

HEALEY ALS Platform Trial

Weekly Q&A – Jan 6, 2022



Healey Center

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

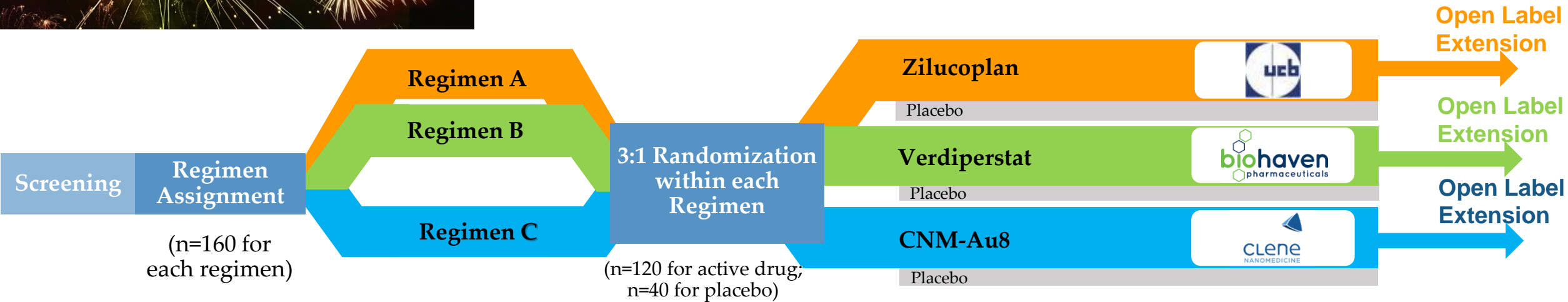


The AMG Foundation

➤ *Thank you!*

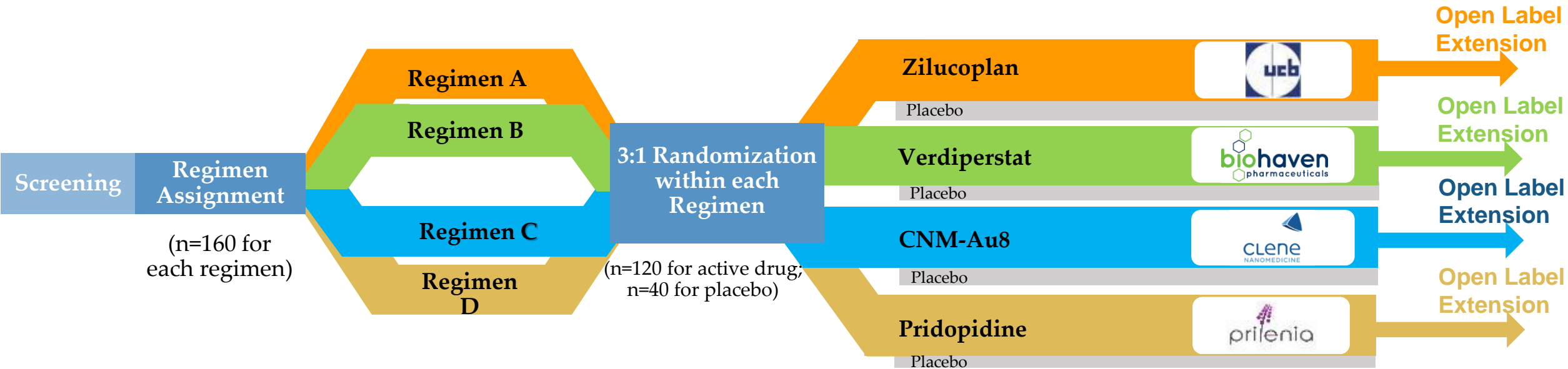


