

Biogen Announces Latest Updates on ALS Clinical Trials During the Pandemic

While social distancing has created challenges to conducting clinical trials during the COVID-19 pandemic, the ALS community can't wait- and won't wait – until the pandemic is over to find treatments and a cure.

In a recent statement, Biogen emphasized the sense of urgency that drives its work.

“We want to reassure (the ALS community) that during this challenging time, we remain committed to developing and delivering potential new treatment options for people with ALS. We continue to move research efforts forward to the best of our ability across our ALS programs.”

The company, a partner of The ALS Association, recently published promising results from its phase 1–2 Trial of Antisense Oligonucleotide Tofersen for SOD1 ALS and is now actively enrolling participants for their [Phase 3 Valor](#) study. It also announced that there is an open-label extension available in the study.

“The ALS Association is proud to be the first funder of antisense technology,” said Kuldip Dave, Ph.D. vice president of research at The ALS Association. “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.”

Two additional clinical trials are also moving forward despite the current challenges presented by the spread of the coronavirus. Below is a statement we would like to share from Biogen providing an update on its current ALS clinical trials.

Read Biogen's full statement below:

ALS Community Statement, July 2020

Dear members of the ALS community,

At Biogen, we understand that our world is facing uncertainty and change caused by the COVID-19 pandemic. The latest resources regarding Biogen's response can be found through our [information center](#).

We want to reassure you that during this challenging time, we remain committed to developing and delivering potential new treatment options for people with ALS. We continue to move research efforts forward to the best of our ability across our ALS programs.

With this commitment in mind, we have a few important updates to share with you.

ALS Clinical Trial Enrollment Status

We recognize that there may be questions about the status and enrollment of our clinical trials due to COVID-19. Currently, we have three ALS clinical studies that are recruiting participants. Please know that we are consistently communicating with each study site across the globe to help solve challenges that may come up.

- **Tofersen:** The safety and potential effectiveness of the investigational drug tofersen are being assessed in the Phase 3 VALOR study and its open-label extension. VALOR

is actively enrolling a cohort of participants with rapidly progressive SOD1-ALS. Additional information and a screener to inform potential eligibility for people living with ALS in the U.S. can be found [here](#).

- **BIIB078:** The safety and tolerability of escalating doses of BIIB078 in people with ALS caused by a mutation of the C9ORF72 gene are being assessed in the Phase 1 study.
- **BIIB100:** The safety and tolerability of escalating doses of BIIB100 in people with all forms of ALS, including genetic subtypes are being assessed in the Phase 1 study.

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

Publication of the Phase 1/2 Tofersen Data in the *New England Journal of Medicine*

The *New England Journal of Medicine*, a prestigious medical journal, [published results](#) from an early stage investigational study of tofersen.

We are encouraged by these early results and look forward to further evaluating the potential of tofersen. Biogen would like to recognize all people who participated in the trial and site staff for their contributions.

We are grateful for your support as we work towards the goal of bringing forth treatments for ALS. We look forward to sharing additional updates with you as they are available.

Sincerely,
Biogen