



Dear Advocacy Leaders,

We are pleased to share with you the topline results from the global Phase 3 TransportNPC™ Study of Trappsol® Cyclo™ in NPC. You may read the press release [here](#). We extend heartfelt gratitude to all trial participants, as well as their families, and the investigators and their teams for their time and continued commitment. With 94 patients studied across 27 sites in 13 countries, your commitment has made the TransportNPC™ study the most comprehensive, placebo-controlled pivotal study regarding patient size, duration and clinical outcomes of an investigational therapy for NPC.

Among the highlights:

- Trappsol® Cyclo™ demonstrated a directionally favorable treatment effect versus placebo on the study's primary endpoint of change in the 4-domain NPC Clinical Severity Scale (4DNPCSS) at Week 96, but did not achieve statistical significance;
- A prespecified analysis in patients receiving background miglustat and/or leucine (a food supplement available in some European countries) therapy, representing approximately 83% of the study population, showed a statistically significant benefit on the 4DNPCSS;
- Over the 96-week treatment period, the safety profile of Trappsol® Cyclo™ was well tolerated. Adverse events were largely consistent with the underlying disease. Treatment-emergent adverse events (TEAEs) were reported as followed: Trappsol® Cyclo™, 93.8%; placebo, 100.0%. Serious TEAEs occurred more frequently with Trappsol® Cyclo™, 35.9%, than with placebo 16.7%; however, serious TEAEs considered related to treatment were reported at similar rates 3.1% vs 3.3%. One treatment-related event of bilateral deafness in the Trappsol® Cyclo™ group was severe. No TEAEs resulted in death, and one participant in the Trappsol® Cyclo™ group discontinued the study early because of a TEAE.
- A separate overall survival analysis comparing infantile-onset patients treated with Trappsol® Cyclo™ with well-matched external controls from the International Niemann-Pick Disease Registry (INPDR) indicated an 85% reduction in the risk of mortality compared with matched INPDR data. A secondary analysis that expanded the control group to include

additional matched historical cohorts from published natural history studies, treatment with Trappsol® Cyclo™ indicated a 94% reduction in the risk of mortality.

As this community knows well, NPC is a devastating, heterogeneous disease, and more treatment options are desperately needed.

We remain on track to submit an NDA for Trappsol® Cyclo™ to the U.S. Food and Drug Administration in the fourth quarter of 2026 based upon a previously announced pre-NDA meeting. The topline data, complete dataset, and overall survival analysis remain subject to full analysis and FDA regulatory review.

Our Trappsol® Cyclo™ program began in 2009, and we share today's exciting milestone with the global NPC community. We look forward to sharing more information with you as we are able.

Together with you, our work continues.

Your devoted team at Cyclo Therapeutics