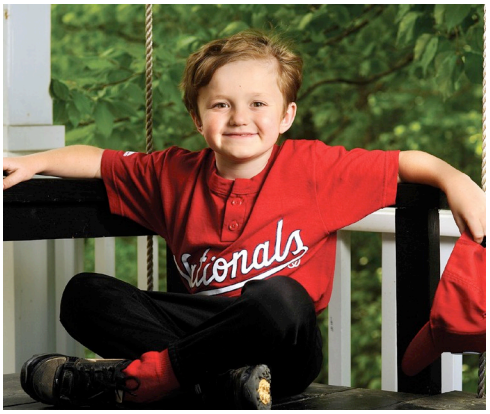
Right To Try: Patient Stories

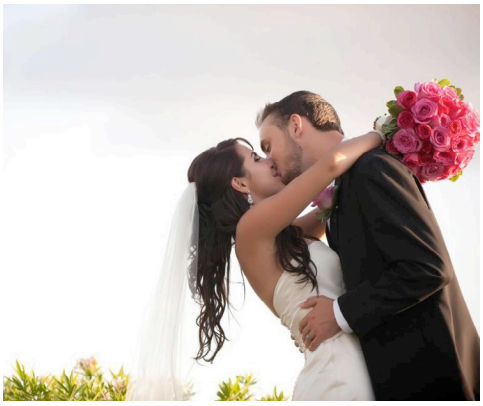
Jordan McLinn

Six-year-old Jordan says he wants to grow up to be a firefighter so he can save lives. Jordan has Duchenne muscular dystrophy, which could leave him paralyzed within 5 years and shortens his life expectancy to only 20 years. There is a drug being used in clinical trials now that is helping young children like Jordan. But it could take another seven years for the drug to be available. His parents say they cannot afford to wait for the FDA to give the drug its final approval. He could be in a wheelchair by then. An investigational drug could add years to Jordan's life, which would give him the chance to save others.



Josh Hardy

By the time Josh Hardy was seven years old he had already beat cancer four times. After a bone marrow transplant, he was infected with a rare virus that no drug on the market could effectively treat. But there was a new medicine being made in North Carolina that was having a positive effect in a small clinical trial. But Josh's doctors couldn't get access to it. Aimee, Josh's mom, started telling Josh's story to anyone who would listen. She created a social media campaign that got worldwide attention. Finally, the FDA and the drug company agreed to let Josh have the drug they were already safely giving to others enrolled in the clinical trial. Now, a year later, Josh is home and healthy. It's no exaggeration to say this investigational drug saved his life.



Mikaela Knapp

At 24, Mikaela was diagnosed with a deadly form of kidney cancer that had already migrated into her bones before she even knew she was sick. She went through every known treatment in a matter of months and nothing worked. Mikaela's high school sweetheart, Keith, heard about a drug under development that was successfully treating people with this same cancer. But Mikaela wasn't allowed to enroll in the clinical trial. Mikaela and Keith launched a social media campaign to try to get access to the drug. But it wasn't enough. The FDA didn't help. Mikaela died on April 24, 2014. Five months later, on September 4, the FDA gave final approval to the drug that could have saved her.

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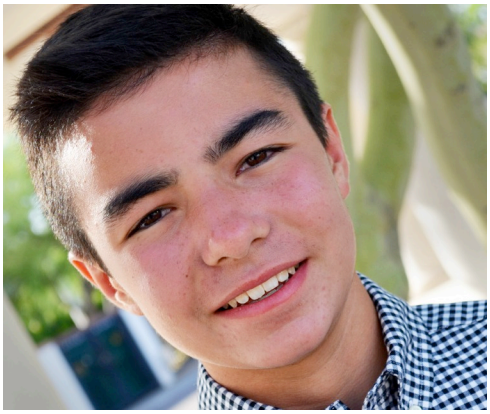
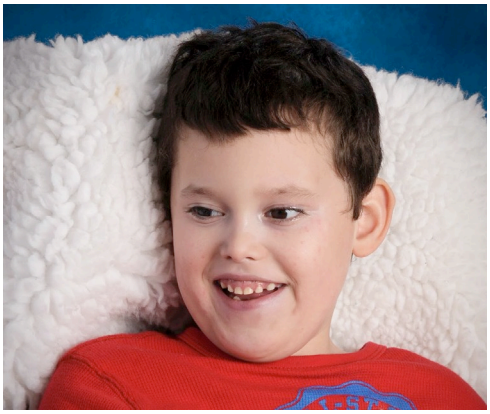


Diego Morris

When 10-year-old Diego woke up with a sore leg, his mom thought "just another sports injury." When the pain didn't go away, they knew something was wrong. But they never expected Osteosarcoma, a rare form of bone cancer. After exhausting all treatment options available, Diego's doctors recommended he try, Mifamurtide, which wasn't available in the United States, but was being safely used and had been given the Prix Galien Award, the gold medal for pharmaceutical research and development, in England. The Morris family wasted no time, and made the move abroad to try to save Diego's life. The treatments worked and now Diego is home in Phoenix, Ariz. and back to playing his favorite sports. Without access to this drug, currently under approval in the U.S., Diego's story could have ended very differently.



Bertrand Might

Bertrand is a very special little boy. He was the first person ever to be diagnosed with a rare, fatal genetic disorder called NGLY1 that has left this seven-year-old paralyzed. Because the disease was only identified by scientists in 2012, and only a few people world-wide have been diagnosed with it, there is no cure and no treatment available. Because the disorder is so rare, a drug may not ever be developed to treat it. But, scientists have found that Bertrand responds to certain investigational therapies. So, Bertrand's family will have to rely on trying new, investigational medications as long as they have access to them.

Ted Harada

Ted was diagnosed with ALS at 38. With no cure, ALS is a certain death sentence—and usually within three years. Ted didn't want to leave his wife and three young children behind in his early 40s. That was just too soon. Lucky for Ted, he was able to enroll in a clinical trial testing a new ALS treatment. Within weeks of beginning the investigational treatment, something miraculous began to happen. Ted set aside his cane and started to regain his strength. While the ALS didn't go away, the symptoms began to subside, allowing him to walk 2.5 miles for ALS awareness in a local campaign. Ted is still going strong because of the investigational treatment he is receiving, and now he is fighting for the right of all terminally ill people to take investigational medications.