

# Evotec Gene Therapy

*Adding value to our partners' research*

# Platforms & technologies for more precision and efficiency

Evotec today – 16 Sites & more than 4,400 employees



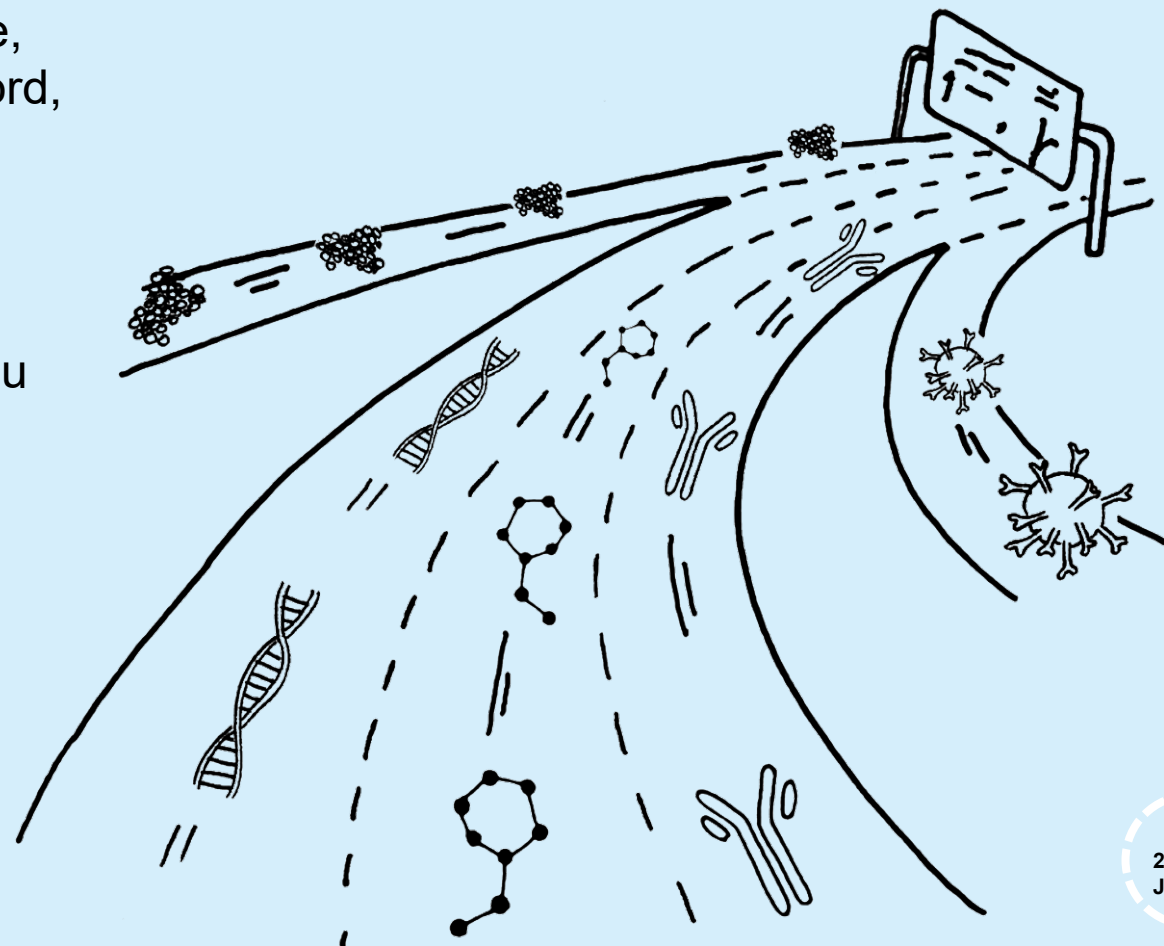
Princeton, Seattle,  
Redmond, Branford,  
Watertown  
~500 FTE



