Global Health Repurposing Philanthropic Award Goes To FRAXA Research Foundation

Dr. Mike Tranfaglia and his kids Andy and Laura cut down their family Christmas tree. Dr. Tranfaglia’s son, who was diagnosed with fragile X syndrome, ignited his commitment to finding a cure for this disease.

In 1992, Dr. Mike Tranfaglia’s son Andy was diagnosed with fragile X, a rare genetic disorder that causes mild to moderate intellectual disability. This diagnosis led Dr. Tranfaglia and his wife, Katie Clapp, to found FRAXA Research Foundation (www.fraxa.org), a nonprofit organization focusing on finding a cure for fragile X. As part of its research strategy, FRAXA has made a significant investment in repurposing research, which is why Cures Within Reach awarded FRAXA with the Golan Christie Taglia Patient Impact Philanthropy Award at the 2017 Global Health Repurposing Awards. The mission of Cures Within Reach is to improve patient quality and length of life by leveraging the speed, safety, and cost-effectiveness of medical repurposing research to help drive treatments to patients. “This is a great honor for FRAXA, and great encouragement to us individually. We spend a lot of time working on a rare disease in relative obscurity, and so this recognition is important to us,” said Dr. Tranfaglia.

When Dr. Tranfaglia’s son was diagnosed, very little was known about fragile X. Only a handful of scientists had studied and published on the disease, and there was no social media to create or join a community for common cause. Dr. Tranfaglia and FRAXA also faced the challenge of debunking the belief that developmental diseases were untreatable. FRAXA’s specific focus and dedicated helped raise awareness and understanding not only of fragile X, but also of other similar conditions such as autism and X-linked developmental disabilities.
Dr. Tranfaglia, a practicing psychiatrist with extensive neuroscience training, coordinates the FRAXA’s research strategy. Over the years, Dr. Tranfaglia and his wife have been able to bring together a group of researchers, patients, families, and caregivers from around the world to work together in developing treatments for fragile X and related developmental disorders. The organization since has funded more than $26 million in biomedical research worldwide, of which over $3 million was dedicated to repurposing research. “Repurposing for rare diseases is a more efficient way to understand how to treat diseases in general,” explained Dr. Tranfaglia. “In our case, repurposed drugs are allowing us to develop better biomarkers and clinical trial methods.”

Dr. Tranfaglia’s son is currently taking the repurposed medication minocycline to treat his fragile X. He was the first patient to intentionally receive this drug to treat fragile X. According to Dr. Tranfaglia, “minocycline is one of the major repurposing success stories in the fragile X field. It helps a lot with Andy’s anxiety, mood, and language. It allows him to function better, and every time we’ve tried to stop it, we realize just how much it’s helping him.” FRAXA has subsequently funded clinical studies of the drug minocycline, and now thousands of others with the syndrome are on it.

FRAXA not only funds research to try to find a cure for fragile X, but also runs scientific meetings, advises pharmaceutical companies, and provides education on college campuses, in community settings, and at international conferences. The organization has the unique advantage of understanding both the science of the disease and the importance of community. FRAXA provides resources, referrals, help, and support to families in need.

Dr. Tranfaglia and FRAXA clearly are pioneers in the fragile X field and leaders in repurposing research, which is why Cures Within Reach recognized Dr. Tranfaglia for his philanthropic work at the 2017 Global Health Repurposing Awards on June 27 in Chicago. FRAXA and Dr. Tranfaglia are certainly deserving recipients.