DNAQOR

Bringing gene therapy to the failing heart

Company Overview

Forward-Looking Statements

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DiNAQOR

OUR MISSION

To bring gene therapy to the failing heart

DNAQOR

Heart failure is a serious disease and a leading cause of death

30M

people globally suffer from heart failure

50%

of heart failure patients die within 5 years of diagnosis

Heart failure in the US costs an estimated

$30B

annually

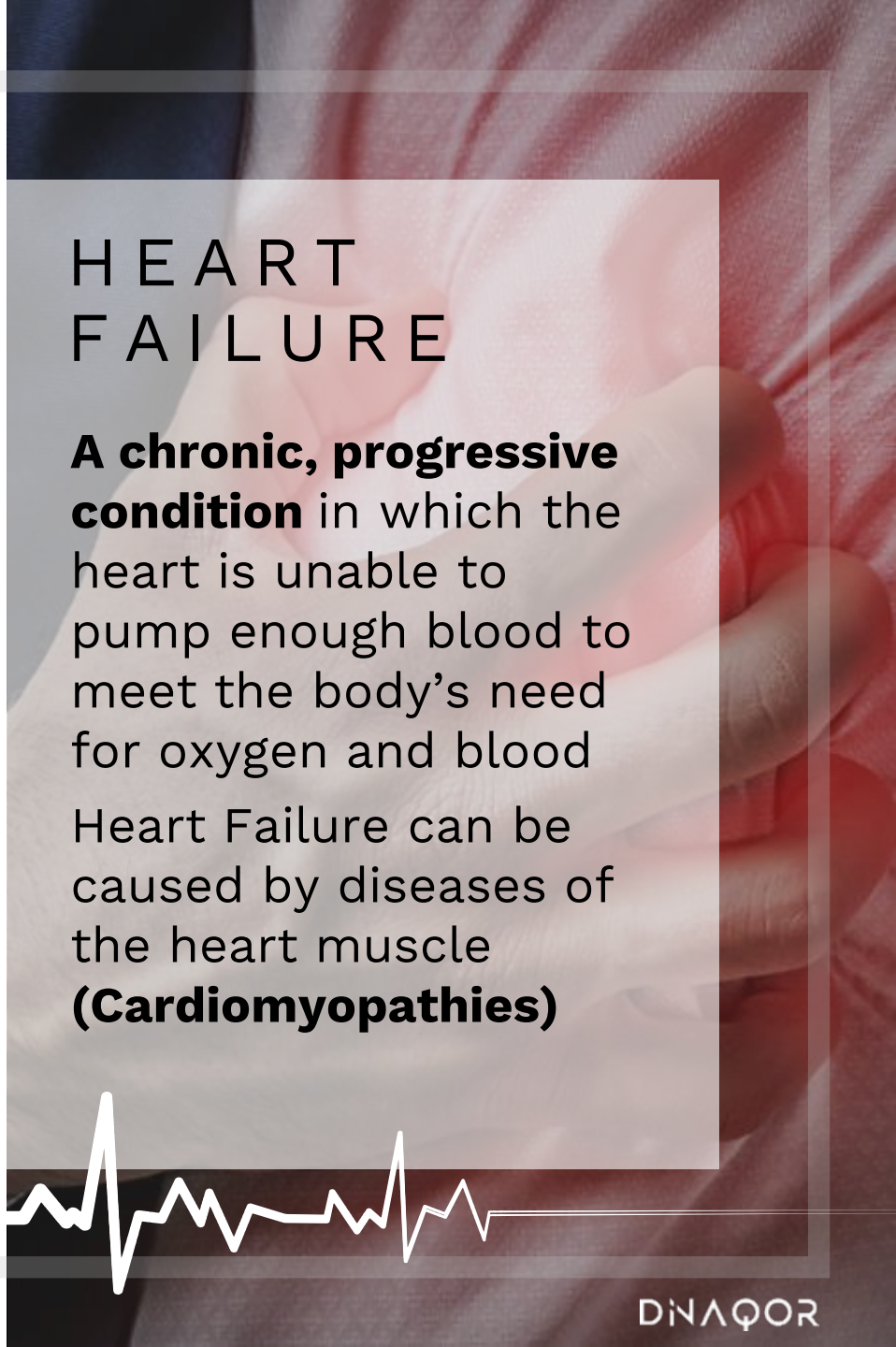
Heart failure in Europe equals an estimated

