Anschutz

ASCEND Definitions of Cellular and Gene Therapy Products

The *Accelerating Solutions for Cell and Gene Therapy Evaluation and Novel Delivery* (ASCEND) program will support **clinical trials of gene-modified and cellular therapies**, as well as **in vivo genome-modifying therapies**, serving as a centralized hub for study intake, feasibility assessment, startup, implementation, and long-term follow up within CU Anschutz and with UCHealth*.

The definitions of what constitute cellular or gene therapy are evolving rapidly. The table below contains the ASCEND program's current working definitions, based largely on U.S. Food & Drug Administration guidance on Tissue & Tissue Products and Cellular & Gene Therapy. These definitions are subject to periodic revision.

If you have an investigational product that **qualifies** or **may qualify** for ASCEND support, please complete the **ASCEND (Cell & Gene Therapy) Intake Form** and consult with the ASCEND team before submitting the study protocol into the HSR Portal. If you have questions about these definitions or about whether a specific study qualifies for ASCEND support, please write to **ASCEND_CGT@CUANSCHUTZ.EDU**.

Products That Qualify For ASCEND Support	**Products That May Qualify** For ASCEND Support	**Products That Do Not Qualify** For ASCEND Support
A. Any product that involves collection of cells (a patient's own cells or third-party cells) that then undergo **any form of permanent gene modification** in the lab prior to infusion into a patient. *Examples: lentiviral vector generated chimeric antigen receptor (CAR) T cells; lentiviral vector generated T cell receptor (TCR) cells; lentiviral vector gene addition to hematopoietic stem cells; and CRISPR edited cells.* B. Any product that involves collection of cells (a patient's own cells or third-party cells) that then undergo **"more than minimal manipulation"** in the lab prior to infusion into a patient. "More than minimal manipulation" is an FDA definition that refers to processing human cells, tissues, and tissue-based products in ways that alter their original relevant characteristics. Examples of "more	A. **Systemic RNA therapies** that involve collection of cells (a patient's own cells or third-party cells) and then infusion into a patient. There is a large spectrum of systemic RNA based therapies and their classification as cellular or gene therapies is debatable. Therapies that involve collection of cells (a patient's own cells or third-party cells) and then infusion into a patient and require the unique clinical resources offered by the cellular and gene therapy clinical program (i.e., stem cellular therapy laboratory, infusion center, clinical toxicity monitoring, etc.). *Example: RNA based CAR therapies.* B. Cell and tissue products that are collected from a patient or donor and then infused into a patient with **no or minimal manipulation** but are being **used for a purpose other than their natural biologic role**. Therapies that	A. Cell and tissue products that are collected from a patient or donor and then infused into a patient with **no or minimal manipulation** and are intended to function consistently with their **natural biologic role**. *Example: unmanipulated hematopoietic stem cells used to reconstitute hematopoiesis following stem cell transplantation.* B. Vaccine therapies that involve stimulation of the **native, unmanipulated** immune system of a patient. C. Bispecific and trispecific **T cell** engaging antibody therapies.

Products That <u>Qualify</u> For ASCEND Support	**Products That <u>May Qualify</u>** For ASCEND Support	**Products That <u>Do Not Qualify</u>** For ASCEND Support
than minimal manipulation" include cell culture/expansion in the lab, enzymatic digestion of tissues to isolate cells, seeding of cells on a medical device, and inducing differentiation of cells to into different cell types in a lab. *Examples: iPSC-derived Cellular Products; ex vivo expanded tumor infiltrating lymphocytes (TILs); dendritic cell therapy with genetic or cytokine modifications; and ex vivo expanded mesenchymal stem cells (MSCs).* C. Therapies that are administered into a patient with the intent of **permanently modifying the genome of cells** within the patient. *Example: Lentiviral based in-vivo CAR T generation platforms.* D. Therapies that deliver genes into a patient's cells, but **do not integrate into the genome** of the patient. *Example: Adeno-associated viral (AAV) therapies.* E. Therapies that require **long-term follow-up** or an **expanded access protocol (EAC)**.	involve collection of cells (a patient's own cells or third-party cells) and then infusion into a patient and require the unique clinical resources offered by the cellular and gene therapy clinical program (i.e., stem cellular therapy laboratory, infusion center, clinical toxicity monitoring, etc.). *Examples: Hematopoietic stem cells to treat orthopedic conditions; mesenchymal stems to treat heart failure.*	

*Children's Hospital-based pediatric cell and gene studies are exempt from ASCEND and will continue to follow existing CCHRI and ExACT pathways. Questions can be directed to PMITherapeuticsResearch@childrenscolorado.org.

*This resource was developed by the ASCEND Steering Committee. More information can be found on Accelerating Solutions for Cell and Gene Therapy Evaluation and Novel Delivery (ASCEND). Contact **ASCEND_CGT@CUANSCHUTZ.EDU** with questions.*

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