

Request for Myozyme Enzyme Replacement Therapy for Brad Crittenden In British Columbia

Prepared by John Crittenden, Brad's father
john@johncrittenden.com

Brad's on Facebook

A HREF="<http://www.facebook.com/group.php?w=wall&gid=275408587627>"

Brad's interview on CHBC News and Global Jan 27, 2010

<http://www.chbcnews.ca/video/index.html?releasePID=uFAQzIqsszNsGkElidP6Vhgs3uEm3WH>

Myozyme for Adult-onset Pompe disease is publicly funded in over 40 countries around the world including the US, numerous European countries like the UK, France, Italy, Germany, and even countries like Croatia, Slovenia, Turkey and Venezuela. In some countries, funding is unrestricted and in other countries it is on a patient by patient basis.

Media contacts - Medical and Patients

Brad Crittenden

112-3201 Wilson Street
Penticton BC V2A 8J3 Canada
bcrittenden@telus.net
Mobile: (250) 488-2571

Dr. Sandra Sirrs MDFRCPC

Medical Director
Adult Metabolic Diseases Clinic
2775 Laurel Street, 4th Floor
Vancouver, BC
Phone: 604-875-5965
(Brad's Doctor)

Dr. Priya Kishnani

Top leading physician in the U.S.
for Pompe Disease
Professor of Pediatrics
Division Chief, Medical Genetics
Duke University Medical Center,
kishn001@mc.duke.edu
919-668-1100 • 1-800-med-duke
kishn001@mc.duke.edu

Dr. Robin E. Casey BSc, MSc, MD

Associate Professor
Departments of Medical Genetics
and Paediatrics
University of Calgary
Tel: (403) 955-2285
Fax: (403) 955-3091
robin.casey@albertahealthservices.ca

Dr. Aneal Khan, MSc, MD, FRCPC, FCCMG

Assistant Professor of Medical Genetics and
Pediatrics, University of Calgary
2888 Shaganappi Tr NW
3rd Floor Metabolic Clinic
Alberta Children's Hospital
403-955-7587
khaa@ucalgary.ca

Dr. Gail Ouellette, Ph.D. CCGC

Geneticist/Certified genetic counsellor
Quebec Portal for Orphan Genetic Diseases
Member, Canadian Association of Pompe
gail.ouellette@pqmgo.org
<http://www.pqmgo.org/>

Dr. Durhane Wong-Regier's

Canadian Organization for Rare Disorders
9011-142 St, NW Edmonton, Alberta
durhane@sympatico.ca
<http://raredisorders.ca/>

Kirsten Harkens

Executive Director
Canadian Society for MPS
and Related Diseases
Kirsten@mpsociety.ca
<http://mpsociety.ca/page/home.aspx>

A few Pompe Patients in Canada

Linda & Trevor Pare

adult now on Myozyme
<http://pompecanada.com>
3423-51 A Ave, Innisfail, Alberta
403-227-4960
403-391-9026
tpare@telusplanet.net

Guy Ashford-Smith

adult now on Myozyme
7051 Cradiz Crescent,
Mississauga, Ontario
905-821-0623
guyashford@aol.com

Ian MacPherson

adult now on Myozyme
23 Baker Street
Hamilton, Ontario
905-524-0870
ian.emagine1980@gmail.com

January 21, 2010

To whom it may concern,

Brad Crittenden, who lives in British Columbia, has Adult-onset Pompe disease. Currently British Columbia does not provide funding for this disease although it can be treated with an enzyme replacement therapy (ERT) using a new drug, Myozyme.

In April, 2008, Alberta began funding ERT for Adult-onset Pompe disease.

In March, 2009 Ontario also began funding ERT for Adult-onset Pompe disease.

British Columbia is not. Brad is not receiving treatment because he lives in BC.

In April 2009, Brad was told that one diaphragm was already paralyzed. His energy level had dropped considerably and he was having difficulty breathing and walking. If he loses the other diaphragm he will be on a ventilator and in a wheelchair for the rest of his life.

In September, 2009, when asked by his doctor, Brad agreed to participate in a two year trial that his doctor was applying to have funded by the BC government. It has been several months since that application was made and there has been no response from the government. Upon inquiring myself a couple days ago I was told by the BC Ministry of Health [paraphrasing] that it will take many months to get a reply and even then it may well be refused because they didn't think ERT helped with Adult-onset Pompe disease.

Yet, I've attached documentation from patients and their doctors that ERT absolutely is effective for Adult-onset Pompe disease. That documentation is included herein.

Brad does not have the luxury of waiting. He is now on a breathing machine at night and part of the day, and his health is continuing to deteriorate.