1-2%

of healthcare costs

46%

Estimated increased prevalence of heart failure in the US by 2030

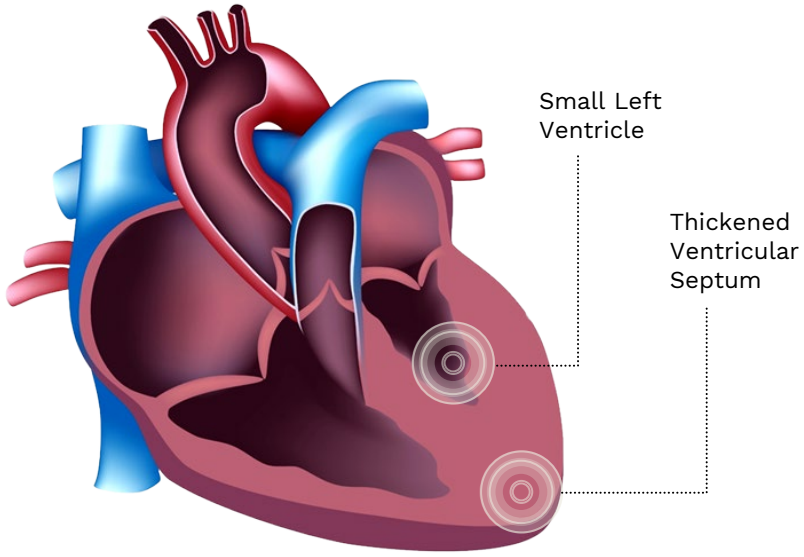


Sources: CDC, DiNAQOR research

Half of the cardiomyopathies are monogenic diseases

Hypertrophic Cardiomyopathy (HCM)

1:500 people



Up to

60%

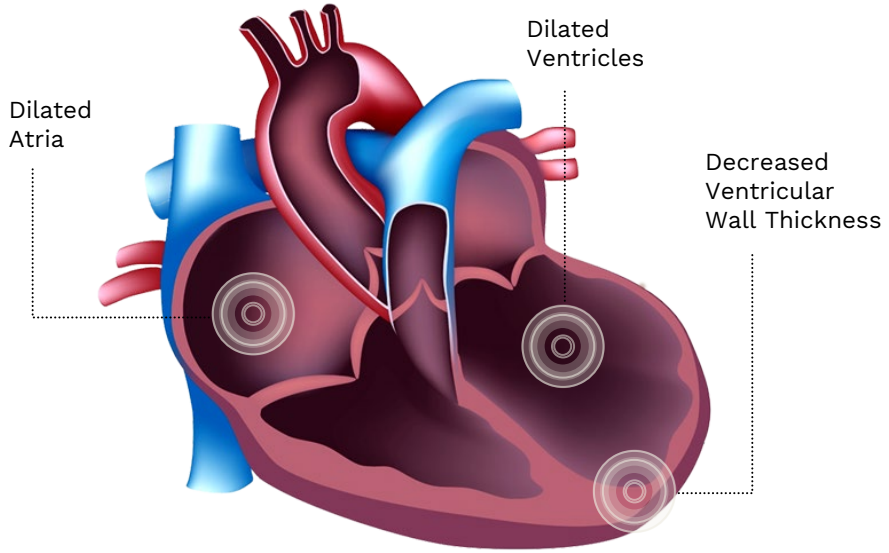
of Cases of Genetic Origin

~800 thousand

1.7M
patients in US and EU

Dilated Cardiomyopathy (DCM)

