



## SMS RESEARCH FOUNDATION

### June 2018 News & Events

## New Research! Request for Proposals

The SMSRF is beginning an **annual grant program to fund novel and scientifically sound ideas** from new and experienced researchers interested in SMS. Our goal is to foster collaboration and stimulate intellectual discussion in the SMS research community as we move the field toward a therapeutic intervention.

**DONATE HERE!**

#### Guidelines for Proposals:

- \$ 75,000 available in direct costs. Indirect costs of up to 8% permitted.
- Investigators need to have a faculty appointment of Instructor level or higher to serve as a principal investigator
- Brief concept paper required for initial review for scientific merit and project feasibility
- Full proposal will be requested after LOI reviewed if found suitable for further consideration
- Concept paper and grant proposals peer reviewed by SMRF scientific advisory board (SAP) utilizing a standardized grading scale

#### Deadlines:

- Concept paper due July 15, 2018
- Full proposal due September 1, 2018

For more information and full concept paper guidelines, please visit our [website](#).

## Gene Therapy Update

Researchers made significant progress on the gene therapy proof-of-concept phase over the last 6 months, and the following milestones were achieved:

- Researchers began exploring the delivery mechanism to modify the critical RAI1 gene, responsible for many of the challenging characteristics of SMS.
- Researchers analyzed, tested, and discovered that the RAI1 gene is too large for the typical virus used for gene therapy.
- Researchers developed five shorter versions of the gene to fit in the viral vector. None of the shorter versions, however, provided adequate functionality.
- Mice with SMS were bred at Baylor College of Medicine.

Baylor  
College of  
Medicine



In response to some of the challenges mentioned above, researchers will be taking three new parallel approaches. Our goals over the next 3-6 months are to:

- Test an alternative virus that could include the full RAI1 gene.
- Split the RAI1 gene into two smaller units and place each half in its own vector that would later be combined to form one full RAI1 gene after injection into the mice.
- Use the widely accepted CRISPR technology with a promoter (which turns on gene expression) in the viral vector. After injection, it would attempt to make one good copy of the RAI1 gene work as if there are two copies, thus re-establishing proper concentrations of the gene.
- Begin testing one or more of these approaches in SMS mice.

# Recent Event

## Logan's Lemonade Stand for Research

Los Angeles, CA - May 20

Thank you to **Jill, Jon and Avery Zemplak** for hosting their 1st Annual Lemonade Stand for Research in honor of their son/brother, Logan. Their event raised a phenomenal \$25,000 for SMS research!

They sold lemonade donated by Whole Foods and Trader Joe's, as well as cookies donated by local bakeries. They also sold t-shirts, cups, and lemon squeeze balls with the adorable lemon logo. Additional money was raised through a raffle and silent auction.

Jill wrote and produced several short videos about life with SMS. We've included one of her videos at the bottom of this newsletter for you to enjoy.



From left, Logan (8), Avery (11), Jill, and Jon Zemplak in front of Logan's Tree of Hope. Supporters bought a lemon for \$1, wrote a message to Logan, and taped it on the tree.



The lemonade stand was held at a recreation center at a local park in LA. If you would like to hold a **lemonade stand for research** in your area, please [contact us](#) for more information!

# Upcoming Events

## Wyatt's Walk & Run 5K Fundraiser

Mt. Juliet, TN - August 11

**Jessica and Eric Rogers** will be hosting their 3rd Annual Wyatt's Walk and Run 5K in honor of their four-year-old son, Wyatt (pictured right), on Saturday, August 11th.

This is a USATF certified course with chip timing. Medals will be awarded to overall first place male and female winners, as well as to the top three finishers in six age categories. Come out to support a great cause while getting your workout for the day!

Event registration will soon be available on the SMSRF website.



## Comedy Fundraiser

Pittsburgh, PA - September 29

**Courtney and Matt Frist** will host their Third Annual Comedy Night at the Pittsburgh Improv on September 29th to raise funds for SMS research.

The event will include a raffle, an auction, a pre-show mixer, and premiere comedians. If you live in the area, please come out for an entertaining evening!

Event registration will soon be on the SMSRF website.



## Recycle for Research Wilton, CT - Ongoing

**Ben and Cole Iannuzzi**, in support of their sister Sydney, are continuing their Recycle for Research fundraiser in their town of Wilton, Connecticut, to raise money for the SMSRF. If you live in the area, please consider supporting their cause by dropping off your empty water bottles so they can recycle them for SMS research.

For pick up or drop off information, contact Ben at [ben@smsresearchfoundation.org](mailto:ben@smsresearchfoundation.org). You can follow them on Facebook at [Recycle for Research](#) or on the SMSRF [website](#).



## Video from

This video was created by SMS mom, Jill Effron, and highlights our SMS kids' extraordinary ability to touch other people's lives with their fearless, outgoing personalities. Jill made a series of videos to support of her fundraiser, Logan's Lemonade Stand for Research, to educate friends and family about the many facets of SMS.



[DONATE TO SMS RESEARCH](#)

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*The mission of the SMS Research Foundation is to support research to improve the knowledge and understanding of SMS so that viable therapeutic options can be developed in order to improve the quality of life of those living with Smith-Magenis Syndrome.*

The SMS Research Foundation is a 501(c)(3) nonprofit organization founded in June 2010.  
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