Children's Hospital LOS ANGELES
THE SABAN RESEARCH INSTITUTE

CHLA Clinical Trial: Next-Generation Therapy Definition

Definition of Next-Generation Therapies:

Next-generation therapies address significant unmet medical needs and offer transformative potential for various diseases. Therapies often involve the following characteristics:

1. **Genetic Modification**: Alteration of the genetic material within cells to correct genetic defects or enhance cellular functions (e.g., Antisense, gene therapy, CRISPR).
    1. Gene therapy aims to treat diseases by replacing, inactivating or introducing genes into cells— either inside the body (in vivo) or outside of the body (ex vivo).
    2. Antisense Therapies involve the use of antisense oligonucleotides (ASOs), designed to bind RNA and selectively alter gene expression.
2. **Cellular Engineering**: Use of engineered cells to treat diseases, often involving immune cells like T-cells (e.g., CAR T-cell therapy).
    1. Cell therapy aims to treat diseases by restoring or altering certain sets of cells or by using the cells themselves as a therapy.
    2. Cells that are altered genetically are tagged under Genetic Modification Cell Therapy in accordance with the standards by the Alliance for Regenerative Medicine (ARM).
3. **RNA-Based Therapies**: Treatments that use RNA molecules to interfere with or modify gene expression (e.g., siRNA, mRNA therapies).
4. **Regenerative Medicine**: Techniques to repair or replace damaged tissues or organs using stem cells or tissue engineering (e.g., stem cell therapy, Regenerative Medical Devices, Bioengineered Implants, Tissue Engineering Scaffolds, Bioprosthetic Devices, Advanced Therapy Medicinal Products (ATMPs), Implantable Biomaterial, Biomimetic Devices.)
    1. Tissue Engineering seeks to restore, maintain, improve, or replace damaged cells, tissues and organs through the combination of scaffolds, cells, and/or biologically active molecules.
5. **Novel Drug Delivery Systems**: Innovative methods to deliver therapeutic agents more effectively and precisely (e.g., nanoparticles, lipid nanoparticles, exosome-based delivery).

Excludes long-term observational or follow-up studies.

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