Evidence for an ALS Survival Benefit with CNM-Au8 Treatment: Interim Results from the RESCUE-ALS Trial Long-Term Open Label Extension
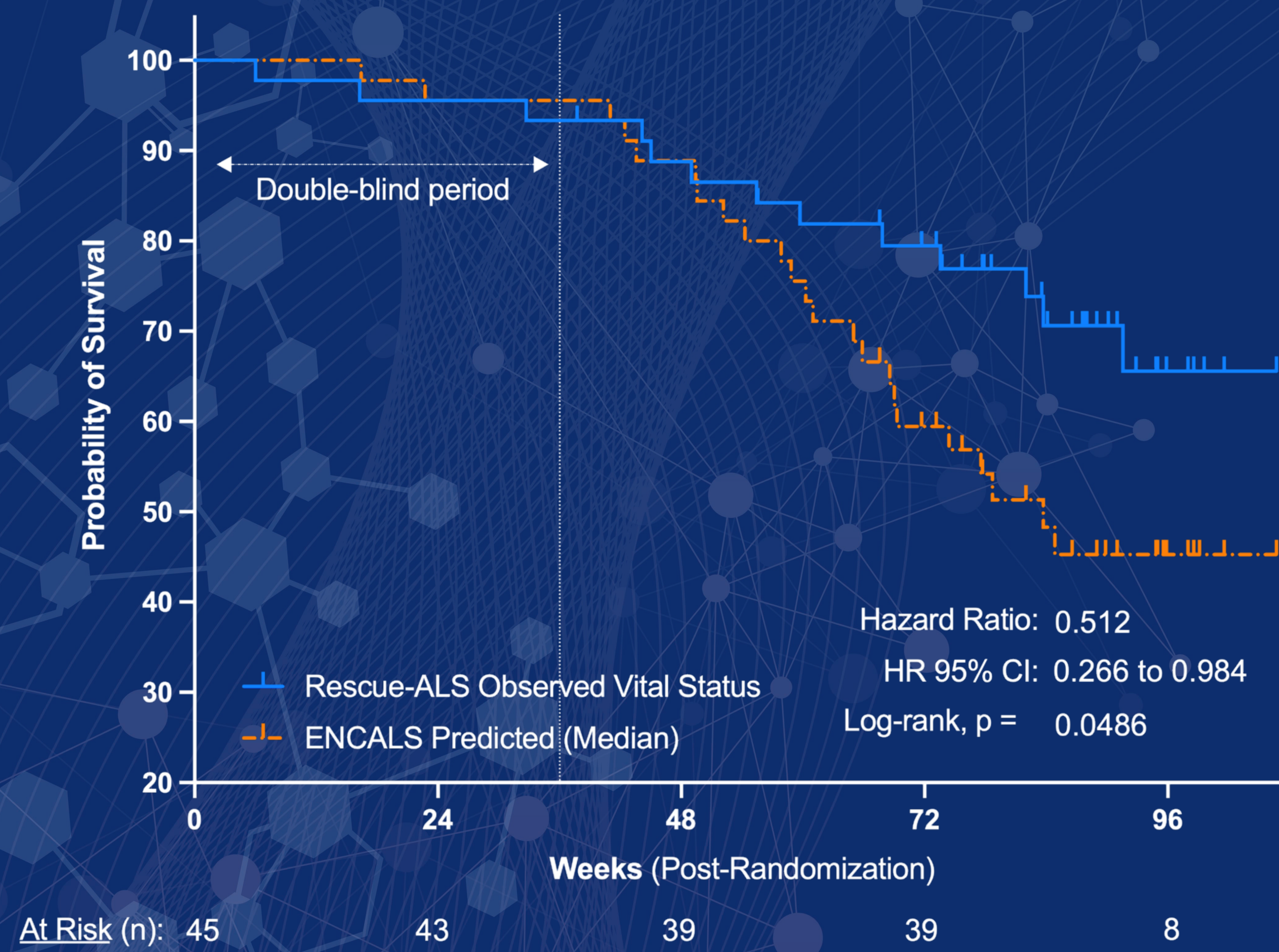
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[1]Concord