



## 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](mailto:healeyamgcenterforals@mgh.harvard.edu)

<b>Date of Submission:</b>	
<b>Title:</b>	
<b>Principal Investigator(s):</b> ( <i>academic and industry</i> )	
<b>Collaborators:</b>	
<b>Company Name:</b>	
<b>Company Address:</b>	
<b>Contact Person:</b>	
<b>Email:</b>	
<b>Phone:</b>	
<b><u>Investigational Drug</u></b>	
<b>Name:</b>	
<b>Class/mechanism of action:</b>	
<b>Molecule type:</b> <input type="checkbox"/> Small molecule <input type="checkbox"/> Biologic	
<i>If biologic, please describe briefly including route of administration (1 sentence)</i>	
<b>What is the current clinical development status of the investigational drug?</b>	
<i>Please describe briefly (1 sentence)</i>	
<b>Was it previously FDA approved for another disease indication (repositioning / repurposing strategy)?</b> <input type="checkbox"/> Yes <input type="checkbox"/> No	
<i>If yes, please describe briefly (1 sentence)</i>	
<b><u>Funding</u></b>	
<b>Company is expected to provide funding for the trial and supply investigational drug and matching placebo as well as small companion expanded access protocol.</b>	
<b>Is funding available?</b> <input type="checkbox"/> Yes <input type="checkbox"/> No	
<i>If no, please briefly describe stage of financing (1 sentence)</i>	
<b><u>Timeline</u></b>	
<b>When would you be ready for First Patient First Visit (FPFV)?</b>	
<b>Briefly describe the relevance of the therapeutic target/pathway in ALS addressing the questions below:</b> ( <i>If additional space is needed you may attach additional pages, Maximum 1 page</i> )	
<ul style="list-style-type: none"> <li>• <b>Is there supportive evidence from human ALS and what is the source?</b> <input type="checkbox"/> Autopsy Tissue <input type="checkbox"/> Blood <input type="checkbox"/> CSF <input type="checkbox"/> Genetic Data <input type="checkbox"/> Other _____</li> <li>• <b>Is there supportive evidence from animal models or other model systems?</b></li> </ul>	

**Briefly describe relevant preclinical and/or clinical evidence used to support the therapeutic effects of the investigational drug, 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)*

**Preclinical**

- **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?**

**Clinical data from healthy volunteers and/or subjects with ALS or related diseases**

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

*HEALEY ALS PLATFORM TRIAL THERAPY APPLICATION FORM*

If you need assistance completing this form, please contact [healeyamgcenterforals@mgh.harvard.edu](mailto:healeyamgcenterforals@mgh.harvard.edu)

**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*

**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.*