

HEALEY ALS Platform Trial

Weekly Q&A – Sept 23, 2021



Healey Center

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

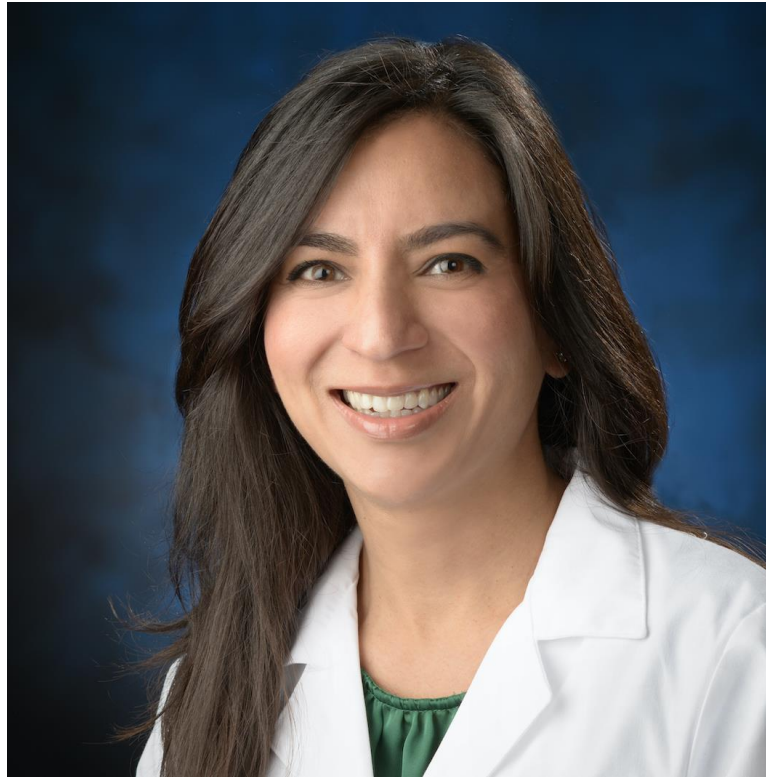


The AMG Foundation



Guest Speaker

Namita Goyal, MD
University of California Irvine, CA
Platform Trial Site Investigator



University of California, Irvine

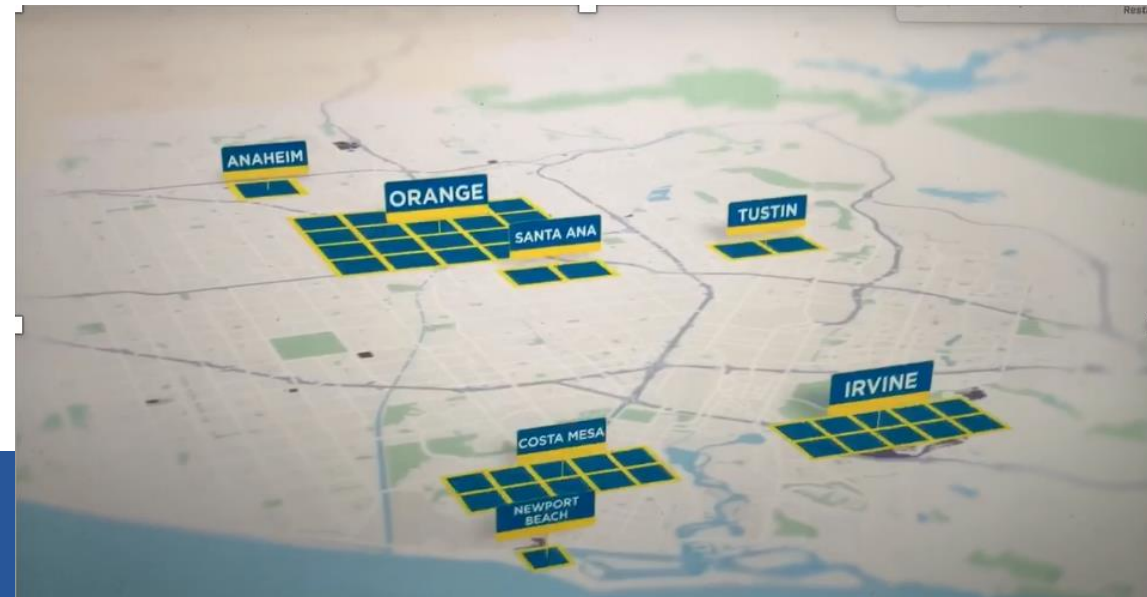


UCI Health



About UC Irvine Health

- Only academic medical center in Orange County
- Level I Trauma Center
- Serve population of 3.4 million people (catchment area > 5 million)
- Diverse patient population in ethnic backgrounds, socioeconomic classes and disease variety



