

HEALEY ALS Platform Trial

Weekly Q&A – April 1, 2021



Guest Speaker

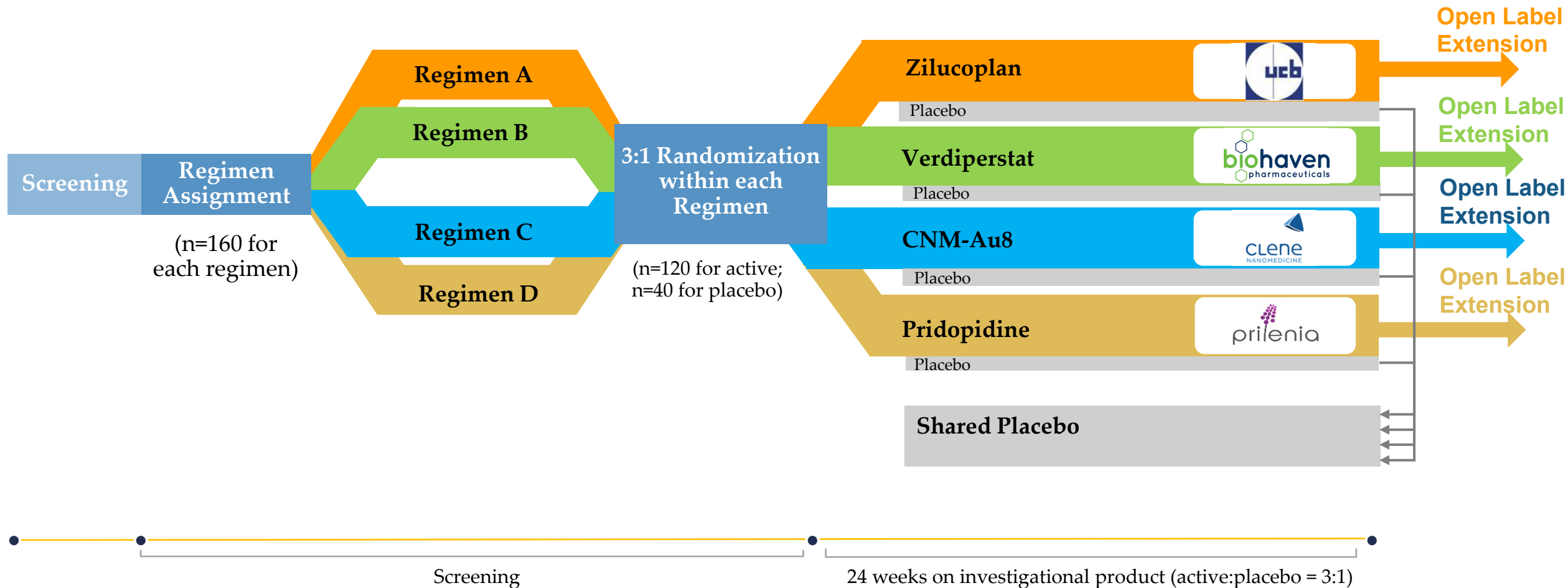
James Berry, MD, MPH
Mass General Hospital Boston, MA
Regimen co-Lead (Regimen C)



Perpetual Adaptive Trial

Randomization Ratio 3:1; Shared Placebo

Open Label Extension (OLE) offered



Endpoints

Primary Endpoint

Change in disease severity - **ALS Functional Rating Scale-Revised (ALSFRS-R)**

Secondary Endpoints

1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival
4. Treatment-specific biomarkers as applicable

Exploratory Endpoints

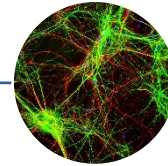
Safety Endpoints

Exploratory Endpoints

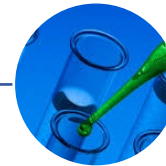
**Endpoint
Development
Engine**



DNA



Neurofilaments



Biomarkers (Blood, Urine, CSF)



Speech Analysis



Home Spirometry


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.

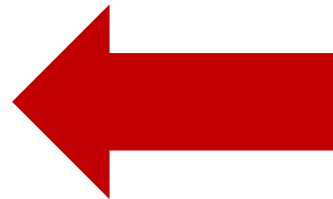


List of Participating Sites by State

| Site | State | Contact Information |
|-------------------------------|---------|-------------------------------|
| Barrow Neurological Institute | Arizona | Jessie Duncan |

49 sites are actively enrolling

Contact Info of Participating Sites by State



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

List of enrolling sites as of April 1, 2021

49 sites are enrolling

- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ UTHSCSA
- ✓ Hospital for Special Care
- ✓ Holy Cross Hospital
- ✓ Thomas Jefferson
- ✓ Houston Methodist
- ✓ Henry Ford Health System
- ✓ Barrow Neurological Institute
- ✓ Ohio State University
- ✓ Northwestern University
- ✓ University of Chicago
- ✓ Wake Forest
- ✓ University of Nebraska
- ✓ Loma Linda University
- ✓ University of Washington
- ✓ University of Iowa
- ✓ Washington University
- ✓ University of Pennsylvania
- ✓ University of Michigan
- ✓ California Pacific Medical Center
- ✓ Penn State Hershey
- ✓ UMass Worcester
- ✓ University of Miami
- ✓ University of Colorado
- ✓ Cedars-Sinai
- ✓ University of Florida
- ✓ University of South Florida
- ✓ Columbia University
- ✓ University of Virginia
- ✓ Emory University
- ✓ University of Maryland
- ✓ SUNY Upstate
- ✓ Beth Israel Deaconess
- ✓ Temple University
- ✓ Dartmouth-Hitchcock
- ✓ Medical College of Wisconsin
- ✓ Spectrum Health
- ✓ University of Missouri
- ✓ University of Minnesota
- ✓ Johns Hopkins University
- ✓ University of CA Irvine
- ✓ University of Kansas
- ✓ Vanderbilt University
- ✓ University of Kentucky
- ✓ Mayo Rochester
- ✓ Duke University
- ✓ Neurology Associates
- ✓ **Ochsner Health System**

Enrollment Updates (as of April 1, 2021)

- **404** individuals with ALS signed informed consent
- **313** individuals were assigned to a regimen
- **275** are receiving investigational product (active or placebo)
- **48** have entered Open Label Extension (OLE)

We will continue to update the ALS community on enrollment (website, webinars)

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

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

Patient Navigator



Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

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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](https://bit.ly/3ad3qel))

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

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

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



Upcoming ALS Webinar

The ALS Association/Northeast ALS Consortium Educational Webinar

ALS Drug Development Webinar Series Part 1: Scientific Considerations



Richard Bedlack, MD, PhD
Duke ALS Clinic



Jeremy Shefner, MD, PhD, FAAN
Barrow Neurological Institute

Wednesday, April 7, 2021
12:00 - 1:00 PM EDT

Drug Development NEALS Webinar Series

This series will include talks on the preclinical development of potential therapies, the regulatory pathway, the science of trials, the clinical trial site level overview, and the importance of patient engagement.

Part 1: Scientific Considerations

April 7th 12:00-1:00pm EDT

Part 2: Regulatory Considerations

April 9th 12:30-1:30pm EDT

Register at <https://bit.ly/3frQsfB>

Send us webinar ideas!

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

