SMA Research Update – Providing Hope!

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

An Open-Label Study to Investigate the Safety, Tolerability, and Pharmacokinetics/Pharmacodynamics of RO7034067 in Adult and Pediatric Patients With Spinal Muscular Atrophy (Jewelfish)

****Active and recruiting****

This is a multi-center, exploratory, non-comparative and open-label study to investigate the safety, tolerability, pharmacokinetic (PK) and PK/pharmacodynamic (PD) relationship of RO7034067 in adults and children with Type 2 and Type 3 Spinal Muscular Atrophy (SMA) who have been previously treated with a survival of motor neuron 2 (SMN2)-targeting therapy.

Eligibility

Ages Eligible for Study:	12 Years to 60 Years (Child, Adult)
Sexes Eligible for Study:	All
Accepts Healthy Volunteers:	No

Inclusion Criteria:

- Confirmed diagnosis of 5q-autosomal recessive SMA
- Previous participation in a study with an SMN2-targeting antisense oligonucleotide or SMN2 splicing modifier other than RO7034067
- Negative blood pregnancy test at screening and agreement to comply with measures to prevent pregnancy and restrictions on sperm donation

Exclusion Criteria:

- Inability to meet study requirements
- Concomitant participation in any investigational drug or device study
- Previous participation in an SMN2-targeting antisense oligonucleotide or SMN2 splicing modifier study other than RO7034067 within 90 days prior to screening
- Previous participation in any investigational drug or device study, other than SMN2 targeting antisense oligonucleotide or SMN2 splicing modifier study, within 90 days prior to screening, or 5 half-lives of the drug, whichever is longer
- Any history of gene or cell therapy
- Unstable gastrointestinal, renal, hepatic, endocrine, or cardiovascular system diseases as considered to be clinically significant by the Investigator
- Presence of clinically significant electrocardiogram (ECG) abnormalities before study drug administration indicating a safety risk for the participant as determined by the Investigator.

- Significant risk for suicidal behavior, in the opinion of the Investigator as assessed by the C-SSRS
- Any major illness within one month before the screening examination or any febrile illness within one week prior to screening and up to first dose administration
- Recently initiated treatment (within less than [<] 6 months prior to enrollment) with oral salbutamol or another beta 2-adrenergic agonist taken orally
- Any prior use of chloroquine, hydroxychloroquine, retigabin, vigabatrin or thioridazine, is not allowed
- Ascertained or presumptive hypersensitivity (e.g., anaphylactic reaction) to RO7034067 or to the constituents of its formulation
- Concomitant disease or condition that could interfere with, or treatment of which might interfere with, the conduct of the study, or that would, in the opinion of the Investigator, pose an unacceptable risk to the participant in this study
- Recent history (less than one year) of ophthalmological diseases that would interfere with the conduct of the study as assessed by an ophthalmologist. Participants in whom Organic Cation Transporter or Optical Coherence Tomography (OCT) measurement of sufficient quality cannot be obtained at screening will not be enrolled

If you would like more information about this study please contact Claudia Chiriboga, MD, MPh or Luz Sanabria at ls2328@cumc.columbia.org

Estimated Enrollment:	24
Actual Study Start Date:	March 2, 2017
Estimated Study Completion Date:	January 31, 2021

Additional participating locations:

Italy: Policlinico Agostino Gemelli; Dipartimento di Neuropsichiatria Infantile

Roma, Lazio, Italy