July 16, 2020

Dear Justin and Joslyn,

I hope you and your family are doing well during these challenging times. I miss our face-to-face meetings but am grateful we can still connect virtually.

I want to share an update on our adrabetadex program. In late April, Mallinckrodt received a general advice letter from the FDA regarding the adrabetadex data package submitted in support of a future regulatory filing. The agency indicated that additional data from our ongoing studies is not sufficient to demonstrate the efficacy of the drug and that an additional study may be necessary to establish benefit. This was obviously disappointing news to us, as we have been expecting to complete our clinical development and supporting data analysis activities to allow for a regulatory filing for the Niemann-Pick type C1 indication. In light of the FDA's advice letter and following consultation with experts in the field, we believe that a regulatory filing is likely to require a sizable randomized clinical trial of extended duration that would be logistically very challenging, if not impossible, to conduct. At this point, we have exhausted all viable options and a clinical trial is not feasible. Therefore we are actively seeking alternative methods of analysis that utilize existing data from both treated and untreated patients with NPC to provide evidence of efficacy.

After careful consideration and extensive review of the recent advice letter, Mallinckrodt has decided to conclude the VTS301 Part C trial and other active clinical studies and offer patients the opportunity to transition to an Expanded Access Program (EAP). The EAP will be available to all investigators currently participating in our active clinical studies and will provide access to the treatment for the foreseeable future. The investigators have been informed of the transition and we will be in contact with them regarding next steps.

We understand that this update may cause patients and families to be concerned with their ability to receive treatment. Please be assured that Mallinckrodt's priority remains with our patients, and we will work closely with the investigators to minimize any delays or disruptions in treatment during the transition process.

We also recognize that this update will also likely cause questions about the regulatory status of adrabetadex. During this time, our regulatory filing remains on hold as we continue to explore opportunities with the scientific and investigative community to identify any additional potential analyses that may aid in a deeper understanding of the benefit of adrabetadex to patients.

Mallinckrodt is deeply grateful to the patients, families and investigators that have participated in the adrabetadex clinical trials. We recognize the dedication and commitment of study participants and are grateful for the essential role they play in scientific research.

We are also thankful for all you do to support the NPC community. While we realize this is disappointing news, please know that Mallinckrodt, Joe Grieco and I will do everything we can to assist the NPC community in this transition.

Please don't hesitate to reach out to me, or Joe, with any questions.

Best wishes,

Sheila

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