

 **Clinical Trials**

C20 CLINICAL TRIAL INTERPRETATION WITH UNKNOWN STEM CELL-SPECIFIC DOSES

 **Cell Therapy**  **Gene Therapy**  **Tissue Engineering** **FUNCTIONAL AREAS**

Stem cell dose is a measure of the viable stem cells present in a given treatment, which can vary within a trial and across trials for different therapies.

CHALLENGE: The mechanisms for stem cell activity are complex and poorly understood, and stem cell counts may vary over time, which makes it difficult to count stem cells and establish standard, effective doses and routes of administration (ROA) in clinical trials. This leads to inconsistent trial results that are hard to interpret and replicate and may not be sufficiently reliable to progress to the next phase of clinical trials.

-  Bioprocessing and Production Standards
-  Analytical and Testing Methodologies Standards
-  Product Quality and Characterization Standards
-  Logistics and Compliance Criteria Standards
-  Preclinical Study Standards
-  Clinical Trial Standards

POTENTIAL FOR STANDARDIZATION

STANDARD OBJECTIVE	Broaden understanding of stem cell activity and variation over time to establish guidelines to identify reliable mechanisms for administering safe, efficacious doses.
POSSIBLE AREAS TO STANDARDIZE	<ul style="list-style-type: none"> <li style="width: 50%;">• Stem cell counting methods/technologies <li style="width: 50%;">• Qualifying ROAs <li style="width: 50%;">• Optimal timing for dose assessment <li style="width: 50%;">• Dose preparation methods

RELATED EFFORTS

- Efforts around cell counting (including an [SCB standard advancement project](#)) can ensure accurate counts are measured when comparing doses across trials.
- USP has standards on [CD34+ stem cell counting methods](#).



NEXT STEPS

- Conduct comparative ROA and dosage studies.
- Assess common causes of inconsistent doses.