BUILDING A GAME PLAN

When a doctor delivered a diagnosis to Pat Furlong that her two sons had the degenerative neuromuscular disease Duchenne Muscular Dystrophy, she was told the boys, then ages four and six, would lose their ability to walk, raise their arms, breathe, and then eventually die.

She refused to sit by and just watch them slowly waste away. She borrowed money to fund her sudden role as a patient advocate, marched off to Washington to corner her Senator and the director of the National Institutes of Health, and brought together academic researchers to get them thinking on how they could tackle the disease.

She eventually lost her two sons to the disease, but continues to be a leading patient advocate and expert on the genetic disorder as the founding president of Parent Project Muscular Dystrophy (PPMD). Furlong shared her experiences at the Global Genes 2015 RARE Patient Advocacy Summit during a session on how patient advocates should go about building a plan for their organizations.

During her talk she focused on the many complex questions and issues a rare disease advocate starting an organization must consider in terms of determining an organization's focus, understanding who it serves, and setting goals.

"When you start an organization, what happens? What can you do? What do you need to think about? What do you need to ask?" she said, explaining that when she borrowed $100,000 to fund her organization, she realized that what she wanted was more time for her sons and to preserve their abilities.

> "When you start a rare disease organization you have challenges. What's your mission? What are you going to do? What do you want to do?" she said. "The answer is you want a cure, but to get there isn't simple or straight forward."

Organizations have a wide range of choices of meaningful activities to engage in and business models to choose from when they set out, whether it's providing support for patients, support for basic research, funding clinical research, engaging in venture philanthropy to support startups, patient advocacy, or some hyrbrid.

> "None of them are wrong. All of them are good and contribute to a whole," she said. "If we all did the same thing, it wouldn't be useful."

The other question to consider is how do you make that organization financially sustainable?

Furlong said government organizations can be helpful, but advocates need to understand the challenges they face.

> They also are financially constrained. They are competitive. And they are slow. We need to recognize that," she said. "There's no way to speed up the NIH. It's a process."

That said, she notes that government organizations bring credibility, and they have access to bigger financial resources. Also, they know how to leverage resources across diseases. "These are real advantages when you are thinking about 'What do I do next? How do I do it? How do I do it better? How do I take a small amount of money and leverage it into much larger money,'" she said.

As an example, Furlong pointed to how a small grant from her organization allowed researchers working on a project to use imaging of muscle to predict the progression of Duchenne. They were able to build on that initial grant to fund work that allowed them to obtain larger grants from the NIH and advance their work.

When Furlong launched her organization, there was little money and effort being invested in understanding Duchenne and developing therapies. Now, 27 companies are developing therapeutics and investment has grown to $5 billion annually from just $5 million a year when she began.

> **"The world has changed for Duchenne. It has changed not just because of a single foundation, or a single government entity, or a single umbrella organization," she said. "It has changed because there are many people contributing to the whole and desiring change."**

Furlong's group entered the policy arena in 2000 and eventually hired a lobbyist to sharpen its message and allow it to better follow regulatory legislation. Their efforts led lawmakers to introduce the MD Care Act in 2001. It was passed that same year and reauthorized in 2008 and 2014. "It's not so sexy, but at the end of the day it's a $500 million investment on the part of the government in muscular dystrophy," she said. "That, next to the cloning of the gene, set the stage for where we are today."

Her group also reached out to the FDA to ask the agency to develop guidance to help drug developers understand the challenges and opportunities they faced in developing therapies for the disease and allow the community to weigh in on them. The FDA said it doesn't write guidance for rare diseases because there are too many of them, but the agency suggested that Furlong's group undertake the effort.

Her organization enlisted the help of the AIDS organizations ACT UP and amfAR, which understood the issues of developing guidances, and engaged the Duchenne community at large to comment. Roughly 200 people became involved in the project. The guidance went through 45 versions, before going to the FDA. Among other things, it called for the minimal exposure of patients to placebos, the use in clinical trials of patients at all points in the progression of the disease, and consideration of patient preferences in creating clinical trials. The agency in June 2014 published it as its first rare disease guidance.

Furlong stressed the importance of working with partners. "'What can you do? What can you leverage? Who do you need to work with? Who's your best partner?'" she said. "You can't do it alone, but we can do it together."

CONTENT SPECIALISTS

Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy
Pat Furlong is the Founding President and CEO of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy (Duchenne). Their mission is to end Duchenne. They accelerate research, raise their voices in Washington, demand optimal care for all young men, and educate the global community.

Duchenne is the most common fatal, genetic childhood disorder, which affects approximately 1 out of every 3,500 boys each year worldwide. It currently has no cure. When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment and the mechanisms for optimal care. Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne.

In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure. Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world.

Session Briefs Summarized by, Daniel Levine
Daniel Levine is an award-winning business journalist who has reported on the life sciences, economic development, and business policy issues throughout his 25-year career. Since 2011, he has served as the lead editor and writer of Burrill Media's acclaimed annual book on the biotech industry and hosts The Burrill Report's weekly podcast. His work has appeared in The New York Times, The Industry Standard, TheStreet.com, and other national publications. He also is the host of RARECast™ podcasts.