

## Dynacure Announces €50M (\$55M) Series C Financing

*-Funding supports ongoing Phase 1 / 2 'Unite-CNM' study of DYN101 in Patients with Myotubular and Centronuclear Myopathies -*

STRASBOURG, France and PHILADELPHIA, April 2, 2020 /PRNewswire/ -- Dynacure, a clinical stage drug development company focused on improving the lives of patients with rare and orphan disorders, announced today the close of a €50 million Series C financing led by Perceptive Advisors.



New investors participating in this financing include Bpifrance Large Venture and funds managed by Tekla Capital Management LLC. Dynacure's existing investors Andera Partners, Bpifrance through its FABS and Fonds Biothérapies Innovantes et Maladies Rares funds, Kurma Partners and Pontifax also meaningfully participated. In connection with the financing, Henry Skinner, Ph.D., Senior Vice President of Tekla Capital Management, will join the Dynacure board of directors.

"We are excited to partner with Dynacure as they pioneer the use of antisense therapy for the treatment of Myotubular and Centronuclear Myopathies," said Ellen Hukkelhoven, Managing Director of Perceptive Advisors. "Perceptive has long been committed to supporting novel approaches in rare diseases and we look forward to working with the dedicated team at Dynacure to bring DYN101 and other medicines to patients faster."

DYN101 is an investigational antisense medicine designed to modulate the expression of dynamin 2 (DNM2) for the treatment of Myotubular and Centronuclear Myopathies (CNM). Proceeds from the financing will be used to advance Dynacure's lead program, DYN101, through an ongoing Phase 1 / 2 clinical trial in adults, expand the DYN101 clinical program to include treatment of pediatric patients and to continue to grow the company and its pipeline.

"Since the company's inception in 2016, Dynacure has made significant progress in a very short time, resulting in the first CNM patient dosed with their antisense medicine, DYN101, in March 2020," said Laurent Higuere, Investment Director at Bpifrance Large Venture. "We view this financing as our commitment to supporting Dynacure as they advance DYN101 and become one of the industry's future rare disease leaders."

"With the support of our outstanding group of investors, this financing positions us well to advance DYN101 through Phase 1 / 2 clinical evaluation in both adult and pediatric patients and expand our pipeline into other rare muscle disorders to strengthen Dynacure's growth," said Stephane van Rooijen (M.D. MBA), Chief Executive Officer of Dynacure.

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 Australia<sup>1</sup>. 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.

## **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, that have XLCNM or ADCNM. The Phase 1 / 2 study will primarily focus on finding an optimal dose for DYN101 based on safety and tolerability. 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 plans to initiate a registrational 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. 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 Australia<sup>1</sup>.

## **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<sup>2,3</sup>. 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 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 Large Venture, Bpifrance through its FABS and Fonds Biothérapies Innovantes et Maladies Rares funds, Idinvest, Ionis Pharmaceuticals, Kurma Partners, Perceptive Advisors, Pontifax and funds managed by Tekla Capital Management LLC.

For more information, please visit [www.dynacure.com](http://www.dynacure.com)

### **Contact:**

Westwicke (an ICR company) and Juniper Point acted as advisors to Dynacure on the series C financing.

### **Juniper Point – U.S. Investor & Media Relations**

Amy Conrad  
[amy@juniper-point.com](mailto:amy@juniper-point.com)  
+1 858 366 3243

**Dynacure**  
[info@dynacure.com](mailto:info@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.