A Patient's Story

For "Extraordinary Measures" Dad, Medical Research is All About Hope

John Crowley remembers hearing the late actor Christopher Reeve once say: "At the end of the day, biotechnology is really just a great big word for hope."

John understands that better than most. In 1998 John's toddler, Megan, and infant son, Patrick, were diagnosed with Pompe Disease, a nearly always fatal neuromuscular disorder. At the time, children with the atypical strain of Pompe that affected Megan and Patrick weren't expected to live more than five years.

"You think, 'this is not supposed to happen to us,'" says John, whose story is chronicled in the movie Extraordinary Measures. "You go through the shock and denial and grief."

John's reaction to the diagnosis was typical of what any parent would experience when blindsided by such devastating news. So was his next step: he learned everything he could about Pompe Disease and the research relating to it. Then he did something extraordinary: he stepped in.

In 2000 John teamed up with Dr. William Canfield, an Oklahoma-based biochemist who was developing an enzyme therapy for Pompe Disease but lacked funding for clinical trials. John, a Harvard trained MBA, left his job as an executive with Bristol-Myers Squibb Co. to become CEO of Dr. Canfield's fledgling company. He took out a second mortgage on his house to help finance the company, raised tens of millions of dollars from venture capitalists and ultimately sold the company to a larger firm, Genzyme, to help secure its future.

John's story as a father turned advocate turned biotech CEO may be unique, but sadly his experience as a father desperate to obtain life-saving therapy for his sick children is not. In that regard, John is the quintessential spokesman for families seeking therapy.

"The trial is the realization of hope," he says. But that hope comes at a price. "The time it takes for individuals to become qualified for studies and for studies to advance through the system is brutally difficult" for patients and their families, says John, whose children were rejected from the first two phases of the enzyme therapy trial.

And, of course, there are the risks. Patrick barely survived having an infusion port inserted into his chest so he could receive the therapy during the third phase of the trial.

Hollywood loves a happy ending and the fact that Megan and Patrick, now both teenagers, are alive today is testament to the power of clinical research and their father's unwavering dedication. The children continue to receive the enzyme therapy every other week, but their health is fragile: both depend on wheelchairs and ventilators and need full-time nurses.

What is the difference between standard treatment and a clinical trial?

The decision to participate in a clinical trial is a deeply personal one. What's right for one person may not be right for another.

Before making this important decision, it's crucial to understand the difference between standard or "routine" medical treatment and care during a clinical trial.

In standard medical treatment, your doctor's only goal is to help you get better. She'll assess your condition, discuss treatment options with you, and recommend an approved treatment that she thinks best meets your needs. You may have a variety of treatment options, including different medications or surgery. What's more, during the course of your treatment, your doctor might alter your treatment to try and achieve better results or lessen any side effects you might be experiencing. For example, she might adjust the dosage of your medication or try a different prescription.

In contrast, clinical research studies are scientific investigations. Scientists ask volunteers to do something -- like take an experimental drug or take a combination of medicines – so they answer a medical question. For example, they might want to see if a drug is safe or if it works better than a drug that is already available or find out how big a dose people need to take.

The researcher's goal is to find out how a drug or device works in your body and the trial is about collecting research data to answer specific medical questions. While researchers are responsible for making sure all volunteers are treated safely and fairly, the range of treatment options in a trial is limited to what's allowed by the study's research design or protocol.

There are medical benefits to participating in a clinical trial. Volunteers have access to new treatments, which may be more effective than standard therapies and may not be available elsewhere. They also receive regular and careful medical attention from a research team that includes doctors and other health professionals.

But participating in a clinical trial involves medical risks. In a clinical trial, researchers are studying something they don't fully understand. They're trying to answer questions such as: Is the treatment safe? Does it have side effects? How much should people take?

Because researchers don't have the answers to all these questions, there are risks to participating in a clinical trial. For example, you may experience unpleasant or even dangerous side effects. The drug or procedure being studied may not work as well as standard treatments, or you may be given a placebo or "dummy drug" instead of an active medication. These are important considerations if you are planning to stop your regular medical therapy in order to participate in a trial.