Guest Speaker

Xiaowei (Bill) Su, MD, PhD
Penn State Health, PA
Platform Trial Site Sub-Investigator
Penn State Health M.S. Hershey ALS Clinic
• 220 active patients (80-100 new annually)
• 4 clinicians, 3 RNs

Penn State ALS Research Program
• One PI, 3 Sub-Is
• 3 Research Coordinators (2 Healey)
Penn State Healey Site Experiences

Healey has been exciting…
• Tremendous patient interest
• Highest enrollment in our ALS clinic & research program history

… And challenging
• Complex logistics & documentation compared to traditional clinical trials
• Ongoing assessment of how many research participants can be managed by each coordinator

Support from MGH and central monitors greatly appreciated
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 October 21, 2021)

- 815 individuals with ALS signed informed consent
- 646 individuals were assigned to a regimen
- 584 individuals were randomized within a regimen (active or placebo)
- 239 have entered the Open Label Extension (OLE)

- 158 individuals were randomized within Regimen A
- 167 individuals were randomized within Regimen B
- 161 individuals were randomized within Regimen C
- 98 individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your partnership
How to Find a Center Near You

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

*New Site*-University of Southern California

53 sites are actively enrolling
Plans for Expansion: 22 New Sites Coming Soon

Atrium Health
Cleveland Clinic
George Washington University
University of California, San Francisco
Yale University
Indiana University
Stony Brook University
Georgetown
University of Arkansas
University of Pittsburgh
University of Southern California
University of Utah
Augusta University
University of Cincinnati
Virginia Commonwealth University
Swedish Medical Center
Las Vegas Clinic
Kaiser, Los Angeles
Lehigh Valley Health Network
St. Alphonsus Regional Medical Center
Hackensack University
Essentia Health

Note: Only those sites highlighted in blue are actively enrolling at this time. Additional sites will be activated on a rolling basis.

as of 10/21/21
Patient Navigator

Catherine Small
Allison Bulat

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

• Biostatistics
• Get to know our sites

Upcoming Guest Speaker:
Oct 28th- Megan Miller, PhD (IAMALS Director of Science and Policy)