I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Apellis' Pegcetacoplan

I AM ALS Patient-Centric Trial Design (PaCTD)	Apellis Pegcetacoplan	
Open Label Extension	Yes	1
Minimize placebo usage - 33% or less	33% placebo	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.)	Yes	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	Yes	1
Part 3 Total		**2**
Part 3 Rating-Patient-Friendly		**0.1**
Total Rating		**0.725**
x5		**3.625**
I AM ALS PaCTD 5-Star Rating:		**4-Star**

I AM ALS Patient-Centric Trial Design (PaCTD)	Cytokinetics Courage Reldesemtiv	
Open Label Extension	Yes	1
Minimize placebo usage - 33% or less	33% placebo	1
A side by side Expanded Access Program	Enrolling 550 in COURAGE. All eligible for OLE + EAP participants in prior trials	1
Part 1 Total		**3**
Part 1 Rating-Seats at the Table		**0.6**
Consideration of disease heterogeneity: e.g., Cross-Over Design or Delayed Start Design	Yes; cross over	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; Two years from symptom onset. Vital capacity of 65%. ALS-FRS-R of 44 or less. Riluzole and Radicava are allowed	1
Investigation of biomarker	Yes; serum (blood), DNA, DME, muscle strength, PROs	1
Independent Unblinded Review Panel that can communicate with FDA where substantial proof of “efficacy” emerges before end of trial	Yes; In the second interim analysis	1
Part 2 Total		**4**
Part 2 Rating-Advancing Science Quickly		**0.3**
Use of Run-In Observation Period - 3 months not acceptable -1 month ideally	No	1
Use of novel methods: wearables, telemedicine visits, financial burden	Yes; Novel methods; telemedicine visits, mobile phone apps, home nursing visit: remote labs, spirometry	1
Part 3 Total		**2**
Part 3 Rating-Patient-Friendly		**0.1**
Total Rating		**1**

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

I AM ALS Patient-Centric Trial Design (PaCTD)	**AB Science Mastinib**	
Open Label Extension		1
Minimize placebo usage - 33% or less	33% placebo	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		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	12 week run-in	0
Use of novel methods: wearables, telemedicine visits, financial burden	Taxi reimbursement	0
Part 3 Total		**0**
Part 3 Rating-Patient-Friendly		**0**
Total Rating		**0.625**
x5		**3.125**
I AM ALS PaCTD 5-Star Rating:		**3-Star**