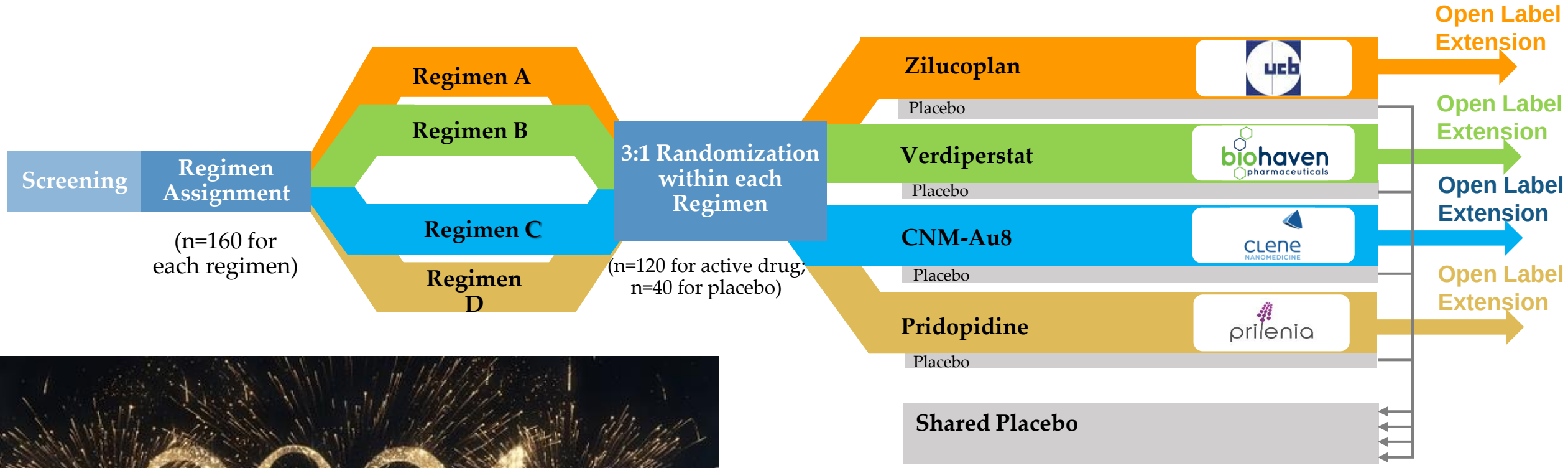
HEALEY ALS Platform Trial

Weekly Q&A – Dec 16, 2021

The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Regimens A, B and C completed enrollment!

- **162** individuals were randomized within Regimen A
- **167** individuals were randomized within Regimen B
- **161** individuals were randomized within Regimen C

159 individuals were randomized within Regimen D

324 have entered the Open Label Extension (OLE)

"I'm looking forward to helping find a cure for ALS."
-Platform trial participant

