

Gene Therapies Pharmaceutical and Healthcare Pipeline Review

Gene Therapies Therapeutic and Drug Pipeline Review

PUNE, INDIA, April 5, 2018 /EINPresswire.com/ -- Summary

"[Gene Therapies](#): A Diverse Range of Technologies with a Promising Long-Term Outlook" discusses all gene therapies under the broad criteria of genetic material introduced to the cell for a therapeutic purpose. It includes not only the insertion of a gene into the cell for expression, but also gene silencing with RNA interference (RNAi) and antisense RNA, aptamers (DNA or RNA polymers which bind to a protein target), and oncolytic viruses.

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Gene therapies have been in development in humans for 25 years, and a number of products have begun to enter the pharmaceutical market. However, due to various challenges and clinical trial setbacks, progress in developing this technology and achieving suitability for commercial usage has been slow. Gene therapies only account for a marginal market share and many efficacy and safety concerns remain unaddressed. However, the pipeline is robust; 906 pipeline gene therapies are currently in development. Most are at an early stage of development, with 76% at the Discovery or Preclinical stage. However, there are currently 23 gene therapy programs in Phase III development and two at the Pre-registration stage. This indicates that although gene therapies are beginning to reach the market after two decades of research in humans, the majority remain in relatively early development.

This report provides a comprehensive view of the clinical, R&D, commercial and competitive landscape of Gene Therapy, and assesses key developments in delivery vector technology, and challenges and advances associated with the production of such vectors.

Scope

Despite 25 years of clinical research, only a few gene therapies of all types have reached the market globally, and none have achieved strong clinical or commercial success -

- Why do gene therapies still occupy only a minimal market share in their respective indications?
- What can be learned from the gene therapies that have already reached the market?

A number of different viral and non-viral vector types are currently in development for the delivery of gene therapies -

- What are the relative advantages and disadvantages of each vector type and which hold the most promise?
- What proportion of the overall gene therapy R&D pipeline is occupied by each vector type?

The current pipeline for gene therapies is diverse in terms of the approaches and vectors covered; 50% are gene silencing therapies, while 31% involve the insertion of a functional gene -

- In which therapy areas is there the highest level of R&D activity for gene therapies?
- At which stage of development does the majority of pipeline gene therapies reside?

- What is the proportion of the pipeline occupied by each intervention and vector type overall?

A number of companies are currently actively developing pipeline gene therapies, including private, public and institutional enterprises -

- How do gene therapies fit into the overall portfolios of these companies?

- What is the level of involvement in gene therapy research from the top 20 Big Pharma companies?

Reasons to buy

This report will allow you to -

- Understand the current status of the field of therapeutic gene therapies, and the relative clinical and commercial success of currently marketed products, comprising Glybera, Kynamro, Macugen, Vitravene, Gendicine, Oncorine, and Neovasculgen.

- Assess the pipeline for gene therapies split by therapy area, vector type and intervention type, and stage of development. Additionally, a granular assessment of the pipeline is provided across the four major therapy areas for gene therapy: oncology, infectious diseases, central nervous system disorders, and genetic disorders.

- Gain a picture of the current competitive landscape, with a detailed breakdown of companies actively involved in the gene therapy pipeline. Understand the level of involvement on the part of big pharma companies, and the extent to which gene therapies fit into the overall portfolios of companies in this field. Additionally, a highly granular breakdown of companies developing multiple gene therapies is provided.

Table of Content: Key Points

Gene Therapy Overview

Types of Gene Therapy

Types of Intervention

Types of Vector

Currently Approved Gene Therapies

Glybera (alipogene tiparvovec)

Kynamro (mipomersen)

Macugen (pegaptanib)

Vitravene (fomivirsen)

Gendicine (rAd-p53)

Oncorine (rAd5-H101)

Neovasculgen (PI-VEGF165)

Gene Therapy Production Strategies

Production of Viral Vectors

Case Study: Challenges in the Manufacture of AAV Vectors

Challenges to Gene Therapy Development

Gene Therapy Pipeline

Gene Therapy Pipeline by Therapy Area and Stage of Development

Gene Therapy Pipeline by Intervention and Vector Type

Company Positioning

...Continued

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