New biotech launched for antisense program targeting Angelman syndrome

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Downers Grove, Ill., Feb. 22, 2018 (GLOBE NEWSWIRE) -- FAST (Foundation for Angelman Syndrome Therapeutics) announced today the launch of GeneTx Biotherapeutics LLC (GeneTx), a subsidiary of FAST, for the development and commercialization of an investigational antisense drug, GTX-101, for the treatment of Angelman syndrome (AS). FAST selected Paula Evans, the chairperson of FAST, to serve as the initial CEO of GeneTx and Allyson Berent, FAST's CSO, to serve as the initial COO of GeneTx. Ms. Evans and Dr. Berent have agreed to serve in such roles on an unpaid basis.

"The launch of GeneTx is the logical next step in FAST's mission to cure Angelman syndrome," said Ms. Evans. "We want to ensure potential treatments for AS are brought to each patient as safely and expeditiously as possible, and being actively involved in the interim process between bench and bedside ensures we will have a strong voice in the pricing and accessibility of possible treatments for Angelman families worldwide."

Angelman syndrome is a rare genetic disorder that affects approximately one in 15,000 live births. AS is caused by a loss of function of the maternally inherited *UBE3A* gene. Symptoms of AS include developmental delay, impaired motor function, loss of speech, and epilepsy. GeneTx Biotherapeutics' GTX-101 is designed to compensate for the deficiencies that result from the underlying genetic cause of the disorder. GeneTx entered into a worldwide license agreement with The Texas A&M University System and a research collaboration agreement with Texas A&M AgriLife Research, under which GeneTx hopes to further develop and commercialize this novel antisense oligonucleotide that could potentially serve as a targeted therapy for patients with the disorder.

"For the past eight years, FAST has narrowly focused on investing in leading-edge technologies to treat the underlying cause and debilitating symptoms of Angelman syndrome," said Dr. Berent. "Our aggressive research agenda and collaboration with Texas A&M AgriLife Research has identified and characterized a promising therapeutic to address this devastating disorder. Angelman syndrome has such a significant unmet need, for which there are no approved therapeutics, and the landscape for bringing meaningful treatments to the community is significantly expanding."

Two research pioneers joined GeneTx as scientific advisors. Arthur L. Beaudet, M.D., is a pioneer in Angelman syndrome research. His lab and the Wagstaff lab independently identified the Angelman gene as *UBE3A* in 1997. Dr. Beaudet was inducted into the Institute of Medicine, the Society of Scholars and the National Academy of Sciences. Dr. Beaudet is the Henry and Emma Meyer chair and professor, department of Molecular and Human Genetics, Baylor College of Medicine.

"Compared to 30 other pediatric neurological disorders, I would make the case that Angelman syndrome holds one of the single most optimistic possibilities for a cure," Beaudet said. "Antisense oligonucleotide technology offers tremendous promise to treat the symptoms of Angelman syndrome."

James M. Wilson, M.D., Ph.D., has been working for three decades to develop effective strategies to treat and cure genetic diseases. Dr. Wilson was recognized as one of 12 leading pioneers in cell and gene research with the Pioneer Award given by Human Gene Therapy, a peer-reviewed journal of the medical research community. Wilson directs the Orphan Disease Center (ODC) in the Perelman School of Medicine at the University of Pennsylvania, which focuses on making rare disease research a priority.

"Rare disease patient organizations are becoming increasingly integral to the development of novel therapeutics and gene therapies," Dr. Wilson said. "The launch of GeneTx demonstrates a keen vision by FAST that I believe can be emulated by other rare disease nonprofits in advancing their research agendas."
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Editor's Note: The Orphan Disease Center receives funding from FAST to develop a gene therapy for treating AS.

About Angelman Syndrome

Angelman syndrome (AS) is a rare, neurogenetic disorder caused by a loss of function of the maternally inherited *UBE3A* gene on the 15th chromosome. *UBE3A* is an imprinted gene where only the maternal copy is expressed in neurons of the central nervous system. Imprinting of *UBE3A* is regulated by expression of the paternally expressed *UBE3A* antisense transcript (*UBE3A-AS*).Individuals with Angelman syndrome generally have developmental delay, balance issues, motor impairment and debilitating seizures. Some people with AS never walk. Most do not speak. Anxiety and disturbed sleep can be serious challenges among those with AS. While individuals with Angelman syndrome have a normal lifespan, they require continuous care and are unable to live independently. Typical characteristics of AS are not usually evident at birth. People with the disorder have feeding difficulties as infants and noticeable delayed development around 6-12 months of age. They need intensive therapies to help develop functional skills. In most cases, Angelman syndrome isn't genetically inherited. AS affects all races and genders. It is often misdiagnosed as autism or cerebral palsy. There is an unmet clinical need for individuals with AS in the areas of motor functioning, communication, behavior and sleep. For more information about Angelman syndrome, please visit CureAngelman.org.

About GTX-101

GTX-101 is an investigational antisense oligonucleotide designed to inhibit transcription of the *UBE3A-AS* across the paternal allele of UBE3A. In vitro studies show that as a result of this inhibition, transcription of the paternal *UBE3A* gene is restored in neurons of the central nervous system.

About GeneTx Biotherapeutics

GeneTx Biotherapeutics LLC is a start-up company dedicated to developing and commercializing safe and effective therapeutics for the treatment of Angelman syndrome.

About FAST

FAST (Foundation for Angelman Syndrome Therapeutics) is a Section 501(c)(3) nonprofit research organization singularly focused on funding research that holds the greatest promise of treating Angelman syndrome. FAST is the largest, non-governmental funder of Angelman-specific research. Paula Evans, the mother of a young girl with Angelman syndrome, founded FAST in 2008. The foundation is based in Downers Grove, Ill.

Wendy Greenfield
Phone: (414) 719-9100
E-Mail: wendy@cureangelman.org