



## I AM ALS Patient-Centric Trial Design (PaCTD) Rating for Cytokinetics' Courage

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
<b>Part 1 Total</b>		<b>3</b>
<b>Part 1 Rating-Seats at the Table</b>		<b>0.6</b>
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
<b>Part 2 Total</b>		<b>4</b>
<b>Part 2 Rating-Advancing Science Quickly</b>		<b>0.3</b>
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
<b>Part 3 Total</b>		<b>2</b>

<b>Part 3 Rating-Patient-Friendly</b>		<b>0.1</b>
<b>Total Rating</b>		<b>1</b>
<b>x5</b>		<b>5</b>
<b>I AM ALS PaCTD 5-Star Rating:</b>		<b>5-Star</b>