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NMD Pharma announces topline results from its Phase 2a study of ignaseclant in Charcot-Marie-Tooth disease Types 1 and 2

- *Exploratory Phase 2a study showed consistent and clinically meaningful functional improvements across muscle strength, motor performance, and patient-reported outcomes in patients with Charcot-Marie-Tooth disease (CMT)*
- *Benefits emerged early, were durable beyond treatment discontinuation, and were supported by patient-reported outcomes, with ignaseclant being safe and well tolerated and no serious adverse events reported*
- *NMD Pharma is accelerating ignaseclant's clinical development in CMT alongside multiple near-term clinical catalysts across its neuromuscular pipeline, with Phase 2 studies in spinal muscular atrophy (SMA) and generalised myasthenia gravis (gMG) expected to deliver additional data in 2026*

Aarhus, Denmark, 3 February 2026 – NMD Pharma A/S, a clinical-stage biotechnology company dedicated to developing novel therapies to restore skeletal muscle health, today announced topline results from its Phase 2a SYNAPSE-CMT study evaluating ignaseclant (formerly NMD670), an investigational first-in-class small molecule inhibitor of the skeletal muscle-specific chloride ion channel 1 CIC-1, in patients living with Charcot-Marie-Tooth disease (CMT) types 1 or 2. There are currently no FDA-approved therapies for the treatment of Charcot-Marie-Tooth disease.

SYNAPSE-CMT (NCT06482437) was a randomized, double-blind, placebo-controlled Phase 2a study designed to explore the clinical activity, safety, and tolerability of twice-daily oral ignaseclant administered over 21 days, with follow-up assessments at day 28. The trial enrolled 81 adult patients with any genetically confirmed CMT1 or CMT2 subtype across clinical sites in the US and Europe.

Although the study did not demonstrate a treatment difference on the 6-minute walk test (6MWT) at 21 days, the pre-specified primary endpoint for this exploratory study, there was improvement across multiple pre-specified secondary endpoints: Patients receiving ignaseclant demonstrated consistent improvements in muscle strength, functional performance, and patient-reported outcomes. These secondary endpoints are recognized as highly relevant by the CMT clinical and patient community.

Key findings from both CMT1 and CMT2 included:

- **Improvements on the validated Charcot-Marie-Tooth Functional Outcome Measure (CMT-FOM)**, a composite scale assessing strength and function across multiple domains, with separation from placebo observed during treatment and maintained through day 28
- **Robust improvements in handgrip strength**, a clinically meaningful domain for patients with CMT
- **Improvements in fine hand function,** supporting functional benefit beyond gross motor performance
- **Patient-reported improvements in physical function and disease impact,** as measured by the CMT-Health Index (CMT-HI), align with objective functional findings

- **Maintenance of functional gains following treatment discontinuation,** suggesting effects that extend beyond short-acting pharmacologic muscle activation

Ignaseclant was safe and well tolerated in the study. The reported adverse events were mild or moderate and did not require drug discontinuation. There were no serious adverse events reported by patients on ignaseclant. The observed safety profile was consistent with prior clinical experience.

"There are currently no approved therapies for people with CMT, and it is encouraging to see improvement trajectories across multiple measures of muscle strength and function in a short clinical study, **said Dr. David Herrmann, MBBCh, Chief of the Neuromuscular Division in the Department of Neurology at the University of Rochester Medical Center and CMT-SYNAPSE study investigator.** "The improvements observed in hand strength and dexterity are particularly relevant for patients, as these functions directly affect daily activities. These findings support further evaluation of ignaseclant in longer-duration studies."

Professor Mary Reilly, MD, Head of Division of Clinical Neurology, UCL Queen Square Institute of Neurology added, "As there are no current approved treatments for any form of CMT, a study such as this current Phase 2a trial of ignaseclant in both CMT1 and CMT2 is very welcome. The results from this exploratory study suggest that targeting skeletal muscle excitability may improve both muscle strength and function in CMT. While further studies are clearly needed, the observed improvements across multiple functional domains, especially in upper limb function, are encouraging and of direct relevance to patients. These results support further longer duration studies of ignaseclant in CMT."

Ana de Vera, MD, Chief Medical Officer at NMD Pharma, commented, "These Phase 2a clinical study results provide encouraging evidence that ignaseclant positively impacts skeletal muscle strength and function within weeks of treatment initiation, with effects that appear to persist beyond the dosing period. Importantly, we did not observe a plateau at the end of the treatment window, suggesting the potential for further benefit with longer-duration therapy. We look forward to reviewing the full dataset and engaging with CMT experts and regulatory authorities to define next steps."

"In a neuromuscular disease where patients typically experience a progressive loss of strength and function, observing consistent functional improvements over such a short timeframe is highly encouraging, **said Thomas Holm Pedersen, PhD, Chief Executive Officer of NMD Pharma.** The clarity and coherence of improvements across other objective and patient-reported measures align closely with our understanding of ignaseclant's mechanisms of action. We believe these results strongly support continued development of ignaseclant as a potential therapy to deliver meaningful functional benefit for patients living with CMT and will be accelerating the clinical development in this indication."

NMD Pharma would like to express its sincere gratitude to the individuals living with CMT who volunteered their time and effort to participate in the SYNAPSE-CMT study, often while managing the daily challenges of living with the disease, as well as to the many patients and families who reached out with interest but were unable to enroll. The willingness of the CMT community to engage in clinical research is deeply appreciated and essential to advancing new therapies, especially in the absence of approved treatment options. NMD Pharma looks forward to continuing to work closely with patients, caregivers, investigators, and patient advocacy organizations and hopes to welcome many of these individuals into future clinical studies.