The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



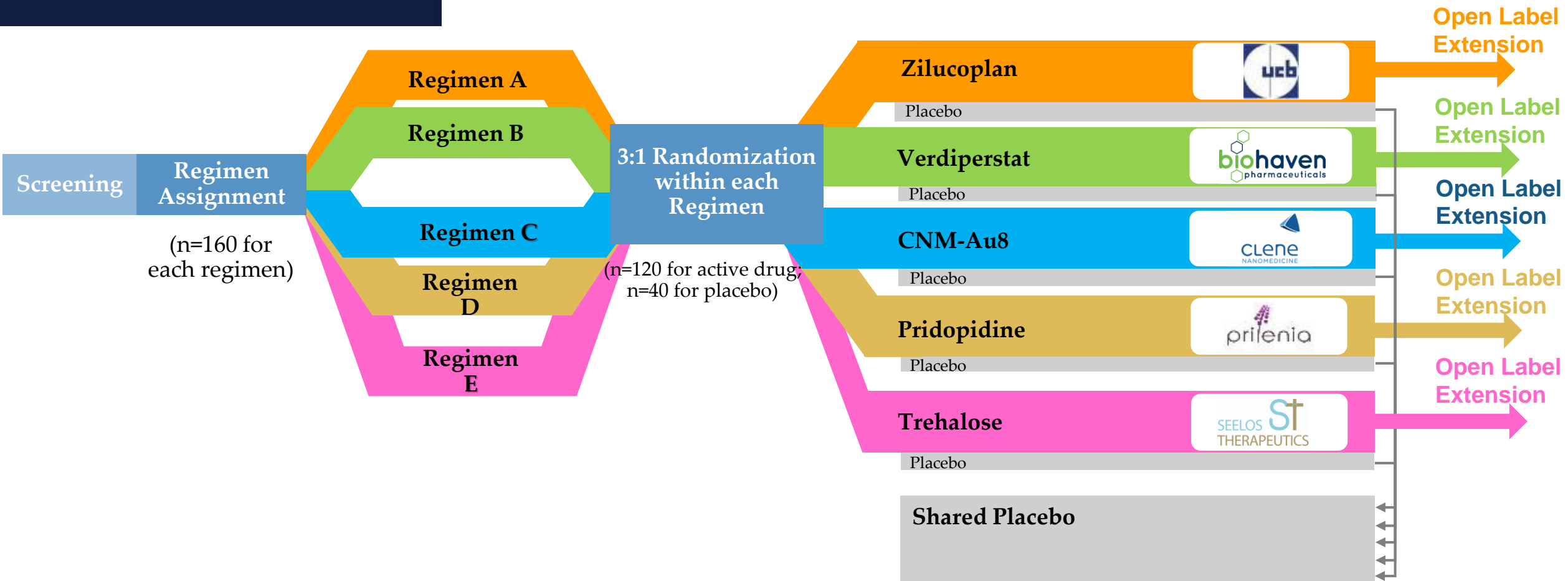


The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



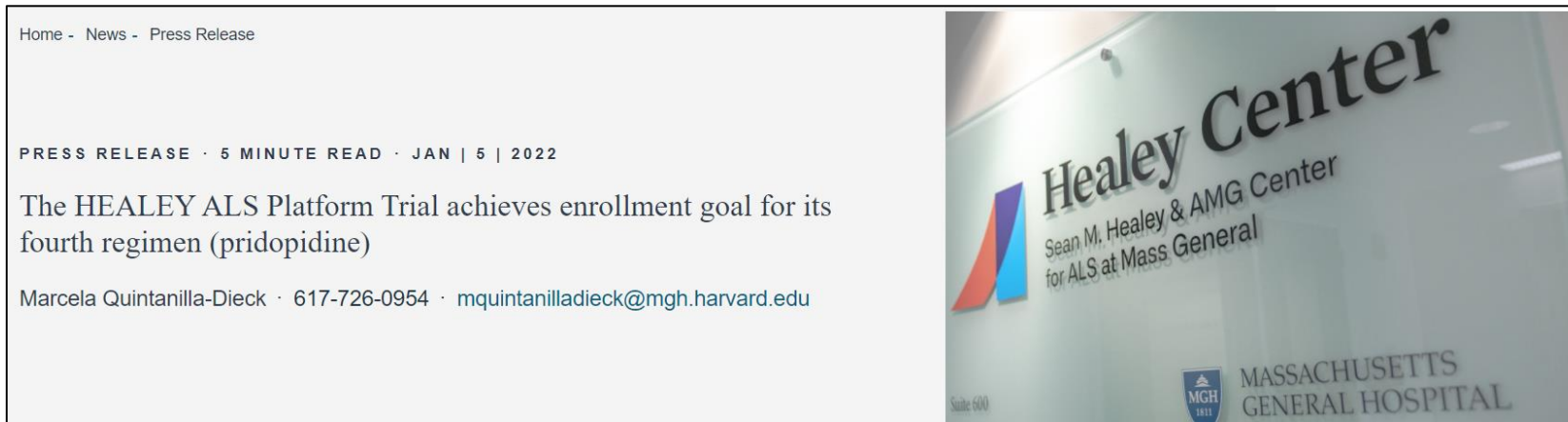


The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Regimens A, B, C and D completed enrollment!

