Expensive drug gives hope to rare disease sufferer

Family wonders who will pay bill

Trevor Pare, 16, suffers from a rare form of muscular dystrophy. He and his mother, Linda, worry his condition will worsen if the province doesn't pay for a drug that costs up to $400,000 a year.
Photograph by : Mikael Kjellstrom, Calgary Herald

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Trevor Pare glides across the floors of Innisfail's R and R Inn and Suites in his wheelchair, making reservations and delivering towels to guests.
Three years ago, the teen's muscles were useless. He could barely hold his head up because of a rare form of muscular dystrophy called Pompe disease.
Now Pare is attending high school, working and dreaming of a career in tourism, thanks, he says, to a new drug called Myozyme, which he is receiving through a compassionate program offered by the drug's manufacturer, Genzyme Canada.
Genzyme Canada put Myozyme on the market Thursday after Health Canada approved the enzyme replacement in August.
But barely a day on the market and the drug is already causing controversy, in large part because of its six-figure price tag.
The drug costs around $60,000 per year to treat an infant and $400,000 or more per year to treat a large adult.
As provincial governments, including Alberta consider whether to pay for the drug, Pare faces a future that could return him to a life without hope.
There are fewer than 100 Canadians with the rare disease. Many of those patients are now fighting with provincial governments to pay for the new treatment -- including Pare, 16, who has been taking the drug for 2 1/2 years. "I was tired all the time. I didn't do anything and I was tired," he says. "I feel so much better now. I have my life back."
Canadian Association of Pompe vice-president Ian Macpherson has been given a free supply of the drug by the manufacturer for now.

"It's scary. I'm fortunate to be on it now, but they can't afford to give it to me for free for a lifetime," he said.
Linda Pare faces that same dread for her son, Trevor. When he was diagnosed at 11 months, "doctors told me to take him home and love him because he'll be gone before he's two," she said.
Trevor was lucky. He thrived, learning to walk when physicians said he never would. But at 13 years old, Pompe disease forced Trevor into a wheelchair. Fatigue made even the most basic movements impossible, including moving his own head.
There was very little doctors could do to treat this condition beyond admitting patients to the hospital until they died, said Dr. Robin Casey, medical director of the inherited metabolic disorders program at Alberta Children's Hospital in Calgary.
Patients with this genetic disorder lack enzymes that break down sugars used for energy. The buildup of sugar in the body weakens muscles until the individual can no longer walk or breath on their own.
Casey had some of his patients participate in the Myozyme drug trial and said it doesn't appear to reverse muscle damage already sustained, but seems to slow or even stop further deterioration.
"It feels wonderful that for the first time we have a treatment that can seriously change the progression of the disease," Casey said.
Genzyme Canada claims its new treatment, given intravenously every two weeks, is also life-saving. Only two per cent of untreated babies diagnosed with Pompe typically live past 18 months. In a drug trial, the pharmaceutical company says 83 per cent of the 18 infants given the drug were living free of ventilators and other muscular conditions at 18 months old.
There are concerns with the drug. According to the company website, there is a risk of life-threatening anaphylactic reactions, including anaphylactic shock, when the medication is infused into the body.
Also, the drug infusions have been associated with heart and lung failure.
Still, one of the biggest concern so far appears to be who will pay for the treatments.
The drug is currently being analyzed by the national Common Drug Review, a committee of experts that conducts rigorous reviews of the clinical and cost effectiveness of new drugs. It will recommend whether provincial governments should fund Myozyme.
Alberta Health and Wellness will decide whether it will pay for the drug once the review is complete, department spokesman Howard May said.
Gaining approval through the review is crucial for the pharmaceutical company because very few can afford $400,000 per year, says University of Victoria drug policy researcher Alan Cassels, who is critical of the company.
He called it obscene for a pharmaceutical company to bring a "life-saving" drug to the market at such prices. "To me it resembles a hostage situation. If your life is being held hostage, how much are you willing to pay?"
Trevor Pare's mother, Linda, lives in fear Genzyme will stop paying for her son's drug supply. She says the province shouldn't wait until the Common Drug Review completes its assessment to start providing the treatment. It needs to pay for Myozyme now.
"There's people across Canada that are living day-to-day with their bodies shutting down and they can't get the drug because no one can pay that kind of money," she said. "This is terminal. It's proven it works. What is a Common Drug Review to us?"
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