Therapeutic Update

Update from Cyclo Therapeutics on the Trappsol® Cyclo™ Development Program

Cyclo Therapeutics, Inc. has received authorization from the FDA to begin enrolling patients in its global Phase III pivotal study evaluating our proprietary formulation of cyclodextrin, Trappsol® Cyclo™, used intravenously (IV), in NPC1 patients. Additionally, the Company expects to receive authorization from the European Medicines Agency in the near term to commence enrollment in European countries, as well as others. Enrollment of the first US patient is expected in March/early April 2021, followed by enrollment of European patients to commence soon after, pending authorization.

The Phase III global pivotal trial will be a 96-week trial, with Interim Analysis at 48 weeks. The dose to be evaluated in the active treatment arm is 2000 mg/kg, and this is based on all of the safety and efficacy data that we have from our Phase I and Phase I/II trials to date (see below for more on these). We will use the 5-Domain NPC Severity Scale to assess disease progression, with additional secondary and exploratory outcome measures.

In its review of the Pediatric Investigational Plan (PIP) associated with our Phase III pivotal trial in the EU, the EMA requested that we include a group of patients in the trial from birth, noting that our drug when administered intravenously has the potential to act as a preventative treatment if given early in the disease course. EMA reviewers also stated that our drug, when provided intravenously, has the potential to serve as a therapeutic for both the systemic and the neurologic features of the disease.

We expect to share all of the parameters of our Phase III clinical trial (A Phase 3, Double-blind, Randomized, Placebo-controlled, Parallel-group, Multicenter Study to Evaluate the Safety, Tolerability, and Efficacy of 2000 mg/kg of Trappsol® Cyclo™ (Hydroxypropyl-β-cyclodextrin) and Standard of Care Compared to Placebo and Standard of Care in Patients with Niemann-Pick Disease Type C1) on ClinicalTrials.gov as soon as possible. For patients interested in learning more about the study, in keeping with Good Clinical Practice, please ask your primary care physician to reach out to Cyclo Therapeutics' Dr. Sharon Hrynkow, Chief Scientific Officer and Senior Vice President for Medical Affairs, Sharon.Hrynkow@cyclodex.com

Our presentations at WORLD 2021 (February 9 and 10) focused on data from the Phase I trial (NCT02939547), which completed in 2020; initial data from the Open-Label Extension Protocol to the Phase I trial (NCT03893071); and the Phase I/II trial (NCT02912793), expected to complete in February 2021. Taken together, these data demonstrate that Trappsol® Cyclo™ has a favorable safety profile, removes cholesterol from cells, crosses the blood-brain barrier, and is associated with disease stabilization or improvement. The Phase I/II trial showed efficacy in the first 7 patients who completed the 48-week study (using agreed measures by EMA outlined in the protocol), and 5 of 7 patients were found to have Improved by their treating physicians while remained stable. The Phase I/II trial will complete this February 2021, and we expect to report Top Line results in the March/April timeframe.

We are continuing our Expanded Access/compassionate use program in multiple countries and will evaluate this program in 2021 in light of new requests for our drug.

All of us at Cyclo Therapeutics are committed to the NPC community and to seeing our IV drug reach market approval swiftly. We are grateful to all of the patients and families who have participated in our clinical trials so far, and to all of the treating physicians who have dedicated so much time and effort to advancing our clinical program. Thank you all!

Warm wishes from the team at Cyclo Therapeutics!

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