

CASE-STUDY

# Next generation of medicine – Gene Therapies

Providing effective & personalized treatments



## Quick overview

Our client has been the front runner when it comes to developing new modalities and investing in advanced technology platforms. With the significant research progression happening in the gene therapy area, they wanted to understand the state-of-the-technology in the industry so that they can augment their internal R&D, manufacturing, & analytical processes

## Client success

FutureBridge strategic program helped the Client team to understand the recent technological advancements happening in the rapidly evolving gene therapy landscape. The program was focused on various aspects of gene therapies, including delivery techniques for gene therapy, to manufacturing/processing techniques, and primary packaging. The key business questions that were answered:

Continuous advancements in gene and cell therapy domain has expanded its horizon to various diseases and research is ongoing on various aspects to address challenges related to delivery and manufacturing. But industry is confident that this momentum will continue in the near future.

- What are the various platforms/approaches used for gene therapy products? How they are addressing the current challenges and what are their associated weaknesses?
- Which technologies offer unique value proposition for the client?
- Who can be the long-term technology partners for the client in accelerating their R&D processes?
- What are the strategic investments made by the competitors/big players in the gene therapy area?

### CRISPR

**Clustered Regularly Interspaced Short Palindromic Repeat (CRISPR) systems**, have been modified for genome engineering. CRISPR was originally employed to **knock out target genes** in various cell types and organisms, but modifications to various Cas enzymes have extended CRISPR to selectively **activate/repress target genes**.

### Zinc Finger Nuclease

**Zinc-finger nucleases (ZFNs)** are artificial **restriction enzymes** generated by fusing a **zinc finger DNA-binding domain** to a **DNA-cleavage domain**. Zinc finger domains can be engineered to **target specific** desired DNA sequences and this enables zinc-finger nucleases to **target unique sequences** within complex genomes.

### TALEN

**Transcription activator-like effector nucleases (TALEN)** a protein-based DNA targeting system that exhibit better specificity and efficiency than ZFNs. Similar to ZFNs, TALENs comprise a nonspecific DNA cleavage domain fused to a customizable sequence-specific DNA-binding domain to generate DSBs.

AstraZeneca SANOFI GENZYME moderna

Flagship Pioneering HOMOLOGY Medicines, Inc. Sangamo

LONZA SIGMA-ALDRICH RODOS BIOTARGET

FutureBridge assessment included 360° approach to cover comprehensive research landscape through early stage patent & scientific literature, as well as point-of-views of pioneers and Subject Matter Experts (SMEs) in the domain covering various aspects on:

- Novel approaches for gene therapies
- Effective delivery techniques for gene therapy
- Advanced manufacturing/processing techniques
- Compatible primary packaging solutions, and
- Analytical techniques and processes

FutureBridge’s assessment helped the client to develop an in-depth understanding of the entire landscape of state-of-the-art technologies related to gene therapy and identify the best-fit technology partners with capabilities to bring synergy to the client’s internal gene therapy programs and ultimately speed up the operations.

## About FutureBridge

FutureBridge tracks from 1 to 25 years, how industries and sectors will evolve, develop, and innovate.

We keep your business ahead of the technology curve. Propel your growth, identify new opportunities, markets, and business models. We can answer your key strategic questions. Facilitate focused, tailored solutions and partnerships using our platforms and programs, with access to global information ecosystems and key industry players for their insights and expertise.