

Single Day Event

Revolutionizing medicine: recent developments
and future prospects in stem-cell therapy

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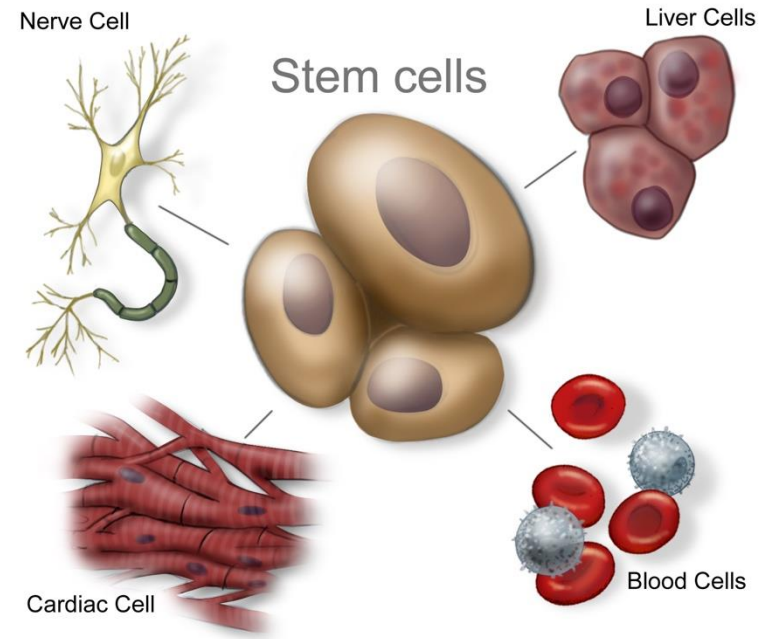


Agenda

- Stem cells: What they are and what they do?
- Advancements in stem cell research
- Characteristics of Cell Gene Therapy studies
- Significant Analysis Elements
- Challenges - oh nay!
- Overcoming the challenges - Let's try!

Stem cells: What they are and what they do?

- Stem cells - The body's master cells are special type of cells which has the properties to make more cells like themselves.
- They self-renew and they can become other cells that do different things in a process known as differentiation.
- Totipotent, Pluripotent: Embryonic stem cells (ESCs), Multipotent: Adult Stem Cells (ASCs), Hematopoietic & Mesenchymal, Induced pluripotent stem cells (iPSCs), Unipotent: Skin cells
- Stem cell therapy holds promise for treating various conditions, including cancer, neurodegenerative disorders, cardiovascular diseases, spinal cord injuries, diabetes, and tissue damage.





Advancements in stem cell research

Biotechnological advancements in stem cell research



Exosome-based Therapeutics

Focuses on stem cell-derived exosomes that modulate immune responses and promote tissue regeneration.

Single-cell RNA Sequencing

Provides insights into stem-cell transcriptomes, cellular heterogeneity, and gene expression patterns.

CRISPR-Cas9 Technology

Enables large-scale genomic studies and therapeutic applications in stem cell-based medicine.

