



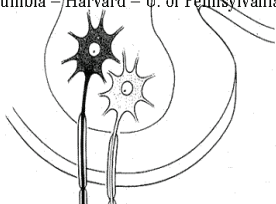
Spinal Muscular Atrophy

Inside this issue:

Meet the Extended SMA Team	2
SMA Clinic	2
Kid's Corner Answers	2
2009 FSMA Conference	2
BioMarker Study	3
PNCr Study	3
EIM Study	3
SMA Registry	3
Upcoming Events	4
Kate's Place	4
Just for Kids	4

PNCr - Network for SMA

North-Eastern Clinical Trials Consortium
Columbia - Harvard - U. of Pennsylvania



From the Desk of Dr. Darras:

Edward Everett Hale once said, "I am only one, but still I am one. I cannot do everything, but still I can do something; and because I cannot do everything I will not refuse to do the something that I can do."

In SMA research, there is no greater asset than the families and children affected by the disease. Without them, there would be no need, passion, or progress in finding a viable treatment or cure for SMA, which is why we cannot praise enough the families that have held strong through this journey with us. Each individual matters in our research; every piece of data collected is invaluable to moving forward.

Our approach is to understand SMA from all angles; the natural history, the best measures to assess the progression of symptoms and discovering a measureable biomarker. The sum of all these parts will help us in evaluating any drug trials that may present themselves for SMA research. Progress is not achieved by leaping bounds; rather it is secured through small steps that we must take together.

Boston SMA Day 2009

Please join us for a day of information, networking and fun at this year's Boston SMA Day. It will be a great time to catch up with the latest developments with SMA research and resources.

worked together to provide a conference that would not only provide an update on research but also be inclusive of topics dealing with every day issues, such as finances, school and of course health and well-being.

Date: Saturday March 14, 2009

Time: 10:00-4:30PM

Place: The Conference Center at Harvard Medical

Together, The Neurology Foundation at Children's Hospital Boston and the SMA Foundation have

We hope that you and your family will join us this year at Boston SMA Day 2009.

If you have any questions or would like to RSVP, please contact Jayson at jayson.caracciolo@childrens.harvard.edu or 617-355-2752.

Meet the SMA Team:

Neurology:

Dr. Basil T. Darras
*Director, NM Program,
Principal Investigator*

Dr. Peter Kang
*Attending Neurologist,
EMG Specialist*

Hailly Butler

Clinical Coordinator

Jayson Caracciolo
Clinical Coordinator

Lindsay Hansen
Administrative Assistant

Orthopedics:

Dr. Frederic Shapiro
Attending Physician

Physical Therapy:
Janet Quigley
Clinical Evaluator

Sue Riley
Clinical Evaluator

Meet the extended SMA Team:

While our Neuromuscular Clinic is multidisciplinary and includes many amazing people, we realized that SMA care extends into other disciplines. To alleviate confusion and enhance communication we have established an amazing team of people in various specialties that work closely with many families with SMA and are willing to work together with us to provide the best care as possible to patients. While these individuals are not directly part of our multi-disciplinary clinic, we hope that may change in the future to minimize the number of medical visits families have to attend.

We understand that some families affected by SMA have found health care providers, not on the list below, very helpful. We encourage families to stay with those providers as this list is not exhaustive. In addition to helping families directly, these individuals have offered amazing support to their colleagues working with patients with SMA.

For purposes of saving space, full titles and contact information is not available here. However, if you would like to get in touch with one of these specialists about making an appointment, please contact Haily Butler at Haily.Butler@childrens.harvard.edu and she can provide you with that information accordingly.

