

HEALEY ALS Platform Trial

Weekly Q&A – Aug 12, 2021



Healey Center

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



The AMG Foundation



Guest Speaker

Michael Weiss, MD, FAAN
University of Washington, WA
Platform Trial Site Investigator



University of Washington Platform Trial Team

- Michael Weiss, MD (site PI)
- Nassim Rad, MD (sub-I)
- Priyank Patel, MD (sub-I)
- Laura Sissons-Ross (senior research coordinator)
- Drew Tschida (research coordinator)
- Anika McManamen (research assistant)
- Wen Pei Ridenour RN, BSN (ALS nurse coordinator)



Notable and Unique Challenges at UW

- COVID19 Protocol for Pulmonary Function Testing
 - Protocol created to permit coordinators to perform vital capacities
 - Requirements for N95 Fit testing and room sanitization requirements
 - So far, no restrictions in performing the testing
- Canadian Participants and Border Crossing
 - Several Canadians are participating in the study at our site
 - Medication transport across US-Canadian border
 - As long as the study drug is kept in original container (bottle or boxes), participants have been able to transport the medication

Letter to US Customs for Border Crossing

UW Medicine
SCHOOL OF MEDICINE

[DATE]

To U.S. Customs and Border Patrol,

[Subject Name], [Passport #], is a Canadian resident living with Amyotrophic Lateral Sclerosis (also known as ALS), a fatal progressive neurological disorder.

[He/She] is travelling to the University of Washington Medical Center in Seattle, WA, to participate in a clinical research trial designed to determine the effectiveness of therapies for people living with ALS, a disease that has only limited treatment at present. [Subject Name] will meet with myself, the Principal Investigator, and members of my research team on [Date].

IF NOT ENROLLED

This clinical trial is not available in Canada. ALS currently does not have any curative treatment and clinical trials are the only option for patients to receive potential therapeutics. [Subject Name] must leave and return to Canada regularly to receive essential medical services in the United States once enrolled.

IF ENROLLED

As [Subject Name] is already enrolled in this clinical trial, their visit is essential for continued medical monitoring and to avoid undue harm. This clinical trial is not available in Canada. [Subject Name] must leave and return to Canada regularly to receive essential medical services in the United States.

IF WITH CAREGIVER

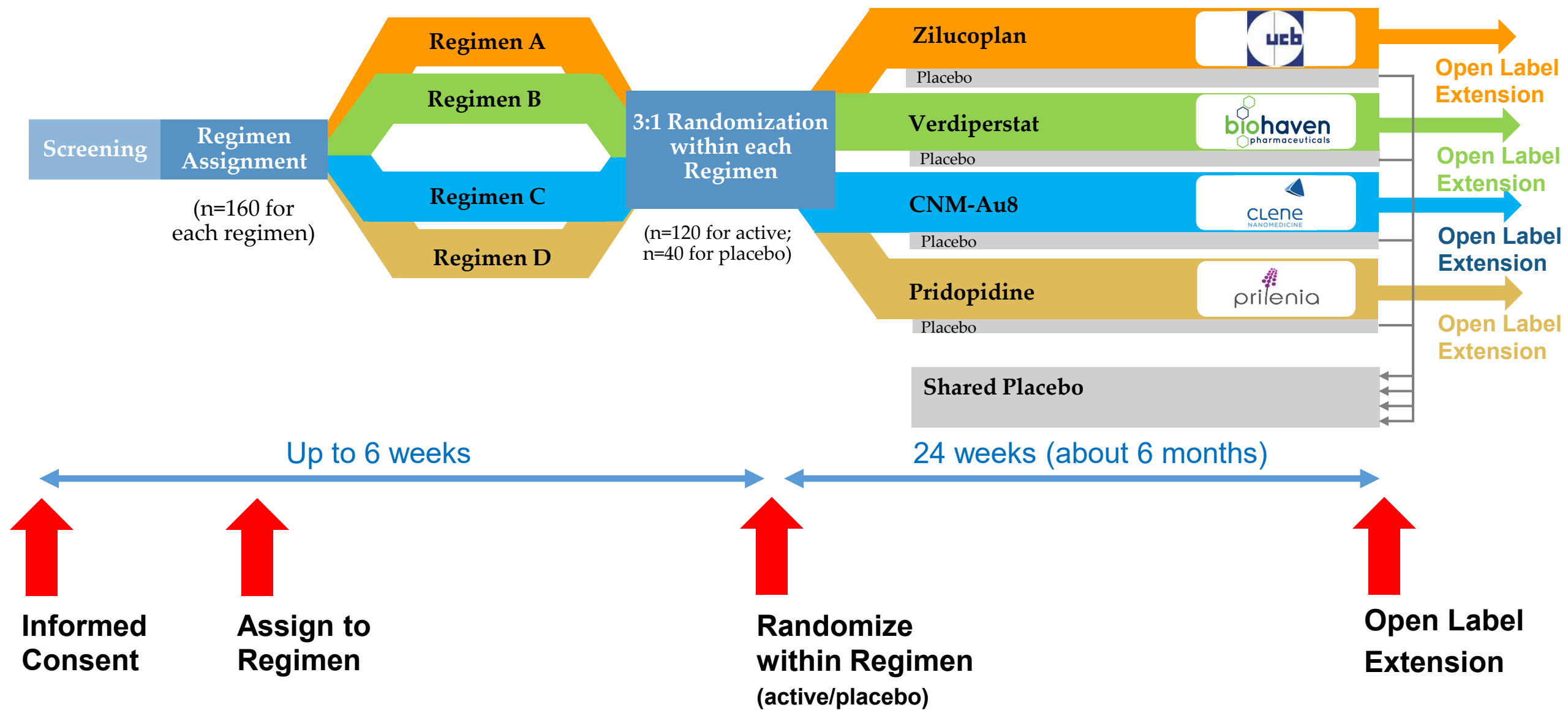
Due to [his/her] medical/physical condition, [Subject Name] will have a caregiver with [him/her]. [His/her] [Caregiver Name], [Passport #] is accompanying [him/her] for this journey. Caregiver presence is essential to [Subject's Name] participation in the trial.

[Subject Name and Caregiver Name] will be staying at [name of Accommodations]. They anticipate returning to Canada on [Date].

Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of August 12, 2021)

- **694** individuals with ALS signed informed consent
 - **551** individuals were assigned to a regimen
 - **505** individuals were randomized within a regimen (active or placebo)
 - **172** have entered the Open Label Extension (OLE)
- | | Total randomized +
Participants in screening |
|---|---|
| • 137 individuals were randomized within Regimen A | = 147 |
| • 153 individuals were randomized within Regimen B | = 156 |
| • 154 individuals were randomized within Regimen C | = 156 |
| • 61 individuals were randomized within Regimen D | = 65 |

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:

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



Send us webinar ideas!

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



Upcoming Guest Speakers:

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

Aug 26th- Niraja Suresh, MD (University of South Florida, FL)

Sept 2nd- Terry Heiman-Patterson, MD (Temple University, PA)

Sept 9th- Jennifer DiMartino (Executive Director, ALS ONE)

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 (recorded- <https://bit.ly/3ad3qel>)
- Feb 4 - Clene/CNM-Au8 (recorded- <https://bit.ly/3jB3WWt>)
- Feb 18- Biohaven/Verdiperstat (recorded- <https://bit.ly/301qdDR>)
- Feb 25- UCB/Zilucoplan (recorded- <https://bit.ly/3b3cpyS>)