>800 people with ALS signed Informed Consent for the Platform Trial

Thank You

This breakthrough trial would not be possible without your partnership

as of 12/16/21

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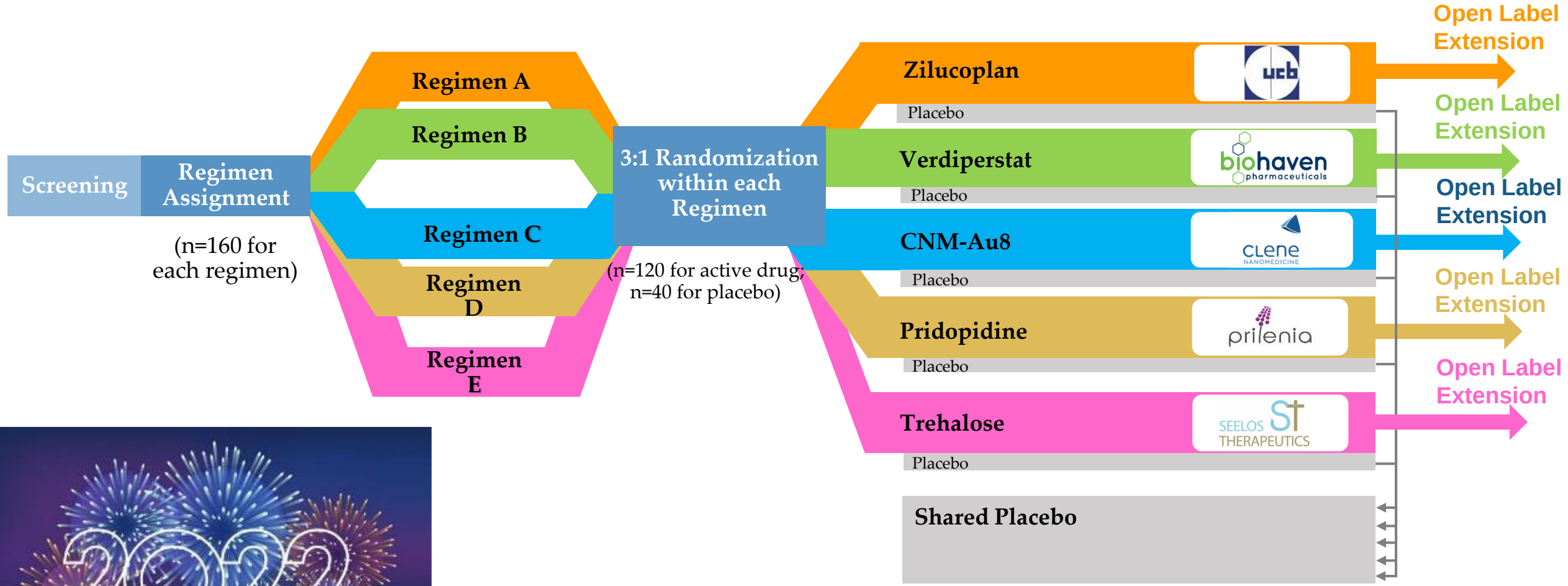


Send us webinar ideas!

Upcoming Guest Speakers:

Dec 23rd- No Webinar

Dec 30th- No Webinar

Jan 6th- Guest TBD

Jan 13th- Sharon Hesterlee, PhD of the Muscular Dystrophy Association

Weekly webinar registration:

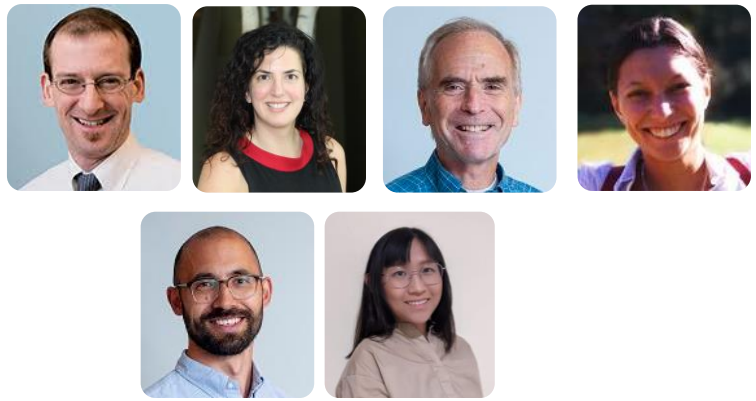
ALS Link sign-up:

Guest Speaker

Lori Chibnik, PhD, MPH
Assistant Professor & Biostatistician
Harvard TH Chan School of Public Health & MGH

Trial Statisticians

MGH Biostatistics



Eric Macklin, PhD; Lori B. Chibnik, PhD, MPH; Douglas Hayden, PhD; Marie-Abele Bind, PhD; James Chan, MA; PoYing Lai, MS

HARVARD MEDICAL SCHOOL

HARVARD T.H. CHAN SCHOOL OF PUBLIC HEALTH

MASSACHUSETTS GENERAL HOSPITAL

Berry Consultants

Michelle Detry, PhD; Melanie Quintana, PhD; Ben Saville, PhD; Matteo Vestrucci, PhD

Berry Consultants
Statistical Innovation

BIOSTATISTICS WEBINAR SERIES

Impetus

- Curated questions from
  - Past webinars
  - Facebook sessions and AMA
  - Emailed questions

What determined n=160 as the magic number for each regimen?

How does the platform randomization work given that there are 3 drugs that have over 200 patients enrolled, the placebo, and now Pridopidine is new with just 1 patient?

