



The Good Fight

FightSMA Annual Meeting
April 23-28, 2006

Melrose Hotel
Washington, D.C.

Welcome


Dear Friends,

Welcome to **The Good Fight**, the FightSMA 2006 Annual Meeting. Thank you for taking the time to join us. Together we are impacting pivotal research for SMA and raising the collective conscience for this cruel disease.

As we have planned for this year's meeting, we have increasingly believed that our time together will produce powerful synergy: great minds and a common vision. This is the perfect equation for success.

We hope that you will be inspired and equipped for the wonderful work you are doing and refreshed by the fellowship of this community.

All best,



Martha and Joe

The Mission

FightSMA has a clear, focused and vital mission:

“To strategically accelerate research toward treatment and a cure for spinal muscular atrophy, a deadlycrippler and the number-one inherited genetic cause of infant death.”

The words that capture the Mission are carefully chosen. Embedded in the Mission is a reminder of what the organization is all about – a deadly children's genetic disease, the leading inherited killer of babies. “Strategically accelerate” means that the group is not only about fundraising for research (though it does a lot of that). It means that in addition to supplying money for medical research, it takes actions that in themselves “strategically accelerate” the work. It is part of the FightSMA mission to bring researchers together to meet and discuss the most promising next steps to take in order to move with maximum speed toward a treatment or cure. An idea may (and does) come out of those meetings that provides new focus for work at several labs, or eliminates redundant efforts, or encourages scientific collaboration, or encourages a new proposal.

The mandate to “strategically accelerate research” also requires that a continuing, energetic effort be made to raise awareness about the disease. People need to hear about SMA. They need to know its terrible power, and they need to know about the sacrificial and heroic work that is being carried on by parents, families and friends. It will always be the task of FightSMA to educate the public about SMA and to bring a constantly higher profile to the disease, through publicity, special events, mailings, on the Web and in meetings with Congress.

Researcher Conference

Sunday, April 23, 2006

3:00 PM	Check-in	Melrose Hotel
7:00 - 8:00 PM	Cocktail Reception	William Penn Reception Area
8:00 - 9:30 PM	Dinner	William Penn

Monday, April 24, 2006

8:00 AM	Breakfast	Potomac I & II
8:00 - 8:15 AM	Welcome	Martha Slay

Basic and Translational Investigation

Potomac I & II

8:15 - 8:45 AM	Intravital murine studies	Anneliese Schaefer
8:45 - 9:15 AM	SMN Assay	Elliott Androphy
9:15 - 9:45 AM	Aminoglycoside approaches	Christian Lorson
9:45 - 10:15 AM	HDACi	Charlotte Sumner

10:15 - 10:35 AM Break

10:35 - 11:05 AM	Mouse models	Arthur Burghes
11:05 - 11:35 AM	Motor Neuron cellular models	Doug Kerr
11:35 - 12:05 PM	Gene therapy for motor neuron disorders	Nick Boulis
12:05 - 12:35 PM	NPTUNE	Robert Leshner

12:35 - 1:10 PM Lunch Potomac I & II

Clinical Investigation

Potomac I & II

1:10 - 1:40 PM	Electrophysiologic assessment of SMA and clinical trials	Katherine Swoboda
1:40 - 2:10 PM	AmSMART update	Karima Ravenell
2:10 - 2:40 PM	Clinical update and outline of challenges	Petra Kaufmann
2:40 - 3:10 PM	The Biochemical SMA phenotype	Ingrid Tein

Researcher Conference

Monday, April 24, 2006 continued

3:10 - 3:30 PM	Break	
3:30 - 4:00 PM	NINDS and SMA Project; the state of play	Jill Heemskerk
4:00 - 4:30 PM	SMA, a grand unifying theory	Alex MacKenzie
4:30 - 5:00 PM	Open Discussion	
7:00 - 9:00 PM	Dinner – SMA Science with visiting from ALS and Stem Cell representatives	William Penn

