Mallinckrodt Pharmaceuticals

August 1, 2019

Dear Joslyn,

On Wednesday, July 31, 2019 Mallinckrodt was notified by the United Kingdom (UK) Medicines and Healthcare products Regulatory Agency and the French Agence Nationale de Sécurité du Médicament that the open label extension portion (Part C) of our Phase 2b/3 study VTS301, evaluating the effects of adrabetadex (VTS-270) in children who have neurologic symptoms of Niemann-Pick Type C (NPC) disease, must be suspended in the UK and France following a preliminary finding by the agencies of an unfavorable benefit/risk balance. This means that patients in the UK and France cannot receive treatment with adrabetedex until the suspension is lifted. Study investigators in the UK and France were formally notified on July 31, 2019 about the study suspension in their respective country.

At present, patients at clinical trial sites for Study VTS301 in other countries can continue to receive treatment in accordance with the protocol. Additionally, expanded access programs in all other countries will remain active.

Mallinckrodt firmly believes that there is a treatment benefit, and there are no new safety findings for adrabetadex seen in Study VTS301 or any other sponsored clinical study or expanded access program. Our firm belief is that there continues to be a positive benefit/risk balance for these fragile patients.

We are actively engaged with the respective regulatory agencies to address this matter and are seeking a timely meeting in order to present a more in depth review of the information that informed our assessment. We are hopeful that there will be a determination that the study may resume.

As legally required, Mallinckrodt will be informing all other study investigators, regulatory agencies and institutional review boards/ethics committees regarding the action taken by UK and France to suspend Study VTS301. This notification will include our assessment of the benefit/risk and why we believe it is appropriate for patients to continue in this study.

Further, we have reached out individually to EU, international and US NPC patient advocacy groups and will continue our efforts to keep the entire NPC community abreast of further developments.

We understand that this news is extremely disappointing and will cause anxiety with the families in these two affected countries. We have been in continued communication with the broader EU and International community regarding this development. Please be assured that we are committed to working around the clock to address this matter on behalf of patients and will keep you informed as soon as we have additional details.

If you have any questions, please do not hesitate to reach out to me at the address below

Sincerely,

Sheila Talafous
Director, Advocacy Relations
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