

HEALEY ALS Platform Trial

Weekly Q&A – Dec 2, 2021



Healey Center

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



The AMG Foundation

Guest Speaker

Neil Thakur, PhD
Chief Mission Officer
The ALS Association





A **rapid** and fundamental transformation in the ALS experience, until we find cure

Longer Lives

- Life-extending treatments made accessible to ALL people with ALS
- Prevention of harms associated with ALS

Improved Quality of Life

- People with ALS empowered to live their lives as they want
- Greater engagement and autonomy
- Reduced or prevented physical, emotional and financial burdens

Preventing Loved Ones from Getting ALS

- Genetic screening to identify those at risk
- Prevention of new cases of ALS



How we get there



Find New Treatments & Cures

- More clinical trials and participation with research
- More focused research

Optimize Current Treatments & Care

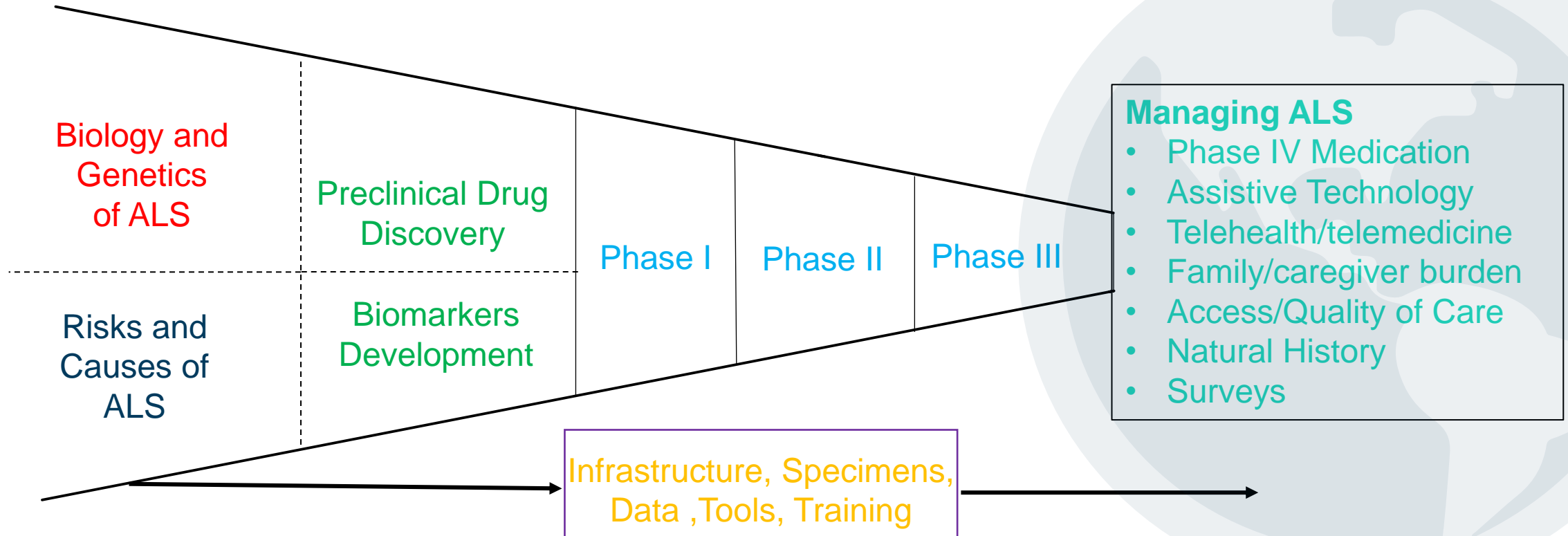
- Improve and deliver state of the art care
- Improve assistive technology

Prevent or Delay Harms Associated with ALS

- Identify risk factors
- Treat as early as possible
- Discover new preventative treatments

