Gene therapy - A one shot cure!

After almost five decades of extensive research, the era of gene therapy has finally arrived. Persistent work by eminent scientist has finally paid off; gene therapy is now considered a promising treatment option for a plethora of human disease.

Gene therapy represents a major shift in medicine. It involves the use of a vector, for example, a modified virus, to insert corrected gene into human cells to correct a mutated gene. Gene therapy aims to correct the underlying genetic cause of a disease and not just the disease symptoms and this, has led to a new ray of hope for patients with rare disease and cancer sufferers.

The term gene therapy comprises a broad range of technologies and is classified into gene silencing, gene replacement, molecular diagnostics, stem cell delivery and gene editing. From these techniques, gene silencing and gene editing have been the most thoroughly studied and both seem to have good market prospects.

Approved products

Gene therapy has been traditionally associated with inherited disease, but as a matter of fact, the majority of work has been in the field of oncology.

FDA has approved three gene therapies and numerous others are under active clinical investigation.

The first gene therapies to get approved by the FDA include Kymriah (Novartis) and Yescarta (Gilead Sciences). Kymriah is approved for acute lymphoblastic leukemia, or ALL and Yescarta is indicated for use in non-Hodgkin lymphoma. CAR-T cell immunotherapy (for chimeric antigen receptor T cell) is the term specifically used to describe these treatments. The process involves removal of patient's T cells from a blood sample and then genetically modifying them to produce artificial proteins on their surfaces. These artificial proteins are called chimeric antigen receptors and they possess the ability to identify cancerous cells and destroy them.

Luxturna is the first gene therapy for inherited disease/ genetic disorder to receive FDA approval. It is developed by Philadelphia-based Spark Therapeutics.

Glybera, a product of uniQure N.V, was the first gene therapy to be approved for use in Europe. Glybera was approved in 2012 for an ultra-rare condition termed hereditary lipoprotein lipase deficiency (LPLD). Nevertheless, the $1.2 million-per-course gene therapy, was finally withdrawn in 2017 by uniQure due to uncertain commercial prospects.

Other potential targets for gene therapy include hemophilia, sickle-cell disease, several serious inherited neurodegenerative disorders, an array of other genetic diseases, and multiple cancers of the bone marrow and lymph nodes.

Market prospects - a hot investment sector!

Due to the significant potential which gene therapy possess, a number of giant Pharma companies have started investing in this field. Novartis has made a $170 million deal with Spark for rights to use its blindness treatment, Luxturna, outside the U.S. Novartis has also acquired AveXis, an Illinois-based gene-therapy developer, and plans to bring on the first gene-therapy option for the treatment of spinal muscular atrophy.

Another company following Novartis steps is Sanofi. Sanofi bought Bioverativ in large part because of its potential to develop gene therapies. With this acquisition, Sanofi aims to target the hemophilia market.

Challenges

A major concern related to the use of gene therapy is the large price tag attached to it. The million dollar price tag will certainly restrict the number of patients who may benefit from this groundbreaking therapy. Kymriah costs $475,000 for a onetime treatment, Yescarta, is listed at $373,000 and Luxturna is priced at a whopping $ 850,000! This may prove to be a major hurdle for both the manufacturer and insurance companies.

Another challenge would be effectively managing the host immune system in order to ensure both patient safety and a durable therapeutic response. Efforts have been initiated in this direction with many studies working towards the same.

Even with all the potential obstacles, gene therapy is certainly a lucrative sector for giant companies. For companies involved in gene therapy, the first to market benefit will certainly be a game changer because the target population is a very smaller market.

As the originator company and payers wrestle with making this therapy affordable, the potential of this breakthrough approach in treating a wide range of challenging diseases is certainly paving its way to a bright future.

References:

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