



MESSAGE

UNDERSTOOD



Innovative therapies for rare disease - 2018

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



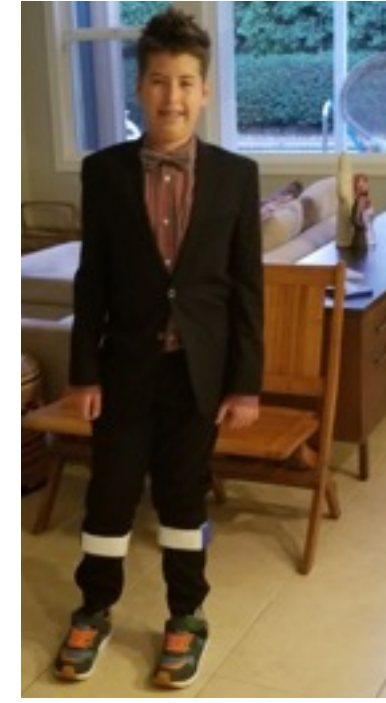
Rian, Ireland



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



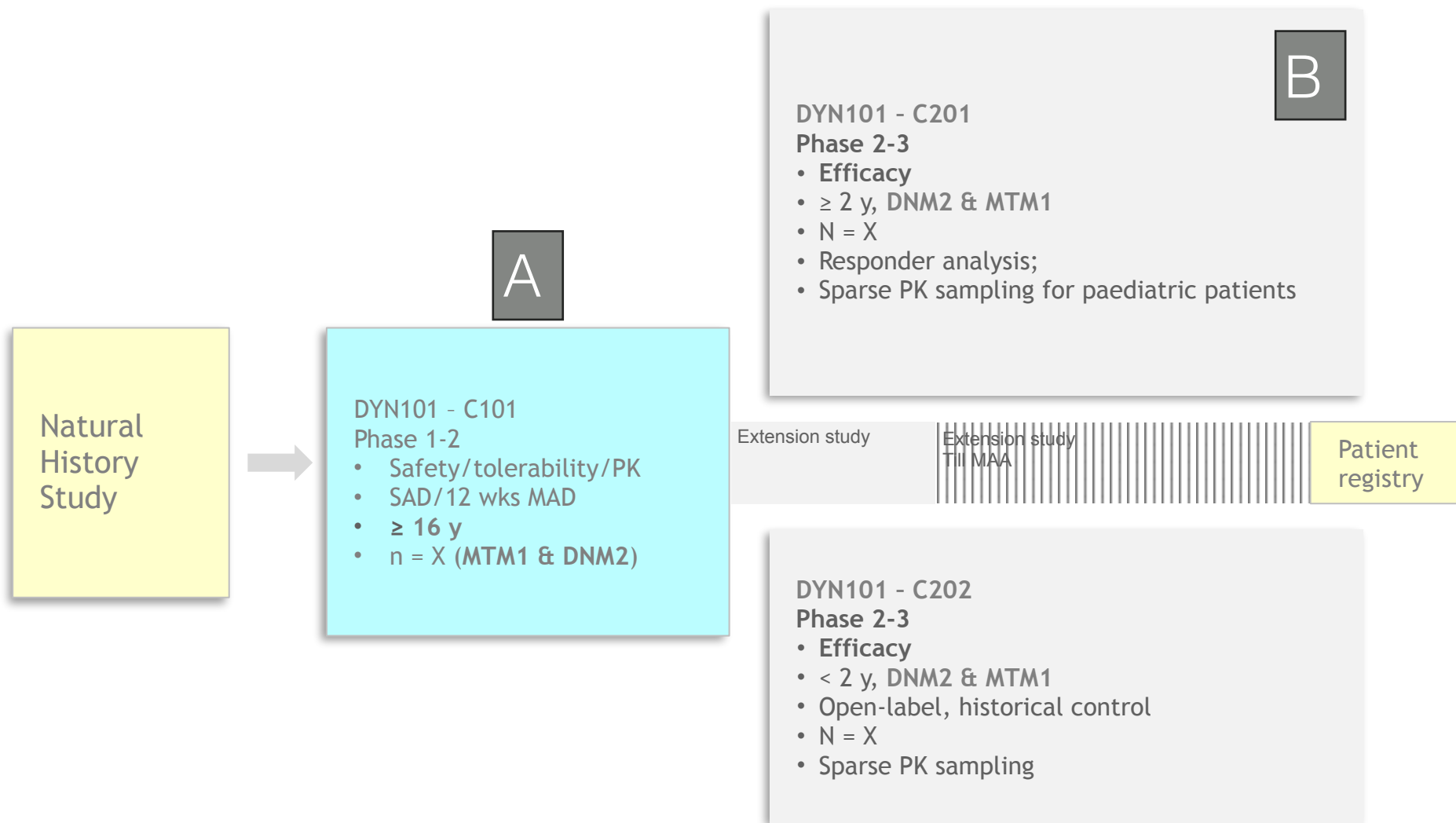
Baseline data from patients with myotubular myopathy enrolled in a European prospective and longitudinal natural history study

M. Annoussamy¹, C. Lilien¹, T. Gidaro¹, E. Gargaun¹, V. Chê¹, U. Schara², A. Gangfuß², A. D'Amico³, A. Daron⁴, C. de Lattre⁵, JM. Cuisset⁶, M. Mayer⁷, JM. Arnal⁸, A. Hernandez⁹, C. Vuillerot¹⁰, S. Fontaine¹⁰, V. Biancalana¹¹, A. Buj-Bello¹², JY. Hogrel¹, H. Landy¹³, L. Servais^{1,4} 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 Mères Enfants, 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. Honnorat, 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



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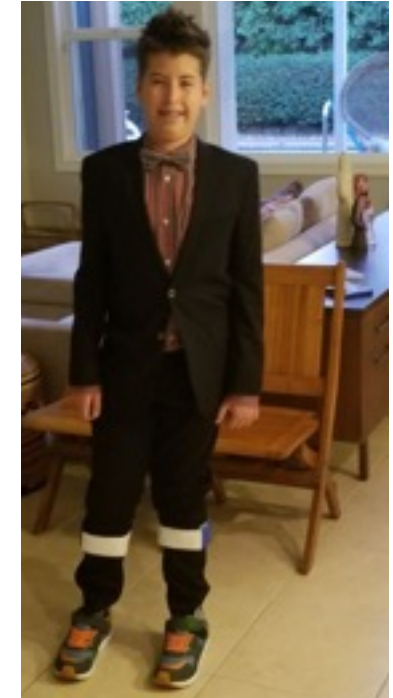
Rian, 7 years old



Morice, 2 years old



Lynn, 17 years old



Sebastian, 14 years old

Dynacure endorses patient organizations



A horizontal banner is positioned across the middle of the image. It is divided into two sections: a white section on the left containing the word "MESSAGE" in teal, and a teal section on the right containing the word "UNDERSTOOD" in white. The background of the banner is a photograph of a modern, circular building with a glass facade and a white, curved roof, set against a clear blue sky.

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