

**Inserm**



Institut national  
de la santé et de la recherche médicale



# SPINAL MUSCULAR ATROPHY FOUNDATION

## **Critical Research Tools for Spinal Muscular Atrophy Drug Discovery New Licensing Opportunity from the SMA Foundation and Inserm**

**NEW YORK, NY**, January 31, 2006- The Spinal Muscular Atrophy Foundation announced the completion of a new licensing agreement that will facilitate access to intellectual property fundamental to drug discovery efforts for the disease. Spinal muscular atrophy (SMA) is the leading genetic killer of infants and toddlers; there is no known treatment or cure. Under the terms of the agreement, the SMA Foundation has licensed, with the option to sublicense, patent rights for the SMN gene as held by Inserm, the National Institute of Health and Medical Research of France. Mutations in the SMN gene are the cause of spinal muscular atrophy and use of gene sequences covered by the patent rights is critical to drug discovery efforts in SMA.

The SMN gene was discovered by Prof. Judith Melki and Prof. Arnold Munnich at the Inserm laboratory located at Necker Hospital in Paris, with the consistent funding support of Inserm and the Association Française Contre les Myopathies (AFM). While the SMA Foundation had no role in the creation, funding or patenting of the original discoveries, it is playing an important role in the advancement of drug discovery efforts in SMA. The novel licensing agreement is focused on the discovery of pharmacological molecules offering a therapeutic solution and it is designed to streamline access to intellectual property associated with the SMN gene and thus speed the development of assays, models and other tools that are critical to the discovery process. It is the hope and desire of each of the contributing organizations that this effort will accelerate the development of a treatment for SMA, a devastating neurodegenerative disease that leads to death by age two in up to 50% of patients.

This license agreement becomes part of a portfolio of critical research tools consolidated by the SMA Foundation for investigators in both academic and

commercial labs working in the field. This portfolio includes licensing opportunities for mouse models of SMA created by the University of Wuerzburg and The Ohio State University and the portfolio also includes tools derived from ongoing research enterprises. Interested parties are invited to contact the Foundation directly at [www.smafoundation.org](http://www.smafoundation.org) for more information about this and other licensing opportunities for SMA research.

### ***About Spinal Muscular Atrophy***

Spinal Muscular Atrophy is a genetic, motor neuron disease caused by mutations in a single gene, SMN1. The mutation causes a loss of motor neurons in the spinal cord and wasting of skeletal muscles, leading to increasing muscular weakness and loss of function. Spinal Muscular Atrophy is often likened to polio because the same spinal cord cells are attacked in each disease. Victims of SMA are at high risk of premature death due to respiratory failure. An estimated 50,000 patients in the US, EU and Japan are affected by the disease.

### ***About SMA Foundation***

The SMA Foundation is a nonprofit organization, founded in 2003, 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. In addition, the Foundation is committed to raising awareness, education and increased federal funding and support for SMA research broadly. Research partners of the Foundation include Columbia University, Massachusetts General Hospital, Washington University, Curis Inc and Regeneron Pharmaceuticals. For more information on the Spinal Muscular Atrophy Foundation, visit [www.smafoundation.org](http://www.smafoundation.org) or call (646) 253-7100.

### ***About Inserm***

Founded in 1964, Inserm is the only French public institute entirely dedicated to biological and medical research and improving public health. Inserm has 13,000 research, technical and administrative staff. In this government-funded institute, all diseases are studied from the most common to the most rare. Inserm, which is involved in all aspects of research, has a mission to conduct basic and clinical research to ultimately improve public health. The institute has more than 360 laboratories throughout France and interacts closely with social and economic partners on a national and international level. Inserm works with about 450 pharmaceutical, biotechnology and biomedical technology companies, and holds more than 1,000 collaborative research and license agreements.

## ***About AFM***

Created in 1958, the French Muscular Dystrophy Association (AFM) is a non-profit association registered under French law which includes patients and families affected by neuromuscular diseases. Its mission is to find a cure for these seriously disabling - and currently incurable- diseases, most of which have a genetic origin, and also help people affected by them. Funding comes from a Telethon, the Association's annual fund-raising event held on the first week-end in December. Thanks to the generosity of millions of donors, AFM has become a major player in genetic research in France. In 1990, it created the Genethon laboratory which produced the first maps of the human genome, thus paving the way for the sequencing of the human genome. AFM supports each year more than 400 research programs, among them more than 30 clinical trials in gene therapy or cellular therapy concerning for example muscular, eyes, skin or blood diseases.

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