International Centre for Genetic Engineering and Biotechnology

Interreg Italia–Österreich | Co-funded by the European Union

PROMOS

FabCure

Transforming lifelong treatment into a one-time cure

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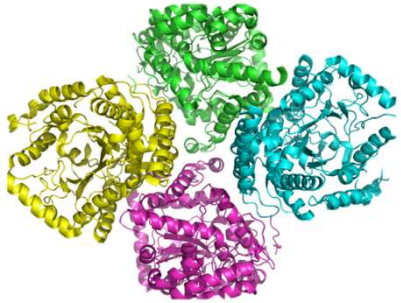
Innsbruck

FabCure

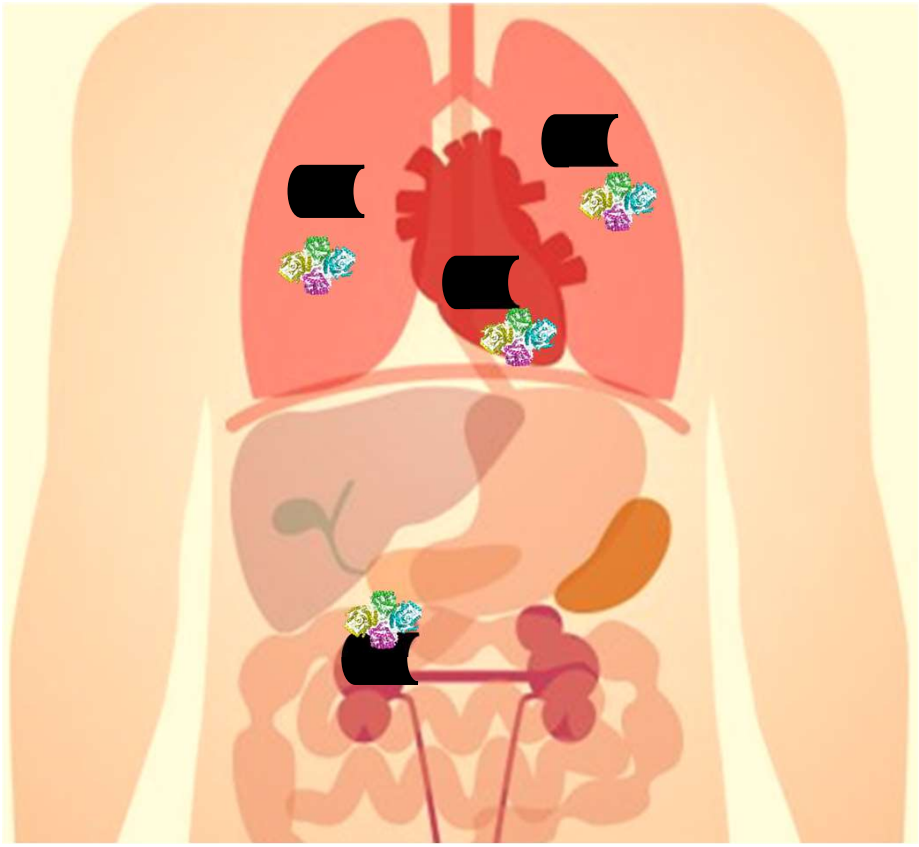
Fabry Disease: X-linked lysosomal storage disorder

Gene



Protein (GLA)

GLA: alpha-galactosidase A

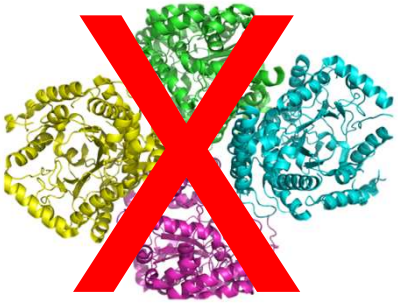


Substrate (Globotriaosylceramide (Gb3)

