

HEALEY ALS Platform Trial

Weekly Q&A – July 22, 2021



Healey Center

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



The AMG Foundation

Guest Speaker

Richard Bedlack, MD, PhD
Duke University, NC
Platform Trial Site Investigator



My Story and Mission

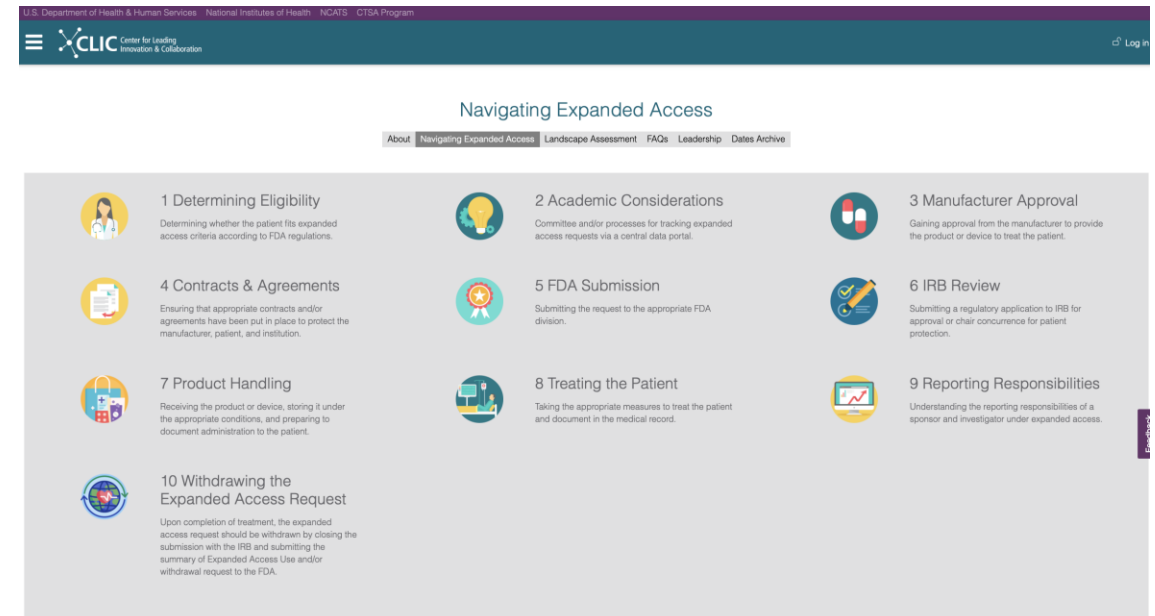
- First encounter
- Building Duke ALS Clinic Team
 - 17 providers, 9 disciplines
- Incorporating ALS Research
 - Part of multi-center trials (many!)
 - Investigator-initiated trials (ex. Levitiracetam, Lunasin, Triheptanoin, Theracurmin, Clenbuterol)
 - Translational (ex. St.A.R., Neurapheresis)
- Education and Advocacy
 - ALSUntangled
 - ALS CRLI
 - PEACe Committee



“Fashionably Fighting ALS”

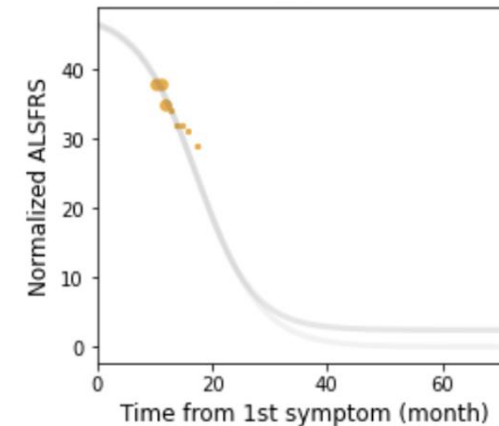
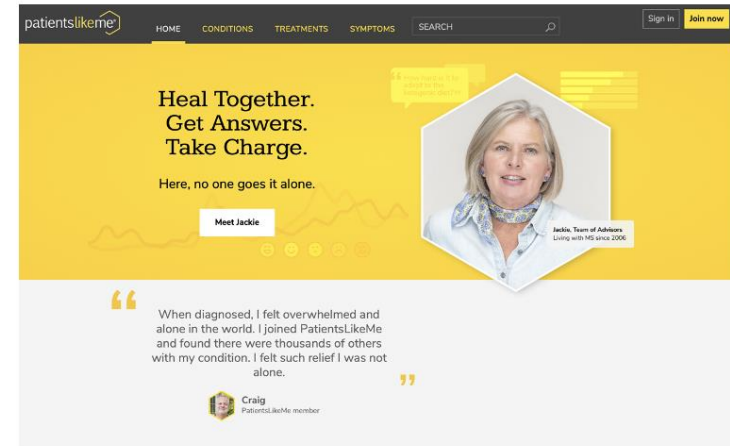
My Roles in Healey ALS Platform Trial

- Site investigator for trial (enrolling)
- Site leader for Verdiperstat EAP
- Developer of EAP educational materials
 - Written materials (to be housed on NEALS website)
 - Book chapter
 - Map and templates (modified from <https://clic-ctsa.org/groups/teamss/navigating-expanded-access>)
 - Virtual training course (to occur at NEALS meeting, be recorded and stored on website)



