



Gene Therapy

The Next Generation of Medicine

A Whitepaper Published By:

Medi-Tech Insights

Medi-Tech Insights

Medi-Tech Insights is a world-leading, Healthcare-focused Business Research & Insights firm.

We are the preferred Knowledge Partner to the world's leading Corporates, Start-ups and Investors.

We help our clients by developing research-based insights, enabling our clients to make fact-based & well-informed business decisions. To do this, we leverage our proprietary knowledge of the healthcare sector & our global industry-physician expert network across U.S, Europe & Emerging Markets. To date we have completed over 100 projects in various areas of healthcare.

Our areas of expertise include:

- Market Scan & Analysis
- Market sizing & Forecasting
- Competitive Landscaping & Market share analysis
- Commercial Due Diligence
- Target Product Profiling
- Pre-Launch Assessment

Copyright © 2021 Medi-Tech Insights

All Rights Reserved. This document contains highly confidential information and is the sole property of Medi-Tech Insights. No part of it may be circulated, copied, quoted, or otherwise reproduced without the explicit written approval of Medi-Tech Insights.

Gene Therapy: The Next Generation of Medicine



Gene therapy is a technique that uses genes for prevention or treatment of genetic disorders. It replaces a faulty gene or adds a new gene to cure disease or improve body's ability to fight disease. Gene therapy holds promise for treating a wide array of diseases such as cancer, cystic fibrosis, heart disease, diabetes, hemophilia and AIDS.

Uses of Gene Therapy

- 01 Replaces missing or defective genes
- 02 Deliver genes that speed destruction of cancer cells
- 03 Supply genes that cause cancer cells to revert back to normal cells
- 04 Deliver bacterial or viral genes as a form of vaccination
- 05 Provide genes that promote or impede the growth of new tissue
- 06 Deliver genes that stimulate the healing of damaged tissue, among others

Vectors in Gene Therapy

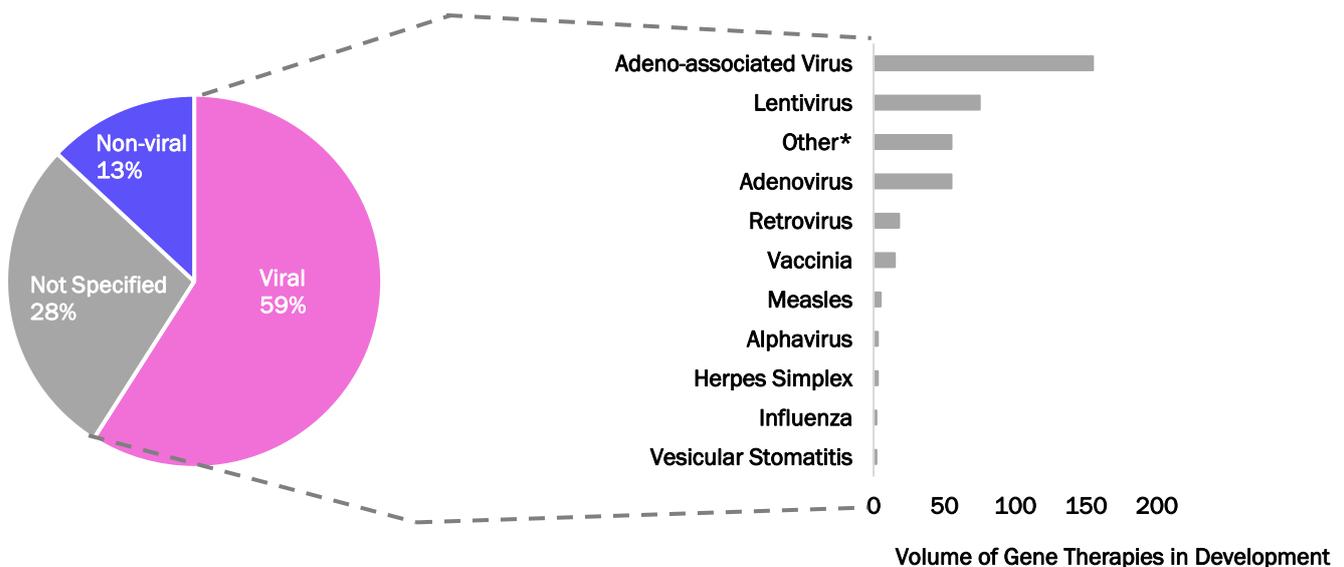
Genes cannot be incorporated into the cells directly; hence viral vectors and non-viral vectors are used as carriers to deliver the genes.

