**FAST's Mission is Clearer Than Ever**

by Paula Evans

Since the announcement of the launch of GeneTx Biotherapeutics, I've been spending time mentoring other parents in the rare disease space on how to control their own destinies in relation to developing effective therapeutics for their children's rare disorders. Some are relatively new to the space, others have already invested millions of dollars but still do not have a clear path for potential treatments. As I explain to those who reach out to me the many steps FAST has taken to develop potential therapeutics for our children, the typical response from these individuals is, “We are never going to be able to do all of that”, or “You are so lucky to be where you are.” The conversation I can't seem to get out of my mind though was the one I had with a mom who has worked so very hard, for years, to develop therapeutics for her child's rare disorder. With tears in her eyes, she explained to me that she didn't have the people to assist her that I have, and have had, on this journey. Each experience has left me awestruck in what we have been able to accomplish at FAST, with of course, the assistance and support of the Angelman community.

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**What FAST Has Accomplished**

From the day I started FAST in 2008, I have been surrounded by the most knowledgeable and talented group of individuals who have worked tirelessly to reshape the future of Angelman syndrome - from assessing the research landscape in order to fund the most cutting edge and translational work, to developing our contract language in order to ensure groundbreaking work didn't languish in a laboratory, to organizing the most relevant symposiums, to attracting new scientists and industry partners, to securing celebrity support, to increasing awareness which in turn increases our fundraising - these amazing individuals made it look easy to move a disorder once thought to be untreatable to the forefront of transformative therapeutic development. After speaking with so many other parents who are trying to emulate what FAST has accomplished, I realize it was anything but easy – it was smart, educated, laser-focused determination and clarity that solidified FAST as the leader in Angelman syndrome research initiatives in order to bring viable therapeutics options to our children.

After funding all the preclinical work on an antisense strategy to treat Angelman syndrome, FAST formed the subsidiary GeneTx Biotherapeutics (“GeneTx”) in order to move this very exciting work out of the laboratory and into clinical development. FAST is the first rare-disease patient organization to take this approach. GeneTx is the very first company to trial a potentially disease-modifying drug in this patient population.

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Other programs that FAST has funded are equally impressive. At FAST, we don't simply fund a research grant; we fund entire preclinical programs to prepare them for pharmaceutical investment. Several of FAST-funded programs, specifically in the gene therapy and gene-altering therapy space, are now moving towards the clinic. These are unbelievably exciting times for the Angelman community.

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An Additional $5 Million Investment

Whereas most rare disease patient organizations would begin to relax and wait (and pray) for the results of their decisions, strategies, and hard work to pay off, FAST is poised to work harder than ever to create meaningful change in the Angelman community. In 2020, we are committing to fund an additional $5 Million dollars to Angelman specific research and invest in caregiver support, an area that is dramatically underserved in this patient community. FAST always strives to think and plan 5+ years ahead, which is likely why Angelman is one of just a handful of central nervous system ("CNS") disorders with an antisense therapy in clinical trial. FAST began funding an antisense therapy program in 2012 and 5 years later, that work was licensed for clinical development. Being forward thinking and aggressive in our mission has served us well and we have no intention of slowing down. “FAST is entering this new decade with a robust scientific plan including expanding our therapeutic translational research initiatives into various areas including support of novel therapeutic platforms, genotype-specific therapeutic exploration, high throughput in vitro and in vivo drug screening, and understanding the impact of deletion positive AS outside of the maternal UBE3A gene deficiency,” said Alyson Berent, FAST’s chief science officer.

In order to continue funding the cutting-edge research FAST is known for, and implementing an caregiver support program, FAST is making some changes to its infrastructure in order to enhance our productivity and efficiency. We are excited to announce that Maiddy Dunigan and Kena Richert, two of FAST’s founding members and fellow Angelman parents, will transition from FAST board members to staff. “I am excited to have the opportunity to focus on all of the projects and programs FAST is planning to initiate in order to better serve the Angelman community in 2020 and beyond,” said Maiddy Dunigan. FAST’s new chief of operations. “Securing funding outside of the community and stretching every dollar we have to better serve the community is my top priority,” said Kena Richert, FAST’s chief financial officer.

Lauren Hoffer
FAST Board

“I’m humbled and excited to further roll up my sleeves to build on the awe-inspiring feats the FAST board has accomplished to date,” added Lauren Hoffer.

All of the advancements in Angelman syndrome, as well as FAST’s mission, would not be possible without the continued support of our community. All of us at FAST remain enormously grateful for your support and look forward to the many advancements that lie ahead. We apologize in advance for blowing up your social media and email with all of our forthcoming announcements.

With excitement and gratitude,

Amelia Beatty
FAST Board

Filling Maiddy and Kena’s board positions are Amelia Beatty and Lauren Hoeffter, two extraordinary Angelman parents who have already made immeasurable contributions to the Angelman community. “I am deeply honored and thrilled to join the FAST board and I look forward to serving our community in this new capacity,” stated Amelia Beatty.

Chairperson