, CAR-T therapy, Cell-based gene therapy, Service,** and **Bioengineering** which account for **33.3%**, **23.9%**, **22%, 11.1%** and **9.7%** of all companies, **respectively**.

Top-10 Cell Therapy Publicly Traded Pharma Companies by Market Capitalization



The chart presents the **Top-10 Cell Therapy** public companies arranged by market capitalization as of end of April 2023. **BioNTech**, German biotechnology company based in Mainz that develops and manufactures active immunotherapies for patient-specific approaches to the treatment of diseases holds the first place with **$27.2B** of market capitalization which keeps rising.

Investment Landscape

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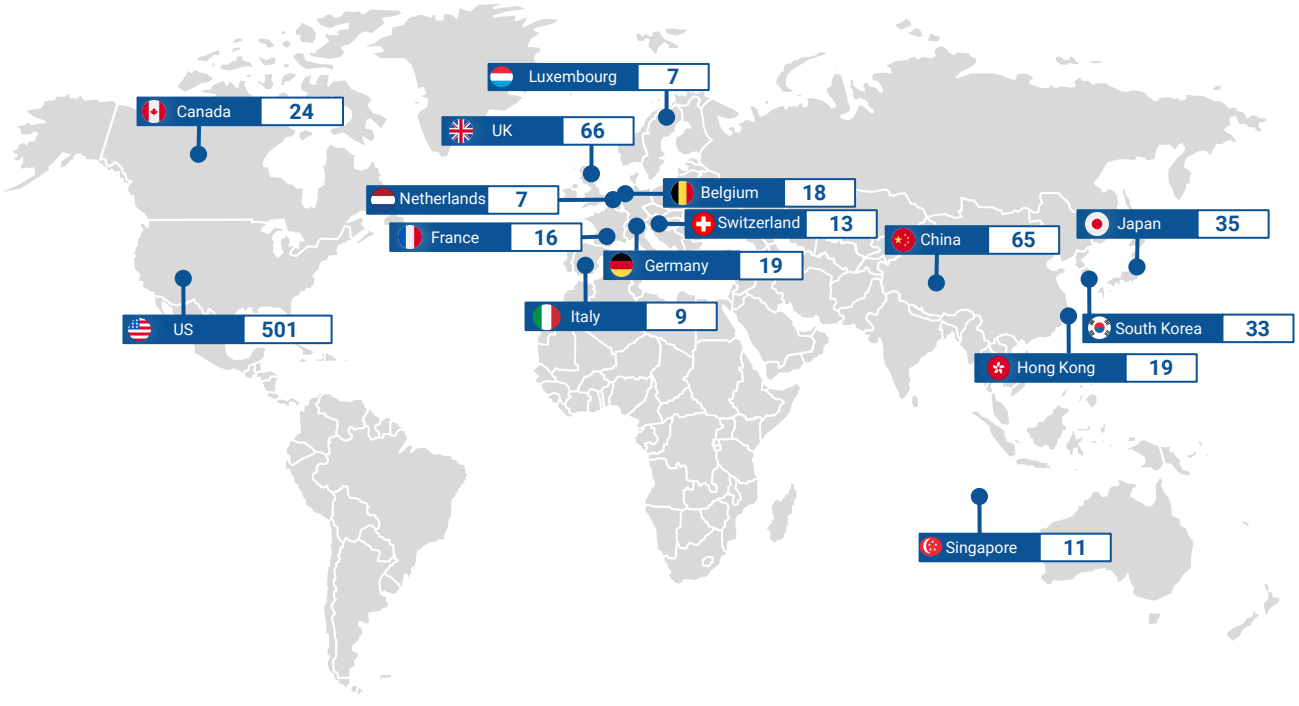
Market at a Glance: Investors



More than half of the investors in **preclinical and clinical optimisation companies** are from the **United States** (around **55%**). **Some** of investors **(7%)** are located in the UK; 7% of the investors are located in **China**; 4% – in **South Korea** and **Japan. Germany, France, Belgium,** and **Hong Kong** each host 2% of all investors. Overall, the **first 10 countries** by number of investors locate **82%** of all investors.

Top 20 Investors

INVESTORS	COMPANIES	INVESTED IN
Alexandria Venture Investments	15	Jasper Therapeutics, Aspen Neuroscience, Ambys Medicines, egenesis, Obsidian Therapeutics, Sonoma Biotherapeutics, Precision Biosciences, Neurona Therapeutics, Wugen, Cartography Biosciences, Kytopen, Garuda Therapeutics, TCR2 Therapeutics, VOR, Nohla Therapeutics
RA Capital Management	15	Orchard Therapeutics, VOR, artiva, be Biopharma, Legend Biotech, Achilles Therapeutics, Bellicum, TScan Therapeutics, Athersys, abcuro, Precision Biosciences, nkarta, Obsidian Therapeutics, Century Therapeutics, bluebirdbio
OrbiMed	14	cogent Biosciences, Iovance Biotherapeutics, magenta Therapeutics, Adicet Bio, Gracell 亘喜生物, Harpoon Therapeutics, Athersys, Aspen Neuroscience, Garuda Therapeutics, Impact Bio, Century Therapeutics, Intellia Therapeutics, Achilles Therapeutics, LifeCell
Fidelity Management and Research Company	>12	Allogene Therapeutics, BioNTech, allo VIR, Intellia Therapeutics, Nohla Therapeutics, Sonoma Biotherapeutics, VOR, Century Therapeutics, Neon Therapeutics, Wugen, Poseida Therapeutics, elevatebio
ARCH Venture Partners	11	Aspen Neuroscience, Sana Biotechnology, egenesis, CERO Therapeutics, Fate Therapeutics, Sonoma Biotherapeutics, Nohla Therapeutics, be Biopharma, 药明巨诺 JW Therapeutics, bluebirdbio, VIR
EcoR1 Capital	11	2seventybio, Abeona Therapeutics, elevatebio, allo VIR, immatics, neogene, Atara Bio, artiva, magenta Therapeutics, Intellia Therapeutics

Top 20 Investors

INVESTORS	COMPANIES	INVESTED IN
California Institute for Regenerative Medicine	10	
Invus	10	
Samsara BioCapital	10	
Surveyor Capital	10	
Google Ventures	9	
National Institutes of Health	9	
Redmile Group	8	
RTW Investments	8	

Top 20 Investors

INVESTORS	COMPANIES	INVESTED IN
Innovate UK	8	
Casdin Capital	7	
Cormorant Asset Management	7	
Janus Henderson Investors	7	
Logos Capital	7	
Perceptive Advisors	7	

Top 20 Investors

California

EcoR1 Capital
San Francisco, California, United States

California Institute for Regenerative Medicine
San Francisco, California, United States