Repatriation General Hospital, University of Sydney, Australia; [2]Brain and Mind Centre, University of Sydney, Australia; [3]Clene Nanomedicine, Salt Lake City, UT, USA
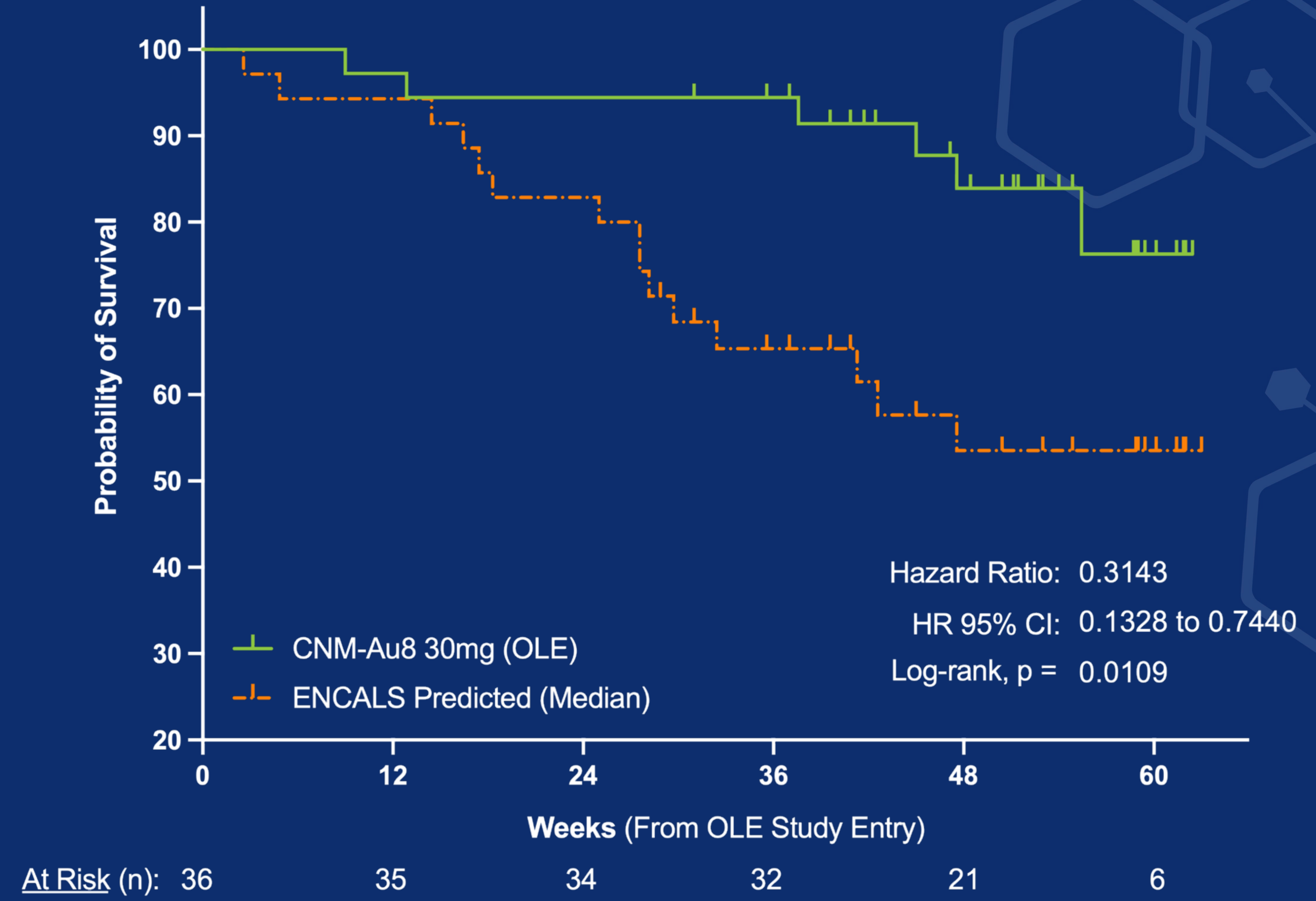
All Randomized

