

**Fight SMA: Researchers' Conference**  
**April 20-22, 2008**  
**L'Enfant Plaza, Washington, DC**

**Schedule of Events**

	<b>Sunday, April 20, 2008</b>	
<b>3:00 PM</b>	Check-in for Researchers Opens	<b>L'Enfant Plaza Hotel</b>
<b>7:30 PM</b>	Cocktail Reception	<b>Renoir Room</b>
	<b>Monday, April 21, 2008</b>	
<b>8:00 AM</b>	Breakfast and Introduction by Dr. Chris Lorson	<b>Renoir Room</b>
<b>8:30 AM</b>	SMN Function	
	<i>A genetic approach to the critical molecular pathway in spinal muscular atrophy using zebrafish and mice</i>	<i>Arthur Burghes</i>
	<i>SMN function in MN</i>	<i>Gary Bassell</i>
	<i>The role of SMN in the MN</i>	<i>Michael Sendtner</i>
<b>10:15 AM</b>	Break	
<b>10:30 AM</b>	SMN Function continued	
	<i>Consequences of SMN deficiency on snRNP biogenesis and function</i>	<i>Livio Pellizzoni</i>
	<i>SMN in muscle</i>	<i>Rashmi Kothary</i>
<b>12:30 PM</b>	Lunch	<b>Renoir Room</b>
<b>1:30 PM</b>	SMN translational research	
	<i>SMN stability and proteasome inhibition</i>	<i>Barrington Burnett</i>
	<i>RNA mediated modulation of SMN2 pre-mRNA splicing</i>	<i>Chris Lorson</i>
	<i>SMN inducing signaling pathways</i>	<i>Alex MacKenzie</i>
<b>3:15 PM</b>	Break	
<b>3:30 PM</b>	SMN translational research continued	
	<i>Gene therapy applications in SMA</i>	<i>Nick Boulis</i>
	<i>Moving AAV from the bench to bedside for neuromuscular disorders</i>	<i>Brian Kaspar</i>
	<i>Development of Therapeutics for SMA</i>	<i>Matthew Butchbach</i>
<b>5:30 PM</b>	Break	
<b>7:00 PM</b>	Dinner	<b>Degas Room</b>
	<b>Tuesday, April 22, 2008</b>	
<b>8:00 AM</b>	Breakfast	<b>Renoir Room</b>
<b>8:30 AM</b>	SMN translational research continued	
	<i>Identification of new SMN inducing compounds</i>	<i>Elliot Androphy</i>
	<i>SMN anti-sense</i>	<i>Adrian Krainer</i>
	<i>HDAC inhibitors in mice</i>	<i>Charlotte Sumner</i>
<b>10:15 AM</b>	Break	
<b>10:30 AM</b>	SMN translational research continued	
	<i>Discovery Initiatives and Research Priorities</i>	<i>Meg Winberg</i>
<b>11:45 AM</b>	Clinical Trials Update	<i>Kathy Swoboda</i>
<b>12:30 PM</b>	Lunch	<b>Renoir Room</b>
	Conclusion	<i>Chris Lorson</i>

### Attending Researchers

Elliot Androphy, M.D.	University of Massachusetts Medical School
Gary Bassell, Ph.D.	Emory University School of Medicine
Nicholas Boulis, M.D.	Emory University School of Medicine
Arthur Burghes, Ph.D.	Ohio State University
Barrington Burnett, Ph.D.	National Institutes of Health
Matthew Butchbach, Ph.D.	Ohio State University
Kenneth Fischbeck, M.D.	National Institute of Neurological Disorders and Stroke
Katrina Gwinn-Hardy, M.D.	National Institutes of Health
Brian K. Kaspar, Ph.D.	The Research Institute at Nationwide Children's Hospital
Rashmi Kothary, Ph. D.	Ottawa Health Research Institute
Adrian Krainer, Ph.D.	Cold Spring Harbor Laboratory
Robert Leshner, M.D.	Children's National Medical Center
Chris Lorson, Ph.D.	University of Missouri-Columbia
Alex MacKenzie, M.D., Ph.D.	Children's Hospital of Eastern Ontario Research Institute
Marco Passini, Ph.D.	Genzyme Corporation
Livio Pellizzoni, Ph.D.	Columbia University Center for Motor Neuron Biology and Disease
Michael Sendtner, M.D.	University of Wurzburg
Charlotte Sumner, M.D.	Johns Hopkins University School of Medicine
Kathy Swoboda, M.D.	University of Utah
Meg Winberg, Ph.D.	Spinal Muscular Atrophy Foundation

## Families and Friends' Conference

### General Information

**When:** April 22-25, 2008  
**Where:** L'Enfant Plaza Hotel  
480 L'Enfant Plaza, SW  
Washington, D.C.

### Schedule of Events

	<b>Tuesday, April 22, 2008</b>	
<b>3:00 PM</b>	Check-in for Families and Friends Opens	<b>L'Enfant Plaza Hotel</b>
<b>7:00 PM</b>	Reception and Check-In	<b>Monet 3 Room</b>
	<b>Wednesday, April 23, 2008</b>	
<b>8:30 AM</b>	Check-In	<b>Outside Monet 1 Room</b>
<b>9:00 AM</b>	Breakfast	<b>Monet 1 and 2 Rooms</b>
<b>9:15 AM</b>	Science Update	<i>Dr. Chris Lorson</i> <i>Dr. Alex MacKenzie</i>
<b>9:45 AM</b>	Break	
<b>10:00 AM</b>	Thriving with SMA Panel	
<b>12:00 PM</b>	Lunch	<b>Monet 1 and 2 Rooms</b>
<b>1:00 PM</b>	Nutrition Panel	
<b>3:00 PM</b>	Break	
<b>3:30 PM</b>	One-on-one Meetings	<b>Monet 1 and 2 Rooms</b>
<b>5:30 PM</b>	Break	
<b>7:00 PM</b>	Dinner	<b>Monet 1 and 2 Rooms</b>
	<b>Thursday, April 24, 2008</b>	
<b>7:30 AM</b>	Breakfast and Capitol Hill Briefing	<b>Monet 1 and 2 Rooms</b>
<b>9:00 AM</b>	Visits to Capitol Hill Representatives	