For Nicole, a young girl with a rare autoimmune disease, the repurposing of the drug Rapamycin was a miracle. Nicole is one of thousands of children for whom repurposed drugs change outcomes. “[Being sick] involved half of my life in the hospital,” Nicole said. To the people who helped get her repurposed treatment, she said, “You saved my life.”

Stories like Nicole’s are why Cures Within Reach is hosting the 5th Annual Global Health Repurposing Awards on June 27, 2017 at the Radisson Blu Aqua Hotel in Chicago, IL, where this non-profit will give out patient impact repurposing awards in philanthropy, industry and research among guests from industry, academia, clinical care, philanthropy, government, and patient advocacy. “The 2017 GHRA Awards will highlight the impact that repurposing research can have on improving patient outcomes and reducing healthcare costs,” says Robert Metz, Senior Vice President of Horizon Pharma, co-chair of the event.

“We have supported the creation of 13 repurposed treatments that are impacting patients today, including the repurposing of Rapamycin,” explains Dr. Bruce Bloom, President of Cures Within Reach. “Every day we receive new repurposing ideas from researchers and clinicians that can save lives, and requests from patient groups and philanthropists wanting to help. And every day we are grateful to the funders and researchers that have helped us help patients with rare diseases.”
More than 7,000 rare diseases exist worldwide, currently affecting more than 350,000,000 people. About one in ten people in the United States live with rare disease, which is defined in the United States as a disease that affects less than 200,000 people. According to the Kakkis EveryLife Foundation, 95 percent of rare diseases have no FDA approved treatment. In addition, 50 percent of rare diseases do not have a specific foundation supporting them or focusing research their rare disease.

Repurposing is one of the most efficient and cost-effective ways to find a “new” treatment for an unsolved disease. Repurposing tests drugs, devices, and nutraceuticals that are already approved to treat one disease or condition to see if they are safe and effective for treating another disease.

Repurposed drugs cost less to test and take less time to get to patients. Repurposing proof of concept clinical trials can cost as little as $250,000 or less in order to get to off-label use to patients, while novel drugs can cost $1 billion or more to get to market. Repurposed drugs can often get to patients in three years or less, while novel drugs can take 12 years or more. In addition, as many as 3 in 10 repurposing trials lead to an off-label use of a drug, while one in 10,000 novel drugs are successfully brought to market.

A repurposed drug has already passed toxicity and other tests, so there is less of a chance of discovering adverse effects associated with the medicine. Patients with incurable diseases often don’t have the ten years it takes to bring a novel drug to market. With lower costs, more patients can access a repurposed drug that will help them with their disease.

And with all those good reasons to support and use repurposing, the best reason is that they change lives. Nicole’s mother, Laura, says with tears in her eyes, “If you would have told me that these two little pills could save my daughter’s life, I never would have believed it. And yet, here we are 7 years later and she’s living an almost normal life. We’re so thankful for the researchers and funders that made this happen. We love Cures Within Reach!”