The most common types of vectors are ‘Viral Vectors’ that include adeno-associated viruses, retroviruses, lentiviruses, adenoviruses, vaccinia viruses, pox viruses, among others. **Viral vectors are considered the most effective way of gene transfer. They are used to modify specific cell tissue and can be manipulated to express therapeutic genes.**

Non-viral vectors are naked DNA/DNA plasmids which are administered to the target cells directly as naked DNA with the help of physical method or by chemical method, formulating it with different chemical substances like polymers, & lipids among others.

Owing to drawbacks of viral vectors such as high costs, failure to transfer large size genes, induction of strong immune response by host cell and carcinogenicity, non-viral vectors are used to avoid these consequences.

Viral Vector Delivery: Most Prominent in Gene Therapy



*Other category contains gene therapies with unspecified viral vector types

Sources: The Alliance for Regenerative Medicine (ARM), ClinicalTrials.gov, Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

In the current clinical trials pipeline, most common choice for developers is a virus. **Approximately 59% of gene therapy candidates use a viral vector, while only 13% are delivered by non-viral means (mainly plasmids).** Other modes that are used are messenger RNA, liposomes, or bacterial vectors.

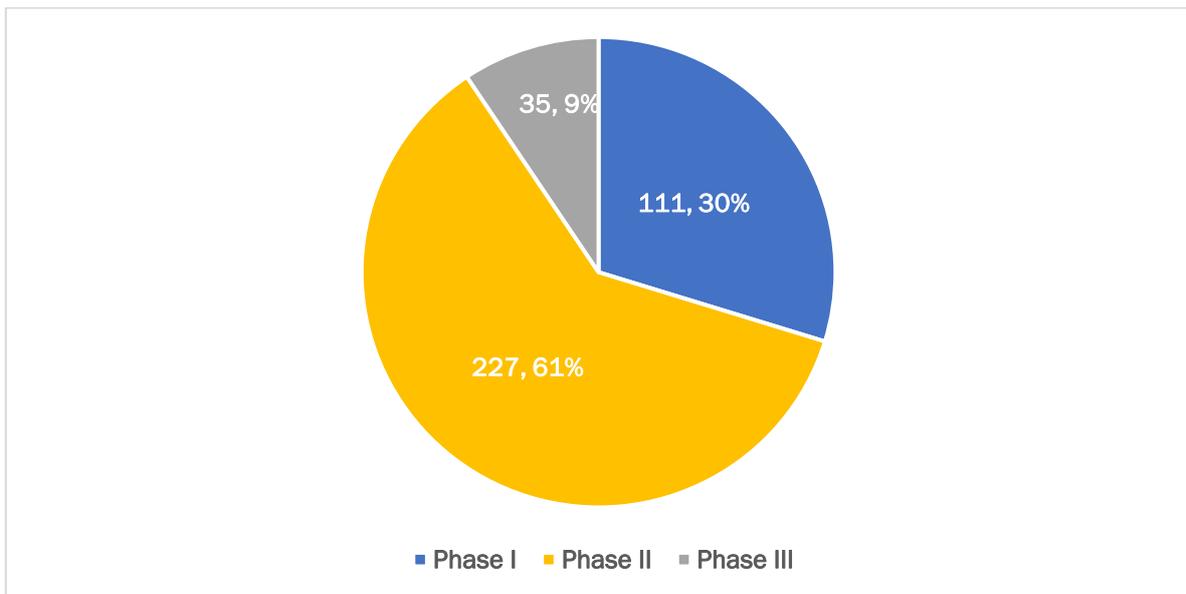
Among viral vectors, **adeno-associated virus (AAV) is the most actively used** owing to its safety, low immunogenicity, and long-term transgene expression. Apart from AAV, **lentivirus and adenovirus** are other commonly used viral vectors in gene therapy development.

Clinical Trial Assessment: Gene Therapy

The number of gene therapy clinical trials has skyrocketed over the years. Till Q3, 2020, there were total 373 gene therapy clinical trials.

Approximately 91% of gene therapy clinical trials were in Phase I and Phase 2.

