

HEALEY ALS Platform Trial

Weekly Q&A – Oct 8, 2020

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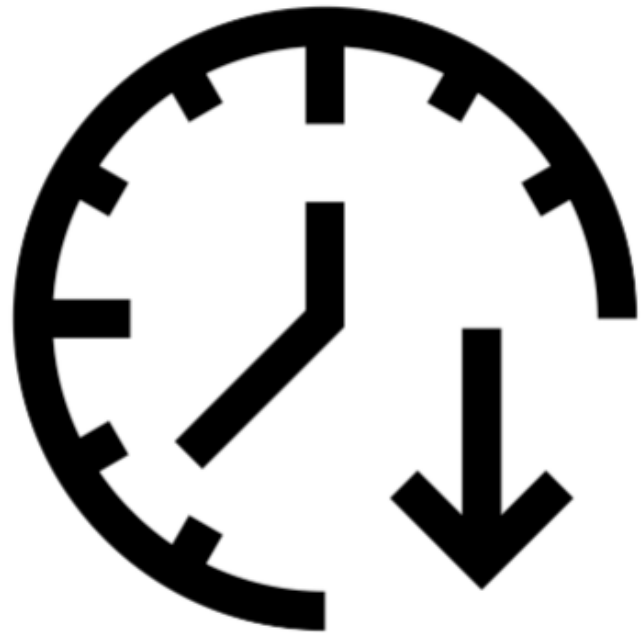
AGENDA

- **Brief review of the HEALEY ALS Platform Trial**
- **Updates**
 - *Enrollment*
 - *Study Treatments*
 - *Sites*
- **How to stay in touch and find a site near you**
- ***This week's FAQ: exclusionary supplements/interventions***

YOUR QUESTIONS

HEALEY ALS Platform Trial

➤ **Accelerates ALS Therapy Development**



Traditional



	Intervention
Disease	Therapy A



Platform



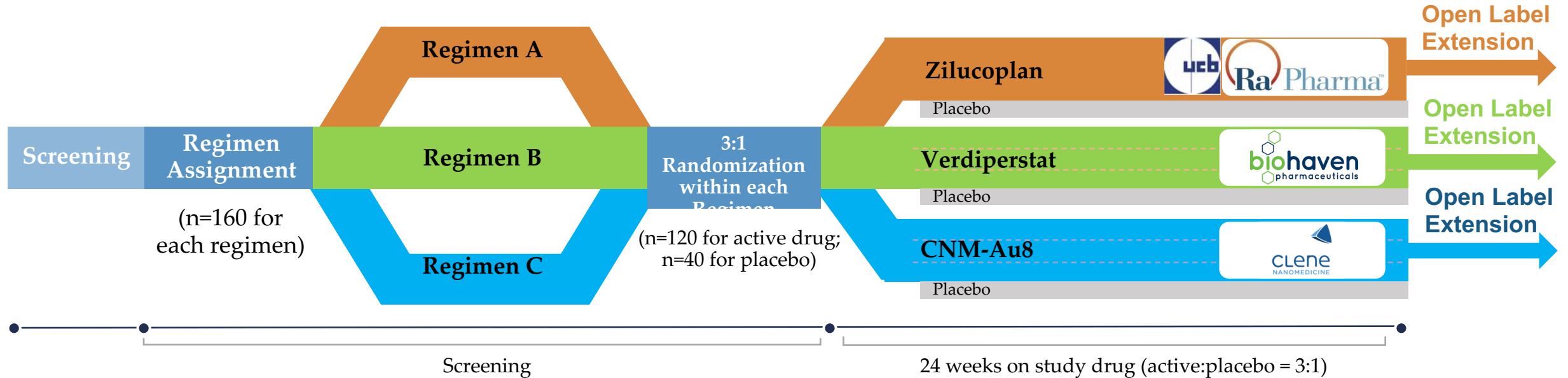
	Intervention		
Disease	Therapy A	Therapy B	Therapy C

Perpetual adaptive trial- Open Label Extension offered



Healey Center

Sean M. Healey & AMG Center
for ALS at Mass General



- Treatments were selected by ALS scientists and experts based on a competitive process
- The platform opened with the first 3 treatments – enrollment started in July 2020
- The next treatment will be added soon (pridopidine by Prilenia)
- We are in discussion with 3 companies for 2021!

Enrollment Updates

- **96** individuals with ALS signed informed consent
- **48** are currently on study drug

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

Questions about enrollment? Please contact the Patient Navigator

Phone: 833-425-8257 (HALT ALS)

Email: healeyalsplatform@mgh.harvard.edu


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.

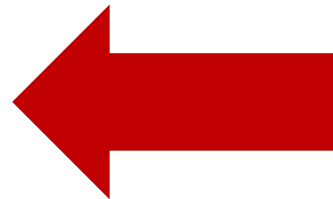


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

- 24 sites are actively enrolling!
- More will be added once the first 54 have been activated

Contact Info of Participating Sites by State




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

How to Receive Platform Trial Updates

Sign up for the ALS Link Newsletter (for info on this trial and other research opportunities)

Not sure which trial might be right for you? The Healey Center for ALS offers coordination services to help you understand your options and make the best choice for your individual situation.

[Introduction to Clinical Trials](#)



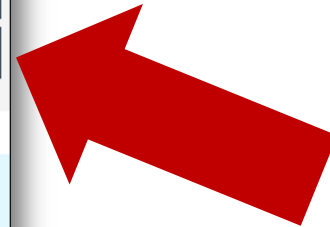
[Enrolling ALS Trials & Studies](#)

[ALS News and Publications](#)

[Sign Up for ALS Link](#)

ALS Platform Trial

The Healey Center is launching the first adaptive ALS Platform Trial to bring novel ALS therapies to patients more quickly.



<https://www.massgeneral.org/neurology/als/services/clinical-trials-enrollment-coordination>

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, methylcobalamin but only at very high doses
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- 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)