



I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Alexion's Ultomiris

I AM ALS Patient Centric Trial Design (PaCTD)	Alexion Ultomiris	
Open Label Extension	Yes - 2 years	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	Subset Analysis & NFL	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.)	No age restriction, symptom onset 36 months, Riluzole and Radicava fine	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	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