

HEALEY ALS Platform Trial

Weekly Q&A – July 15, 2021



Guest Speaker

Shafeeq Ladha, MD
Barrow Neurological Institute, AZ
Platform Trial Site Investigator



Barrow ALS Research Team

- ALS Clinical Team
- Clinical Research Unit

- ALS Scientists



- BNI CRO



Barrow Healey Platform Trial Team



Nicole Turcotte



Jessie Duncan



Dianna Thiessen



Arubah Ahmed



Bill Jacobsen

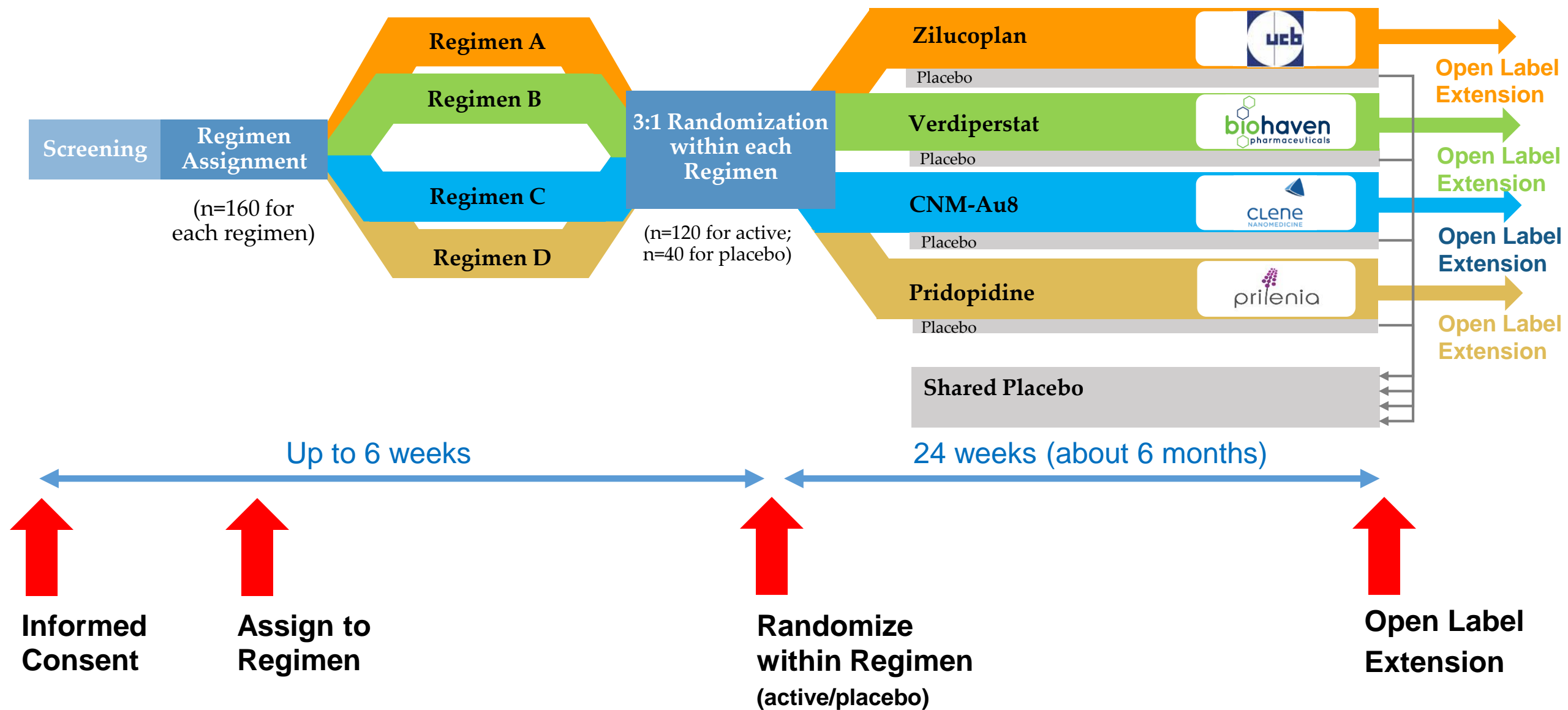


Shafeeq Ladha

Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of July 15, 2021)

- **651** individuals with ALS signed informed consent
- **504** individuals were assigned to a regimen
- **458** individuals were randomized within a regimen (active or placebo)
- **139** have entered the Open Label Extension (OLE)

- **124** individuals were randomized within Regimen A
- **143** individuals were randomized within Regimen B
- **141** individuals were randomized within Regimen C
- **50** individuals were randomized within Regimen D

Thank You

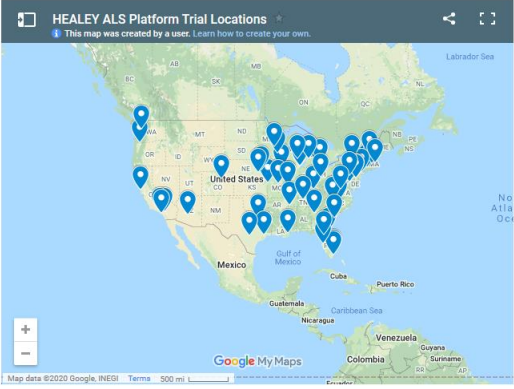
This breakthrough trial would not be possible without your partnership

How to Find a Center Near You

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites



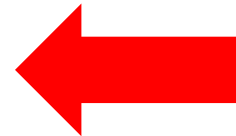
HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

52 sites are actively enrolling

Contact Info of Participating Sites by State



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

Patient Navigator



Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu



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

July 29th- James Berry, MD, MPH (Mass General Hospital, MA)

Hristelina Ilieva, MD, PhD (Thomas Jefferson, PA)

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>)