Tuesday, April 25, 2006

8:00 AM	Breakfast	Potomac I & II
8:00 - 8:30 AM	Introduction	Drs. Alex MacKenzie and Christian Lorson

SMA, ALS, and Stem Cell Joint Strategies Meeting

Potomac I & II

8:30 - 9:00 AM	Axonal biology in the pathogenesis of motor neuron diseases	Jonathan Glass
9:00 - 9:30 AM	Transport defects and damaging secretion in motor neuron disease	Jean-Pierre Julien
9:30 - 10:00 AM		Jeffrey Rothstein
10:00 - 10:30 AM	Discussion	
10:30 - 10:45 AM	Break	

10:45 - 11:15 AM	Stem cells in motor neuron diseases	Doug Kerr
11:15 - 11:45 AM	Generation of high purity motor neuron cultures from human embryonic stem cells	Hans Keirstead
11:45 - 12:15 PM	Combining growth factor and stem cell therapy for ALS	Clive Svendsen

12:30 - 1:30 PM Lunch and Discussion Potomac I & II

7:00 - 9:00 PM Dinner sponsored by Ansar Landmark Restaurant

Researcher Roster

Elliott Androphy, M.D.	<i>University of Massachusetts Medical School</i>
Nicholas Boulis, M.D.	<i>The Cleveland Clinic</i>
Arthur Burghes, Ph.D.	<i>Ohio State University</i>
Kenneth Fischbeck, M.D.	<i>National Institute of Neurological Disorders and Stroke</i>
Jonathan Glass, M.D.	<i>Emory University School of Medicine</i>
Jill Heemskerk, Ph.D.	<i>National Institute of Neurological Disorders and Stroke</i>
Jean-Pierre Julien, Ph.D.	<i>Laval University, Quebec</i>
Petra Kaufmann, M.D.	<i>Columbia University</i>
Hans Keirstead, Ph.D.	<i>University of California-Irvine</i>
Doug Kerr, M.D., Ph.D.	<i>Johns Hopkins Hospital</i>
Robert Leshner, M.D.	<i>Children's National Medical Center, Washington D.C.</i>
Christian Lorson, Ph.D.	<i>University of Missouri-Columbia</i>
Alex MacKenzie, M.D., Ph.D.	<i>Children's Hospital of Eastern Ontario</i>
Karima Ravenell, M.S.	<i>University of Texas at Southwestern Medical Center</i>
Jeffrey Rothstein, M.D., Ph.D.	<i>Johns Hopkins Hospital</i>
Anneliese Schaefer, Ph.D.	<i>Washington University School of Medicine</i>
David Shine, Ph.D.	<i>Baylor College of Medicine</i>
Charlotte Sumner, M.D.	<i>National Institute of Neurological Disorders and Stroke</i>
Clive Svenden, Ph.D.	<i>University of Wisconsin-Madison</i>
Kathy Swoboda, M.D.	<i>University of Utah School of Medicine</i>
Ingrid Tein, M.D.	<i>The Hospital for Sick Children, Toronto</i>

Thriving with SMA Panel Members

John Bach, M.D.	<i>University of Medicine and Dentistry of New Jersey</i>
Jennifer Barnes, M.A.	<i>Children's Healthcare of Atlanta</i>
Jennifer Hewitt	<i>Children's Healthcare of Atlanta</i>
Sherri Katz, M.D.	<i>Children's Hospital of Eastern Ontario</i>
Robert Leshner, M.D.	<i>Children's National Medical Center</i>
Chris Makris, M.D.	<i>Children's Hospital, Birmingham, Alabama</i>
Eleanore Rothenberg, Ph.D.	<i>The Sibling Center, New York</i>
Mary Schroth, M.D.	<i>University of Wisconsin</i>
Kathy Swoboda, M.D.	<i>University of Utah</i>
Brian Weaver, MS	<i>University of Medicine and Dentistry of New Jersey</i>