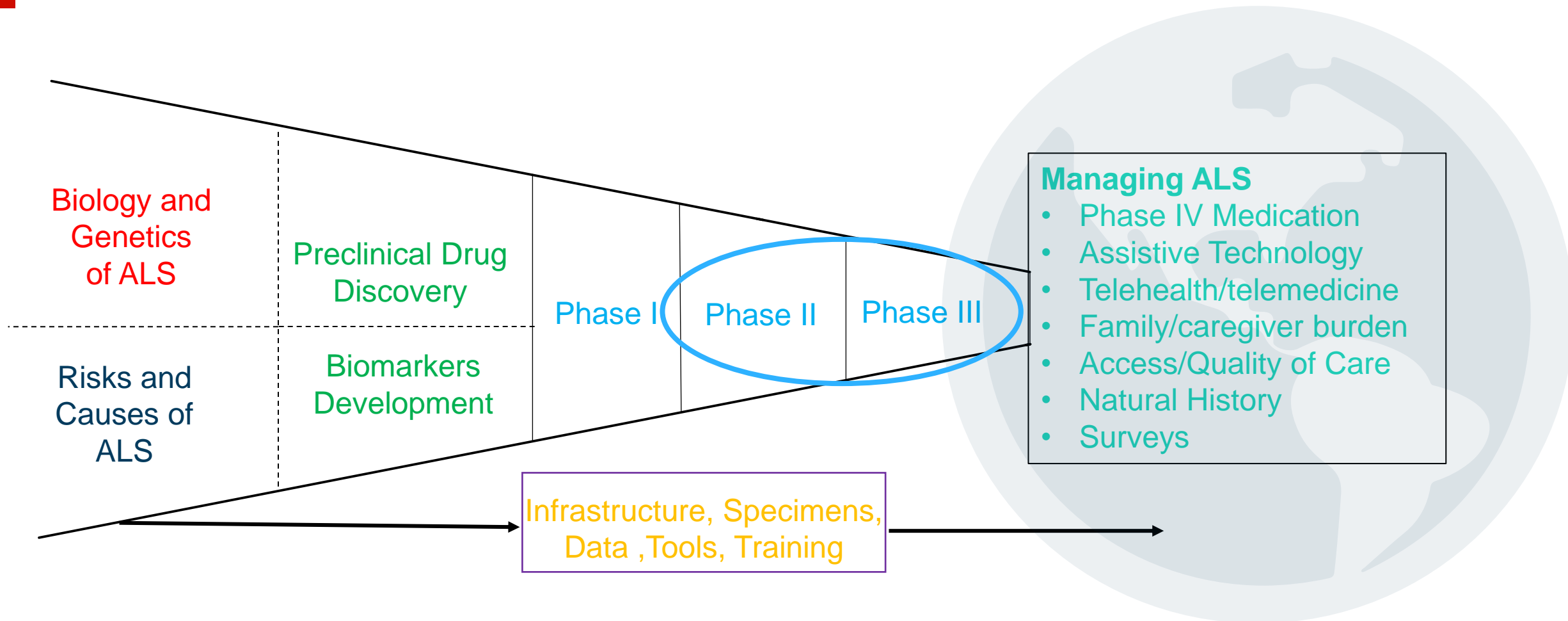


The ALS Association's Research Program Currently Funds Widely Across the ALS Ecosystem

