



Advertisement

News:

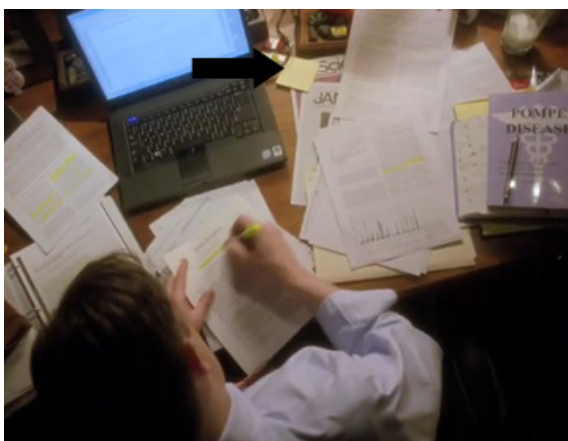
## A review of *Extraordinary Measures*

Posted by [Jef Akst](#)

[Entry posted at 22nd January 2010 12:05 PM GMT]

[Comment on this news story](#)

Rare diseases and drug discovery don't usually make for Hollywood blockbusters. But today (January 22) a film about a genetic affliction that strikes fewer than 10,000 people worldwide hits movie screens, and it has some serious star power behind it. Harrison Ford and Brendan Fraser head up the cast of *Extraordinary Measures*, a new movie that may lift Pompe disease from the shadows of obscurity into the spotlight, as the focal point of an inspirational story of paternal love and scientific innovation.



A screen shot from the movie. Note *The Scientist's* poking cameo appearance next to the computer (black arrow).

Image: YouTube trailer (screen shot)

"The movie is a great exposure for a rare genetic disease," said Duke University School of Medicine's [Priya Kishnani](#), who studies Pompe and participated in much of the research that led to the first and only approved treatment for the disease -- a quest that forms the central plot of the film. "I would have never thought in my lifetime, a disease that I'm so passionate about would make it into mainstream Hollywood cinema."

*Extraordinary Measures* tells the tale of businessman [John Crowley](#) (played by Fraser) who makes it his mission to promote the development of an enzyme therapy to treat his two youngest children, who are sick with Pompe. Teaming up with University of Nebraska researcher Robert Stonehill (Ford), Crowley starts a small biotech company called Priozyme dedicated to his purpose. (In reality, the researcher who helped Crowley was William Canfield of the University of the Oklahoma Health Sciences Center, and the company Crowley launched was called Novazyme.)

Pompe disease is a genetic lysosomal storage disorder. Any of the nearly 300 mutations in the lysosomal enzyme acid  $\alpha$ -glucosidase (GAA) gene that affect its function can result in either infantile- or late-onset accumulation of glycogen in muscle tissues. Pompe destroys skeletal muscle, impairing motor skills and usually relegating patients to wheelchairs. The disease also compromises lung and heart function, with most Pompe patients ending up on ventilators with inflamed and failing hearts. Infants with Pompe disease typically survive less than one year, while late onset patients can suffer only mild symptoms for years before experiencing an abrupt decline.

As the movie depicts, Novazyme was eventually bought out by [Genzyme](#) (Zymagen in the movie), a larger biotech company that was also developing a Pompe therapy and had purchased two others. Now with four different enzymes to choose from, Genzyme ran what they call the "Mother of All Experiments," pitting the four candidates against one another in blinded biochemical analyses and mouse model tests to see which held the most promise. Once the results were in, a group that included Canfield, as well as Genzyme's vice president, Robert Mattaliano, selected the winner, revealing the drug's identity by decoding the color-schemed key.

### LATEST NEWS

[A review of Extraordinary Measures](#)

[deCODE reborn](#)

[DNA factory launches](#)

[Wanted: Records of revoked grants](#)

[NSF slaps school over grant](#)

[Marshall Nirenberg dies](#)

[Ancient humans more diverse?](#)

[News in a nutshell](#)

[Tips to safely provide records](#)

[Engineering cellular synchrony](#)

[Is America competing?](#)

[Playing with plastic](#)

[Cancer lines contaminated](#)

[Haitian AIDS clinic still standing](#)

[Glial cells aid memory formation](#)

[More Entries...](#)



### Supplements

[NRW: Biotechnology in North Rhine-Westphalia](#)

[Life Sciences in Ireland](#)

[Schizophrenia](#)

[Autoimmunity](#)

### Survey Series

[Best Places to Work](#)

[Salary Survey](#)

[The Scientist Video Awards](#)

[Lab Web Site and Video Awards](#)



### The Scientist Daily

Science headlines delivered daily. Register today.

### Institutions

a Chinese hamster ovary cell line -- was the subject of three clinical trials: one for infantile-onset Pompe patients, one for patients aged 3 months to 3.5 years, and one for the two Crowley children, who were able to receive treatment at a local New Jersey hospital near their home after their father left his position at Genzyme.

The trials showed significantly decreased mortality, and patients were able to survive longer without the aid of a ventilator, and some even demonstrated increased motor function. In 2006, the treatment, an intravenous enzyme replacement therapy dubbed Myozyme, became the first and only approved treatment for Pompe disease. While Crowley's children are still on respirators, they are responding well to their Myozyme treatments, which they continue to this day.

*Extraordinary Measures* tells this story with remarkable accuracy (albeit with some minor Hollywood name changes and plot tweaks), and shines a light on Pompe that rarely touches most rare diseases. But somewhere in the recounting of this emotionally driven tale, the viewer, at least the scientifically-inclined one, is alienated from the realities of the disease. The sick children, while on ventilators and in wheelchairs, speak with ease -- an overly optimistic picture of Pompe patients, who often have much more difficulty communicating. The movie also typecasts theoretical endeavors as idealistic and even useless, dismissing a branch of research that provides the basis of most bench and clinical work.



The film does a better job of representing the hard-to-swallow fiscal issues of drug development. "The movie emphasizes how these things can be monetized," University of Florida pediatric cardiologist [Barry Byrne](#) told *The Scientist*. "Everything cannot be done at the research level," added Byrne, who was involved in the clinical trials of Myozyme. "Without investments from a company like Genzyme to bring these products to patients there would be no benefit to patients in the long run."

Since the development of Myozyme, research on treatments for Pompe disease has continued and expanded. Most recently, Byrne opened a [clinical trial](#) to test a gene therapy supplement to the enzyme replacement therapy. A handful of biotech companies are also developing new Pompe drugs, including Genzyme which is currently working on their second generation compound.

"It's really striking that for such a small community of patients that there is so much interest," Byrne said. "And [hopefully it will be] catalyzed by the public interest in the film."

#### **Related stories:**

- [A lab goes to Hollywood](#)

[March 2006]

- [The Scientist Video Awards: 2009](#)

[August 2009]

- [Communicating through movies](#)

[14th February 2005]

---

Advertisement

---

**Rate this article**

---

Rating: 4.67/5 (3 votes )

[Comment on this news story](#)

---