

## Families of SMA and Cambria Biosciences Partner to Find New Treatment Avenues for SMA

**March 1, 2006 Libertyville, IL** -- Families of Spinal Muscular Atrophy today announced the funding of an industrial research program at Cambria Biosciences LLC to identify novel therapeutic targets for Spinal Muscular Atrophy (SMA). Cambria will utilize the model organism *Caenorhabditis elegans* to mimic genetic aspects of SMA

SMA is a genetic disorder that causes a chronic deficiency in the production of the Survival Motor Neuron (SMN) protein due to mutations in the *SMN1* gene. This protein is essential to the proper functioning of the motor neurons that originate in the spinal cord, as well as control of muscles in the limbs, neck and chest. In the United States there are more than seven million carriers of the genetic risk factors for SMA--and the disease affects approximately one in every 6,000 live births. Scientific research funded by Families of SMA and others has shown the presence of a second gene called *SMN2* strongly influences SMA disease severity.

“It is now well established that the loss of the *SMN1* gene leads to motor neuron loss and muscle degeneration in SMA patients and that the number of *SMN2* gene copies modulates disease severity,” said Dr. Beth Westlund of Cambria Biosciences and lead researcher on this project. “Additional studies suggest that other genes can affect the onset and severity of motor neuron loss in this condition. The focus of our research will be to search for genes that can alleviate the problems associated with defects in *SMN1* using the roundworm *Caenorhabditis elegans* or *C. elegans*, which is a very powerful model organism that is widely used by scientists to identify novel genetic interactions.

“This research should provide new targets for the development of novel therapeutics for SMA,” said Dr. Jill Jarecki, Families of SMA Research Director. “We are very pleased that Cambria, with its expertise in applied genetic research, is interested in partnering with Families of SMA in order to further the progress towards an effective treatment for SMA.”

Families of SMA awarded the grant to Dr. Westlund and Cambria to conduct this research as part of an ongoing commitment to funding the most promising research in the field of Spinal Muscular Atrophy. Cambria’s research plan was reviewed by Families of SMA’s Scientific Advisory Board, which is comprised of top SMA researchers from around the world.

### **About Spinal Muscular Atrophy**

Spinal muscular atrophy (SMA), the number one genetic killer of children under the age of two, is a group of inherited and often fatal diseases that destroys the nerves controlling voluntary muscle movement, which affects crawling, walking, head and neck control, and even swallowing. In the United States alone there are more than seven million carriers of the genetic risk factors for SMA--and the disease affects approximately one in every 6,000 live births. SMA is usually diagnosed at less than 18 months of age.

### **About Families of SMA**

FSMA is the largest international organization dedicated solely to eradicating SMA by promoting and supporting research in both the private and public sector, helping families cope through informational programs and support, and educating the public and the medical community about SMA. The organization, originally founded in 1984 by small group of parents, has grown to more than 32 chapters and affiliates worldwide and more than 5,000 member families and is a founding member of the International Alliance for Spinal Muscular Atrophy. FSMA receives the majority of its funding through volunteer efforts, funding over \$25 million to date, and continues to increase its funding commitments each year with \$15 million in new research planned over the next three years. For more information visit the website [www.curesma.org](http://www.curesma.org) or call 1-800-886-1762.

### **About Cambria Biosciences**

Cambria Biosciences LLC is a Boston-area drug discovery company focused on underserved neurological disorders for which there are well-defined disease mechanisms, animal models, and criteria for clinical trials. Cambria's scientists combine the power of genetics and high-throughput chemical screening to discover new drug candidates and how they work. By characterizing the biological mechanisms of active compounds in living biological systems that serve as models for human disease, these technologies provide a rapid way to advance promising therapeutic candidates to the clinic. In addition, Cambria earns revenues from corporate partnerships that leverage its technology platform for multiple applications, including agriculture and animal health. For more information, please visit [www.cambriabio.com](http://www.cambriabio.com).

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