Brad should be receiving Myozyme now before he deteriorates any further. The BC government needs to do the right thing and fund treatment for Brad immediately until their assessment is completed.

Anything you or anyone you know could do to help us would be appreciated. Brad needs your help.

Sincerely,

John Crittenden

A very concerned father

Testimonies from Patients and their doctors

This shows that Myozyme definitely does work

Pompe disease survivor eager to see movie concerning the affliction

<http://www.albertalocalnews.com/reddeeradvocate/news/local/>

[Pompe_disease_survivor_eager_to_see_movie_concerning_the_affliction_81964417.htm](http://www.albertalocalnews.com/reddeeradvocate/news/local/Pompe_disease_survivor_eager_to_see_movie_concerning_the_affliction_81964417.htm)

Trevor [Pare] was accepted in [a] clinical trial in the United States starting in January of 2004 on the [Myozyme] therapy which Linda [Trevor's mother] said saved his life. She believes that, had Trevor been three years younger when he first started the therapy, he would still be walking today.

Trevor and Linda launched a very public campaign, making their case with the media and begging the provincial government for help on behalf of all people who suffer from rare diseases that require expensive treatments.

Then Health Minister Ron Liepert made two unescorted visits to their Innisfail home to talk about the problem. Trevor never asked for help for himself. He asked for help for everyone in his predicament, she said.

The family was away on Mother's Day in 2008 when Liepert hand-delivered an envelope containing unbelievably good news. The province had found a way to fund drug programs for people with Pompe and other rare diseases.

It was public awareness, made possible through the media, that helped push for that change, said Linda.

Time for my miracle

<http://www.mississauga.com/opinion/columns/article/249647--time-for-my-miracle>

Guy Ashford-Smith, 52, is a longtime Mississauga resident. He was diagnosed with Pompe Disease in 2001.

The tag line for the movie is, "Don't wait for a miracle. Make one!" Crowley, a Harvard MBA, formed a company, financed research and ultimately found a treatment for this disease. He truly made a miracle.

This is where I come in. I also have Pompe. I'll be having my first dose of John Crowley's miracle tomorrow. I have the adult onset form of this rare disease.

Since shortly after my diagnosis, I've been working to get this treatment for myself and others here in Canada. My fellow American sufferers have been receiving this treatment for years. Since suffering respiratory failure and being admitted to Credit Valley Hospital (where their miracle workers saved my life), I have fought with the Ontario Health Ministry for this treatment.

I am tired most of the time. I wake up in the morning with no energy. Even worse, I don't look "sick." To the uninformed, there's nothing wrong with me.

The Ministry of Health has said that the delay in approval for this treatment was for them to develop a protocol. They have taken years to do this. These are years I can never get back.

I get my first treatment tomorrow. I'm truly grateful for John Crowley and his miracle and I hope it works for me. I'm way too tired to make my own.

Perhaps I'll regain some energy and truly be able to engage the Health Ministry on the treatment for rare diseases issue. I'd enjoy the irony of them treating me so that I can beat up on them. Turnabout is fair play.

True-story film tells of ordeal with rare disease

<http://www.toledoblade.com/apps/pbcs.dll/article?AID=/20100123/NEWS32/1230362>

Mr. Goethals has been on a real-life drug for Pompe disease since he was selected five years ago to be one of 90 people nationwide in a clinical trial, first at the University of Pittsburgh and, starting in September, 2006, at St. Vincent.

Three patients at St. Vincent participated in the clinical trial for Myozyme, which was developed by Genzyme Corp. and has been approved by the Food and Drug Administration.

"The trial suppressed the progression of the disease and saved my life," said Mr. Goethals, who continues to get Myozyme at St. Vincent, which is registered to administer the drug.

Mr. Goethals' pulmonologist, Dr. James Tita, said he first developed an interest in Pompe disease 25 years ago. Traditionally, infants who exhibited Pompe symptoms died from heart and respiratory failure within a year, while those who did not have problems until later in life typically needed ventilators to survive, he said. There still is no cure for Pompe disease, but Myozyme provides hope for infants and slows the disease's progression in adults, Dr. Tita said.

"There's just been tremendous advances," said Dr. Tita, St. Vincent's section chief of pulmonology.

Summary of enclosed documentation

Pompe LOTS early abstract

Safety and efficacy results from a randomized, double-blind, placebo-controlled study of alglucosidase alfa for the treatment of Pompe disease of juveniles and adults.

Patients were >8 years old, ambulatory, free of invasive ventilation, and had quantifiable respiratory and lower extremity muscle weakness.

