



BREAKING NEWS: Advancing GM2 and GM1 Gene Therapy

Last month we shared the exciting news from [UMass Medical School \(UMMS\)](#) about a significant grant that they received to advance the work of the Tay-Sachs Gene Therapy Consortium (TSGT).

Today there is another significant announcement...

Axovant Sciences, Inc. announced they are licensing the GM1 and GM2 gene therapy programs from UMMS. Read more about this news [here](#) in today's press release from Axovant.

Why is this fantastic news?

Axovant has the expertise, experience, and financial resources to partner with UMMS and NIH to implement the clinical trials, work with the regulatory authorities, and hopefully, if successful, make the gene therapy available more broadly.

We are thrilled to learn that Axovant will lead the development for the GM2 and GM1 gene therapy programs. NTSAD looks forward to helping them move forward as the leading patient advocacy group for Tay-Sachs, Sandhoff and GM1 diseases.

Together, we helped to make this possible!

Since 2007, over \$2 million of Research Initiative gifts we've received from you have gone to fund the work of the Tay-Sachs Gene Therapy Consortium (Miguel Sena-Esteves, PhD, Doug Martin, PhD, Heather Gray-Edwards, PhD, Florian Eichler, MD, Professor Tim Cox and Tom Seyfried, PhD).

There have been an additional \$800,000 in grants awarded to other investigators for relevant grants in related gene therapy, biomarker, and natural history studies in all of our diseases.

NTSAD grants that have supported gene therapy include:

- * animal studies from mice to cats to Jacob Sheep
- * the care for the herd of Jacob Sheep
- * gene therapy vector development and subsequent validation studies
- * biomarker research
- * natural history studies
- * pilot assay for Tay-Sachs newborn screening
- * toxicology and efficacy studies

Each step of progress is a step for all the families and every one is tied to the everlasting legacies of all the children and adults who are no longer with us.

Thank You!



INVITAE

to ALL the families, their friends and community who have supported research over the years.

In particular we want to thank our largest donors to date...

Katie & Allie Buryk Research
Fund of NTSAD

Cameron & Hayden Lord Foundation

Cure Tay-Sachs Foundation

Drive for Dylan (Manning)

Mathew Forbes Romer Foundation

It is clear that a family's experience with the disease is important to the FDA in addition to the data about the progression of the diseases in all its forms.

Please take a moment. **Register for the GM2, GM1 or CANAVAN PINs.**

You can save what you enter and go back to it when you have the time.

If you're a bereaved family, your experience can also be translated to these surveys despite the language being in the present. We understand this may be emotionally difficult, but your experience still matters and can make a difference.

New York Area of NTSAD

Vera Pesotchinsky Research
Fund of NTSAD

All of our families who have done
Day of Hope events!

***Be sure to post
#PinnedGM2, #PinnedGM1,
or #PinnedCanavan
when you've registered with
any of the PINs!***

Susan Kahn, Executive Director
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