

Thank you for joining the webinar!

We are admitting audience members from the waiting room.

**Please allow a few moments for the webinar to begin.**



# HEALEY ALS Platform Trial

## Expanded Access Q&A – Aug 8, 2024



## Healey & AMG Center

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



Calico



THE ARTHUR M. BLANK  
FAMILY FOUNDATION



The AMG Foundation

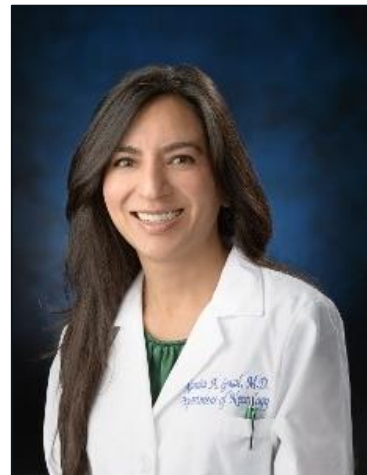
# Guest Speakers

Amanda Stevens



Co-Founder of axeALS Foundation  
Wife, Mother, ALS Advocate

Namita Goyal, MD



Director, UCI Health ALS & Neuromuscular Center  
HEALEY ALS Platform Trial Investigator



## **Our Mission:**

To raise awareness and funding for ALS treatments and clinical trials; to help patients and their families live as full and normal lives as possible; and to encourage research for the prevention, alleviation, care, treatments, and (one day) cure of ALS.

For more information, please contact  
Amanda Stevens, [amanda@axeals.org](mailto:amanda@axeals.org)



# About axeALS

- The axeALS Foundation was founded by Eric and Amanda Stevens after Eric was diagnosed with ALS at just 29 years old.
- Eric is approaching 5 years since being diagnosed with ALS. Since his diagnosis, Eric and Amanda have welcomed 2 beautiful babies into the world.
- axeALS has granted over \$100,000 to families that are fighting this devastating disease.
- In 2023, axeALS Joined forces with the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital to establish a groundbreaking Expanded Access Protocol (EAP) program with a commitment of \$486,000.









**We know how destructive ALS can be.  
Help us change this disease from TERMINAL to TREATABLE!**



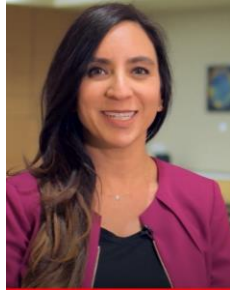
For more info visit: [www.axeALS.org](http://www.axeALS.org)



# UC Irvine ALS Clinical Team



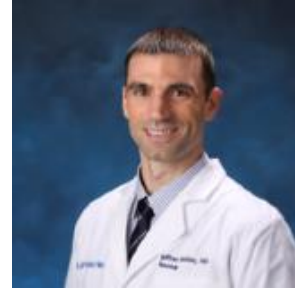
Tahseen Mozaffar, MD  
*Division Head*



Namita Goyal, MD  
*Director of ALS Clinic*



Ali Habib, MD



Jeff Mullen, MD



Minha Han, RN



Multidisciplinary Team



Research Team

# UC Irvine ALS Trials

Interventional Drug Trials	Observational Trials	Upcoming Drug Trials
Annexon (ANX005)	CREATE ALS	Novartis (CVHB937B12201)
Calico (ABBV-CLS-7262)	ALL ALS	AB Science (Masitinib)
Healey Platform	Possible – UCC4ALS	
Medicinova (Ibudilast)	<b>Expanded Access Programs</b>	
MT Pharma (Edaravone)		
PTC (Utreloxastat)	Trehalose EAP	
Sanofi (SAR43820) (RIPK1)	CNMAu8 EAP	
Transposon (for C9)	Upcoming – Pridopidine EAP	
UniQure (SOD1 gene therapy)	Upcoming – RAPA EAP	



# Expanded Access Programs in ALS

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- Opportunity for patients who don't meet eligibility criteria for clinical trials
- ALS symptoms > 3 years
- FVC <50 %

***Thank you to AXE ALS for making EAPs possible at UCI!***



**Healey & AMG Center**

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



**AxeALS Foundation Establishes Expanded Access Protocol (EAP) Program at the University of California, Irvine with The Sean M. Healey & AMG Center for ALS**

# Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Webinar  
registration:



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

ALS Link sign-up:



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



Allison Bulat

**Summer Series Upcoming Webinar:**

**August 22<sup>nd</sup>- Monthly Platform Trial Update**