

## Cure SMA Presents Isis Pharmaceuticals with SMA Breakthrough Award

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ELK GROVE VILLAGE, Ill., Nov. 17, 2014 /PRNewswire-USNewswire/ -- On November 15, Isis Pharmaceuticals [ISIS, -0.53%](#) was presented with the 2014 SMA Breakthrough Award as part of the Cure SMA Gala of Hope in Seal Beach, CA. The award was accepted by Stanley T Crooke, Ph.D., M.D., who is the founder, chairman, and chief executive officer of Isis Pharmaceuticals.

The award was presented by Kenneth Hobby, president of Cure SMA, an organization that has grown from grassroots beginnings to become a leading funder of research into spinal muscular atrophy (SMA), with nearly \$57 million funded in its 30-year history.

Isis was recognized for their work with ISIS-SMNRx, an antisense drug that is being developed to treat SMA. SMA is the leading genetic cause of death for children under two, and approximately 1 in every 50 individuals is a carrier of the mutation that causes SMA.

SMA is caused by a mutation in the survival motor neuron gene 1 (SMN1). Because of this mutation, individuals with SMA don't produce enough survival motor neuron (SMN) protein and this prevents the motor neuron cells in the spinal cord from functioning properly.

ISIS-SMNRx is a drug designed to increase of the SMN protein by inducing the survival motor neuron gene 2 (SMN2), a related gene that typically makes only a small amount of functional SMN protein, to make more functional SMN protein.

Earlier this year, ISIS-SMNRx advanced to Phase 3 clinical trials. This study represents the first Phase 3 trial in the world for a drug that addresses the underlying cause of SMA.

"SMA is a devastating disease. At Isis, we are working hard to develop a drug, which we hope could make a difference in the lives of patients with SMA," said Dr. Crooke. "On behalf of everyone who contributed to the discovery and development of ISIS-SMNRx, I am honored to accept this recognition, and I applaud the efforts of Cure SMA, SMA patients and their families for their support in our efforts to develop ISIS-SMNRx. We are pleased to have rapidly advanced this important investigational drug to late-stage development. We are evaluating ISIS-SMNRx in infants with SMA and plan to initiate the second Phase 3 study before the end of the year in children with SMA. In addition, we will continue to dose the infants and children in our earlier clinical studies who are being evaluated in open-label extension studies."

"We are thrilled to present Isis with the first-ever SMA Breakthrough Award. The pace of research into SMA has increased considerably in the last 5-10 years, and Isis has been at the forefront of that," said Kenneth Hobby, president of Cure SMA. "Programs like ISIS-SMNRx give us hope that we will find a treatment for SMA."

Cure SMA made several grants to help develop the approach behind ISIS-SMNRx, beginning with a grant in 2003. The most recent grant was given just last year. Cure SMA and Isis have also partnered in patient recruitment for ongoing clinical trials.

SOURCE Cure SMA

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