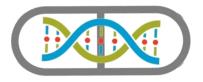
# EUKARYS

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EuroQuity, 9th June 20

#### The Synthetic Gene Therapy Company



#### EuroQuity, E-pitch session June 9, 2020

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## **PROJECT IDEA**

To develop a pipeline of synthetic gene therapies – a novel therapeutic approach for gene compensation, also adaptable to vaccination - able to fill unmet needs of existing gene therapy approaches

# PROBLEM

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- Gene therapy technologies existing to date, primarily based on recombinant viruses, have many drawbacks:
  - Safety: insertional mutagenesis,
  - Immunogenicity: readministration,
  - IFN response: tolerance,
  - Gradual collapse of efficiency,
  - Packaging capacity: gene length,
  - Tissue-specific targeting,
  - Very expensive production process,

#### Limited to monogenic diseases.

\* Other technical developments are on going for synthetic gene vaccination

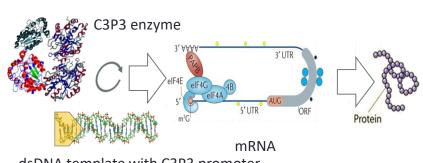
# SOLUTION

Synthetic gene therapy is made possible by two technologies invented and developed by Eukarÿs\*

(1) Synthetic DNA produced with Möbius<sup>®</sup> system



C3P3<sup>®</sup> artificial expression system



dsDNA template with C3P3 promoter

C3P3-G1: WO 2011/128444 →2031 - C3P3-G2: EP2018/070479→2037 -C3P3-G3: on-going

## COMPETITION

#### Viral gene therapy:

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 Main competitor with most products registered in the last 5 years,
 Competitors: Novartis, Pfizer, Spark, UniQure, BluebirdBio, Immune Design, Audentes...

The other approaches have not demonstrated their effectiveness:

Synthetic mRNA (except for vaccination),

• Viral gene therapy with plasmid DNA.

Main competitor for synthetic gene vaccination: synthetic mRNA (Moderna, Curevac, BioNTech)

## **EXPECTED ADVANTAGES**

- Safe: without risk of insertional mutagenesis,
- Well-tolerated: without CpG dinucleotides
- Immuno-masked: for the enzyme C3P3,
- Transient and readministrable,
- Expression and/or inhibition of one or more genes,
- No known limits in gene length,
- GMP-compliant with reduced production costs,
- Conjugated with small molecules or others technology for synthetic gene vaccination

#### **MARKET OPPORTUNITY & TRACTION**

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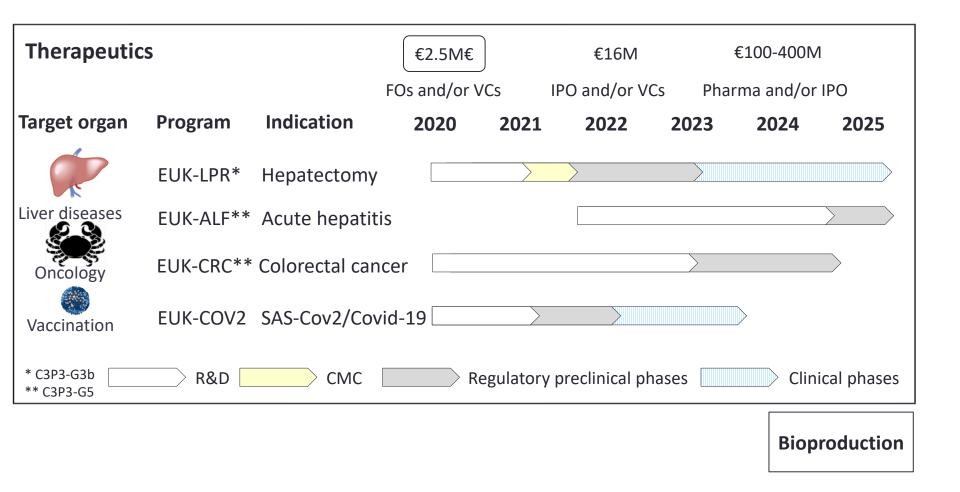
• Gene therapy is rapidly growing market:

- Gene therapy market US\$9.7 billion, with CAGR of 36% →US\$45 billion in 2025,
- FDA: 10-20 CGT predicted to be approved per year by 2025, >200
  CGT IND expected to be submitted by 2020,
- Estimates to be taken with caution: e.g. commercial failure of Glybera (Uniqure).

Nucleic acid vaccination accounted for US\$ 8.99 Mn in 2019, and was expected to grow at a CAGR of 61.5% ... before the Covid-19 pandemics

#### **REVENUE MODEL**

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Special Advisory

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Historical investor

**Pierre Grange** 

Immobilier Trust

President of IDEVI

Historical investor

Ecole des Ponts et Chaussées,

Pr. Philippe Kourilsky

Professor of Immunology

Immunology Network

Professor Collège de France

Former President of SORIF (Vinci

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Administrator

Po – EcoFi)

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Scientific Advisory Board

University of Paris II Panthéon-Assas

Institute of Political Studies (Sciences-

## TEAM

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Executive Board of Directors



Philippe Jais

Executive President &CSO, Founder of Eukarÿs Inventor of all technologies MD (Hepato-gastroenterology), PhD (Molecular Genetics) Pharma and Biotech



Patrice Vernet Experienced entrepreneur, Former elected representative of Essonne Historical investor



**Pr. Pierre Chambon** Emeritus Professor of Biochemisty IGMC and ICS, Strasbourg Professor Collège de France Albert Lasker Award Member of the



**Dr. Daniel Metzger** Research Director at CNRS IGMC and ICS, Strasbourg Grand Prix Jules Martin Prix René Turpin Member of European Academy of

Cancer Sciences and EMBO

French, Swedish and US National

Academy of Sciences









**Pr. Christian Bréchot** Professor of Hepatology Paris Descartes University Former Former CEO of INSERM Former CEO of Paris Pasteur Institute President Global Virus Network











#### **Guillaume Prunier**

Special advisor Polytechnique/Mines Areva, head of innovation French Ministry of Economy, former CEO Flaminem Deputy Executive Director, INRIA

#### Pr. Dominique Franco

Administrator Emeritus Professor of Surgery University Paris XI Expert in liver surgery Pioneer of liver transplantation President of the French Academy of Surgery

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Administrator Institute of Political Studies, Sorbonne Law School, Edmond de Rothschild, BNP Paribas, SFAF Eponyme Partners. President of PPRS

#### Pr. Jean-Charles Duclos-Vallée

Professor of Hepatology University Paris XI Expert in severe acute and chronic liver diseases Head of the DHU Hepatinov, Paris

#### **Dr. Steve Pascolo** PhD, ENS of Paris Former CSO of Curevac Pioneer of mRNA uses for vaccination and immunomodulation Head of Immunology, University Hospital of Zürich

## FINANCIALS

Economic model dependent on investment by private investors, i.e. business angels, family offices and VCs.

Objectives:

- To reach to clinical POC with our most advanced synthetic gene therapy,
- Followed by co-development agreement with Pharma,
- Expected in 2024.
- Estimated financial needs: €18 million to reach final objective in several tranches.

## INVESTMENT

- Amount sought: €2.5 million,
- Target period: 3Q or 4Q 2020,
- Uses of the funds:
  - Recruitment of key people: CEO, CFO part time, R&D staff,
    R&D: CMC of synthetic DNA of EUK-LR up to submission to regulatory agencies,
     Intellectual Property

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THERAPY

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