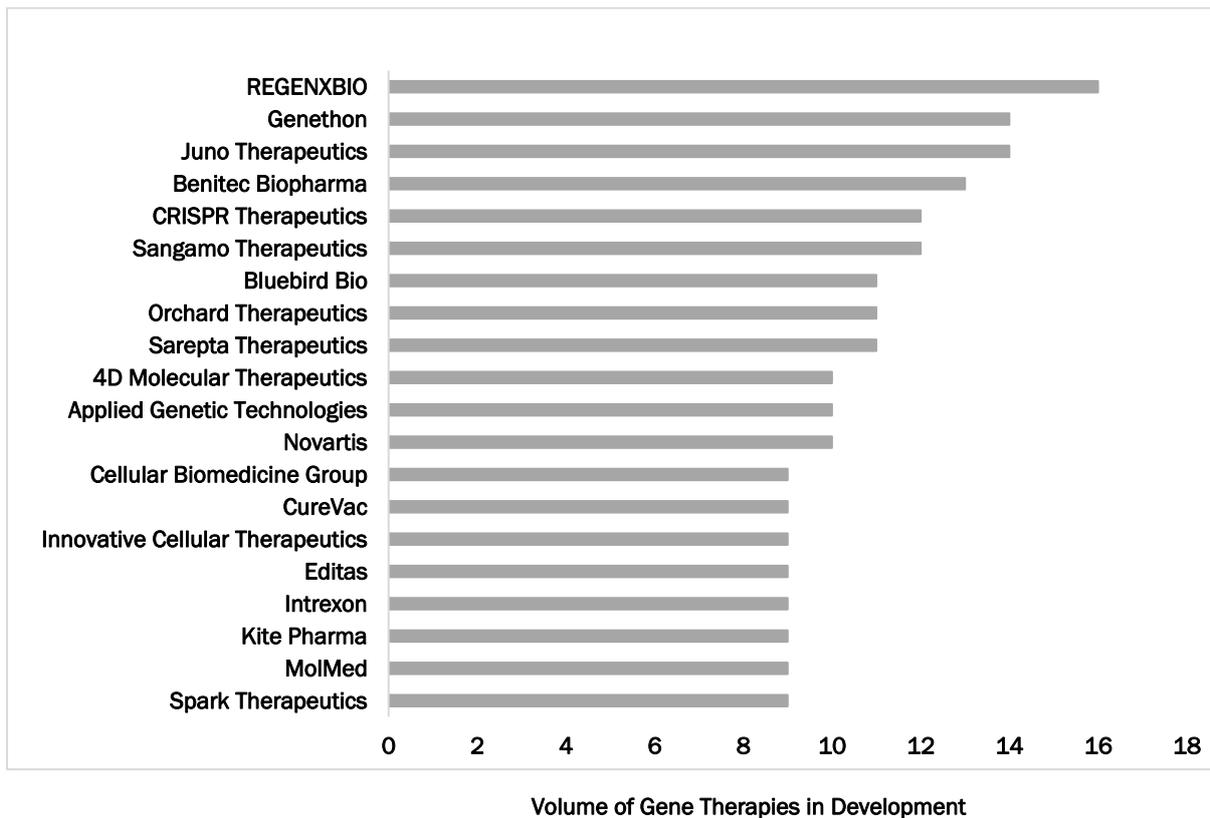
Gene Therapy: Clinical Trials



Sources: The Alliance for Regenerative Medicine (ARM), ClinicalTrials.gov, Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

In the gene therapy market, there are approximately 370+ unique companies with development-stage candidates. The group includes biotech, emerging pharma, mid pharma, and big pharma companies. The gene therapy marketplace is marked by presence of very small players too who are only working on one or two therapies while there are active companies with larger pipelines totalling over a dozen programs.

Top 20 Gene Therapy Companies, By Size of Pipeline



Sources: The Alliance for Regenerative Medicine (ARM), ClinicalTrials.gov, Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

Within the top 20 most active companies by pipeline size, **REGENXBIO leads by volume with 16 gene therapies in development. At 14 candidates, Juno Therapeutics ties with Genethon as the second most active gene therapy company.**

