

Help us celebrate our

15th ANNIVERSARY

Greg Marzolf Jr.

FOUNDATION

helping people with muscular dystrophy

2002-2017

15 Years of Fighting for a Cure for Families

Read just one family's story of how our fight for a cure is making a difference

Sophie Lathe, a smiley 10-year-old girl, was diagnosed with Spinal Muscular Atrophy (SMA) at a young age. SMA is a form of MD that causes weakness and muscle wasting.

In December 2016, everything changed for Sophie. The Food & Drug Administration approved Spinraza, the first drug for SMA. As Holly, Sophie's mother, states "We've been waiting and cheering on medical researchers and those who fund medical research for years. To know that we're now in the circle of those who will benefit from that research is an incredible feeling."

In February, Sophie was the first patient in the entire Midwest to commercially receive Spinraza. The team at the University of Minnesota MD Center worked diligently to make this possible – coordinating the procedure, working with the pharmaceutical company, managing financial needs, and appreciating family requests.

It will take some time before Sophie sees and feels the full benefits of Spinraza – but in the meantime, she is grateful.

"I hoped that researchers would find something to help people like me, but I never thought about what it would be like if I stopped getting worse. I think differently now. I feel blessed that so many people cared so much that they would research for so long to make Spinraza. I believe my life could be different than what I imagined before Spinraza. I think my life will be different--the better kind of different." – Sophie Lathe



15 Years of Fighting for a Cure with Local Researchers

The GMJF is proud to support Dr. Townsend's innovative work

The cost of clinical trials presents a significant hurdle for the evaluation of new therapeutic approaches in muscular dystrophy. The high costs of trials result from the need to enroll large numbers of ambulatory patients. These trials require large numbers, in part because of the current reliance on the 6- minute walk test (6-MWT). The 6-MWT is used to assess skeletal muscle function but is imprecise and highly variable. This test is used because it provides measure of how well muscles are working; but is also

affected by respiratory and heart disease, both of which are common in muscular dystrophy. In addition to these disease-related issues, the motivation of the patient to perform the test introduces additional variability.

To address these deficiencies, Dr. Townsend and his lab have developed a hand-held device that functions as a video game controller. The force applied to the hand-held device controls the movement of a character in a video game. This novel approach allows exercise protocols to be implemented in both ambulatory and non-ambulatory patient populations. Furthermore, the use of the video game will limit the motivational effects that affect almost all current assessments of muscle function. Dr. Townsend believes that this technique will dramatically lessen the costs of clinical trials, allowing more therapies to be evaluated and more quickly moving us closer to a cure.

15 Years of Invaluable Volunteers

The Innovation Award:



James Moeller, board member.

Patty Marzolf, president of GMJF, said: "Jamey never says no, has served in many roles, is willing to take risks, thinks big and does whatever is best for the vision and future of the foundation and ultimately for the patients and mission we serve."

The Champion Award:



Kathleen Moriarty, board member.

"There is nothing better to have in your corner than someone who truly and outwardly believes in your work and actually talks about it, encourages others to participate in events and recruits people to get involved," said Patty.

Greg Marzolf Jr. Foundation 2017 Grant Recipients:

The GMJF provided \$35,000 grants to two research labs at the University of Minnesota's MD Center. The winning grant recipients are:

Dr. Rita Perlingeiro: The GMJF grant committee determined to renew this grant due to the impressive headway that is being made by the Perlingeiro Lab. They have begun communication with the FDA, and a pre-IND meeting will be scheduled for March. They state in their application: "The support from the GMJF will be essential to move this research program forward, not only to enable the potential future treatment of DMD patients, but to lay the groundwork for what we believe may be one of the first diseases to be treated by a pluripotent-cell based therapy."

Dr. DeWayne Townsend: The high costs of trials result from the need to enroll large numbers of ambulatory patients. To address these deficiencies, Dr. Townsend's lab has developed a hand-held device that functions as a video game controller. See story above ^

15 Years of Fighting for a Cure by Fundraising



Join us Aug. 19 for
Rock the Pavement 5K & 10K

With the best 5K and 10K course in Minnesota, this is an event you won't want to miss! Join the Greg Marzolf Jr. Foundation as we Rock the Pavement on August 19, 2017. Choose to run or walk a 5K or 10K around beautiful Lake Calhoun! All proceeds from the Rock the Pavement 5K and 10K benefit the Greg Marzolf Jr. Foundation in the fight for a cure to end Muscular Dystrophy! Visit our website at www.gregmarzoljr.org to learn more and to register.

All Made Possible with the Support of Amazing Sponsors

Cause to Cook



Rock the Pavement



Here's How You Can Help Us Celebrate

Thank you for giving from your heart as we celebrate 15 years of ground-breaking change in our quest for a cure!

DONATE Visit GregMarzolJr.org/donate to donate online or mail in attached form.

PARTICIPATE Join us Aug. 19, 2017 for Rock the Pavement or Jan. 13, 2018 for our Cause to Cook for a Cure gala.

SPONSOR Contact us at sponsors@gregmarzoljr.org for information on becoming a sponsor.

SPREAD THE WORD
Invite family and friends to get involved and raise awareness.

DONATION FORM

Mail donations to:

Greg Marzolf, Jr. Foundation P.O. Box 16190 St.Paul, MN 55116

Name _____

Address _____

Email _____

Phone (_____) _____ - _____

Donate online @ www.gregmarzoljr.org/donate

check for \$ _____ . _____

Did you know...

...that since 2002, GMJF has raised over **\$1.5 million** to support the GMJF Basic Research Program, GMJF Clinical Grant Program and MD Center Family Camp?