

HEALEY ALS Platform Trial

Weekly Q&A – Jan 27, 2022



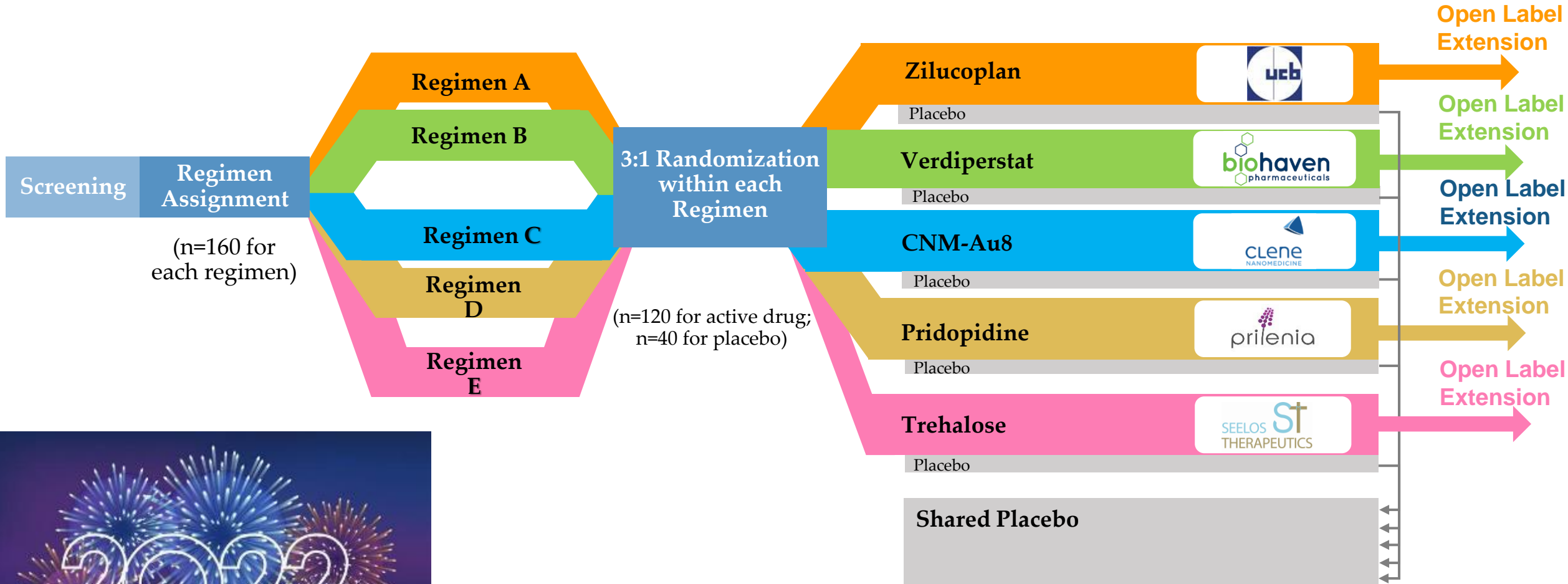
Healey Center

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



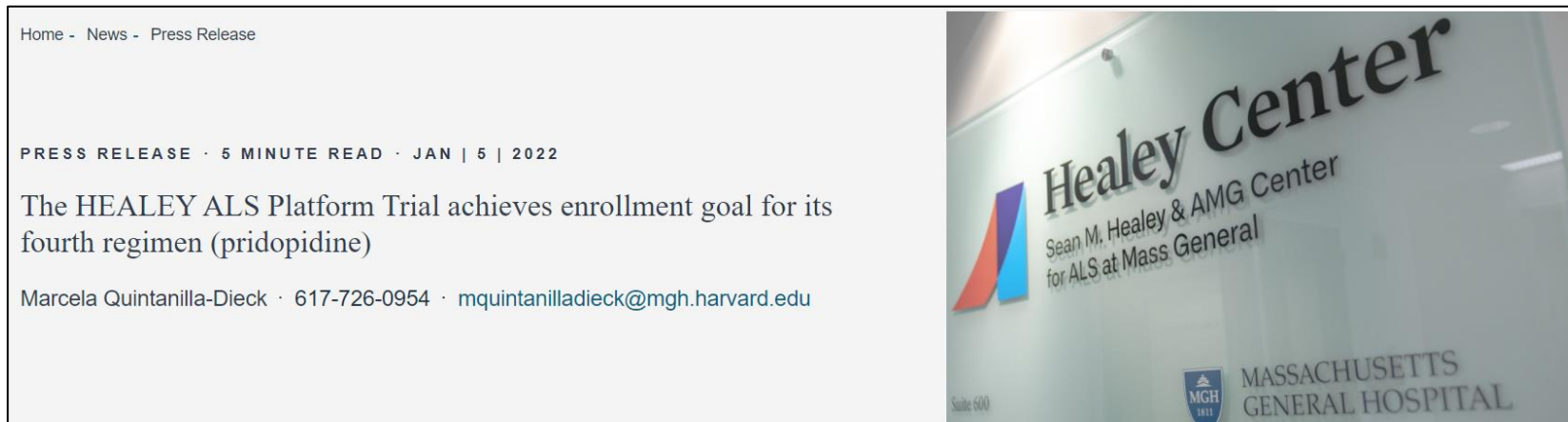
The AMG Foundation

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



365 participants have entered the Open Label Extension (OLE)

= Thank You =

This breakthrough trial would not be possible without your partnership

52 Sites Activated for Regimens A-D



