Press Release

Dynacure receives Research Grant from the Programme d'Investissements d'Avenir (PIA3) Grand Est operated by Bpifrance to Expand Development of Lead Antisense Program Dyn101 for Rare Disease 'Centro Nuclear Myopathies' (CNM)

STRASBOURG, April 25, 2019 – Dynacure, a biotechnology company developing new treatments for patients affected by serious orphan disorders, today announced the award of a research grant of 450 000 euros from Programme d'Investissements d'Avenir (PIA3) Grand Est operated by Bpifrance.

The research grant will advance and expand the development of Dynacure's lead drug program (Dyn-101), an antisense oligonucleotide designed to modulate the expression of dynamin 2 (DNM2) for the treatment of Centronuclear Myopathies. Centronuclear and myotubular myopathies (CNM) are rare congenital myopathies with variable inheritance ranging from X-linked recessive (XLCNM), autosomal dominant (ADCNM), and autosomal recessive (ARCNM), all associated with poor prognosis. CNM myopathies affect between 4 000 and 5 000 patients in the EU, US, Japan and Australia[1].

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, ADCNM and ARCNM. The research grant announced today allows Dynacure to accelerate and expand its development plans for Dyn101, including the identification and development of novel biomarkers to support upcoming clinical studies in CNM and additional preclinical research to expand the spectrum of human diseases for which Dyn101 may be applicable. Dynacure expects to begin its first in human study with Dyn101 in the second half of 2019.

Belinda Cowling (PhD), Chief Scientific Officer of Dynacure, stated: "We are thrilled to receive funding from the region for research and development of a treatment for CNM patients. This research grant will allow us to accelerate our clinical plans in CNM and broaden the investigation of Dyn101 in other indications where modulating DNM2 may have therapeutic potential. We believe this strategy will maximize the number of patients that may be treated by our potentially transformative therapy."

Frédéric Legros (PhD), Chief Operating Officer of Dynacure, added, "This source of non-dilutive funding is essential to drive Dynacure's research forward and is a recognition of the quality of our project by the French State, Région Grand Est and Bpifrance."

About Dynacure: www.dynacure.com

Dynacure develops new treatments for patients suffering from serious orphan disorders. Dynacure's lead program, Dyn101, is designed to address centronuclear myopathy (CNM), a rare and debilitating disease affecting children and young adults. The Dyn101 development program targets the Dynamin 2 protein using a cEt antisense oligonucleotide candidate developed in collaboration with Ionis Pharmaceuticals. Dynacure is also building a complementary research portfolio targeting other orphan disorders.

Dynacure's investors are Andera Partners, Bpifrance, IdInvest, Kurma Partners and Pontifax.

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1. *Neuromuscul Disord. 2018 Sep;28(9):766-777. doi: 10.1016/j.nmd.2018.06.012. Epub 2018 Jul 1.*