FabCure

Fabry Disease: X-linked lysosomal storage disorder

Mutated Gene



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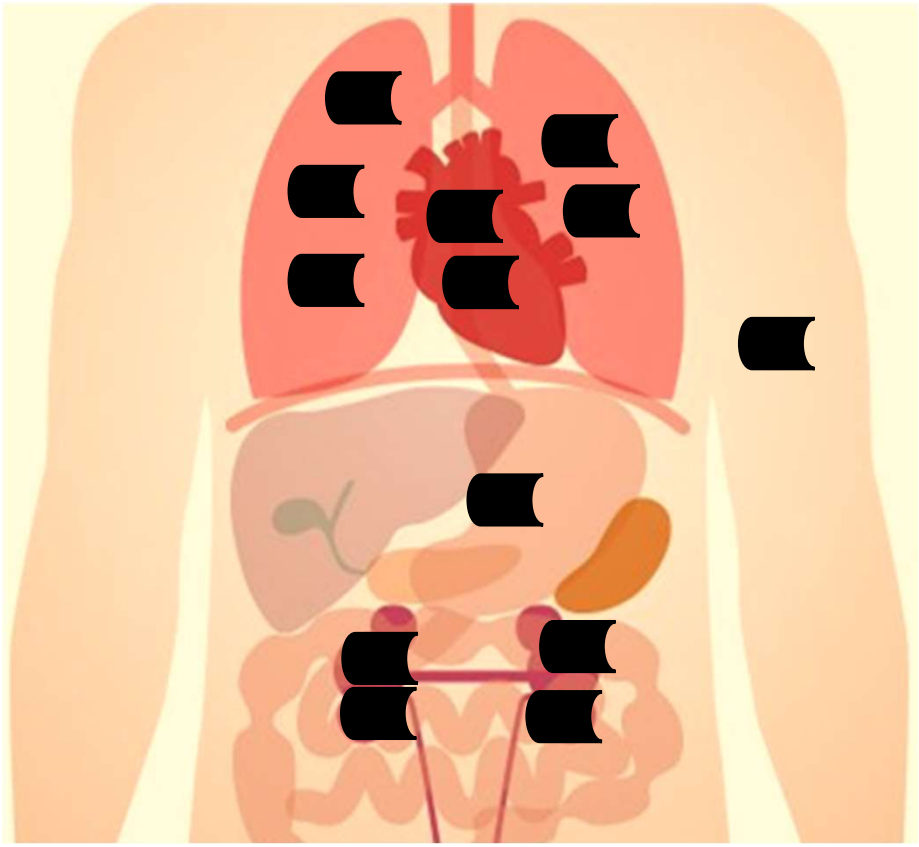


Affects the organs, and the patient may die in early life

Substrate (Globotriaosylceramide (Gb3)

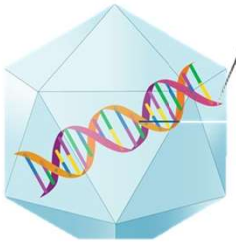
Our Solution:

Liver-targeted single-dose gene therapy for Fabry disease.