RESCUE-ALS Long-Term Observed Survival (All Randomized) vs. ENCALS Predicted Median Survival
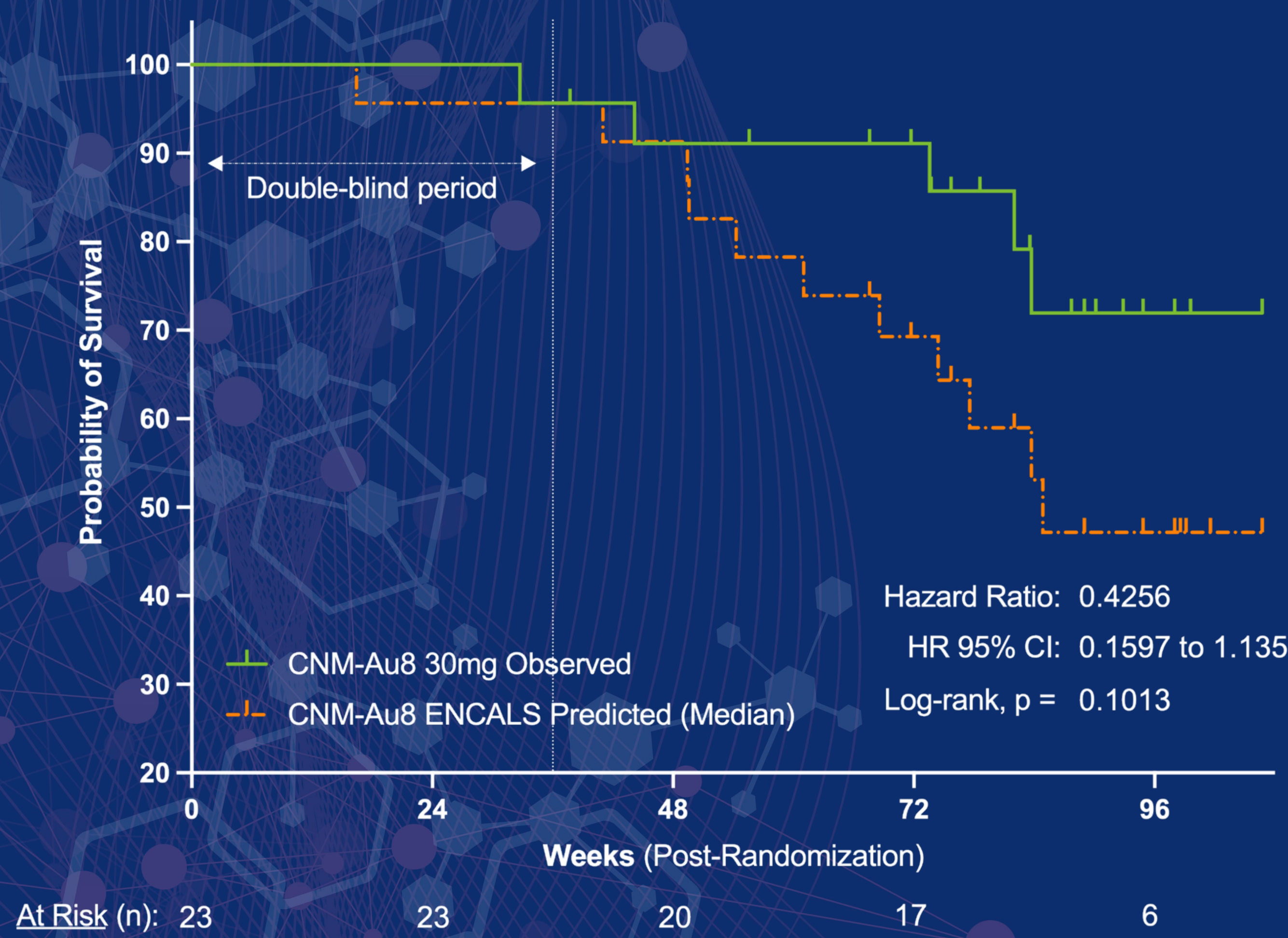ITT Population, All Subjects from Randomization (Active & Placebo)

