

Optimizing Outcomes: The Impact of Endpoint Selection in CGT Trials

Why Endpoint Selection is Critical in Cell and Gene Therapy (CGT) Trials



3 months added

and \$535,000 in unplanned costs per protocol amendment.¹

In CGT trials, where nearly **47% of trials enroll fewer than 20 patients²**, the consequences of incorrect endpoint selection are amplified. With small and complex populations, selecting the right endpoints is crucial to avoid protocol amendments, which drive up costs and delay timelines.

By carefully aligning endpoints from the outset, sponsors can prevent these costly delays and ensure more efficient trial execution.

¹ <https://www.centerwatch.com/articles/25921-no-end-in-sight-for-trial-complexity-csdd-report-reveals>

² <https://bmchealthservres.biomedcentral.com/articles/10.1186/s12913-023-09494-5>

Navigating Key Challenges in CGT Endpoint Selection



Overcome Confounding Factors



Challenge CGT patients often have complex comorbidities, making it difficult to select endpoints that clearly link outcomes to the therapy.

Approach Emmes uses adaptive trial designs to adjust endpoints as data evolves, reducing the impact of confounding factors.

Balance Efficacy & Safety



Challenge Efficacy and safety endpoints are often interwoven, complicating measurement in CGT trials.

Approach Emmes uses interim analyses to adjust endpoints over time, ensuring both efficacy and safety are balanced without compromising patient outcomes.



Maximize Impact in Small Populations



Challenge Small patient numbers in CGT trials make achieving statistical significance difficult.

Approach Emmes applies patient-centered trial designs to optimize data collection and ensure statistical rigor, even with small sample sizes.

Ensure Relevance in Long-Term Follow-Up



Challenge CGT trials often require years of follow-up, making it challenging to select endpoints that remain relevant.

Approach Emmes designs endpoints that capture both short- and long-term outcomes, allowing sponsors to collect meaningful data throughout the trial.



Emmes' Proven Approach to CGT Trial Success

With over three decades of experience and a track record of more than 125 CGT trials, Emmes combines innovation, AI-driven solutions, and specialized expertise to tackle endpoint selection challenges.

By ensuring statistical rigor, feasibility, and regulatory alignment, Emmes guides sponsors to successful outcomes from trial design to approval.



Partner with Emmes to Accelerate Your CGT Trial Success.
Contact us at info@emmes.com