Alexandria Venture Investments
Pasadena, California, United States

Google Ventures
Mountain View, California, United States

Redmile Group
San Francisco, California, United States

Logos Capital
San Francisco, California, United States

New York

OrbiMed
New York, New York, United States

Invus
New York, New York, United States

RTW Investments
New York, New York, United States

Casdin Capital
New York, New York, United States

Perceptive Advisors
New York, New York, United States

Samsara BioCapital
Palo Alto, California, United States

Massachusetts

RA Capital Management
Boston, Massachusetts, United States

Fidelity Management and Research Company
Boston, Massachusetts, United States

Cormorant Asset Management
Boston, Massachusetts, United States

Illinois

ARCH Venture Partners
Chicago, Illinois, United States

Surveyor Capital
Chicago, Illinois, United States

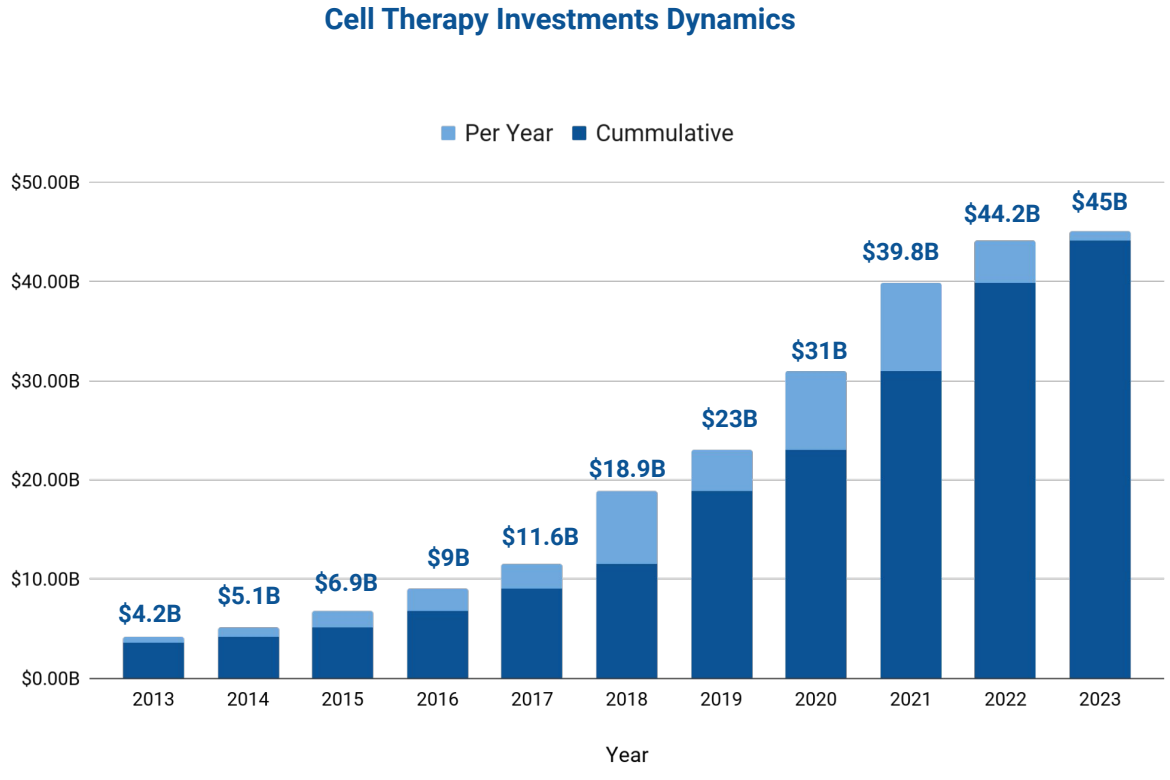
Maryland

National Institutes of Health
Bethesda, Maryland, United States

Janus Henderson Investors
London, England, United Kingdom

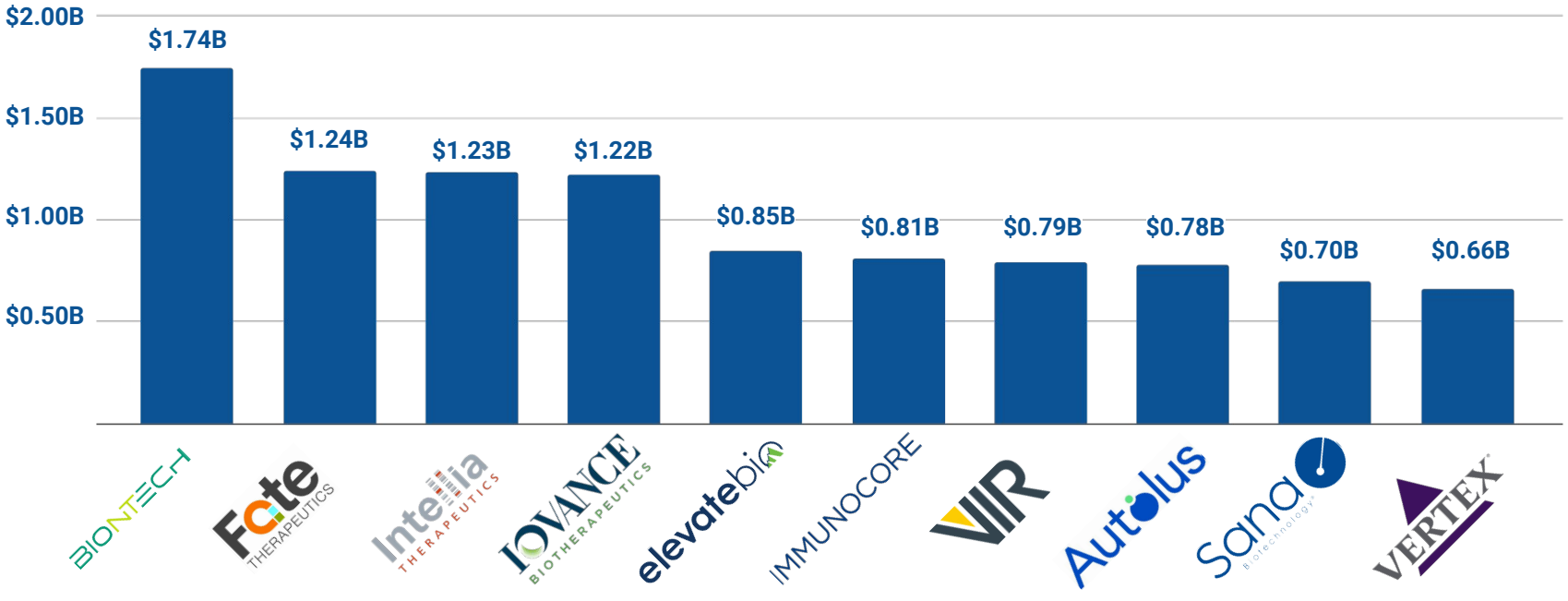
Innovate UK
Swindon, Wiltshire, United Kingdom

