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.

HEALEY ALS Platform Trial

Weekly Q&A – February 8, 2024

Massachusetts General Hospital
Founding Member, Mass General Brigham

HARVARD
MEDICAL SCHOOL

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

TACKLE ALS
"DON'T BE SORRY LETS BEAT THIS"

ALS ASSOCIATION

NEALS Northeast Amyotrophic Lateral Sclerosis Consortium

MDA Muscular Dystrophy Association

SALSA For a Cure

alsFINDINGaCURE

I AM ALS

TAMBOURINE

NCRI NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

BARROW Neurological Institute

ALS ONE

RUN2REVIVE MIND · BODY · POTENTIAL

Berry Consultants
Statistical Innovation

1850 MELIORA

ucb

biohaven pharmaceuticals

CLENE NANOMEDICINE

prilenia

THE ARTHUR M. BLANK FAMILY FOUNDATION

BASEBALL WINTER MEETINGS SAN DIEGO

SEELOS THERAPEUTICS

Calico

DENALI THERAPEUTICS

AMG The AMG Foundation

Guest Speakers

Rick Bedlack, MD, PhD, MS
(Duke University)

Gwen Petersen
(Patient Advocate)

My Perspective on Expanded Access Programs (EAPs) for People Living with ALS

Richard Bedlack MD PhD
Stewart, Hughes, and Wendt Distinguished Professor
Director of ALS Program
Duke University

My Job

- For 23 years, I have worked to create options and hope for people living with ALS (PALS)
  - Built multiple unique multidisciplinary ALS clinics and tele-ALS Clinics
    - “Fashionably Fighting ALS”
  - Led ALS Clinical Research Learning Institutes to empower PALS to be more effective research partners
  - In partnership with PALS, designed and participated in multiple research studies, including trials

A Problem…and A Solution

- Unfortunately, most of the PALS I have seen can’t participate in trials due to exclusions, travel burdens, unacceptable design features
  - These PALS “self experiment” with alternative and off-label treatments (AOTs) they find on the Internet
    - ALSUntangled can help them make more informed decisions, but there can still be questions and doubts about these products (ex. purity)
    - As a field, we do not learn anything from this self-experimentation

- EAPs
  - Provide a strong experimental option for PALS who cannot be in trials
  - Our field can lean from these

EAPs at Duke ALS Clinic

- 2018: BHV-0223 (n=1)
- 2022-2023: Verdiperstat (n=10)
- 2022-2023: AMX0035 (n=26)
- 2023-2024: CNM-Au8 (n=26)
- 2023-2024: Ibudilast (n=1)

- Coming later in 2024:
  - Pridopidine
  - CNM-Au8 (part 2)

Challenges I have Encountered with EAPs

- There aren’t enough of them
  - At Duke, 4000 PALS seen in 23 years, 64 got into an EAP

- They require site resources, some of which are not reimbursed
  - My first EAP took 4 hours of physician effort to get 1 thing for 1 PALS

- There remains a widespread lack of knowledge on how to get these open at sites

The Future

- More products will become available via EAPs
  - I would love to see every PALS who cannot be in a trial participate in an EAP

- More resources will become available to sites who want to offer EAPs (ex. Duke now has an entire office that will help with startup and conduct of EAPs; Act for ALS EAPs currently offer reasonable budgets)
  - I would love to see EAP endowments

- New training materials will empower knowledge of how to participate (ex. https://neals.org/als-trials/expanded-access; Annual EAP Training Program at NEALS meeting)
  - I would love to see a NEALS EAP mentoring program

