Family Engagement Pioneers

Adding to Rettsyndrome.org's Unique Value in the Clinical Trial Process

Paige Nues, Director of Family Empowerment Rettsyndrome.org, April 2018

In a world of complexity, I aim for simplicity. My work at Rettsyndrome.org has always been to focus around the age-old basics: Why, What, Where and When.

My 'Why' started simply: our daughter Katie was diagnosed with Rett syndrome and I couldn't sit by and wait for someone else to make the changes we wanted. A lot of people were doing great things, but I was not convinced it was enough. So I left life in the Silicon Valley to see what I could do to fill the gaps.

My 'What' at Rettsyndrome.org has evolved and become a buzz-word in the research world. It's called 'patient-tricity' or patient engagement. Katie trained me in the profession of Rett syndrome: the art of advocacy, the art of engagement. I embedded myself in the tribe called Rett, as a member of the club we didn't voluntarily join. My role here is unique and incredibly personal: advancing the safety and health of our kids, and improving the process of participating, in research.

Delivering hope, dignity and equal access to clinical trial design and identifying clinical research opportunities, whether it's a drug trial or a communication study, are priorities that I strongly believe in. Rett syndrome takes away far too many life choices, but it doesn't steal all of them, especially our ability to have Hope and Participation. We believe it is our responsibility to take a leadership position here if we are going to ask families to participate. I want to share what we mean by this, and what Rettsyndrome.org is doing to influence family-focused, patient-centric clinical trials and clinical research.

My 'Where' has expanded over the past decade as we have strategically built the North American Rettsyndrome.org Clinics Network. The existence of this growing network means that our children who are seen at these clinics and/or participate in the Natural History Study have access to the best care practices available, and become well positioned to participate in clinical trials because they are under medical supervision of Rett experts. In turn, a broad team of clinicians learn every day from our children. This network creates trust. Trust is crucial to families, and to successful work with a clinic or a drug company. **This is where Rettsyndrome.org brings value to the equation.** We know the syndrome, we have the clinical network, we have deep relationships with our families, and we have experience in clinical drug trials. We collaborate with Industry partners in a way that is safe and protects families. Our team at Rettsyndrome.org is rich in scientific and health care skill sets, experiences and perspectives, and we hold ourselves to a level of accountability in the research process that says we won't just *fund* research; we will participate in the *design* of research, *for the benefit of our children*. We have experience & assets that the pharmaceutical industry values. That is why they call on us to engage on a regular basis.

My 'When' is NOW. When Rettsyndrome.org is part of the clinical trial design process, we bring the context of family life and professional healthcare experience to the table. In this process, we are also preparing for the future - after treatments come. What will life be like after treatment? How will therapies and school plans evolve as the disease process is changed by these treatments? Post-trial vigilance will be needed and we are positioned to lead it. We are enriching the soil from which our children will grow and change once scientific discovery has its success.

In closing, let me remind you that I have the same hopes and dreams for Katie that parents everywhere share: at most a cure, at the very least symptomatic relief from the many aspects of Rett syndrome. Very few rare diseases have completed the hard journey from clinical trials to prescribable treatments; therefore very few foundations are good at ushering this process. Let me tell you a secret: we are good. We are very good. And we are getting better with each and every trial. We are here to help families make decisions that are safe and to ensure that we are all partners in Hope and Possibilities. I speak from experience.

In this vein, here's a piece of good news: on January 25th, the FDA issued this updated guidance document:

Payment and Reimbursement to Research Subjects: Recruitment Incentives are Not Undue Influence

This means that it is no longer considered "coercion" for a pharmaceutical company to reimburse participants for things like travel, care provider assistance, etc. This is very good news for all of us. Hopefully, this will lower a common financial hurdle for families who wish to contribute to research progress. We'll keep an eye on this:
https://www.fda.gov/RegulatoryInformation/Guidances/ucm126428.htm