I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Orphazyme's Arimoclomol

I AM ALS Patient Centric Trial Design (PaCTD)	Orphazyme Arimoclomol[1]	
Open Label Extension	Yes-18 months	1
Minimize placebo usage - 33% or less	Yes (33%)	1
A side by side Expanded Access Program	No	0
Part 1 Total		**2**
Part 1 Rating-Seats at the Table		**0.4**
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.)		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
Part 2 Total		**3**
Part 2 Rating-Advancing Science Quickly		**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 burden	telemedicine visits, travel reimbursement, drug shipped to home, home nursing visits	1
Part 3 Total		**2**
Part 3 Rating-Patient-Friendly		**0.1**
Total Rating		**0.725**

[1] Orphazyme's clinical trial design was created before the FDA updated its ALS clinical trial guidance in the Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment Guidance for Industry in September 2019.

x5		**3.625**
I AM ALS PaCTD 5-Star Rating:		**4-Star**