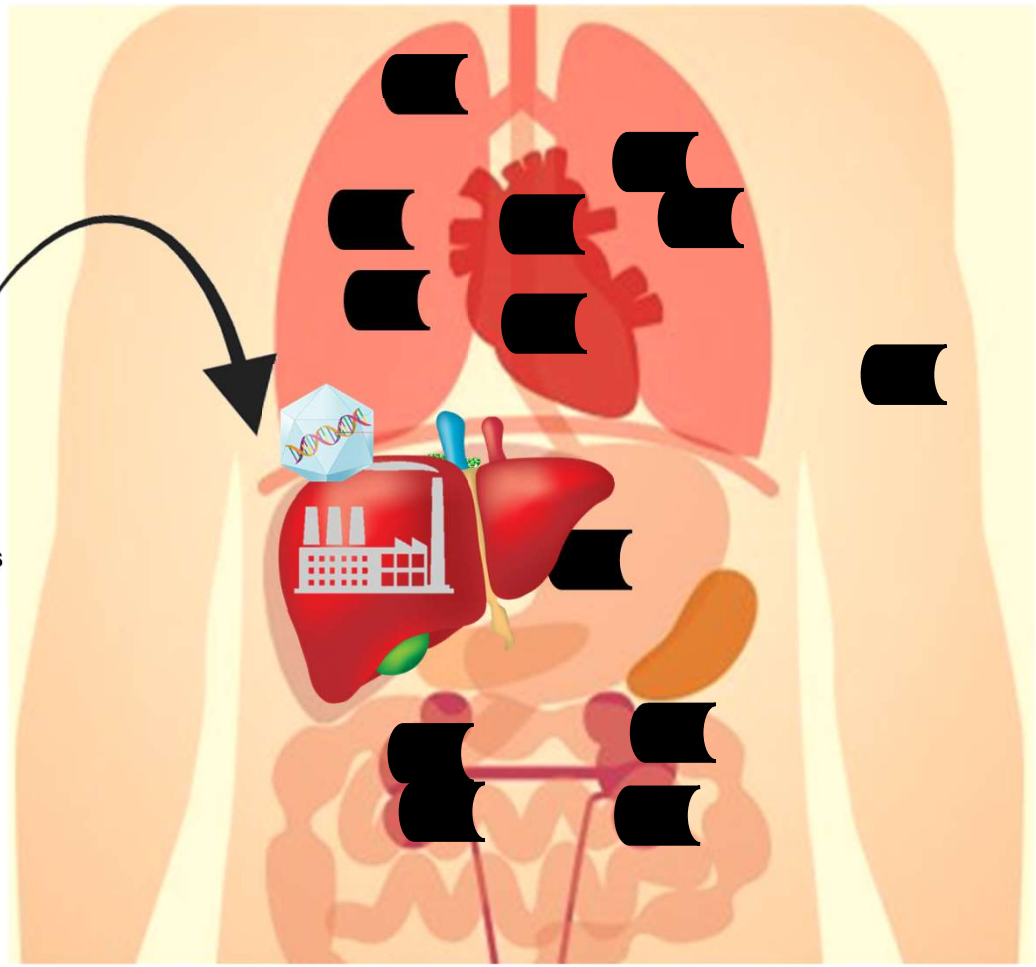
Strategy: Liver as a bio factory

Designed Highly expressing cDNA

Patent international application No. :
PCT/EP2024/055793



Adeno-associated virus with cDNA



Preclinical Study in a disease animal model ✓

Long-term efficiency and Dose study ✓

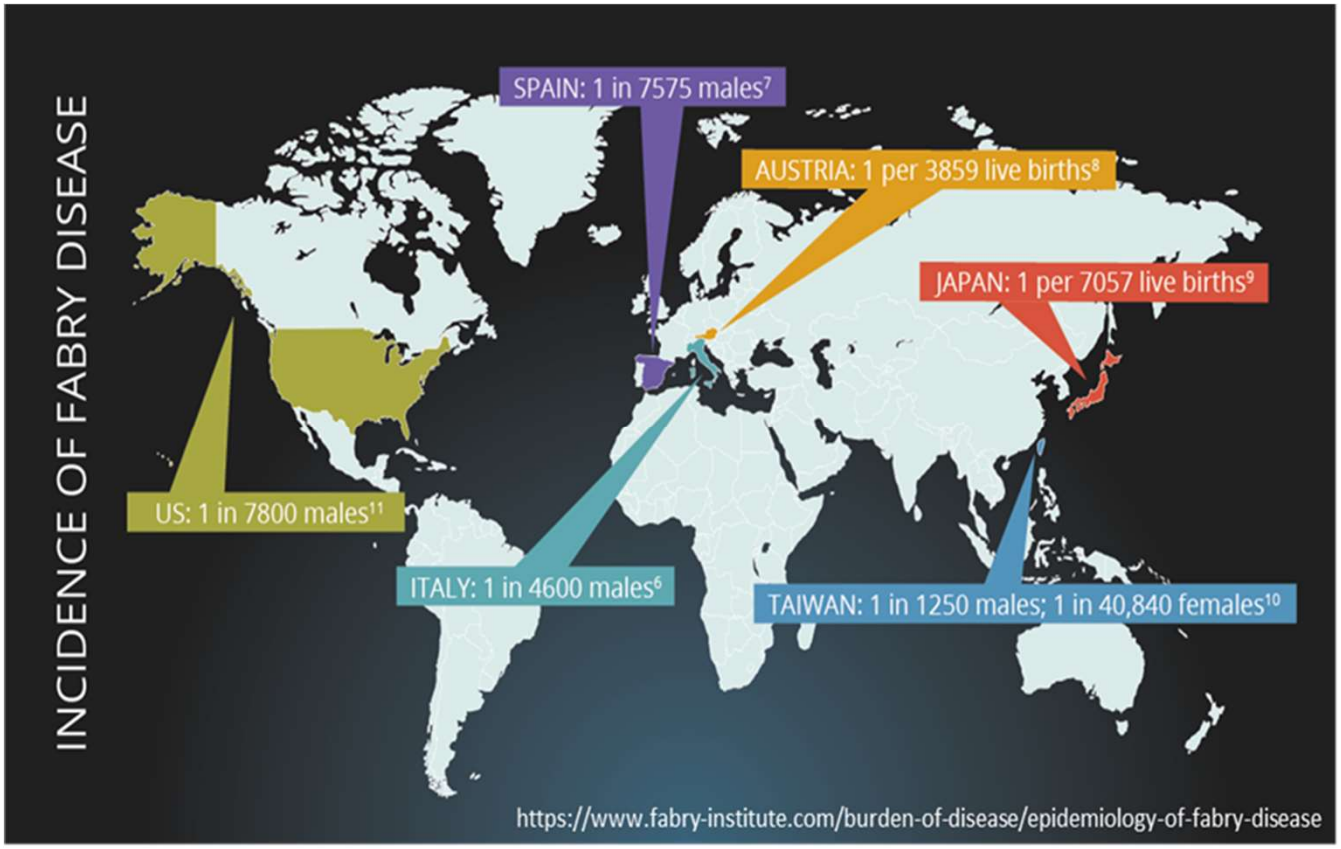
WHY?

