HEALEY ALS Platform Trial

Weekly Q&A – Oct 8, 2020

Sabrina Paganoni, MD, PhD
Merit Cudkowicz, MD, MSc
Sandy Morris - IAMALS

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

Northeast Amyotrophic Lateral Sclerosis Consortium

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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
<table>
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<th>Intervention</th>
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<td>Disease</td>
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**Traditional**

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<th>Intervention</th>
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<tr>
<td>Disease</td>
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<td>Therapy A</td>
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**Platform**

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Perpetual adaptive trial-
Open Label Extension offered

- 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

- 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)

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

→ 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)