

October 7, 2019

Re: Study VTS301 – Study Resumption in the United Kingdom

Study Title: A Phase 2b/3 Prospective, Randomized, Double-Blind, Sham-Controlled Trial of VTS-270 (2-hydroxypropyl- β -cyclodextrin) in Subjects with Neurologic Manifestations of Niemann-Pick Type C1 (NPC1) Disease

Dear Toni and Niemann-Pick UK Members,

Good news! We are pleased to inform you that Mallinckrodt has confirmed with the Medicines and Healthcare Products Regulatory Agency (MHRA) that the temporary halt of Study VTS301 Part C has been lifted and the study may resume in the United Kingdom.

Mallinckrodt is working with the MHRA and the study investigators to ensure all appropriate steps and notifications are being conducted. Investigators are being informed and will reach out to their patients to discuss next visits and procedures to be performed.

It is suggested that the first visit back to the site be conducted as an unscheduled visit with the following procedures performed: physical and neurological examination (abbreviated), vital signs, audiologic testing, Clinician Clinical Global Impression of Change (CGIC), NPC-SS intake, adverse events, concomitant medications and administration of adrabetadex (VTS-270). In addition, clinical laboratory testing (chemistry, hematology, coagulation) may be included (only) if this assessment was not completed in the past 26 weeks. Auditory brainstem response may also be performed, (only) if this assessment was not completed in the past 52 weeks.

Thereafter, the patient should resume the biweekly scheduled visits.

On behalf of my colleagues at Mallinckrodt, I want to thank the MHRA for its thorough review of the data in making its decision to resume the study. We especially want to thank all the patients, caregivers, families and advocates for their patience throughout this challenging time. We know how difficult and stressful the situation has been for you and the NPC community, and we thank you for your continued understanding. We are so proud to support this amazing community.

Sincerely,

Sheila Talafous
Director, Advocacy Relations
Mallinckrodt Pharmaceuticals
1425 Route 206
Bedminster, NJ 07921
908-238-6351 T
908-581-5123 M
sheila.talafous@mnk.com