1:250 people



Up to

35%

of Cases of Genetic Origin

~900 thousand

Source: DINAQOR research

DINAQOR

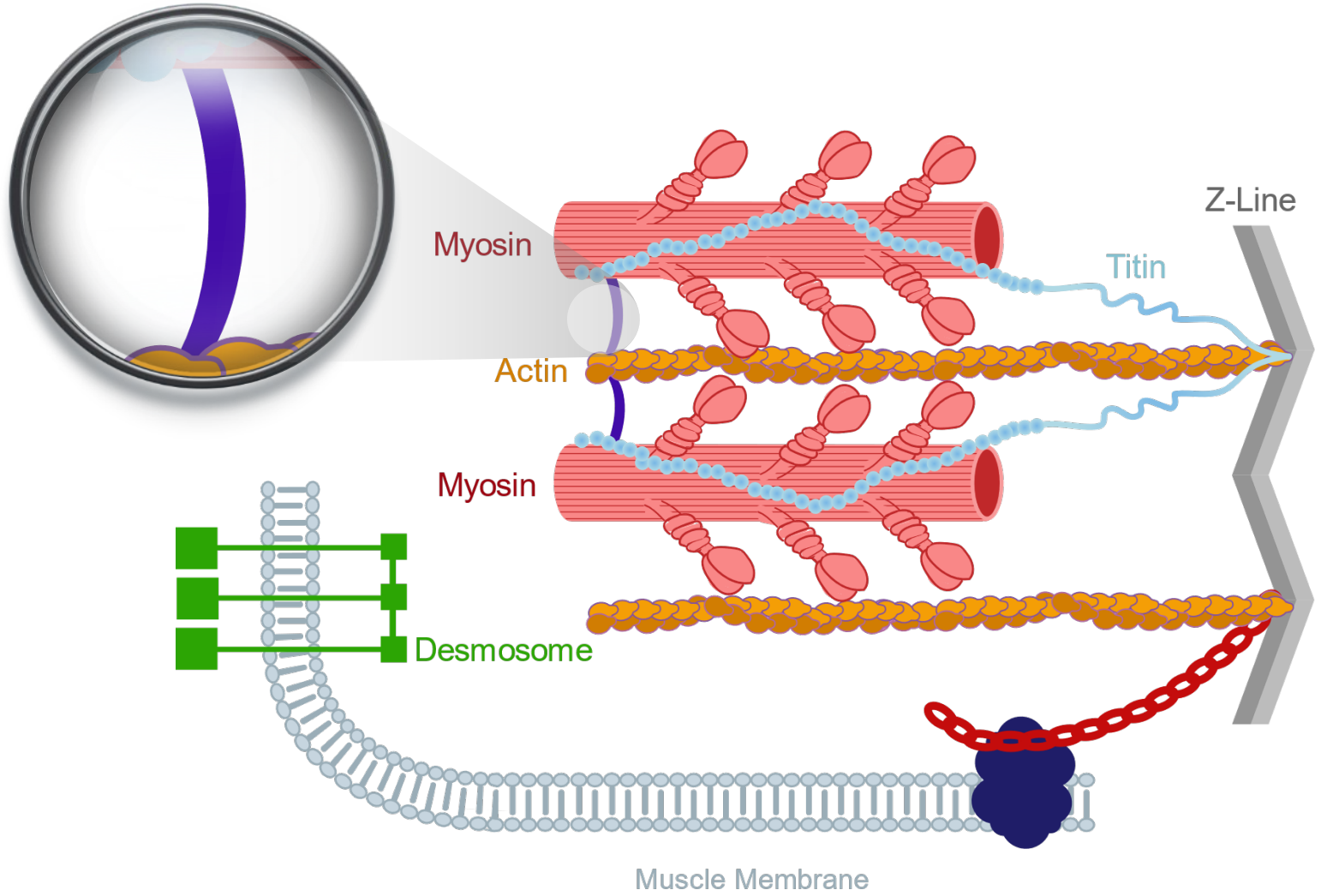
Mutations of *MYBPC3* found in approximately 40% of genetic HCM

