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Giving and getting: Mom turns son's death into new rare-disease drug voucher program

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Jacob Froman was a typical, healthy 8-year-old boy until his diagnosis seven years ago with medulloblastoma, a rare pediatric brain cancer. He died in January 2009 at age 10.

But Jacob's story doesn't end there. Instead, it could spark a new wave of drugs for rare childhood diseases, benefiting companies such as **BioMarin Pharmaceutical Inc.** and other companies as well as patients.

San Rafael-based BioMarin (NASDAQ: BMRN) this month was rewarded for its development of a drug for a childhood rare disease with a voucher that will shave four to six months off FDA review for one of its future drugs. The pilot program was created by the Creating Hope Act, which was folded into the FDA Safety and Innovation Act that Congress passed in 2012.

The program's transformation of personal loss into life-saving treatments for many children is a testament to the perseverance of Jacob's mother, Nancy Goodman. In the wake of Jacob's death, she created Kids v. Cancer, a Washington, D.C., nonprofit, to change the way pediatric drug development is done.

I spoke with Goodman about the program and its potential impact on drug research and development.

How were you involved in the creation of the Creating Hope Act?

My focus has been looking at the impediments to pediatric cancer research and pediatric drug development research. My first step was the Creating Hope Act.

The problem is the markets are too small and it's not easy for pharmaceutical companies to

enter the market of drugs for kids with serious illnesses and get an acceptable return on investment. The Creating Hope Act creates an incentive in the form of a voucher that entitles the drug developer to faster drug approval.

Three years after Jacob's death, you had approved legislation; two years later, BioMarin is the first company awarded a voucher. That's fast work.

I'm a lawyer by training, but I have no lobbying training. We lived in New York City and, after Jacob died, I followed my husband to D.C.

I had never been on Capitol Hill before as a lobbyist — I had been there as a tourist — and I started meeting with Hill staffers and we put together a grassroots lobbying campaign with Jacob's friends and my son, Ben. You could bring these kids into Congressional offices and they would make senators and representatives cry. We had email days and letter writing.

Our whole budget for lobbying was under $100,000.

It was really this great exercise in American democracy.

We're now working with companies and researchers to help them realize what this act will enable them to do.

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Were you lucky as far as timing? FDA fees were on track to be reauthorized at the time you were talking to lawmakers about the voucher program.

Our timing was great, and I'm grateful that we were taken on as a pro bono client of a leading law firm with an FDA practice. [Goodman did not identify the firm, which she said does not want to be identified.]

There also was a first effort — around rare tropical diseases — and the law firm put together a very professional draft bill. We took that tropical disease legislation and talked to health care economists, Hill staffers, pharmaceutical company staffers, venture capital executives and biotech executives, and we identified the problems with the tropical disease legislation. And we fixed them.

What were those fixes?

A drug manufacturer for pediatric rare diseases already is aiming for the U.S. market and the trial is designed for FDA approval, where with the tropical disease scenario drug developers don't usually submit (new drug applications) and (biologics license applications) to the FDA, so there's a much greater burden to redesign their trials.

The value of the (tropical disease) voucher essentially is zero.

Why is that?

You have to give a year's notice before you exercise the voucher, and a lot can change in that year. Under our system, there is a 90-day notice, but we feel with 90 days you're just polishing up your documents. There are no surprises left.

I've heard that this is a pilot program.

It is a pilot program. Under the Creating Hope Act, the secretary of Health and Human Services will issue three vouchers. After the third, the clock will start ticking and as many vouchers that can be created in the next 12 months can be issued. Then the program will sunset, there will be a (General Accounting Office) report and then we can ask for reauthorization.

Does the voucher sunset as well?

The voucher does not expire.

A lot of companies have seen rare diseases as a lucrative area. Did drug developers really need another incentive to focus on rare diseases? Or are childhood rare diseases particularly overlooked?

With premium pricing, some drug companies have identified ways to create drugs for rare diseases and achieve an acceptable rate of return. When the markets are ultra-small, it's still difficult; when the market is kids, it's still difficult; drug development for rare cancers is still very difficult.

It's hard to develop cancer drugs. Even with the Creating Hope Act, it will be hard to develop immunotherapies, for example.

Are there market failures for pediatrics? For certain indications, the answer is yes. For cancer, we hope the Creating Hope Act will give them a reason to embrace pediatric rare cancers.

Why has there been this "market failure"?

I don't know if anyone knows. Is it because the numbers are so small? Maybe not. We have BioMarin now in MPS IV.

Is it because pediatric cancer drugs are used for only a certain period of time and enzyme replacement therapies are for a lifetime? Is it because we know how to create enzyme replacement therapies as opposed to cancer immunotherapies? Or is it just because the companies have not thought about pediatric rare diseases?

What does this program mean for patients with rare diseases and their families?

It gives us a lot of hope.

On our home page is a picture of a little girl, Annabelle, riding a bicycle. This really means there's a kid out there with a terrible disease who can lead a natural life — little girls such as Annabelle, who should have a chance to ride their bikes and laugh and play with their friends. It gives drug developers an easier time to develop drugs for kids like that.

Companies can make hefty profits from these drugs. A cynic asks why we should provide a benefit for companies to make more profit with these vouchers.

Drug development for kids with serious illnesses is a public good. We've organized our society for drug development companies — for-profit companies — to provide that. We have to give them an opportunity to have a reasonable return on that investment.

Why should we be concerned about reasonable return on investment if we give a kid like Annabelle a reasonable chance at a happy life?

And, by the way, our organization does not accept donations from pharmaceutical and biotech companies.

What have you learned through this process?

I've learned how much you can accomplish without that many resources but with a little bit of chutzpah.

We need to think long and hard to provide opportunities to help companies to development drugs for kids.

Companies still aren't developing pediatric cancer drugs, and we need to think about how to provide the conditions for them to do that, or we have to role up our sleeves and seriously fund entities that will do drug development for rare diseases. If we believe that rare disease drug development is a public good, then we need to fund it as if it's a public good.

The great thing about energizing the biotech and pharmaceutical industries is they have incredible resources if you find a way to help them develop a drug.

> Ron Leuty covers biotech, higher education and China for the San Francisco Business Times.