Dynamics of Investments in Cell Therapy Industry



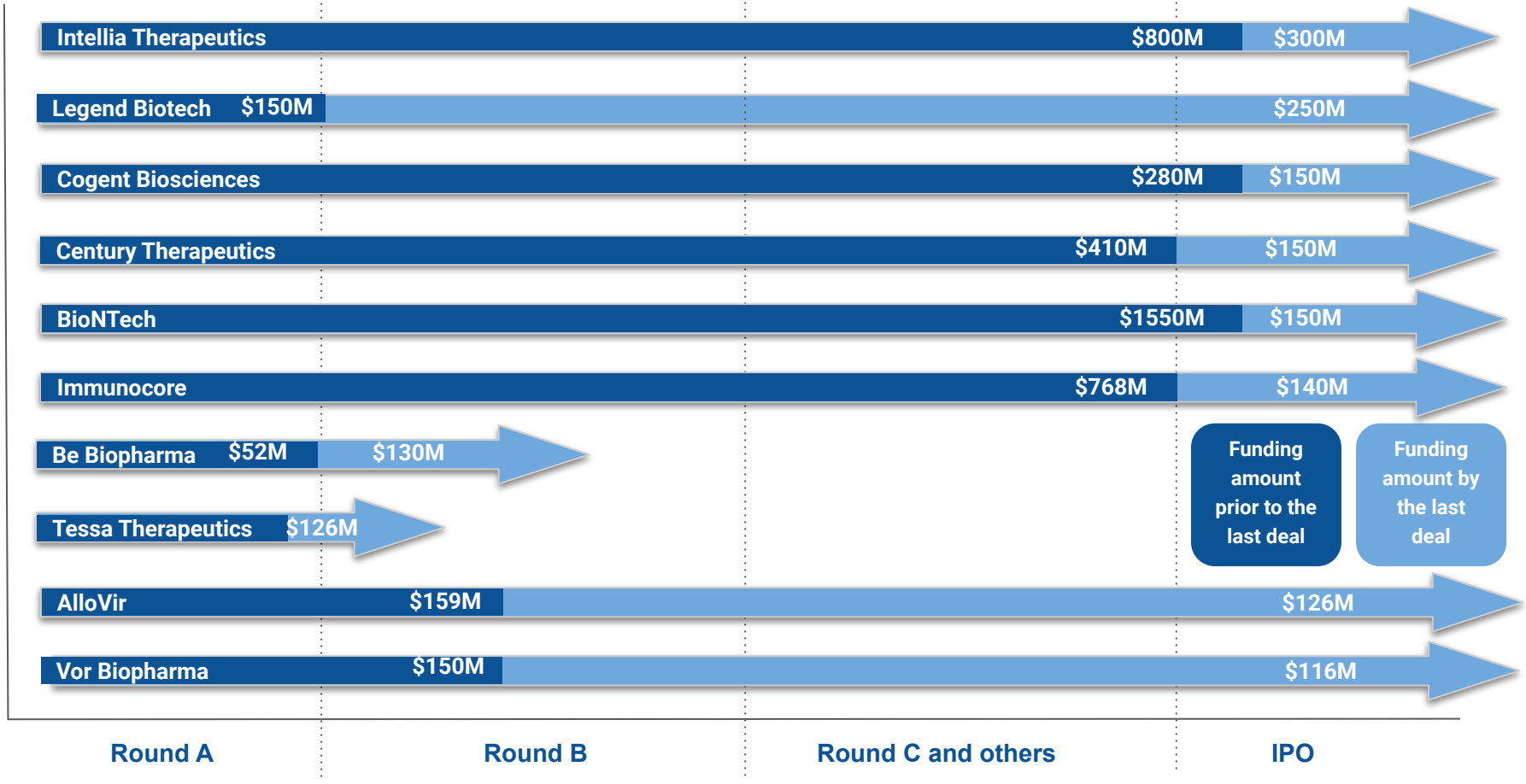
There has been a substantial increase in the amount of capital invested in Cell Therapy companies **since 2017.** During the last 6 years, the annual amount of investments in **400** companies has increased **4 times** (to **$45B** in total as of April 2023). The most rapid growth was in 2021, when the year investment was $8.81B. We can suggest, that COVID-19 pandemia was the catalizator of this rapid growth. But because of the global economic recession, the investments in Cell Therapy development companies in 2022 did not have these high growing increase in several times as in previous years ($4.36B in 2022 compared to $8.81B in 2021). On the April 2023, the total investments in Cell Therapy development companies are **$45B.**

