

# Global Gene Therapy Market Analysis & Forecast to 2022

<https://marketpublishers.com/r/G99E8358534EN.html>

Date: February 2017

Pages: 310

Price: US\$ 4,900.00 (Single User License)

ID: G99E8358534EN

## Abstracts

This report provides the reader with:

Current Global Market Worth and Forecast with CAGR Through 2022

Sub-Market Worth by Therapeutic Area (Cancer, Rare Diseases, Cardiovascular, Neurological, Ocular) and Forecast with CAGR Through 2022

Sub-Market Worth by Geography (Americas, Europe, RoW) and Forecast with CAGR Through 2022

Sub-Market Worth by Technology (Gene Product, Service, Viral Vectors) and Forecast with CAGR Through 2022

Insight into gene therapy technologies, challenges associated with developing therapeutic genes and disadvantages of gene therapy.

Full outline of the gene therapy industry from the formative years through to products discovered during 1990 and 2017.

Detailed descriptions of commercialized products approved between 2003 and 2017 that include: Gendicine, Rexin-G, Oncorine, Neovasculgen, Glybera, Imlygic, Strimvelis, Zalmoxis, Kymriah, Yescarta and Luxturna.

Description of seven of the Phase III product candidates that include: Generx, Collategene, LentiGlobin, Lenti-D, VM-202, Invosa and GS-010.

Description of 21 Phase II product candidates that are set to have significant market share.

Commercialization status of gene therapies in by geographic region

Evaluation of gene therapy pricing

Description of the firstever warranty offer by GSK for Strimvelis.

A detailed analysis of various types of viruses used as vectors.

Description of clinical applications of gene therapy and the various genetic and infectious diseases addressed by gene therapy.

Description of 77 companies that are directly and indirectly associated with gene therapy industry.

### 1.3 Key Questions Answered in this Report

What is the size of gene therapy market?

What is the CAGR and market size over the next five years?

What are the different sub-markets and their worth/CAGR over the next five years?

What is gene augmentation therapy?

What is suicide gene therapy?

How is ex vivo gene delivery different from in vivo gene delivery?

What are the types of gene therapies classified on the basis of targeted cell types?

What is the role of CRISPR technology in gene therapy?

What are the approved gene therapy products?

How many gene therapy product candidates have reached the Phase III stage?

How many Phase II gene therapy product candidates are there?

What is the commercialization status of gene therapies in E.U. member countries?

What are the prices of gene therapy products?

What are the reasons for this extortionate pricing of gene therapies?

Which company is offering warranty for its gene- therapy product?

What is the current strength of gene therapy industry?

Is it true that the real strength of gene therapy industry is based on the number of clinical trials?

What is the total number of ongoing clinical trials as of 2017?

What is the distribution of clinical trials by geography?

Which countries are associated with gene therapy clinical trials?

What are the major indications addressed by the clinical trials?

Which genes are transferred in these clinical trials?

How many Big Pharma are associated with the gene therapy industry?

What are non-viral and viral vectors?

What are the various features of viral vectors?

Which viral vectors are predominantly used in gene therapy clinical trials?

What are the major diseases addressed by therapeutic genes?

Where is the gene therapy market heading, and what opportunities and challenges will it face?

## EXECUTIVE SUMMARY

Growing at a CAGR of over x% the global gene therapy market is forecast to hit \$363 million by 2022 from \$x million in 2017. Strengthened by recent approvals of Kymriah, Yescarta and Luxturna in the US, and a committed European, Japanese and Chinese environment, gene therapy is set to become a significant player in the biopharmaceutical industry. The space covers many therapeutic areas specifically, oncology, rare diseases, Parkinson's, HIV, severe combined immuno-deficiencies (SCID) and hemophilia. Gene therapy is driven by over 2,200 clinical trials globally, with over 55% of this occurring in the US, followed by Europe, Canada and China. Recently, in November 2017, the FDA indicated that gene therapies will now qualify for a fast approval process, which will bring more therapies to market faster. However, the space also has significant challenges, such as manufacturing logistics, reimbursement and its high cost. This 310 page market analysis cutting-edge report tackles this growing but challenging industry, it highlights its strengths, weaknesses and opportunities and provides a comprehensive account of major companies, clinical trials and technological advancement.