Orth an der Donau  
~40 FTE



Verona (Campus  
Levi-Montalcini),  
Modena  
~850 FTE



Hamburg (HQ),  
Goettingen (Manfred  
Eigen Campus)  
Cologne, Munich,  
~1050 FTE



Abingdon (Dorothy  
Crowfoot Hodgkin),  
Alderley Park  
~950 FTE



Lyon, Toulouse  
(Campus Curie)  
~950 FTE

# Evotec GT<sup>1)</sup> – Adding value to our partners' research

Innovative and flexible solutions from target identification to clinical candidates

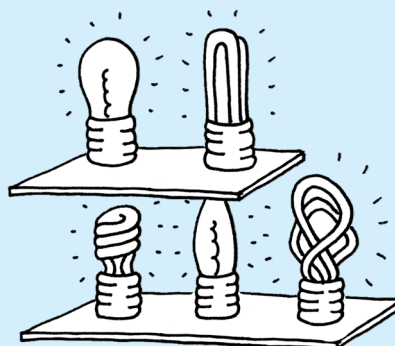
## The people

Outstanding scientists  
Strong experience in gene therapy and drug development for rare diseases  
Poised to progress pipeline assets into clinic



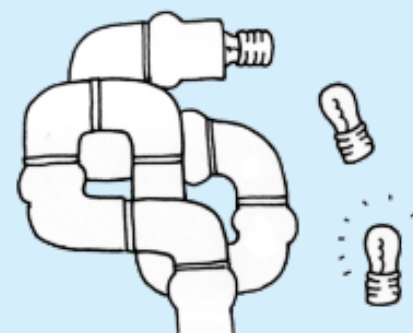
## Therapeutic area expertise

Team leverages therapeutic area insights from years of industry experience and across Evotec.



## Integrated drug discovery & development

State-of-the-art capabilities  
Best-in-class technology platforms



## Flexible deal structures

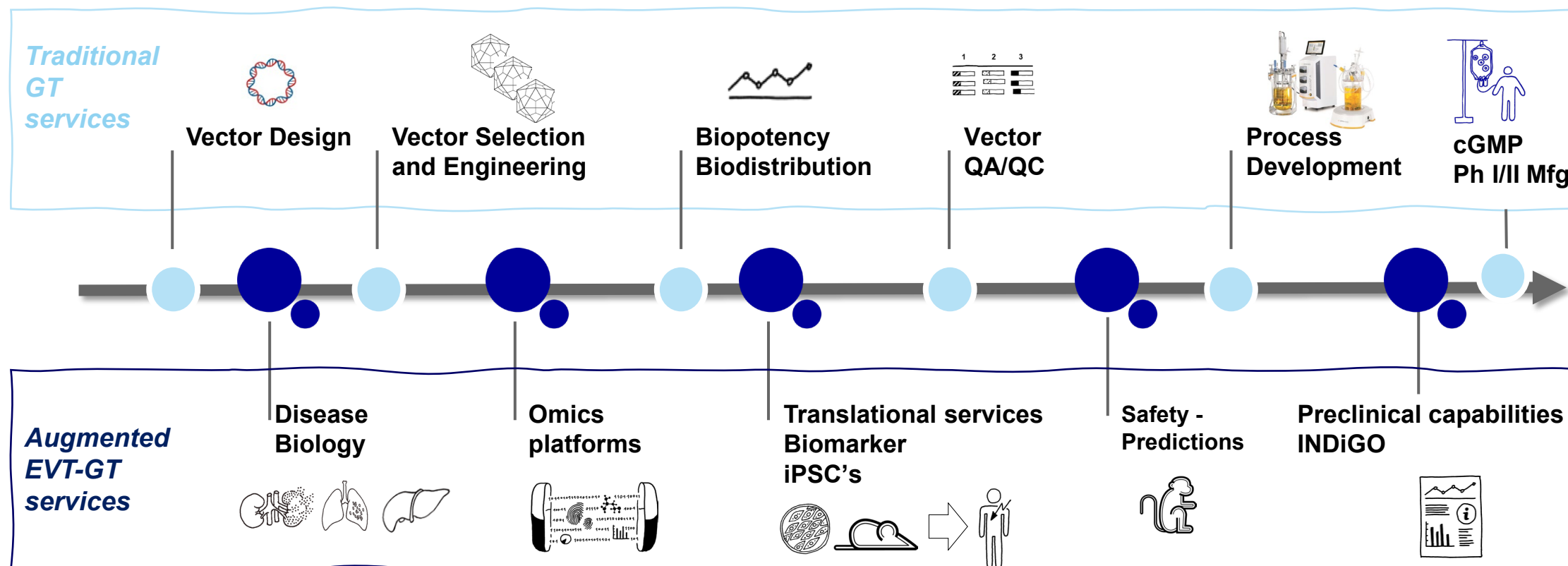
Integrated collaborations and stand-alone services