“Thriving with SMA” Panelists

Tuesday, April 25, 2006

4:00 - 5:30 PM	Panelists' meeting with Dr. Alex MacKenzie, and Dr. Christian Lorson	Potomac I & II
5:30 - 6:00 PM	Ansar Presentation	Ben Aysin
7:00 - 9:00 PM	Dinner sponsored by Ansar	Landmark Restaurant

Wednesday, April 26, 2006

8:00 AM	Breakfast	Potomac I & II
8:00 - 10:00 AM	Science Presentation	Drs. Alex MacKenzie, Christian Lorson, Doug Kerr, and Robert Leshner
10:00 - 10:15 AM	Break	
10:15 - 11:15 AM	“Thriving with SMA” Panel Presentation	Potomac I & II
	Moderator	Robert Leshner
	Speech Therapy	Jennifer Barnes
	Psychotherapy	Eleanor Rothenberg
	Pulmonology	Chris Makris
	Pulmonology	Mary Schroth
11:15 - 12:00 PM	Question and Answer Session	Potomac I & II
12:00 - 12:30 PM	Lunch	Potomac I & II
12:30 - 1:30 PM	“Thriving with SMA” Panel Presentation	Potomac I & II
	Moderator	Robert Leshner
	Occupational Therapy	Jennifer Hewitt
	Physiatry and Rehabilitation	John Bach
	Respiratory	Brian Weaver
	Respiratory	Sherri Katz
	Nutrition	Kathy Swoboda

“Thriving with SMA” Panelists

Wednesday, April 26, 2006 continued

1:30 - 2:15 PM	Question and Answer Session	Potomac I & II
2:15 - 2:30 PM	Break	
2:30 - 5:00 PM	Consultations with Panelists	Potomac I & II
6:30 - 7:30 PM	Cocktail Reception	William Penn
7:30 - 9:00 PM	Dinner & Program	Potomac I & II

Special Thanks



To The Ansar Group, Inc. for sponsoring Tuesday night's Researcher and Panelist Dinner at the Landmark Restaurant and to Ben Aysin for demonstrating Ansar's ANX 3.0 Autonomic Nervous System Monitoring Software. Ansar is providing the ANX 3.0 lease-free for the FightSMA funded research of Dr. John Bach at the University of Medicine and Dentistry of New Jersey.

Hospitality Suite

The Hospitality Suite is located in Room 823 of the Melrose Hotel and is available for Annual Meeting attendees to socialize, to snack, or to relax. The Suite is also available for guests to store their baggage between the time they check out (12 noon) and the time they depart. Please contact Heather Lennon regarding additional Hospitality Suite questions.

Contact Information

Heather Lennon, FightSMA Staff
Cell Phone 804.241.3028

The Melrose Hotel, Washington, D.C.
2430 Pennsylvania Avenue, NW
Washington, DC 20037
Phone 202.955.6400
Fax 202.955.5765
www.melrosehoteldc.com

Families and Guests

Rod Adams	Richmond, Virginia
Melissa and Jeff Andrade	Wayzata, Minnesota
Cheryle and Icesis Augustino	Powhatan, Virginia
Linda Baker	Richmond, Virginia
Elaine Bolton	Atlanta, Georgia
Holly and John Bolton	Huntersville, North Carolina
Mary Bodzo	Bellevue, Florida
Priscilla Burbank	Richmond, Virginia
Aubrey Campbell	Richmond, Virginia
Joe Cueter	Troy, Michigan
Sally Curran	Richmond, Virginia
Laurie and Duncan Elliott	Tampa, Florida
Carol Feaver	Whitby, Ontario
Elise Finkner	Chatbam, Virginia
Maureen and Bill Higgins	Westfield, New Jersey
Dan Jorgensen	Madison, Connecticut
Patti Kemp	Hueytown, Alabama
Tracy Lacey	Whitby, Ontario
Deborah Landon	Virginia Beach, Virginia
Heather Lennon	Richmond, Virginia
Tonya, Ben, and William Mason	McDonough, Georgia
Rob Mazzenga	Dumfries, Virginia
Anne Meguiar	Richmond Hill, Georgia
Mary Peabody	Virginia Beach, Virginia
Carl Reed	Dublin, Ohio
Stacy and Bill Saville	Virginia Beach, Virginia
Terri Sawyer	Virginia Beach, Virginia
Nadine Schaper	Beaverton, Oregon
Kathie Shetler	Winchester, Virginia
Barbara Shuman	Richmond Hill, Georgia
Martha and Joe Slay	Richmond, Virginia
Carice and Wayne Smith	Mechanicsville, Virginia
Jane Smith	Austin, Texas
Trevor and Tom Smith	St. Petersburg, Florida
Amy and Scott Stein	Birmingham, Alabama
Bubba Sumerlin	Richmond Hill, Georgia
Jennifer Sutton	Middleton, New York
Susan Tedesco	Richmond, Virginia
Scott Therriault	Southport, Connecticut
Kathy Tompkins	New York, New York
Sarah Williams	Richmond, Virginia
Susan Youngs	Richmond, Virginia

