



12730 Triskett Road
Cleveland, Ohio 44111
(216) 812 - 5855
Fax (216) 251-6728
ken.bihn@curetay-sachs.org

Research Update – February 2015

Research summary

Let me try and give you a little research news - there has been a lot of activity going on behind the scenes - some of which I am permitted to talk about and some I am supposed to keep confidential. Let me try and walk the line.

First our **UC Davis project**. Gerhard and his team are trying to use a combination of gene therapy, bone marrow and stem cell to attack the Hex A issue. They are basically trying to replicate the success the French researchers have had in another disease. Early on, the research was going well when tested in some Tay-Sachs blood. At the same time the group was establishing a mouse model for testing at UC Davis. Early testing on the mouse model was not good. We had a lot of discussion about the research in January and everyone believes the research still holds great promise but could use some expert animal guidance. Lucky for us we know two such people in Miguel Esteves and Doug Martin. And still luckier for us Miguel and Doug get along very well with Gerhard, Jan and Joe (the UC Davis team). So the five of them are going to work together. Gerhard, Jan and Joe believe the research will work - and so do Miguel and Doug. So they are going to talk monthly - and we'll keep funding the research to see what we can create. If they believe - we believe!!

The **TSGT** (Tay-Sachs Gene Therapy Consortium) also issued a research update - on Super Bowl Sunday none the less. These guys are always working. If you remember we had an issue with vectors being toxic (Miguel hates that word) in monkeys. We tested some new vectors - and identified three we thought would be safe. The most effective vector still showed some unacceptable side effects in the monkeys (mostly near the injection site) - but the other two vectors proved to be safe. The issue then became - do these two "safer" vectors still create enough Hex A to effectively reduce the GM2? They did a short study in mice (using the monkey vectors) and found that one of the vectors - was still able to reduce GM2 by 80+% in two of four mice. The team suspect using the monkey vector is causing an immune response in the other two mice. But the 80+% is promising so they would like to move forward with this particular vector. It looks safe in monkeys and still effective in mice. We are going to test the vector more before proposing we put it in a child. Next is to make this vector in mouse and cat versions (to counter the immune response) and do more testing (we are talking short term - 3 to 6 month testing) while at the same time approaching the FDA with our results to discuss clinical trials and formal toxicity testing. We want to test this vector in a way that makes our team and the FDA comfortable that it is safe - and effective. The team still hopes to get a trial approved this year - but how the mice and cats do will have a significant impact on that timeline.

So now you know just about everything I know. Most of the more confidential stuff is super technical stuff that I don't really understand and some funding and partnering things that I do understand but are not all that relevant to the actual research. The research teams send me highly detailed reports - I use them to help me sleep at night. But we do have experts that can read and understand them - and they believe (unanimously I might add) that both these research project still have great potential. The researchers often talk about how the final **CURE** may be a



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combination of several therapies. I look at this as a two step process. Step one is to stop Tay-Sachs disease from killing our kids. Step two is to repair the damage done to their brains before we stop the disease - some of that happens in the womb. The only population of people that cares about stopping Tay-Sachs disease is us. There is a huge population of people that cares about fixing a damaged brain. So we need to only address Step one and the big money charities will figure out step two. So guess what we are going to do - accomplish step one. It will not be easy and it will not be cheap. But **we are not an ordinary group of people**. We have lived with and cared for some of the most remarkable kids in the history of the world. Strong kids that fought a horrible disease while making people all around them feel better. We need only be half as strong as them to win this fight.

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. If you have any questions or comments about this update I can be reached at ken.bihn@curetay-sachs.org or you can call the foundation offices at (216) 812-5855

Kenneth Bihn
President
Cure Tay-Sachs Foundation