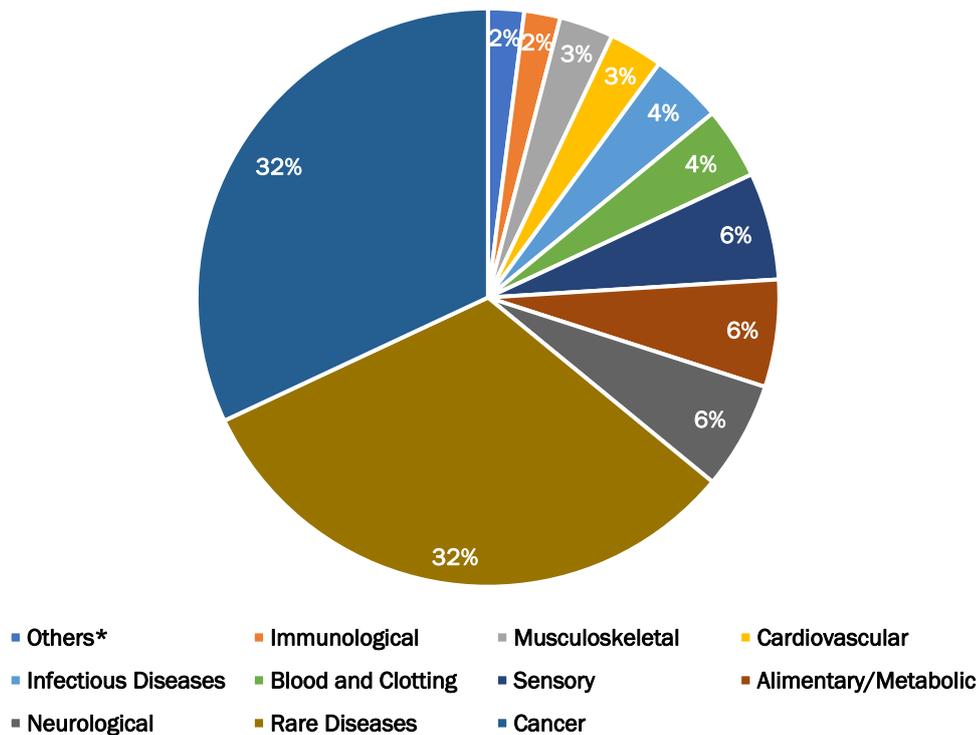
Among larger sized pharma firms, Novartis and Kite Pharma (now owned by Gilead) make the top 20 list. Also included are the gene editing players Sangamo Therapeutics, CRISPR Therapeutics, and Editas.

While not in the top 20, other big pharma players that are pursuing gene therapies include Sanofi, Biogen, GlaxoSmithKline, Pfizer, and Johnson & Johnson.

Application Horizon: Gene Therapy

Gene therapy finds application in oncological disorders, cardiovascular diseases, neurological disorders, rare diseases, infectious diseases, among others. **Oncological disorders and rare diseases are top two applications of gene therapy. Maximum number of companies are having their product pipeline in these two application areas.**

