

Future Developments in Gene Therapy

Developments In Current Landscape

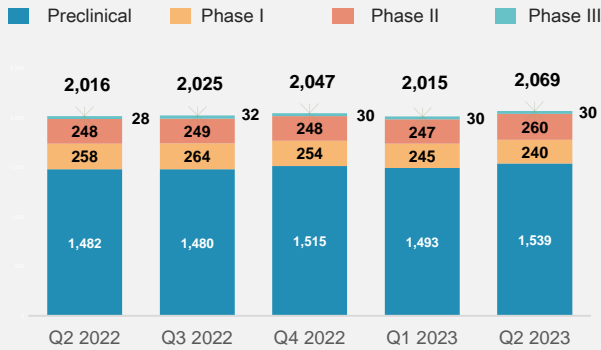


Gene therapy involves direct manipulation of genes to correct/replace faulty information, mitigating associated conditions. It is a rapidly evolving field in medical science, providing innovative treatment for genetic disorders and rare diseases. Gene therapy faces several challenges including gene delivery to cells, risks and side effects, regulatory environment, and high costs. However, there have been some promising advances in this field that would result in more innovative treatments for a wide range of diseases.

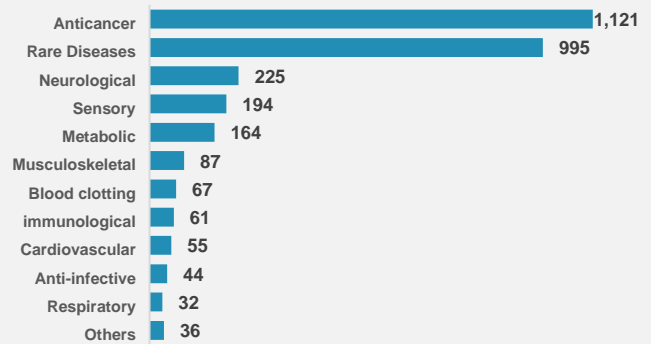
CURRENT DRUG LANDSCAPE

In 2022, the gene therapy pipeline witnessed 6% growth with five new gene therapies receiving approval in that year, two of which are specifically for hemophilia. Oncology and rare diseases are the primary focus of gene therapies in the pipeline.

Clinical trial status of gene therapies | Figures in units

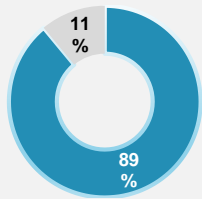


Therapeutic segmentation of gene therapy trials | Figures in units



RECENT DEVELOPMENTS

With the evolving pipeline of gene therapy, continued R&D has led to newer and more efficient gene delivery systems, such as viral and non-viral vectors, which are crucial for the success of gene therapy treatments. Investments in this sector have increased owing to low interest rates and global recognition of the importance of innovative healthcare technologies.



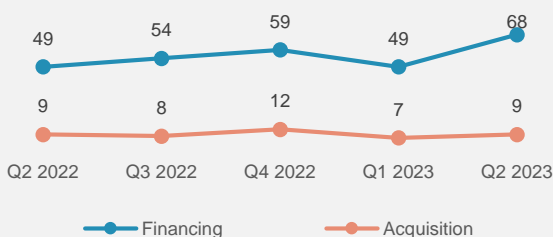
89% of gene therapies in development use viral vectors for delivery. Adeno-associated virus and lentivirus are the most common viral vectors used in development.

■ Viral vectors ■ Non-viral vectors

~68% of gene therapies in development are genetically modified cell therapies. Among them, Chimeric Antigen Receptor T cells (CAR-T) make up 49% and TCR-T 12%. The remaining therapies are less commonly utilized.

310 gene therapy deals were signed in 2021, as reported by IQVIA, in contrast to only 164 deals in 2017. The total value of gene therapy M&A deals in 2021 amounted to \$18.9 Bn, showing a significant increase from \$6.7 Bn in 2017.

- 2021** ● Sarepta Therapeutics acquired Solid Biosciences for **\$11 Bn**, gaining access to gene therapy for Duchenne muscular dystrophy.
- 2021** ● Astellas Pharma acquired Audentes therapeutics for **\$3.3 Bn**, gaining access to gene therapy for spinal muscular atrophy.
- 2022** ● Lilly acquired Akouos for **\$1.6 Bn**, gaining access to gene therapy for sensorineural hearing loss.



68 public financing (IPOs) plus privately raised funding (venture rounds, debt offerings, or private investment in public equity) deals were secured by advanced molecular therapy companies.

Strategic alliances have risen from 54 deals in 2018 to 70 in 2022, increasing at a CAGR of ~12.5%.

Novartis-Intellia therapeutics; develop and commercialize CRISPR-based gene editing therapies for in-vivo liver delivery

Sangamo-Sarepta therapeutics; develop gene therapies for Duchenne muscular dystrophy

Pfizer-Beam therapeutics; develop in-vivo base editing programs for rare genetic disease targets

Sanofi-Scribe therapeutics; develop in-vivo genetic therapies for sickle cell and other genomic diseases

UNMET NEEDS

Gene therapy is showing promise as a treatment for diverse diseases. However, several gaps and challenges must be addressed before it can achieve widespread application.

Method of Delivery	<ul style="list-style-type: none">▪ The delivery of gene therapy products to the target cells is a major challenge.▪ Viral vectors are the most common delivery method. However, they can be immunogenic and cause insertional mutagenesis, which can lead to cancer. Non-viral delivery methods are being developed, but they are not yet as effective as viral vectors.
Regulatory Hurdles	<ul style="list-style-type: none">▪ Gene therapy products have a strict regulatory oversight, slowing down the development and approval process and making it difficult for patients to access these treatments.▪ FDA has announced plans to diminish regulatory barriers by working with the National Center for Advancing Translational Sciences and the Foundation for the NIH to create a revised regulatory framework.
Cost of Care	<ul style="list-style-type: none">▪ Gene therapy is expensive and complex to develop and manufacture. Moreover, the small patient pool limits the potential market for these treatments. The average gene therapy is likely to cost ~\$1.5 Mn.▪ However, there are a few organizations such as the Gene Therapy Access Coalition and National Organization for Rare Disorders that offer financial assistance to cover the cost of gene therapy.

Despite the challenges, gene therapy has the potential to change the treatment landscape, especially for rare and genetic diseases. Through several programs, governments and private foundations are funding R&D of gene therapy. Efficient manufacturing, safer gene delivery methods, and new pricing models would allow more patients to access these treatments.

Right from understanding key issues to advising you through the right set of insights and recommendations, Aranca Research, consolidation, and insightful analysis to aid in-depth understanding of therapy and effective decision-making

HOW CAN ARANCA HELP?

01

Market assessment: Disease burden/disease landscape, Diagnosis and Treatment Paradigm, Humanistic and Economic Burden

02

Current and Future Landscape: Current treatments and latest pipeline landscape assessment for a particular therapy area

03

Strategic initiative analysis: Understanding key market trends in terms of M&As, funding, deals and other pertinent strategic initiatives

04

Health Technology Assessment: Analysis of various HTA decisions published by various countries

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