HEALEY ALS Platform Trial

Weekly Q&A – Jan 26, 2023

Healey Center
Sean M. Healey & AMG Center for ALS at Mass General
Regimen E Updates (as of Jan 26, 2023)

- 212 participants consented to Master Protocol since RGE initiated
- 157 participants assigned to RGE
- 148 participants randomized within RGE

148 out of 160 participants randomized within RGE
Schema for Each Regimen

- **Screen for eligibility**
- **Randomization 3:1**
- **Placebo**
- **Active**

**Screening Period**

**Randomized Period**
- 24 weeks

**Open-Label Extension Period**
- (Active Treatment Extension)
October 2022: **Regimen C** top line results announced

While the primary endpoint was not met, exploratory analyses of the secondary survival endpoint demonstrated a significant reduction in risk of death or permanently assisted ventilation when adjusting for baseline risk imbalances in the CNM-Au8 regimen for the 30 mg dose.

Based on Phase 2 study results, Clene is preparing for a Phase 3 Trial of CNM-Au8.

Early 2023: **Regimen C** Open Label Extension is closing.

CNM-Au8 Expanded Access Protocol (EAP) is launching.
Open Label Extension (OLE) to transition to Clene Expanded Access Program (EAP)

- Participants in OLE for Regimen C will be prioritized for the Clene EAP
- During transition from OLE to EAP, participants in Regimen C will have option to transfer to a different Platform Trial site
- EAP broadens access to CNM-Au8, so it will be available to more people (including those who were not eligible to participate in Platform Trial Regimen C)
- EAP details, eligibility criteria, site locations, and contact info will be posted on ClinicalTrials.gov
CNM-Au8 Q&A

James Berry, MD, MPH
Regimen Co-Lead (Regimen C)
Massachusetts General Hospital

Michael Hotchkin
Chief Development Officer
Clene Nanomedicine, Inc.