Families and Guests Conference

Tuesday, April 25, 2006

3:00 PM Check-in **Melrose Hotel**
7:00 - 9:00 PM Drop-in Dinner **Hospitality Suite**

Wednesday, April 26, 2006

8:00 AM Breakfast **Potomac I & II**
8:00 - 10:00 AM Science Presentation Drs. Alex MacKenzie, Christian Lorson,
Doug Kerr, and Robert Leshner

10:00 - 10:15 AM Break

10:15 - 11:15 AM “Thriving with SMA” Panel Presentation **Potomac I & II**
Moderator Robert Leshner
Speech Therapy Jennifer Barnes
Psychotherapy Eleanor Rothenberg
Pulmonology Chris Makris
Pulmonology Mary Schroth

11:15 - 12:00 PM Question and Answer Session **Potomac I & II**

12:00 - 12:30 PM Lunch **Potomac I & II**

12:30 - 1:30 PM “Thriving with SMA” Panel Presentation **Potomac I & II**
Moderator Robert Leshner
Occupational Therapy Jennifer Hewitt
Physiatry and Rehabilitation John Bach
Respiratory Brian Weaver
Respiratory Sherri Katz
Nutrition Kathy Swoboda

1:30 - 2:15 PM Question and Answer Session **Potomac I & II**

2:15 - 2:30 PM Break

Families and Guests Conference

Wednesday, April 26, 2006 continued

2:30 - 5:00 PM Consultations with Panelists **Potomac I & II**

5:00 - 6:00 PM SMA Coalition Meeting **Hospitality Suite**

6:30 - 7:30 PM Cocktail Reception **William Penn**

7:30 - 9:00 PM Dinner **Potomac I & II**

Thursday, April 27, 2006

8:00 AM Breakfast **Potomac I & II**

8:00 - 9:00 AM Briefing by Winning Strategies
Donna Mullins and
Laura Breitenicher

9:00 - 11:00 AM FightSMA Board of Director’s Meeting **Potomac I & II**

10:00 - 5:00 PM Congressional Visits according to
Winning Strategies’ Schedule **Capitol Hill**

Friday, April 28, 2006

8:00 - 10:00 AM Breakfast **Hospitality Suite**

12:00 PM Check-out for all rooms

Capitol Hill Visits

Winning Strategies suggests that those visiting Capitol Hill leave an hour before scheduled appointments to allow time to travel and to pass security. Transportation to Capitol Hill has not been prearranged. Please hail a taxicab and submit a receipt to Heather Lennon for reimbursement.

FightSMA is also pleased to reimburse the cost of lunch while visiting the Hill. Eateries are located in many of the Capitol office buildings, including the Rayburn, Longworth, Dirksen, Russell, and Cannon Buildings. Museums and institutions around Capitol Hill also offer dining options.

fightsma

1807 Libbie Avenue, Suite 104

Richmond, Virginia 23226

P 804.515.0080

F 804.515.0081

www.FightSMA.org