

SPINAL MUSCULAR ATROPHY FOUNDATION

Spinal Muscular Atrophy Foundation Awards \$1.125 Million In Grants To Support SMA Research Efforts

Funding to Promote and Advance Clinical Research for the Cause, Treatment and Potential Cure for Spinal Muscular Atrophy

New York, NY – April 19, 2004 – The Spinal Muscular Atrophy Foundation today announced that it has awarded new research grants totaling \$1.125 million to four outstanding Young Investigators specializing in research on Spinal Muscular Atrophy (SMA), the leading genetic killer of infants and toddlers. These grants support the implementation and advancement of clinical research into the cause, treatment and potential cure for SMA.

Spinal Muscular Atrophy is a genetic, motor neuron disease characterized by the wasting away of skeletal muscles. More than 25,000 Americans have the disease. Up to 1,000 new babies – an estimated one in every 6,000 live births – are afflicted with SMA each year. Over 50% of patients with SMA die before the age of two. Currently there are no treatments.

“This initial round of research grant funding represents a significant first-step in our Foundation’s active involvement in the search for a cure for SMA,” said Loren Eng, Founder and President of the SMA Foundation. “These grants are critically important to our efforts and will provide the funding to enhance innovative research initiatives. The results from these projects will advance our goals in learning more about the cause of this debilitating and fatal disease and ultimately bring us closer to finding a treatment or cure.”

The Young Investigator Award is an incentive and mentoring program that allows a new generation of scientists to explore an early professional interest in studying SMA, with a mentor who has an established track record as an investigator.

The awards will be presented at a special ceremony held during the 56th Annual Meeting of The American Academy of Neurology in San Francisco, California on April 28, 2004. The award provides each young investigator \$85,000, plus allowances for tuition, research expenses and an annual mentor stipend, for three years. The Young Investigator Awards are co-sponsored by the American Academy of Neurology Foundation.

The SMA Foundation is pleased to award grants to the following Young Investigators:

Christine DiDonato, PhD
Children's Memorial Institute for Education and Research (CMIER)
Chicago, IL
Project Title: *"Pharmacological Treatment Strategies Using Neuroprotective Agents in Cellular and Animal Models of SMA"*
Mentor: Leon Epstein, MD

Umrao R. Monani, PhD
Neurological Institute of New York at Columbia University
New York, NY
Project Title: *"The Role of SMN in MotorNeuron Development"*
Mentor: Tom Jessell, PhD

Charlotte J. Sumner, MD
National Institute of Neurological Disorders and Stroke
Bethesda, MD
Project Title: *"Epigenetic Regulation of the SMN Gene: Identifying Molecular Targets for SMA Therapeutics"*
Mentor: Kenneth Fischbeck, MD

Kathryn J. Swoboda, MD
University of Utah School of Medicine
Salt Lake City, UT
Project Title: *"Refinement of Outcome Measures for Clinical Trials in Infants and Children with Spinal Muscular Atrophy"*
Mentor: Mark Bromberg, MD, PhD