May 8, 2019

Dear Advocacy Partner,

At the end of last year, Mallinckrodt Pharmaceuticals reached out to you to communicate the fact that we were delaying the start of Study VTS-270-303.

In response to your most recent questions about the status of VTS-270-303, we wanted to provide a brief update.

Mallinckrodt has not yet begun enrolling patients in this Phase 2 open-label clinical study, which is designed to evaluate the effects of VTS-270 in infants and toddlers up to age four who have neurologic symptoms of Neimann-Pick type C disease. Given the Phase 2b/3 pivotal study (VTS-301) results, we are closely evaluating the potential benefit of VTS-270 before initiating this study in an especially vulnerable population.

As discussed in other venues, Mallinckrodt is continuing our efforts to understand the totality of the data that is available for understanding the potential benefit and safety of VTS-270.

As always, we appreciate your interest and ongoing support. Feel free to share this update with your community as you deem appropriate.

I look forward to our continued engagement. Please feel free to contact me with any questions.

Best wishes,

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