



# SMS RESEARCH FOUNDATION

## March 2017 Research News & Events

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Greetings!

In January, the Smith-Magenis Syndrome Research Foundation (SMSRF) announced a new [Challenge Grant](#) in the amount of \$70,000. All donations made until **July 15, 2017**, will be matched dollar-for-dollar. You can double the impact of your donation by giving during this time!

Our gene therapy project is now underway at Baylor College of Medicine. Gene therapy is a promising method to treat rare genetic diseases, especially when they are primarily caused by a single gene defect, as is the case with SMS.

To help finance this project, two SMS families held fundraisers in March, and an additional three SMS families will host events in the coming months. For more details, please see below. If you are interested in hosting an event, please [contact us](#), and we will help you every step of the way.

Thank you for your continued support!

Sincerely,  
SMSRF Board of Directors

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**DONATE NOW - Challenge Grant**

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## Research Update

Of approximately 80 genes missing in SMS individuals, one gene in particular, called RAI1, is responsible for the majority of clinical abnormalities, specifically the neurobehavioral features.

Our gene therapy project aims to deliver RAI1 into the central nervous system to restore its expression levels and consequently correct aberrant neuronal function.

Researchers at Baylor College of Medicine, with support from Sanofi-Genzyme, will be generating proof-of-concept preclinical data, in mice with SMS, that support the potential testing of gene therapy in humans.



## A Interview with Liqun Luo (Scientific Advisory Panel & SMS Parent)

Dr. Liqun Luo is a member of the SMSRF Scientific Advisory Panel, a professor at Stanford University, and an SMS parent. Dr. Luo was recently interviewed by the Simons Foundation regarding a paper he published last year on the functions of the RAI1 gene and on his lab's future plans for studying SMS. [Read the full article here.](#)



## International Partnerships

We are incredibly grateful that two international SMS organizations have partnered with us on the gene therapy project. We would like to thank [Smith Magenis México](#) and [Pas à pas avec Alexia](#) for their generous support!

This project would not be possible without the generosity of our phenomenal donors and the SMS community worldwide.



Smith Magenis México



## Recent Events

### Strikes for SMS Bowling Fundraiser

Thank you to **Matthew Krautheimer** who hosted a bowling fundraiser called "Strikes for SMS" on March 19, 2017, in honor of his sister Emma. Matthew is becoming a Bar Mitzvah, and as part of his preparations, he is required to do a mitzvah project, or social action. His sister was recently diagnosed with SMS, so he knew right away that he wanted to help improve the lives of children with SMS. His event raised over \$6,000! Congratulations Matthew!



### Yard Sale in Florida

Thank you to the **Smallwood family**, who organized a yard sale on March 4, 2017, in Saint Cloud, Florida and raised \$2,590!

Shannon Smallwood is pictured with her daughter, Charlotte, who is 4 years old with SMS.



## Upcoming Events

### Laugh It Up! Pittsburgh *A Comedy Night for SMS - April 8*

The **Frist Family** will be hosting their second annual comedy night at the Pittsburgh Improv. It will be a night of fun and laughter in celebration of their daughter, Ellie. There will be stand-up comedy with premier comedians, as well as amazing auction items and raffle prizes.

For more details, as well as registration and donation information, go to [Laugh it Up! Pittsburgh](#).



### LIGHTS, CAMERA, RESEARCH!

#### *CARS 3 Fundraiser - June 17*

The **Iannuzzi Family** will be hosting a movie fundraiser for the whole family on Saturday, June 17. Pixar's newest movie, *Cars 3*, will be featured, along with a video game contest, a silent auction, and refreshments.

If you are an SMS family attending the event, you are welcome to join the Iannuzzis at their home afterwards for a backyard picnic to meet other SMS families and members of the Scientific Advisory Panel. Please contact [Jennifer Iannuzzi](#) for more details.

For event registration and donation information, go to [LIGHTS, CAMERA, RESEARCH!](#)

2017  
**LIGHTS \* CAMERA RESEARCH!**  
SMSRF fun family morning

FOR SMITH-MAGENIS SYNDROME  
at the PROSPECTOR THEATER

For sponsorship & tickets please visit:  
[www.lightscameraresearch.kintera.org](http://www.lightscameraresearch.kintera.org)

SAVE THE DATE  
8:30A.M. - 11:30A.M.  
**SATURDAY JUNE 17TH CARS 3**  
VIDEO GAME TOURNAMENT  
FACE PAINTING & BALLOON BAKING  
RAFFLES AND PRIZES TOO!  
Admission: \$30 per person

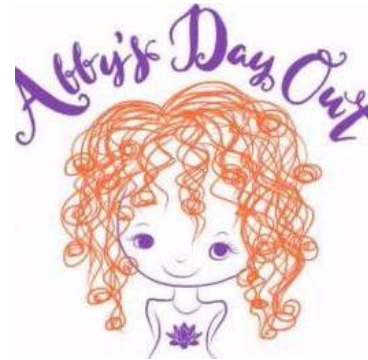
SMS RESEARCH FOUNDATION

## 2nd Annual Abby's Day Out Walk

Lloyd Harbor, NY - June

The **Gerberg Family** will be hosting their second annual 5K walk fundraiser in June 2017, in honor of their daughter, Abby. More details and website information will be coming soon!

Please [contact us](#) if you are interested in hosting an event! We will help you every step of the way.

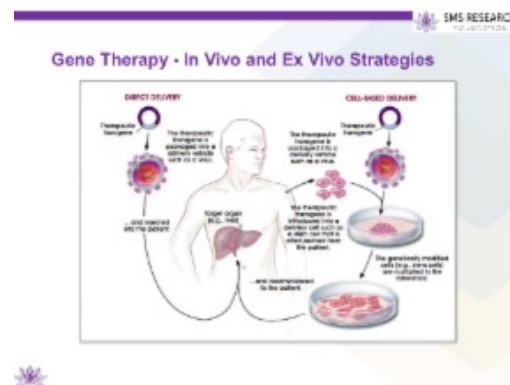


## Watch Our Past Webinar

Did you miss the webinar explaining our new gene therapy project? Or do you want to watch it again to gain a better understanding?

Our Scientific Advisory Panel members, Chris Iannuzzi and Gianluca Pirozzi, give a detailed presentation of the project.

[Watch the webinar here.](#)



*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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