

Jonah's Just Begun



PO. Box 150057
Brooklyn, NY 11215

www.jonahsjustbegun.net



Mission

Jonah's Just Begun Foundation to Cure Sanfilippo is dedicated to driving research for Sanfilippo Type C and raising awareness about all rare diseases. The foundation raises funds and distributes them to academic researchers to further their work in this disease area. JJB also encourages others affected by rare disease to advocate for cures. JJB was founded by Jeremy Weishaar and Jill Wood after their son, Jonah, was diagnosed in 2010.

Strategy/Approach

Since beginning its journey to cure Sanfilippo Type C in 2010, Jonah's Just Begun has focused on developing basic and translational research for the disease as well as educating the public about rare diseases and the need to advocate for cures. In working with other Sanfilippo Type C focused foundations across the globe, JJB is able to advance research and cures for this subtype of a rare disease. Laying the foundation for how to treat the disease, through mouse model development and basic research, has positioned JJB to move forward with finding a compound to fit the treatment needs of the disease.

JJB Foundation hosts annual events and fundraisers throughout the year to raise funds for Sanfilippo Type C research. Crowdfunding and grassroots organizing have also been crucial for this foundation to get their message out. The foundation attends symposiums and works closely with organizations who promote open source scientific data.

Research Portfolio

Research sponsored by the foundation focuses on three main areas of potential treatment: chaperone therapy, gene therapy and substrate reduction therapies. Additional areas of interest include using animal models to understand and cure Sanfilippo disease, treating neuropathy associated disease progression, and understanding the cause of dementia in Sanfilippo disease.

- Chaperone therapy—efforts to identify chaperone molecules with high throughput screening and computational screening.
- Gene therapy—direct brain delivery of a gene vector, assessing issues of immune response & scale up problems.
- Substrate Reduction Therapies (SRT)—reduce substrate production, address long term toxicity of drugs and ability to cross the blood brain barrier.

Partnership Practices

Jonah's Just Begun is a partner in the HANDS (Helping Advance Neurodegenerative Disease Science) consortium. This

consortium includes other patient groups supporting research for Sanfilippo Type C and global leaders in research and treatment of neurodegenerative lysosomal storage disorders.

Consortium Members:

- Jonah's Just Begun Foundation to Cure Sanfilippo Inc.
- JLK-Sanfilippo Research Foundation
- Sanfilippo Sud
- Sanfilippo Barcelona
- Alexey Pshezhetsky, University of Montreal
- Brian Bigger, University of Manchester
- Alessandro Fraldi, TIGEM

The alliance does not partner with biotech and pharmaceutical companies. *FasterCures* is not aware of any formal collaborative initiatives between JJB and industry.

Financials

Jonah's Just Begun is a non-profit 501(c)(3) foundation. The most recent financial information available is for 2012.¹

Year Ending 12/31/12

- Revenue: \$92,653
- Assets (market value): \$16,026
- Grants: \$98,100
- Gifts Received: \$92,653
- Expenditures: \$102,131

Key Accomplishments

- Formation of HANDS Consortium
- Ongoing natural history study by Dr. Paul Levy, Children's Hospital at Montefiore
- Participation in CONNECT open-access patient registry by PatientCrossroads
- Developed mouse model for Sanfilippo Type C
- Founded Phoenix Nest, a biotechnology company, to commercialize discoveries made during research

Leadership

Board of Directors

- Jeremy Weishaar, President
- Jill Wood, Treasurer
- Raquel Marques, Vice President (Portugal)
- Nancy Burke, Secretary (JLK Foundation)

¹ Information obtained from GuideStar. Accessed March 2014.
<http://www.guidestar.org/organizations/27-2957730/jonahs-just-begun-foundation-cure-sanfilippo.aspx>

- Antonio Vicente, Director (Portugal)
- Sue Burlison, Director
- Chrisit & Jason Omeroid, Co-Directors (Levi's Life, Love & Laughter)

Scientific Advisory Board

- Wendy Chung, MD PhD
- Robert D. Steiner, MD
- Gregory M. Pastores, MD
- Sean Ekins, PhD DSc
- Sandra Catarina Conceicao Alves