HEALEY ALS Platform Trial

Weekly Q&A – Sept 23, 2021

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

[Logos of various organizations associated with HEALEY ALS Platform Trial]
Guest Speaker

Namita Goyal, MD
University of California Irvine, CA
Platform Trial Site Investigator
University of California, Irvine

UCI Health
About UC Irvine Health

- Only academic medical center in Orange County
- Level I Trauma Center
- Serve population of 3.4 million people (catchment area > 5 million)
- Diverse patient population in ethnic backgrounds, socioeconomic classes and disease variety
ALS Neuromuscular center (overview)

• Nationally Recognized: ALS Certified Center of Excellence
• Comprehensive multidisciplinary clinics
• Electrodiagnostic lab - state of the art
  EMG, single fiber EMG, quantitative sensory testing and autonomic testing
• High volume of Muscle and Nerve histopathology- weekly conferences
• Cutting edge Clinical Trials in a number of Neuromuscular Disorders:
  ALS, Myasthenia gravis, Gene therapy, Myositis
ALS Neuromuscular Faculty

Tahseen Mozaffar, MD
*Director of Center*

Namita Goyal, MD
*Director of NM Fellowship*
*Co-Director of Center*
*Director of ALS Clinic*

Ali Habib, MD
*Director of EMG Lab*

Manisha Korb, MD

Trained from:
*Wash U*
*MGH/Harvard*
*Columbia*
*Univ of Chicago*
Neuromuscular Junior Attendings

Jeff Mullen, MD

Shadi Milani, DO
ALS Center at UC Irvine

• ~250 ALS patients seen in our Center
• Patients seen in Multidisciplinary Team
  • Respiratory, Physical therapy, Speech therapy, RN, Social worker
• Offered cutting edge diagnostic studies
  • including genetic testing
• Opportunity for Clinical trials
Research Team

Cutting edge Clinical Trials
• Oral drugs
• IV infusions
• Stem cell therapy
• Gene therapy
Questions?

Program Director
Namita Goyal, MD namitag@hs.uci.edu

ALS Clinical Trial Coordinator
Jeanette Overton jtoverto@hs.uci.edu
Thank you
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening

Regimen Assignment

(n=160 for each regimen)

Regimen A

Regimen B

Regimen C

Regimen D

3:1 Randomization within each Regimen

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

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Shared Placebo

Open Label Extension

Up to 6 weeks

24 weeks (about 6 months)

Informed Consent

Assign to Regimen

Randomize within Regimen (active/placebo)

Open Label Extension
Enrollment Updates (as of September 23, 2021)

• 761 individuals with ALS signed informed consent
• 603 individuals were assigned to a regimen
• 544 individuals were randomized within a regimen (active or placebo)
• 210 have entered the Open Label Extension (OLE)

Total randomized + Participants in screening

• 148 individuals were randomized within Regimen A = 161
• 163 individuals were randomized within Regimen B = 168
• 160 individuals were randomized within Regimen C = 161
• 73 individuals were randomized within Regimen D = 81

Thank You
This breakthrough trial would not be possible without your partnership
Patient Navigator

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Allison Bulat
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To see whether you might qualify, view the list of eligibility criteria:
Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites

Upcoming:
Oct 7th- No webinar this week
Oct 14th- David Walk, MD FAAN (University of Minnesota, MN)
In this webinar, Dr. Richard Bedlack (Duke ALS Clinic) and Dr. Merit Cudkowicz (Sean M. Healey & AMG Center for ALS at Mass General Hospital) discussed why research engagement by people living with ALS is important, examples of how it is helping, continued challenges, and plans for further improvements in the future.