



Gene Therapy

GENE THERAPY OVERVIEW

Genetic disorders result from inherited or spontaneous changes in the DNA code (mutations).

These changes can lead to the loss of function or gain in toxic function of a protein and subsequent alteration of cell function. There are two major types of gene therapy:

Gene Addition

Enable the body to produce a functional protein that it could not adequately make before.

Gene Editing

Targeted modification of a specific site in the genome, including gene correction/insertion and gene inactivation/disruption.

370

Clinical trials in monogenic diseases

1793

Phase 1 clinical trials

654

Targeted genes are receptors

263

Clinical trials with AAVs

80%

Rare diseases have a genetic background

320 Millions

People could be affected world wide

GENE THERAPY AT EVOTEC

1

Gene therapy Center of Excellence (Austria)

35

Scientists and research associates

>100

Years of cumulated gene therapy R&D experience

GENE THERAPY PLATFORMS AT EVOTEC

Viral gene delivery

- ▶ AAVs
- ▶ Novel platform - undisclosed
- ▶ Other viral

Non-Viral gene delivery

- ▶ LNP
- ▶ Exosomes
- ▶ Other non-viral

Gene editing



GENE THERAPY-RELATED TEAM ACCOMPLISHMENTS

33

Publications

>10

Patents

3

INDs

GENE THERAPY PAYLOADS AT EVOTEC

- ▶ DNA
- ▶ mRNA
- ▶ siRNA
- ▶ dbDNA



GENE THERAPY THERAPEUTIC AREAS AT EVOTEC

- ▶ CNS
- ▶ Lysosomal Storage Disorders
- ▶ Muscular diseases
- ▶ Hematology
- ▶ Metabolic diseases