Pulmonary:

Dr. Virginia Kharasch

Lauren Perlman,
*Respiratory Care Continuing
Care Coordinator*

Dr. Robert Graham
Intensivist / Home Care

Nutrition:

Stacey Tarrant,
Clinical Nutrition Specialist

Gastroenterology:

Dr. Laurie Fishman

Anesthesiology:

Dr. Nevil Sethna
Dr. Chuck Berde

Surgery (non-orthopedic):

Dr. Craig Lillehei

Complex Care Service:

Dr. Laurie Glader

PACT Team:

Dr. Joanne Wolfe
Dr. Tamara Vesel
Dr. Christy Ullrich

Janet Duncan,
Nurse Practitioner

Marsha Joselow,
Social Worker

Kids' Corner Answers
(questions on page 4):

- 1) Racecar
- 2) Garbage truck
- 3) Footprints

SMA Clinic:

We have begun the new clinic dedicated solely to SMA. The clinic is held from 1:30-5:00pm the first Thursday of every month.

The clinic includes Dr. Darras, Dr. Kang, Dr. Shapiro, Janet Quigley, Lauren Perlman, Dr. Graham Stacey Tarrant and

Haily Butler. The purpose of this clinic, in addition to providing consistent clinical care, is to facilitate participation in research. We understand how busy everyone's lives are and hope that this clinic will ease the burden of research participation as study visits and clinical visits can be

combined on the same day. We also hope that this clinic will allow for families affected by SMA to meet and interact while waiting for their appointments. If you have any questions about SMA clinic or would like to schedule a visit, please contact Lindsay Hansen at 617-355-8235.

2009 FSMA Conference

Hopefully many of you were able to attend last year's national FSMA Family and Professionals Conference in Boston and enjoyed the experience.

This year the conference will be held in Cincinnati, Ohio on June 18-21. FSMA will be celebrating their 25th Anniversary at the conference, which is taking place at the Hyatt Regency

Hotel. Registration is now open and information for the conference can be found at:

<http://www.fsma.org/FSMACommunity/Conference/>

This conference is a wonderful opportunity to hear about the newest ideas in treatment, to learn about updates in the SMA

research, and to just meet other families that have been affected by SMA.

Please call (800) 886-1762 or email info@fsma.org if you have any questions about the conference

Please help us welcome **Jayson Caracciolo** to our SMA Research Team. Jayson joined us as a Clinical Research Coordinator in October 08' and will help with all of the research studies detailed here.

Jayson received a Masters in Public Health from the University of Florida with a concentration in epidemiology. He has previous experience working on registries and international clinical drug trials

We are excited to have Jayson on our team so please help us welcome him at your next visit!

If you are interested in more information about any of these studies please contact:

Hailly Butler at:
857-218-4677

or

Hailly.Butler@childrens.harvard.edu

Pilot Study of Biomarkers for SMA study:

This study is intended to learn more about SMA with the hopes of finding a measurable biomarker in the blood or urine.

We hope to have about 20-25 participants in this study at our site. The study involves doctors and families at eighteen sites across the nation with hopes that 120 families like yours will participate in

this research. The SMA team here at Children's is very excited to participate in the Biomarker study as it is the first major effort to find a marker to measure disease progression in SMA patients.

The study includes just one visit per participant, which includes SMA patients and controls. In addition to collecting blood and urine samples, we

will also collect physical therapy measures and medical history information from each patient.

Enrollment is scheduled to conclude March 1, 2009 and we welcome any interested participants. Please contact Hailly Butler with any questions or to schedule a visit.

PNCR study:

As many of you know, we teamed up with Columbia University NY, Children's Hospital of Philadelphia, and Rochester University a few years ago to form the Pediatric Neuromuscular Clinical Research (PNCR) Network. The goal of this group is to further research on the east coast for SMA.

We have been very successful at building our infrastructure

and gathering important information through our natural history study.

The data collected as part of this study has been highly requested by many groups working on SMA and we are thrilled to be able to provide information that moves research forward.

At this time, we are focusing on completing the study for those

participants already enrolled. We are not actively recruiting but are able to accommodate any interested participants.

In addition, we are still awaiting word about funding from the NIH for a medication study. We hope to be able to provide you good news through the next addition of the newsletter!

