

Prosetin – Phase I Clinical Trial FAQs

What is prosetin?

Prosetin is the first potential new drug under investigation for the treatment of ALS to emerge from The Project ALS Therapeutics Core at Columbia (THE CORE). Prosetin is an orally administrable, brain penetrant, small molecule shown in multiple laboratory models of ALS to block MAP4K, a specific protein kinase that several leading academic researchers & drug companies have identified as a key player in ALS.

As of August 2021, Project ALS has obtained permission from the Food & Drug Administration to start human studies of prosetin and is initiating a Phase I clinical trial in healthy volunteers and ALS patients.

What is a Phase I clinical trial?

In a Phase I clinical trial, a new investigational treatment like prosetin is given to people for the first time. In a Phase I study, initial participants receive one very low dose of the drug. If there are no serious side effects, the drug may be given at higher doses, and for longer periods of time, until doctors find a dose that's likely to be effective without causing harm.

By the end of Phase I study, we hope to establish the optimal dose for longer-term studies—how much drug can be given without causing serious side effects, and how the drug behaves in a person's body.

What will happen in prosetin's Phase I clinical trial?

We plan to study prosetin in a three-part Phase I trial:

- (1a) A single ascending dose study, where healthy volunteers receive one dose of prosetin at different dose levels and are watched to be sure there are no serious side effects,
- (1b) A multiple ascending dose study, where healthy volunteers receive several doses of prosetin at different dose levels and are observed to be sure there are no serious side effects,
- (1c) A multiple dose study where people with ALS receive several doses of prosetin at the level that seemed optimal in healthy volunteers. In Part 1c, we will also test to be sure that prosetin is crossing the blood-brain barrier, and that it is blocking MAP4K, in people with ALS.

This study's planned *hybrid design*—testing prosetin in both healthy volunteers and people with ALS—will allow us to more rapidly learn how prosetin behaves in people, and to confirm the best dose for continued ALS clinical trials.

It is important to note that Phase I clinical trials often change in real-time. Sometimes, the optimal dose will be found faster than expected, while sometimes, additional doses must be studied. Thus, while we will move as quickly as responsibly possible and keep you closely updated via our website and [email list](#), we are not projecting a specific timeline for this study.



I have ALS—how do I participate in prosetin’s Phase I clinical trial?

The ALS patient portion of prosetin’s Phase I trial will take place at Columbia University Medical Center in New York City. This portion of the trial will not begin until the healthy volunteer portion is completed. To receive more information about this trial, please email info@projectals.org with the subject “Prosetin Phase I ALS Patient Volunteer”