Getting to know Sophia Zilber, Associate Director, Statistical Programming

April 18, 2024

The voices and perspectives of patients play an essential role in rare disease research and development (R&D). They are often our best resource for understanding these rare conditions to bring forward meaningful treatment options. Sophia Zilber is an Associate Director of Statistical Programming. We spoke with her about her personal connection to rare disease, extensive work as a community advocate, and the many ways that patient registries can help to accelerate R&D.

How did you get involved in statistics and biostatistics?

After my family immigrated from the former Soviet Union, and right after college, I was looking for a job opportunity where I could apply my education in computer science. I had good technical skills, and I liked math, so I chose to work in software development and programming. After a few years working in this field, I knew that I wanted to do more with my programming and technical skills.

I decided to further develop my skills and pursue my master's degree in biostatistics, because I was drawn by the opportunity to apply my skills with greater potential for helping patients and improving the lives of people.

How did you come to work in rare disease at Alexion?

When I was introduced to Alexion, I quickly realized that I'd be able to make a difference and bring my passion and my personal connection to rare disease to work every day.

It is critically important for a rare disease patient to have support because there's a lot less available to them: less knowledge of rare diseases, fewer clinical trials available, and fewer companies working on treatments. It's very meaningful for me to work in this field and at Alexion because I lost my newborn daughter, Miriam, to a rare, mitochondrial disease called Leigh Syndrome—a disease that has no approved treatments.

This personal experience of losing my daughter to a rare disease, really changed my perspective and my career focus. Since then, my work is driven in her memory, and focused on rare disease—because nearly 50% of all rare disease patients are children, and more than 90% of rare diseases have no approved treatment.

In addition to my work at Alexion, I am a board member for the Cure Mito Foundation, which is dedicated to advancing research of Leigh Syndrome, and I lead a worldwide Leigh Syndrome patient registry. We work really hard to bring this community together to build awareness and advance the science of this disease.

What about your work at Alexion excites you?

Every little thing we do here matters. For rare disease patients, small progress is significant and can lead to much bigger advancements. The rare disease community is so connected. We know that everything we do for one disease can have an impact on the broader rare community, and that progress spreads very quickly.

What's one lesson you've learned from working in rare disease R&D?

I'm always impressed by the drive and determination that I see every day. There are a lot of motivated people that work in rare disease R&D, but there are also inevitably setbacks, which can feel discouraging. Setbacks are a part of our process, in the rare disease community in general, in the scientific community, and in patient advocacy. And these setbacks can be important opportunities to learn.

You have to take it day by day and keep going forward, because we have a bigger purpose— driving science forward for the patients counting on us and for the rare disease community.

Learn more about our scientists, innovative culture and career opportunities at Alexion.com.