Neuromuscular center



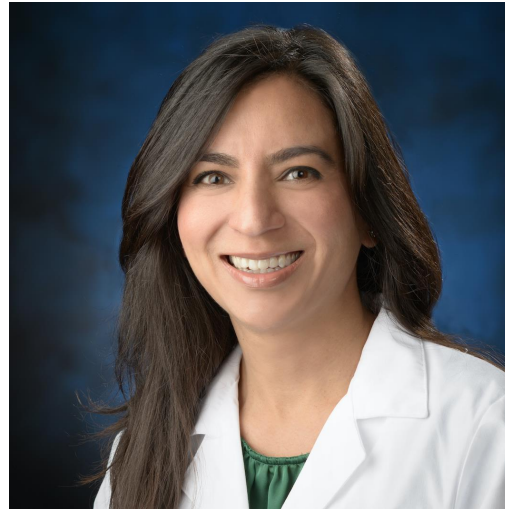
ALS Neuromuscular center (overview)

- **Nationally Recognized: ALS Certified Center of Excellence**
- **Comprehensive multidisciplinary clinics**
- **Electrodiagnostic lab - state of the art**
EMG, single fiber EMG, quantitative sensory testing and autonomic testing
- **High volume of Muscle and Nerve histopathology-** weekly conferences
- **Cutting edge Clinical Trials** in a number of Neuromuscular Disorders:
ALS, Myasthenia gravis, Gene therapy, Myositis

ALS Neuromuscular Faculty



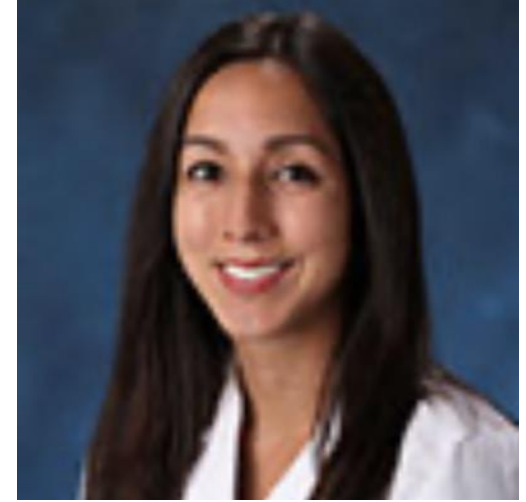
Tahseen Mozaffar, MD
Director of Center



Namita Goyal, MD
Director of NM Fellowship
Co-Director of Center
Director of ALS Clinic



Ali Habib, MD
Director of EMG Lab



Manisha Korb, MD

Trained from:
Wash U

MGH/Harvard

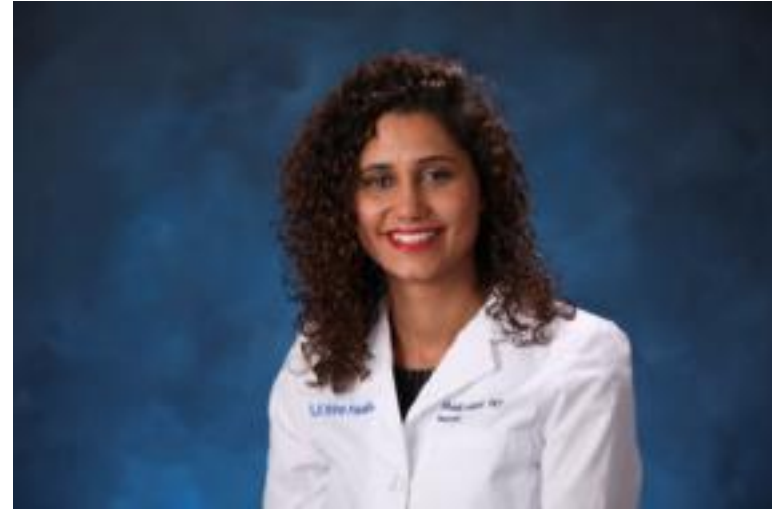
Columbia

Univ of Chicago

Neuromuscular Junior Attendings



Jeff Mullen, MD



Shadi Milani, DO

ALS Center at UC Irvine

- ~250 ALS patients seen in our Center
- Patients seen in Multidisciplinary Team
 - Respiratory, Physical therapy, Speech therapy, RN, Social worker
- Offered cutting edge diagnostic studies
 - including genetic testing
- Opportunity for Clinical trials

Research Team

Cutting edge Clinical Trials

- Oral drugs
- IV infusions
- Stem cell therapy
- Gene therapy



Questions?

