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

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
• 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
**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

January 11, 2024 –
EAP Discussion with the NIH

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)

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)

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

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

https://bit.ly/3r6Nd2L
https://bit.ly/3S4P0Sb
https://bit.ly/3Obj2m6
The ALS Association/Northeast ALS Consortium Educational Webinar

ALS Treatments: Riluzole, Radicava, and Relyvrio

Register Here:


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

Moderator: Senda Ajroud-Driss, M.D., Northwestern University
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

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

Weekly webinar registration: [QR Code]
ALS Link sign-up: [QR Code]

https://bit.ly/3r6Nd2L
https://bit.ly/3o2Ds3m