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**COLUMBIA RECEIVES UP TO \$15 MILLION FROM
SPINAL MUSCULAR ATROPHY FOUNDATION**

***Supports Center for Motor Neuron Biology & Disease,
Targeting Deadliest Genetic Disease of Infants & Toddlers***

NEW YORK, NY, February 16, 2006 – Columbia University has been awarded up to \$15 million from the Spinal Muscular Atrophy (SMA) Foundation. The grant will fund activities by Columbia's newly established Center for Motor Neuron Biology and Disease to accelerate the discovery of medical advances for SMA, a devastating disease that is the number one genetic killer of infants and toddlers.

"We're excited to work with Columbia's Center for Motor Neuron Biology and Disease--in addition to being one of the best centers worldwide for the study of motor neuron biology and SMA, it has a staff of internationally renowned doctors eager to test the latest therapeutic approaches that emerge from laboratory research," said Loren Eng, co-founder and president of the SMA Foundation. "We are confident that the center's leadership has the drive and expertise to fulfill the scientific promise of SMA research and create new, effective treatments."

The Center for Motor Neuron Biology and Disease, also referred to as the Motor Neuron Center (www.ColumbiaMNC.org), was founded at Columbia University in November 2005. It is a unique research center committed to focusing on the biology of the motor neuron and two diseases caused by motor neuron degeneration: SMA and amyotrophic lateral sclerosis (ALS, or Lou Gehrig's Disease). Its mission is to create a cohesive translational research center where laboratory findings are quickly translated into new treatments for patients, and clinical learnings are regularly brought into the laboratory to shape new directions in research.

More than 40 leading researchers from numerous disciplines at Columbia University Medical Center and Columbia University's Morningside campus, including neurobiology, neurology, genetics, pathology, cell biology, physiology, anatomy, chemistry and pediatrics, have converged to form the center. The members include a Nobel Prize winner and three Investigators of the Howard Hughes Medical Institute.

"This is a remarkable time in SMA research," said Gerald D. Fischbach, M.D., executive vice president for Health and Biomedical Sciences and dean of the Faculty of Medicine, Columbia University Medical Center. "A decade ago we knew very little about what causes SMA, and now we know the gene that causes the disease. The SMA Foundation funding will allow our Center for Motor Neuron Biology and Disease to rapidly advance the knowledge of how this gene causes motor neurons to degenerate, and turn this into improved clinical care for patients."

The gift, which will be distributed over five years, is the largest ever made by a private foundation for SMA research. The grant symbolizes a new type of funding between private foundations and universities – "active funding," a style popularized by the Gates Foundation. As part of the grant agreement, the Center for Motor Neuron Biology and Disease will recruit new investigators to complement existing Columbia expertise and is incentivized to reach specific scientific goals.

"The SMA Foundation is a tremendous partner in our work," said Christopher E. Henderson, Ph.D., co-director of the Center for Motor Neuron Biology and Disease, along with Serge Przedborski, M.D., Ph.D., professor of neurology, pathology and cell biology, and Darryl De Vivo, M.D., the Sidney Carter Professor of Neurology. "We value their ongoing input and collaboration, which provide a new perspective on how to take basic research findings and move them toward the clinic. We hope that this approach will generate both new biological insights and relief for families living with SMA."

"We have known about SMA for more than a century; however, there remain significant gaps in our knowledge about this disease. We need to fill in these gaps in order to develop effective treatments," said Dr. De Vivo, who is also director of the SMA Clinic at Columbia University Medical Center. "Although it is the most common genetic cause of infant death and is the second most common autosomal recessive disease – a disease caused by a mutation of a single gene passed on by both parents – SMA has not received as much attention as many other less-common genetic disorders."

Since its inception, the SMA Foundation, led by co-founders Dinakar Singh and Loren Eng, has pledged nearly \$30 million to researchers at academic medical centers and biotech companies. The grant to the Center for Motor Neuron Biology and Disease is meant to unite, consolidate and amplify previous funding for SMA research and clinical efforts at Columbia University. These include:

- The SMA Clinic at Columbia University Medical Center, directed by Darryl De Vivo, M.D., the Sidney Carter Professor of Neurology
- The Pediatric Neuromuscular Clinical Research Network, a multi-center network that will conduct clinical trials in SMA patients as drug candidates are discovered. The center is also led by Dr. De Vivo.
- A 2004 Young Investigator Award in Spinal Muscular Atrophy to Umrao Monani, Ph.D., assistant professor of neurology, who was instrumental in developing a mouse model of SMA
- Brent Stockwell, Ph.D., assistant professor of biological sciences and chemistry, who uses new technology to screen tens of thousands of compounds for potential SMA drugs. In 2004, indoprofen, a close cousin of ibuprofen, was the first candidate identified by this new screening technology.

About Spinal Muscular Atrophy

Spinal muscular atrophy is a genetic, motor neuron disease characterized by the wasting of skeletal muscles due to progressive degeneration of nerve cells in the spinal cord. Loss of function leads to premature death due to respiratory problems; half of its victims die before reaching age two. The SMA Foundation estimates that there are currently 25,000 to 55,000 people suffering from SMA in the United States, Europe, and Japan and that the annual market potential for a drug to treat SMA could exceed \$500 million.

Currently there is no treatment for SMA, only palliative care. Research progress in the last decade, however, has made SMA scientists and clinicians optimistic that treatments can be developed in the next few years.

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About the Spinal Muscular Atrophy Foundation

The SMA Foundation is a nonprofit organization founded in 2003 that is dedicated to finding a treatment and potential cure for Spinal Muscular Atrophy (SMA). The Foundation provides funding for the full range of research from basic to clinical work conducted in academic laboratories as well as corporate therapeutics development: since its inception, the Foundation has awarded nearly \$30 million in sponsored research agreements. In addition, the Foundation is committed to raising awareness and generating support for increased federal and corporate funding for the disease. For more information on the Spinal Muscular Atrophy Foundation, visit www.smafoundation.org or call (646) 253-7100.

Columbia University Medical Center provides international leadership in pre-clinical and clinical research, in medical and health sciences education, and in patient care. The medical center trains future leaders in health care and includes the dedicated work of many physicians, scientists, nurses, dentists, and public health professionals at the College of Physicians & Surgeons, the College of Dental Medicine, the School of Nursing, the Mailman School of Public Health, the biomedical departments of the Graduate School of Arts and Sciences, and allied research centers and institutions. Columbia University Medical Center researchers are leading the discovery of novel therapies and advances to address a wide range of health conditions. www.cumc.columbia.edu

Founded in 1754 as King's College, **Columbia University** in the City of New York is the fifth oldest institution of higher learning in the United States and today is one of the world's leading academic and research institutions. For more information about Columbia University, visit www.columbia.edu.