- Cases are increasing because of the implementation of newborn screening
- Pan ethnic disease
- Incidence rates are increasing in the US, Japan, Taiwan, and the EU (Germany, France, Italy, and Spain)
- Without therapy, patients can't survive

Under diagnosed

Newborn screening

1 in 1,250 to 1 in 21,973



Available treatments are not enough!

- None of the available ones are 100% efficient
- Lifelong treatment
- Antibody against the drug.
- High cost (200k to 300k USD per year)

NO EFFECTIVE GENE THERAPY IN THE MARKET....

SIZE OF THE MARKET

Total Available Market (TAM):

~30,000+ DIAGNOSABLE PATIENTS.

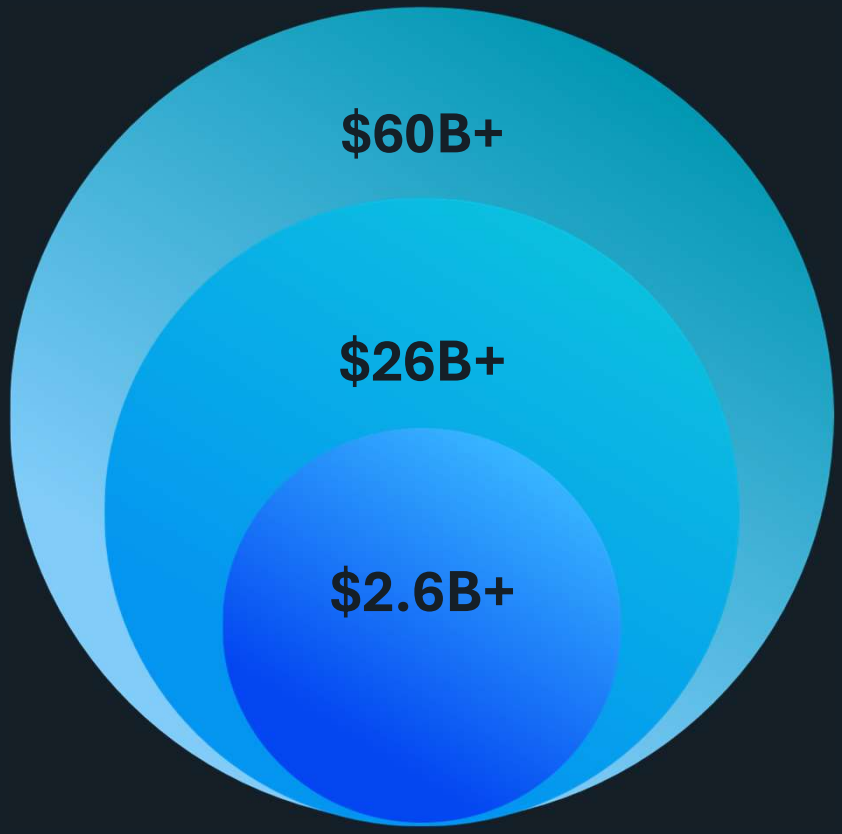
At ~ **$2M** price per dose

Serviceable Available Market (SAM):

~13,050+ PATIENTS ACROSS US/EU - **$26B**

Serviceable Obtainable Market (SOM):

10% OVER 5 YEARS = ~1305+ PATIENTS : **$2.6B**



https://www.delveinsight.com/report-store/fabry-disease-epidemiology-forecast-insight

https://www.fabrydisease.org/about-fabry-disease/how-many-people-have-fabry-disease?utm_source=chatgpt.com

Looking For....

Looking for **industries/Investors** to collaborate with to translate the therapy to clinical applications. (We have collaboration with hospitals to arrange patients in Italy)

Regulatory path planning

cGMP low-scale manufacturing for clinical trials.

Your feedback.

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Thank You..

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