Top 10 Cell therapy Industry Companies by Total Investments in Q1 2023

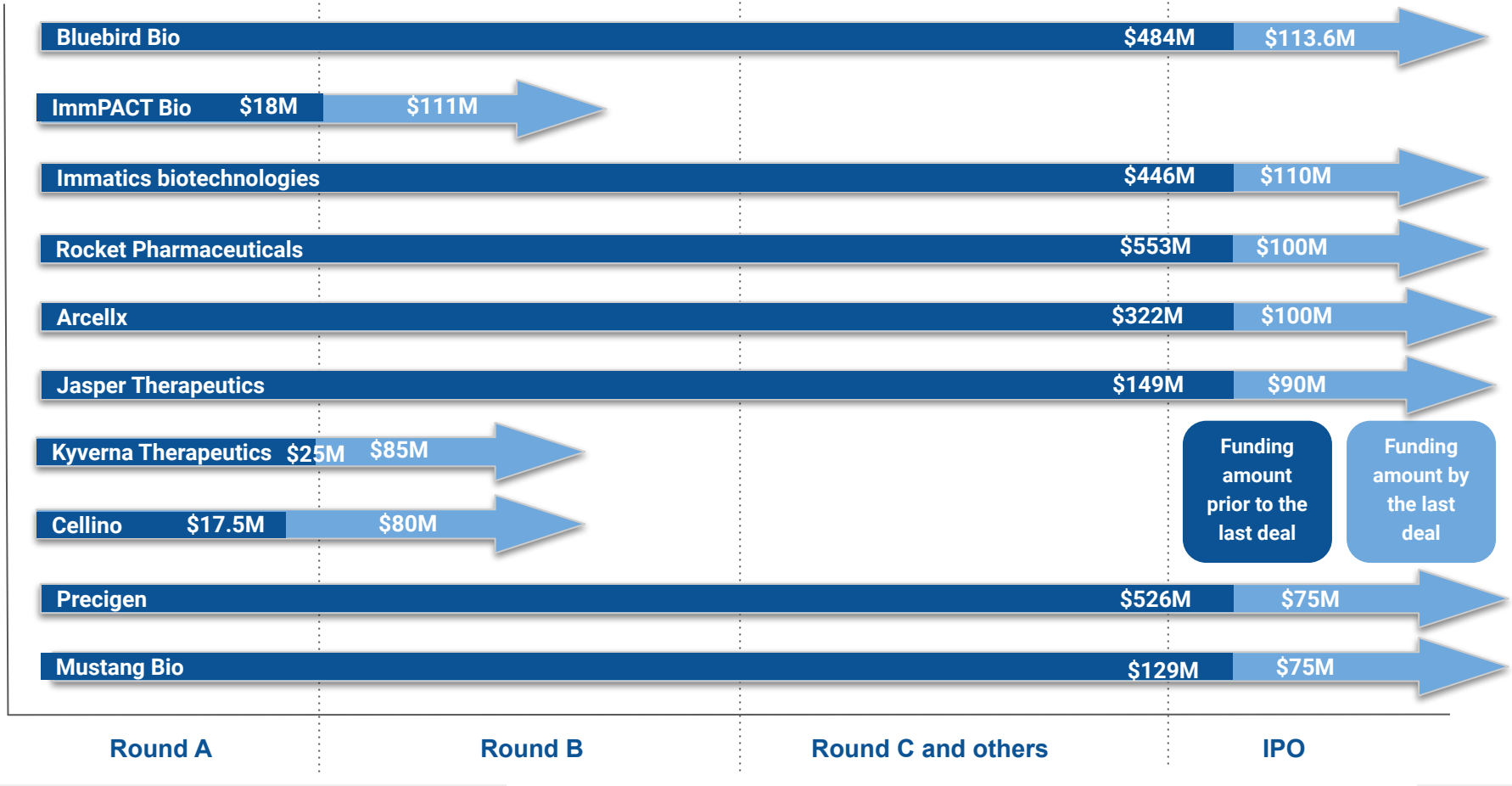


The chart shows the top 10 AI-driven drug discovery companies sorted by the **total funding** raised by the end of Q1 2023. **BioNTech,** a biopharmaceutical company pioneering the development of individualized therapies for cancer and other diseases is now at the top of the list. The company has the total funding raised to **$1.74B**. **Fate Therapeutics**, a s a stem cell and developmental biology research company using biological mechanisms to develop stem cell therapeutics could finance **$1.24B** in capital market. Other companies from the chart researched from **$0.66B** to **$1.23B** in total funding in Q1 2023.

Leading Companies by Amount and Stage of Funding Raised in 2022-2023



Leading Companies by Amount and Stage of Funding Raised in 2022-2023



Notable Use Cases of Cell Therapy Applications in Pharmaceutical R&D and Medicine

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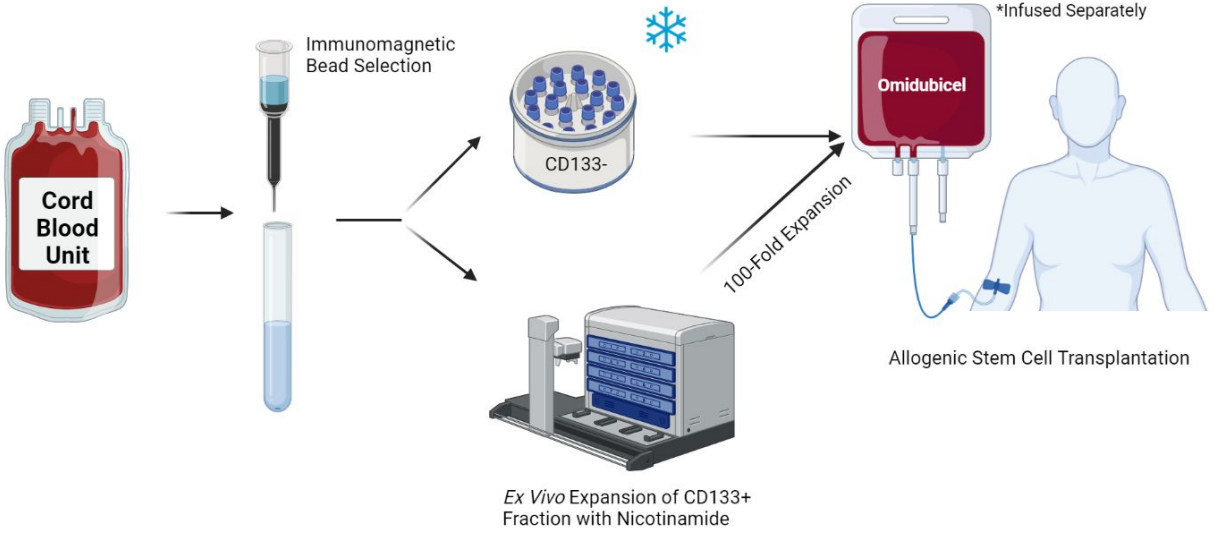
AGING ANALYTICS AGENCY

Cell Therapy Application Use Cases: Gamida Cell

Gamida Cell is a cell therapy pioneer working to turn cells into powerful therapeutics. The company's research and development efforts have produced potentially **curative cell therapy candidates** for patients with blood cancers. In Q1 2023, **The U.S. Food and Drug Administration** approved **Gamida Cell's** cell therapy **Omisirge** (omidubicel-onlv), a substantially modified allogeneic (donor) cord blood-based cell therapy to quicken the recovery of neutrophils (a subset of white blood cells) in the body and reduce the risk of infection.

Omisirge is a **nicotinamide modified** allogeneic hematopoietic progenitor cell therapy derived from cord blood indicated for use in adults and pediatric patients 12 years and older with hematologic malignancies who are planned for umbilical cord blood transplantation. This proprietary nicotinamide-based, or **NAM-based, expansion technology** enables donor cells to grow while maintaining functionality. Omidubicel consists of NAM-expanded hematopoietic stem cells and differentiated immune cells, including T cells. The final cell therapy product may be cryopreserved until the patient is ready to begin the transplant, when it is thawed and infused.