Oncology and Rare Diseases are Focus Therapy Areas

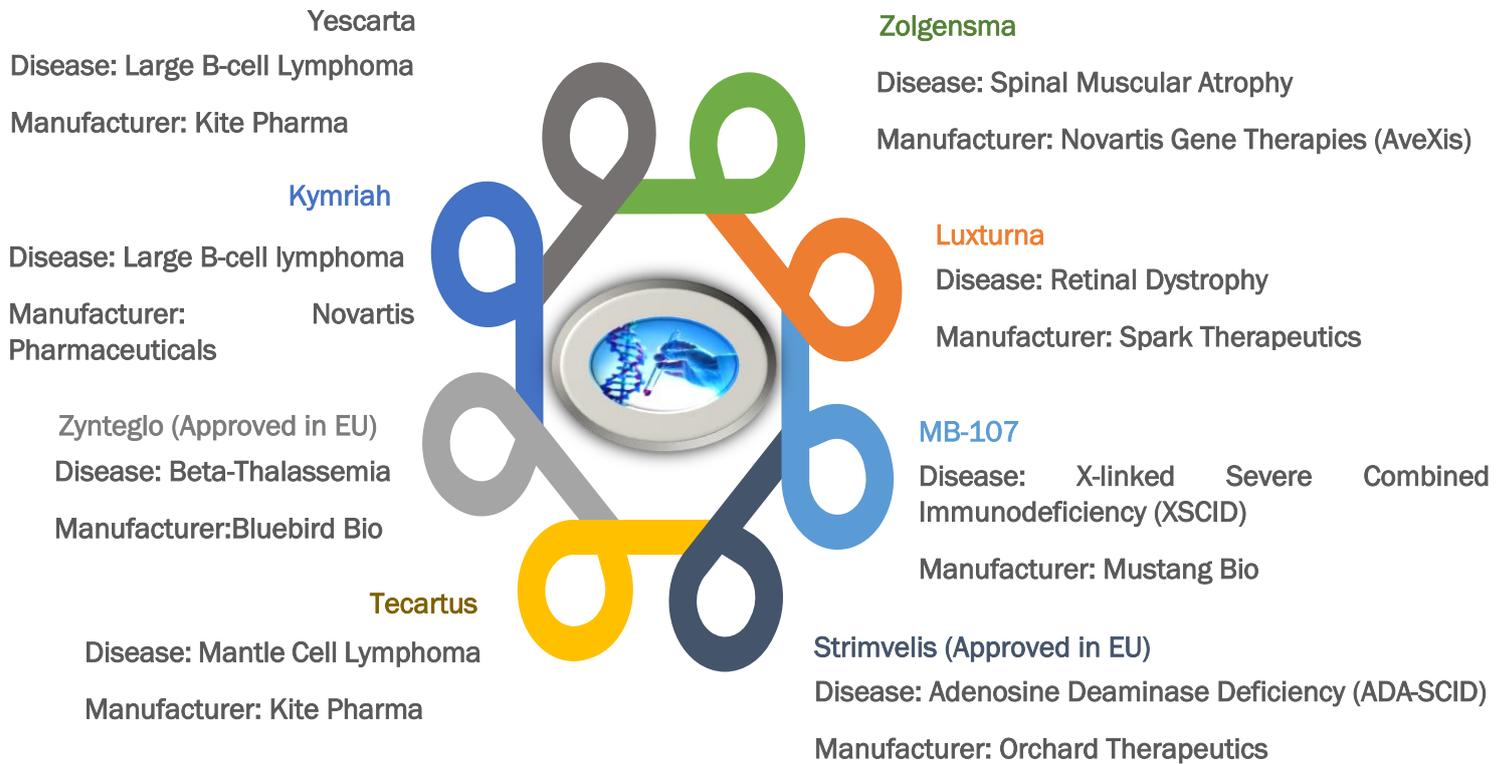


*Others represents dermatological, respiratory, and genitourinary

Sources: The Alliance for Regenerative Medicine (ARM), ClinicalTrials.gov, Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

Key gene therapies under development are for treatment of cancer, hemophilia, heart disease, rare eye diseases, Duchenne muscular dystrophy (DMD), retinal diseases, fatal muscle condition, Huntington's disease, Parkinson's, ALS (Lou Gehrig's disease), among others.

