



ISCT North America 2019 Abstract Categories

<p>Mesenchymal Stem/Stromal Cells – Preclinical</p> <ul style="list-style-type: none"> • Genetic modification of MSCs for various indications • Basic biology and characterization of tissue-specific MSCs (Omics-based methods, functional assays, genetic approaches, etc.) • Novel methodologies for cell/tissue processing and large-scale manufacturing • Role of MSCs and the MSC niche in disease progression (cancer, hematological diseases, etc.) • MSCs in regenerative medicine and immune modulation (cardiology, CNS, pulmonary, musculoskeletal, dental, GI, GVHD, etc.)
<p>Hematopoietic Stem Cells – Preclinical</p> <ul style="list-style-type: none"> • Genetic modification of HSCs/HPCs for various indications • Stem cell mobilization and collection • Novel Hematopoietic cell and tissue processing • Enhancing conditioning and/or expanding indications for HSC transplantation • Novel Hematopoietic stem cell characterization (markers, functional assays, proteomics, genomics, etc.) • Enhancing cell engraftment and persistence • Cord Blood and other approaches to expanding the available donor pool
<p>Engineered Tissues and Stem Cell Products – Preclinical</p> <ul style="list-style-type: none"> • Bioengineering of genetically modified cells for various indications • iPS Cells, ES Cells, Tissue-specific Stem Cells (CNS, corneal/limbal, etc.), islets and other differentiated cell types (e.g. fibroblasts, hepatocytes, myoblasts, etc.) • Stem Cell Plasticity • 3D Scaffolds, 3D bioprinting, smart surfaces, etc. for Cell and Tissue generation • Engineered organs and tissues including organoids and organ-on-a-chip • Combination products (implantable device + cells)
<p>Immunotherapy – Preclinical</p> <ul style="list-style-type: none"> • Genetic modification of immune cells for various indications • Antigen presenting cells (DCs, B cells, macrophages, etc.) • T cell therapy in cancer, infectious disease, etc. • Innate immunotherapy (NK/NKT/CIK etc) cells and cell therapy • Immune modulation for autoimmunity and alloimmunity –Treg / MDSC / MSC / ToIDC etc

Improving Cell and Gene Therapy Tools – Preclinical

- Viral vector delivery systems (Retro-, Lenti-, AAV, etc)
- Non-viral delivery systems (electroporation, liposomal, etc.)
- Controlling gene expression and toxicity (suicide genes, inducible genes, etc.)
- Gene editing (e.g., CRISPR)
- RNA therapy
- Exosomes for manufacturing or therapy
- Equipment and devices for manufacturing (including point-of-care), testing, storage and transport, thaw and product administration, etc.

Clinical Studies in Cell and Gene Therapy

- Phase I/II/III clinical investigations and post marketing studies in any therapeutic indication
- Clinical trial management, implementation and other operational topics
- Correlative science, biomarkers, immune monitoring, cell persistence & tracking, etc.

Process Development and Facility Operations

- Technology transfer, intellectual property, commercialization
- Clinical process development and validation
- Obtaining, qualifying and validating ancillary products, equipment
- Potency Assays
- Facility Design
- Facility Operations
- Supply Chain Logistics
- Cost of Goods

Regulatory Affairs and Quality

- Regulatory inspections, reporting & submissions (ex. INDs, BLAs)
- Compliance
- Accreditation (JACIE/FACT, etc.)
- Legal aspects including consent and liability
- Policy development and implementation
- Ethical issues in cellular therapy
- Quality Control (proficiency testing, in-process and release testing)
- Education, training & competency
- Quality Assurance / Quality Systems for process monitoring and auditing; CAPA, investigations & deviation management; Validations, verifications, qualifications, etc.