

What is SMA?

Spinal Muscular Atrophy (SMA) is the number 1 genetic killer of children under 2 years of age. It can strike a person anytime throughout their life - infant, toddler, teen or adult.

It is a group of inherited and sometimes fatal diseases that destroy the nerve cells (motor neurons) controlling voluntary movement, such as, crawling, walking, head and neck control, as well as swallowing and breathing. It affects the anterior horn cells located in the spine.

Facts About SMA

- 1 in 35 people are carriers of the SMA gene. (7 million unknowingly carry this gene)
- 1 in every 6,000 live births is affected.
- Over 25,000 people in the USA have SMA
- SMA does not discriminate when it comes to sex, age, race or ethnic background.
- Both parents must carry the SMA gene.
- There is a 1 in 4 chance of having a child with SMA.
- There is a 50% chance of having a child who carries the gene.

There are 3 types of SMA:

- TYPE 1 (WERDNIG-HOFFMAN): Infantile; Most Fatal
- TYPE 2: Strikes between 9 - 18 months
- TYPE 3: Can strike at any time between 18 months to teen years to adult years

The OSU SMA LAB Dr. Arthur Burghes

The main focus of the laboratory is the molecular understanding of genetic neuro-muscular disorders. In particular, the laboratory focuses on unraveling the molecular biology of Spinal Muscular Atrophy (SMA). SMA is an autosomal recessive disorder that is characterized by destruction of motor neurons in the anterior horn of the spinal cord. The disorder can be classified according to clinical severity into three types. SMA is caused by loss or mutation of the SMN1 gene but not the virtually identical SMN2 gene. The two genes essentially differ by a single nucleotide that affects the incorporation of exon 7 into the SMN message. This results in the SMN2 gene producing insufficient SMN protein for motor neurons. SMN functions in the biogenesis of SnRNPs which is essential for all cells. Currently it is not clear why high SMN levels are so critically important for motor neurons. We have developed an animal model of SMA in the mouse and shown that high copy number of the SMN2 gene can rescue the SMA mouse. We are using this animal model of SMA to understand why motor neurons are affected and to develop treatments for SMA. A high throughput screen to identify compounds that can activate SMN has been started. It is hoped that these compounds can act as therapeutic reagents for SMA. The laboratory is also developing gene therapy methods for treatment of SMA in collaboration with...



The OSU SMA Lab has created mice with SMA and have cured them. Mice are being sent to other labs for drug, gene therapy and stem cell testing.

Dr Brian Kaspar—The Research Institute at Nationwide Children’s Hospital.

The Kaspar Lab’s main focus is utilizing the (AAV) adeno-associated viral gene therapy to all areas of the central nervous system (CNS). SMA mouse models were made at the OSU Lab and sent to the Kaspar lab. An AAV with the SMA gene was injected into the mice to restore motor neuron function. Success was achieved and mice were cured of SMA. Mice with no leg motor function began to walk and run. The next step before human trials will be clinical trials in monkeys to develop therapeutic practices for humans. This research is very promising for SMA patients who have been living with SMA for many years.



Miracle for Madison and Friends is excited about this research. Not only will this method help SMA but will also help in treatment discoveries for ALS, Spinal Cord injuries, Huntington disease and other neurodegenerative disease.

Researchers at Nationwide Research Institute are working to find a cure through gene therapy.

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 Dublin Ohio 43017

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HITTING THE LINKS

Tartan Fields Golf Club

October 13 2009

Registration: 10 am

Silent Auction: 10:30 am

Lunch: 11 am

Golf Start: High Noon

Format: Shotgun Start Best Ball

Golfers will enjoy playing on one of Central Ohio's premier courses. The success of the fundraiser greatly depends on friends and family within our community.

DO SOMETHING GREAT- and show your dedication and support for this worthy cause by either donating, becoming a sponsor and or playing golf.

Thank you for helping us find a cure for SMA

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- Company information in program
- Full page ad in program
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- Half page ad in program
- 1 Foursome

SILVER—BIRDIE \$750.00

- Name on MFMF website
- Event signage
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- Quarter page ad in program
- 1 Foursome

Bronze—PAR \$250.00

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- Name and or logo at hole of your choice

**Tartan Fields
Golf Club**

**Madison's
Angels
Hitting the Links
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**Miracle for
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Friends**



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