

Introducing Gene Therapy

part of UCB's
innovation strategy

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Neil, UCB

Disclaimer & safe harbor

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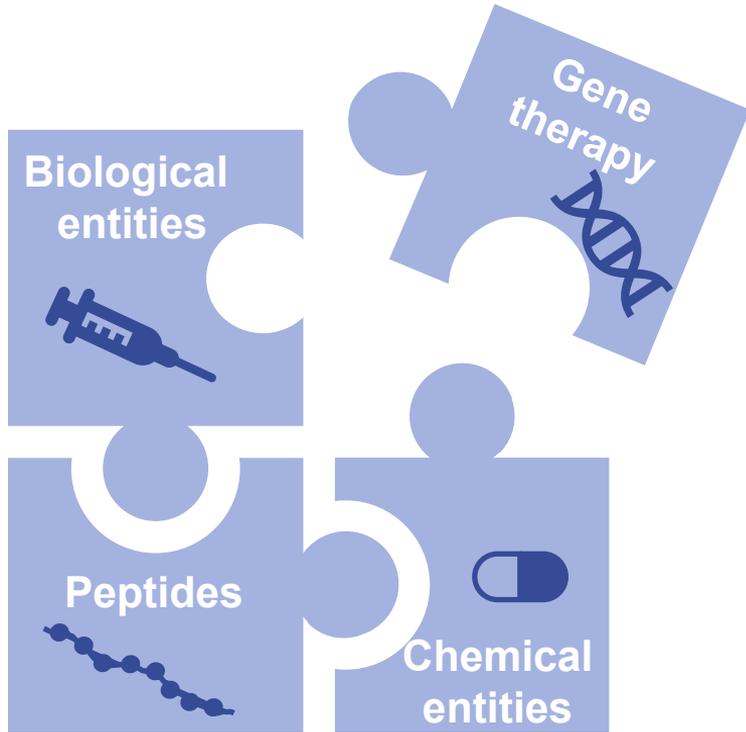
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Gene Therapy – Excellent strategic fit with UCB

Strengthens UCB's capabilities to discover & develop differentiated solutions with unique outcomes for patients



- **Chemical – UCB legacy**
- **Biological – Celltech (2004)**
- **Peptides – Ra Pharma (2020)**
- **Gene therapy:**
 - **Element Genomics (2018)**
 - **Handl Therapeutics (2020)**

Gene therapy to contribute fulfilling our Patient Value Ambition



Gene therapy to support UCB's ability to lead in CNS

UCB's Patient Value Ambition



Innovate to bring **differentiated** solutions with **unique outcomes** that help specific patients achieve their goals



Best individual patient **experience**



Access to all those who need these solutions in a way which is viable for patients, society and UCB

Gene
Therapy

Cure

Disease
modification

Symptomatic
Treatment

Gene therapy pipeline: to build-up and expand

AAV-platform & capabilities

- UCB has selected AAV for its platform in CNS
- To address **root cause** and/or **enhance targeted CNS delivery** of payload

Evolving technology



Start in CNS for initial development

Diseases in scope:

- **Neurodegeneration**
- **Movement disorders**
- **Epilepsy**

Expand in more populations*

UCB aims to build an AAV platform that could represent
~25% of UCB's pipeline in 2030



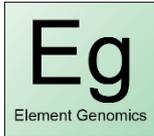
CNS: Central Nervous System

* Explore where gene therapy could offer solution for new patient populations

UCB enhances its R&D engine by adding gene therapy

Acquisition of Handl Therapeutics BV augments existing capabilities

2018



Acquisition of Element Genomics, Inc.

a discovery stage biotechnology company based in Durham, NC (U.S.), focused on finding breakthrough cures for severe human disease through the use of functional genomics

2020



Acquisition of Handl Therapeutics BV

a neurodegeneration gene therapy company with headquarters in Leuven, Belgium

next

- Evolve existing internal gene therapy capabilities
- Add partnerships & external resources
- Build gene therapy pipeline

Boosting UCB's capabilities

Acquisition

Handl Therapeutics



- **Founded in 2019**
- **Headquarter in Leuven, Belgium**
- **12 employees with strong gene therapy expertise & capabilities**
- **Focus on gene therapy to treat complex neurodegenerative diseases**
- **Combining state of the art technology platforms and scientific advances**
 - KU Leuven (Belgium)
 - Centre for Applied Medical Research (CIMA Universidad de Navarra, Spain)
 - University of Chile (Chile)
 - King's College London (UK)
- **Strong international network**
- **Access to global capabilities and expertise**

