



evotec

DD*in*

DRUG DISCOVERY INSIGHTS



**EVOTEC
EXPANDS
INTO GENE
THERAPY**

EVOTEC — GENE THERAPY

State-of-the-art gene therapy site in Orth an der Donau, Austria with an acknowledged and experienced team dedicated to gene therapy drug discovery and development

- ▶ World-class expertise and know-how in the field including vectorology, virology, analytics & assays, etc.
- ▶ Extensive disease insights and expertise in gene therapy applications for hemophilia, hematology, metabolic and muscle diseases
- ▶ Strong leadership team with acknowledged expertise in gene therapy

“GENE THERAPY IS A PROMISING APPROACH IN THE DEVELOPMENT OF POTENTIALLY CURATIVE OR STRONGLY DISEASE-MODIFYING MEDICINES FOR PATIENTS, ESPECIALLY FOR INHERITED AND RARE DISEASES”

Friedrich Scheiflinger, EVP Head of Gene Therapy

Rationale behind the step into gene therapy

- ▶ Evotec is now able to find the best suited drug candidate agnostic of modality for any given biology
- ▶ Development of own and co-owned assets in a completely unbiased manner
- ▶ Gene therapies offer hope for a wide array of so far untreatable or difficult-to-treat diseases
- ▶ Gene therapy market is expected to grow exponentially in the next decade

Agreement with Takeda

- ▶ Multi-year research and development collaboration covering selected Takeda gene therapy projects
- ▶ Financials: Undisclosed upfront payment + various payments over time

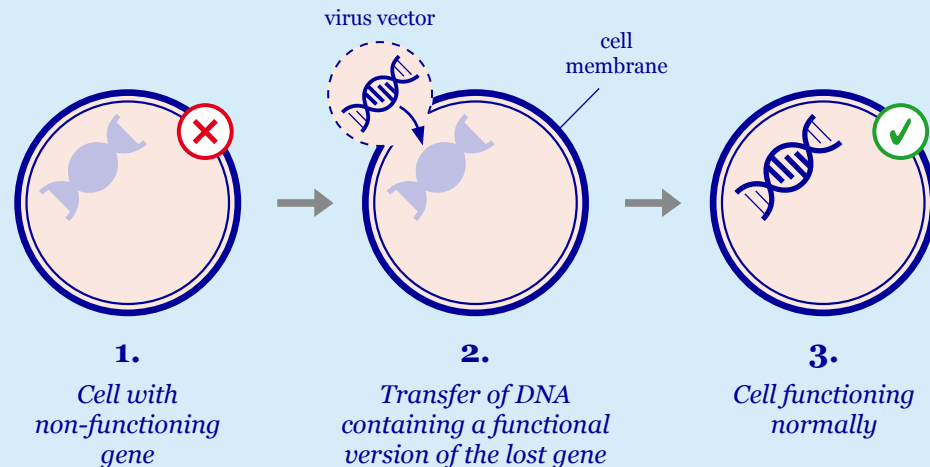


WHAT IS GENE THERAPY?

Gene therapy is a technique that delivers genetic materials into the cells or body of a patient as a therapy. Gene therapies can work through several mechanisms:

- ▶ Replacing a disease-causing gene with a healthy copy of the gene
- ▶ Inactivating a disease-causing gene that is not functioning properly
- ▶ Introducing a new or modified gene into the body to help treat a disease

How does it work – simplified illustration:



There is a variety of types of gene therapy products, including:

- ▶ Plasmid DNA
- ▶ Viral vectors
- ▶ Bacterial vectors
- ▶ Human gene-editing technology
- ▶ Patient-derived cellular gene therapy products

The history of gene therapy

1972 – A seminal paper titled ‘*Gene therapy for human genetic disease?*’ was published in Science

1990 – Launch of the first approved gene therapy trial

2003 – China became the first nation to approve a gene therapy (oncology indication)

