



Phase I Trial using Intravenous Trappsol® Cyclo™ for Niemann-Pick disease type C:

Information for Physicians

Sponsor: CTD Holdings, Inc. (OTCQB: CTDH), Alachua, Florida, www.ctd-holdings.com

Drug: Trappsol® Cyclo™, CTD's proprietary formulation of hydroxypropyl betacyclodextrin (HPBCD), a ring of glucopyranose molecules with affinity for cholesterol. Orphan Drug designation in US and Europe, Fast Track designation in US.

Pre-clinical: Studies in the NPC mouse and cat models show delay of symptoms, increase in lifespan, and decrease in cholesterol with subcutaneous administration of HPBCDs.

Compassionate use of Trappsol® Cyclo™: In 2009, CTD began providing its product on an expanded access basis in the US, then Brazil and Europe. Initial safety data with IV administration showed safety, only expected AEs, and efficacy in systemic features as well as neurologic features of the disease. Heterogeneity in responses across individuals.

General parameters of the US Phase I trial: Participants over age 18 years with confirmed diagnosis of NPC1; Miglustat/Zavesca allowed; 14 week treatment period; infusion of drug every two weeks; outcome measures include safety; pk, pD, liver size; impression of disease. Full enrollment 12 patients across 2 sites:

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Physicians are encouraged to contact the sites directly to determine availability of space in the study. Additional resources for this study or the Phase I/II companion study in Europe and Israel are ClinicalTrials.gov NCT02939547 and NCT02912793 or via CTD's Senior Vice President for Medical Affairs Sharon H. Hrynkow PhD at Sharon.Hrynkow@cyclodex.com.