Partnership

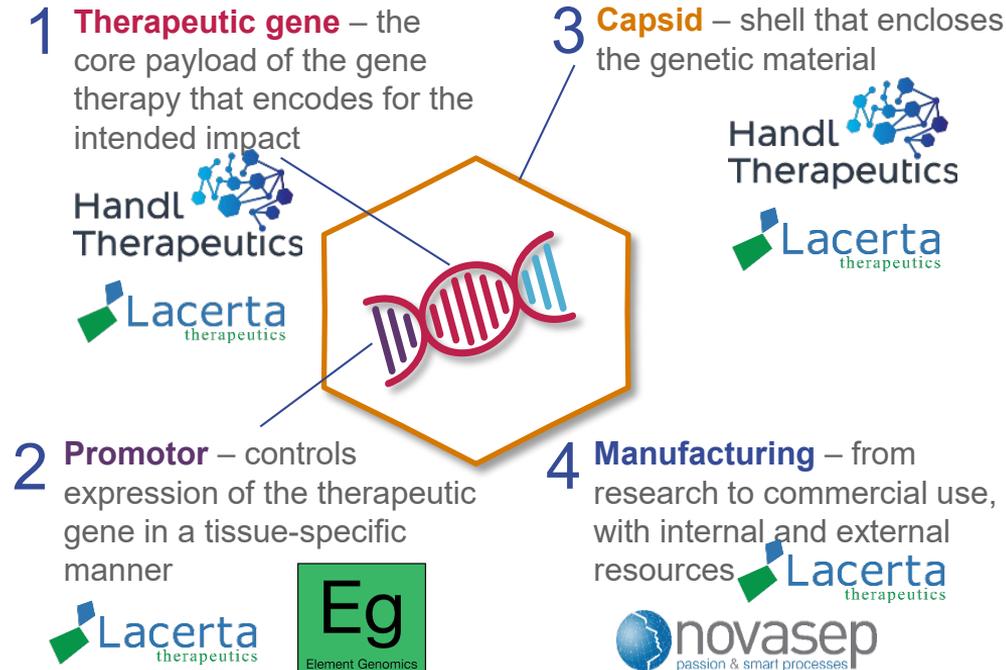


- **Founded in 2017**
- **Headquarter in Alachua, Florida (U.S.)**
- **Spin-off of University of Florida**
- **Dedicated to discovering & developing AAV-based therapies, by leveraging proprietary AAV capsid technology platform and proprietary scalable manufacturing**
- **Strong expertise in AAV-based central nervous system (CNS) targeted gene therapies**
- **Lacerta Therapeutics will lead research, preclinical activities & early manufacturing process development**
- **Enabling UCB to strengthen its ability to lead in movement disorders & neurodegeneration**

AAV gene therapy, a collaborative approach

Internal & external resources

Gene therapy consists of key components ...

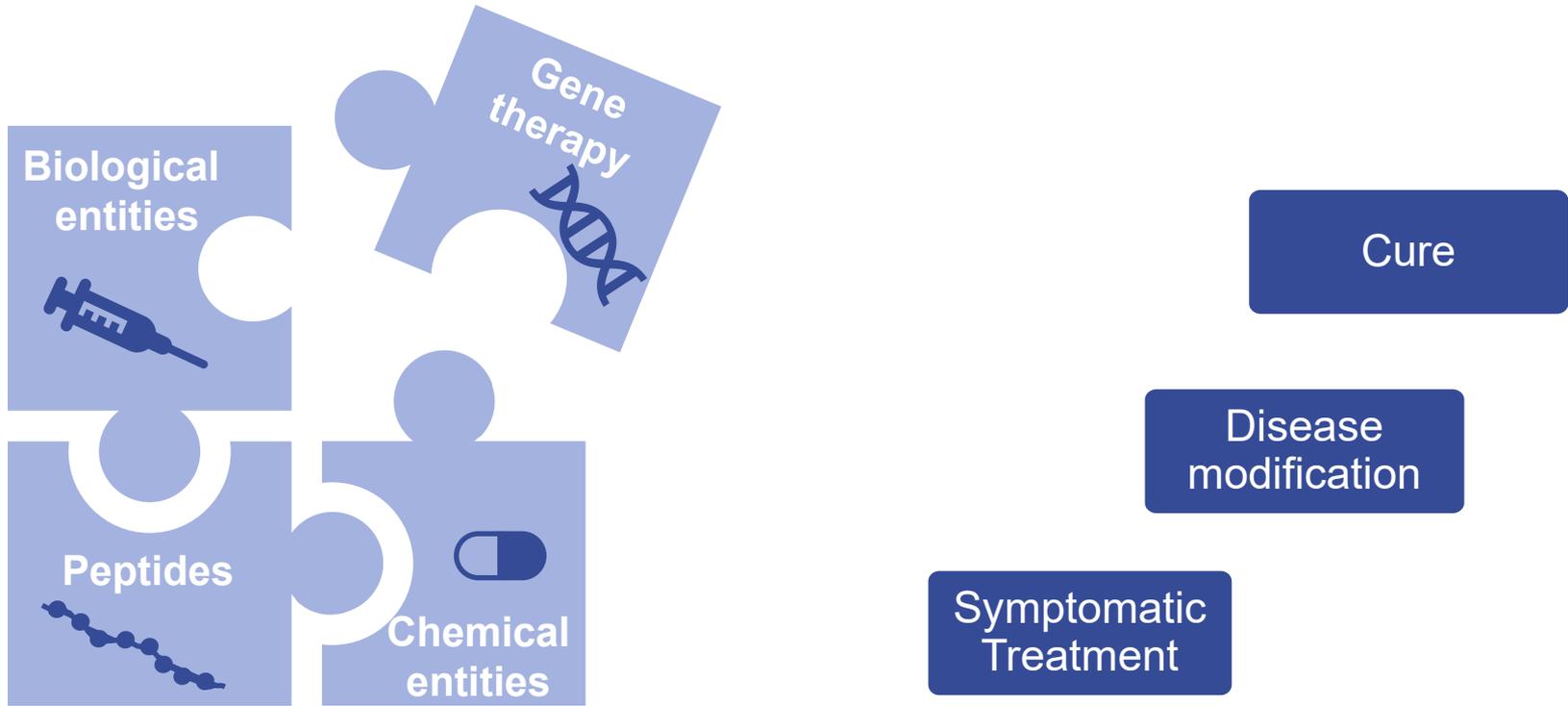


... and is a platform in itself

Vectorized antibody	Produce antibodies in a specific organ / type of cell, mostly utilized to deliver antibodies across the blood-brain barrier
Gene replacement	Replace a disease causing mutated gene with a healthy copy of the gene
Gene silencing	Regulate the gene expression of a disease causing gene to inhibit its negative effects

Gene Therapy – Excellent strategic fit with UCB

Develop differentiated solutions with unique outcomes for patients



Thank you
for your attention

Your question,
please

