Biogen to Present Promising Results of Phase 1/2 Trial of Antisense Targeting SOD1

Next week at the American Academy of Neurology Meeting (AAN) in Philadelphia, Biogen will present promising results of the phase 1/2 study of its newly named investigational therapy tofersen (previously BIIB067), which is now enrolling in a phase 3 trial. Tofersen is an antisense oligonucleotide (ASO), a type of designer DNA drug, targeting SOD1.

The ALS Association is proud to be the first funder of antisense technology. Our support dates back to 2004, when antisense was just an idea in Dr. Don Cleveland's lab at University of California San Diego (UCSD). This new potential treatment targets the second most common cause of inherited ALS, mutations in the SOD1 gene.

While this news is promising, it is important to note that the drug (tofersen) is still experimental. Biogen also plans to provide updates to their clinical program include the enrolling phase 1 antisense trial (BIIB078) targeting C9orf72, the most common genetic cause of ALS, and an upcoming phase 1 trial testing drug BIIB100 for people with sporadic ALS.

These drugs are applications of research the Association has been funding for many years, including with Ice Bucket Challenge donations.

Click here and here 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.
Below is a statement we would like to share from Biogen that gives an update on its current and upcoming ALS trials.

ALS Community Statement *May 2019*
Dear members of the ALS community,

Thank you for your continued support as we advance the development of our investigational therapies to treat people living with ALS. We truly value your interest, feedback and partnership as we continue to move our programs forward. In our commitment to keep you informed of our progress, we have a few exciting updates that we want to share with you.

Tofersen (BIIB067) for the Treatment of ALS with SOD1 Mutations
Along with our partner Ionis Pharmaceuticals, we have made great progress in moving the tofersen (previously called BIIB067) program forward into Phase 3 development. Tofersen is an antisense oligonucleotide (ASO) being studied for the treatment of ALS in adults with a confirmed superoxide dismutase 1 (SOD1) genetic mutation.

Data from the interim analysis of the Phase 1/2 tofersen trial will be presented on May 7, 2019 at the American Academy of Neurology (AAN) Annual Meeting in Philadelphia, PA, expanding on the study results that we shared with you last December.

In summary, out of the 38 individuals who received study drug, the 10 trial participants who received the highest dose tested of tofersen (100 mg) over a three-month period had a statistically significant reduction in the amount of SOD1 protein in their cerebrospinal fluid when compared to the 12 trial participants who received placebo ($p<0.002$). Furthermore, the participants who received tofersen 100mg showed a numerical trend towards slowing of decline in clinical function, respiratory function, and muscle strength compared to those who received placebo.

While we continue to analyze the data, we are encouraged by the initial findings and will be collecting additional data to further assess the efficacy and safety of tofersen in a larger SOD1 population and over a longer duration. To this end, we recently enrolled the first patient in our Phase 3 trial, called VALOR, for which we aim to enroll approximately 60 individuals at 32 sites across 10 countries. We are working with regulatory agencies to further define the scope of the clinical data package that is required to support an approval to market tofersen. We believe that, if successful, the ongoing trial will be an important component of this package. To learn more about this trial, please visit https://clinicaltrials.gov/or reach patientcenter@biogen.com.

You can also access additional information in our news release, here.

BIIB078 for the Treatment of C9orf72-Related ALS

Individuals diagnosed with C9orf72-related ALS are being recruited by selected sites for the first-in-human clinical trial of BIIB078.

This study is designed to investigate the safety, tolerability, and pharmacokinetics of increasing doses of BIIB078 compared to a group that receives a placebo. As is common in a clinical trial in which several dose strengths are being evaluated, Biogen assesses data obtained from each dose group before enrolling participants to receive the next highest dose. This means that, by design, the clinical trial goes through periods of more or less active recruitment. This period of evaluation may not always be reflected on clinicaltrials.gov, but is critical to ensure the safety of participants.

Also, since the safety and tolerability profile of BIIB078 has not yet been fully studied in humans, as is typical with trials in this phase of development, the number of participants to be included in the clinical trial will be limited. While we realize there is a great unmet need in the C9orf72 community and strong desire for access to investigational therapies, we appreciate your understanding as we uphold the highest standards of care.

Currently we have 9 active sites in the U.S. and Canada and hope to have several more open globally in the next few months to complete enrollment as soon as possible.

BIIB100 for the Treatment of Sporadic ALS

Finally, BIIB100 is the newest addition to our ALS research program; it is an investigational therapy for the treatment of ALS due to sporadic causes. We will begin investigating the safety and tolerability of BIIB100 for the treatment of ALS in a Phase 1 trial as early as June 2019. The trial will be a single ascending dose study, meaning that participating individuals will receive one dose of the treatment. We will share further updates once study sites begin enrolling patients.

Our Commitment

We are incredibly grateful for those in the ALS community who believe in the science behind our investigational treatments and have chosen or sought to participate in one of our trials. We realize that we cannot accommodate every person who wants to join a study and how frustrating that can be for people who hope to seek access to these treatments.

We recognize that there are limited trials and treatments available, so we will continue to work very closely with study sites and investigators to enhance the study participant experience while ensuring that investigators can properly conduct the study and evaluate the data.

With all of our investigational treatments, our goal is to evaluate the data as quickly as we can, looking for positive safety and efficacy signs that will support the advancement of our development programs and the broadest access to appropriate individuals.

As part of our commitment to finding a treatment for people living with ALS, we will continue to provide you and the greater ALS community with regular updates of our clinical development plans, including opportunities for participation in Biogen clinical trials.

For more information or resources on clinical trial enrollment for any of our ongoing studies, please reach out to patientcenter@biogen.com.

Sincerely,
Biogen