I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Healey Platform Trial Regimen's A, B, & C

I AM ALS Patient Centric Trial Design (PaCTD) The HEALEY ALS Platform Trial tests multiple treatments in one trial. This listing will be updated if additional drugs are added to the trial.	**Platform Trial** **Clene Nanomedicine** **CNM-Au8** **Biohaven Pharmaceutical Holding Co** **Verdiperstat** **Ra Pharmaceuticals** **Zilucoplan**	
Open Label Extension	Yes - up to 1 year +	1
Minimize placebo usage - 33% or less	Yes (25%)	1
A side by side Expanded Access Program	CNM-Au8 - Yes Verdiperstat - Yes Zilucoplan - Pending[1]	1[2]
Part 1 Total	**3**	**3[3]**
Part 1 Rating-Seats at the Table	**0.6**	**0.6[4]**
Consideration of disease heterogeneity: e.g., Cross-Over Design or Delayed Start Design	Yes	1
Use of scientifically supportable inclusion criteria, pre-defined subset analysis, re-randomization at trial conclusion to equalize outlier progressors between trial arms, or alternative controls (historical, algorithmic etc.)	Yes (36 months from symptoms). No upper age limit.	1
Investigation of biomarker	Yes	1
Independent Unblinded Review Panel that can communicate with FDA where substantial proof of "efficacy" emerges before end of trial	No	0

[1] Ra Pharmaceuticals' Zilucoplan Expanded Access Program is pending.
[2] Ra Pharmaceuticals' Zilucoplan rating is 0 until the Expanded Access Program begins.
[3] Ra Pharmaceuticals' Zilucoplan receives a 2 until the Expanded Access Program begins.
[4] Ra Pharmaceuticals' Zilucoplan receives a 0.4 until the Expanded Access Program begins.

Part 2 Total	**3**	**3**
Part 2 Rating-Advancing Science Quickly	**0.225**	**0.225**
Use of Run-In Observation Period - 3 months not acceptable -1 month ideally	No	1
Use of novel methods: wearables, telemedicine visits, financial reimbursement	Yes	1
Part 3 Total		**2**
Part 3 Rating-Patient-Friendly		**0.1**
Total Rating		**0.925**[5]
x 5		**4.625**[6]
I AM ALS PaCTD 5-Star Rating:		**5-Star**[7]

[5] Ra Pharmaceuticals' Zilucoplan receives a 0.725 until the Expanded Access Program begins.
[6] Ra Pharmaceuticals' Zilucoplan receives a 3.625 until the Expanded Access Program begins.
[7] Ra Pharmaceuticals' Zilucoplan receives a 4-Star rating until the Expanded Access Program begins. A 5-Star rating is an average of the three drugs in the trial.