

# HEALEY ALS Platform Trial

Weekly Q&A – July 29, 2021



## Healey Center

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

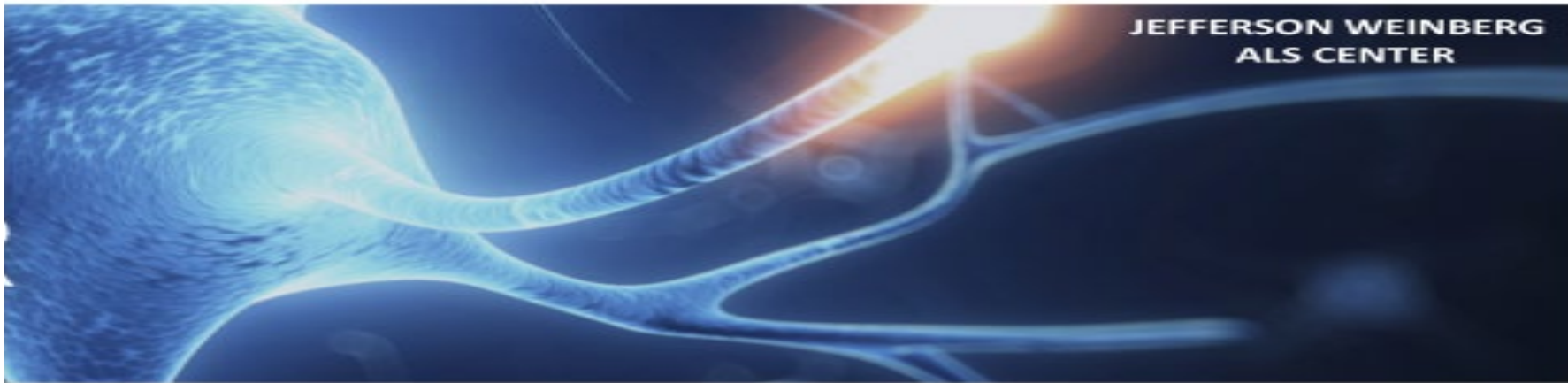


The AMG Foundation

# Guest Speaker

**Hristelina Ilieva, MD, PhD**  
**Thomas Jefferson, PA**  
**Platform Trial Site Investigator**





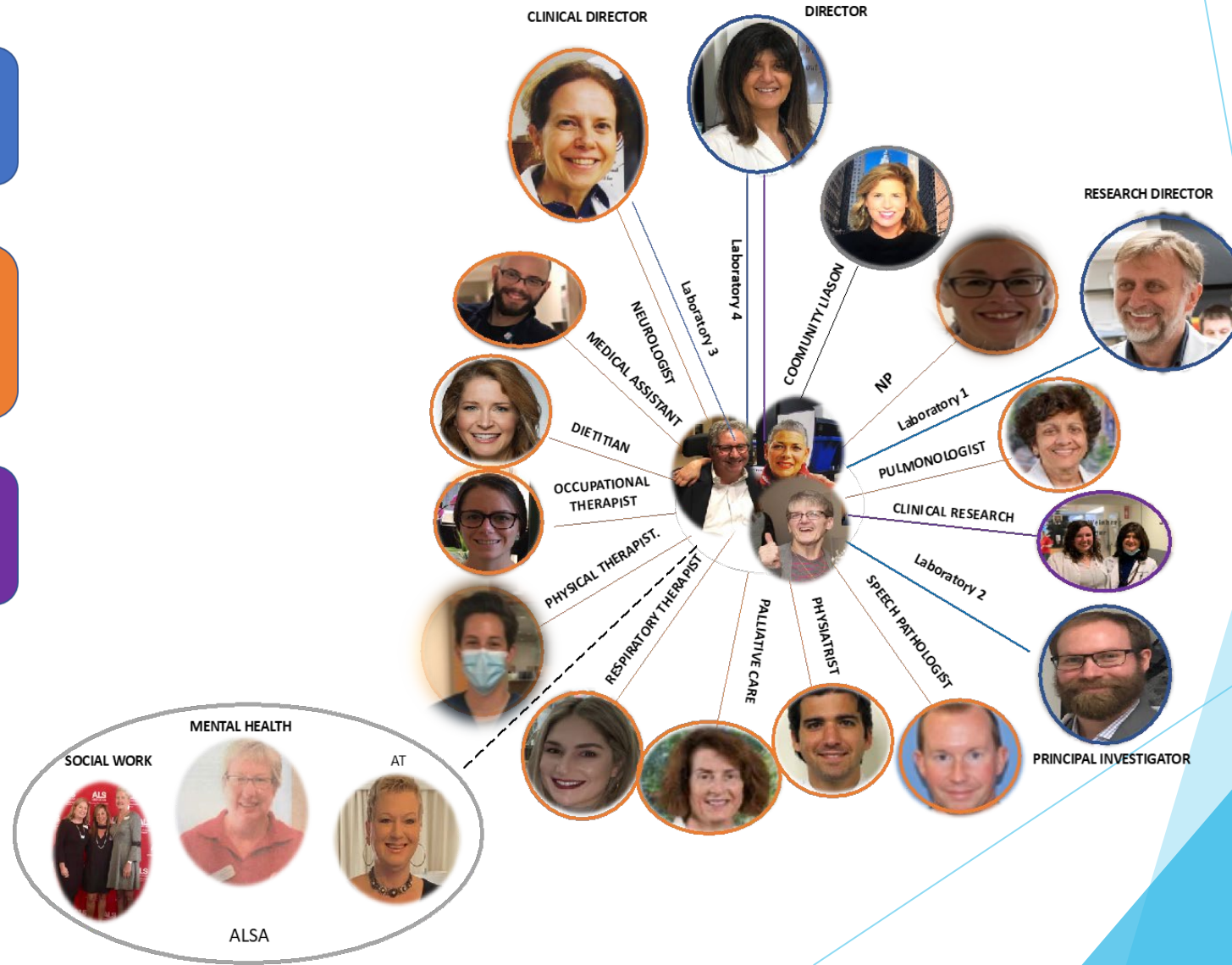
- ▶ Piera Pasinelli, PhD -
  - ▶ Director
- ▶ Hristelina Ilieva, MD, PhD -
  - ▶ Clinical Director and PI of Healey Platform Trial
- ▶ Davide Trotti, PhD
  - ▶ Research Director
- ▶ Jessica Rinaldi -
  - ▶ Associate Director of Clinical Research
- ▶ Stephanie Hansen, Madeleine Singson, Sofia Nastri, Christopher Hague, Melissa Warren
  - ▶ Clinical Research Team

# Jefferson Weinberg ALS Center

4 RESEARCH LABORATORIES  
(BASIC-TRANSLATIONAL)

INTER-DISCIPLINARY CLINIC  
(Standard of care +  
*Pulmonology; Physiatry;  
Palliative Care; Research Education*  
ALSA Staff)

