Therapeutic Update



Message from Lise Kjems, MD Chief Medical Officer at Cyclo Therapeutics

Dear NNPDF Community,

Having recently returned from the WORLDSymposium[™], I am energized by the discussions and progress across lysosomal storage disorders, and particularly for the Niemann-Pick community. I had the opportunity at the meeting to present a poster about our Phase 3 trial,



known as TransportNPC™. This pivotal clinical trial was designed in close collaboration with regulatory agencies, Key Opinion Leaders, patient advocacy groups and patient communities to best enable a potentially positive outcome. With the severity of this disease and the limited treatment options currently available to patients, there is a significant unmet need for a product that can treat the systemic and neurologic manifestations of NPC.

We continue to be encouraged by the potential of Trappsol® Cyclo™ and are leveraging this critical product, its mechanism of action and route of administration with the hope of providing a much-needed clinical benefit to the NPC patient population. Additionally, very recently, we established a global steering committee, led by the Principal Investigator for our TransportNPC study, Dr. Caroline Hastings, to shepherd our study forward. I am grateful to Dr. Hastings and to each member of this committee for their tireless effort to improve the lives of those living with NPC. You can read our full announcement here.

For more information about the Company's TransportNPC™ pivotal Phase 3 study, visit ClinicalTrials.gov and reference identifier NCT04860960.

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