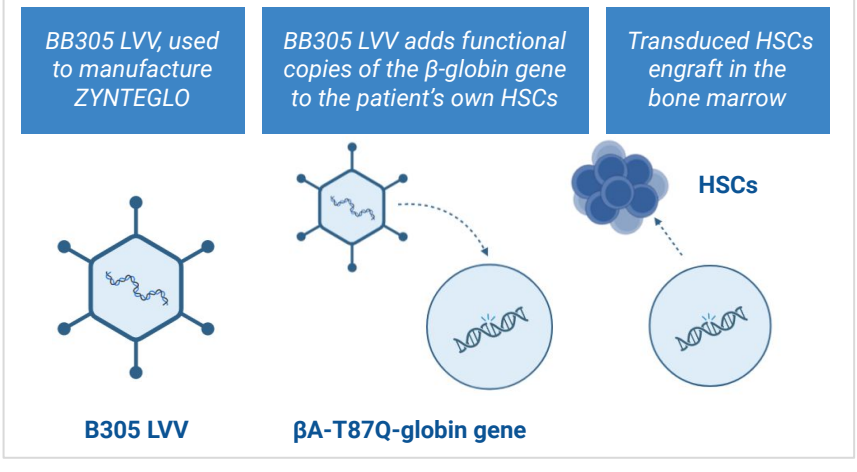
Omidubicel At A Glance



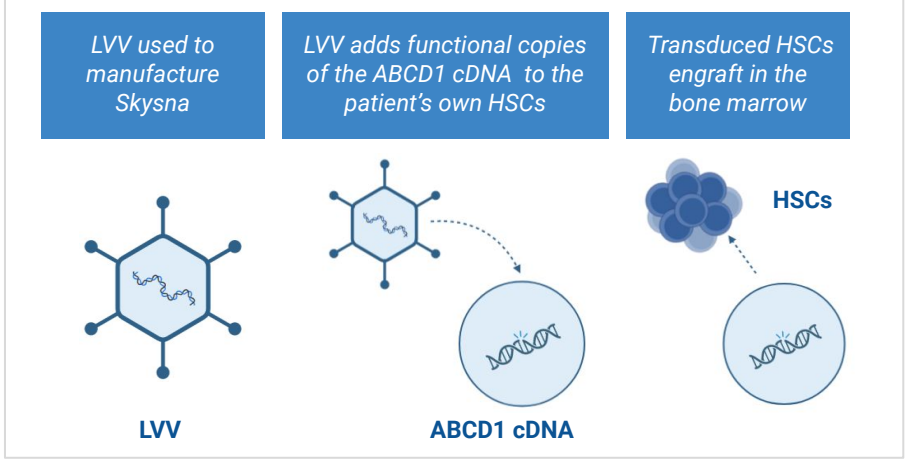
Cell Therapy Application Use Cases: bluebird bio

bluebird bio specializes in **cell-based gene therapy** with four primary diseases in its crosshairs: **Cerebral Adrenoleukodystrophy (CALD)**, **Sickle Cell Disease (SCD)**, and **Transfusion-Dependent Beta-Thalassemia (TDT)**. bluebird bio is focused on **gene addition**. In gene addition therapies, functional copies of a gene are delivered to a patient's **stem cells** using a delivery system called a "vector." bluebird bio uses **lentiviral vectors (LVVs)** because they have unique properties that are well-suited to treating a range of severe genetic diseases. bluebird.bio hqs two therapies approved by FDA: **Zynteglo** and **Skysona**.

Zynteglo works by adding functional copies of a modified form of the beta-globin gene (βA-T87Q-globin gene) into a patient's own **hematopoietic stem cells** (**HSCs**) to allow them to make normal to near normal levels of total hemoglobin without regular RBC transfusions



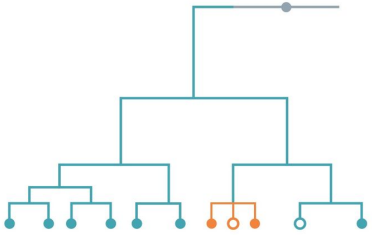
Skysona is the first FDA-approved therapy shown to slow the progression of **CALD**. Skysona adds functional copies of the ***ABCD1*** cDNA into patients' **hematopoietic stem cells** (HSCs) through transduction of autologous CD34+ cells with LVV.



Cell Therapy Application Use Cases: MOGRIFY

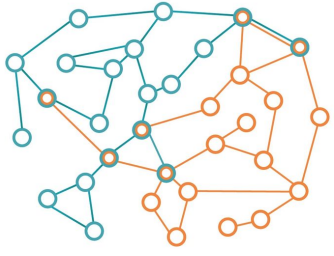
Mogrify has developed a proprietary suite of platform technologies that utilize a systematic big-data approach to direct cellular reprogramming and the maintenance of cell identity. The platforms, **MOGRIFY®** and **epiMOGRIFY®** deploy next-generation sequencing, gene regulatory and epigenetic network data to enable the prediction of the transcription factors and growth factors required **to produce any target human cell type** from any source human cell type.

MOGRIFY®



- Source cell type
- Target cell type
- Background selected for analysis
- Excluded for being highly related
- Excluded for being too distant

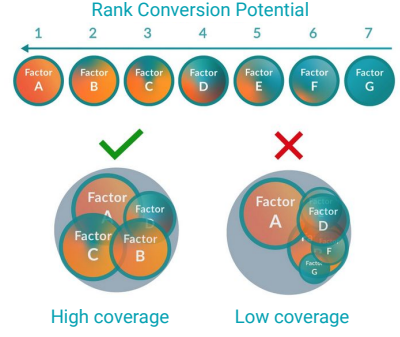
Step 1 Calculation of contextual gene expression



- Gene expressed in source cell type
- Gene expressed in target cell type
- Gene expressed in both cell types

Step 2 Identification and scoring of genes that require change from source to target cell

Step 3 Building local regulatory network of influence around each transcription factor (TF)



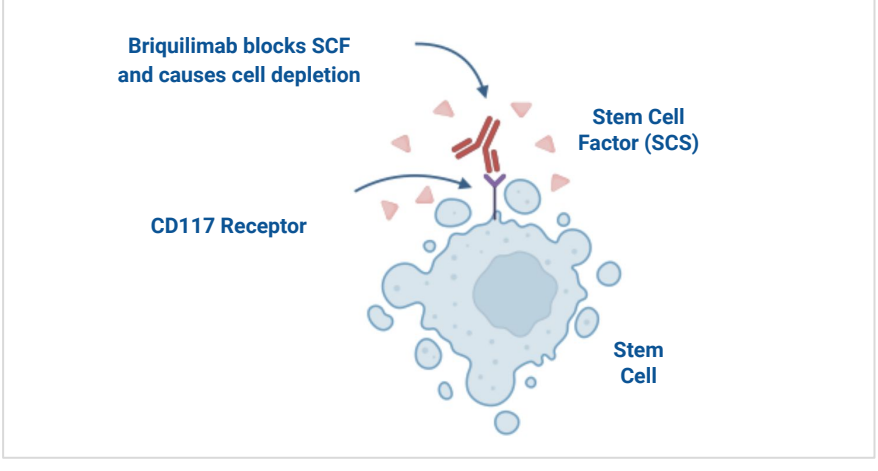
- Set if required genes for target cell type
- Set if required genes for TF