Double-blind period

- Rescue-ALS Observed Vital Status
- ENCALS Predicted (Median)

Hazard Ratio: 0.512
HR 95% CI: 0.266 to 0.984
Log-rank, p = 0.0486

At Risk (n): 45   43   39   39   8
Weeks (Post-Randomization)

All OLE Participants (CNM-Au8 Treated)

RESCUE-ALS Long-Term Observed Survival (OLE Participants) vs. ENCALS Predicted Median Survival
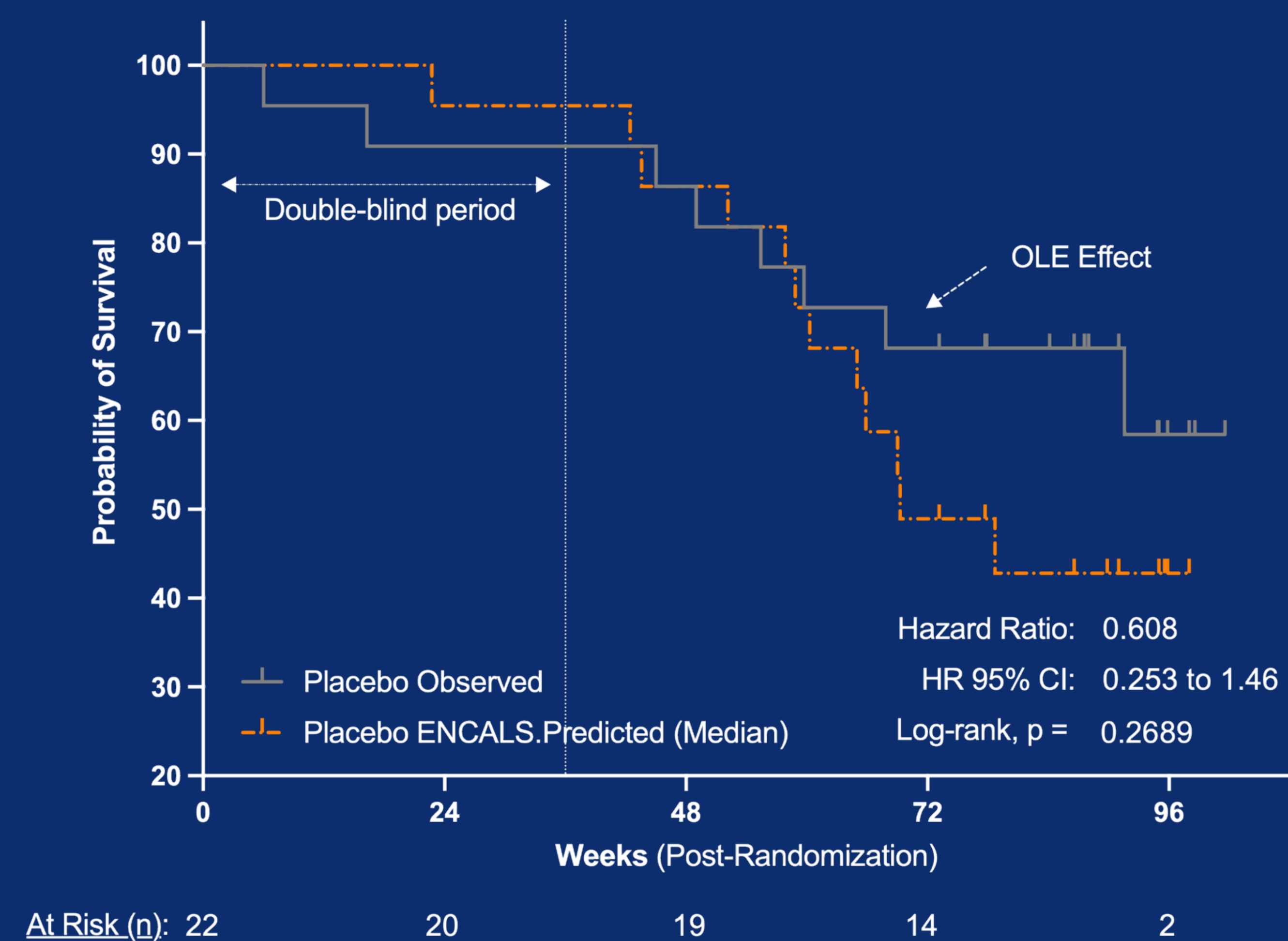All Open Label Extension Participants

