HEALEY ALS Platform Trial

Weekly Q&A – Oct 20, 2022



MGH	MASSACHUSETTS
1811	GENERAL HOSPITAL



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











Berry Consultants





























The Arthur M. Blank FAMILY FOUNDATION





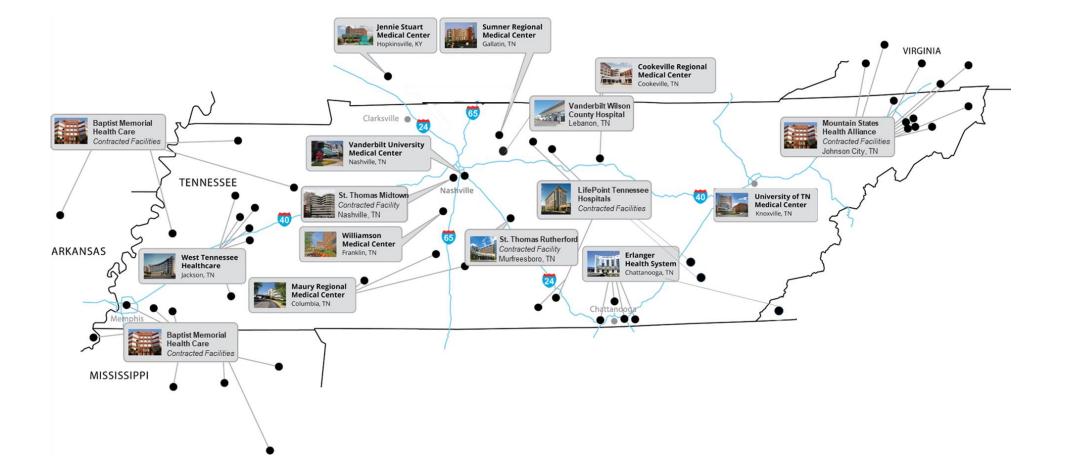
Guest Speaker



Amanda Peltier, MD, MS

Platform Trial Site Investigator Vanderbilt University Medical Center Nashville, TN

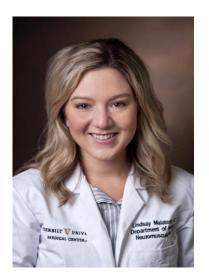
VUMC and Allied Hospitals, TN



VUMC Neuromuscular Division Faculty











The Vanderbilt ALS Multidisciplinary Clinic



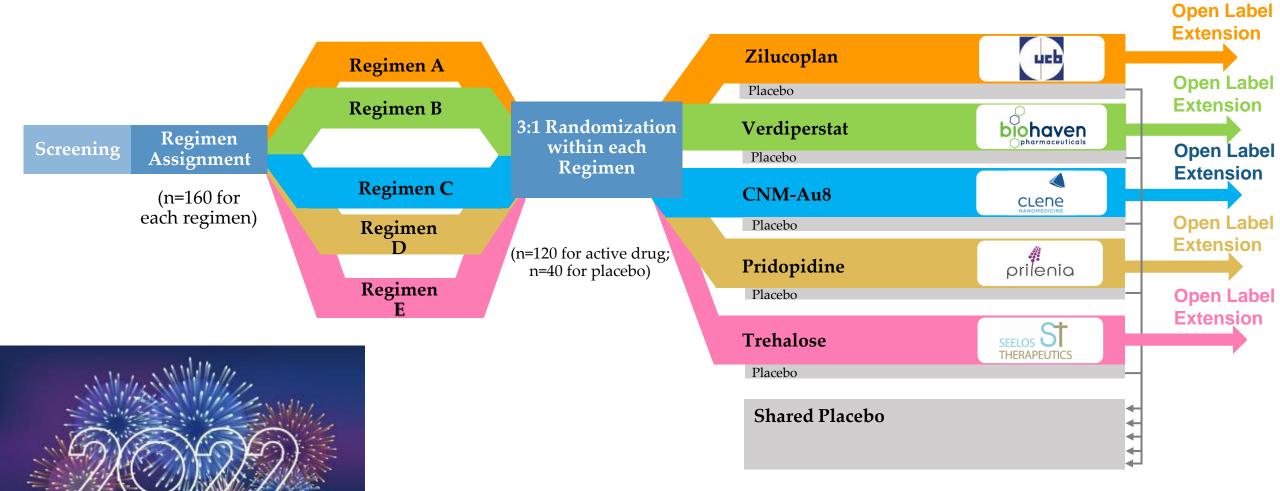
ALS at Vanderbilt

- We have a multidisciplinary clinic monthly which currently serves ~125 patients from Tennessee, Kentucky and Alabama.
- We are supported by MDA and ALSA.
- Our clinic includes 4 neuromuscular specialists, 2 neuromuscular NP, pulmonology (1 MD, 1 NP), respiratory therapy, social work, palliative care, speech therapy, physical therapy, occupational therapy, assistive technology, nutrition
- We have 2 full time research coordinators.
- We have 10 patients who have been randomized in Healey

Thank You!!