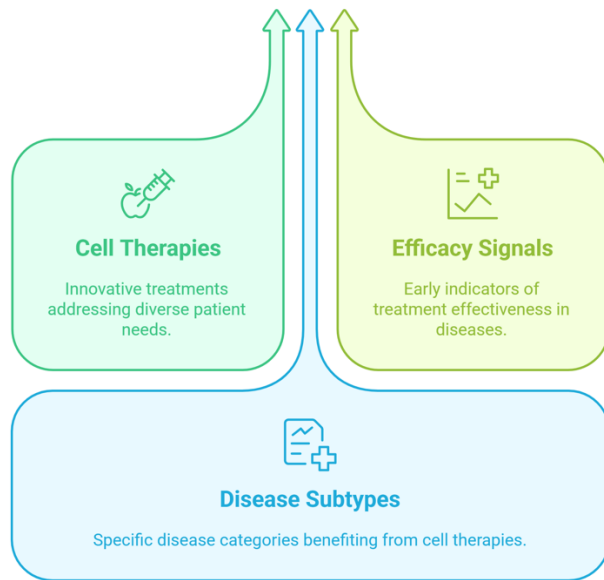


Characteristics of Cell Gene Therapy studies

Vein-to-Vein Cell Therapy Cycle



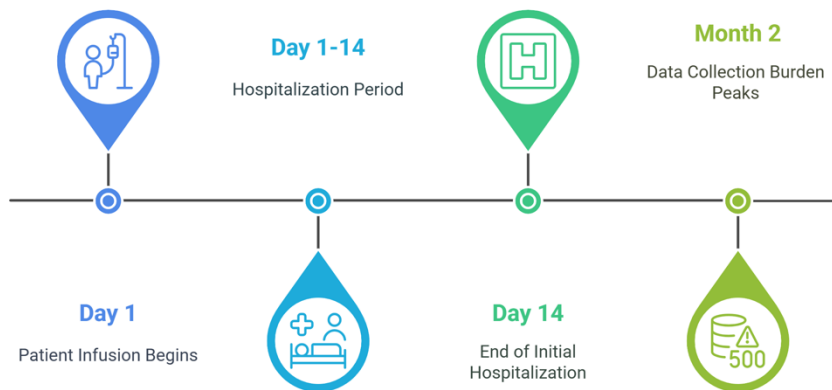
Fast-Tracking Medical Innovation





Characteristics of Cell Gene Therapy studies - cont.

