



Patient-Centric Trial Design (PaCTD) Rating Criteria

The PaCTD rating system was created by a group of patient and caregiver volunteers on the I AM ALS Clinical Trials Team. Their goal is to outline criteria for humane and efficient trial design. This rating system objectively evaluates trials based on select trial design elements in three key categories: optimizing access to investigational therapies, advancing scientific progress and if the trial is patient friendly.

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What are PaCTD ratings?

The PaCTD rating system is a 5-star rating system. The I AM ALS Clinical Trials Team used nine elements to assess clinical trial design. For definitions of the nine elements, see the definition section below. These elements fell into three primary categories and were given percentage weighting for the overall rating as listed below:

- **Optimizing access to investigational therapies (60%).** This category addresses whether a trial includes the following elements:
 - Open-Label Extension
 - Minimizes placebo usage
 - An Expanded Access Program
- **Advancing scientific progress (30%).** This category addresses whether a trial includes the following elements:
 - Consideration of disease heterogeneity
 - Use of scientifically justified eligibility criteria
 - Investigation of one or multiple biomarkers
 - Independent unblinded review panel for interim efficacy check-ins if warranted
- **Being patient friendly (10%).** This category addresses whether a trial includes the following elements:
 - Use of run-in observation period
 - Reduce travel burden by use of novel methods

PaCTD ratings do *not* measure or evaluate the treatment's safety or efficacy. A high rating on this scale does not indicate promising science and a low rating on this scale does not mean the treatment is ineffective -- it purely measures the design of the trial from the patient and caregiver perspective across the criteria outlined.

Within the ALS Signal dashboard you can hover your mouse over an individual PaCTD rating to see the specific elements included in a particular trial. If you have any questions about this rating system, please contact community@iamals.org.

Helpful Definitions

The document referenced throughout this page as “FDA” is the FDA ALS Clinical Trial Guidance Document. [It can be found here](#).

Open-Label Extension: The trial allows for trial participants to continue to access the treatment after the participation commitment in the trial has ended through Open-Label Extension (FDA p.5).

Minimize placebo usage: The clinical trial design seeks to minimize placebo administration to 1/3 of trial participants or less (FDA p.4 B1).

An Expanded Access Program: The drug sponsor provides access for patients who do not meet trial inclusion/exclusion requirements through an Expanded Access Program (FDA p.4).

Consideration of disease heterogeneity: The trial utilizes novel design features to sort out the effects of disease heterogeneity (e.g. crossover design; delayed start design; re-randomization of outlier disease progressors as part of a post-trial subset analysis) (referred throughout the FDA guidance). For more on ALS disease heterogeneity, click [here](#).

Use of scientifically justified eligibility criteria: All trial inclusion/exclusion requirements are scientifically justified and do not reflect a cut and paste attempt to copy prior unsuccessful trials (FDA p.3).

Investigation of biomarker: The trial attempts to identify and substantiate novel disease-related biomarkers (FDA p. 3 and 7).

Independent Unblinded Review Panel: The trial utilizes an Independent Unblinded Review Panel. This panel has the ability to halt a trial due to patient safety concerns. It also has the ability to identify early indicators of success so that there can be an adjustment to the trial to speed consideration of review (FDA p.3).

Use of minimal run-in observation period: The trial utilizes a run-in period of one month or less. A run-in period is a period of time between when a patient is accepted into a clinical trial and undergoes initial tests/screening and when the investigational drug/treatment is administered to a patient (FDA p.6 #3).

Reduce travel burden by use of novel methods: The trial design utilizes telemedicine, wearable technologies, financial reimbursement and/or other novel methods to limit patient travel and the number of clinic visits throughout the trial (FDA p.7).

