The Scientific and Medical Community Says SMA Treatment or Cure is within Reach

Spinal Muscular Atrophy (SMA) is the leading genetic killer of infants and toddlers. Each year, up to 1,000 babies are born with this deadly genetic disease that dooms them to a life of suffering and premature death, usually before the age of two. An estimated 25,000 Americans suffer from this untreatable, incurable, and fatal disease. Over seven million potential parents are carriers of this deadly gene.

SMA attacks the spinal cord nerve cells and causes skeletal muscles to waste away, trapping healthy minds inside withering bodies. Over time, SMA victims lose muscle control and strength, leading to a progressive inability to walk, stand, sit up, move and, eventually, breathe.

However, dramatic discoveries in the past seven years have catapulted SMA from being a poorly understood disease to one with better prospects for treatment than perhaps any other major genetic disease.

And, in an extraordinary show of support, the international scientific community agrees.

In a recent letter to the director of the National Institutes of Health (NIH), leading scientists and clinicians worldwide urged NIH to rapidly implement translational research for SMA, which will focus on translating basic science into actual treatments and drugs for the disease.*

Translational research is critical to the development of a treatment not only for SMA, but also for other similar diseases.

It is their most educated expectation that with NIH funding of $20 to $30 million a year, an effective therapy for spinal muscular atrophy can be achieved in the near term of five years or less.

Moreover, they believe that the development of an SMA treatment would be an example of the potential return on the tremendous investments that have been made in molecular genetic research.

Tens of thousands of families devastated by SMA, and millions of parents who live with this gene, thank these highly respected researchers for lending their names and expertise to accelerate the development of treatment or cure for this horrific disease.

To view the full text of the letter to NIH, log on to: **www.smafoundation.org/science.**

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"...expectation that with NIH funding of $20 to $30 million a year, an effective therapy for SMA can be achieved in five years or less."

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* Letter to Dr. Elias A. Zerhouni, Director, and Dr. Audrey S. Penn, Acting Director, National Institutes of Health, signed by leading scientists and clinicians worldwide. March 2003.

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