

**Neurobiology of Disease in Children Symposium: Spinal Muscular Atrophy
October 18, 2006
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The Neurobiology of Disease in Children conference series was established in 2001 with the original aims of bringing together clinicians, scientists, caregivers and NIH program officers to (1) review scientific advances and determine their relevance to current and future clinical practice in child neurology, (2) to coordinate efforts across clinical and basic science disciplines, lay organizations, and the NIH to review current research initiatives and define future directions, and (3) to disseminate the proceedings of the symposia to ensure that clinicians and basic scientists are informed about scientific advances, current research initiatives and future directions.

Spinal Muscular Atrophy was the disorder selected for the 2006 symposium, and the conference format included formal presentations, question and answer sessions, panel discussions and open discussions to directly address the conference objectives. The first session (Clinical Neurology of SMA) was moderated by Dr. Susan Iannaccone, and described the historical descriptions, classification, diagnosis and clinical management of SMA. The second session (Molecular Mechanisms in SMA), moderated by Dr. Christine DiDonato, described the molecular mechanisms of the disorder, the importance of the SMN gene on the cellular level and in neurodevelopment, and the applications of animal models in SMA research. The third session (Translational Research), moderated by Dr. Kathryn Swoboda, covered the search for active compounds, animal models in preclinical testing, clinical trials, and the current standard of care for SMA. These sessions were followed by an executive summary and a panel discussion of future directions for SMA research moderated by Dr. Jill Heemskerk of the NINDS.

The proceedings of this year's Spinal Muscular Atrophy symposium will be published, along with peer-reviewed papers by symposium participants in an August 2007 special issue of the *Journal of Child Neurology* (published by BC Decker), entirely dedicated to Spinal Muscular Atrophy. This is an ideal mechanism to thematically bundle peer-reviewed papers for publication in the journal with the highest impact factor in the field of child neurology.

In addition to meeting the aims outlined above, the 2006 Spinal Muscular Atrophy symposium was impressive in terms of attendance. Attendance was capped by space limitations and exceeded expectations with 270 total attendees. With support from FightSMA, a patient organization, we were able to invite 19 young investigators and medical students interested in the neurosciences or in SMA research to attend the symposium.

The proposed topic for the 2007 Neurobiology of Disease in Children conference is Tumors of the Central Nervous System, and the symposium directors are working on developing a similarly diverse and comprehensive agenda. We are grateful to the Office of Rare Diseases for supporting the 2006 Spinal Muscular Atrophy Symposium, and contributing to the series' success.