The Foundation for Angelman Syndrome Therapeutics (FAST) announced the establishment of an associated drug development accelerator called AS^{2}Bio Inc. The company is creating a drug development ecosystem to bring transformative therapeutics to those living with Angelman syndrome (AS).

“The team at FAST brought together expertise, experience and commitment to patients in order to create a unique platform—one now poised to make transformative change a reality, as safely and quickly as possible, for those living with Angelman syndrome,” said Julien de Bournet, CEO of As2Bio. “We want to use the success we have had so far to turbocharge that future.”

Since its founding, FAST had a vision to disrupt the traditional research model and create an innovative drug development culture to advance therapeutic discovery for Angelman syndrome (AS) and other like-disorders. In 2016, as FAST’s chief science officer, Dr. Allyson Berent spearheaded a team that created a focused roadmap that interlaced conventional as well as new and emerging therapeutic approaches. Based on that vision, FAST began funding what would become over 90 translational research grants, resulting in 13 different therapeutic programs, each focused on different mechanisms to potentially address Angelman syndrome, a monogenic non-degenerative neurologic disorder.

After an influx of funding to expand its drug discovery pipeline and clinical infrastructure, FAST took a venture philanthropy approach, launching the company GeneTx Biotherapeutics, to advance the first investigational antisense oligonucleotide (ASO) through early stage research, IND-enabling studies, and eventually into a Phase 1/2 clinical trial. The GeneTx team was able to file an IND application and partner the program with Ultragenyx Pharmaceutical in under 10 months from the declaration of a clinical candidate for the first clinical trial of a disease modifying therapy in AS.

In 2022, GeneTx was acquired by Ultragenyx Pharmaceuticals after preliminary Phase 1/2 open-label clinical data was disclosed, supporting continued clinical development of the program and representing a critical milestone in the advancement of potential therapies for AS.

Capitalizing on this success, FAST and AS^2 Bio are creating an integrated approach to drug development, leveraging collective expertise, resources, data, and vital networks to provide a “bridge” for new technologies so they can move, quickly and safely, from proof-of-concept to first-in-human. Each AS program will be part of a structured and supportive ecosystem with the purpose of leveraging managerial talent and resources across all.

With FAST financing, companies are being created to address the therapeutic modalities that show promise in AS, ensuring priorities for these programs do not waiver and their potential human application is seen through, if deemed scientifically appropriate. Those investigational development programs currently being advanced include: in vivo AAV-gene replacement therapy, in-vivo CRISPR gene editing, and ex vivo hematopoietic progenitor stem cell gene replacement therapy.

Moreover, nearly all of the learnings from these translatable therapeutic modalities can be leveraged to enable advancement beyond AS. In fact, the entire platform model—from the scientific modalities themselves to the organizational infrastructure that supports it—can be transferable to other neurodevelopmental disorders.

Employees include Julien de Bournet, chief executive officer; Allyson Berent, chief development officer; and Jennifer Panagoulias, chief operations officer.