Message from Cyclo Therapeutics

May 30, 2024

Dear Advocacy Partners,

We are pleased to share today's press release announcing that our TransportNPC™ study is fully enrolled. You can read the full details here.

Among today's highlights:

1. TransportNPC™ is the most comprehensive ongoing controlled pivotal study regarding patient size, global footprint, duration and clinical outcomes for the treatment of NPC.
2. The study has dosed the 93rd (final) and 94th (over enrolled) patients, and ten patients in the substudy treating newborn to 3 years of age.
3. Topline data from the 48-week interim analysis of 104 enrolled patients is anticipated in the first half of 2025.
4. If 48-week data demonstrate significance, we will target submitting a New Drug Application to FDA and Marketing Authorization Application to EMA in the second half of 2025.

We are grateful to our study investigators and to the heroic patients and families who have joined this truly global endeavor. Today, over 100 physicians, inclusive of principal and sub-investigators from 13 nations, have worked together to advance this program as quickly as possible in our collective global effort aimed at bringing a much-needed approved treatment to the NPC community. Given the unmet medical need that remains in the NPC community, we will re-open our Expanded Access Program (EAP). Details are still being finalized, and physicians should reach out to patients@cyclodex.com to discuss potential usage.

Our efforts to bring forward a treatment for NPC began in 2009 with our compassionate use treatment programs, and later our Phase 1 and Phase 2 studies demonstrated Trappsol® Cyclo™ is well-tolerated and has the potential to treat both the systemic and neurological symptoms of NPC. To each of you, thank you for being alongside us on this journey. We are grateful for your mentorship and partnership, and together with you our work will continue.

Kind regards,

Lori McKenna, Head of Global Patient Advocacy
Scott Fine, Chief Executive Officer