

COMPASS

A Publication Dedicated to Research Updates **FALL 2015**

19th Annual SMA Researcher Meeting

The Annual SMA Conference has two parts: our family conference, for those affected by SMA, and the largest SMA Researcher Meeting worldwide. This year's research meeting set a record with over 300 attendees.

The SMA Researcher Meeting promotes sharing of unpublished scientific data to accelerate the pace of research. It also helps build research collaborations, integrate new researchers and drug companies into the community, and educate future SMA researchers.

But the most unique aspect of the conference is the interaction between researchers and families, through sharing research updates or fun activities like the researcher relay race.

Here are a few of the research highlights from the Annual SMA Conference.

New Clinical Trial Materials Debut

As more SMA drugs progress through clinical trials, Cure SMA has been working to address the increasing need for accurate information.

"I attend a number of medical meetings. Never have I been so moved by the quality of the interactions together with researchers [and] families...Excellent scientific content in close proximity to the purpose driven research that aligns our interests."

The centerpiece of these education efforts is our new booklet, *Learning About Clinical Trials*. Developed with expert reviewers, this booklet explains the clinical trial process. Released to families at the conference, it is now available on our website, along with the updated SMA drug pipeline and a list of trials currently recruiting.

Six Programs Participate in Drug Discovery Panel

During a special panel discussion, representatives from six drug programs in clinical trials updated families. This session also included informational presentations from Dr. Jill Jarecki, our research director, and Dr. John Kissel.

Four of the six programs—gene therapy, ISIS-SMNRx, RG7800, and LMI070—treat the underlying genetics of SMA. The other programs, Olesoxime and CK-2127107, work to protect the muscles and nerves.

Pharmaceutical Companies Release New Data From Clinical Trials

The SMA Researcher Meeting also provides companies with a worldwide platform to announce new results. Several of our pharmaceutical partners released new clinical trial data at our SMA Researcher Meeting. These include:

- Isis Pharmaceuticals provided an update on children with SMA enrolled in the open-label extension (OLE) study for the Phase 2 study of ISIS-SMNRx.
- Cytokinetics released data from their Phase 1 study of CK-2127107, a skeletal muscle activator. Cytokinetics, in partnership with Astellas, has announced a Phase 2 study will begin later in 2015.



"I consistently heard the same thing from families: '[T]his conference changed (or saved) our lives.' The experience of meeting with families was incredible and I left the conference feeling very inspired and moved by the many stories I heard."

- BioBlast announced positive preclinical proof-of-concept results of its possible SMA drug.
- Nationwide Children's Hospital updated their Phase 1 trial of gene therapy.

Updates from the 2015 SMA Researcher Meeting

Each year after the conference, we publish session summaries in the news section of our website that are written by our Scientific Advisory Board. These updates explore the major research questions in SMA.

Following are introductions to these sessions. Complete summaries of all can be found on our Cure SMA website, in the news section.

Emerging Trends in Motor Neuron Pathobiology

We recognize that the research in SMA and in related motor neuron disorders share similar challenges: to know WHAT cells to protect, WHEN we need to protect them, and HOW we can identify new potential therapies. This session brought in scientists who work in other motor neuron disorders to explore connections to SMA.

Newborn Screening

Data generated in mouse models of SMA suggest that drug delivery is most effective when given early. This session explored how the SMA research community is pursuing newborn screening for SMA.

Clinical Research

As SMA drugs move closer to FDA approval, an important goal is to represent the patient perspective on topics like the impact of SMA, what constitutes meaningful change, and risk/benefit analysis. The clinical research session explored this, as well as biomarkers and clinical trial outcome measures.



Drug Discovery

The 2015 SMA Researcher Meeting closed with a much anticipated session on SMA drug development. Eight talks were given, four focused on drug programs at early stages in the SMA pipeline and four focused on drugs being tested in clinical trials.

SMA Pathology

Electrical current must flow from sensory neurons to the motor neurons in the spinal cord and then to the muscle itself to promote muscle contraction. The session discussed the formation of these circuits, as

“The meeting was unlike anything I’ve attended in my 25 year career working in the pharmaceutical field. I’m not sure I can express what it meant to me. I left the meeting feeling inspired...and touched by the stories of the families.”

well as how to measure their electrical flow and function.

MN Partners and Therapeutic Targets, Parts 1 and 2

Individuals with SMA do not correctly produce survival motor neuron (SMN) protein at high enough levels. There may be ways to treat SMA other than increasing SMN levels. The drugs targeting alternative pathways could be used in combination with SMN-enhancing therapies.

To read more on these topics, go to www.cureSMA.org/news. Select **Conference** from the news topics box to see all conference updates, or search for **2015 SMA Researcher Meeting Summary** to see only the session summaries.

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