PaCTD Ratings for Individual Trials

Click below to view a PaCTD Rating for:

Click to view a PaCTD Rating for current trials:

- [MediciNova \(Ibudilast\)](#)
- [PTC Therapeutics \(CARDINALS\)](#)
- [Denali Therapeutics \(DNL343- Healey Platform Regimen G\)](#)

Click to view a PaCTD Rating for non-enrolling trials:

- [Brainstorm \(NurOwn\)](#)
- [Orphazyme \(Arimoclomol\)](#)
- [Alexion \(Ultomiris\)](#)
- [Biogen \(BIIB067 \(SOD1\)\)](#)
- [Platform Trial](#)
 - Clene Nanomedicine (CNM-Au8)
 - Biohaven Pharmaceutical Holding Co (Verdiperstat)
 - Ra Pharmaceuticals (Zilucoplan)
- [Duke University \(Theracurmin\)](#)
- [Apellis \(Pegcetacoplan\)](#)
- [Reldesemtiv \(Cytokinetics Courage\)](#)
- [AB Science \(Mastinib\)](#)

I AM ALS Patient-Centric Trial Design (PaCTD)		
Manufacturer: PTC Therapeutics	NCT05349721	
Therapy: PTC857	CARDINALS (Phase 2)	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.	OLE as long as active treatment phase with good data collection	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.	2:1 Active:Placebo	1
Expanded Access Program 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 co. website	Not Available	0
Part 1 Total		2
Part 1 Rating-Seats at the Table		0.4
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 the science of clinical trials in ALS. 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 accelerometers. - Independent unblinded review panel for interim efficacy check-ins if warranted	Multiple scales used as secondary endpoints helps to develop alternatives to ALSFRS-R. Biomarkers NfL, Urine P75.	0.25
Part 2 Total		0.25
Part 2 Rating-Advancing Science Quickly		0.075
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	8 week run-in to enrich the primary ITT population.	0.5

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.	Home health provider comes to home for blood draws. 3 clinic visits in blinded phase and 3 in OLE. Travel reimbursement	1
Part 3 Total		1.5
Part 3 Rating-Patient-Friendly		0.075
Total Rating		0.55
x 5		2.75
I AM ALS PaCTD 5-Star Rating:		3-Star

I AM ALS Patient-Centric Trial Design (PaCTD)		
Manufacturer	MediciNova	
Drug	Ibutilast (NCT04057898)	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.	6 mo OLE included in protocol per clinicaltrials.gov. Duration should match blinded treatment phase	0.5
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.	Traditional 1:1 Randomization	0
Expanded Access Program 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 co. website	Not accepting requests for EAP	0
Part 1 Total		0.5
Part 1 Rating-Seats at the Table		0.1

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 the science of clinical trials in ALS. 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 accelerometers. - Independent unblinded review panel for interim efficacy check-ins if warranted	No publicly available evidence of novel trial design	0
Part 2 Total		0
Part 2 Rating-Advancing Science Quickly		0
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	Pre-trial screening takes approximately 1 month	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.	MedACT program helps to remove barriers to participation by providing financial and other support as needed	1
Part 3 Total		2
Part 3 Rating-Patient-Friendly		0.1
Total Rating		0.2
x 5		1
I AM ALS PaCTD 5-Star Rating:		1-Star

Manufacturer: Denali Therapeutics	NCT05842941	Rating
Therapy: DNL343	Healey Platform Regimen G	
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.	Committed to a 52 week minimum OLE (aka Active Treatment Extension)	1

<p>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.</p>	<p>Following Healey Platform protocol ratio of 3:1 active to placebo</p>	<p>1</p>
<p>Expanded Access Program 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 co. website</p>	<p>Awaiting additional safety and efficacy data before launching an Expanded Access (NOTE: if an Expanded Access Program is implemented this rating will be updated.)</p>	<p>0</p>
<p>Part 1 Total</p>		<p>2</p>
<p>Part 1 Rating-Seats at the Table</p>		<p>0.4</p>
<p>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 the science of clinical trials in ALS. 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 accelerometers. - Independent unblinded review panel for interim efficacy check-ins if warranted</p>	<p>The Healey Platform trial design has several innovative aspects including: - Broad inclusion criteria including no upper age limit, symptoms onset of less than 36 mo, 3:1 placebo ratio - Looking at several biomarkers - NfL, ISR, TDP-43, GFAP</p>	<p>1</p>
<p>Part 2 Total</p>		<p>1</p>
<p>Part 2 Rating-Advancing Science Quickly</p>		<p>0.3</p>
<p>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</p>	<p>Minimal run-in period to allow for evaluation and testing</p>	<p>1</p>
<p>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.</p>	<p>Some visits are virtual, use of smart phone apps, remote lab tests</p>	<p>1</p>
<p>Part 3 Total</p>		<p>2</p>
<p>Part 3 Rating-Patient-Friendly</p>		<p>0.1</p>
<p>Total Rating</p>		<p>0.8</p>