FDA Approved Gene Therapies



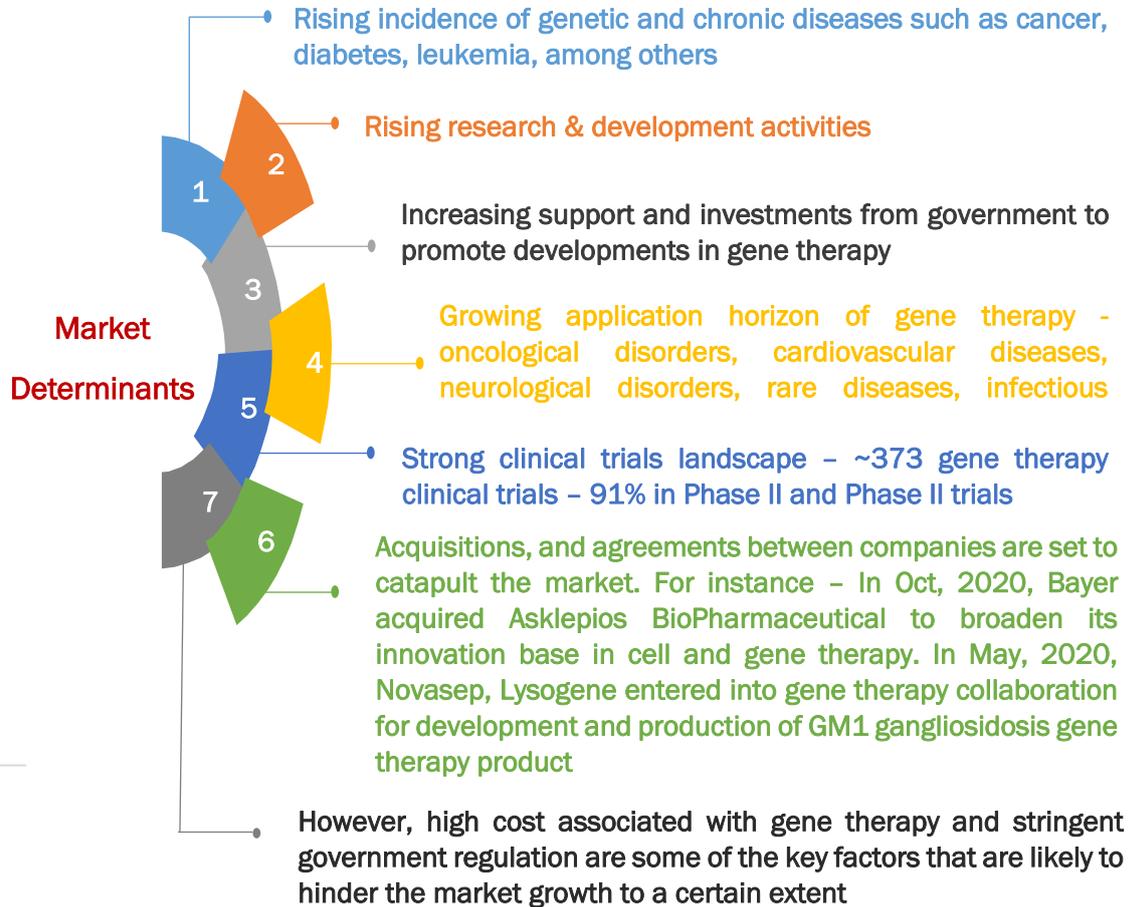
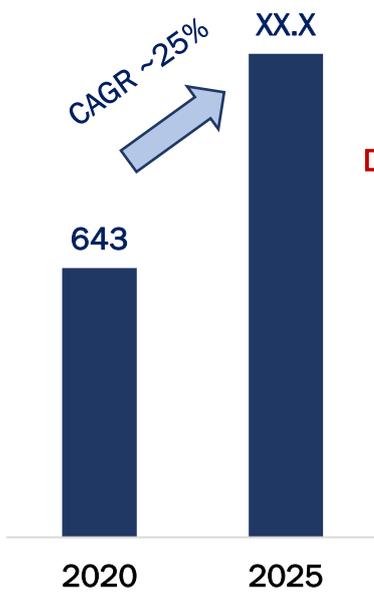
Sources: U.S. FDA, Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

Gene Therapy Market: Global Snapshot



The global gene therapy market was pegged at ~US\$ 643 million in 2020 and is anticipated to witness a CAGR of ~25% in next 5 years

Global Gene Therapy Market (US\$ Million)



Sources: Annual Reports, Investor Presentations, SEC Filings, Press Releases of Companies, Primary Research, Medi-Tech Insights Analysis

Future Outlook: Gene Therapy Market



- The next decade in gene therapy is expected to be positively transformational & exciting!
- With the current rate of trials and treatments in the queue for approval at the FDA and key U.S. and EU approvals for the next generation of gene therapies, manufacturers and other stakeholders have ample reasons to be optimistic about the future prospects of this market
- Gene therapies have high prices as they are expensive to manufacture. Over the next few years, **payers all across the globe** are expected to value gene therapies through a new lens and **adopt payment mechanisms that may be completely novel**
- From recent **outcomes-based agreements established by Novartis and Spark Therapeutics**, it is evident that **payers and pharmaceutical companies are willing to work together** and this is a positive sign for all stakeholders involved
- To meet the high demand as the pipeline advances and grows, **gene therapy manufacturers are expected to turn to contract manufacturing organizations (CMOs) or contract development and manufacturing organizations (CDMOs) for vector supply services**
- The demand for manufacturing is expected to offer lucrative opportunities for CMOs and CDMOs in the specialized market of cell and gene therapy. There are only a select group of companies currently with these capabilities, which are considered highly specialized, and **it is likely that new CMO/CDMO players will emerge as well to address this demand**

DISCLAIMER:

This whitepaper is the property of Medi-Tech Insights and shall at all times remain the property of Medi-Tech Insights. Users may quote or share content from the whitepaper with explicit acknowledgement only.

Interested to discuss how we could support you?

Reach us at info@meditechinsights.com or visit our website www.meditechinsights.com

Medi-Tech Insights
Avenue Franklin Roosevelt 121/b6
1050 Brussels
Belgium

Thank You

Medi-Tech Insights

Avenue Franklin Roosevelt 121/b6

1050 Brussels, Belgium

info@meditechinsights.com