



**MESSAGE**

**UNDERSTOOD**



Innovative therapies for rare disease - 2018

# Our Mission : Serving patients and their parents by developing innovative therapies for rare diseases



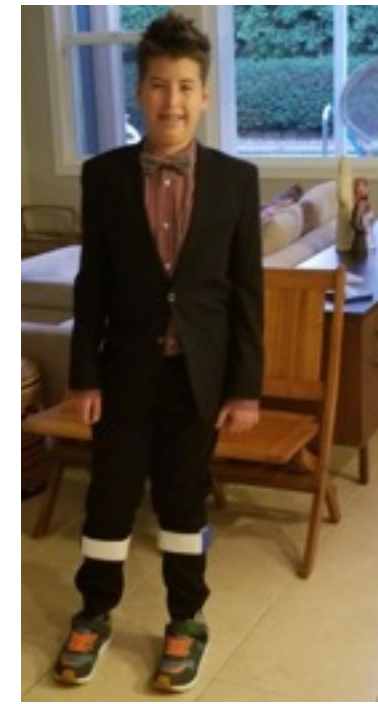
Rian, Ireland



Morice, Germany



Lynn, The Netherlands



Sebastian, USA

# Executive management and board of Directors

**Stephane van Rooijen, MD MBA**  
Chief Executive Officer



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

**Frédéric Legros**  
PhD. Msc  
Chief Operating Officer



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

**Chris Freitag**  
MD  
Chief Medical Officer



Formerly head of clinical development Debiopharm, Shire, Roche

**Leen Thielemans**  
Ir, PhD, MSc  
Head of Development



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

**Belinda Cowling**  
PhD  
Head of Research



Former thematic leader INSERM, Department of Translational Medicine of neuromuscular diseases, IGBMC

# What makes Dynacure unique

Build leader in rare diseases



Leverage validated technology platforms



## 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



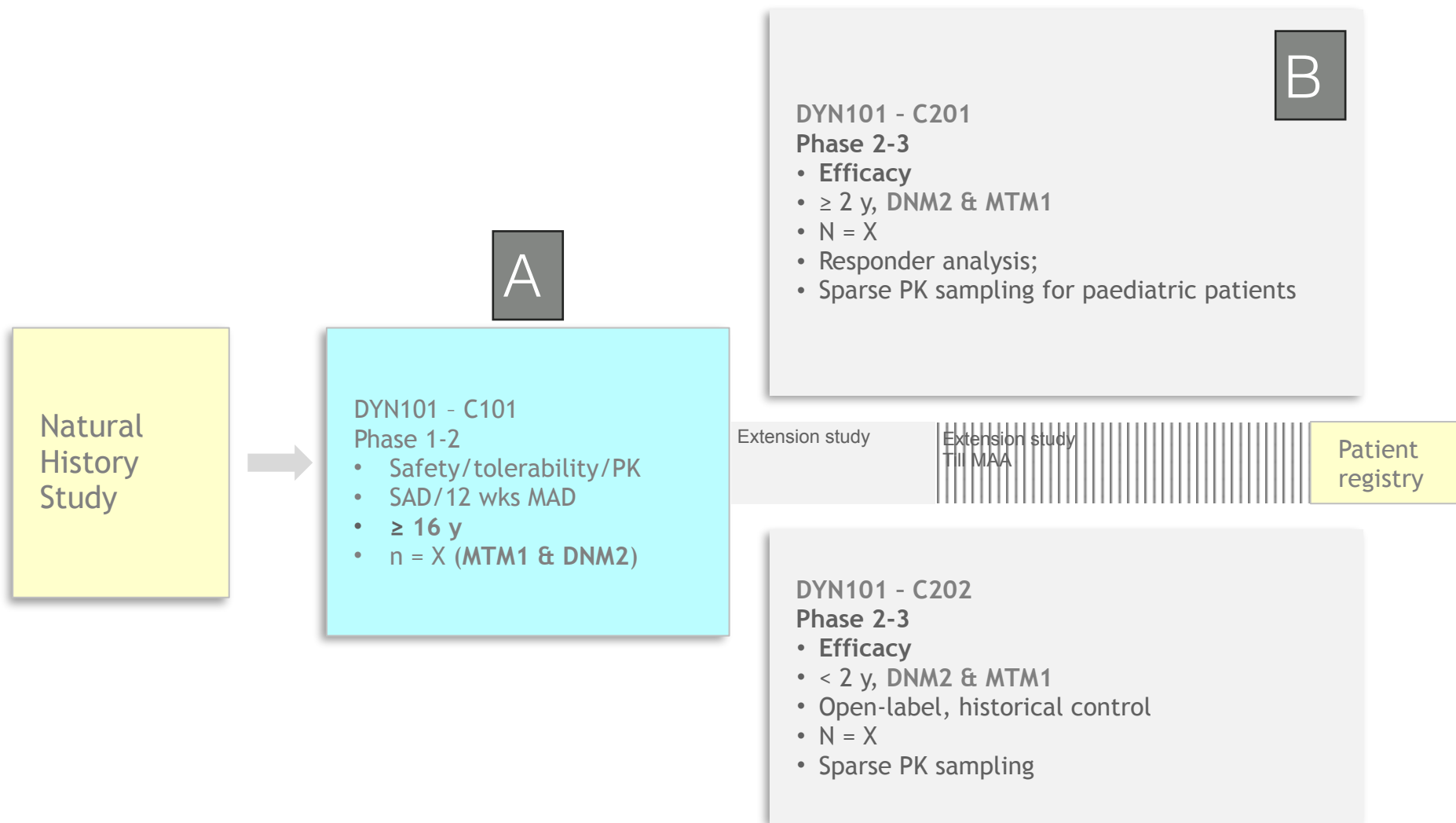
## Baseline data from patients with myotubular myopathy enrolled in a European prospective and longitudinal natural history study