MYBPC3
Gene

cMyBP-C
Cardiac Myosin-binding Protein C

- "Molecular brake" slowing down Actin sliding velocities
- Loss decreases maximal force development
- Mutation found in approximately 40% of genetic HCM cases

~310,000 patients in US/EU, 25,000 with Heart Failure



PLATFORM

We have the platform and the people to implement our mission

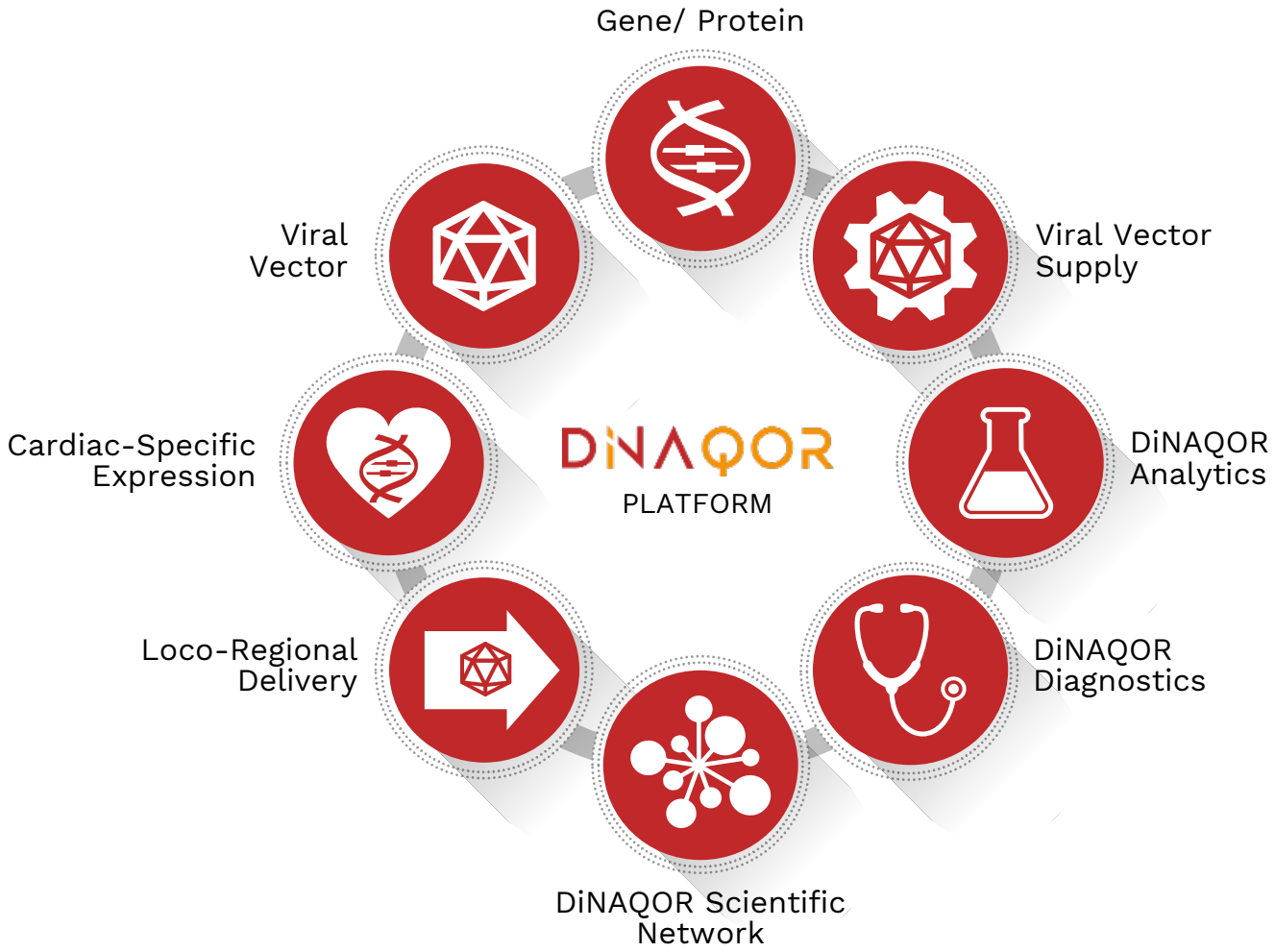
- SCIENCE AND TECHNOLOGY
- PRODUCT AVAILABILITY
- PATIENT ACCESS

PEOPLE

- EXPERIENCED CLINICIANS
- IMPRESSIVE SCIENTIFIC TRACK RECORD
- BUSINESS BUILDERS

DiNAQOR

Our platform provides all elements to go to clinic now



Our modular technology platform accelerates path to clinic by allowing faster, more predictable development

Our platform accelerates the path to the clinic

Gene/Protein	Lead program addresses mutations of cardiac myosin-binding protein C gene (MYBPC3) causing cardiomyopathies in children and adults
Viral Vector	A non-replicating adeno-associated virus (AAV9) delivers the genetic material to a patient's heart cells
Cardiac-Specific Expression	A highly heart-specific human cardiac troponin T promoter activates the transgene and ensures selective expression in cardiac tissue
Loco-Regional Delivery	A loco-regional delivery system will allow to route gene therapy directly to the cardiac muscle minimizing risks and reducing the costs

DNAQOR

We delivered proof of concept for the gene-virus-promoter construct

1

nature COMMUNICATIONS