Results: [This study consisted of] 90 patients (45 male and 45 female, 93% Caucasian; age range 10-70 years) were randomized. Baseline mean 6MWT distance was 327.4+/- 128 meters (50.1% predicted) and mean FVC was 54.6+/- 14.8% predicted, indicating considerable disease burden at baseline. By last evaluation, estimated mean absolute differences of 28.1+/- 13.1 meters in 6MWT distance ($p=.03$) and 3.4 +/- 1.2% in % predicted FVC ($p=.003$) were observed in favor of alglucosidase vs placebo.

Conclusions: In this first placebo-controlled study of alglucosidase in juveniles and adults with Pompe disease, treatment was shown to improve walking and pulmonary outcomes compared to placebo.

Improved or stabilized respiratory function in 3 patients with Pompe disease after starting recombinant alfa-alglucosidase (rhGaa) - a case to maintain a steady course with treatment.

Aneal Khan, Colleen McNeil and Robin Casey
University of Calgary, Alberta Health Services, Calgary Health Regions

Subjects: 3 patients of which all showed impairment of respiratory function measured by the % predicted FVC when upright prior to start of rhGAA.

All patients initially started on 20 mg/kg qow (every other week) rhGAA.

RESULTS: [I've shown only the results for the 44 year old patient here because this is closer to Brad's age]

- Patient 1 showed a decline in % predicted FVC from 78% to 67% prior to enzyme replacement and has shown a steady and consistent increase to 87% over 2 years at 20 mg/kg rhGAA qow.
- Individual measurements of FVC were quite variable, but using serial measurements, there appears to be a gradual but consistent stabilization of % predicted FVC or improvement after 1.5 years of rhGAA.
- Patients 2 and 3, who were using nocturnal BiPAP at the start of rhGAA have not shown progression to invasive ventilation for over 2 years since the start of enzyme replacement.

(Please see the attachment for additional details.)

Eight years experience with enzyme replacement therapy in two children and one adult with Pompe disease

http://www.sciencedirect.com/science?_ob=ArticleURL&_udi=B6T9T-4SM2062-2&_user=10&_rdoc=1&_fmt=&_orig=search&_sort=d&_docanchor=&view=c&_searchStrId=1176000997&_rerunOrigin=google&_acct=C000050221&_version=1&_urlVersion=0&_userid=10&md5=0203a08ae2342eebef1671c81b4568e2

"Two severely affected patients, wheelchair and ventilator dependent, who had shown stabilization of pulmonary and muscle function in the first 3 years, maintained this stabilization over the 5-year extension period. In addition patients became more independent in daily life activities and quality of life improved.

The third moderately affected patient had shown a remarkable improvement in muscle strength and regained the ability to walk over the first period. He showed further improvement of strength and reached normal values for age during the extension phase."

Improvement with ongoing Enzyme Replacement Therapy in advanced late-onset Pompe disease: A case study

http://www.sciencedirect.com/science?_ob=ArticleURL&_udi=B6WNG-4TPND7C-2&_user=8895552&_coverDate=12%2F31%2F2008&_alid=1176026254&_rdoc=9&_fmt=high&_orig=search&_cdi=6962&_sort=r&_docanchor=&view=c&_ct=213&_acct=C000050221&_version=1&_urlVersion=0&_userid=8895552&md5=7a1f2cda3ed1853033fcd2755a54c50d

"We report a case of a 63-year-old patient with significant morbidity who made notable motor and pulmonary function gains after two years on therapy. Thus, improvements in those with advanced disease may be possible after long-term treatment."

Australian Pompe's Association

July 2009: This is an informal survey of Australian patients who suffer from Pompe's Disease, to show the effects of treatment with Enzyme Replacement Therapy - MYOZYME.

The ages at the start of Myozyme treatment range from 24 to 67 years old, with an average being 50 years, 6 months. The period of treatment ranges from 3 months to 3 years 5 months, with the average period being 1 year 11 months.

Comments and summary: Had treatment been available to us at the time of diagnosis (see table on page 3), we would not have suffered the devastation that is Pompe's Disease, with its always progressing weakening of muscles, loss of functioning and compromised breathing - and we would not now need the care of our partner, parents, siblings or children. Or worse, be placed in a Nursing Home at a much too early age. Not only does that bring much emotional trauma and heartache to the patients and their families, but it will also bring with it considerable costs to the Australian Government for many years to come.

(Please see the attachment for the dramatic results in the patients own words.)

Attachments

POMPE LOTS EARLY ABSTRACT _ LAFORET.pdf

Copy of Survey2-AusPatientsOnMyozyme-July2009-FINAL (2)-2.pdf

Pompe respiratory Munich Khan.pdf

Article: **Pompe disease survivor eager to see movie concerning the affliction**

Article: **Time for my miracle**

Article: **True-story film tells of ordeal with rare disease**