“Drugs in Bodies”

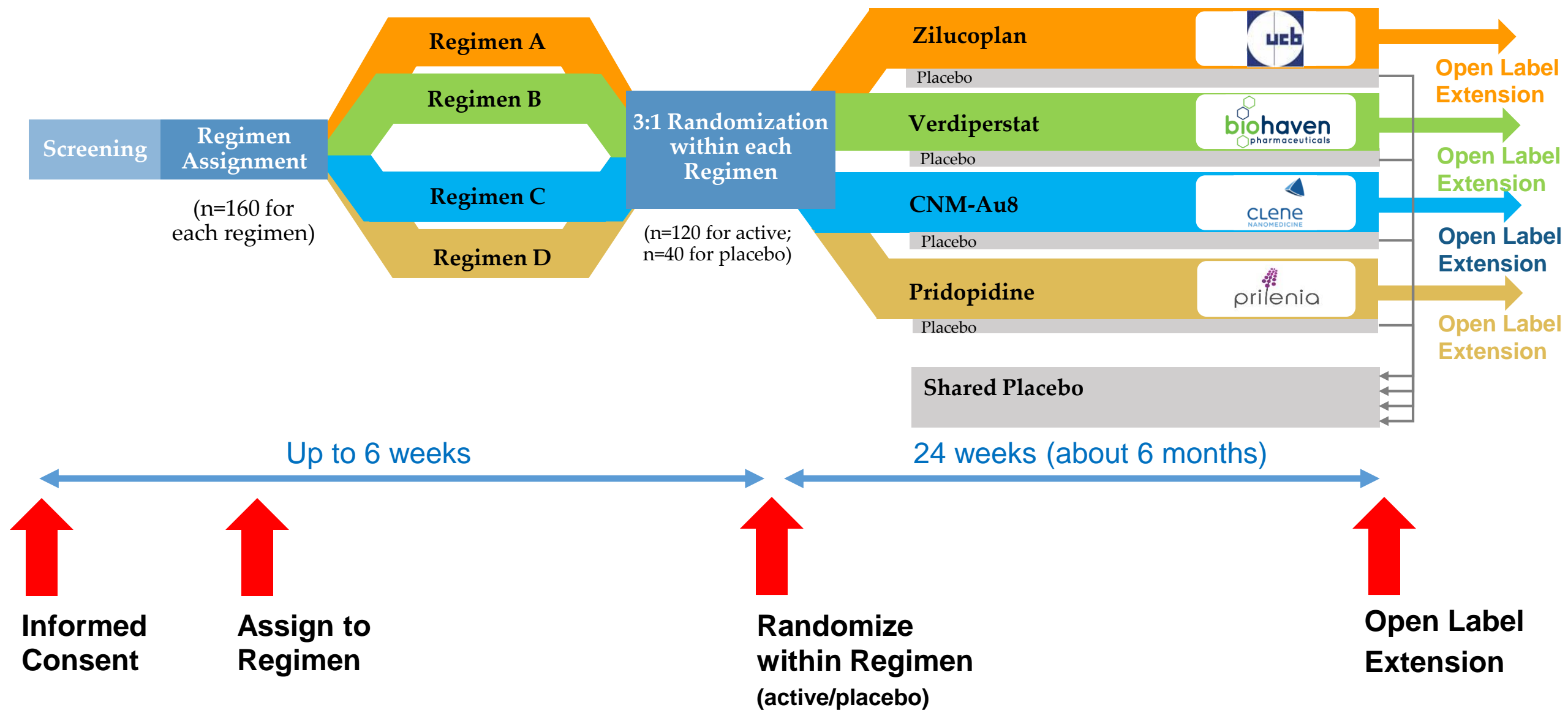
- I believe every person with ALS should have access to experimental therapies
 - Why: patients want them, “high-throughput screening”
 - How: trials, EAPS, alternative and off-label treatments (AOTs)
 - More trials than ever
 - Platform EAPs are a HUGE step forward (more spots, standardized documents, educational materials for neurologists, centralized data collection)
 - What we learn from Platform, new laws (“Act for ALS”) will facilitate more EAPs
 - For those who cannot find a trial or EAP, ALSUntangled (www.alsuntangled.org) can help people make more informed decisions about AOTs they might try, and a new “Personal Experience Tool” I am developing with PatientsLikeMe will facilitate determination of whether these might be working



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of July 22, 2021)

- **661** individuals with ALS signed informed consent
- **520** individuals were assigned to a regimen
- **468** individuals were randomized within a regimen (active or placebo)
- **146** have entered the Open Label Extension (OLE)

- **127** individuals were randomized within Regimen A
- **144** individuals were randomized within Regimen B
- **144** individuals were randomized within Regimen C
- **53** individuals were randomized within Regimen D

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 Guest Speakers:

July 29th- James Berry, MD, MPH (Mass General Hospital, MA)

Hristelina Ilieva, MD, PhD (Thomas Jefferson, PA)

Aug 12th- Michael Weiss, MD, FAAN (University of Washington, WA)

Aug 19th- Laura Foster, MD (University of Colorado, CO)