Mearini, G. *et al. Mybpc3* gene therapy for neonatal cardiomyopathy enables long-term disease prevention in mice. Nat. Commun. 5:5515 doi: 10.1038/ncomms6515 (2014).

2

Molecular Therapy Nucleic Acids

Prondzynski, M. *et al.* Evaluation of *MYBPC3* trans-Splicing and Gene Replacement as Therapeutic Options in Human iPSC-Derived Cardiomyocytes. *Mol. Ther. Nucleic Acids 7* (2017).

Mybpc3 gene therapy **dose-dependently corrects protein levels**

Dose-dependent suppression of mutant mRNA by *Mybpc3* gene therapy

Mybpc3 gene therapy **incorporates exogeneous cMyBP-C protein correctly into the sarcomere**

Mybpc3 gene therapy dose-dependently **prevents hypertrophy and dysfunction**

Cardiac-specificity of the **TNNT2 promoter lacking off-target expression**

MYBPC3 gene transfer **corrects hypertrophic phenotype**

Source: DiNAQOR

DiNAQOR

AAV9 vector consistently shows strong cardiac tropism

Human

SMA

- High tropism to the human heart

Mouse

Pompe

- 10x diaphragm
- >2x skeletal muscle

SMA

- >20x skeletal muscle
- 5x liver

AAV9

Cardiac Tropism

NHP

MPSIIIB

- 10x skeletal muscle

Pompe

- 40x skeletal muscle

Dog

DMD

- >2x skeletal muscle

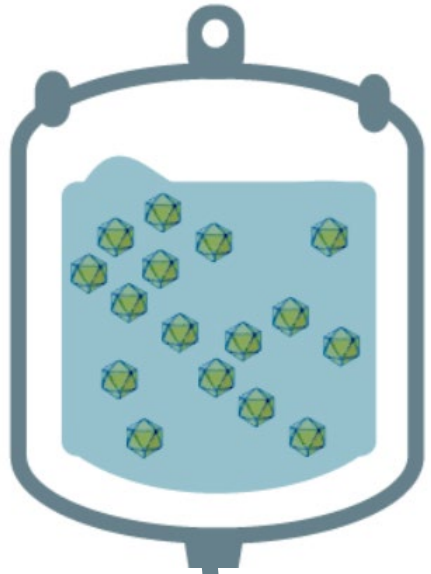
Sources: Mouse: Falk *et al.* 2015, Pacak *et al.* 2006, Meyer *et al.* 2015. Dog: Yue *et al.* 2015. NHP: Murrey *et al.* 2014, Tarantal *et al.* 2016, Pacak *et al.* 2006. Human: www.fda.gov/media/127961, Kaspar *et al.* WMS 2019 WMS 2019.

