Healey & AMG Center
Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital

HEALEY ALS Platform Trial Therapy Application Form

For drug candidates to be considered for clinical trial phase 2/3 development. Send Completed form to: HealeyAMGCenterforALS@mgh.harvard.edu.

Date of Submission:

Title:

Principal Investigator(s): *(academic and industry)* **Collaborators:**	

Company Name:
Company Address:
Contact Person:
Email:
Phone:

Investigational Drug
Name:
Class/mechanism of action:
Molecule type: ☐ Small molecule ☐ Biologic
If biologic, please describe briefly including route of administration (1 sentence)

What is the current clinical development status of the investigational drug?
Please describe briefly (1 sentence)

Was it previously FDA approved for another disease indication (repositioning / repurposing strategy)? ☐Yes ☐No
If yes, please describe briefly (1 sentence)

Funding
Company is expected to provide funding for the trial and supply investigational drug and matching placebo as well as small companion expanded access protocol.
Is funding available? ☐Yes ☐No
If no, please briefly describe stage of financing (1 sentence)

Timeline
When would you be ready for First Patient First Visit (FPFV)?

Briefly describe the relevance of the therapeutic target/pathway in ALS addressing the questions below: *(If additional space is needed you may attach additional pages, Maximum 1 page)*

- **Is there supportive evidence from human ALS and what is the source?**
  ☐Autopsy Tissue ☐Blood ☐CSF ☐Genetic Data ☐Other _____
- **Is there supportive evidence from animal models or other model systems?**

NEALS
TACKLE ALS "DONT BE SORRY LETS BEAT THIS"

Briefly describe relevant preclinical and/or clinical evidence used to support the therapeutic effects of the <u>investigational drug</u>, addressing the bullets below where applicable:
(It is important to include supportive experimental data-If additional space is needed you may attach additional pages, Maximum 3 pages)

<u>Preclinical</u>

- **Relevant *in vitro* or *in vivo* pharmacology data**
- **Which animal models were used for the preclinical evaluation?**
- **Describe alternative model systems if not tested in animal models (e.g., IPS model systems)**
- **Describe the route/timing of the intervention delivery/dosing**
- **Is there evidence that the investigational drug reached and engaged the target?**
- **Describe the relevant preclinical efficacy data**
- **Have the preclinical results been independently replicated?**

<u>Clinical data from healthy volunteers and/or subjects with ALS or related diseases</u>

- **Relevant pharmacokinetic data**
- **Relevant target engagement data**
- **Other relevant pharmacodynamic data**
- **Relevant clinical efficacy data**

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Platform Trial Considerations
(If additional space is needed you may attach additional pages, Maximum 2 pages)

Briefly address the following below:

- **Comment on timing of availability of drug, placebo and IND status**
- **Dose selection rationale**
- **Clinical Data at the intended dosage (safety/tolerability)**
- **Primary and Secondary Goals of the trial**
- **What will result in go/no-go decisions for a future phase III efficacy trial?**

Participant selection criteria
Please describe briefly

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Is the intervention most likely to be effective in a selected population of people with ALS? If so, please specify and provide rationale.

Describe the choice of any general or investigational drug-specific biomarkers/outcome measures to evaluate target engagement and pharmacodynamic effects in the trial. Briefly describe the assay to be included and any validation data supporting the assay.
(If additional space is needed you may attach additional pages, Maximum 1 page)

Additional information (optional) **please list applicable references for your proposal here**:

**Please attach any additional pages as a single document.*

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