



Dynacure Announces First Patient Dosed in Phase 1 / 2 'UNITE-CNM' Study of DYN101 for the Treatment of Myotubular and Centronuclear Myopathies (CNM)

-Safety, PK/PD and preliminary efficacy results in 2021-

STRASBOURG, France and PHILADELPHIA, March 5, 2020 /PRNewswire/ -- Dynacure, a clinical stage drug development company focused on improving the lives of patients with rare and orphan disorders, today announced that the first patient has been dosed with DYN101 in a Phase 1 / 2 study called 'Unite-CNM'. This milestone marks the first time any company has dosed a CNM patient with an antisense medicine.

DYN101 is an investigational antisense medicine designed to modulate the expression of dynamin 2 (*DNM2*) for the treatment of Myotubular and Centronuclear Myopathies (CNM). CNMs are serious, rare, life-threatening disorders that affect skeletal muscles from birth. Centronuclear Myopathies affect between 4,000 and 5,000 patients in the EU, US, Japan and Australia¹. DYN101 is being developed in collaboration with Ionis Pharmaceuticals, the leader in RNA-targeted drug discovery. Preclinical efficacy observed in multiple mouse models of the disease gives confidence in the ability to demonstrate potential disease-modifying results in humans.

"We are committed to providing CNM patients and their families with a therapeutic solution to treat this devastating, progressive muscle disease. Today, we are pleased to have made progress toward this goal with the first dose of DYN101 in a patient in our Phase 1 / 2 'Unite-CNM' study," said Chris Freitag (M.D.), Chief Medical Officer of Dynacure. "We look forward to gaining valuable clinical insights from this study and we expect to report interim results from 'Unite-CNM' in 2021."

Nicol Voermans (M.D. PhD), Neurologist Radboud University Medical Centre (the Netherlands) commented, "The start of clinical evaluation of the effect of DYN101 in patients greater than 16 years of age with X-linked and autosomal dominant CNM is a promising advancement in the treatment of CNM. This is the only trial providing therapeutic hope to this population of CNM patients."

"This clinical trial, based on Ionis' technology platform, is the first investigational study testing an antisense medicine to treat Centronuclear Myopathies," said Brett Monia (Ph.D.), Dynacure's board member and Chief Executive Officer at Ionis Pharmaceuticals. "Dynacure's approach has the potential to provide therapeutic benefit to a wide range of CNM patients by modulating DNM2. We look forward to continue our fruitful collaboration with Dynacure."

About the Phase 1 / 2 Study 'Unite-CNM' (DYN101-C101)

'Unite-CNM' (DYN101-C101) study is a multicenter, ascending dose study to evaluate the safety, tolerability, pharmacokinetics and preliminary efficacy of DYN101 in approximately 18 patients greater than 16 years of age with X-linked (XLCNM) or autosomal dominant CNM (ADCNM). Enrolled patients will have a run-in period or be rolled over from an ongoing natural history study, sponsored by the Institute of Myology in France, which includes 60 subjects that have XLCNM or ADCNM. While the Phase 1 / 2 study will primarily focus on finding an optimal dose of the drug via safety, tolerability and after 12 weeks of treatment, multiple domains of efficacy will also be assessed in an exploratory analysis, which include muscular function, respiratory function and muscle strength. After completing the Unite-CNM study, Dynacure expects to investigate a potentially registration-directed Phase 2 / 3 study (all age groups) that would include US and European sites.

More information can be found at <https://clinicaltrials.gov/ct2/show/NCT04033159?term=dynacure&rank=1>.

About Centronuclear Myopathies

Myotubular and Centronuclear Myopathies (CNM) are serious, rare, life-threatening disorders that affect skeletal muscles from birth. CNMs derive their name based on the central location of the muscle fiber nucleus, which is an abnormal finding observed in muscle biopsies. The disease is driven by mutations in multiple genes including MTM1, DNM2 and BIN1 and Dynacure scientists have discovered the link between an increase in DNM2 and the direct cause of the disease (Cowling et al 2014 JCI). There are many genetic forms of CNM including X-linked recessive (XLCNM/ Myotubular Myopathy), autosomal dominant (ADCNM), and autosomal recessive (ARCNM), which are all associated with poor prognosis. Centronuclear Myopathies affect between 4,000 and 5, 000 patients in the EU, US, Japan and Australia¹.

About DYN101

DYN101, an investigational antisense oligonucleotide using Ionis' proprietary antisense technology, is designed to modulate the expression of dynamin 2 (DNM2) for the treatment of Myotubular and Centronuclear Myopathies (CNM). Preclinical studies have demonstrated that DYN101 has the potential to be disease modifying in CNM, with compelling preclinical efficacy in treating animal models of XLCNM and ADCNM^{2,3}. Prevention and reversion of the disease was observed with a clear dose-dependent improvement in whole body strength and mice survival.

The development plan for DYN101 was designed to be very broad and it is the only known program being investigated for most CNM populations, XLCNM and ADCNM. DYN101 has been granted Orphan Drug designations by the US FDA and EMA.

About Dynacure

Dynacure is a clinical-stage drug development company focused on improving the lives of patients with rare and orphan diseases. The Dynacure team leverages its proven track record in rare disease drug development to build a pipeline of novel drugs. Dynacure is developing DYN101, an investigational antisense medicine designed to modulate the expression of dynamin 2 for the treatment of Myotubular and Centronuclear Myopathies, with Ionis Pharmaceuticals. Dynacure is also building a complementary research portfolio targeting other orphan disorders. The company maintains its headquarters in Strasbourg, France and a corporate office in Philadelphia, PA, USA. Dynacure's investors are Andera Partners, Bpifrance, IdInvest, Ionis Pharmaceuticals, Kurma Partners and Pontifax.

For more information, please visit www.dynacure.com.

1. [*Neuromuscul Disord.* 2018 Sep;28\(9\):766-777. doi: 10.1016/j.nmd.2018.06.012. Epub 2018 Jul](#)
2. [*Nat Commun.* 2017 Jun 7;8:15661. doi: 10.1038/ncomms15661.](#)
3. [*Proc Natl Acad Sci U S A.* 2018 Oct 23;115\(43\):11066-11071. doi: 10.1073/pnas.1808170115. Epub 2018 Oct 5.](#)

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