Healey & AMG Center

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

ALS MyMatch Therapy Application Form

For investigational product candidates to be considered for clinical trial phase 1b/2a development

Please send Completed form to: healeyamgcenterforals@mgh.harvard.edu

Key Considerations:

- Only Phase 1b/2a biomarker-driven or dose finding studies in ALS are suitable for ALS MyMatch.
- Only 3-6 month long treatment duration trials are best fit for ALS MyMatch
- This program is not suitable for Phase 1 healthy volunteer studies.
- Please consider applying to the HEALEY ALS Platform trial if the primary objective is a Phase 2b/3 clinical efficacy trial in ALS: ALS Platform Trial Therapy Application Form

Date of Submission:

Title:

Principal Investigator(s): (academic or industry)

Other Key Personnel:

Company/Institution Name and Address:

Contact Person:

Email:

Phone:

Investigational Drug/Device Name:

Therapeutic Class: ☐ Small molecule ☐ Cell Based Therapy ☐ Monoclonal Antibody ☐ Antisense Oligonucleotide/RNAi Therapeutic ☐ Gene Therapy ☐ Device

Was IP previously FDA approved for another disease indication (repositioning / repurposing strategy)? ☐Yes ☐No

If yes, please describe briefly (1 sentence):

Primary Objective(s) for the MyMatch trial (Check all that are applicable):

☐ Target Engagement ☐ Dose Finding ☐ Pharmacokinetics or PK/PD modeling ☐ CNS Penetrance ☐ Other

Route of Administration (1 sentence):

Mechanism of action:

Name of the drug/mechanism-specific target engagement biomarker, if identified and available to use:

Current Clinical development status:

Regulatory Status (Do you hold or have you applied for an IND/IND exemption/IDE for this protocol? Please outline regulatory agency interactions within U.S and outside of U.S for this IP and Orphan Drug designation status, if any):

Future Development and Commercialization plan (ALS and other indications):

Manufacturing and IP Status, including cGMP status, IP handling, IP procurement (for repurposing projects):

[Note: For all clinical trials, the Company/Academic group is required to supply investigational drug, device and matching placebo as applicable for the project]

Funding

For the first 2-3 industry/academic investigator-initiated partnerships, the Healey & AMG Center will provide partial funding support to coordinate and run the trial. MyMatch Trial Partner is expected to cover any additional costs. For subsequent trials, the Healey & AMG Center will assist with some infrastructure costs and the Company is expected to fund all other costs.

Is funding available? ☐Yes ☐No

If no, please briefly describe stage of financing:

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:

Is there supportive evidence from human ALS and what is the source?

☐ Prior ALS trial ☐ Autopsy Tissue ☐Blood ☐CSF ☐Genetic Data ☐Other

Is there supportive evidence from animal models or other model systems?

Maximum 3 pages including:

MyMatch Trial Design Synopsis

Brief outline specific aims and intended preliminary trial design (Note: ALS MyMatch Design Consultation Team will collaborate on developing and finalizing study design)

- Intended treatment duration to achieve primary and key secondary objectives
- Participant selection criteria and if any biomarker or enrichment criteria requirement for trial entry
- Dosage(s) selection rationale
- What will result in go/no-go decisions for a future clinical trial testing?

Briefly describe relevant preclinical and/or clinical preliminary evidence used to support the therapeutic effects of the investigational drug and the relevant biomarkers to either select best candidate participants and/or assess target engagement, addressing the bullets below where applicable :

(It is important to include supportive experimental data)

Preclinical

- Relevant *in vitro* or *in vivo* pharmacology data
- Describe results in any applicable animal models 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?

Relevant preclinical target biomarker data that can be used for selection of participants and/or assessing pharmacodynamic effects in proposed clinical trial

- Describe the relevant preclinical efficacy data
- Have the preclinical results been independently replicated?

Clinical evidence from healthy volunteers and/or people living with ALS or related diseases if available

- Relevant pharmacokinetic data
- Relevant target biomarker data if available
- Other relevant biomarker data that might inform on best selection of participants