CLINICAL RESEARCH



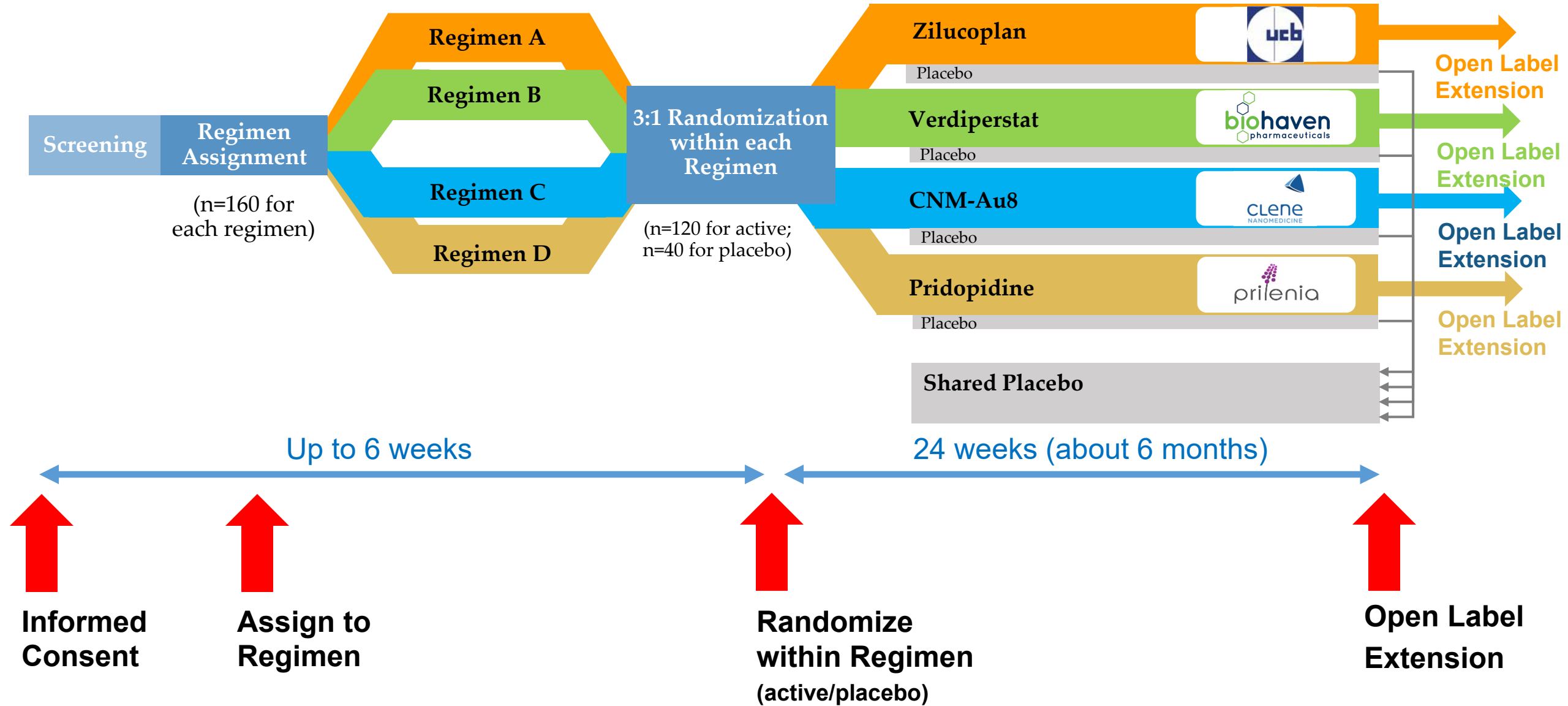
# Healey Platform Trial

- ▶ Our site started enrolling patients in August of 2020, and enrollment is still ongoing.
- ▶ Our site currently has 11 patients participating in this trial.
  - ▶ 3 on **Regimen A**, 3 on **Regimen B**, 4 on **Regimen C**, 1 on **Regimen D**.
  - ▶ 5 patients have moved on to the open label extension, 6 remain on double-blind.
  - ▶ 2 patients have completed DB phase but unable to continue with OLE due to disease progression.

# Perpetual Adaptive Trial

## Shared Placebo; Randomization Ratio 3:1

### Open Label Extension (OLE) offered





# Enrollment Updates (as of July 29, 2021)

- **676** individuals with ALS signed informed consent
- **534** individuals were assigned to a regimen
- **482** individuals were randomized within a regimen (active or placebo)
- **155** have entered the Open Label Extension (OLE)
  
- **130** individuals were randomized within Regimen A
- **149** individuals were randomized within Regimen B
- **148** individuals were randomized within Regimen C
- **55** 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	<a href="#">Jessie Duncan</a>

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](mailto: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 5<sup>th</sup>- TBD**

**Aug 12<sup>th</sup>- Michael Weiss, MD, FAAN (University of Washington, WA)**

**Aug 19<sup>th</sup>- Laura Foster, MD (University of Colorado, CO)**

**Aug 26<sup>th</sup>- TBD**

# 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>)