Blu Genes Foundation | Tay Sachs Sees Hope in First Gene Therapy Clinical Trial

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Blu Genes Foundation Gives UMMS $1.4M(USD) to Bring Tay-Sachs Gene Therapy Approach to Clinical Trial

The Blu Genes Foundation, a Toronto-based foundation dedicated to the development of gene therapy treatments for rare diseases, has donated $1.4 million to University of Massachusetts Medical School for the advancement of a Phase I/II clinical trial for the genetic disorder known as Tay-Sachs. The gift from the Blu Genes Foundation will be a catalyst in moving research from the preclinical phase to early stage human trials, according to Heather Gray-Edwards, PhD, DVM, assistant professor of radiology at UMass Medical School (UMMS).

"This philanthropic investment in our Tay-Sachs research from the Blu Genes Foundation will allow us to take more than a decade of scientific discovery into the clinic, where our novel gene therapy approach will directly impact patient lives," Dr. Gray-Edwards said.

Tay-Sachs, and a similar disease, Sandhoff disease, are inherited neurologic diseases that occur when genetic mutations prevent cells from producing enzymes needed to break down and recycle materials. Without these enzymes, the materials accumulate to toxic levels, slowly destroying the nervous system. A team of researchers from UMMS and Auburn University in Alabama have been working on a gene therapy to correct the enzyme deficiency using adeno-associated virus, or AAV, vectors.

The average life expectancy for children with infantile Tay-Sachs or Sandhoff disease is about three to five years; there is currently no treatment for either disease. The gene therapy in development has shown promise in animal models of these diseases by extending lifespans up to four times those of untreated animals.

In addition to Dr. Gray-Edwards, the development team includes Miguel Sena-Esteves, PhD, associate professor of neurology at UMMS and principal investigator of the program; Terence R. Flotte, MD, professor of pediatrics, microbiology & physiological systems, and RNA therapeutics, and clinical principal investigator of the trial; and Douglas Martin, PhD, professor of anatomy, physiology and pharmacology at the Auburn University College of Veterinary Medicine.

"We have spent a great deal of time over the past two years conducting preclinical toxicology and efficacy tests and the results provide the supporting data to submit an Investigational New Drug (IND) application to the FDA," said Dr. Sena-Esteves. "We now are beginning work to file an IND with the FDA for the Phase I/II trial and we hope to secure approval in early 2019. We also have initiated manufacturing of the vectors, which should take approximately five months to complete. These are important milestones in this process which demonstrate real progress towards our goal of launching a clinical trial."

"Once we have secured the go-ahead from the FDA, we will be ready to quickly enter the clinical phase of this project, which we anticipate happening as early as March 2019," said Dr. Flotte. "We are excited to work with the National Tay-Sachs & Allied Diseases Association (NTSAD) and the Cure Tay-Sachs Foundation (CTSF), two patient organizations that have been our partners from the beginning of this project and will continue to partner with us as we move into the upcoming trial. NTSAD and CTSF have together funded over $4 million in our research over the past decade to help get us to this point. Now together with this very generous and catalytic gift from the Blu Genes Foundation, we are one big step closer to making this trial a reality." The Blu Genes Foundation is a recently established nonprofit organization that is raising funds to advance gene therapy and find a cure for genetic disorders such as Tay-Sachs. "By strategically investing our philanthropic resources in world-class research, our goal is to offer hope to patients and families where currently there is none," said Joseph

Cordiano, chairman of the Blu Genes Foundation. “We are excited to join NTSAD and CTSF in partnering with UMass Medical School and Auburn University at a critical juncture to help make this first-in-human clinical trial a successful reality.”

“The proof-of-concept studies in affected animals are compelling, and the FDA provided a clear path forward to human clinical trials,” said Dr. Martin. “Too many children with Tay-Sachs and Sandhoff have died since we started this project. The time has finally arrived to push back on these diseases. Our single-minded goal is to get a safe and effective therapy to patients and their families as quickly as possible. Thanks to the support of the Blu Genes Foundation, NTSAD and CTSF, achieving our goal is within sight.”

About the University of Massachusetts Medical School

The University of Massachusetts Medical School, one of the fastest growing academic health centers in the country, has built a reputation as a world-class research institution, consistently producing noteworthy advances in clinical and basic research. The Medical School attracts more than $264 million in research funding annually, 80 percent of which comes from federal funding sources. The mission of the Medical School is to advance the health and wellbeing of the people of the commonwealth and the world through pioneering education, research, public service and health care delivery with its clinical partner, UMass Memorial Health Care. For more information, visit **www.umassmed.edu.**

About the Blu Genes Foundation

Blu Genes Foundation was established to advance research in gene therapy for genetic disorders, offering hope where currently there is none. This need is the driving force behind Blu Genes Foundation’s mission. Gene therapy research takes dedicated time and funding support to bring treatments from bench to bedside.

The Foundation was established initially because of a personal mission to find a cure for a rare and fatal genetic disorder called Tay-Sachs disease and quickly recognized a greater need to include all genetic disorders. Blu Genes Foundation is committed to raising funds to advance gene therapy research to find a cure for genetic disorders.

To learn more about the Blu Genes Foundation and how your tax-deductible donation can help to advance gene therapy research, please visit **www.blugenes.org**

To learn more about Blu Genes Foundation, CTSF, and NTSAD or to support Tay-Sachs research, visit: Blu Genes Foundation: **www.blugenes.org**; **info@blugenes.org** Cure Tay-Sachs Foundation: **www.curetay-sachs.org**; **rick.karl@curetay-sachs.org**; National Tay-Sachs & Allied Diseases Association: **www.ntsad.org**; **info@ntsad.org**

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