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Helping Jerry's Kids

SPINAL MUSCULAR ATROPHY
FOUNDATION

Enhance Efforts to Find a Treatment for Spinal Muscular Atrophy: Cosponsor the SMA Treatment Acceleration Act (HR 2149; S 2118)

Spinal Muscular Atrophy (SMA) is a hereditary disorder that destroys the nerves controlling voluntary muscle movement. It is the number **one** genetic killer of children under the age of two. SMA is a relatively common “rare disorder.” It is estimated that SMA occurs in about 1 in every 6,000 births. Approximately 1 in 40 individuals (7.5 million Americans) carries the gene that causes SMA, making it the second most common autosomal recessive genetic disorder. This incidence rate shows neither racial nor gender bias.

Presently, there is no known treatment for SMA, though there have been several exciting research breakthroughs over the past decade. Among more than 600 neurological disorders, SMA has been identified by the National Institutes of Health as being one of the diseases closest to treatment based on scientists’ advanced genetic understanding of the disease.

In order to support the investigators, clinicians, and families who are working to find a treatment or cure for SMA, the SMA community, including Families of SMA, FightSMA, the Muscular Dystrophy Association (MDA), and the SMA Foundation, has united behind the “**SMA Treatment Acceleration Act.**” This legislation authorizes federal funding in order to:

- Upgrade and unify SMA clinical trials sites and establish a national clinical trials network for SMA.
- Establish a Data Coordinating Center to provide expert assistance and advice to SMA clinical trials sites.
- Expand and intensify federally supported research programs with respect to pre-clinical translational research related to SMA.
- Enhance the SMA patient registry and for expanded research on the epidemiology of SMA.
- Establish an Interagency SMA Research Coordinating Committee, consisting of representatives from relevant government agencies and the public, to coordinate government activities relating to SMA, develop a comprehensive strategy for improving and expanding SMA research, make recommendations to strengthen collaborative research across multiple institutes at NIH, and identify barriers to the development of drugs for treating SMA.
- Promote collaborative research at the National Institutes of Health to ensure cooperation across multiple Institutes regarding research related to SMA.
- Establish and implement a program for providing information and education on SMA to health professionals and the general public related to advances in the screening, diagnosis and treatment of SMA and the provision of care to SMA patients.

The SMA Treatment Acceleration Act provides federal support to complement the substantial private funding that national non-profit organizations are investing to find a treatment for SMA. Passage of this landmark legislation will enable investigators to mount national clinical trials to demonstrate that identified therapeutics are safe and effective for SMA patients.

If you have any questions about the SMA Treatment Acceleration Act, or if you would like information about how you can become a cosponsor, please contact Spencer Perlman of Families of SMA (spencer@fsma.org / 202-333-5750), Caroline Gibson of FightSMA (carolinegibson@fightasma.com / 804-515-0080), Annie Kennedy of MDA (akennedy@mdausa.org / 202-828-8560), or Laura Lay of the SMA Foundation (llay@swdc.com / 202-589-0800).