Step 4 Scoring of the conversion potential of each TF and optimization of network coverage

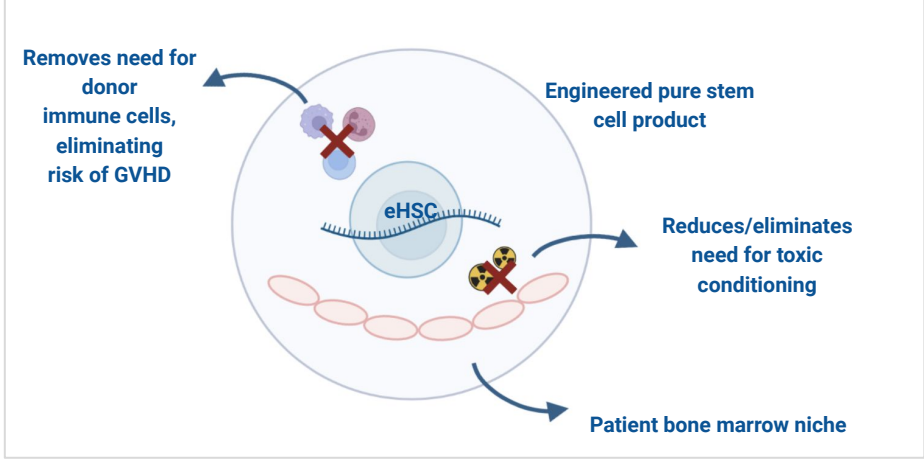
Cell Therapy Application Use Cases: Jasper Therapeutics

Jasper Therapeutics focuses on developing novel stem cell transplantation approaches with higher proliferation and survival rate, as well as reduced immune response and improved engraftment. Their main drug candidate **Briquilimab** is an antibody used to assist **hematopoietic stem cells (HSCs)** transplants for **severe combined immunodeficiency (SCID), sickle cell disease and myelodysplastic syndromes (MDS)**. The company also started developing revolutionary **mRNA Stem Cell Grafts** to overcome key limitations of allogeneic donor and autologous gene-edited **stem cell transplants**.

Briquilimab is an antibody that functionally blocks the interaction of the c-Kit receptor from its ligand, stem cell factor (SCF). For **stem cell transplant**, briquilimab **clears the niche** in the bone marrow for donor or gene-corrected hematopoietic stem cells to engraft and does not carry a toxic payload or recruit immune cells.



mRNA Stem Cell Grafts are based on **reprogramming mRNA or DNA** in stem cells to transiently modify donor or gene-edited stems to have a proliferative and / or survival advantage over a patient's endogenous stem cells, permitting higher levels of engraftment without the need for toxic conditioning of the patient.



Selected Cell Therapy Pharma Collaborations in 2022-2023

Jan 2022

Allogene Therapeutics announced exclusive collaboration with **Antion Biosciences** for Multiplex **miCAR™ Technology**

Apr 2022

Artiva Biotherapeutics announced agreement with **Merck** to Evaluate Combinations of **NK Cells** with Tri-Specific NK-Cell Engagers

Jul 2022

Mogrify and **Astellas** announced collaboration to conduct research on in vivo **regenerative medicine** approaches to address sensorineural hearing loss

Aug 2022

Poseida Therapeutics collaborated with **Roche** for development allogeneic CAR-T cell therapies for hematologic malignancies

Sep 2022

Arsenal Biosciences partnered with **Genentech** to identify features of successful **T-Cell therapies** for oncology

Oct 2022

Bristol Myers Squibb has inked a multiyear extension to its collaboration with **Obsidian Therapeutics,** securing itself the exclusive option to license cell therapie

Selected Cell Therapy Pharma Collaborations in 2022-2023

Kite and **Arcellx** closed agreement to co-develop late-stage clinical **CART-ddBCMA** in **multiple myeloma**

Kite, A GILEAD Company

ARCELLX

Thermo Fisher Scientific and **Arsenal Biosciences** have updated their strategic collaboration to further the development of **autologous T cells** for the treatment of cancer

ThermoFisher SCIENTIFIC

LyGenesis and **Imagine Pharma** partner for type 1 diabetes therapies development

LyGenesis

imagine PHARMA

Jan 2023 | Feb 2023 | Mar 2023 | Mar 2023 | Apr 2023 | Apr 2023

Boehringer Ingelheim and **3T Biosciences** collaborated to discover and develop next-generation **cancer TCR therapies**

Boehringer Ingelheim

3T BIOSCIENCES

Regeneron Pharmaceuticals and **Sonoma BioTherapeutics** partnered to develop anovel regulatory T **cell (Treg) therapies** for autoimmune diseases

REGENERON

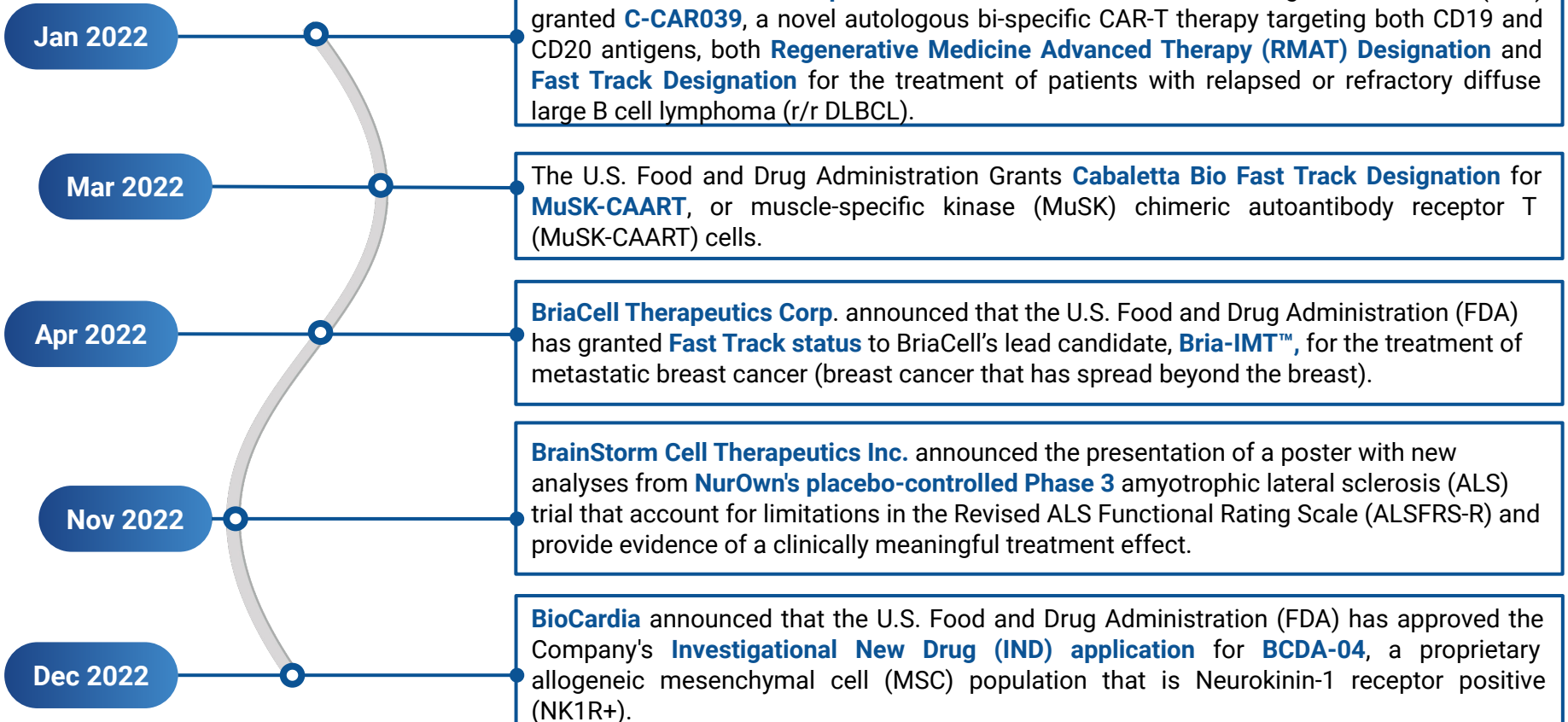
SONOMA BIOTHERAPEUTICS

Aspect Biosystems and **Novo Nordisk** entered partnership to develop **bioprinted tissue therapeutics** for diabetes and obesity

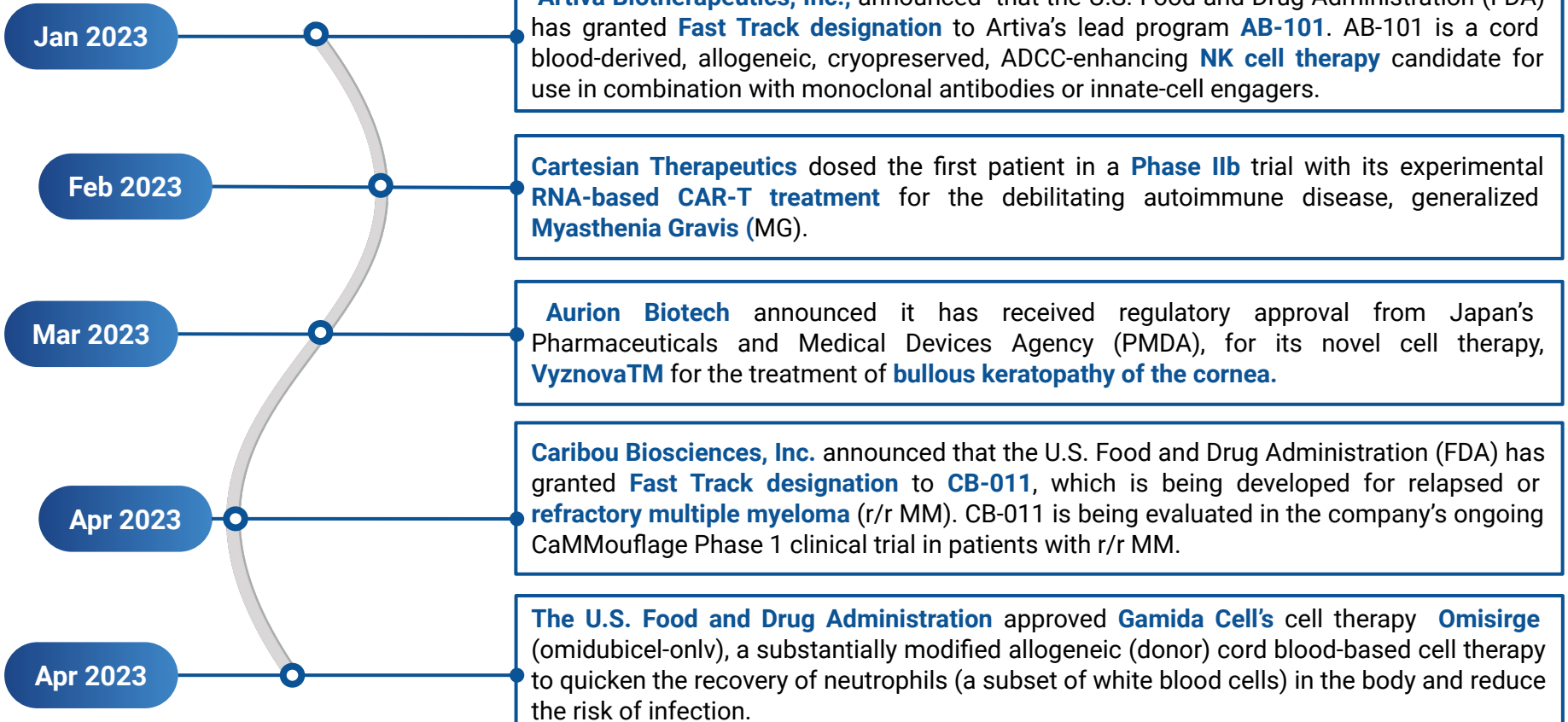
Aspect biosystems

novo nordisk®

Clinical Trials News in 2022



Clinical Trials News in Q1 2023



Key Takeaways

Cell Therapy is a **rapidly growing industry** that has the potential to revolutionize healthcare by providing new treatments for diseases that were previously untreatable. There are currently **over 1,000** registered cell therapy **clinical trials** underway worldwide, and the growing number of clinical trials in cell therapy reflects the increasing interest and investment in this field, driven by the potential to develop new treatments for a wide range of diseases.

CAR-T cell therapy is one of the most promising areas of cell therapy, with FDA-approved treatments for certain types of blood cancer and ongoing clinical trials for other types of cancer. The market is valued at approximately **$12.56 billion** in Q1 2023. However, the high cost of CAR-T therapy and regulatory challenges remain significant obstacles for the industry's growth. Despite these challenges, the CAR-T therapy industry is expected to continue its **growth trajectory**, offering new and improved treatment options for cancer patients.