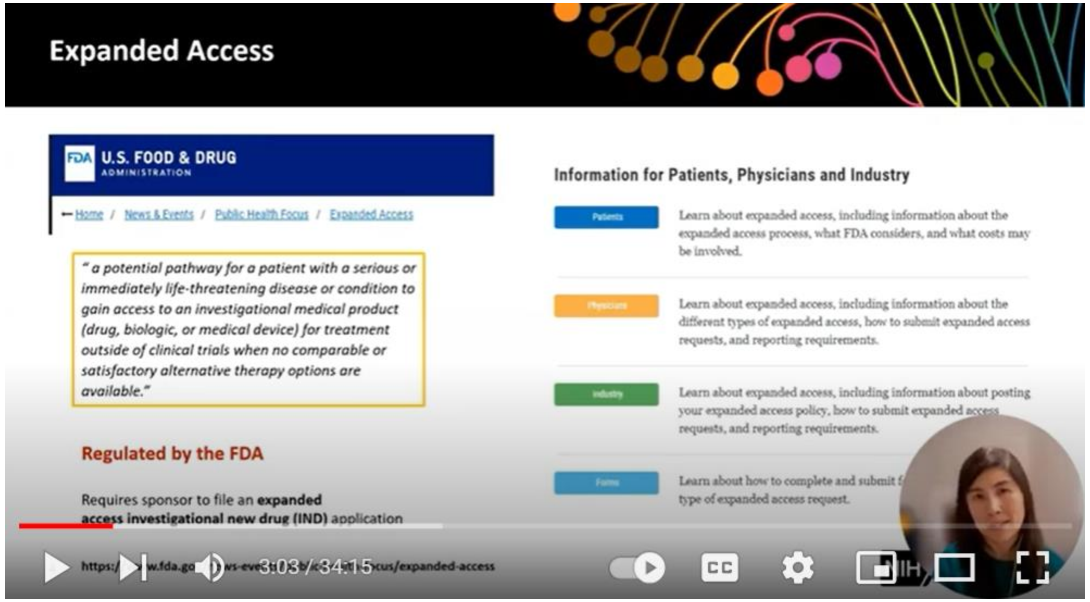
EAP Survey Presented by Gwen Petersen

Survey–

Q: If you have participated in an EAP or tried to get into an EAP, what was your experience like?

10 respondents - all people living with ALS

Monthly Expanded Access Q&A Webinars



"a potential pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available."

Regulated by the FDA

Requires sponsor to file an **expanded access investigational new drug (IND)** application

Information for Patients, Physicians and Industry

Learn about expanded access, including information about the expanded access process, what FDA considers, and what costs may be involved.

Learn about expanded access, including information about the different types of expanded access, how to submit expanded access requests, and reporting requirements.

Learn about expanded access, including information about posting your expanded access policy, how to submit expanded access requests, and reporting requirements.

Learn about how to complete and submit f

type of expanded access request.

January 11, 2024 –
EAP Discussion with the NIH
Recording: https://bit.ly/3HvfJCz

December 14, 2023 –
EAP Update with Dr. James Berry (MGH)
Recording: https://bit.ly/3UfCAJU

November 9, 2023 –
EAP Update with Dr. Suma Babu (MGH)
Recording: https://bit.ly/48HAEOy

March 14, 2024 – Thurs 5:00-5:30 EST
EAP Update with Dr. Jinsy Andrews (Columbia)

Expanded Access Resources and Webinar Opportunities

Join us for updates about Expanded Access and EAPs **on the second Thursday of each month!**

(or view recordings online)

https://bit.ly/3r6Nd2L

Visit the NEALS website to explore upcoming educational webinar opportunities or view previous recordings

https://bit.ly/3S4P0Sb

Find more webinar opportunities and recordings through the **International Alliance of ALS/MND Associations**

https://bit.ly/3Obj2m6

The ALS Association/Northeast ALS Consortium Educational Webinar

ALS Treatments: Riluzole, Radicava, and Relyvrio

Monday,
March 18th
4:30pm EST

Moderator: Senda Ajroud-Driss, M.D., Northwestern University

Register Here:

https://bit.ly/3HyYobW

Recording will later be available under "educational webinars" on neals.org

Patient Navigation
Central resource for people living with ALS

Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail:healeyalsplatform@mgh.harvard.edu

Weekly webinar registration:

https://bit.ly/3r6Nd2L

ALS Link sign-up:

https://bit.ly/3o2Ds3m

Upcoming Webinars:

February 15th- Weekly Q&A and Platform Trial Update

February 22nd- Genetics Discussion with Dr. Mark Garret (MGH)

February 29th- Weekly Q&A with Dr. Kourosh Rezania from UChicago