- CNM-Au8 30mg (OLE)
- ENCALS Predicted (Median)

Hazard Ratio: 0.3143
HR 95% CI: 0.1328 to 0.7440
Log-rank, p = 0.0109

At Risk (n): 36   35   34   32   21   6
Weeks (From OLE Study Entry)

All CNM-Au8 Randomized

RESCUE-ALS Original CNM-Au8 Randomized Long Term Observed Survival vs. ENCALS Predicted Median Survival
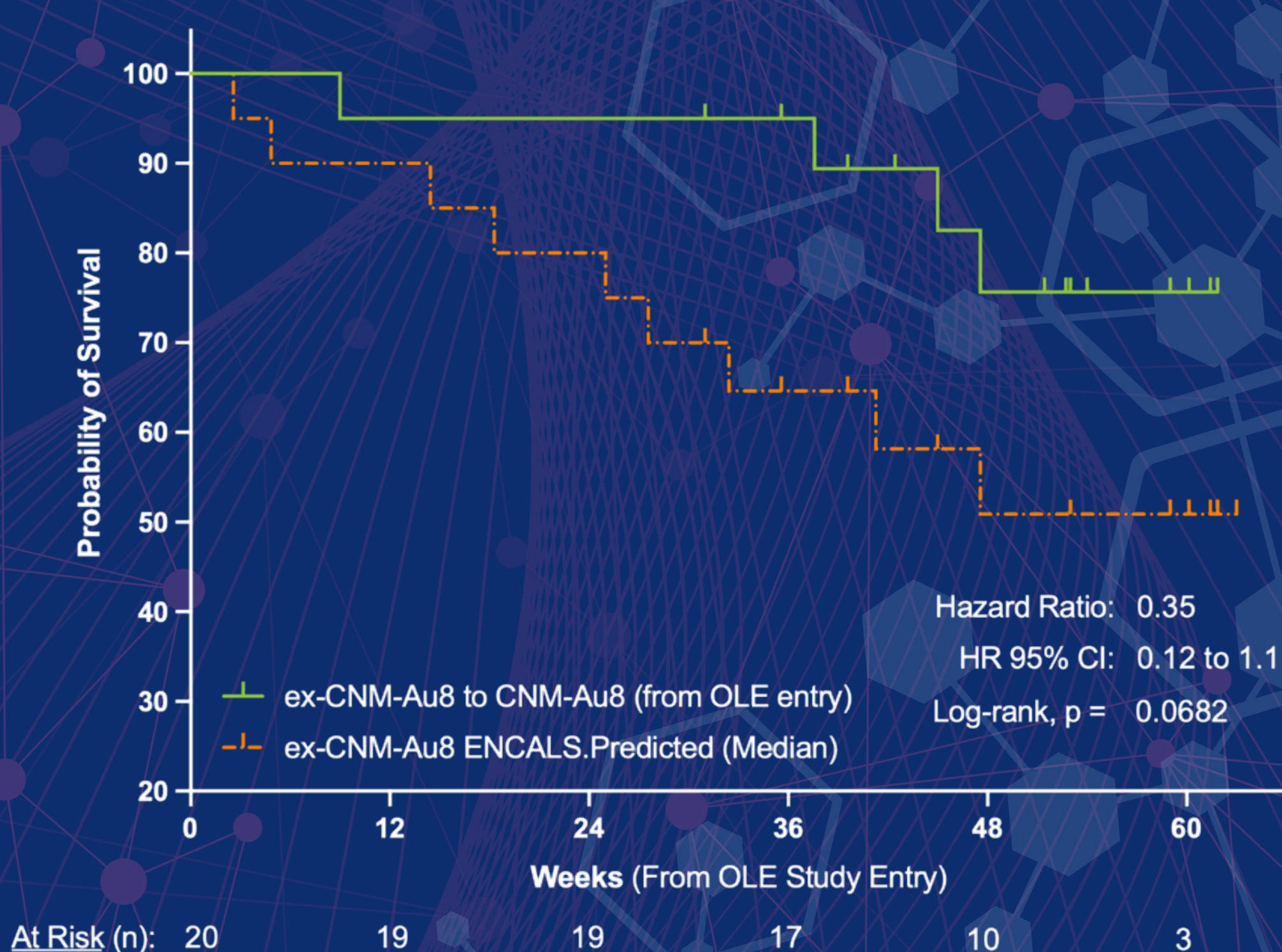All CNM-Au8 Treated Subjects, Survival from Randomization, ITT Population