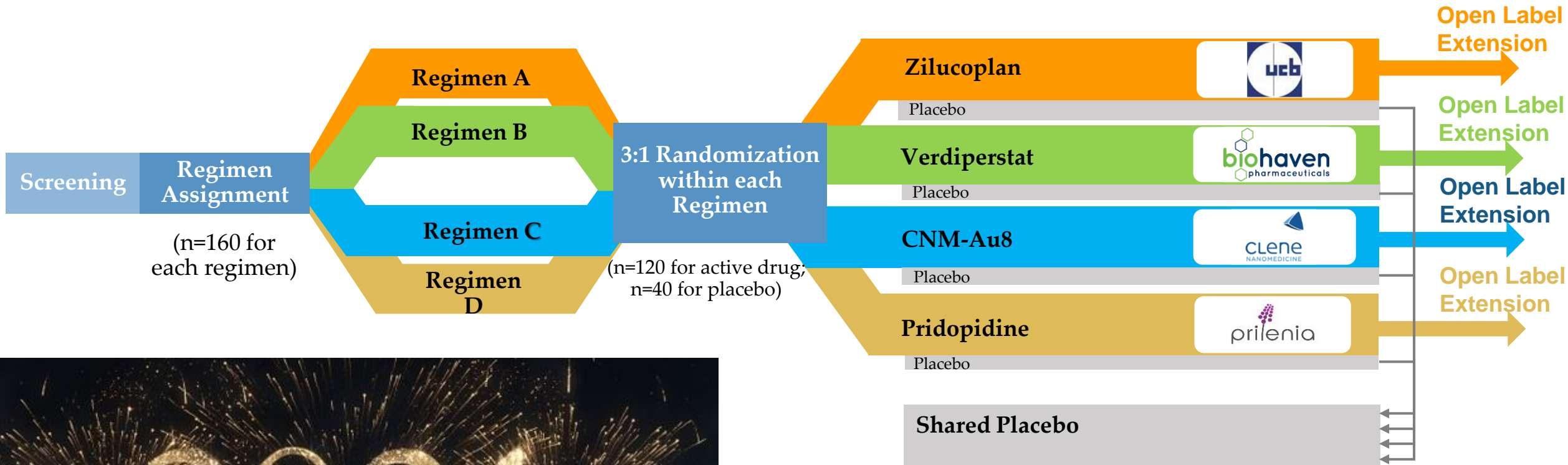


~150 Active Projects, 12 countries around the world, \$50M research commitment

The Healey ALS Platform trial makes part of the pipeline faster



The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Regimens A, B and C completed enrollment!

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

156 individuals were randomized within Regimen D

299 have entered the Open Label Extension (OLE)

*"I'm looking forward to helping find
a cure for ALS."*

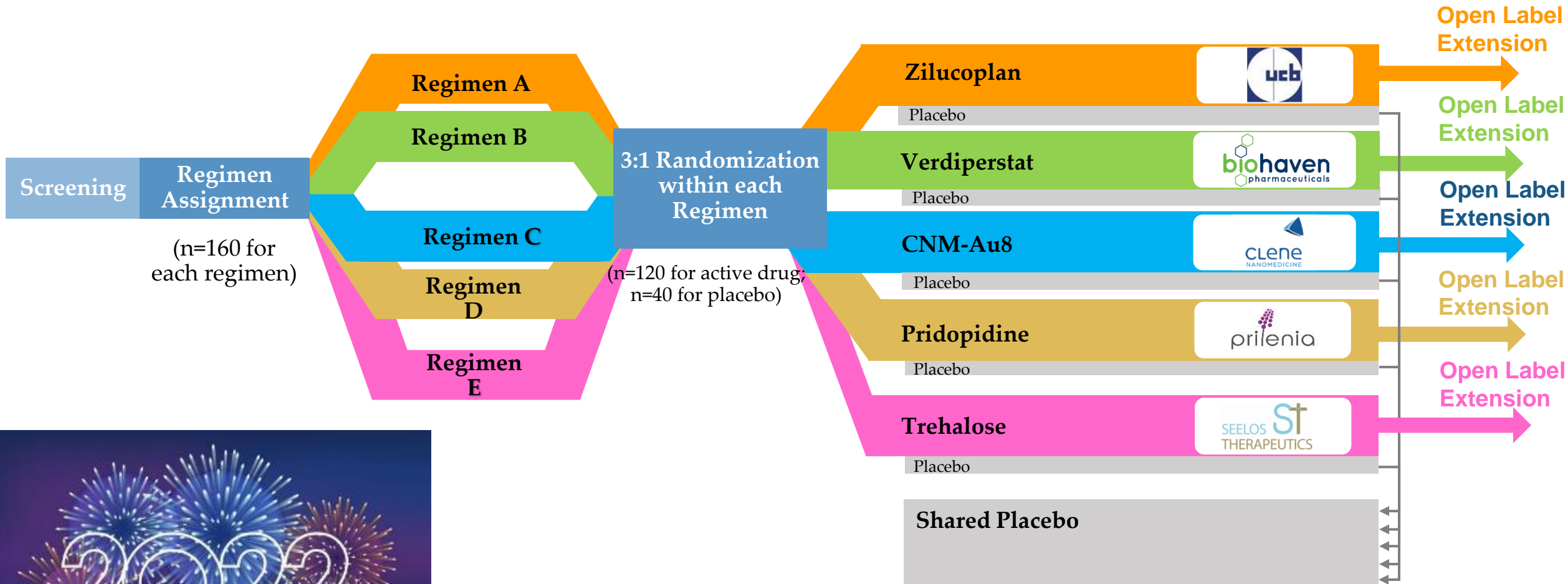
-Platform trial participant

>800 people with ALS signed Informed Consent
for the Platform Trial

Thank You

This breakthrough trial would not be possible
without your partnership

The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



54 sites enrolling, 20 more selected



- ✓ 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. Alphonus 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:



ALS Link sign-up:



Allison Bulat

Send us webinar ideas!

Upcoming Guest Speakers:

Dec 9th- Nicholas Olney, MD (Providence ALS Clinic, OR)

Dec 16th- Biostatistics with Lori Chibnik, PhD, MPH

Dec 23rd- No Webinar

Dec 30th- No Webinar

Weekly webinar
registration:



ALS Link sign-up:

