

WE WILL BEGIN SHORTLY

- **To submit text questions to today's presenters, please type your questions into the 'Q&A' box**
- **Today's presentation is being recorded and will be posted on the Healey & AMG Center for ALS website**

<https://www.massgeneral.org/neurology/als/news/webinars>

HEALEY ALS Platform Trial

Weekly Q&A – Nov 12, 2020



AGENDA

- **Updates**

- *Enrollment*
- *Supplements and other interventions*
- *Patient Navigator*

YOUR QUESTIONS

➤ Accelerating ALS Therapy Development

Traditional



| | Intervention |
|---------|--------------|
| Disease | Treatment A |



Platform

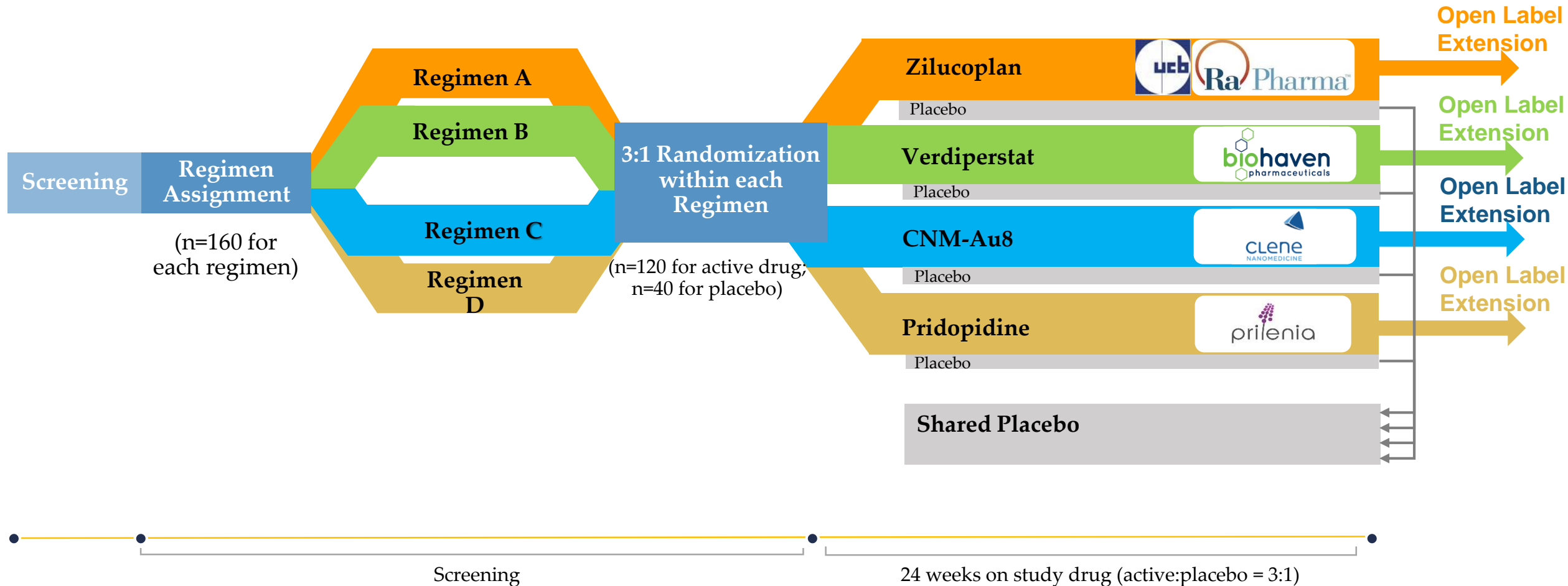


| | Intervention | | | |
|---------|--------------|-------------|-------------|-------------|
| Disease | Treatment A | Treatment B | Treatment C | Treatment D |

Perpetual Adaptive Trial

Randomization Ratio 3:1; Shared Placebo

Open Label Extension offered

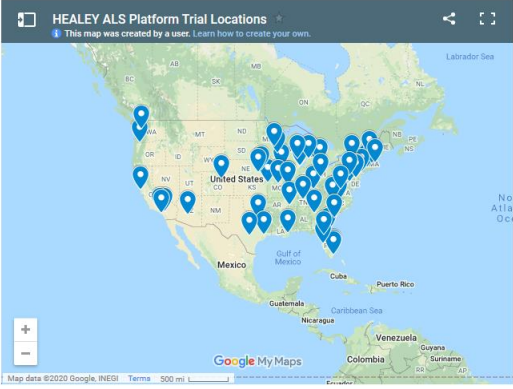


How to Find a Center Near You

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites



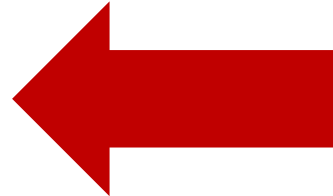
HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

List of Participating Sites by State

| Site | State | Contact Information |
|-------------------------------|---------|-------------------------------|
| Barrow Neurological Institute | Arizona | Jessie Duncan |

- * 29 sites are actively enrolling
- * A total of 49 sites are already IRB approved
- * Goal is to activate the other sites soon and add more in early 2021!

Contact Info of Participating Sites by State



<https://www.massgeneral.org/neurology/als/research/platform-trial-sites>

Enrollment Updates (as of Nov 12, 2020)

- **190** individuals with ALS signed informed consent
- **139** individuals were assigned to a regimen
- **113** are currently receiving study drug (active or placebo)

We will continue to update the ALS community on enrollment (website, webinars)

FAQ:

Which supplements/interventions are exclusionary?

Guiding Principles:

- Any supplements/medications that are in a trial are considered exclusionary (as they might affect the efficacy of the treatment under evaluation)
 - Additional supplements/medications might be exclusionary based on safety and interactions with the treatment under evaluation
 - Examples of exclusionary agents: TUDCA, sodium phenylbutyrate, curcumin, high doses of methylcobalamin
-
- Most trials have a list of exclusionary supplements/medications but each trial has a different list
 - The list might change as new trials open and others end
 - Note: a washout period prior to Master Protocol Screening is required (30 days or 5 half-lives if known, whichever is longer)

FAQ:

Which supplements/interventions are exclusionary?

Stem Cells

- Prior use of stem cells via intrathecal or intravenous administration is allowed after appropriate wash-out
- Any prior use of stem cells via injection into the brain or spinal cord is exclusionary

Treatment for familial ALS

- Prior use of antisense oligonucleotides is allowed after appropriate wash-out
- Any prior exposure to gene therapies is exclusionary

- The following interventions are **allowed** within dosing limits or for indications as described.
- If taking higher dosages prior to Master Protocol Screening, washout is **not** required. However, we ask the participant to agree to reduce dosages to remain within the limits outlined below

Acetyl-L-Carnitine (at dose of 3 grams or less per day)

Basis (nicotinamide riboside and pterostilbene) (at dose of 2 capsules or less per day)

Deanna's protocol (no dosing limit)

Inspiratory and expiratory muscle training (no dosing limit)

L-serine (at dose of 4 grams or less per day)

Lithium if prescribed for bipolar disease (no dosing limit)

Mexiletine if prescribed for muscle cramps at dose of 900mg or less per day

Nicotinamide (at dose of 500mg or less per day)

Cannabinoids such as medical marijuana, hemp, cannabis and other CBD containing products (no dosing limit)

Patient Navigator

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For More Updates

- **Weekly informal Q&A + monthly webinars**

Find the schedule, registration links, slide decks and recordings on our website

<https://www.massgeneral.org/neurology/als/news/webinars>

- **Coming up- webinars about each drug's mechanism of action and science**