SMA Research Update – Providing Hope!

Currently, there are (7) active studies within Columbia University Medical Center:

An Open-Label Extension Study for Patients With Spinal Muscular Atrophy **Who Previously Participated in Investigational Studies of ISIS 396443 (SHINE)**

****Closed for enrollment ****

This study was initiated and the protocol was registered by Ionis Pharmaceuticals, Inc. In August 2016, Biogen assumed responsibility for this study. The primary objective is to evaluate the long-term safety and tolerability of nusinersen (ISIS 396443) administered by intrathecal (IT) injection to participants with Spinal Muscular Atrophy (SMA) who previously participated in investigational studies of nusinersen. The secondary objective is to examine the long-term efficacy of nusinersen administered by IT injection to participants with SMA who previously participated in investigational studies of nusinersen.

Eligibility

Ages Eligible for Study:	Child, Adult, Senior
Sexes Eligible for Study:	All
Accepts Healthy Volunteers:	No

Key Inclusion Criteria:

- Signed informed consent of parent or guardian and signed informed assent of participant, if indicated per participant's age and institutional guidelines.
- Completion of the index study in accordance with the study protocol or as a result of Sponsor decision (e.g., early termination of the index study) within the preceding 16 weeks

Key Exclusion Criteria:

- Have any condition or worsening condition which in the opinion of the Investigator would make the participant unsuitable for enrollment, or could interfere with the participant participating in or completing the study
- Clinically significant abnormalities in hematology or clinical chemistry parameters or electrocardiogram (ECG), as assessed by the Site Investigator, at the Screening visit that would render the participant unsuitable for participation in the study

- Participant's parent or legal guardian is not willing or able to meet standard of care guidelines (including vaccinations and respiratory syncytial virus prophylaxis if available), nor provide nutritional and respiratory support throughout the study
- Treatment with another investigational agent, biological agent, or device within one month of Screening, or 5 half-lives of study agent, whichever is longer

  NOTE: Other protocol defined Inclusion/Exclusion criteria may apply.

Estimated Enrollment:	289
Actual Study Start Date:	November 4, 2015
Estimated Study Completion Date:	August 1, 2022

Additional participating lcoations:

36 locations throughout the United States, Australia, Canada, Europe, China, Japan, Korea, Turkey, and the UK (for a complete list please go to clinicaltrials.gov).