American Society of Gene + Cell Therapy

asgct.org/advocacy

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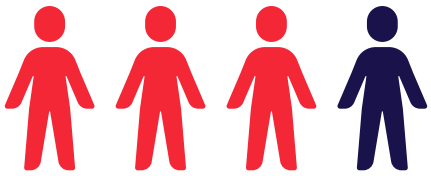
patienteducation.asgct.org

@asgctherapy

MEMBERSHIP OVERVIEW



77% are researchers

5,800+ Members Worldwide

All numbers as of Nov. 2022

Other members include:

- Medical Providers
- Advocates
- Policy Professionals
- Regulatoy Professionals

ADVOCACY AREAS OF FOCUS

Research Funding → Engaging with NIH Institutes and Centers to highlight research needs and funding gaps and to advocate for ways to address them.

Responsible Use of Technology → Supporting dialogue and exchange of ideas as new gene and cell therapy technologies prompt new questions about their use.

Genetic Testing & Screening → Ensuring access to, modernization of, and adequate funding for federal newborn screening legislation, including the RUSP nomination process.

Regulatory Policy → Advocating for predictable, efficient regulatory requirements to facilitate development of safe and effective therapies to alleviate human disease.

Patient Access → Supporting patient access to approved therapies including full coverage of FDA-labeled indications, appropriate use of utilization management tools, and establishing innovative payment mechanisms.

VISION & MISSION

To bring together scientists, physicians, patient advocates, and other stakeholders to transform the practice of medicine. Through which, we aim to advance knowledge, awareness, and education that will lead to the discovery of genetic and cellular therapies that will alleviate human disease.

GENE & CELL THERAPY LANDSCAPE

All numbers as of Jan. 2023

2,053
gene and cell therapies in development (pre-clinical > pre-registration)

697
gene therapy clinical trials, including genetically modified cell therapies

24
globally approved gene therapies, including genetically modified cell therapies

Cell, gene, and RNA therapies are approved worldwide to treat a range of conditions including blood cancers, beta thalassemia, spinal muscular atrophy, Duchenne muscular dystrophy, inherited blindness, and hemophilia A.

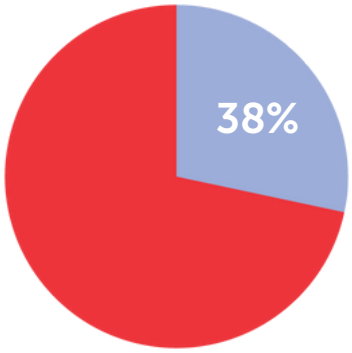
PIPELINE INDICATIONS

Rare Disease

Of gene therapies likely to be submitted for FDA approval over the next five years, 38% (nearly 4 out of 10) are expected to treat rare inherited genetic disorders.

Non-oncology disease targets:

- Hematology (blood) disorders like sickle cell disease
- Neurological disorders that affect the brain and spinal cord
- Musculoskeletal (muscle) diseases
- Retinal (eye) disorders

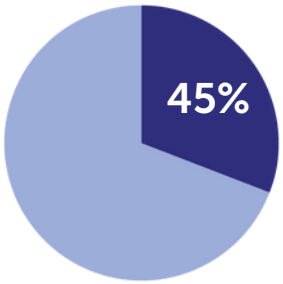


Gene therapies expected to treat rare, inherited genetic disorders.

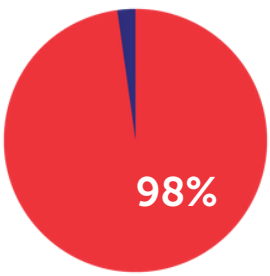
Oncology

Of gene therapies likely to be submitted for FDA approval over the next five years, 45% are anticipated to focus on cancer treatments.

As found in Q4 2021, 98% of CAR-T, CARM, and TCRNK T cell therapies are in development for cancer indications. (Visit asgct.org/research/landscape-report for full data).



Gene therapies to be focused on cancer treatment.



In development for cancer indications.