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NMD Pharma plans to submit detailed topline data from SYNAPSE-CMT as a late breaking abstract or presentation at a leading neuromuscular scientific meeting in the first half of 2026 and to publish the full study results in a peer-reviewed journal.

Ignaseclant has previously completed a randomized, double-blind, placebo-controlled, three-way crossover Phase 2a study of two single oral doses versus placebo in 12 patients with symptomatic gMG. Published in *Science Translational Medicine*, the study showed clinically meaningful and statistically significant improvements in Quantitative Myasthenia Gravis (QMG) total score, along with a favorable safety profile. In January 2025 NMD Pharma announced that the U.S. Food and Drug Administration granted Orphan Drug Designation to ignaseclant for the treatment of CMT.

The Company is also conducting a Phase 2a study in adults with SMA and a Phase 2b study in gMG with AChR and MuSK positive autoantibodies, with top-line results expected in the first half and second half of 2026, respectively. These studies build on the previously reported positive Phase 2a results in gMG and support the potential of NMD Pharma's skeletal muscle-targeted ClC-1 inhibition platform across neuromuscular diseases arising from both pre- and post-synaptic dysfunctions.

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About NMD Pharma
NMD Pharma A/S is a privately held, clinical-stage biotechnology company dedicated to enabling better lives through novel therapies designed to restore skeletal muscle health. The company is advancing a first-in-class platform of small-molecule therapies that selectively target skeletal muscle to address rare neuromuscular diseases as well as broader age-related conditions with significant unmet medical need.

Building on more than 15 years of pioneering research in muscle physiology, NMD Pharma has established a world-leading muscle electrophysiology and translational research platform integrating deep biological insight, proprietary enabling technologies, small-molecule drug discovery capabilities, and robust in vivo pharmacology models. The platform is designed to translate fundamental muscle biology into clinically meaningful and sustained improvements in strength, function, and quality of life for patients.

NMD Pharma's lead development candidate, ignaseclant (formerly NMD670), is a first-in-class skeletal muscle-targeted therapy currently in clinical development for Charcot-Marie-Tooth disease (CMT),

generalized myasthenia gravis (gMG), and spinal muscular atrophy (SMA). The company is also advancing next-generation compounds and exploring additional biologic pathways that may support durable neuromuscular function across a range of disorders that result in a lack of normal muscle function.

Headquartered in Aarhus, Denmark, with operations in the United States, NMD Pharma has raised approximately $180 million from leading life science investors, including Novo Holdings, Lundbeckfonden BioCapital, INKEF Capital, Roche Venture Fund, and Jeito Capital.

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

About ignaseclant (NMD670)
Ignaseclant (formerly known as NMD670) is NMD Pharma's lead development compound and an investigational first-in-class small molecule inhibitor of the skeletal muscle-specific chloride ion channel 1 (CIC-1). By inhibiting ClC-1 ignaseclant enhances skeletal muscle excitability and the muscle's responsiveness to weak signals, improving neuromuscular transmission, restoring muscle activation and skeletal muscle function.

Preclinical and clinical studies have demonstrated that ClC-1 inhibition can improve muscle strength and functional performance across multiple disease settings. Emerging preclinical findings also suggest that ClC-1 modulation may influence additional biologic pathways relevant to sustained muscle and nerve function, areas that remain under active investigation.

Ignaseclant has previously demonstrated clinically meaningful improvements in a Phase 1b/2a study in generalized myasthenia gravis (gMG) and has shown preclinical evidence of activity in spinal muscular atrophy (SMA), Charcot-Marie-Tooth disease (CMT), and age-related muscle disorders such as sarcopenia. Ignaseclant has received orphan drug designations from the U.S. Food and Drug Administration for the treatment of gMG and CMT.

About the SYNAPSE-CMT Phase 2a Trial
SYNAPSE-CMT was a randomized, double-blind, placebo-controlled Phase 2a clinical trial evaluating the investigational drug ignaseclant in 81 adult patients with genetically confirmed Charcot-Marie-Tooth disease (CMT) types 1 or 2.

The study incorporates a range of validated clinical and functional assessments, including measures of muscle strength, motor performance, walking ability, endurance, balance, and patient reported outcomes, to evaluate the impact of skeletal muscle activation via inhibition of the ClC-1 ion channel in patients with CMT. The trial was designed to explore clinical activity, characterize safety and tolerability, inform dose and endpoint selection, and support advancement into subsequent stages of clinical development.

About Charcot-Marie-Tooth Disease (CMT)
CMT is a rare, inheritable neuromuscular disease affecting approximately one in 2,500 people worldwide, including an estimated 135,000 individuals in the United States, making it one of the most prevalent rare orphan neuromuscular diseases. CMT causes progressive dysfunction of peripheral nerves, leading to sensory loss, debilitating muscle weakness, impaired balance, declining motor control and substantial limitations in daily function that typically worsen over time. There are no FDA-approved treatments for CMT, and patient

care is supportive, relying on physical therapy, orthotic devices, and mobility aids. The significant and lifelong burden of CMT underscores the urgent need for new therapeutic approaches, including strategies designed to directly improve muscle strength and function independent of underlying nerve degeneration, such as the mechanism being investigated with ignaseclant.