- ✓ **162** individuals were randomized within Regimen A
- ✓ **167** individuals were randomized within Regimen B
- ✓ **161** individuals were randomized within Regimen C
- ✓ **163** individuals were randomized within Regimen D



> 300 participants have entered the Open Label Extension (OLE)

PRESS RELEASE · 5 MINUTE READ · JAN | 5 | 2022

The HEALEY ALS Platform Trial achieves enrollment goal for its fourth regimen (pridopidine)

Marcela Quintanilla-Dieck · 617-726-0954

· mquintanilladieck@mgh.harvard.edu



<https://bit.ly/31s5Y6y>

“Completing enrollment for the fourth regimen in the Platform Trial provides further hope towards discovering treatments for people living with ALS using this novel approach to therapy development in the disease. We are grateful for our partnerships with people with ALS who provide advice and input and without whose participation we could not develop effective ALS treatments.”

Merit Cudkowicz, MD, MSc

Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital



“The exceptional enrollment rates we are seeing are a testament to the power of the platform approach”

Sabrina Paganoni, MD, PhD

physician-scientist at the Healey & AMG Center and co-lead investigator of the trial

Our first collaborative paper was accepted for publication!

Adve



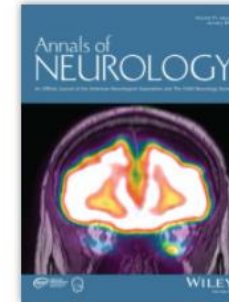
Neurology Grand Rounds |  Free Access

Adaptive Platform Trials to Transform ALS Therapy Development

Sabrina Paganoni MD, PhD , James D. Berry MD, MPH, Melanie Quintana PhD, Eric Macklin PhD, Benjamin R. Saville PhD, Michelle A. Detry PhD, Marianne Chase, Alex Sherman, Hong Yu, Kristin Drake MBA, Jinsy Andrews MD, Jeremy Shefner MD, PhD, Lori Chibnik PhD, MPH, Matteo Vestrucci PhD, Merit E. Cudkowicz MD, MSc, for the HEALEY ALS Platform Trial Study Group

First published: 21 December 2021 | <https://doi.org/10.1002/ana.26285>

This article has been accepted for publication and undergone full peer review but has not been through the copyediting, typesetting, pagination and proofreading process, which may lead to differences between this version and the Version of Record. Please cite this article as doi: 10.1002/ana.26285.



Advertisement

Accepted Articles

Accepted, unedited articles published online and citable. The final edited and typeset version of record will appear in the future.



Plans for Expansion: 22 New Sites Coming Soon



- ✓ Atrium Health
- ✓ Cleveland Clinic
- ✓ George Washington University
- ✓ University of California, San Francisco
- ✓ Yale University
- ✓ Indiana University
- ✓ Stony Brook University
- ✓ **Georgetown University**
- ✓ University of Arkansas
- ✓ University of Pittsburgh
- ✓ **University of Southern California**
- ✓ University of Utah
- ✓ Augusta University
- ✓ University of Cincinnati
- ✓ Virginia Commonwealth University
- ✓ Swedish Medical Center
- ✓ Las Vegas Clinic
- ✓ Kaiser, Los Angeles
- ✓ Lehigh Valley Health Network
- ✓ St. Alphonsus Regional Medical Center
- ✓ Hackensack University
- ✓ Essentia Health

Note: Only those sites highlighted in blue are actively enrolling at this time. Additional sites will be activated on a rolling basis.

Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

- ▶ Enrollment updates shared weekly
- ▶ Over 1,000 questions about ALS research answered live
- ▶ Over 13,000 viewers from 43 countries
- ▶ Special guests and topics added each week

Weekly webinar
registration:



<https://bit.ly/3pT9H6C>

ALS Link sign-up:



<https://bit.ly/3EWEp38>



Allison Bulat

Send us webinar ideas!

Upcoming Guest Speaker:

Jan 13th- Sharon Hesterlee, PhD of the Muscular Dystrophy Association

Weekly webinar
registration:



<https://bit.ly/3pT9H6C>

ALS Link sign-up:



<https://bit.ly/3EWEp38>