



Parents of sick Canadian boy on 'Lorenzo's Oil'-like quest for treatment

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By Tobi Cohen

TORONTO (CP) - Jacob Trossman can't speak, eat or sit up by himself but his parents make sure that he leads as full a life possible.

The four-and-a-half-year old goes to summer camp, has been attending a special school with other children since he was about 18 months old and adores his two-year-old twin sisters Jamie and Sierra, who like to make him laugh.

On Wednesday, the bright-eyed Toronto boy will make his North American television debut on ABC's "Nightline."

Jacob has a severe case of Pelizaeus-Merzbacher disease (PMD), a degenerative neurological disorder that can't be cured let alone treated with any proven medication.

His family has decided to hit the airwaves in the hopes of generating awareness about the rare genetic disease and support for a new drug that's shown promise in treating PMD in lab mice.

"I'm not kidding myself in thinking that it's a magic bullet that will make everything perfect," said Jacob's mother, Marcy White.

"But if it's able to give him some type of normalcy, or some way to vocalize, or a little bit of head control or muscle tone to help him do some things independently, it would just... open a whole world for him."

Jacob has trouble breathing and swallowing, has to be fed by a tube inserted into his stomach, is confined to a wheelchair and requires round-the-clock, one-on-one care. Caused by a lack of myelin, the fatty tissue that insulates nerve fibres in the brain, the disease is expected to get worse as Jacob gets older.

Any treatment that would make Jacob's life easier and more enjoyable "is just something that we have to do our best to track down to the ends of the earth," said White.

That's precisely what his parents have done since Jacob was diagnosed when he was 10 months old.

Their quest has drawn parallels with the 1992 Oscar-nominated movie "Lorenzo's Oil," in which the parents of a boy afflicted with a rare disease go to great lengths in search of a cure.

White and husband Andrew Trossman have taken it upon themselves to comb through medical journals, scour the Internet and set up meetings with researchers in an effort to find a treatment.

They eventually found a McMaster University veterinary researcher who was studying a drug for other purposes, but considered its potential and brought it to the attention of Dr. Jim Garbern, a neurology professor and PMD researcher at Wayne State University School of Medicine in Detroit, Mich.

Garbern received permission from the pharmaceutical company that's been testing the drug as a treatment for spinal chord injuries to begin testing its effectiveness on the symptoms of PMD.

Over the last month, he's finally begun to see some positive results.

"Where the mice that would normally, without any treatment, have a very exaggerated tremor in their hind limbs when they're walking, the animals, after about a day of treatment, had much better control of their limbs," said Garbern.

"They could walk more like normal mice and the effects seem to be sustained."

Garbern said the drug has so far had little effect on lifespan and doesn't appear to control the seizures which are common in severe cases of PMD. Pending further testing, he suggested it might be most effective in treating PMD when used in combination with other medications.

"Generally speaking, scientists don't like to announce results until they've really been through the grinder... so I wouldn't say that we're there yet," he said.

"I'm guardedly optimistic that it's working, but I want to make absolutely sure of that before we review all of our data and present it to the pharmaceutical company."

Should the drug's producer agree with his final analysis, Garbern is hopeful he'll be able to begin clinical trials soon though he doesn't want to get "hopes up too high, too early."

As far as White is concerned, time is running out.

Since children's brains develop most within the first five years of life, White would like to get the drug into Jacob before his brain starts showing serious signs of degeneration.

"The earlier we can get this drug into him the better chance he has," she said. "Our window of opportunity is closing as he gets older."

Besides generating awareness and much needed funding for research, White hopes publicizing her son's story will spark interest among those in the Canadian medical community which has so far focused little on PMD, which is estimated to affect just one in about 500,000 live births.

She's hoping for help lobbying the government so Jacob can gain compassionate access to the drug once it's approved for clinical trials.

Jacob's story will air Wednesday on ABC's "Nightline" at 11:30 p.m. EST.

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