

Functional and Long-Term Survival Data of AMX0035 in Amyotrophic Lateral Sclerosis: Results of the CENTAUR Trial

Sabrina Paganoni^{1,2}, Suzanne Hendrix³, Sam Dickson³, Newman Knowlton³, Joshua Cohen⁴, Justin Klee⁴, Kent Leslie⁴, Rudolph E. Tanzi¹, Patrick D. Yeramian⁴, David Schoenfeld⁵, Merit E. Cudkowicz¹, on behalf of the CENTAUR Study Group

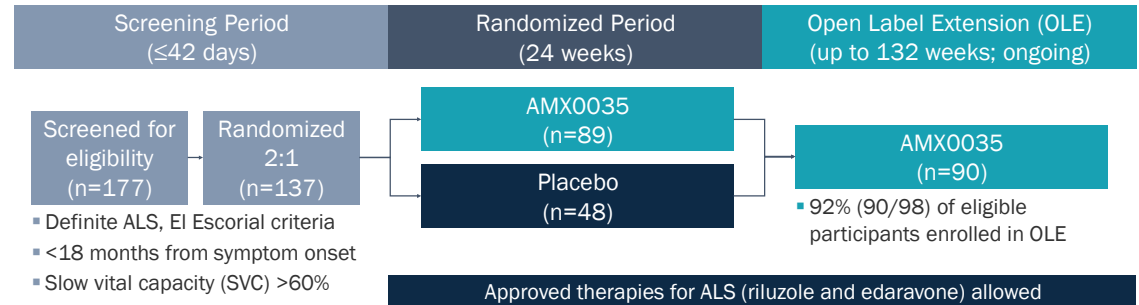
¹Sean M. Healey and AMG Center for ALS & the Neurological Clinical Research Institute, Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts; ²Spaulding Rehabilitation Hospital, Harvard Medical School, Boston, Massachusetts; ³Pentara Corporation, Millcreek, Utah; ⁴Amylyx Pharmaceuticals, Inc., Cambridge, Massachusetts; ⁵Biostatistics Center, Massachusetts General Hospital, Harvard Medical School, Boston, Massachusetts



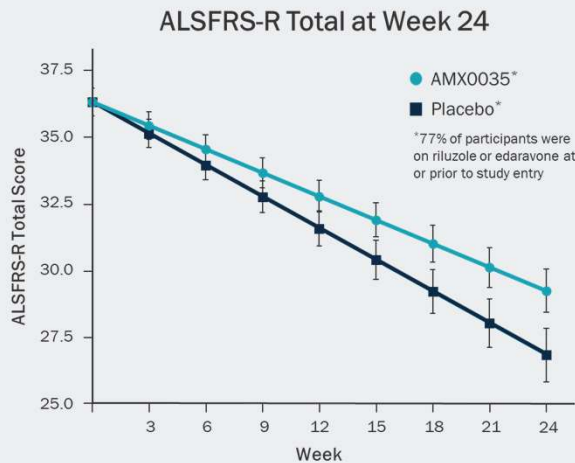
AMX0035 is an oral, fixed-dose coformulation of the compounds sodium phenylbutyrate (PB) and taurursodiol (TURSO) designed to reduce neuronal death in people with ALS by simultaneously mitigating endoplasmic reticulum stress and mitochondrial dysfunction. CENTAUR was a randomized, double-blind, placebo-controlled trial conducted at 25 centers of the Northeast Amyotrophic Lateral Sclerosis Consortium (NEALS) in the United States from June 2017 through September 2019

- Packaged in sachets (3g PB, 1g TURSO per sachet)
- Dissolved in water and administered BID by mouth or via feeding tube
- Taste, appearance, and solubility matched to placebo

CENTAUR Phase 2 Study Design



Change of slope in ALSFRS-R (Primary Efficacy Endpoint) Randomized Period



- Significant slowing of functional decline among participants randomized to AMX0035 relative to placebo
- Difference: 0.42 points/mo, (95% CI 0.03–0.81), P=0.03
 - 2.32 points at the end of the six-month study

References

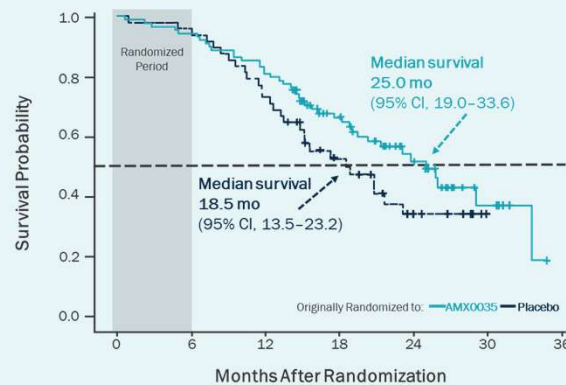
1. Paganoni S, et al. N Engl J Med. 2020;383:919-930.
2. Paganoni S, et al. Muscle Nerve. 2020. <https://doi.org/10.1002/mus.27091>.

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Prespecified Overall Survival Analysis Spans Randomized Period and OLE

Overall Survival for All Participants Randomized in CENTAUR



- Risk of death was 44% lower in the originally randomized to AMX0035 group; HR 0.56 (95% CI 0.34-0.92), P=0.02
- 6.5 month longer median survival in the group originally randomized to AMX0035

Safety Randomized Period

- Nearly all participants (AMX0035, 97%; placebo, 96%) reported one or more AEs during the trial. Most did not lead to modification or interruption of study drug dosing and were not considered related to treatment
- Events that occurred with greater ($\geq 2\%$) frequency in the AMX0035 group were primarily gastrointestinal



- Administration of AMX0035 is associated with both a statistically significant functional and survival benefit in people with ALS
- Similar rates of adverse events were recorded in the AMX0035 and placebo groups during the 24-week randomized period

For more information, join the Pipeline Session Oral Presentation Tuesday, February 23 11:30 AM EST

Disclosures

SP reports research grants from Amylyx, Revaliesio Corporation, Ra Pharma, Biohaven, Clene, Prilenia, The ALS Association, the American Academy of Neurology, ALS Finding a Cure, the Salah Foundation, the Spastic Paraplegia Foundation, and the Muscular Dystrophy Association, and consulting fees from Orion and Amylyx; SH, SD, NE, KH, and NK report other personal fees from Pentara Corporation; JC, JK are co-CEOs of Amylyx Pharmaceuticals; KL and PY are full-time employees of Amylyx Pharmaceuticals; RT reports founding equity in Amylyx and is the head of their Scientific Advisory Board; DS reports grants from The ALS Association and consulting fees from Immunity Pharma and Alexion; MC reports grants from Mass General Hospital during the conduct of the study; and grants from Clene Nanomedicine, Ra Pharma, Biohaven, and Prilenia; consulting fees from Amylyx, Takeda, Biogen, Wave Life Sciences, QurAlis, Avexis, Disarm, ALSpharma, Helixmith, Orion, Transposon, Cytokinetics, and Immunity Pharma.