DNAQOR

We are developing an innovative loco-regional delivery system for the heart

Route gene therapy directly to the cardiac muscle maximizing biodistribution and transduction of the cardiac cells.

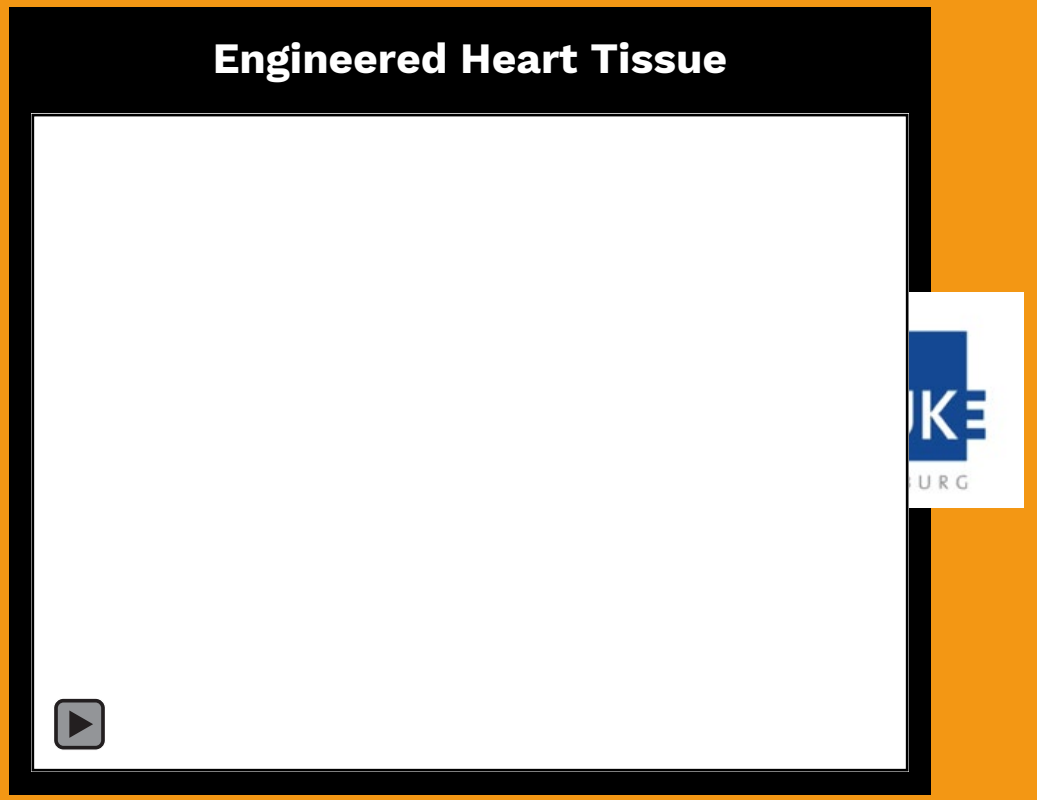
Minimize potential adverse effects of systemic gene therapy delivery and lower the costs.



Patent Pending.

We are testing our innovative gene therapies in Engineered Heart Tissue

We established a collaboration with the Department of Experimental Pharmacology and Toxicology of the University Medical Centre Hamburg-Eppendorf, Germany to test our innovative gene therapies in human induced pluripotent stem cell-derived engineered heart tissues



We are targeting strategic partnerships with leaders in cardiology and gene therapy

DNAQOR

Established strategic partnership with Lonza for GMP production

Lonza

Timely delivery of near-term viral vector quantities to accelerate time to phase I