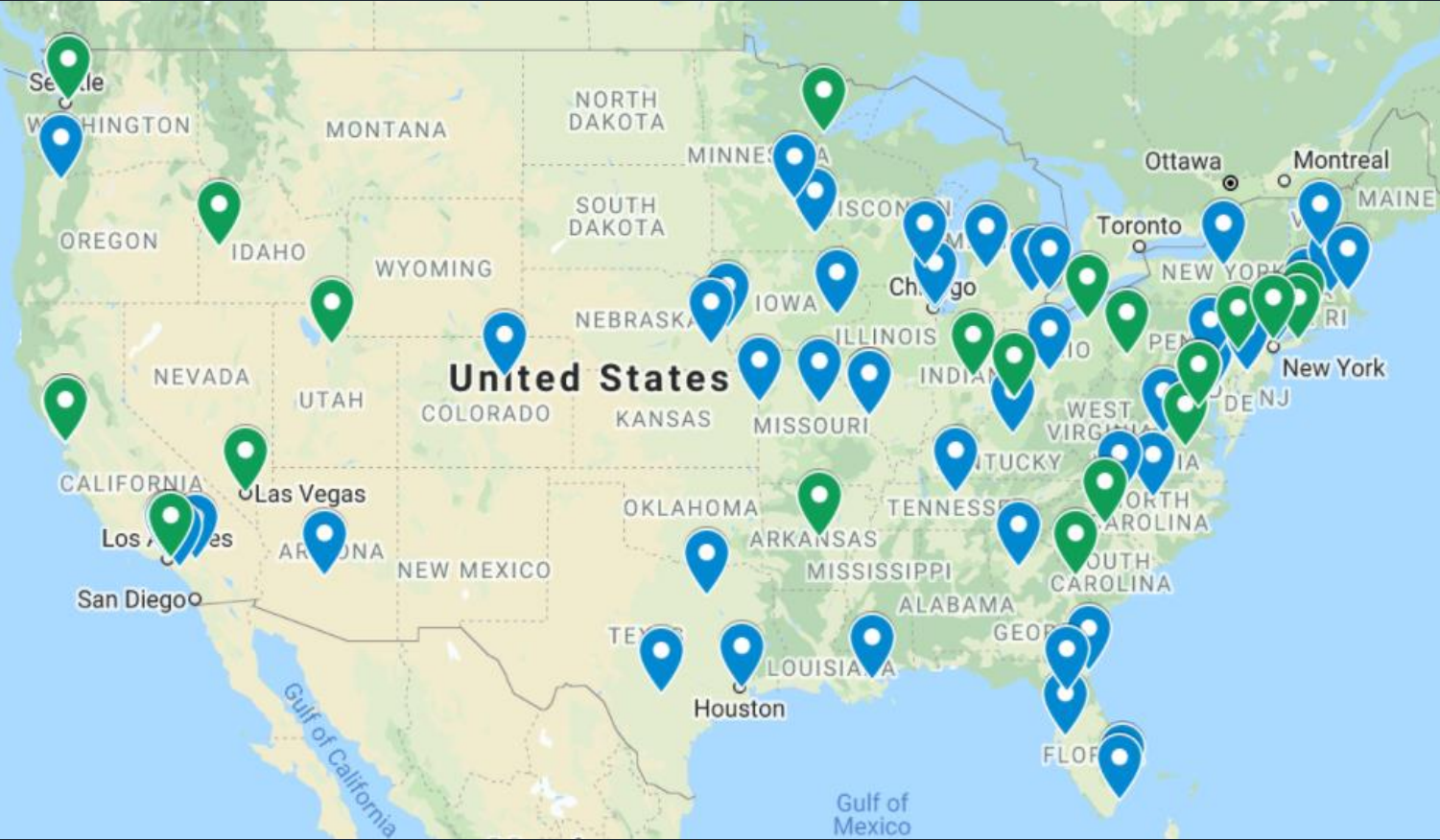
- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ UTHSCSA
- ✓ Hospital for Special Care
- ✓ Holy Cross Hospital
- ✓ Thomas Jefferson
- ✓ Houston Methodist
- ✓ Henry Ford Health System
- ✓ Barrow Neurological Institute
- ✓ Ohio State University
- ✓ Northwestern University
- ✓ University of Chicago
- ✓ 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
- ✓ St. Louis University
- ✓ Providence Brain and Spine

