

HEALEY ALS Platform Trial

Weekly Q&A – July 1, 2021



Healey Center

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



The AMG Foundation

Guest Speaker



Jeffrey Rosenfeld, PhD, MD, FAAN **Loma Linda University, CA** **Platform Trial Site Investigator**

- Professor and Associate Chairman Department of Neurology
- Director, Neuromuscular & ALS/MND Programs
- Medical Director, Center for Restorative Neurology

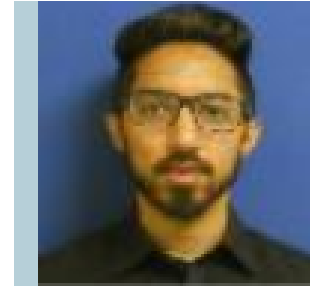




CRN at LLUH Platform Trial Research Team



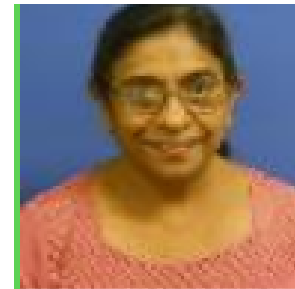
Sedano, Josie



Qasim, Syed



Borg, David



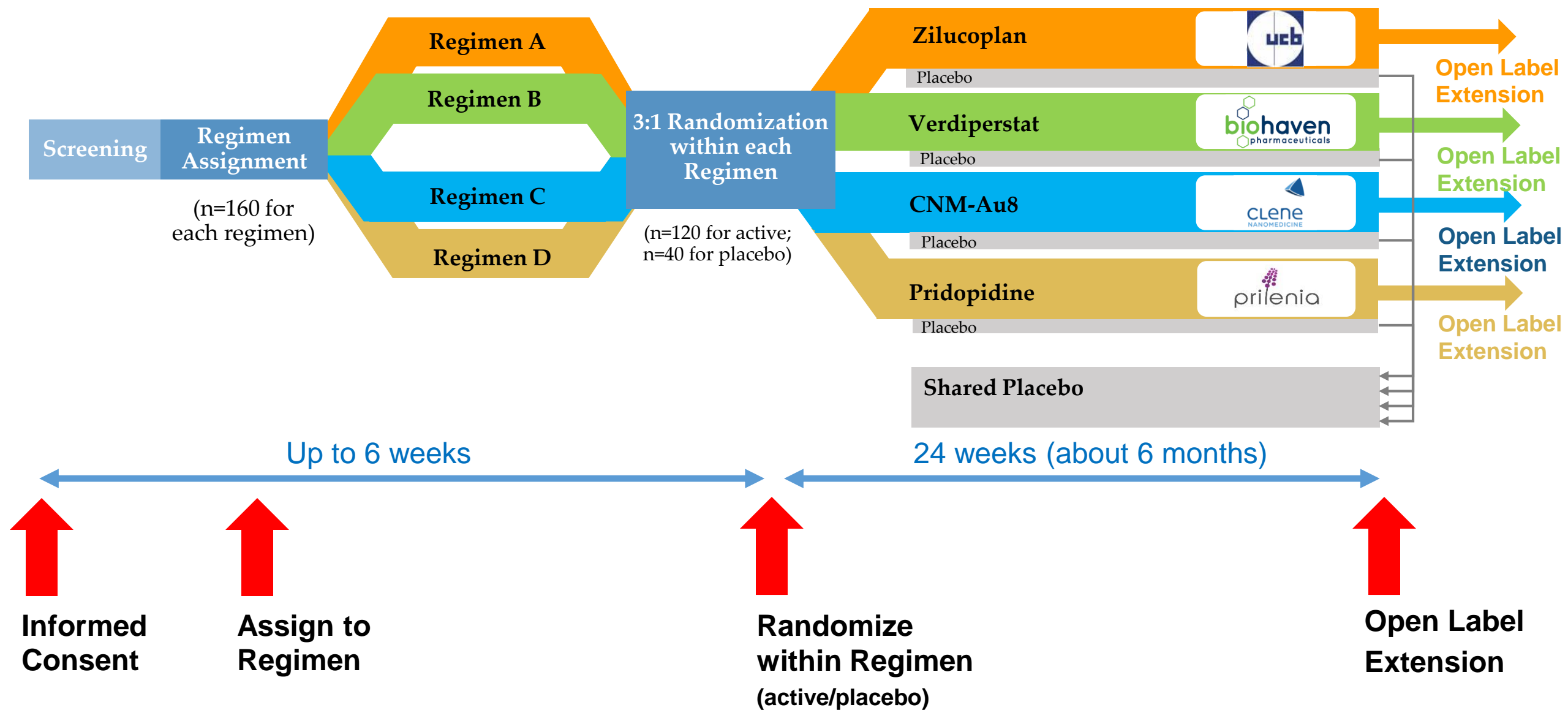
Baroya, Sheba S.



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of July 1, 2021)

- **627** individuals with ALS signed informed consent
- **492** individuals were assigned to a regimen
- **444** individuals were randomized within a regimen (active or placebo)
- **135** have entered the Open Label Extension (OLE)

- **120** individuals were randomized within Regimen A
- **138** individuals were randomized within Regimen B
- **139** individuals were randomized within Regimen C
- **47** individuals were randomized within Regimen D

Thank You

This breakthrough trial would not be possible without your partnership

Patient Navigator



Catherine Small

Allison Bulat

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To see whether you might qualify, view the list of eligibility criteria online:

<https://bit.ly/3p4PrLL>

Send us webinar ideas!

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



Upcoming Guest Speakers:

July 15th- Shafeeq Ladha, MD (Barrow Neurological Institute, AZ)

July 22nd- Richard Bedlack, MD, PhD, MS (Duke University, NC)

Aug 12th- Michael Weiss, MD, FAAN (University of Washington, WA)

Aug 19th- Laura Foster, MD (University of Colorado, CO)

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/>

Previously: Drug mechanism of action and science webinars

Jan 21- Prilenia/Pridopidine (view recording- <https://bit.ly/3ad3qel>)

Feb 4 - Clene/CNM-Au8 (view recording- <https://bit.ly/3jB3WWt>)

Feb 18- Biohaven/Verdiperstat (view recording- <https://bit.ly/301qdDR>)

Feb 25- UCB/Zilucoplan (view recording- <https://bit.ly/3b3cpyS>)