

# HEALEY ALS Platform Trial

Weekly Q&A – April 8, 2021



## Healey Center

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

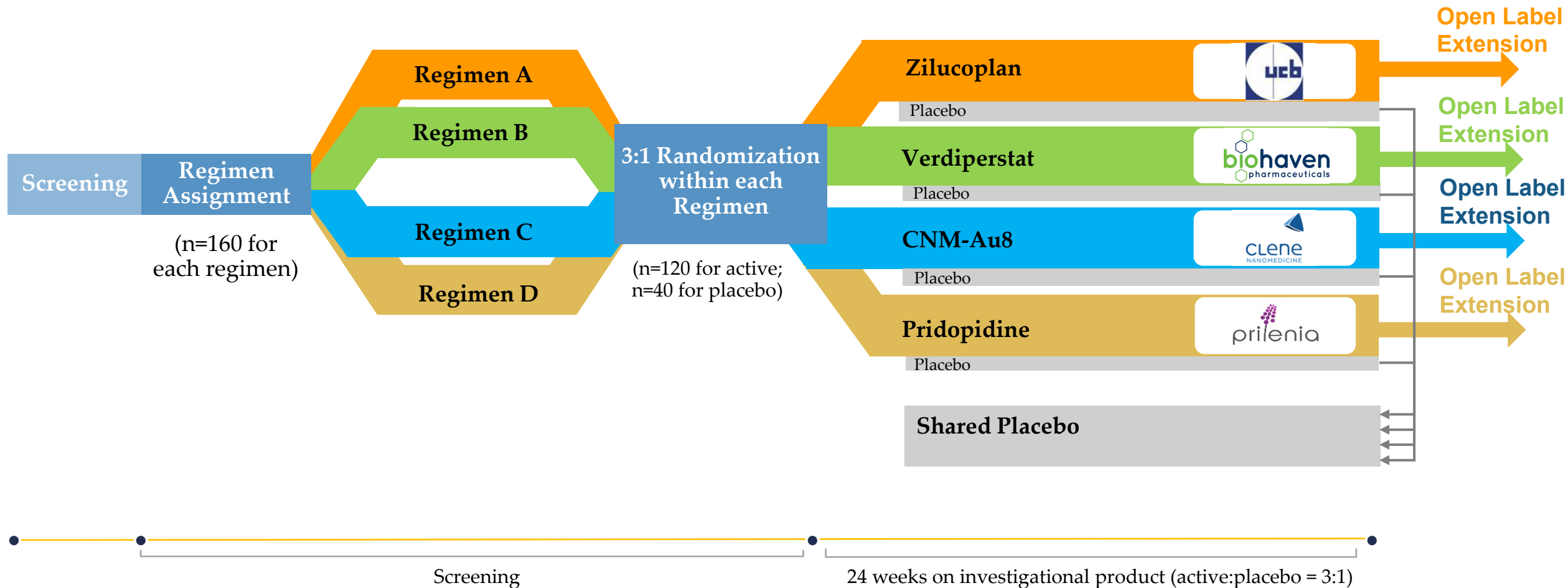


# Guest Speaker

**Jeremy Shefner, MD, PhD**  
**Barrow Neurological Institute Phoenix, AZ**  
**Regimen Lead (Regimen D: Pridopidine)**



# 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)**

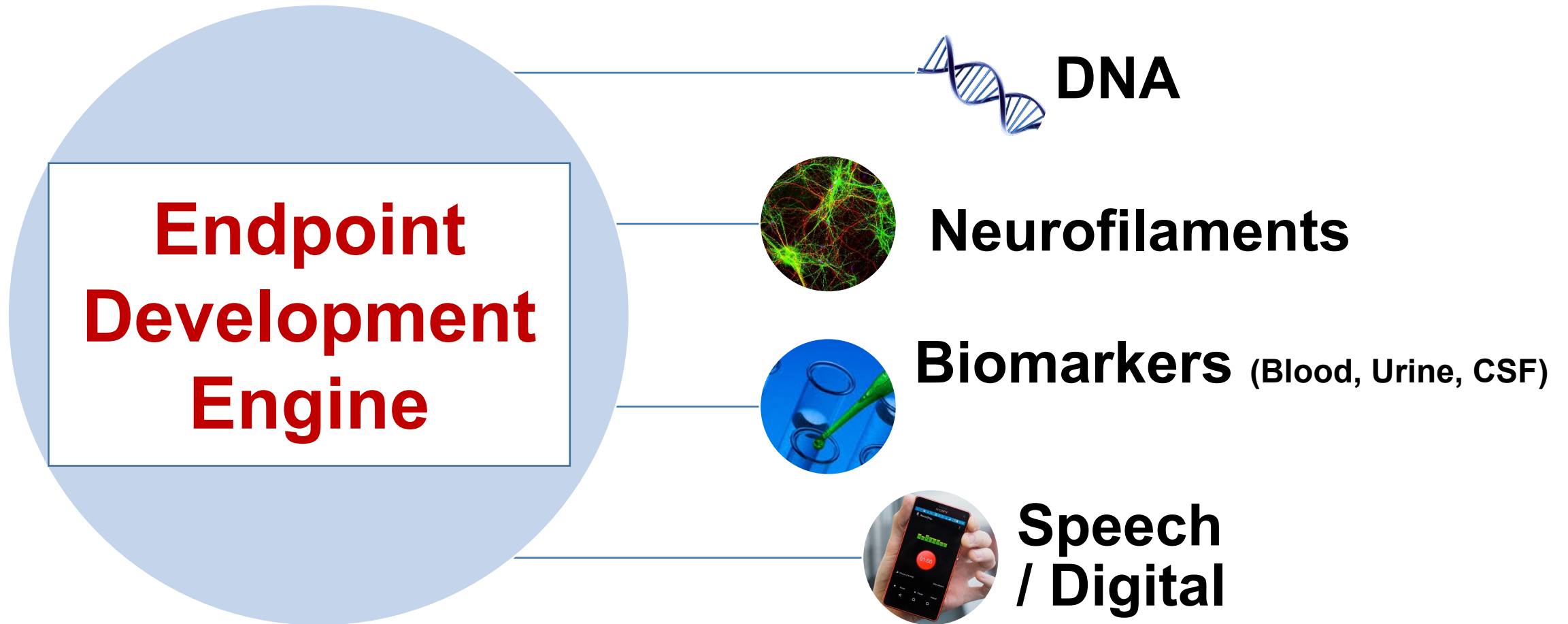
## Secondary Endpoints

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




# 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.

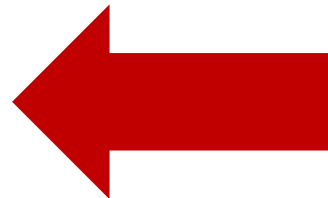


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	<a href="#">Jessie Duncan</a>

50 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 8, 2021

## 50 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
- ✓ [Mayo Clinic Florida](#)

# Enrollment Updates (as of April 8, 2021)

- **420** individuals with ALS signed informed consent
- **329** individuals were assigned to a regimen
- **280** are receiving investigational product (active or placebo)
- **56** 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**

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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 2: Regulatory Considerations



Peter DiRoma and Holly Fernandez Lynch, JD, MBe  
Friday, April 9, 2021  
12:30 - 1:30 PM EDT

Join us for the second installment of the NEALS webinar series on ALS drug development moderated by Dr. Jinsy Andrews from Columbia University. 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.

### 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 (Recorded)

April 7<sup>th</sup> 12:00-1:00pm EDT

#### Part 2: Regulatory Considerations

April 9<sup>th</sup> 12:30-1:30pm EDT

Register and view recordings at

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

# Send us webinar ideas!

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



## Next Week's Guest: Dr. Adam Quick from Ohio State University