2 Additional Sites Activated for Regimen D



- ✓ 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
- ✓ St. Louis University
- ✓ Providence Brain and Spine
- ✓ Georgetown University
- ✓ University of Southern California

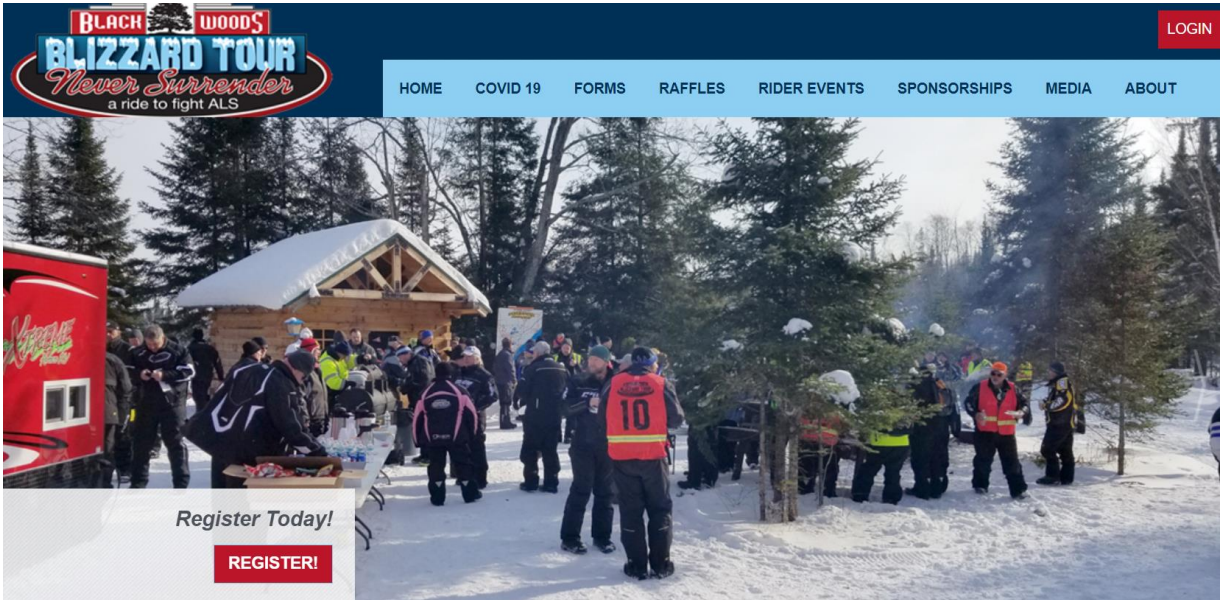
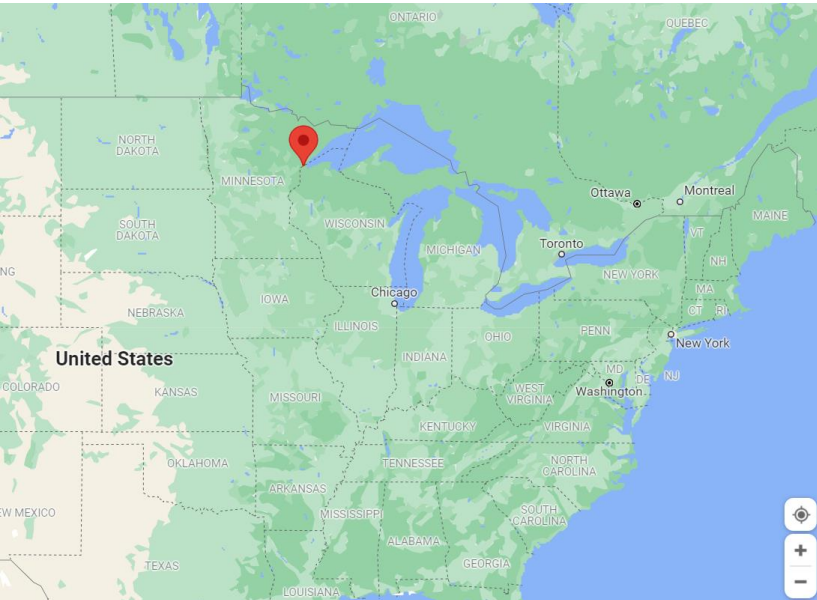
20 New Sites to be Activated for Regimen E



