Alliance for Regenerative Medicine

A COMMUNITY FOCUSED ON BUILDING THE FUTURE OF MEDICINE

The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments.

ARM is the global voice of the cell and gene therapy sector representing...

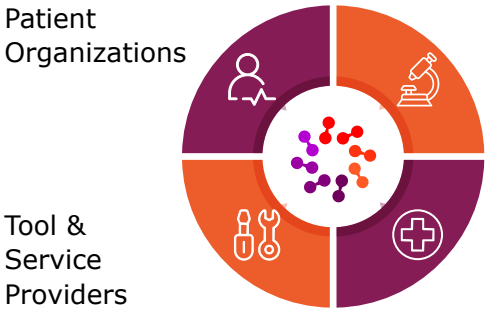
475+ members in **25** countries

See who is part of the alliance: alliancerm.org/members

What we do:

- Convene the cell and gene therapy sector
- Provide data & analysis on the sector's progress
- Engage with key stakeholders including regulators & policymakers
- Enable the development of advanced therapies
- Modernize healthcare systems to facilitate patient access

Our members include:



The future of medicine

Jimi Olaghere has spent his life dealing with the painful complications of sickle cell disease. In 2020, he participated in a ground-breaking gene-editing clinical trial to treat his condition. Jimi says he has not gone to the emergency room since treatment, and aside from regular follow-up for the clinical trial, has no additional sickle cell care ongoing.

Cell and gene therapies augment, repair, replace, or regenerate organs, tissues, cells, genes, and metabolic processes in the body. These therapies can durably treat and **potentially cure a range of diseases –** often with just a single treatment.

The FDA has approved **27 cell and gene therapies to date**, including treatments for aggressive forms of blood cancer and serious rare genetic diseases, including hemophilia B and spinal muscular atrophy. The FDA expects to approve 10-20 cell and gene therapies each year starting in 2025; ARM anticipates that the agency could approve at least five new gene therapies for rare genetic diseases this year.

Gene Therapy

Gene therapy seeks to introduce genes into a patient's body with the goal of durably treating, preventing, or potentially curing disease, including rare genetic diseases and some prevalent diseases.

Cell Therapy

Cell therapy is the administration of viable, often purified cells into a patient's body to grow, replace, or repair damaged tissue for the treatment of a disease. A variety of different types of cells can be used in cell therapy.

Gene Editing

Gene editing is a technique by which DNA is inserted, replaced, removed, or modified at particular locations in the human genome for therapeutic benefit in order to treat cancer, rare inherited disorders, or other diseases.

Tissue Engineering

Tissue engineering seeks to restore, maintain, improve, or replace damaged tissues and organs through the combination of scaffolds, cells, and/or biologically active molecules.

The sector at a glance

	North America	Asia Pacific	Europe	Other Regions	TOTAL
Developers	686	492	244	35	1,457
Clinical Trials	964	848	403	139	2,220

Possible FDA regulatory decisions on cell and gene therapies in 2023

Adaptimmune Therapeutics
Cell Therapy: Advanced synovial sarcoma

Atara Biotherapeutics
Cell Therapy**:** Epstein-Barr virus-associated post-transplant lymphoproliferative disorder

BioMarin Pharmaceutical
Gene Therapy: Hemophilia A

bluebird bio
Gene Therapy: Sickle cell disease

Gamida Cell
Cell Therapy: Hematological malignancies

Iovance
Cell Therapy: Metastatic melanoma

Krystal Bio
Gene Therapy: Dystrophic epidermolysis bullosa

Mesoblast
Cell Therapy: Steroid-Refractory Acute Graft Versus Host Disease

Sarepta Therapeutics
Gene Therapy: Duchenne muscular dystrophy

StemCyte
Cell Therapy: Unrelated donor hematopoietic progenitor cell transplantation

Vertex Pharmaceuticals & CRISPR Therapeutics
Gene Editing Therapy: Sickle cell disease, ß-thalassemia