For the next round, will the current placebo data be combined with that to be acquired in the next round?

One of the slides mentioned a non-statistically significant difference. Can you explain that please, since you were so positive on the positive results.

If someone re-randomizes into a second regimen, is it possible then could be randomized into placebo 2x?

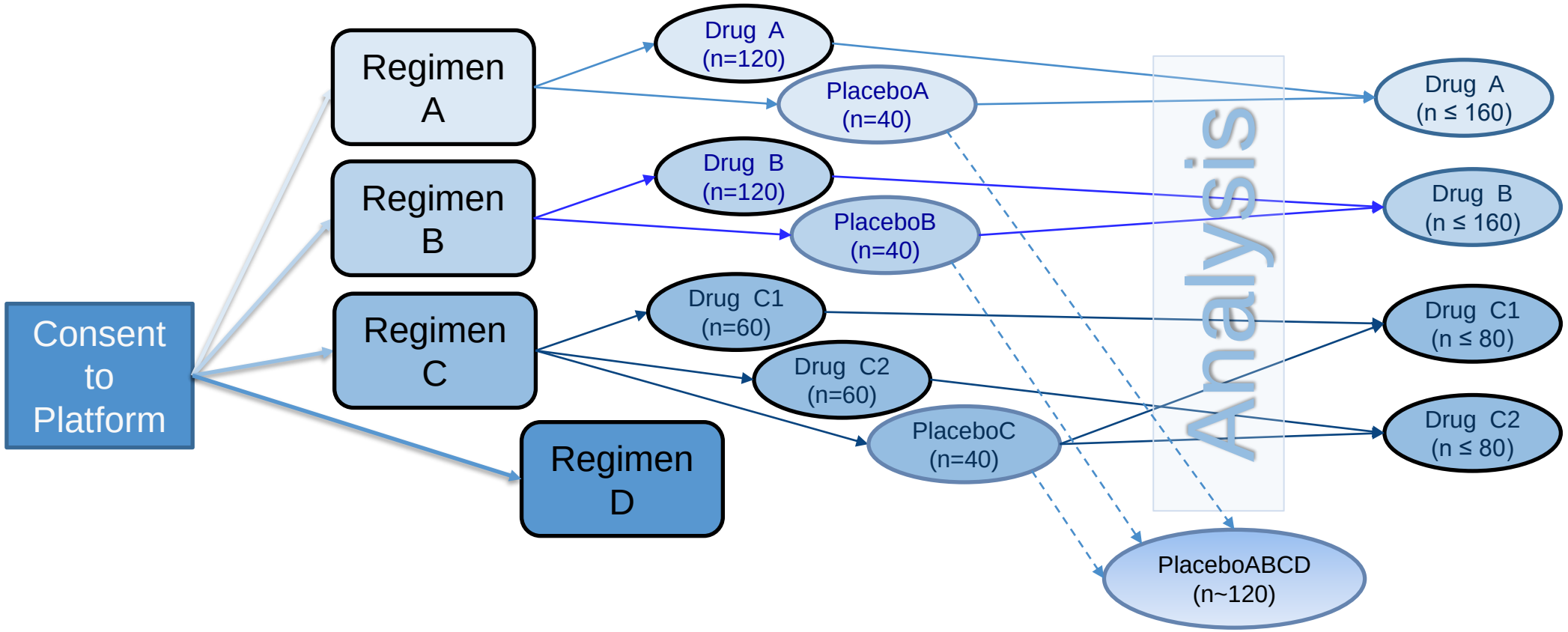
How come trials are set up in a way to “prove statistical significance”, but even when they are deemed “positive”, they are not “significant” ENOUGH for the FDA?

How are deaths included in the statistical analysis of adverse consequences if the death is from ALS and not trial complications?

Can you explain to people how the FDA's statistical penalty works for manufacturers who decide to do an interim analysis and how you or a manufacturer decides if they will be doing those in the Healey trials?

ALS PLATFORM TRIAL THROUGH THE EYES OF A STATISTICIAN

Healey ALS Platform trial



unblinded unblinded blinded *unblinded*

Healey ALS Platform trial

Study design

'clinically meaningful' results

Sample Size

Randomi-zation