

Request for Proposals of Investigational Products for the HEALEY ALS Platform Trial

The Sean M. Healey & AMG Center for ALS at Mass General is seeking applications of experimental therapies for inclusion in the HEALEY ALS Platform Trial. While applications are always accepted on a rolling basis, to decide the next investigational products to launch, we are looking for partners interested in starting their regimen in 2022 or early 2023 to send an application in by June 1st, 2022. Launched in July 2020, the HEALEY ALS Platform Trial is a phase 2/3 multicenter, double blind, placebo controlled, perpetual, adaptive platform trial testing multiple regimens. For more information about the HEALEY ALS Platform Trial, please see our recent publication (<https://doi.org/10.1002/ana.26285>).

The trial incorporates several design and operational innovations:

1. Increased power through the use of sharing of data from participants assigned to placebo across treatment regimens
2. More rapid entry of treatment regimens into the ongoing platform trial
3. Planned interim analyses
4. A Bayesian primary analysis model for proportional slowing of disease progression as measured by ALSFRS-R total score
5. Innovative statistical modelling to account for effects of time and administration in placebo participants
6. Inclusion of biomarkers and novel outcome measures to improve understanding of disease progression
7. Active engagement and interest of ALS community

Participants are randomized in two stages: first to a treatment regimen, where a regimen is defined as both an active treatment and the corresponding randomized placebo, followed by 3:1 randomization to either the active treatment or placebo within each regimen. The recommended sample size for each regimen is 160 (120 on active and 40 on placebo). Each regimen includes an Open Label Extension (OLE) so that participants who complete participation in the placebo-controlled trial have the option to receive active treatment long term. The current full protocol can be found [here](#).

We encourage applications for investigational products from the academic and industry community to be considered for inclusion in the HEALEY ALS Platform trial.

Review Criteria:

- Relevance of target to human disease
- Pre-clinical data to support target and therapy
- Clinical trial readiness (availability of compound and placebo, IND, previous human trial experience as applicable)
- Availability of relevant biomarkers

Time line for Submission:

Release Date:	Friday April 1, 2022
Informational Webinar:	Thursday, April 14, 2022 05:30 PM EDT
Application Due Date:	Wednesday, June 1, 2022

Informational Webinar:

We invite you to register and participate in an upcoming informational webinar on Thursday, April 14, 2022 05:30 PM Eastern Time to learn about the platform trial and the therapy application process.

Please use this link to register:

https://partners.zoom.us/webinar/register/WN_0pLHK1vHTI2zj9vCQfwNyg

Please contact us at healeycenterforals@mgc.harvard.edu for any inquiries.

Industry partners are expected to provide investigational product, matching placebo and funding support for their treatment regimen. There may be resources available from Healey Center and foundation partners to help lower total costs for selected companies.

Confidential Disclosure Agreements (CDAs):

Confidential Disclosure Agreements (CDAs) are executed between MGH (on behalf of the Review Committee members) and the institution or company before the ALS Platform Trial Therapy Application Form is submitted.

Click here for [CDA template](#) and submit completed CDA form to

healeycenterforals@mgc.harvard.edu

ALS Platform Trial Therapy Application:

To access the application form please click here [ALS Platform Trial Therapy Application Form](#) and submit your completed application to healeycenterforals@mgc.harvard.edu

Learn more about Healey & AMG Center, NEALS Consortium, Tackle ALS and how to partner with the HEALEY ALS Platform Trial:

<https://www.massgeneral.org/als/healeycenter/>

<https://www.neals.org/>

<https://www.tackleals.com/>

<https://www.massgeneral.org/neurology/als/research/research-partners>