

Dynacure joins ongoing Natural History Study in Centronuclear Myopathies with the Institute of Myology (France)

Strasbourg (France), September 26, 2017

Dynacure is a biotechnology company developing new treatments for patients affected by serious orphan disorders. In its first drug discovery program, Dynacure is focusing on Centronuclear Myopathy (CNM), a debilitating rare disease affecting children and young adults. Dynacure's development program is using an antisense oligonucleotide developed in collaboration with Ionis Pharmaceuticals. Dynacure today announced its participation in the 'Natural History Study and Functional Status of Patients with Myotubular Myopathy and other Centronuclear Myopathies' (NatHis - CNM).

Natural History Study in Centronuclear Myopathy

This prospective non-interventional longitudinal study investigates the natural history and function of 60 patients with MTM and other CNM from Europe, United States and Canada. The study was started in 2014 by the Institute of Myology, Genethon and Valerion Therapeutics to collect data on patients with CNM resulting from a mutation in the MTM1 gene (XLCNM). Dr Laurent Servais from the Institute I-Motion (located at the Hôpital Trousseau, Paris France) is the coordinating investigator. Due to its expertise in the field of CNM, Dynacure will continue the study with the Institute of Myology (its sponsor) and extend it for 24 months. In addition to XLCNM, other subtypes of CNM patients resulting from a mutation in BIN1 (ARCNM) and DNM2 (ADCNM) will be enrolled.

The study objective is to follow-up the disease course, changes in disease severity and any progression of symptoms. Study specific functional assessments and patient questionnaires will be used, and will be based on age and ambulatory status of the participant.

Data from the study will be used to characterize the disease of CNM and determine which outcome measures will be the best to assess the efficacy of potential therapies in future clinical trials.

Anne Lenox, CEO of UK's Myotubular Trust said: *"The Myotubular Trust thank Dynacure and the Institute of Myology for the extension of this NatHis-CNM study which will be crucial in understanding and quantifying the evolution of the disease. We are particularly pleased to see the inclusion in the study of more forms of centronuclear myopathy. Participants are children and young adults affected by this debilitating disease for which no treatment exists yet".*

Laurent Servais, MD PhD, coordinating investigator added: *“I’m grateful to Genethon and Valerion for their support in the first 36 months of the study. The generated data are a source of valuable scientific medical knowledge. I’m very excited to expand this unique study with Dynacure to build a solid comparator platform for future clinical trials to evaluate new therapies”.*

Stephane van Rooijen, MD MBA, CEO of Dynacure concluded: *“Dynacure is well positioned to drive forward its lead program in Centronuclear Myopathies in order to develop new therapeutic approaches and help patients who suffer from CNM. By joining the NatHis-CNM effort, Dynacure confirms its commitment to support patients and physicians to enhance medical knowledge on the natural course of this rare disease”.*

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About Dynacure: www.dynacure.fr

Dynacure is a biotechnology company developing new treatments for patients affected by serious orphan disorders. In its first drug discovery program, Dynacure is focusing on Centronuclear Myopathies (CNM), a rare debilitating disease affecting children and young adults. Dynacure’s Dyn101 development program is based on the modulation of the expression of the Dynamin 2 protein through the use of an antisense oligonucleotide developed in collaboration with Ionis Pharmaceuticals, the leading biopharmaceutical company in RNA-targeted drug discovery.

Dynacure was founded in 2016 as a spin-off from the IGBMC (Institute of Genetic and Molecular and Cellular Biology – Unistra/INSERM/CNRS) of Strasbourg.

About Institute of Myology: <http://www.institut-myologie.org>

Located in Paris, at the heart of Europe’s largest hospital, The Pitié-Salpêtrière Hospital, the Institute of Myology was created in 1996 under the leadership of an association of patients and their parents, the AFM-Telethon (www.afm-telethon.fr).

With a patient focus, the Institute of Myology coordinates the medical management, basic, applied and clinical research and education. It is an international reference centre that participates in numerous trials and clinical studies, mainly concerning neuromuscular diseases but also muscle damage related to high performance sports or ageing. Since 2005, the Institute of Myology is an association governed by the law of 1 July 1901. The Institute of Myology Association’s mission is to facilitate the coordination of site activities, in partnership with five public guardianship: Public Assistance – Paris Hospitals (Assistance publique -Hôpitaux de Paris – AP-HP), France’s Atomic Energy Commission (Commissariat à l’Energie Atomique – CEA), the French National Health and Medical Research Institute (Institut National de la Santé and de la Recherche Médicale – INSERM), Université Pierre et Marie Curie (UPMC) and the National Centre of Scientific Research of Paris (Centre National de la

Recherche Scientifique – CNRS).

Today, the Institute of Myology boasts 250 experts of muscle and its diseases, 28,270 patient records since its beginning, 4100 annual consultations, a myology research centre.

About I-Motion: <http://www.institut-myologie.org/imotion/?lang=en>

The Institute I-Motion is located at the Hôpital Trousseau (Paris France) and results from a collaborative initiative of the Institute of Myology, the Assistance Publique – Hôpitaux de Paris (AP-HP), the University Pierre et Marie Curie (UPMC) and the Association Française contre les myopathies (AFM) in view of conducting pediatric clinical studies in the neuromuscular diseases' field.

About Genethon: <http://www.genethon.fr>

Created in 1990 by AFM-Telethon, Genethon is fully dedicated to the design and development of gene therapy treatments for rare diseases. Its objective is to provide these innovative treatments to patients affected with rare disorders.

Genethon is developing therapies for rare neuromuscular diseases, immune system or blood disorders and liver diseases.

About Myotubular Trust: <http://www.myotubulartrust.org>

Myotubular Trust was founded in 2006 by two mothers of boys with x-linked myotubular myopathy. The Trust raise research funds to invest in proof of principle projects, designed to find a treatment or cure for all forms of myotubular and centronuclear myopathy.

£1.75M has been raised by an extensive network of affected families, and their communities – 12 international research grants have been awarded to date.

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