

To the NPC1 Community,

It's been a little while since we provided our last communication back in February and we're excited to share three important updates on our progress.

- We have completed our first meeting with the U.S. Food & Drug Administration (FDA).
- We are in the midst of putting together a manuscript for publication summarizing the data and experience from the VTS-301 trial, which we recognize as an important part of advancing understanding of the trial, of adrabetadex*, and of NPC1. More detail below.
- Last, but not least, the North American Expanded Access Program ("EAP") continues to support patients who meet criteria for benefit and wish to continue to receive treatment with adrabetadex.

Regulatory Interactions

We recently had an FDA invited introductory call to discuss plans regarding the development of VTS-270 and introduce the Mandos team. It was a positive and collaborative meeting and our key takeaways from the meeting included:

- FDA provided their perspective to consider as we evaluate the most appropriate data analysis methods and development options.
- FDA encouraged us to engage in frequent interactions with them to review and discuss the overall development plan and next steps.
- We shared our plan to identify NPC sub-groups in our analyses and FDA offered their feedback on this new approach and interest in learning more.

We appreciated the invitation by FDA, and we look forward to the next interaction as well as further collaboration towards a path or paths forward for the adrabetadex program.

Publications

We are well into the process of drafting the VTS-301 manuscript in order to publish and share with the community. Recognizing all that the community contributed to this trial, we are committed to ensuring that every contribution is valued and evaluated so that we can share back the learnings. For example, a number of patients in the trial chose to allow collection and analysis of biospecimens to help advance our understanding of NPC1 progression and the potential response to adrabetadex.

We are working to complete those biomarker analyses within the context of a full data and operational analysis from the trial before we publish.

We want to thank the patients, families, investigators, sites, advocacy organizations, and expert advisors who have contributed to this trial and continue to help us advance this work.

Looking Ahead

As we move ever deeper into data gathering, analyses, and regulatory preparations, you can expect to see regular updates from us on a quarterly basis, with ad hoc updates as may be warranted. As always, we're pushing as fast and hard as we can without sacrificing data quality and look forward to continuing to keep everyone updated on our progress.

Sincerely,

Scott Riccio
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**Adrabetadex is an investigational drug that has not been approved by FDA, and FDA has not found it to be safe and effective for use to treat NPC1 or for any other use.*

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