- 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
- St. Louis University
- Providence Brain and Spine
- Georgetown University
- University of Southern California
- Atrium Health
- Cleveland Clinic
- George Washington University
- University of California, San Francisco
- Yale University
- Indiana University
- Stony Brook University
- University of Arkansas
- University of Pittsburgh
- 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

New site spotlight: Essentia Health

First ever ALS platform drug trial comes to Duluth



Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants in regimen E.

Sites marked "Active, Not recruiting" are active in the platform (for example, they are following participants in regimens A-D) but are not enrolling new participants at this time. Most active sites are expected to start enrolling in regimen E soon.

Site	State	Enrollment Status	Contact Information
Jefferson Weinberg ALS Center, Thomas Jefferson University	PA	Active, Not recruiting	Stephanie Hansen
Lehigh Valley Health Network	PA	Recruiting	Andrew Orzel (610-402-9543)
Penn State Hershey	PA	Active, Not recruiting	Heidi Runk



<https://bit.ly/3g2NZr5>

Regimen E Site Activation Plans

- Plan to activate about 20 sites per month
- Target number: about 65 sites total

Sites on track for January activation:

- | | | |
|---|---------------------------------|--|
| ➤ Lehigh Valley Health Network- activated! | ➤ University of Nebraska | ➤ University of South Florida |
| ➤ Mass General Hospital | ➤ University of Cincinnati | ➤ University of Kansas Medical Center |
| ➤ University of Massachusetts | ➤ Spectrum Health | ➤ Wake Forest Health Sciences |
| ➤ Northwestern University | ➤ University of Colorado | ➤ SUNY Upstate |
| ➤ Hospital for Special Care | ➤ Georgetown University | ➤ Washington University |
| ➤ University of Maryland | ➤ Barrow Neurological Institute | ➤ Beth Israel Deaconess Medical Center |
| ➤ Virginia Commonwealth University | ➤ Indiana University | ➤ California Pacific Medical Center |

Patient Navigation

Central resource for people living with ALS



Catherine Small

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Weekly webinar
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

Upcoming Guest Speakers:

Feb 10th- Ximena Arcila-Londono, MD (Henry Ford, Detroit MI)

Feb 17th- Jim Caress, MD (Wake Forest, Winston-Salem NC)



Allison Bulat