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.

MEMBERSHIP OVERVIEW

5,800+ Members Worldwide

77% are researchers

Other members include:
- Medical Providers
- Advocates
- Policy Professionals
- Regulatory 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.

All numbers as of Nov. 2022
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

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).