In Q1 2023, **The U.S. Food and Drug Administration** approved new stem cell therapy. **Gamida Cell's** cell therapy **Omisirge** (omidubicel-onlv)is a substantially modified allogeneic (donor) cord blood-based cell therapy to quicken the recovery of neutrophils (a subset of white blood cells) in the body and reduce the risk of infection.

The total market value of companies that use or develop new cell therapies is **$78B** as of end of April 2023 which includes more than 125 companies that reached IPO and their number continue to rise. Top 3 companies by market capitalization are **BioNTech $28.5B**, **Legend Biotech $11.51B and CRISPR Therapeutics $3.88B .**

Overview of Deep Pharma Intelligence

DEEP PHARMA INTELLIGENCE

AGING ANALYTICS AGENCY

Deep Pharma Intelligence — New Era in Pharma Analytics

Deep Pharma Intelligence (DPI), an analytical subsidiary of Deep Knowledge Group, is a highly specialised think tank in the area of BioTech innovation profiling, market intelligence, and BioTech development advisory. The company is dedicated to producing powerful data mining and visualisation systems, interactive analytics tools, and industry reports, offering deep technical insights, market intelligence, and strategic guidance in the high growth and significant opportunity areas.

DPI is Focusing on Three Key Activities:

Conducting Market Intelligence

Producing regular **open-access** and **proprietary reports** on the emerging topics and trends in the pharmaceutical and healthcare industries. All reports are supported by our back-end analytics systems and tools that allow to receive fresh insights and updates about opportunities and risks.

Creating Big Data Analytical Dashboards

Building a comprehensive **Big Data Analytical Dashboard** (SaaS) as a one-stop-platform for all market and business intelligence operations our customers may need, including profiling thousands of companies, market signals and trends based on tens of millions of constantly updated data points.

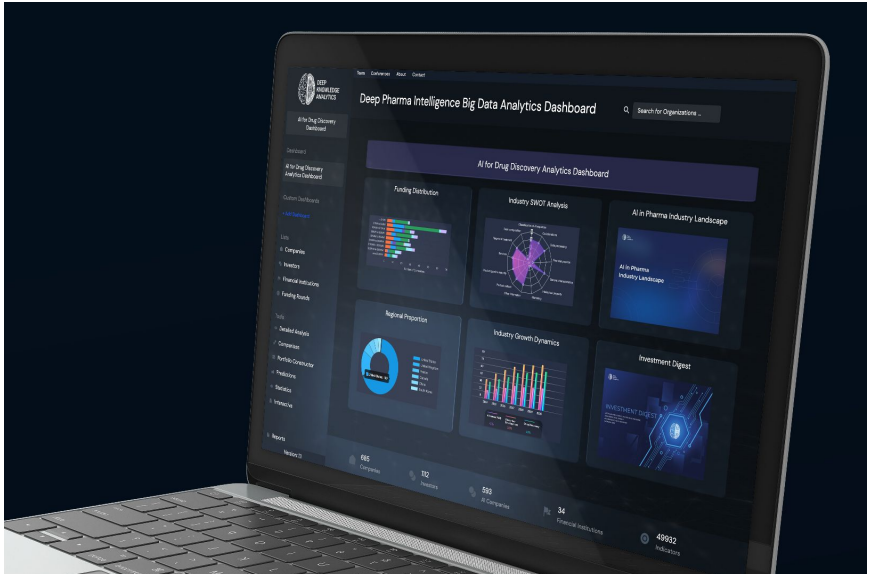
Producing Scientific Content

DPI provides a **full-cycle development of articles, scientific journals, and books**. We are ready to develop a detailed Requirement Specifications document, including layout of the journal, fully designed brand book, with example templates for each chapter.

AI in Drug Discovery Analytical Dashboard

AI in Drug Discovery Analytical Dashboard is a fundamental tool for strategic insights, opportunity evaluation, competitor profiling, and other purposes relevant to Pharma and BioTech decision-makers, life science investors, consulting companies, and regulatory agencies.

700	Companies
1,800	Investors
265	R&D Collaborations
3200	Clinical Trials
170	Parameters of Automated SWOT Analysis



Market Intelligence Focus

Automated SWOT Analysis	Stock Price Forecasting	Interactive Chart Builder
Automated Competitive Analysis	Financial Portfolio Constructor	Matching Tool for Investors

Comprehensive Market Intelligence

Deep Pharma Intelligence's proprietary services include **custom consulting projects based on the specific customer needs,** as well as a collection of preproduced 'ready-to-use' proprietary reports, developed by our research team and covering general trends and specific action ideas and strategy insights related to the most promising business prospects (e.g. new technologies, BioTech start-ups), M&A prospects (e.g. pipeline development targets), and strategic growth ideas (trends profiling, industry overviews, etc.).

Selected Open Access Reports

World's AI for Drug Development Landscape: Focus on Asia gives a complete picture of the industry environment in terms of AI usage in drug discovery, clinical research, and other elements of pharmaceutical research and development with the focus on Asia.

Artificial Intelligence in Nuclear Medicine Q1 2023 report aims to provide a comprehensive overview of the current state of nuclear medicine markerand research. This overview highlights the trends and insights in a form of informative mind maps and infographics.

Artificial Intelligence for Drug Discovery Landscape Overview Q1 2023 offers a thorough analysis of the market environment with regard to the use of AI in drug development, clinical research, and other areas of pharmaceutical R&D.

Business Consulting Services

Deep Pharma Intelligence offers a comprehensive range of **consulting services**, including **market and competitor research, technology scouting and due diligence, investment landscape profiling, and comprehensive analytics support for investment decision-making.**

Investment Landscape Profiling

Identifying investment trends in the pharma, BioTech, medicine, healthcare, drug development technological space, investments risk profiling based on risk tolerance, risk capacity, and risk requirements.

Technology Scouting and Due Diligence

Identifying, locating, and evaluating existing or developing technologies, products, services, and emerging trends. The service includes business, science and technology, intellectual property (IP) profiling, and potential assessment.

Market Research

Thorough market assessment within a specific industry in the field of pharma, BioTech, medicine, healthcare, drug development, AI, and others.

Competitor Research

Competitive analysis of companies, technologies, technological sectors, etc. Competitive analysis includes SWOT analysis and competitive profiling.

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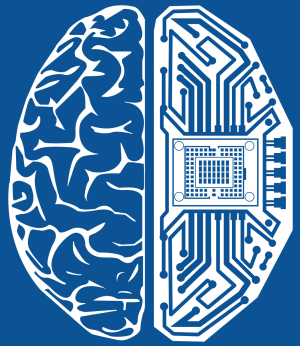

Link to the Report: www.deep-pharma.tech/ai-in-dd-q3-2022-subscribe

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