Key Phases in CGT Study Data Collection



Complex Analysis Process in Cell Therapy Studies



Lab Analyses

Conducting detailed laboratory tests on samples



Repeat Outputs

Repeating analyses for different subgroups



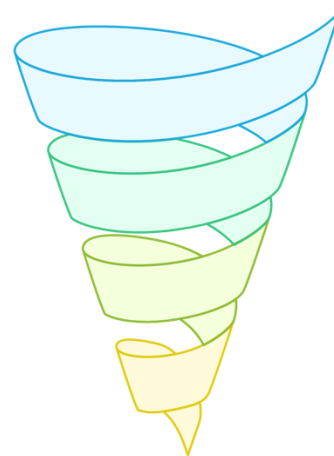
Safety Analysis

Focusing on adverse events and safety data



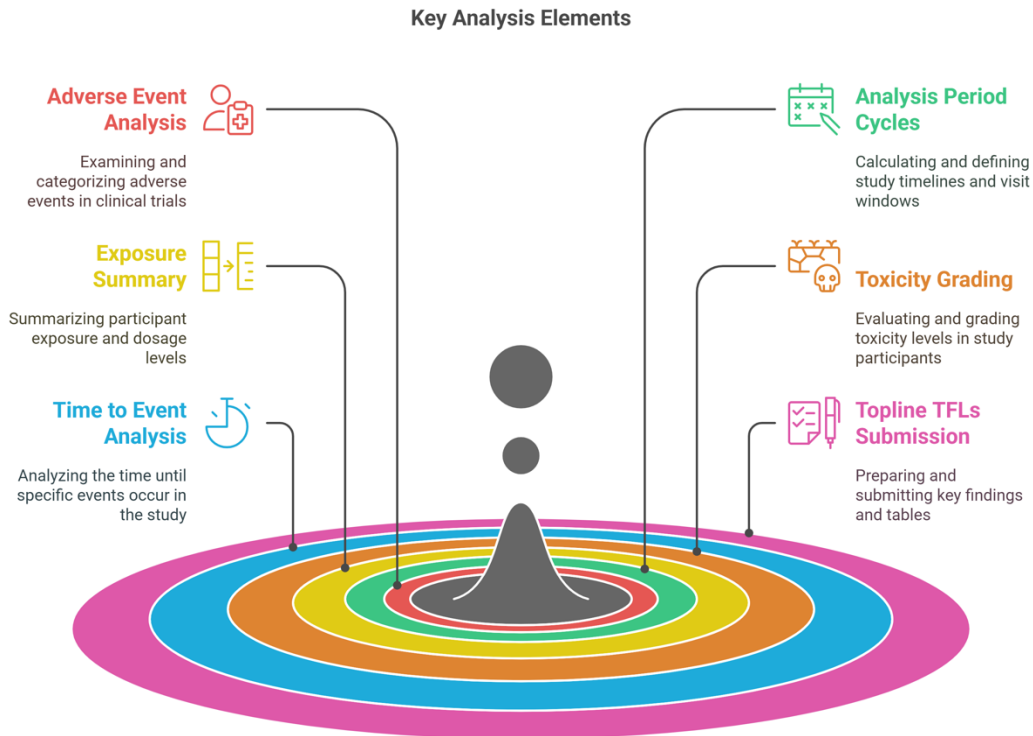
Ad-hoc Analyses

Performing additional analyses for biomarker identification





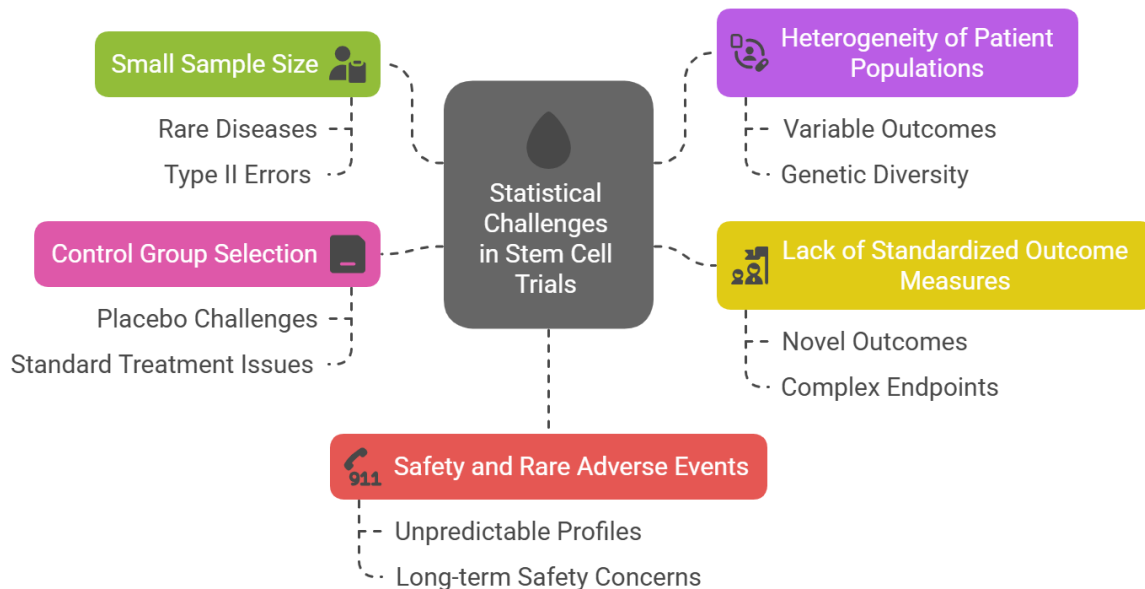
Significant Analysis Elements





Challenges – oh nay!

Statistical Challenges in Stem Cell Clinical Trials





Overcoming the challenges - Let's try!

Addressing challenges stem cell clinical trials

Control Group Selection

Use historical controls or sham procedures, considering ethical implications.

Heterogeneous Populations

Stratify patients and employ pre-specified subgroup analyses.

Safety and Adverse Events

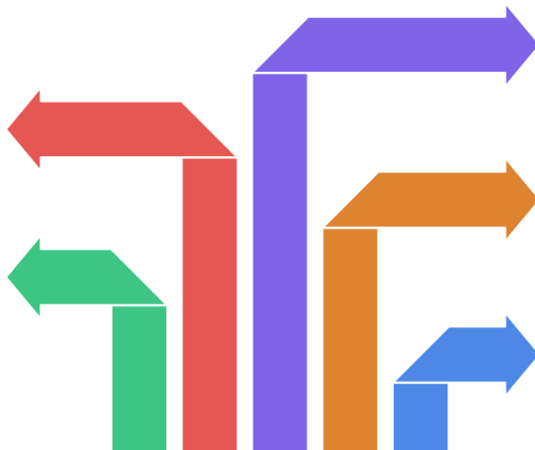
Use dose-escalation designs and post-marketing surveillance.

Outcome Measures

Use composite endpoints and collaborate with regulators.

Small Sample Size

Use adaptive designs or multi-center trials to increase recruitment.





Overcoming the challenges - Let's try!

Enhancing data management and analysis

Creating User-Friendly Platform

Developing an intuitive interface for data collection



Utilize Open Data Sharing

Engage with community for agile data sharing



Generate Synthetic Datasets

Offer synthetic data to preserve essential properties



Implement Frequent Data Cleaning

Conduct regular data cleaning cycles



Develop Applicable Programs

Create programs for diverse studies



Focus on Data Linkage and Validation

Strengthen data linkage and validation processes





The Global Healthcare Data Science Community

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