Electrical Impedance Myography study:

In the summer of 2007 we began a study using a technology called electrical impedance myography (EIM). EIM is a new method that we hope will prove effective at measuring nerve changes in children and adults with SMA in a reliable way.

This study involves evaluations at the hospital every six

months but can be combined with other clinical or research visits as it only takes an hour. At this time we are actively enrolling into this study. We are seeking 30 patients with SMA, all types, over age of 1 month and 30 individuals without SMA, also over age of 1 month. Thus far we have studied approximately 35 children with and without

SMA, and we are happy to report that all of the children found the testing painless and many found the testing fun.

We are hoping to fill all of the research spots for this study in the next 6 months so please call or email Hailly Butler if you are interested in more information!

SMA Registry:

There is a lot of research happening throughout the world on SMA. However, no single study is capable of learning from every individual in the world with SMA.

As many of you know there is a registry for SMA, housed at

Indiana University.

According to their website: "The purpose of the International Spinal Muscular Atrophy Patient Registry is to provide a resource by which individuals and families affected by SMA

and researchers interested in studying SMA can come into contact with one another."

For more information about the registry or to participate please visit their website at: smaregistry.iu.edu/

Upcoming Events:

FSMA New England Chapter - 9th Annual Spring
Walk & Roll.
Saturday May 16, 2009 at 10 am
Wompatuck State Park in Hingham, MA.

Walkers and rollers will enjoy a 1.5 loop on paved paths through scenic woods. There will be t-shirts, refreshments, an exciting raffle, and fun for the children. The requested donation to Families of SMA is \$15 per person ages 12 and up. Please contact Silvia Murphy for more information at brianandsilvia@verizon.net.

FSMA New England Chapter -
1st Annual Disc Golf Tournament
**Second Weekend of June in Medfield,
MA.**

All proceeds from the event will benefit the New England Chapter of Families of SMA. Come learn from the pros, enjoy a family day on the course, and celebrate the start of summer with this new sport that is sweeping the nation. For more information please contact Heidi Johnson at heidikjohnson@comcast.net.

For more information regarding Families of SMA, please contact Michael Barrett, president of the New England Chapter. He can be reached at:
newengland@fsma.org

Please keep visiting our website for the most up to date information about research and upcoming events. This newsletter is also posted on the website to make it easier to share with interested friends and family.

www.childrenshospital.org/sma



**Children's
Hospital
Boston**

Win a Free Vacation!!!

Kate's Place is located on Long Lake in Harrison, Maine and is named in honor of Kate Pecora who has Type III SMA. Applications from families, couples, and individuals affected by SMA are once again being accepted to enter a drawing for a free week at this wonderful lakeside vacation home with ramped entrance and first floor accommodations. Applications will be accepted from January 1st through March 16th and the drawing will be held on March 17th. For more information, contact Kristine Pecora at acureforkate@comcast.net or 978-664-8488 or visit: <http://mysite.verizon.net/>

Just For Kids

Hey kids! How smart are you? Can you figure out these fun brainteasers?

(Answers on page 2)

#1

What vehicle is spelled the same backwards as forwards?

#2

What has wheels and flies, but is not an aircraft?

#3

The more of them you take, the more you leave behind. What are they?

Riddles from:

<http://kids.niehs.nih.gov/braint.htm>

Word Fun...

Can you find these school words?

TEACHER
CLASSES
BOOKS
RECESS
PRINCIPAL
GRADES
PENCIL
HOMEWORK
MATH
READING

ECHECCEGMICCRSR

TSEBPPNACRKCNS

AELRCESLTC SHGD

ERARNASGHLLEESA

OHPCGBOOKSTCHAE

OEIBHRMSMEIEPGS

CLCRREADINGMOAG

KSNLWCRDCCDGTSE

ATIOAEISEBOMICH

OARRESEANSPHOIR

GKPTCSSTMDCHSED

NRERSNLETRCRGED

GAAKAEESISRERRPS

ALEOMGEGSERLRLI