

Phase I/II Study using Trappsol® Cyclo™

Initial safety and efficacy findings for a phase I/II trial of hydroxypropyl beta cyclodextrins administered intravenously in patients with Niemann-Pick Type C disease.

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Niemann-Pick Disease Type C is a rare and fatal genetic disorder characterized by cholesterol accumulation in every cell of the body. Hydroxypropyl beta cyclodextrins (HPBCDs) have been found in pre-clinical studies to release cholesterol from cells, normalize cholesterol homeostasis, delay symptom onset, and increase lifespan. We present data from the first four patients participating in a Phase I/II study using Trappsol® Cyclo™, the proprietary formulation of HPBCD of CTD Holdings. The trial is randomized, double-blinded, with no control group (NCT02912793). Patients were randomized to receive one of three doses of Trappsol® Cyclo™ (1500 mg/kg, 2000mg/kg or 2500 mg/kg) administered intravenously over 8 to 9 hours twice monthly for 48 weeks. Results presented remain blinded with respect to dose. The review of individual and cumulative safety data to date has shown the study drug to be well tolerated with no serious safety signals observed. In particular, no clinically significant or permanent hearing problems were observed from IV dosing of Trappsol® Cyclo™ as measured by standard audiometric testing. A plasma biomarker for severity of NPC disease, lysosphingomyelin-509, shows a clear downward trend with successive dosings: in 3 of 4 patients, there was a 30% to 50% reduction. Blinded results from standardized tests for ataxia, cognitive capacity, and NPC Severity Scores and Global Impression of Disease as well as subjective assessments from investigators show variation among patients in terms of outcome measures. Three of four patients showed improvement in one or more outcome measures, including ataxia, saccadic eye movements, speech and overall well-being. Initial impressions are encouraging. Clinical efficacy will be fully evaluated on unblinding.