



Alliance *for*
**Regenerative
Medicine**

Regenerative Medicine
& Rare Disease

February 2019



The Alliance for Regenerative Medicine (ARM) is the preeminent global advocate for regenerative and advanced therapies. ARM fosters research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

By leveraging the expertise of its membership, ARM empowers multiple stakeholders to promote legislative, regulatory and public understanding of, and support for, this expanding field.

www.alliancerm.org

Special thanks to our data partner:

informa

Informa is one of the world's leading knowledge providers. We create and deliver highly specialized knowledge to individuals, business, and organizations around the world.

Informa provides authoritative research and analysis and up-to-the-minute business news, comment, and events for all sector of healthcare, medical, and life science communities. Informa Business Information (IBI) is one of the world's leading providers of industry and drug news, analysis, and data to the global pharmaceutical industry.

Regenerative Medicine & Rare Disease

Rare diseases affect fewer than roughly five in 10,000 people worldwide. Approximately 80% of rare diseases have identified genetic origins, and are often debilitating; about 30% of children with a rare disease will die before their fifth birthday. There are roughly 30 million people in the U.S. living with a rare disease and more than 300 million people worldwide. Taken as a whole, there are nearly 7,000 different rare diseases currently identified; only about 5% have an FDA-approved treatment.

Significant progress in the fields of gene and cell therapy have opened up the possibility of durably treating and potentially curing many of these rare genetic diseases. The premise behind these therapies is relatively straightforward: patients with the rare disease gene are producing too much of, not enough of, or a diseased version of a particular protein or chemical, and the therapy is intended to add in or replace the proper version of that gene.

Current therapeutic approaches include gene and cell therapy:

In **gene therapy**, a delivery vehicle (often an engineered virus) will deliver a correct version of the gene of interest or will silence the diseased gene in a patient's cells.

In **cell therapy**, an engineered cell line is used to introduce a properly functioning gene in order to deliver the intended therapeutic effect.

ARM members active in developing therapies for rare disease include:

- 4D Molecular Therapeutics
- Abeona Therapeutics
- Adaptimmune
- Adverum Biotechnologies
- Aegle Therapeutics
- AGTC
- American Gene Technologies
- Angiocrine
- Asterias Biotherapeutics
- Atara
- Athersys
- Audentes
- AveXis
- AVROBIO
- Axovant Sciences
- Bellicum Pharmaceuticals
- BioMarin
- Biostage
- BioTime
- Bioverativ
- bluebird bio
- BrainStorm Cell Therapeutics
- Caladrius Biosciences
- Capricor
- Casebia Therapeutics
- Celgene Corporation
- Cell Medica
- Cellerant Therapeutics
- CRISPR Therapeutics
- Cynata Therapeutics
- Editas Medicine
- Enzyvant Therapeutics
- ExCellThera
- Fate Therapeutics
- Fibrocell Science
- GenSight Biologics
- Genethon
- Glycostem
- GlaxoSmithKline
- Homology Medicines
- Immusoft Corporation
- Intellia Therapeutics
- In Vivo Therapeutics
- Iovance Biotherapeutics
- Kiadis Pharma
- Kite Pharma
- Krystal Bio
- Legend Biotech
- LogicBio
- Longeveron
- Lysogene
- Magenta Therapeutics
- MaxCyte
- MEDIPOST
- MeiraGTx
- Mesoblast
- MolMed
- Mustang Bio
- Myonex
- Neuralstem
- NightStar Tx
- Nohla Therapeutics
- Novartis
- Orchard Therapeutics
- Oxford BioMedica
- Pfizer
- Pluristem Therapeutics
- Poseida Therapeutics
- Promethera Biosciences
- PTC Therapeutics
- Regenerex
- REGENXBIO
- ReNeuron
- Rocket Pharma
- SanBio
- Sangamo Therapeutics
- Sanofi Genzyme
- Sarepta Therapeutics
- Sentien Biotechnologies
- Sernova
- Sigilon Therapeutics
- Solid Bio
- Spark Therapeutics
- Synpromics
- Tessa Therapeutics
- Terumo BCT
- Thermo Fisher Scientific
- Tmunity Therapeutics
- Ultragenyx
- uniQure
- Vericel
- Vivet Therapeutics
- Voyager Therapeutics
- WindMIL Therapeutics

Regenerative Medicine & Rare Disease

As of the end of 2018:

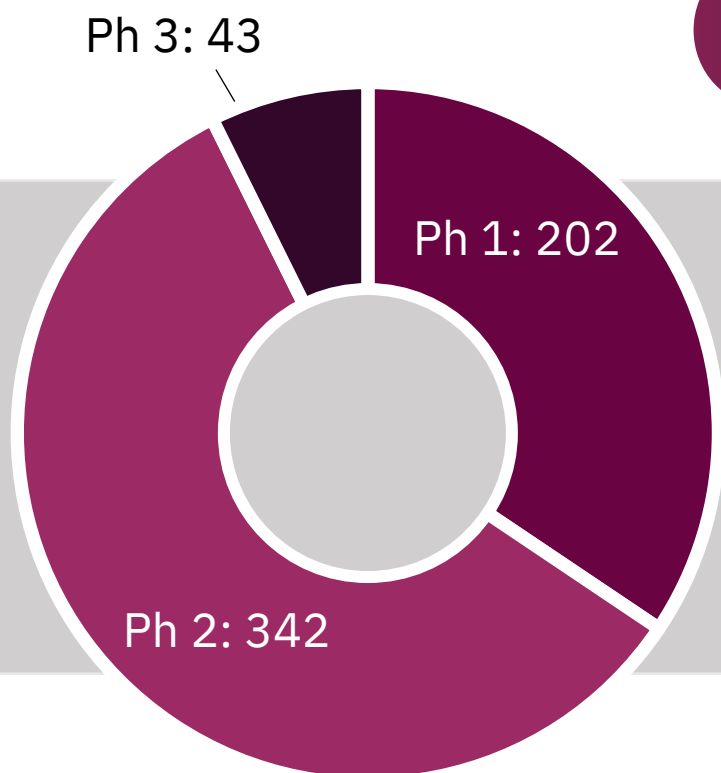


There are more than 323 companies worldwide active in developing regenerative medicines and advanced therapies for rare diseases.

There are currently 600+ clinical products and product candidates in clinical development or marketed.



There are an estimated 300 million patients suffering from rare diseases worldwide.



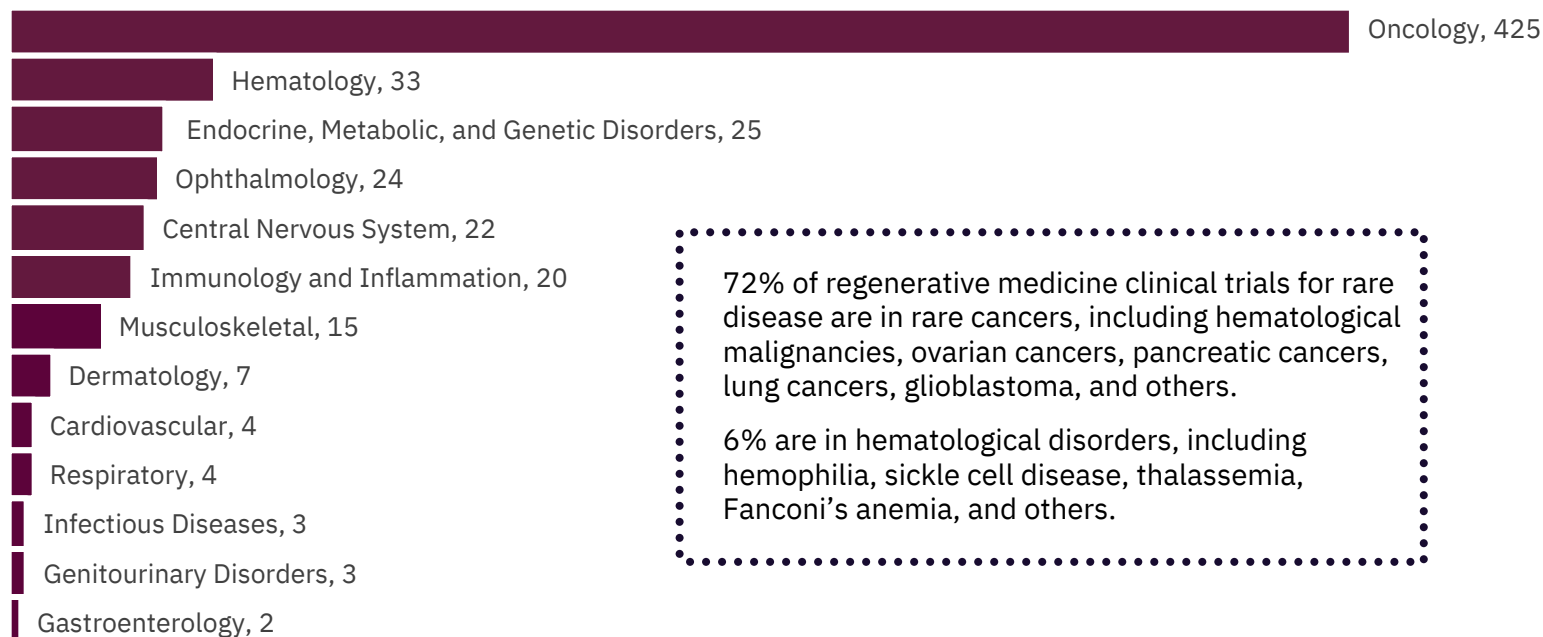
Clinical Trials of Regenerative Medicines for Rare Disease

ARM tracks info from

587
clinical trials
in rare disease

Clinical Trials by Indication & Technology Type

Clinical Trials by Indication



Clinical Trials by Technology



Gene Therapy

Ph I: 61
Ph II: 141
Ph III: 22



Gene-Modified Cell Therapy

Ph I: 121
Ph II: 148
Ph III: 12



Cell Therapy

Ph I: 20
Ph II: 51
Ph III: 7

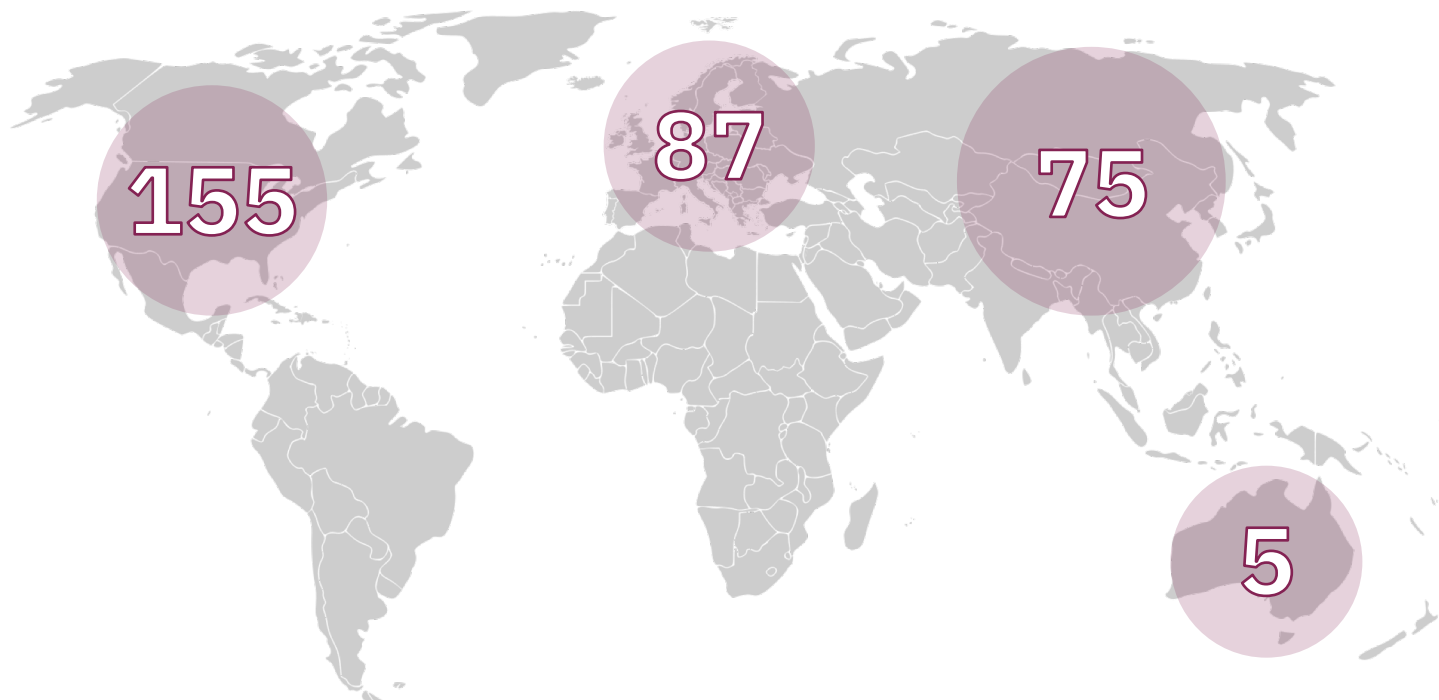


Tissue Engineering

Ph I: 0
Ph II: 2
Ph III: 2

Global Landscape

Companies Active in Developing Regenerative Medicines for Rare Disease



Global Financings of Companies Active in Developing Therapies for Rare Diseases

Total Global Financings

\$9.7B

↑ **48%** from 2017

Gene & Gene-Modified
Cell Therapy

\$8.2B

↑ **39%** from 2017

Cell Therapy

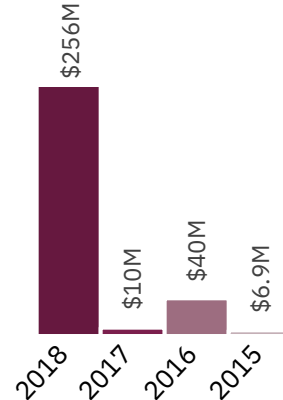
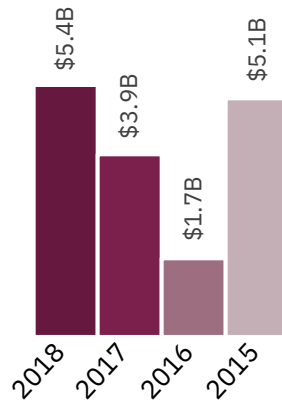
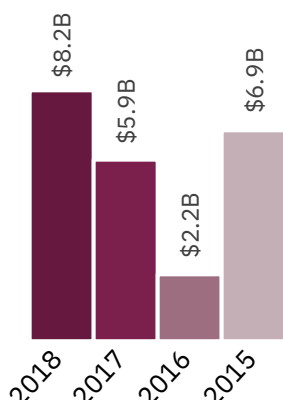
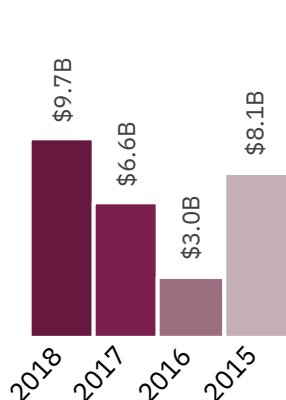
\$5.4B

↑ **39%** from 2017

Tissue Engineering

\$256.2M

↑ **2,552%** from 2017*



All data and figures calculated through the end of 2018. Financings for companies active in both cell & gene therapies are counted in both categories.

*The large year-over-year increase in financings for tissue engineering was primarily driven by the launch of Celularity, a company active in cell therapy and tissue engineering, with \$250M.

Commentary from Developers



John Gray, Ph.D.
Senior Vice President &
Chief Scientific Officer
Audentes Therapeutics

Those patients affected by rare diseases are beginning to see gene therapy as a real possibility in their lifetimes. Because more than half of rare disease patients are children, these advances are especially meaningful.

