



NMD Pharma initiates Phase II trial of NMD670 in Spinal Muscular Atrophy

Aarhus, Denmark, 26 September 2023 – NMD Pharma A/S, a clinical-stage biotech company developing first-in-class, small molecule CIC-1 inhibitors directly targeting muscle function within neuromuscular disorders, today announces that the first patient has been dosed in a Phase II clinical trial with the CIC-1 inhibitor NMD670 in patients impacted by spinal muscular atrophy (SMA). The patient was dosed under the direction of Prof. dr. Kristl Claeys, MD, PhD at the Kliniekhoofd Neurologie, Neuromusculaire Ziekten, Laboratory for Muscle Diseases and Neuropathies at UZ Leuven University Hospital in Belgium.

The Phase II clinical trial ([NCT05794139](https://clinicaltrials.gov/ct2/show/study/NCT05794139)) is a randomized, double-blind, placebo-controlled, 2-way crossover study to evaluate the efficacy, safety, and tolerability of twice-a-day oral dosing of NMD670 for 21 days in ambulatory adult patients with SMA Type 3. The study is an international multicenter study including sites in North America and Europe.

“SMA is a rare neuromuscular disorder characterised by severe muscle weakness and fatigue that greatly affects the quality of life of patients and their families.” commented Jorge A. Quiroz, EVP and Chief Medical Officer NMD Pharma. “NMD670 has already been shown to be safe and efficacious in a proof of mechanism study in patients with myasthenia gravis (MG) and we are confident that it could also be beneficial for the treatment of patients affected by SMA.”

Thomas Holm Pedersen, Chief Executive Officer of NMD Pharma, added: *“Despite great advances in the therapeutic armamentarium of therapies to treat SMA, patients can still experience muscle weakness and impaired mobility. This represents a significant unmet need for new interventions that can help patients independently perform daily activities and better manage the symptoms of SMA. We are very pleased to announce the dosing of the first patient in this Phase II study. This represents a significant milestone for NMD Pharma as we further expand our pipeline into new indications, and I look forward to keeping the patient communities and market updated as we progress.”*

“We are excited for the start of this Phase II clinical trial that will test a new approach for SMA. Building on the impact from our current genetically targeted disease-modifying therapies, NMD670 may help to restore muscle strength and function by targeting the neuromuscular junction which is known to be affected in SMA,” said Kenneth Hobby, President, of Cure SMA.

NMD670 is a first-in-class small molecule inhibitor of the muscle-specific chloride ion channel, the CIC-1 ion channel. NMD Pharma has pre-clinically demonstrated that CIC-1 inhibition can enhance neuromuscular transmission and ultimately skeletal muscle function in multiple animal models of neuromuscular diseases. NMD670 has already demonstrated positive clinical study results in a Phase I/II study in patients with MG. Based on these preclinical and clinical data, it is expected that this novel approach could be beneficial in the treatment of patients impacted by SMA.

Ambulant adult type 3 SMA patients in the US and Europe are encouraged to participate in the study. Further information and a list of currently active investigational sites can be found on clinicaltrials.gov or via email at contact@nmdpharma.com.

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About NMD Pharma

NMD Pharma A/S, is a clinical-stage biotech company leading in the development of novel first-in-class therapies for severe neuromuscular disorders. The Company was incorporated as a spin-out from Aarhus University, Denmark in 2015 and was founded on more than 15 years of muscle physiology research with a focus on regulation of skeletal muscle excitability under physical activity. NMD Pharma has built a world-leading muscle electrophysiology platform leveraging its in-depth know-how of muscle physiology and muscular disorders and is developing a pipeline of CIC-1 inhibitors for the treatment of patients with neuromuscular disorders including myasthenia gravis and spinal muscular atrophy. Positive top-line data reported from a Phase I/II clinical trial of lead program NMD670 in myasthenia gravis has provided clinical validation of CIC-1 inhibition to restore neuromuscular function. NMD Pharma has raised ~€80 million from investors including Novo Holdings, Lundbeckfonden BioCapital, INKEF Capital, Roche Venture Fund, and Jeito Capital. Find out more about us at <http://www.nmdpharma.com/>.

About Spinal Muscular Atrophy

Spinal muscular atrophy (SMA) is a genetic disease characterized by progressive destruction of nerve cells that control essential skeletal muscle activity leading to weakness and fatigue. SMA is the most common genetic cause of mortality in infants with an incidence of 1:5,000 to 1:15,000 new-born babies. Typically, SMA is caused by a defect in the survival motor neuron 1 gene (SMN1) on chromosome 5q13 and is commonly classified into Types 1 to 4 dependent on age of onset, symptom severity and the number of copies of the SMN2 gene.