



Summit on Spinal Muscular Atrophy Investigators meet to advance clinical research for SMA, the leading genetic cause of infant and toddler mortality

(Bethesda, MD) – With the recent introduction of SMA legislation in both the U.S. House of Representatives and the U.S. Senate to create a national clinical trials network for spinal muscular atrophy (SMA), the leading advocacy organizations hosted a two-day summit to work toward reducing or eliminating barriers to development of the drugs that would be tested in that network.

The SMA Summit on Drug Development, that was held on September 28 and 29 at the Hyatt Regency Bethesda, in Bethesda, MD, included representatives from the pharmaceutical industry, family support groups, clinicians and government. These stakeholders in the clinical research process discussed infrastructure, regulatory, and community support needs for evaluating treatments in development. The goal is to speed the development and approval of drugs designed to treat SMA, for which there is currently no approved treatment.

Spinal muscular atrophy is a neuromuscular disease that kills more children under two than any other genetic disorder. It affects the victim's motor neurons, causing muscle atrophy, loss of strength, disability and premature death due to respiratory complications in the majority of affected children.

Both the bill and the drug summit were conceived and implemented through the collaboration of SMA organizations and other parties that have an interest in accelerating a treatment and cure for this disease. These efforts are in anticipation of clinical trials to test new drug treatments for SMA.

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