At Audentes, our portfolio of product candidates employs the use of AAV, a small, non-pathogenic virus that is genetically engineered to function as a delivery vehicle, or vector, and is administered to a patient to introduce a healthy copy of a mutated gene to the body. AAV gene therapy vectors are modified such that they will not cause an infection like a normal virus but are capable of delivering therapeutic genes into patients' cells. Vectors derived from AAV have a well-established safety profile in humans and have been shown to effectively deliver genes to skeletal and cardiac muscle, and the liver, eye, and nervous system.

For me, having in mind the patient that desperately needs new therapeutic alternatives makes all the difference in finding the motivation to keep striving to develop them. Beyond that most meaningful goal, developing gene therapies gives me an opportunity to use my understanding of the molecular biology of life to provide a contribution to society. Each product we develop teaches us a little bit more about the wonderful miracle of life and helps us develop new ways to use that understanding to design the next product, which can improve the life of yet another patient that truly needs it. My hope is that by continuing to develop gene therapies we can not only improve the lives of a great number of patients, but also learn important lessons about life and what it means to be human.



Tim Hunt, J.D.
Senior Vice President
Corporate Affairs
Editas Medicine

There are more than 6,000 genetically-defined diseases, and more than 95 percent of these diseases do not have any approved medicines. Genome editing has the potential to deliver a variety of genome editing medicines that have the promise and potential to provide transformative value – not only to the patients, but to the entire healthcare system. At Editas Medicine, we are defining the field of gene editing and are beginning to unlock its vast potential. We think that's great news for the people we are trying to help.



Alliance *for*
**Regenerative
Medicine**

Alliance for Regenerative Medicine

Contact: Lyndsey Scull | Vice President, Communications | lscull@alliancerm.org
www.alliancerm.org