Program Director

Namita Goyal, MD

namitag@hs.uci.edu

ALS Clinical Trial Coordinator

Jeanette Overton

jtoverto@hs.uci.edu

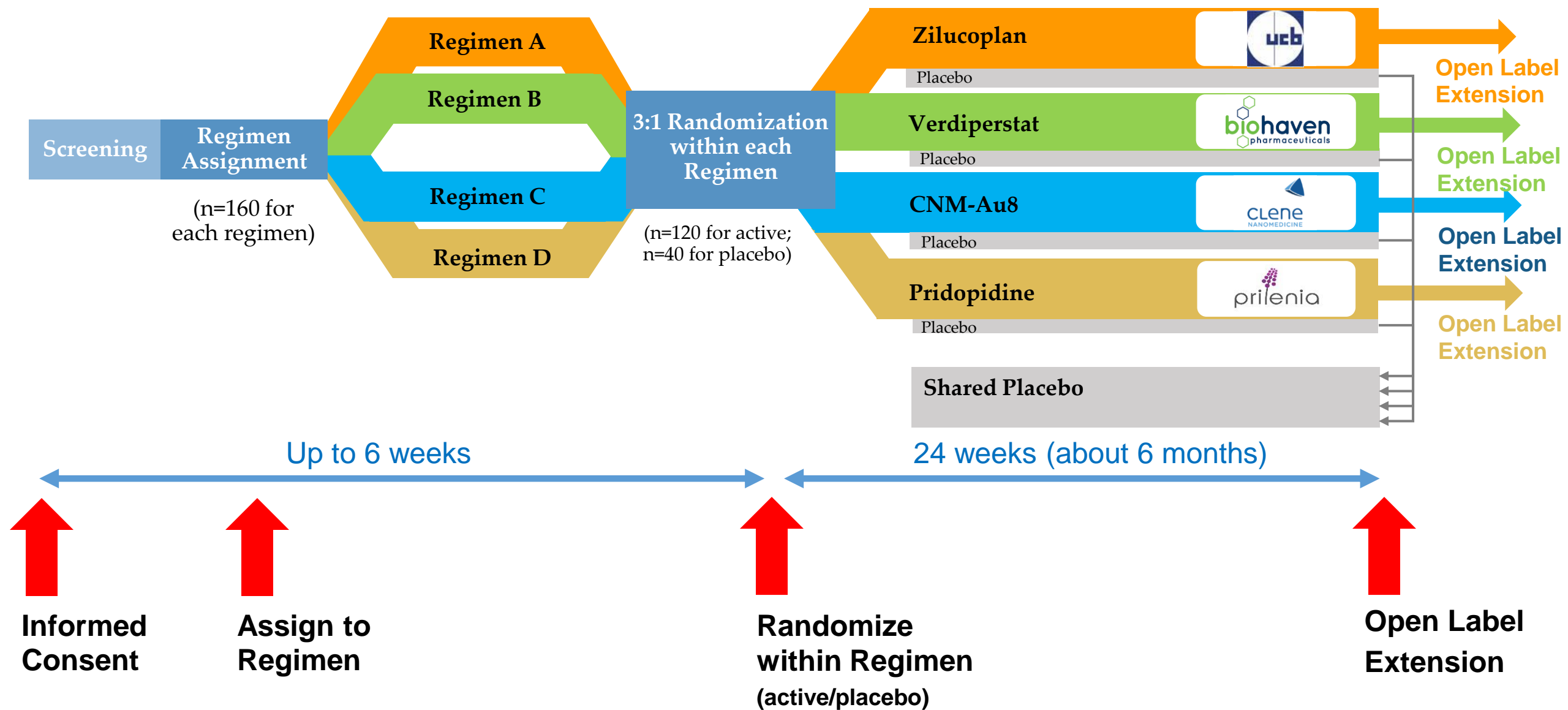
Thank you



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of September 23, 2021)

- **761** individuals with ALS signed informed consent
 - **603** individuals were assigned to a regimen
 - **544** individuals were randomized within a regimen (active or placebo)
 - **210** have entered the Open Label Extension (OLE)
- | | Total randomized +
Participants in screening |
|---|---|
| • 148 individuals were randomized within Regimen A | = 161 |
| • 163 individuals were randomized within Regimen B | = 168 |
| • 160 individuals were randomized within Regimen C | = 161 |
| • 73 individuals were randomized within Regimen D | = 81 |

Thank You

This breakthrough trial would not be possible without your partnership

Patient Navigator



Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail: 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:

Oct 7th- No webinar this week

Oct 14th- David Walk, MD FAAN (University of Minnesota, MN)



ALS Drug Development Webinar Series Part 6: Research Engagement by People Living With ALS



Richard Bedlack, MD, PhD
Duke ALS Clinic



Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for
ALS at Mass General Hospital



View recording at
<https://bit.ly/3frQsfB>

In this webinar, Dr. Richard Bedlack (Duke ALS Clinic) and Dr. Merit Cudkowicz (Sean M. Healey & AMG Center for ALS at Mass General Hospital) discussed why research engagement by people living with ALS is important, examples of how it is helping, continued challenges, and plans for further improvements in the future.

Recorded

Wednesday, September 15, 2021
4:00 - 5:00 PM EDT