evotec

DDin
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

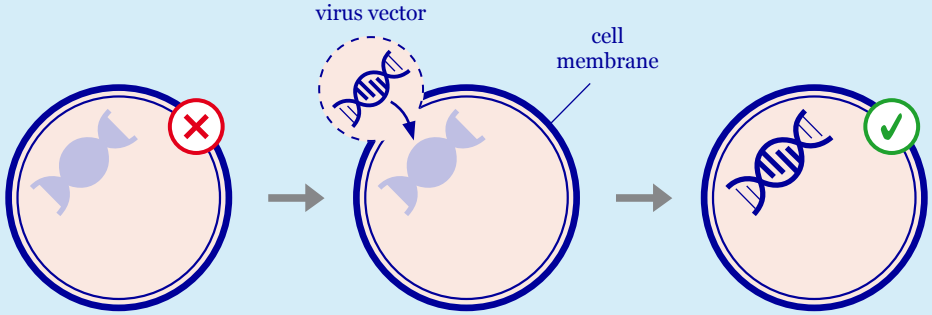
Takeda + evotec

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

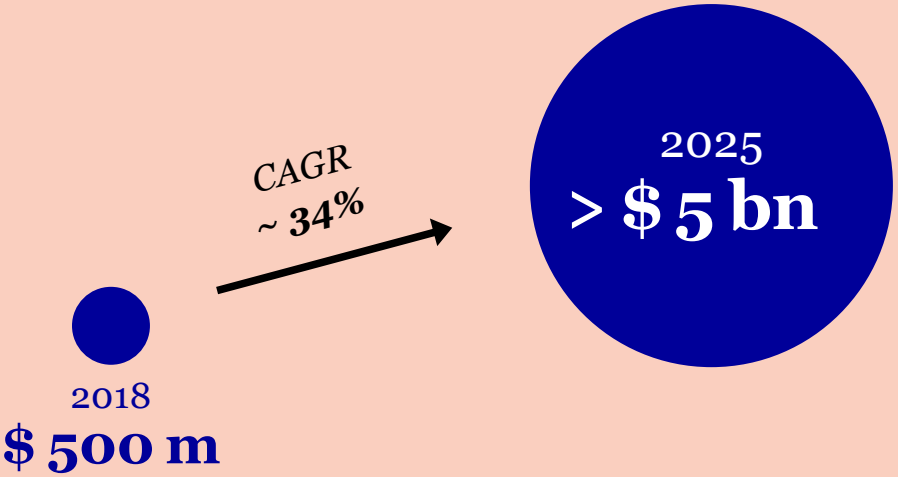
Sources
https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/what-gene-therapy
https://www.labiotech.eu/features/gene-therapy-history/

FACTS & FIGURES

Market

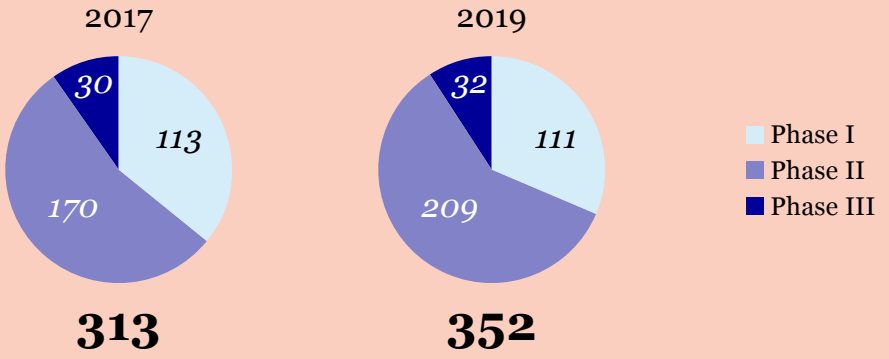
According to various analyst reports, the gene therapy market was valued at approx. $ 500 m in 2018, a relatively low number due to still only very few drugs on the market.

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
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