**A gene therapy platform combined with world-class drug discovery & development expertise to accelerate and maximize our partners' success**

# Early de-risking of gene therapy programs through addressing the translational gap

Unlocking the promise of gene therapy



„Competitive gene therapy development proposition, from program discovery up to entry into the clinic“

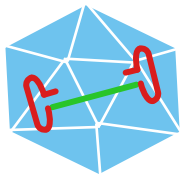
Standard viral *in vivo* gene therapy development technologies are strongly augmented by readily available, highly innovative, in-house capabilities

## In vitro Sciences

Full bandwidth of vector design and characterizations

### Vector design

- Payload
  - Transgenes
  - Antisense approaches (e.g. shRNA, miRNA)
  - Vectorized antibodies
- Codon-optimization
- Capsids
  - Natural serotypes
  - Engineered capsids
- Genome editing
- Non-viral GT



### Vector production

- Lab scale transfection & vector harvest
  - 1 to 5L benchtop bioreactor transfections
- Chromatography Systems (Äkta platform)
  - Affinity chromatography
  - Ion exchange chromatography
- Ultracentrifugation
- TFF



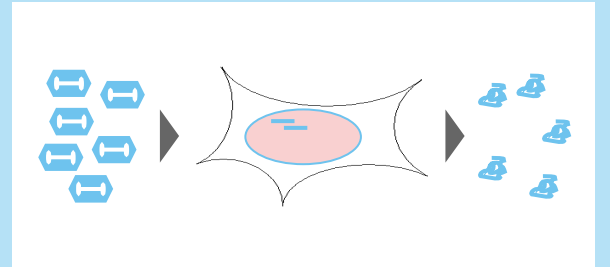
### Vector characterization

- Vector quantification
  - Real time qPCR, ddPCR
  - Fluorometry
  - ELISA capsid protein
- Vector integrity
  - Agarose gel
  - DNA Sequencing
  - SDS-PAGE and Immunoblot
- Full to empty capsid particle ratio



### Functional assays

- Cell-based assays
  - Celigo S – analysis platform
  - FACS analysis and (single) cell sorting
- Transgene expression assays
- Biopotency assays
  - Enzymatic assays
  - Functional ELISA and other biochemical assays



We cover within Evotec the entire value chain of preclinical drug discovery and development, from early discovery to lead candidate identification and preclinical development candidate characterization

# Non-Clinical Sciences

We cover a broad range of preclinical work under one roof with clear line of sight

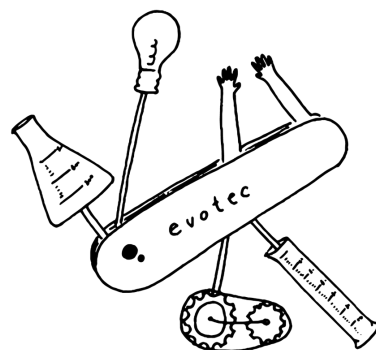
## Discovery Sciences

- Target biology / pathway: *in vivo* proof of concept studies
- Available mouse models or customised disease models
- On-site capacity: 5,000 rodents (IVC housed); AAALAC