2012 – First approval granted in Europe

2017 – FDA made the first gene therapy available in the United States

Sources

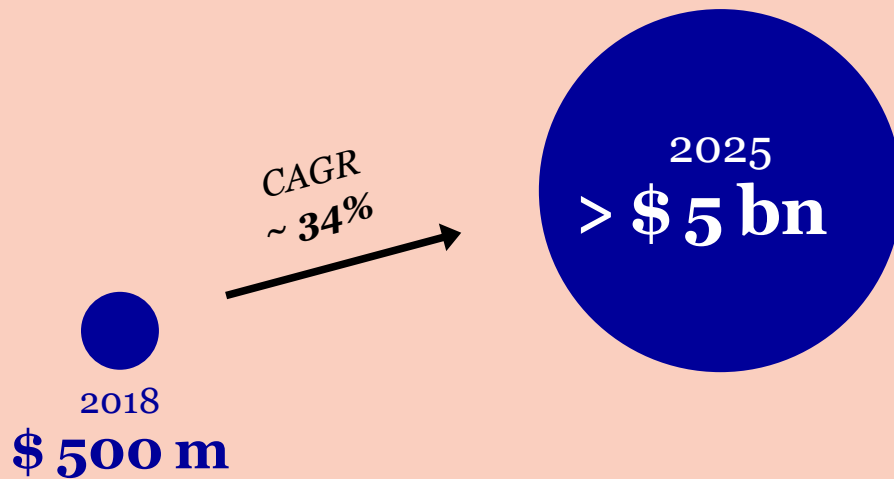
<https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy>
<https://www.labiotech.eu/features/gene-therapy-history/>

FACTS & FIGURES

Market

According to various analyst reports, the gene therapy market was valued at approx. \$ 500 m in 2018, a relatively low number due to still only very few drugs on the market.

The market is expected to reach > \$ 5 bn by 2025 with an impressive CAGR of ~34% over the forecast.

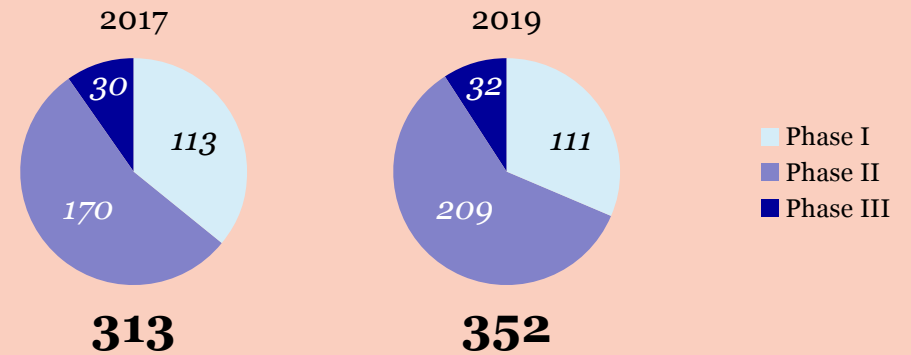


Total global financings
for gene & gene-modified
cell therapy:

2017
\$ 4.5 bn

2019
\$ 7.6 bn

Clinical trials underway worldwide by end of



In early 2018, there were more than 950 molecules in the pipeline with various regulatory bodies in various clinical phases. However, around 76% of the molecules are in the developmental or pre-clinical stages and are expected to be available by the late 2020s.

→ ***This will lead to another further boost for the gene therapy market.***

By the end of January 2020, the FDA has approved four gene therapy products and the agency anticipates many more approvals in the coming years, as evidenced by the **more than 900 investigational new drug (IND)** applications for ongoing clinical studies in this area.

By 2025, it is predicted that the FDA will be approving **10 to 20** cell and gene therapy products a year.

Sources

<https://www.pharmaceutical-technology.com/comment/fda-gene-therapy/>
<https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics>
<https://www.fda.gov/news-events/press-announcements/fda-continues-strong-support-innovation-development-gene-therapy-products>