HEALEY ALS Platform Trial

Weekly Q&A – Nov 19, 2020

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

Northeast Amyotrophic Lateral Sclerosis Consortium

Tackle ALS
ALS Association

ALS ONE
MDA
Muscular Dystrophy Association

ALS FINDING a CURE

I AM ALS

SALSA

RUN 2 REVIVE

The Arthur M. Blank Family Foundation
AGENDA

- Updates
  - Enrollment
  - Sites
  - Patient Navigator

YOUR QUESTIONS
Accelerating ALS Therapy Development

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<th>Platform</th>
<th>Intervention</th>
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<td>Disease</td>
<td>Treatment A</td>
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Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension offered

Screening
Regimen Assignment
(n=160 for each regimen)

3:1 Randomization within each Regimen

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

Regimen D
(n=120 for active drug; n=40 for placebo)

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Screening
24 weeks on study drug (active:placebo = 3:1)

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension
How to Find a Center Near You

* 30 sites are actively enrolling

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites
List of enrolling sites as of Nov 19, 2020
30 sites are enrolling

- Texas Neurology
- MGH
- UTHSCSA
- Hospital for Special Care
- Holy Cross Hospital
- Thomas Jefferson
- Houston Methodist
- Henry Ford Health System
- Barrow Neurological Institute
- Ohio State University
- Northwestern University
- University of Chicago
- Wake Forest
- University of Nebraska Medical Center
- Loma Linda

- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- Forbes Norris MDA/ALS Research Center, CPMC
- Penn State Hershey
- UMass
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- University of South Florida
- Columbia University
- University of Virginia
Enrollment Updates (as of Nov 19, 2020)

- 201 individuals with ALS signed informed consent
- 148 individuals were assigned to a regimen
- 123 are currently receiving study drug (active or placebo)

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

Catherine Small
Allison Bulat

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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**