Double-blind period

- CNM-Au8 30mg Observed
- CNM-Au8 ENCALS Predicted (Median)

Hazard Ratio: 0.4256
HR 95% CI: 0.1597 to 1.135
Log-rank, p = 0.1013

At Risk (n): 23   23   20   17   6
Weeks (Post-Randomization)

All Placebo Randomized

RESCUE-ALS Original Placebo Randomized Long-Term Observed Survival vs. ENCALS Predicted Median Survival
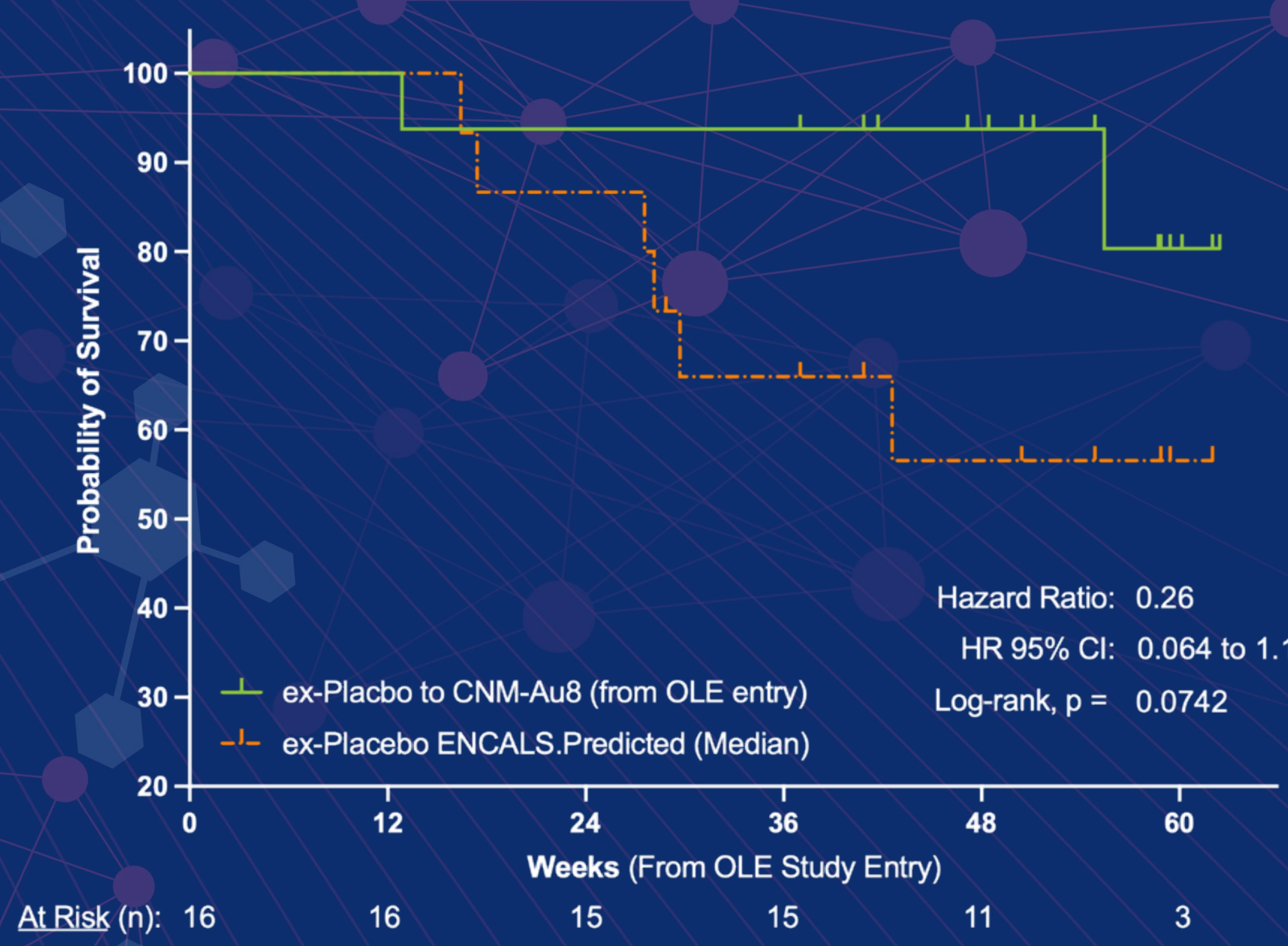All Placebo Treated Subjects, Survival from Randomization, ITT Population

Double-blind period

OLE Effect

- Placebo Observed
- Placebo ENCALS Predicted (Median)

Hazard Ratio: 0.608
HR 95% CI: 0.253 to 1.46
Log-rank, p = 0.2689

At Risk (n): 22   20   19   14   2
Weeks (Post-Randomization)

