



Balancing Life's Tough Times™

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## Families of Spinal Muscular Atrophy and deCODE Open Screening Capabilities to Third-party Compounds

*Latest stage in aggressive drug development drive for Spinal Muscular Atrophy*

Libertyville, ILLINOIS, January 7, 2004 — Families of Spinal Muscular Atrophy (FSMA), an organization founded to promote research leading to effective treatment of Spinal Muscular Atrophy (SMA), a debilitating and often fatal disease, today announced the next step in its partnership with deCODE genetics (Nasdaq:DCGN). FSMA and deCODE are making available deCODE's high-throughput screening capabilities to test promising compounds that may increase SMN levels or supplement SMN function.

Using compounds identified through previous FSMA-funded research, deCODE's Chicago-based pharmaceuticals group has completed initial screening and is now working to optimise hits to obtain the best activity and drug like properties. FSMA and deCODE are now hoping to collaborate with other parties in order to make available the necessary chemistry resources to progress other potentially effective compounds through the evaluation process. These services will be made available under the current collaboration that is being funded by FSMA.

SMA is a genetic disorder involving the deletion or mutation of one or both copies of the Survival Motor Neuron (SMN1) gene on chromosome 5. This causes a chronic deficiency in the production of the SMN protein, essential to the proper functioning of the motor neurons in the spinal cord and to the control of muscles in the limbs, neck and chest. [The initial hits](#) developed by FSMA and deCODE [increase expression of the SMN2 gene](#), raising the level of SMN protein.

"Families of SMA is committed to finding a cure for SMA," said Audrey Lewis, executive director of FSMA. "[With deCODE we have made significant strides in identifying promising new compounds, and we want to extend this effort to](#) identify any and all compounds that may lead to an effective treatment or cure [for SMA](#)."

FSMA-sponsored research has contributed not only to the identification of both the SMN1 and SMN2 genes. Previous FSMA-sponsored drug discovery work has identified a series of compounds that may increase the expression of the SMN2 gene and thus the amount of fully functional protein produced. If a compound can be found that can do this safely and effectively, it may be possible to restore the proper amount

of SMN protein in the body and slow or reverse the disease process. deCODE's current work for FSMA focuses on identifying and developing such a compound.

“We are pleased to be bringing to our collaboration with FSMA the full capabilities of an experienced drug discovery partner,” said Kari Stefansson, CEO of deCODE. “We are committed to helping FSMA develop new experimental drugs for therapy of SMA as quickly and effectively as possible.”

In the U.S. alone there are more than 7 million carriers of the genetic risk factors for SMA, and the disease affects approximately one in every 6000 live births. SMA is usually diagnosed when babies are less than 18 months old, although certain types of the disorder can appear in later life.

#### **About FSMA**

Families of SMA is a volunteer-driven nonprofit organization based in Libertyville, IL. It was founded in 1984 by parents of children suffering from SMA. FSMA is the largest international organization dedicated solely to eradicating SMA by promoting and supporting research, helping families cope through informational programs and support, and educating the public and the medical community about SMA. The organization has more than 24 chapters worldwide and more than 5,000 member families and is a founding member of the International Alliance for Spinal Muscular Atrophy. FSMA has funded \$16 million towards SMA research, and has already committed an additional \$12 million over the next three years. In addition, FSMA has provided more than three million dollars in patient services, which includes an equipment pool, conferences, family newsletter and phone support. For more information visit the website [www.curesma.com](http://www.curesma.com) or call 1-800-886-1762.

#### **About deCODE**

deCODE is using population genetics to create a new paradigm for healthcare. With its uniquely comprehensive population data, deCODE is turning research on the genetic causes of common diseases into a growing range of products and services — in pharmaceuticals, gene and drug discovery, DNA-based diagnostics, pharmacogenomics, bioinformatics, and clinical trials. deCODE's pharmaceuticals group, based in Chicago, and deCODE's biostructures group, based in Seattle, conduct downstream development work on targets derived from deCODE's proprietary research in human genetics as well as contract service work for pharmaceutical and biotechnology companies. deCODE is delivering on the promise of the new genetics.<sup>SM</sup> Visit us on the web at [www.decode.com](http://www.decode.com).