Since the FDA approved Kymriah (tisagenlecleucel), Yescarta (Axicabtagene ciloleucel) and Luxturna (voretigene neparvovec-rzyl) in 2017, the US gene therapy space has expanded significantly, underlined by the fact that over 55% of completed and ongoing trials are located in this geographic. Growth in the gene therapy industry has resulted in new commercial initiatives and the emergence of new startups and spin-off biotechs. Furthermore, gene therapy specifically has raised well over \$600 million of venture capital in the last five years. Early stage companies have raised seed, Series A and Series B investment steadily since the market took off, including Spark Therapeutics, Avalanche Biotech, uniQure, Voyager Therapeutics, Editas Medicine and GenSight.

In 2017, the gene therapy market for technologies, services and products was estimated to be worth \$x million, with a potential to reach \$363 million by 2022. The main market space is cancer which currently holds x% market share. This indication generated \$x million in 2017 and will generate \$x million in 2022. This is followed by rare diseases, cardiovascular, neurological and ocular indications. Looking at the market by technology, at present, gene product therapeutics generate the majority of revenue with over \$x million in 2017, growing to \$x million by 2022. Viral vectors are set

to generate \$x million in 2017, and will rise to \$x million in 2022, and by then gene therapy services such as vector development and transfection will hit \$x million. At present, the Americas have penetrated the market significantly with 65% geographic share, followed by Europe (x%) and the RoW (x%).

Gene therapy products approved between the years 2003 and 2017 include Gendicine, Oncorine, Rexin-G, Neovasculgen, Glybera, Imlygic, Strimvelis, Zalmoxis, Kymriah, Yescarta and Luxturna. Gendicine was approved for head and neck squamous cell carcinoma and has been in the Chinese market since 2003. Rexin-G was approved in the Philippines back in 2007 for the treatment of primary and metastatic cancer. Oncorine was approved in China in 2005 for nasopharyngeal carcinoma. The Russian market has Neovasculgen from 2011 for the treatment of peripheral arterial disease (PAD) and critical limb ischemia. The first gene therapy approved in E.U. was Glybera in 2012 for the treatment of familial lipoprotein lipase deficiency (LPL), however in October 2017 it was pulled from the market due to lack of patient demand. In 2015, Imlygic was approved in E.U. and also in the U.S. to treat melanoma, and Phase II results released in 2017 indicated its efficacy in combination with the checkpoint-inhibitor, Yervoy. In the E.U., Strimvelis was approved in 2016 for the treatment of adenosine deaminase severe combined immunodeficiency (ADA-SCID). In 2016, Zalmoxis was approved in E.U. for the treatment of leukemia. 2017 was a bumper year for gene therapy with Kymriah, Yescarta and Luxturna all gaining FDA approval.

Renewed interest has encouraged start-up companies to affiliate with academic centers for tech know-how. As clinical trials advance towards licensure, more meticulous product characterization using improved analytical methods and progressively higher regulatory compliance will be required. Some of the ongoing clinical trials are closing on to produce promising results, including one for hemophilia B caused by the deficiency of Factor IX using a recombinant adeno-associated virus (AAV) as a vector. The product candidate if succeeds will be a relatively cheaper alternative to the expensive and lifelong factor replacement therapy.

A second example of a successful outcome in gene therapy are studies conducted by independent laboratories focusing on sub-retinal delivery of recombinant AAV expressing retinal pigment epithelial RPE65 for Leber Congenital Amaurosis Type 2. A third example is the clinical trial involving nine children with X-linked severe combined immunodeficiency (SCID-X1) treated with autologous bone marrow CD34+ cells transduced with a self-inactivating (SIN)  $\gamma$ -retroviral vector expressing the IL-2 receptor  $\gamma$ -chain. The most significant achievement in gene therapy is the spectacular clinical results obtained by many independent teams using CAR-T-cell technology. This novel

strategy involves ex vivo gene transfer using recombinant retroviral or lentiviral vectors of chimeric antigen receptors consisting of antibody-binding domains fused to T-cell-signaling domains into patient T lymphocytes.

As gene therapies are generally meant for one time or short duration treatments, they are customized to individuals confined to small patient populations. Therefore, manufacturing firms are expected to seek premium prices for these therapies. Because of this, these therapies will have to face valuation and reimbursement challenges. Stakeholders will show reservations about the hefty price tags and they will require significant data to be convinced. With the removal of Gylbera from the EU market in 2017, due to the fact that only one patient was treated with the drug, all eyes are focused on the number of end patients that will be treated, and their ability to pay. To that end, launching of new drugs may have to be delayed in order to collect more data for payers. Furthermore, annuity based reimbursement agreements and pay-for-performance scenarios will have to be tackled.

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