

Mallinckrodt Pharmaceuticals is continuing to evaluate the totality of clinical evidence for adrabetadex (VTS-270) including additional analysis of data from VTS301 (our Phase 2B/3 study) as well as data from the Rush University Medical Center (RUMC) Expanded Access Program (EAP). A summary of the VTS301 post-hoc analysis, ***Intrathecal adrabetadex for the treatment of Niemann-Pick disease, type C1***, was presented via a poster presentation at the 16th Annual WORLD Symposium on February 12th.

We continue to gather data from VTS301 Part C in all participating countries, including the UK and France -- where the study has resumed following a brief suspension last year. We also have a U.S. open-label study currently enrolling patients aged four-years and older. All current EAPs, including the Rush EAP and EAPs in Germany, Australia and various sites in the U.S. are continuing. Finally, we are taking a close look at data from the NIH Natural History Study of NPC to gain a better understanding of the disease course and plan to share these insights to help advance the scientific knowledge of NPC.

We look forward to continued collaboration with INPDA and the entire NPC community throughout 2020. Our outreach this year includes a third-year grant to the Newborn Screening effort of the Firefly Fund, sponsorship and participation at several upcoming conferences and support of various programs including Global Genes' Impact 2020 Initiative. We were also pleased to highlight NPC among our Rare Disease Day/Week outreach at MNK. Follow [@MNK](#) on Twitter to see all our #RareDiseaseDay activity.

Adrabetadex is an investigational agent. The safety and effectiveness of adrabetadex have not been established by the FDA or any Regulatory authority.

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