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 – Dec 3, 2020

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

Northeast Amyotrophic Lateral Sclerosis Consortium

HARVARD MEDICAL SCHOOL

MASSACHUSETTS GENERAL HOSPITAL

NEALS

NEUROLOGICAL CLINICAL RESEARCH INSTITUTE
BARROW Neurological Institute

UCB Pharma
biohaven pharmaceuticals
Clenie Nanomedicine
prilenia

Berry Consultants

The Arthur M. Blank Family Foundation

Tackle ALS

ALS ASSOCIATION

MIDA
Muscular Dystrophy Association

alsFINDINGaCURE

SALSA

ALS ONE

RUN2REVIVE
AGENDA

• Updates on the HEALEY ALS Platform Trial
  - Study Treatments
  - Sites
  - Enrollment

• How to stay in touch and find a site near you

YOUR QUESTIONS
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension offered

Screening
Regimen Assignment
(n=160 for each regimen)

Regimen A
Regimen B
Regimen C
Regimen D

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

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

Screening
24 weeks on study drug (active:placebo = 3:1)
How to Find a Center Near You

33 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 Dec 3, 2020
33 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
✓ Emory
✓ University of Maryland
✓ SUNY Upstate
Enrollment Updates (as of Dec 3, 2020)

• 214 individuals with ALS signed informed consent
• 170 individuals were assigned to a regimen
• 143 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

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu
For More Updates

• **Weekly webinars**
  The idea of came from our Patient Advisory Committee: we are excited to be talking with you on a weekly basis and take any questions you might have

• **Find the schedule and registration links on our website**

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

  **Coming up: webinars about each drug’s mechanism of action and science**