

October 9, 2019

Re: Study VTS301 – Study Resumption in France

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

Dear Advocacy Partner,

We are pleased to inform you that the Agence Nationale de Securite du Medicament (ANSM) has informed Mallinckrodt that Study VTS301 Part C may resume in France.

Mallinckrodt is working with ANSM and the study investigators to ensure all required steps and notifications are underway. While we are pleased with the developments in both France and the UK, it's important to note that a new data monitoring committee has been established to review the benefit/risk balance of the data and the future status of the study could be impacted by the findings of this independent committee.

Currently, investigators have been informed that the suspension has been lifted and are reaching out to their patients to discuss next steps. It is suggested that the first patient visit back to the site include the following procedures: 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 adrebetadex (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 biweekly scheduled visits.

On behalf of my colleagues at Mallinckrodt, I 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,

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