FOR IMMEDIATE RELEASE

MavriX Bio Receives FDA Fast Track Designation for MVX-220 for Treatment of Angelman Syndrome

- Designation intended to facilitate development and expedite review of drugs for serious conditions with unmet medical needs

MIDDLETON, Mass., September 22, 2025 — MavriX Bio, a clinical-stage biotechnology company focused on the development of transformative genetic therapies for Angelman syndrome (AS), today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for MVX-220, an investigational adeno-associated virus (AAV) gene therapy for the treatment of AS.

“The FDA’s decision to grant Fast Track designation for MVX-220 reflects the urgent need for therapies for individuals living with Angelman syndrome” said Allyson Berent, DVM, DACVIM, Chief Development Officer of MavriX Bio. “This designation highlights the promise MVX-220 holds as a potential treatment for AS, and importantly, enables closer collaboration with the FDA and an accelerated path forward.”

In May 2025, MavriX announced that FDA had cleared the Investigational New Drug (IND) application for MVX-220. MavriX is preparing to initiate ASCEND-AS, a Phase 1/2 first-in-human study of MVX-220 in Angelman syndrome.

“GEMMABio has been deeply honored to support MavriX Bio on the MVX-220 development program. With Fast Track status, this program gains crucial momentum — it means the team can engage more closely with regulators and accelerate development milestones. For the Angelman syndrome community, Fast Track designation opens up a path toward bringing gene-targeted therapies to patients sooner,” said James M. Wilson, MD, PhD, Chief Executive Officer at GEMMA Biotherapeutics (GEMMABio), whose team developed MVX-220.

MavriX Bio will join the Foundation for Angelman Syndrome Therapeutics (FAST) and the Angelman Syndrome Foundation (ASF) for an Angelman Community Webinar on Thursday, October 9, 2025, at 1:00 p.m. EST to provide a summary of the trial design, enrollment criteria, study procedures, etc. for the ASCEND-AS study. Registrants have the ability to submit questions before the webinar. Registration link:
https://us02web.zoom.us/webinar/register/WN_YgtRsvq6QTWNgs4sSW7yxA#/registration

About Angelman Syndrome

Angelman syndrome is a rare, non-degenerative, monogenic, neurological disorder characterized by severe developmental delay, lack of verbal speech, sleep disturbances, seizures, motor and balance impairments, among other symptoms. It is caused by the loss of function of the *UBE3A* gene in neurons. Angelman syndrome is thought to affect 1:12,000-1:20,000 individuals. There are currently no approved treatments for AS.

About MVX-220

MVX-220 is an investigational gene therapy designed to restore functional expression of the *UBE3A* gene in neurons, the underlying cause of AS, using targeted AAV delivery. The first-in-human study ASCEND-AS will evaluate the safety, tolerability, and efficacy of MVX-220 in adult and pediatric individuals living with various genotypes of AS, including deletion, uniparental disomy (UPD) and imprinting center defects (ID).

MVX-220 was developed at the University of Pennsylvania with full support from the Foundation for Angelman Syndrome Therapeutics (FAST), which funded both the development and nonclinical research activities. The program was subsequently licensed to MavriX Bio, a portfolio company of FAST's drug development accelerator AS^{2}Bio, to enable clinical translation in collaboration with GEMMABio, a therapeutics company focused on developing transformative gene therapies and ensuring global access to treatments.

About MavriX Bio

MavriX Bio is a biotechnology company developing genetic medicines for severe neurological disorders with high unmet need. The company advances programs from discovery through early clinical development and collaborates with academic centers, investigators, and patient organizations to accelerate responsible innovation. For more information, visit mavrixbio.com.

About ASCEND-AS

ASCEND-AS (NCT07181837) is a Phase 1/2 first-in-human clinical study of MVX-220 in Angelman syndrome that will evaluate safety and tolerability as well as clinical efficacy assessments. Further details can be referenced at
https://clinicaltrials.gov/study/NCT07181837?term=NCT07181837&rank=1

About AS^{2}Bio

AS^{2}Bio is drug development accelerator established by FAST to create an integrated approach to drug development in Angelman syndrome, leveraging collective expertise, resources, data and vital networks to provide a "bridge" for new technologies so they can move from proof-of-concept to early-stage clinical trials. MavriX Bio is one of the portfolio companies that AS^{2}Bio supports. For more information, please visit as2bio.com.

About GEMMABio

GEMMABio is a therapeutics company focused on advancing research and global access to life-changing advanced therapies for those living with rare diseases. The company provides research

and product development functions to bring gene therapy discoveries from the bench to the bedside faster and affordably.

GEMMABio is led by gene therapy industry pioneer Jim Wilson and his team of experts, who previously conducted their work in academia. Wilson is also the Chairperson of Franklin Biolabs, a Contract Research Organization that provides a full range of services from discovery to clinical vector manufacturing to the global genetic medicines industry. For more information, please visit gemmabiotx.com.

About the Foundation for Angelman Syndrome Therapeutics (FAST)

FAST is the leading patient advocacy organization working to cure Angelman syndrome. As the largest non-governmental funder of Angelman syndrome research in the world, our goal is to drive forward transformative research and development programs as efficiently as possible for those living with AS — regardless of age or genotype. Learn more at cureangelman.org.

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