Fighting the World’s Worst Childhood Disease You’ve Never Heard Of This Spring

Cure for Rare Children’s Disease Could Impact Millions... If The Doctors Desperate For A Cure Can Get Anyone To Listen

WASHINGTON, DC, USA, March 24, 2016 /EINPresswire.com/ -- Springtime is when the world awakens with a burst of colors…green grass, flowers blooming bright pink and white flowers, blue skies pushing the gray of winter away… and kids coming out of their indoor winter exile to enjoy it all. Except for the ones who can’t experience the beauty and color of spring, robbed of their sight by a rare childhood disease with an even grimmer long-term prognosis: a genetic disorder that strikes otherwise healthy children, and causes them to first go blind, then lose their hearing, balance, coordination, ability to breathe on their own, and eventually die. Today, there is no cure. It’s called Wolfram Syndrome, and you’ve probably never heard of it.

But a small team of doctors, researchers, and parents of Wolfram’s syndrome sufferers hope to change that very soon, because every day counts for kids like ten-year-old St. Louis girl Raquel Gebel. As a toddler, Raquel began showing symptoms of severe diabetes: constant thirst and frequent urination. A year later, her vision began deteriorating and she was diagnosed with optic atrophy.

Doctors eventually determined Raquel had Wolfram’s, the rare disease that leads to the death of brain and pancreatic cells, causing severe diabetes, progressive vision and hearing loss, as well as problems with balance, coordination — and even breathing. Now, it’s a race against time for Raquel and other children suffering from Wolfram’s.

There is hope, and it could have huge wide-ranging implications for millions of people suffering from much more common diseases including type one and type two diabetes; medical researchers have found the root cause of Wolfram syndrome and identified a handful of potential FDA-approved drugs for treatment, as well as a new group of drugs that may stop the brain and pancreatic cell death caused by the disease.

Here’s the problem: even though the research required to get things finalized and to get the right drug into the hands of suffering children before it’s too late is relatively inexpensive, the power brokers in the government and the billion dollar pharmaceutical industry that could help with completing clinical testing for the drug candidates before it can move on to clinical trials aren’t lifting a finger. And kids are dying while miles of red tape clog the system.

“Funding could come from the U.S. government via Department of Defense grants, but it is very hard to get to them. You really need political contacts to get to the Appropriations Committee to make any real headway,” says Wolfram’s expert Dr. Saad Naseer. “What is true is that the government has grossly underfunded rare disease research over the years and only recently has the NIH (National Institutes of Health) focused more on this. If the government won’t help, there needs to be a shift where big pharma invests more money in rare disease as opposed to just small biotechs who relatively speaking have only pennies.”

“At the end of the day, the world of big pharmaceuticals and medical research is incredibly competitive and very money driven; any number of groups could fund this research and potentially find a life changing treatment not just for Wolfram’s, but for people with diabetes. The amount of money needed is so relatively small, it would essentially be a rounding error for these guys, but we can’t even get them to acknowledge us at all. It’s a real David vs. Goliath type situation. I know that we’re on the cusp of a cure that can save kids like Raquel, but also have even major significance beyond implications for Wolfram syndrome that could help millions with diabetes. I just need someone to listen to us, and to care. Maybe as we head into the holidays, someone will,” says Dr. Naseer.

Talk with Dr. Saad Naseer (pronounced ‘Sod Nah-seer,’ born in the US, perfect English, Midwestern accent) about:
- What life is like for children with Wolfram’s syndrome
- What’s the link between Wolfram’s and diabetes, and how are they different?
- What’s the real holdup on this research, and how close are they to a cure?
- If people want to help fight Wolfram’s syndrome, what can they do?

Schedule your interview with Dr. Saad Naseer today by contacting Allen Media Strategies Jessica Lloyd at (703) 589- 8960 or jessica@allenmediastrategies.com. Find out more about Wolfram’s syndrome at www.thesnowfoundation.org.

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