I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Biogen's ATLAS

I AM ALS Patient-Centric Trial Design (PaCTD)		
Manufacturer: Biogen	**NCT04856982**	
Therapy: BIIB067 (Tofersen)	**ATLAS**	Rating
Open Label Extension Rating: 0 if not offered, .5 or 1 depending on if announced/implemented, how the OLE is structured, looking at length of time, amount of patient data collected that can help in the approval process, etc.	Open label extension is offered to as many participants as possible given study design. Two yrs minimum; can be up to 5.5 years depending on study enrollment date	1
Minimize placebo usage Rating: 0, .5 or 1 depending on how progressive design is. Are the odds of receiving placebo less than 50%? For example the Healy Platform trial only randomizes 25% of participants into the shared placebo control and received a score of 1. Traditional 50/50 randomization gets a 0 score.	Score of zero for 1:1 placebo ratio however the sponsor's rationale is sound given anticipated effect size and recruiting challenges.	0
Expanded Access Program (EAP) A side by side Intermediate (or larger) Rating: 0 if not offered, .25 proposed, .5 filed with FDA, .75 approved by FDA, 1 implemented. Other considerations: number of slots, time length and amount of patient data collected that can help in the approval process, or, once drug is approved, to help convince payors to cover all, policy posted on company website	EAP is not a consideration for this trial since toferson is a marketed product.	N/A
Part 1 Total		1
Part 1 Rating-Seats at the Table		0.3
Advancing Science Quickly A trial is awarded a rating of 0-1.0 depending on whether it incorporates design elements that may increase the chance of producing definitive trial results and advance ALS science.The following list provides examples but is not exhaustive. • Consideration of disease heterogeneity such as using a predictive algorithm for trial inclusion or a crossover design • Investigation of potentially regulatory grade biomarkers such as neurofilament light or digital biomarkers such as	Innovative and inclusive by the nature of the trial. Four part study design allows individuals to be studied at various phases of the disease	1

accelerometers. ● Independent unblinded review panel for interim efficacy check-ins if warranted		
Part 2 Total		1
Part 2 Rating-Advancing Science Quickly		0.3
Minimize Use of Run-In Observation Period and Washout Period Rating: 0. .5, 1 depending how accommodative the trial with patient friendly features like no run in period	Run-in period of up to 6 weeks is appropriately patient centric for this trial design to allow to test/confirm genetic mutation and establish neurofilament light (NfL) levels	1
Use of novel methods: wearables, telemedicine visits, financial reimbursement Rating: 0, .5, 1 depending how accommodative the trial design is to patient participation such as use of patient friendly features like travel reimbursement for patient and caregiver, home collection of patient data during the trial.	Travel costs for participants and caregivers are covered. The trial uses a mix of clinics and at-home visits. Unlimited genetic counseling is available.	1
Part 3 Total		2
Part 3 Rating-Patient-Friendly		0.1
Total Rating		0.7
x 5		4
I AM ALS PaCTD 5-Star Rating:		**4-Star**