SOD1 Phase I Antisense Trial Shows Promise and C9orf72 Phase I Antisense Trial Begins

The ALS Association is proud to be the first investor in antisense technology, dating back to 2004 when antisense was just an idea in Dr. Don Cleveland's lab at University of California San Diego (UCSD). Fast forward to 2018 and we are seeing promising results in antisense drugs targeting the two most common causes of inherited ALS, mutations in the SOD1 and C9orf72 genes.

After years of hard work by The ALS Association-funded investigators, Biogen recently announced promising results of the phase 1 SOD1 antisense trial (BIIB067) that is now moving into the next clinical phase and announced the initiation of a phase 1 antisense trial (BIIB078) targeting C9orf72.

The phase 1 SOD1 antisense trial enrolled 70 people with ALS that demonstrated proof-of-biology and proof-of-concept for the BIIB067. At the highest dose tested in 10 people with ALS over a three-month period, compared to placebo, BIIB067 showed a statistically significant lowering of SOD1 protein in the cerebral spinal fluid and a numerical trend towards the slowing of clinical decline as measured by the ALS Functional Rating Scale-Revised.

With these encouraging interim results, Biogen is urgently moving BIIB067 forward into a pivotal study. This means that Biogen could use the results of the pivotal study, if they are positive, as a basis to file a new drug application to the FDA.

Read more for a primer about antisense technology and how The ALS Association has played an integral part in moving this promising technology from the laboratory into clinical trials.

https://alsadotorg.wordpress.com/2018/08/17/the-als-associations-early-investment-in-antisense-technology-is-having-a-real-impact/
https://alsadotorg.wordpress.com/2018/08/22/meet-dr-don-cleveland-prominent-als-researcher-who-pioneered-antisense-technology/
Below is a statement we would like to share from Biogen that explains the company's exciting progress in antisense technology.

Dear members of the ALS community,
As we look towards 2019, we wanted to provide an update on our investigational therapies for ALS to those of you around the world who are impacted by this disease.
We have made important progress along with our partner Ionis Pharmaceuticals in moving forward the development of our investigational therapies for familial ALS, BIIB067 and BIIB078. BIIB067 is designed to treat ALS in adults with confirmed mutations in the superoxide dismutase 1 (SOD1) gene. BIIB078 targets the chromosome 9 open reading frame 72 (C9ORF72) mutation (often called a "repeat expansion"), which is the leading familial cause of ALS.
Both of our ongoing studies in ALS are progressing and we wanted to share some important updates with you:

BIIB067 for SOD-1 Related ALS

- *Our Phase 1 study of BIIB067 recently completed enrollment. A total of 70 people enrolled in this effort.*
- *An additional study of BIIB067 remains underway, which evaluates outcomes over a longer period of time.*
  - *On December 6, we announced positive results from an interim analysis of this Phase 1 study, as well as Biogen's decision to obtain a license to develop and commercialize BIIB067 for SOD1 ALS from Ionis Pharmaceuticals.*
  - *Results of the interim analysis were encouraging, and Biogen is urgently advancing BIIB067 to a pivotal clinical study, meaning it could potentially serve as a basis for regulatory filing. Study timing and enrollment plans are not yet confirmed but we will communicate these as soon as we are able to.*
  - *You can learn more about the status of the BIIB067 program in our news release, here.*

BIIB078 for C9ORF27 Related ALS

- *Our Phase 1 clinical trial for BIIB078 was initiated this fall, and the first patients have now been dosed. We hope to have 12 U.S. as well as several Canadian and European sites open and enrolling within the next several months to complete enrollment as urgently as possible. These studies will help us better understand the safety, tolerability and pharmacokinetic profile of the investigational treatments and appropriately plan for future studies.*
  We're grateful to those in the ALS community who have chosen or sought to participate in one of our clinical trials and for their belief in our science. Groundbreaking clinical research cannot be accomplished without a commitment from people living with ALS who are willing to join a clinical study.
  While we wish we could accommodate every person who wants to join a study, clinical studies at the early stages typically enroll a small number of participants and have a limited number of sites available. We recognize that this can be very frustrating, especially for people who are eager to access investigational compounds when there are few treatment options available.
  We are humbled by the overwhelming response for participation in our studies. Please know we are working tirelessly with study sites and investigators to optimize the study participant experience while ensuring that investigators have the resources they need to properly conduct a study and evaluate study data on our behalf.
  Our goal is to evaluate the data for both studies as quickly as we can, looking for suitable signals of safety and efficacy that will help move our development programs forward and advance future studies.
  In 2019, we will continue to provide quarterly updates on progress in our development plans, including opportunities for participation in Biogen clinical trials. Biogen is committed to finding a treatment for ALS. This commitment extends to individuals participating in our studies, their families, caregivers and the greater ALS community.
  For more information or resources on clinical trial enrollment, please reach out to patientcenter@biogen.com.

  Sincerely,

  Biogen