Thank you for joining the weekly webinar!
We are admitting audience members from the waiting room.
Please allow a few moments for the webinar to begin.
Guest Speakers

Shawn Sarbacker
Founder of TechVsALS

Hristelina Ilieva, MD, PhD
Clinical Director of Jefferson Health’s Weinburg ALS Center
TechVsALS Patient-Led Fundraising For Increased ALS Expanded Access

Shawn Sarbacker & Healey Center for ALS at Mass General Hospital

For more info, please contact
Shawn Sarbacker, shawn@techvsals.org
Alexandra Van Strein, avanstrein@mgh.harvard.edu
www.techvsals.org
About Shawn

- 56 year-old father of 3 + 1, remarried Aug ’22
  #ALoveStory
- Lives in Philly; Sports and fitness aficionado
- Runs a global computer chip assembly equipment business for Kulicke & Soffa
- Diagnosed with ALS Nov. 2020
- Under care of “Dr. Merit” at MGH Healey ALS Center since Dec. 2020
- Joined Clene CNM-Au8 Expanded Access Program
- Founded the “TechVsALS” campaign in Feb. 2022

THANK YOU!
While over 80 promising new ALS therapies are in the clinical trial pipeline...

- Only 50% of persons with ALS are eligible to participate in clinical trials to gain access to these new life-extending or life-saving therapies.

- Phase 3 Clinical Trials + FDA approval of successful new therapies takes an average of 3-4 years, which exceeds ALS patients’ typical life expectancy.

There IS a solution... Expanded Access Programs.
Why is Expanded Access critical?

- Moral imperative – no more #DyingWaiting
- Expanded clinical learnings
- Supports therapy FDA approval by augmenting primary clinical trial data (& insurance approval...)
- Bridges the availability gap between successful trials and FDA approval
TechVsALS Strategy

- Partnership with the Healey Center
- Solicit sponsorship from entities in the "Tech" world for HEALEY PT Companion Expanded Access program to combat ALS
- Initiate EAP programs at sponsor-designated sites
- Companies, Foundations, Organizations, Individuals
HEALEY ALS Platform Trial

Weekly Q&A – July 13, 2023
HEALEY Platform Trial Companion EAP is Creating a Bridge to Livable ALS

Platform Trial Design
- Accelerated trial results
- Reduced placebo
- Lower costs

Active Treatment Phase
- Guaranteed patient access to active drug
- Longitudinal / survivability data
- Enhanced safety & efficacy data

Expanded Access
- Greatly increased ALS patient access to emerging treatments
- Broader trial data
- Bridges gap to regulatory approval

Greatest Unmet Need
The ALS drug development pipeline is robust
Efficient trials are needed to rapidly screen drugs and move them to Phase 3

ALS Clinical Trials Enrolling in the US (Phase 2, 2/3, 3)

More “shots on goal” leads to:
→ More promising therapies available for EAPs
→ More bridges to regulatory approval needed

Note: Trials are listed within the date range in which enrollment began
Kulicke & Soffa sponsors Companion EAP at Jefferson Weinberg ALS Center in Philadelphia

Ribbon cutting ceremony
31 Aug 2022
Life Changing Impact

"We strive to provide ALS patients, including for those that are not eligible for clinical trials, access to promising new therapies with good underlying science. The K&S gift supporting that mission not only established the Weinberg EAP but also has been pivotal in accelerating Expanded Access for ALS patients nationwide."

Dr. Merit Cudkowicz & AMG ALS Ctr at Mass General

"Since being on EAP my progression has been much, much slower than typical. This has allowed me to live a full life and ‘stay in the game’ as additional promising therapies come available!"

Shawn Sarbacker

"The EAP gave Joe a chance to receive a treatment that he would not have received otherwise. We are both thankful for the opportunity to have additional hope...it made us feel that Joe was still valued and seen."

Joe and Helen Brooks

"EAPs represent hope for those who wouldn't otherwise have access to any investigational therapies that are in clinical trial."

Philip Green

"Thanks to the funds provided by K&S via TechVsALS & the establishment of the EAP, we have been able to make promising therapies available to patients who wouldn’t have access to them. And this is priceless. There is no more pressing need than having access to these therapies."

Dr. Piera Pasinelli, Director, Jefferson Weinberg ALS Ctr

"Inclusion

Healey & AMG Center

Advancing Science

Access

Hope

Inclusion

Google “Curtis Richter Hope Experiment”
axeALS & Never Surrender Sponsor Three Additional Healey Platform Trial Companion EAP Sites

The axeALS Foundation in partnership with the Sean M. Healey & AMG Center for ALS establishes Expanded Access Protocol (EAP) Program at the University of California, Irvine

The nonprofit’s $485,000 donation provides options for ALS patients

Irvine, CA (March 10, 2023) — The axeALS Foundation is partnering with the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital to establish a new Expanded Access Protocol (EAP) program at the University of California, Irvine. Under the direction of, Namita Goyal, MD, professor of neurology, UCI School of Medicine, and director of UCI Health ALS Services, thanks to multyear support from the axeALS Foundation, UCI will add expanded access as an option for people living with ALS who do not qualify for trials.

“We are so excited to bring this EAP program to our ALS patients at UCI Irvine with these and other expanded access opportunities,” said Dr. Goyal.

Eric & Amanda Stevens
www.axeals.org

www.neversurrenderinc.org
ALS Expanded Access Progress

- Act for ALS was signed into law in Dec. 2021 providing substantial funding for EAPs.
- As of May 2024, over 400 participants have enrolled in EAPs across 21 investigational therapies [2x increase vs 2022].
- 45 sites are planned across the U.S. for the Pridopidine EAP and 10 are planned for RAPA-501 EAP [7x increase vs 2022].
- Even with this great progress, less than 10% of eligible ALS patients will have access to expanded access => more work to do!
What’s Next?

- Now widening scope of TechVsALS fundraising to support an innovative new Healey Platform Trial accelerator program
- Provide multi-year funding to augment staffing and infrastructure at leading sites
- Our goal: greatly accelerate trial enrollment and increase EAP access for all ALS patients
Here’s how you can help!

- Help us connect to companies, foundations
- Join our team – awareness, outreach, grassroots fundraising
- Donate today! Every dollar counts and goes directly to the Healey Center  www.techvsals.org

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Thank you!

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