Mallinckrodt Pharmaceuticals

January 20, 2021

Dear Joslyn and Justin,

I hope you, your families and the NPC community are doing as well as can be expected during these challenging times.

I'm writing to share important news regarding the adrabetadex (VTS-270) clinical development program. Today, Mallinckrodt disclosed that after an extensive and comprehensive review of all available clinical data, there is no clear evidence of potential benefit for adrabetadex in Niemann-Pick Type C1 disease (NPC). Following this determination, the company has concluded that the benefit / risk balance for adrabetadex as a potential treatment for the neurologic symptoms of NPC is negative. In other words, the risks associated with the treatment, outweigh the potential benefit. It is important to note that there were no new safety findings resulting from this comprehensive data review.

The independent Data Monitoring Committee (DMC) for Study VTS301 has informed Mallinckrodt that it agrees with the assessment of a negative benefit / risk balance following an independent review of the analyses conducted to-date.

As a result of these findings, Mallinckrodt will conclude the adrabetadex development program, including discontinuing all ongoing Company-sponsored clinical studies, expanded access protocols and investigator initiated research studies. **Effective immediately, Mallinckrodt is recommending that treatment with adrabetadex be discontinued as soon as possible, with the appropriate physician oversight.**

In appreciation of the complexity of care for NPC patients, Mallinckrodt will make adrabetadex available to existing patients in Company-sponsored clinical studies, expanded access programs (EAPs) and investigator initiated research programs (IIRs) for up to nine (9) months (*until October 20, 2021*) to allow time for physicians, patients and their families to develop a transition plan.

It is important to understand that continued treatment with adrabetadex for up to this 9-month timeframe will be contingent upon a number of factors including re-consent by the patient/family, and approval by the respective Institutional Review Boards (IRB)/Ethics Committees (EC) and health authorities. Also, please be aware that patients who are not currently receiving adrabetadex will not be able to start treatment. The independent DMC for Study VTS301 has agreed with the recommendation to discontinue treatment as soon as possible, while allowing for up to 9 months for transition.

We understand that this news will be difficult for those involved in the management of NPC and for the individuals, families and caregivers affected by this devastating and rare neurodegenerative disease. We have been dedicated to advancing adrabetadex since acquiring the compound in February 2018, and we share in the disappointment of the entire NPC community.

We are deeply grateful to the patients, caregivers, clinicians and patient advocacy groups for their support of the adrabetadex clinical development program. Advances in drug development would not be possible without trial participants who, despite their individual battles, give their time and dedication to make an impact for others. You have made a difference in the lives of the NPC patients and the community as we have collectively worked to advance our understanding of this disease.

It is important to Mallinckrodt that you hear from us directly and have an opportunity to ask questions. The Ara Parseghian Medical Research Fund has graciously offered to host a webinar for the NPC patient and family community during which Dr. Steve Romano, Executive Vice President and Chief Scientific Officer at Mallinckrodt, and Dr. Susan VanMeter, Senior Clinical Director, will address this latest news and take your questions.

Please join us on Friday, January 22, from 12:30-1:30 p.m. EST via the following Zoom link:

https://notredame.zoom.us/j/93703585673?pwd=emxnRmxTb2NaZjFNQ2tySGN6elFMUT09

Passcode: 050409

On a personal note, when I joined Mallinckrodt two and a half years ago, I was introduced to this wonderful NPC community and have had the honor of working with you to advance awareness for NPC. This is a tenacious community – one that will not acquiesce, but instead will continue to work together to drive disease awareness, education, screening and research. Your voice is strong, and we hope that our work has helped you to increase engagement around NPC in the hopes of one day finding additional treatment options for this disease.

On behalf of Mallinckrodt, I'd like to extend our thanks and gratitude to the entire global NPC community for its continued partnership on the adrabetadex development program. You are all very important to us.

Sincerely,

Sheila Talafous

Sheila Talafous
Director, Advocacy Relations