



Extraordinary Measures the Movie: A Discussion by Pompe Disease Support Groups



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For Immediate Release

Background

Extraordinary Measures is a new movie scheduled for release in America on January 22, 2010. Starring Harrison Ford and Brendan Fraser it tells the story of a family struggling with a rare disease—the neuromuscular condition known as Pompe Disease (aka Acid Maltase Deficiency).

The Acid Maltase Deficiency Association (AMDA) is a US patient support group for sufferers of Pompe Disease. The International Pompe Association (IPA) is a federation of nearly 40 national patient groups around the world who collectively support over 1000 patients and their families.

Pompe disease is an extremely rare, progressive neurological condition that affects people of all ages; the classical infantile form is particularly severe as it particularly damages the heart, respiratory muscles and skeletal muscle. Older infants, children and adults are affected to varying degrees and without treatment all may suffer from loss of muscle function and breathing difficulties. Quality of life is reduced and life expectancy is considerably shortened; most untreated infants do not live beyond their first birthday. Every patient has their own unique story, and every family is affected in its own way.

Extraordinary Measures

The film Extraordinary Measures focuses on the actions of one highly-motivated individual and his collaboration with a medical researcher. It shows how Pompe disease, a devastating illness, changes family life forever, something that is true whatever the severity or age of the sufferer.

It is a remarkable story about the man and his quest to find a treatment for his children – but it is NOT the story of the development of a treatment for Pompe disease; that is a misconception implied by the film and spread by other commentators.

The real story behind the development of an effective treatment involved close collaboration between families, researchers and industry and was underway long before John Crowley's involvement. The presence of his company did not lead to the development or acceleration of a therapy for Pompe disease; that process was already making good progress elsewhere.

The missing part of the Hollywood story is that the development of a treatment involved an *international* Pompe community; this has been airbrushed out of the picture by the movie and the associated book. Since the early 1990's the AMDA and other IPA affiliated organizations have together been very closely involved with the research and subsequent development of an Enzyme Replacement Therapy (ERT) for Pompe disease. We have long worked with the Dutch team of researchers at the Erasmus Medical Centre (Rotterdam). These trail-blazing pioneers, proved the principle of ERT for Pompe disease, carried out the first successful clinical trial and led the path towards a commercial therapy. They did so on a shoestring budget, in the face of indifference from

the medical establishment. It is therefore particularly disappointing that no mention is made of their contribution.

It is apparent by the end of the movie that the therapy is certainly not a miracle cure and it is for that reason that the Pompe community, through the AMDA and IPA, continues to work with researchers and industry towards future therapies. However, it is important to remember that ERT is helping hundreds of patients around the world *today*.

Research and patient communities fighting rare diseases such as Pompe yearn for publicity to help them reach out to the public, patients, families, medical professionals and politicians, and for that reason we are very grateful to this movie.

As a community we hope that this film will lead to a greater awareness of Pompe disease and thereby improved diagnosis of the condition. We hope that the information about the disease will help families quickly find support from both medical professionals and patient support groups, and that screening programs to accurately identify the disease will be given a high priority.

We hope the community at large will realize the critical importance of newborn screening – by far the best way to identify the disease so that timely treatment can limit irreparable muscle damage, giving those affected the best opportunity to lead a normal life

Recommended Reading

IPA founding member Kevin O'Donnell is currently writing a chronicle of events that led to the development of the commercial Enzyme Replacement Therapy for Pompe disease.

“Pompe Disease – The Real Story” can be read online at:

<http://pompestory.blogspot.com>

Whilst we would recommend that you read the whole story, if time is limited there is a search facility to locate subjects of interest.

Contacts

The AMDA and IPA have connections with a number of families touched by Pompe disease who are eager to share their stories; if you would like more information about our work or would like to interview these families please use the contact details below.

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