



## TAY-SACHS GENE THERAPY CONSORTIUM A Big Step Closer to Clinical Trials

The UMass Medical School issued a press release today announcing that UMass Medical School and the Tay-Sachs Gene Therapy Consortium (TSGT) are beginning work to file an **Investigational New Drug (IND)** application to the FDA. **This application is for a Phase I/II clinical trial for GM2 Gene Therapy.** At the same time, they have begun the work to manufacture vectors for the clinical trial!

The **Tay-Sachs Gene Therapy Consortium** research team, including Drs. Miguel Sena-Esteves, Heather Gray-Edwards, and Doug Martin, has spent a great deal of time over the past two years conducting preclinical toxicology and efficacy tests (funded by NTSAD and Cure Tay-Sachs Foundation) which has brought them to filing this IND.

This announcement is something we've been preparing for since NTSAD sat at the table with the Tay-Sachs Gene Therapy Consortium team at Boston College in March 2007. Over \$2 million of Research Initiative gifts we've received from you have gone to funding this research since 2007. NTSAD's grants to the Consortium have included:

- 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
- toxicology and efficacy studies; and
- facilitating meetings, making introductions to experts in the field of drug development, and being in constant communication with the consortium team all along the way.

Read more about this news and a significant grant for this research [HERE](#) in today's press release from UMass Medical School.

**TWO ACTIONS** you can take now that are important for the GM2 Gene Therapy clinical trial moving forward are:



**1. Register for the GM2 Patient Insight Network (PIN).**

It is becoming clear that a family's experience with the disease is important to the FDA in addition to the data that about the progression of the diseases in all its forms. Please take a moment. **Register for GM2 PIN.** 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.

Click [HERE](#) to begin the registration process.



**2. With your support, we have been able to get to this point.**

It is your continued support that we seek to ensure that these clinical trials move forward. Please consider making a gift — it is vital to the outcome of this work and to our entire community!

Make a gift today to fund the clinical trial at UMass Medical Center and other future trials.

**GIVE TO RESEARCH**

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