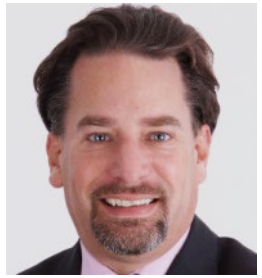
Manufacturing technology to deliver long-term clinical trial and commercial viral vector quantity needs

Process development and AAV expertise to optimize AAV9 platform for tropism and immunogenicity

DiNAQOR established a strategic collaboration with Lonza, one of the world's leading manufacturers of adeno-associated viral gene therapy vectors

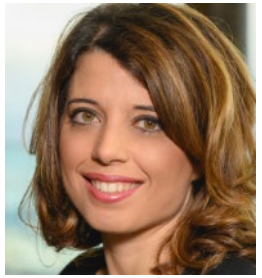
DiNAQOR

Experienced leadership with deep cardiology and gene therapy expertise



JOHANNES HOLZMEISTER, M.D.
CHAIRMAN & CEO

- Founder, Chairman & CEO of multiple biotech ventures, internationally recognized cardiologist
- Led interventional device group for heart failure patients at the University Hospital, Zurich



VALERIA RICOTTI, M.D.
EVP, CHIEF MEDICAL OFFICER

- Leading expert in design and execution of clinical trials, including gene-therapy, >40 articles
- Medical Director, BioMarin Pharmaceutical; Director of Translational R&D, Solid Biosciences; Honorary clinical lecturer UCL

PROF. THOMAS VOIT, MD, PhD
CHIEF SCIENTIFIC OFFICER

- Internationally recognized authority on AAV-based gene therapies, >300 articles and 20 patents
- Professor, Marie Curie/ Sorbonne University; Director NIHR Great Ormond Street Hospital Biomedical Centre

RETO WITTWER, M.L.
CHIEF FINANCIAL OFFICER, BOARD MEMBER

- Experienced CFO and pharma leader, Chambre Fiduciarie Suisse certified tax expert
- 20+ years experience in pharma and biotech, executive roles at Magna, Sandoz, Novartis

HANNS E. ERLE, PhD
CTO & HEAD OF GLOBAL SUPPLY

- Global manufacturing & product supply expert and experienced pharma leader
- 30+ yrs. experience in pharma and biotech, Executive roles at Merck, Aventis, Hoechst

DNAQOR

Scientific advisory board: World-leading experts in heart failure and gene therapy research

THOMAS VOIT, MD, PhD
CHAIRMAN

University College London, Professor of Pediatrics, NIHR Great Ormond Street Hospital and UCL Institute of Child Health, Director of the Biomedical Research Centre

EDUARD AYUSO, DVM, PhD

University of Nantes, Scientific Director of The Translational Vector Core

LUCIE CARRIER, PhD

University Medical Center Hamburg-Eppendorf, Professor of “Functional Genomics of Cardiomyopathies”

PERRY ELLIOTT, MD

Bart’s Heart Centre, Clinical Lead of the Inherited Cardiovascular Disease Unit, University College London, Professor of Cardiovascular Medicine

THOMAS ESCHENHAGEN, MD

University Medical Center Hamburg Eppendorf, Institute Director, Center for Experimental Medicine and Institute of Experimental Pharmacology and Toxicology

CALUM A. MACRAE, MD, PhD

Brigham and Women’s Hospital, Vice Chair, Scientific Innovation, Harvard Medical School, Associate Professor, Cardiovascular Medicine

