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## Research Update – October 2015

### Research summary

I got brief research updates from both the TSGT (Tay-Sachs Gene Therapy Consortium) and UC Davis. The TSGT update was much like the one in August - the team has met and agrees upon the strategy moving forward. Looks like they have selected a clinical trial location so now they just need to work up a budget. It is possible Miguel will be able to talk on that at the auction. It is exciting to finally be working on a clinical trial budget - but its also scary to know its time to fund a clinical trial. The UC Davis research is looking better and better.

**Here is the go to quote from the update *"We have recently completed all of our in vitro studies evaluating the safety, stability, and functionality of our HexA/HexB lentivector. The vector appears to be safe, stable, and we get high overexpression of both HexA and HexB in transduced cells. We also get a high level of beta-Hex activity in the MUG/MUGS assay. For our mouse studies, we have observed significant decreases in GM2 in the brain of HexA/HexB lentivector mice compared to HexB-/- mice."***

Simple and clear yes? Basically what they are saying is the therapy is creating high levels of enzymes (Hex A and Hex B) and is reducing the waste (GM2) in the brain as compared to an untreated brain without an adverse impacts on the mice. Over the years we have learned Hex A works better in concert with Hex B. But we've also learned what works in mice does not always correspond to what works in humans. But testing in mice is always the starting point. These results are good - and worth pursuing. I'll try and get the entire context of the update and the accompanying slides on the CTSF website in the next couple days - if you want me to email you a copy let me know and I'll sent it. The UC Davis update is for public release - the TSGT update is more technical and not for public release.

This update will be posted on the Cure Tay-Sachs website under Quarterly Updates. You can also learn more about the TSGT at [www.tsgtconsortium.com](http://www.tsgtconsortium.com). If you have any questions or comments about this update I can be reached at [ken.bihn@curetay-sachs.org](mailto:ken.bihn@curetay-sachs.org) or you can call the foundation offices at (216) 812-5855

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PS Sorry I got a little lazy posting these updates. I have posted all three of the 2015 updates on 2/2/16