CNM-Au8 OLE

RESCUE-ALS CNM-Au8 to CNM-Au8 OLE Long-Term Observed Survival vs. ENCALS Predicted Median Survival
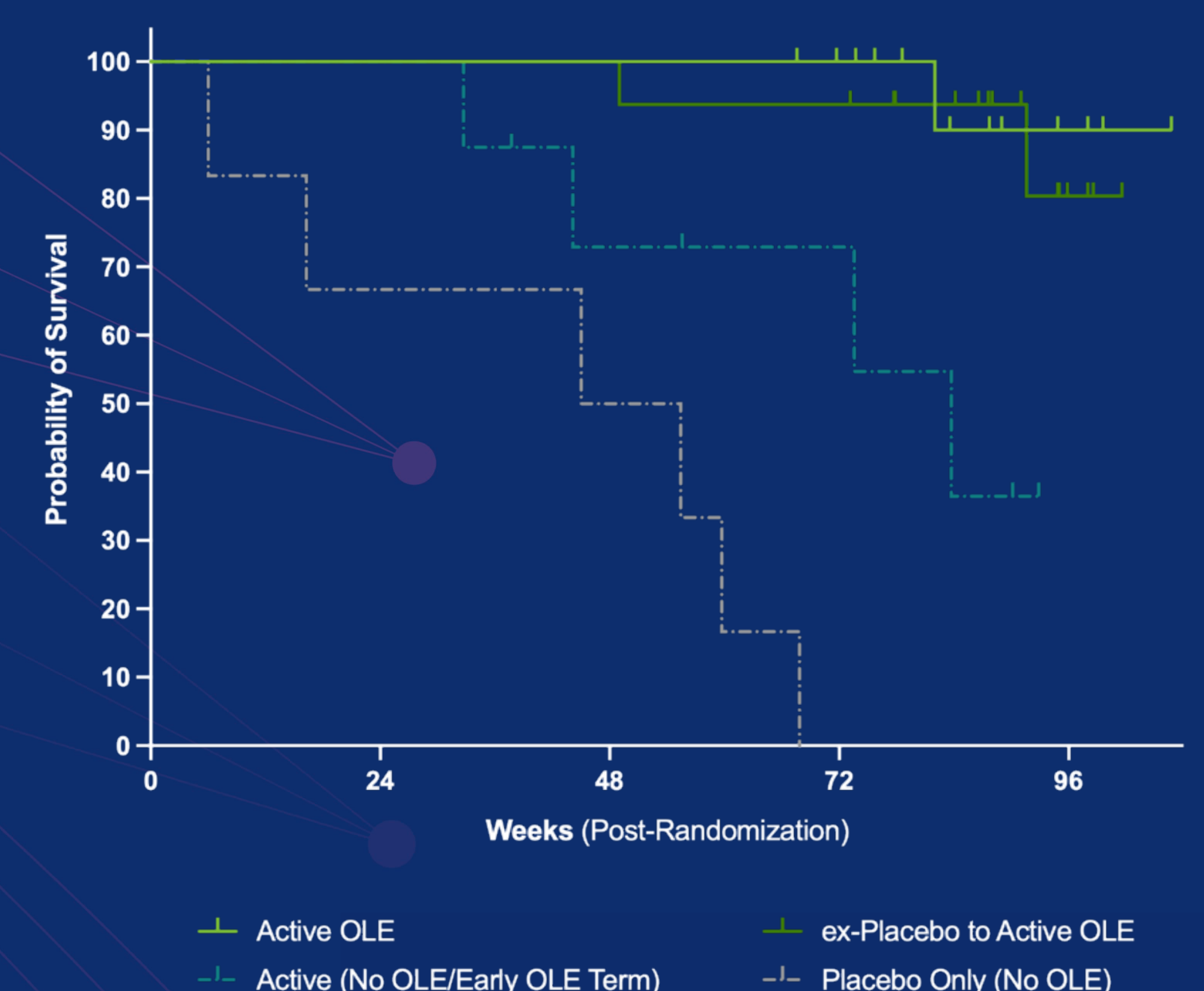All CNM-Au8 Subjects Entering OLE, Survival from OLE Entry, ITT Population Subset

- ex-CNM-Au8 to CNM-Au8 (from OLE entry)
- ex-CNM-Au8 ENCALS.Predicted (Median)

Hazard Ratio: 0.35
HR 95% CI: 0.12 to 1.1
Log-rank, p = 0.0682

At Risk (n): 20   19   19   17   10   3
Weeks (From OLE Study Entry)

ex-Placebo to CNM-Au8 OLE

RESCUE-ALS ex-Placebo (to CNM-Au8 OLE) Long-Term Observed Survival vs. ENCALS Predicted Median Survival
All Placebo Subjects Entering OLE, Survival from OLE Entry, ITT Population Subset

- ex-Placbo to CNM-Au8 (from OLE entry)
- ex-Placebo ENCALS.Predicted (Median)

Hazard Ratio: 0.26
HR 95% CI: 0.064 to 1.1
Log-rank, p = 0.0742

At Risk (n): 16   16   15   15   11   3
Weeks (From OLE Study Entry)

Survival by OLE Status

Rescue-ALS Study Participants Long-Term Observed Survival by OLE Entry Group

- Active OLE
- Active (No OLE/Early OLE Term)
- ex-Placebo to Active OLE
- Placebo Only (No OLE)

Weeks (Post-Randomization)

RESCUEALS

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