FEDERICO MINGOZZI, PhD

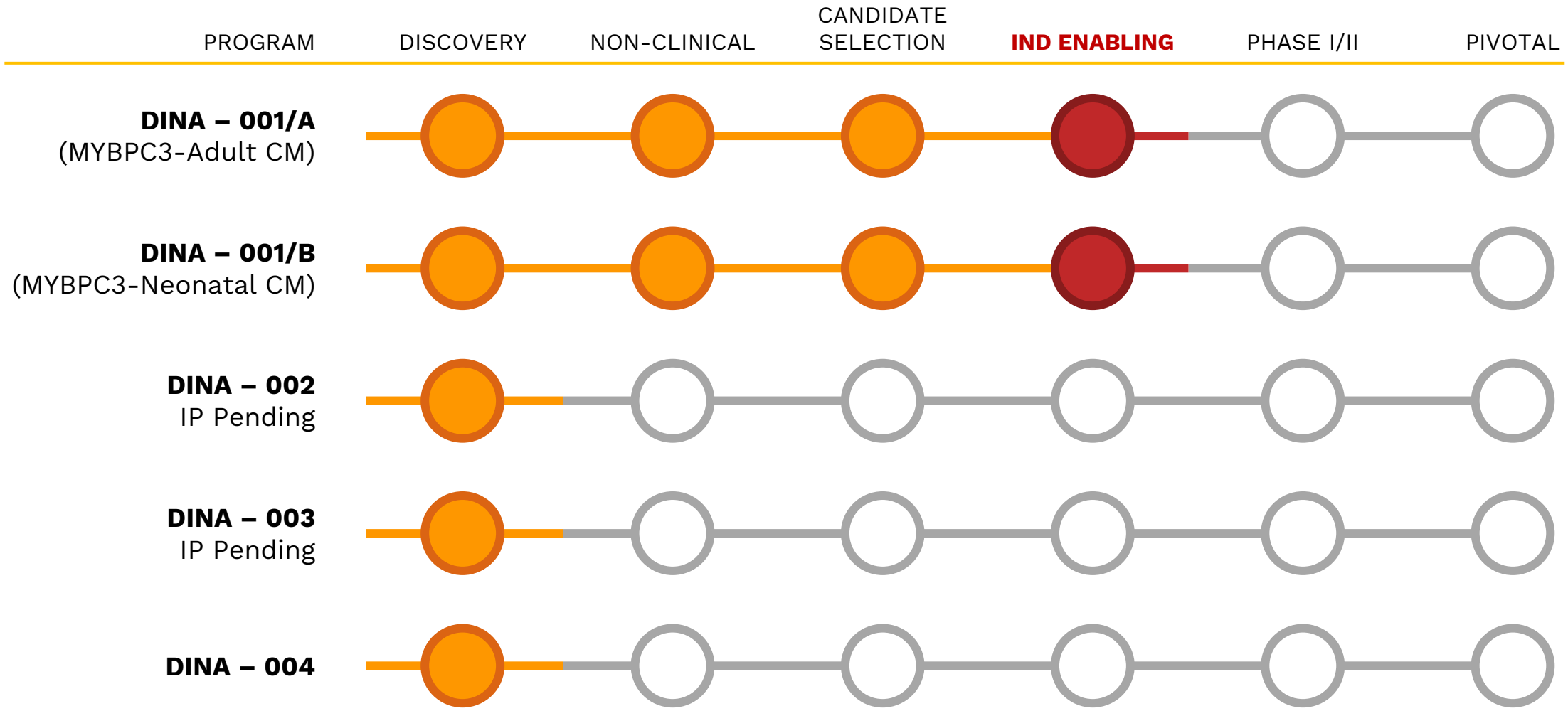
Spark Therapeutics, Chief Scientific Officer

SCOTT SOLOMON, MD

Harvard Medical School, Brigham and Women’s Hospital, Professor of Medicine, The Edward D. Frohlich Distinguished Chair

DNAQOR

Strong pipeline to address a range of genetic cardiomyopathies



Source: DiNAQOR

DiNAQOR is uniquely positioned to deliver cardiac gene therapy now

Optimized distribution of construct in target tissue due to effective dose*, and high tropism of AAV9 capsid to the heart

On-target expression of transgene due to high cardiac specificity of troponin T promoter

Anticipated long-lasting effect of transgene in postmitotic cardiomyocytes

Low immune response and reduced costs with loco-regional gene delivery

* learned from current clinical GT trials*in SMA, DMD and XLMTM

DiNAQOR


DNAQOR
Pioneering the route of advanced therapies for the human heart
DNAQOR