

25 Voices of SMA

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Who am I?



I am a scientist and administrator at a biopharmaceutical company, Repligen. My job is to help identify new opportunities to develop treatments for CNS diseases especially those with no current treatments. I direct a small but dedicated group of scientists with the task of trying to design and manage all the parts of developing novel drugs.

I have spent 25 years trying to advance new therapies for childhood diseases such as autism, Friedreich's ataxia and spinal muscular atrophy.

Why did I become active in SMA?

My company has been keen to find areas where new scientific findings provide a chance for novel treatments to advance quickly. In attending a conference sponsored by FSMA, I became aware of the astonishingly fast progress made in the molecular understanding of the cause of SMA. At this meeting it became apparent that approaches to increasing smn production could be a powerful therapy and potential therapeutic molecules were being identified. This started a three year communication with FSMA leaders to understand their program and provide feedback about what needed to be done to make this a successful therapeutic that could attract the resources needed for commercial development. As a small company we take a personal involvement in our programs. The combination of a large unmet need in SMA, a promising pre-clinical compound identified by years of good effort by FSMA, and the resources available at Repligen there seemed to be a good match.

What is my contribution to SMA?

Last fall we licensed the quinazoline compounds from FSMA to advance these molecules and develop a potential treatment for SMA. I hope to coordinate the drug development operations that can advance this experimental drug through safety evaluations, manufacturing methods development, testing in non-clinical settings all in preparation for testing in patients. New drugs with novel protein targets are challenging to develop as there is no existing roadmap. My contribution is to create this roadmap for this compound and give it every chance to succeed to be an effective treatment for SMA.

What do I like about FSMA?

There are two aspects that drew me to become involved with FSMA. The people of the organization are bright, hard working and upbeat while being pragmatic of the difficult task of being involved in developing treatments for the patients they represent. The meetings held by FSMA for scientists and families provided me as a non-clinician an opportunity to see the faces of families that deal with SMA daily and made a strong connection for me that brought my personal goals and work goals together.