- Your participation makes it possible for advances in ALS.
- We could not do it without you!

The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Enrollment Updates (as of Oct 20, 2022)

• 153 individuals have signed informed consent

112 individuals have been randomized within Regimen E

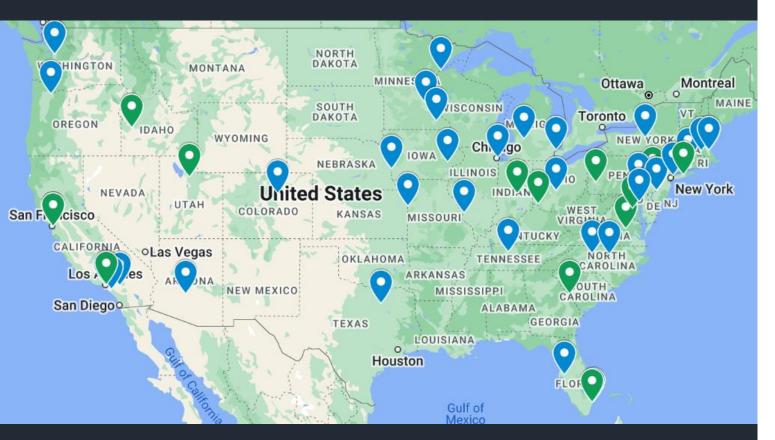
Thank You

This breakthrough trial would not be possible without your participation

Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality

51 Sites Currently Activated for Regimen E



(as of 10/20/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

<u>Lehigh Valley Health Network</u>	UC San Francisco
Mass General Hospital	Mayo Rochester
University of Kansas	University of Washington
University of Maryland	Vanderbilt University
California Pacific Medical Center	UPMC
Northwestern University	Indiana University
Virginia Commonwealth University	Augusta University
University of Nebraska	University of Utah
Washington University	Holy Cross Hospital
Wake Forest University	Penn State Hershey
Hospital for Special Care	University of CA, Irvine
Saint Alphonsus Regional	Cedars Sinai Medical Center
University of Massachusetts	University of Pennsylvania
Duke University	Nova Southeastern University
Barrow Neurological Institute	Johns Hopkins University
Georgetown University	Columbia University
Texas Neurology	Stony Brook University
Beth Israel Deaconess Medical Center	<u>Kaiser, Los Angeles</u>
SUNY Upstate	
Spectrum Health	
Henry Ford Hospital	
<u>Essentia Health</u>	Site Map & Contact
University of Southern California	and canada and

 $\mathbf{\nabla}$

 $\mathbf{\nabla}$

V

 $\mathbf{\nabla}$ $\mathbf{\nabla}$

 $\mathbf{\nabla}$

 $\mathbf{\nabla}$

 $\mathbf{\nabla}$

University of South Florida

Providence Brain and Spine

University of Colorado

University of Minnesota Loma Linda University

Swedish Medical Center

Ohio State University

University of Cincinnati

Thomas Jefferson University

University of Iowa



https://bit.lv/3g2NZr5

Patient Navigation Central resource for people living with ALS

E-mail:healeyalsplatform@mgh.harvard.edu



Weekly webinar registration:

Catherine Small



Allison Bulat



Phone: 833-425-8257 (HALT ALS)

https://bit.ly/3r6Nd2L

ALS Link sign-up:



https://bit.ly/3o2Ds3m

Upcoming Cancelations:

November 3rd- Webinar canceled due to annual research conference **November 24th-** Webinar canceled for holiday, Happy Thanksgiving!

Weekly Recordings Available on Website

Webinar Recordings

Science & Mechanism of Action Series

Weekly & Monthly Updates

October 6, 2022: Weekly Q&A featuring discussion of Regimen C/CNM-Au8 results Merit Cudkowicz, MD, MSc and Sabrina Paganoni, MD, PhD presented this week's updates on the HEALEY ALS Platform Trial and answered questions from the audience. We were joined by the co-lead investigators for Regimen C and representatives from Clene Nanomedicine, Inc. to discuss the trial results for CNM-Au8. Watch recording_

September 29, 2022: Weekly Q&A featuring discussion of Regimen B/Verdiperstat results

Merit Cudkowicz, MD, MSc and Sabrina Paganoni, MD, PhD presented this week's updates on the HEALEY ALS Platform Trial and answered questions from the audience. We were joined by the co-lead investigators for Regimen B and representatives from Biohaven Pharmaceuticals to discuss the trial results for Verdiperstat. <u>Watch recording</u> 0

θ

Visit the Website:



https://bit.ly/3g4kzfv