M. Annoussamy<sup>1</sup>, C. Lilien<sup>1</sup>, T. Gidaro<sup>1</sup>, E. Gargaun<sup>1</sup>, V. Chê<sup>1</sup>, U. Schara<sup>2</sup>, A. Gangfuß<sup>2</sup>, A. D'Amico<sup>3</sup>, A. Daron<sup>4</sup>, C. de Lattre<sup>5</sup>, JM. Cuisset<sup>6</sup>, M. Mayer<sup>7</sup>, JM. Arnal<sup>8</sup>, A. Hernandez<sup>9</sup>, C. Vuillerot<sup>10</sup>, S. Fontaine<sup>10</sup>, V. Biancalana<sup>11</sup>, A. Buj-Bello<sup>12</sup>, JY. Hogrel<sup>1</sup>, H. Landy<sup>13</sup>, L. Servais<sup>1,4</sup> and NatHis-MTM Study Group

1. Institute of Myology, Paris, France – 2. Paediatric neurology and neuromuscular center, University of Essen, Germany – 3. Bambino Gesù Children's Research Hospital (IRCCS), Laboratory of Molecular Medicine, Rome, Italy – 4. Centre de Référence neuromusculaire, CHU La Charité, Liège, Belgium – 5. Centre de référence maladies neuromusculaires adultes, Hôpital de la Croix-Rouge, Lyon - France – 6. Service de neuropédiatrie, Hôpital Roger Lacroix, Lille, France – 7. Service de neurologie pédiatrique, Hôpital Armand Trousseau, Paris, France – 8. Service de réanimation polyvalente, Hôpital Sainte Justine, Toulouse, France – 9. UCI Pediatrics, Hospital Puertas del Mar, Cadix, Spain – 10. Service de rééducation pédiatrique infantile "L'Escalier", Hôpital Mère Enfant, CHU-Lyon, Lyon, France – 11. Laboratoire de Diagnostic Génétique, Nouvel Hôpital Civil Strasbourg, France – 12. Genethon, INSERM U951, Gory, France – 13. Valerion Therapeutics, Concord, MA, United States – NatHis-MTM Study Group: B. Andreu, S. Davis, C. Walgren-Peterson, E. Bertini, J. Laporte, F. Mengozzi, G. Honnart, O. Ramodal

Funders: Valerion Therapeutics – USA / Genethon – France ; Sponsor: Valerion Therapeutics – Boston – USA ; Sponsor for EU: Association Institut de Myologie – Paris –France

# Clinical Development plan



# Our Mission : Serving patients and their parents by developing innovative therapies for rare diseases



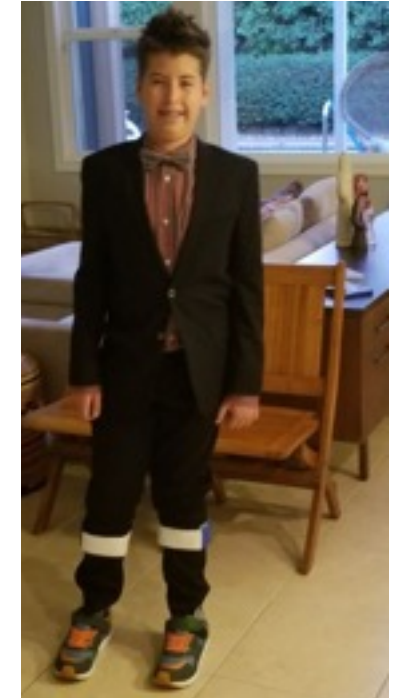
Rian, 7 years old



Morice, 2 years old



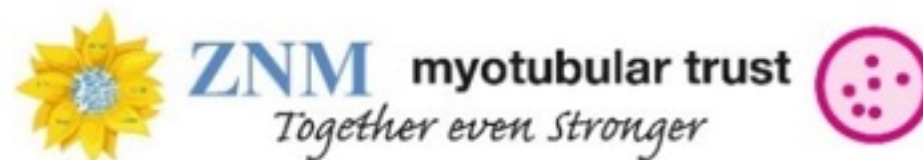
Lynn, 17 years old



Sebastian, 14 years old



# Dynacure endorses patient organizations



A banner with two overlapping rectangular sections. The left section is white with the word "MESSAGE" in teal, bold, uppercase letters. The right section is teal with the word "UNDERSTOOD" in white, bold, uppercase letters. The banner is positioned over a background image of a modern building with a large, curved, metallic structure.

**MESSAGE UNDERSTOOD**

## **DYNACURE SAS**

Module 2 du bio-incubateur de l'ESBS, Pôle API.  
300 boulevard Sébastien Brant, CS 10413, F-67412 ILLKIRCH  
Cedex  
France

[info@dynacure.com](mailto:info@dynacure.com)