Innovative therapies for rare disease - 2018
Our Mission: Serving patients and their parents by developing innovative therapies for rare diseases.

Rian, Ireland

Moric, Germany

Lynn, The Netherlands

Sebastian, USA
Executive management and board of Directors

Stephane van Rooijen, MD MBA
Chief Executive Officer

Frédéric Legros PhD, Msc
Chief Operating Officer

Chris Freitag MD
Chief Medical Officer

Formerly CEO Confo Therapeutics, Head of Genzyme’s European Cardiovascular Business Unit, ViroPharma

Formerly Vice President and Head of Business Development Valneva / Head of TTO Institut Pasteur Paris

Formerly head of clinical development Debiopharm, Shire, Roche

Leen Thielemans
Ir, PhD, MSc
Head of Development

Belinda Cowling
PhD
Head of Research

Managing Partner at 2 Bridge, formerly J&J, Director Translational Sciences at Shire-Movetis

Former thematic leader INSERM, Department of Translational Medicine of neuromuscular diseases, IGBMC
What makes Dynacure unique

Build leader in rare diseases

Build pipeline

Bring innovative treatments to

Leverage validated technology platforms

Other platforms/modalities
Clinical Development objectives

• Obtaining the broadest label possible for X Linked CNM patients and Autosomal Dominant CNM patients in the shortest time

• Simultaneously minimising safety risk to patients
Natural History Study - NatHis-CNM

• Started as an International study EU and Canada, USA in 2014
  - Currently 45 +11 enrolled patients in EU, 7 of which in US
  - Total study duration 48 months, ending May 2019
• New amendments: enrol an additional 20 patients, including DNM2 mutated patients
• Lead investigator: Dr Laurent Servais (Hôpital Trousseau, Paris)
• Participating centres: Belgium, France, Spain, Italy, Germany
Clinical Development plan

**Clinical Development Plan**

**DYN101 - C101**
- **Phase 1-2**
  - Safety/tolerability/PK
  - SAD/12 wks MAD
  - ≥ 16 y
  - n = X (MTM1 & DNM2)

**DYN101 - C201**
- **Phase 2-3**
  - Efficacy
  - ≥ 2 y, DNM2 & MTM1
  - N = X
  - Responder analysis;
  - Sparse PK sampling for paediatric patients

**DYN101 - C202**
- **Phase 2-3**
  - Efficacy
  - < 2 y, DNM2 & MTM1
  - Open-label, historical control
  - N = X
  - Sparse PK sampling

**Natural History Study**

**Extension Study**

**Patient Registry**
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Dynacure endorses patient organizations
MESSAGE UNDERSTOOD

DYNA Cure SAS

Module 2 du bio-incubateur de l’ESBS, Pôle API.
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