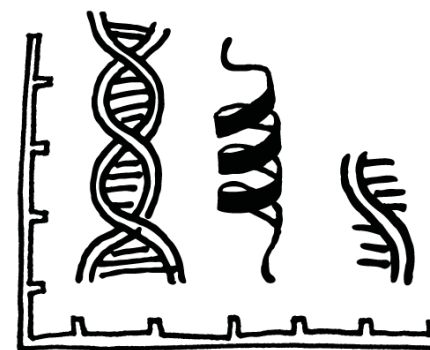
## GT Pharmacology

- Biopotency and pharmoco-dynamic readouts
- Translational pharmacology
- Immunohistochemistry and histology
- Small and large animal species



## Biodistribution / Safety

- Vector biodistribution to target and off-target organs
- *In vivo* imaging
- Combined pharmacology and safety studies
- Safety & Efficacy Biomarker ID



## Seamless Road to Clinic

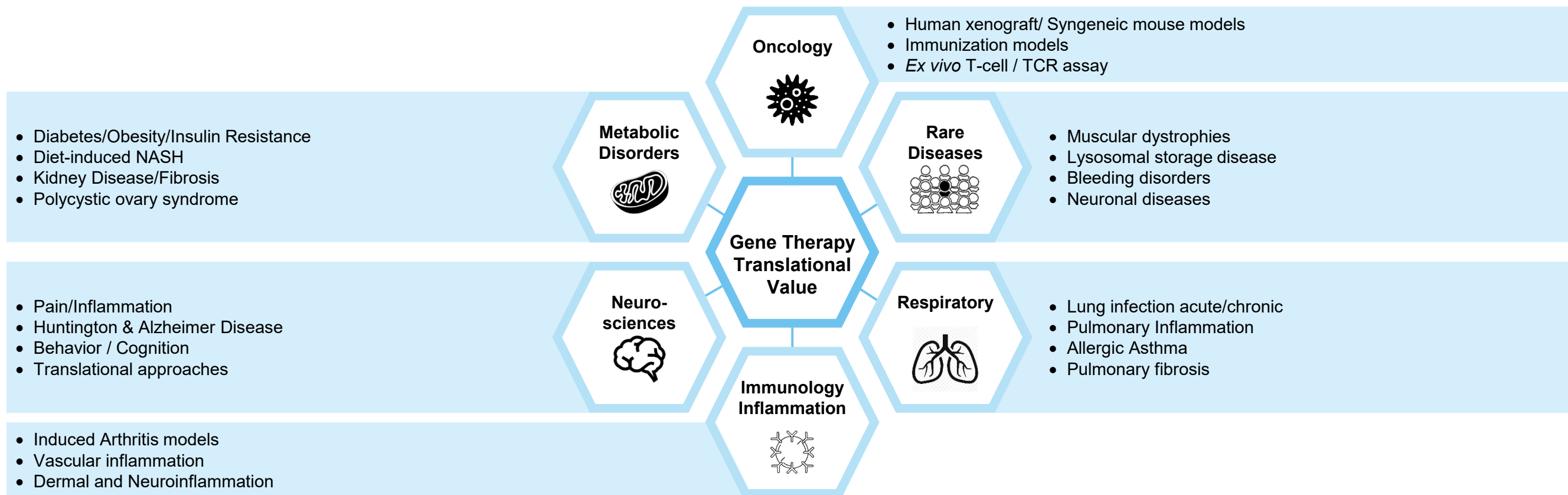
- Vector shedding study
- Immunogenicity assessment
- IND-enabling Pharm / Tox packages
- *In vivo* biopotency screening of gene therapy candidates



We integrate a multitude of complementary best-in-class technology platforms within Evotec's scientific network such as large animal models, biomarker discovery, single nuclei RNA sequencing and Evotec's computational bioinformatic powerhouse.

# Gene therapy – Translational Sciences

We offer unique synergies with therapeutic area expertise at Evotec



**Broad translational sciences & pre-clinical expertise in multiple disease areas**



#RESEARCHNEVERSTOPS

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