x 5		4
I AM ALS PaCTD 5-Star Rating:		4-Star

I AM ALS Patient Centric Trial Design (PaCTD)	Brainstorm NurOwn¹	
Open Label Extension	No	0
Minimize placebo usage - 33% or less	No (50%)	0
A side by side Expanded Access Program	No	0
Part 1 Total		0
Part 1 Rating-Seats at the Table		0
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.)	24 months from onset, no older than 60 years of age. Some were scientifically justified.	0.5
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		2.5
Part 2 Rating-Advancing Science Quickly		0.1875
Use of Run-In Observation Period - 3 months not acceptable -1 month ideally	Yes (3 months)	0
Use of novel methods: wearables, telemedicine visits, financial burden	Telemedicine visits through COVID-19.	0.5
Part 3 Total		0.5
Part 3 Rating-Patient-Friendly		0
Total Rating		0.1875
x5		1.0625
I AM ALS PaCTD 5-Star Rating:		1-Star

¹ Brainstorm’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.

I AM ALS Patient Centric Trial Design (PaCTD)	Orphazyme Arimoclomol²	
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
x5		3.625
I AM ALS PaCTD 5-Star Rating:		4-Star

² 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.

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

I AM ALS Patient Centric Trial Design (PaCTD)	Biogen BIIB067 (SOD1)³	
Open Label Extension	Yes	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	SOD1	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, state 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

³ Biogen’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.

<p>I AM ALS Patient Centric Trial Design (PaCTD)</p> <p>The HEALEY ALS Platform Trial tests multiple treatments in one trial. This listing will be updated if additional drugs are added to the trial.</p>	<p>Platform Trial</p> <p>Clene Nanomedicine CNM-Au8</p> <p>Biohaven Pharmaceutical Holding Co Verdiperstat</p> <p>Ra Pharmaceuticals Zilucoplan</p>	
	Open Label Extension	Yes - up to 1 year +
Minimize placebo usage - 33% or less	Yes (25%)	1
A side by side Expanded Access Program	CNM-Au8 - Yes Verdiperstat - Yes Zilucoplan - Pending ⁴	1 ⁵
Part 1 Total	3	3⁶
Part 1 Rating-Seats at the Table	0.6	0.6⁷
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
Part 2 Total	3	3
Part 2 Rating-Advancing Science Quickly	0.225	0.225
Use of Run-In Observation Period - 3 months not	No	1

⁴ Ra Pharmaceuticals’ Zilucoplan Expanded Access Program is pending.
⁵ Ra Pharmaceuticals’ Zilucoplan rating is 0 until the Expanded Access Program begins.
⁶ Ra Pharmaceuticals’ Zilucoplan receives a 2 until the Expanded Access Program begins.
⁷ Ra Pharmaceuticals’ Zilucoplan receives a 0.4 until the Expanded Access Program begins.

acceptable -1 month ideally		
Use of novel methods: wearables, telemedicine visits, financial reimbursement	Yes	1
Part 3 Total		2
Part 3 Rating-Patient-Friendly		0.1
Total Rating		0.925⁸
x 5		4.625⁹
I AM ALS PaCTD 5-Star Rating:		5-Star¹⁰

⁸ Ra Pharmaceuticals' Zilucoplan receives a 0.725 until the Expanded Access Program begins.

⁹ Ra Pharmaceuticals' Zilucoplan receives a 3.625 until the Expanded Access Program begins.

¹⁰ 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.

I AM ALS Patient-Centric Trial Design (PaCTD)	Duke University Theracurmin	
Open Label Extension	Yes - the whole trial is OLE	1
Minimize placebo usage - 33% or less	No placebo	1
A side by side Expanded Access Program		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	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 - microbiome compared to healthy controls	1
Independent Unblinded Review Panel that can communicate with FDA where substantial proof of “efficacy” emerges before end of trial		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	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)	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