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## UCLA Policy #: Human Gene and Cell Therapy Program Draft for Review 5/16/2023

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Issuing Officer: Vice Chancellor for Research and Creative Activities  
Responsible Department: Vice Chancellor for Research and Creative Activities  
Effective Date: TBD  
Supersedes: New

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### I. PURPOSE & SCOPE II. DEFINITIONS III. POLICY STATEMENT IV. CONTACT INFORMATION V. REFERENCES VI. ATTACHMENTS

#### I. PURPOSE & SCOPE

The UCLA Human Gene and Cell Therapy Program (HGCTP) is a joint effort of the David Geffen School of Medicine, the Jonsson Comprehensive Cancer Center, and the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research.

The HGCTP includes scientific and quality assurance oversight of all Human Gene Transfer Clinical Trials conducted at UCLA and a manufacturing facility for cell and gene transfer products. Attachment A is a schematic representing the essential components of the program.

This Policy sets forth the requirements for oversight of the conduct of Human Gene Transfer Clinical Trials at UCLA and applies to all Human Gene Transfer Clinical Trials regardless of funding source (i.e. extramural industry funding, extramural non-profit or government funding, and/or intramural funding).

#### II. DEFINITIONS

For the purposes of this Policy:

**Clinical Trial** as defined by the NIH is a research study in which one or more human subjects are Prospectively Assigned to one or more Interventions (which may include placebo or other control) to evaluate the effects of those interventions on Health-Related Biomedical or Behavioral Outcomes. Refer to the NIH Clinical Trial Decision tool (<https://grants.nih.gov/ct-decision/index.htm>).

**Human Gene Transfer** is defined as the deliberate transfer into human research participants of either:

1. Recombinant nucleic acid molecules, or DNA or RNA derived from recombinant nucleic acid molecules, or
2. Synthetic nucleic acid molecules, or DNA or RNA derived from synthetic nucleic acid molecules, that meet any one of the following criteria:
  - a. Contain more than 100 nucleotides; or
  - b. Possess biological properties that enable integration into the genome (e.g., cis elements involved in integration); or
  - c. Have the potential to replicate in a cell; or
  - d. Can be translated or transcribed.

**Key Personnel** is defined as the Principal Investigator, other investigators and research personnel who are directly involved in conducting research with study participants or who are directly involved with handling private information related to study participants during the course of a research project. Key Personnel also include Faculty Sponsors who oversee student Principal Investigators in their conduct of human subjects research.

**Principal Investigator** is defined in [UCLA Policy 900](#).

### III. POLICY STATEMENT

All Human Gene Transfer Clinical Trials at UCLA must complete the requirements as outlined in this Policy.

#### **A. Approval by Institutional Bodies**

All Human Gene Transfer research studies at UCLA are required to receive favorable review/regulatory approvals by the following institutional bodies.

1. Institutional Review Board (IRB)
2. [Jonsson Comprehensive Cancer Center \(JCCC\) Internal Scientific Peer Review Committee](#) (ISPRC) for oncology studies OR [UCLA Scientific Review Committee](#) (SRC) for non-oncology studies
3. Institutional Biosafety Committee (IBC)
4. Medical Radiation Safety Committee (MRSC) (if applicable)

#### **B. Scientific and Quality Assurance Oversight**

Scientific review, data, and safety monitoring and quality assurance must be completed.

1. The JCCC ISPRC for oncology clinical trials and UCLA SRC for non-oncology clinical trials, will review the clinical protocol, statistical plan, and other factors such as adequate staffing, competing trials etc.
2. In addition to scientific integrity, the committees also review data and safety monitoring plans for the studies and if necessary, recommend trial oversight by their respective data and safety monitoring board (DSMB).
3. All studies overseen by an institutional DSMB are subject to monitoring and auditing by the quality assurance officers within either the JCCC or the Clinical and Translational Science Institute (CTSI).

For more information, please see the JCCC ISPRC website: <https://cancer.ucla.edu/research/clinical-research-unit/internal-scientific-peer-review-committee> and CTSI ORA website: <https://www.researchgo.ucla.edu/office-regulatory-affairs>

#### **C. Training**

##### **1. Required Training**

All UCLA Key Personnel involved with Human Gene Transfer Clinical Trials must complete and be up to date with the following:

- Good Clinical Practice course via CITI (Collaborative Institutional Training Initiative Program)([See UCLA Policy 917](#));
- NIH Recombinant DNA Guidelines course via CITI; and
- Human Gene Transfer course via CITI.

## 2. Training Responsibility

Principal Investigators are responsible for assuring that all Key Personnel associated with Human Gene Transfer research have completed these training requirements.

## 3. Training Enforcement

During initial and continuing scientific review of Human Gene Transfer Clinical Trials, the designated Scientific Review Committee will ensure that the Key Personnel have completed the three required courses. All Key Personnel must complete the required trainings before Scientific Review Committee approval can be issued.

## **D. Manufacturing of Gene Transfer Products**

The UCLA Human Gene and Cell Therapy Facility (HGCTF) supports manufacturing of gene and cell therapy products for UCLA Principal Investigators as well as other academic and industry partners conducting Clinical Studies in which a cell or gene therapy product is manufactured under an FDA IND. For more information, please see the HGCTF website: <https://medschool.ucla.edu/research/human-gene-and-cell-therapy-facility>

## **IV. CONTACT INFORMATION**

For oncology-related studies, please contact the [Jonsson Comprehensive Cancer Center \(JCCC\) Internal Scientific Peer Review Committee](#) (ISPRC): [CORA@mednet.ucla.edu](mailto:CORA@mednet.ucla.edu)

For non-oncology studies, please contact the Clinical and Translational Science Institute (CTSI) Office of Regulatory Affairs (ORA): [ctsiora@mednet.ucla.edu](mailto:ctsiora@mednet.ucla.edu)

For questions related to manufacturing a cell or gene transfer product, please contact the Human Gene and Cell Therapy Facility: [GMP@mednet.ucla.edu](mailto:GMP@mednet.ucla.edu)

## **V. REFERENCES**

1. OHRPP - Human Subjects Protection Certification via CITI  
(<http://ora.research.ucla.edu/OHRPP/Pages/CITITraining.aspx>)
2. CITI Program Homepage (<https://about.citiprogram.org/en/homepage/>)
3. Human Gene and Cell Therapy Facility (HGCTF)  
(<https://medschool.ucla.edu/research/research-resources/research-cores>)

## **VI. ATTACHMENTS**

- A. UCLA Human Gene and Cell Therapy Program

**Issuing Officer**

**/s/ Roger Wakimoto**

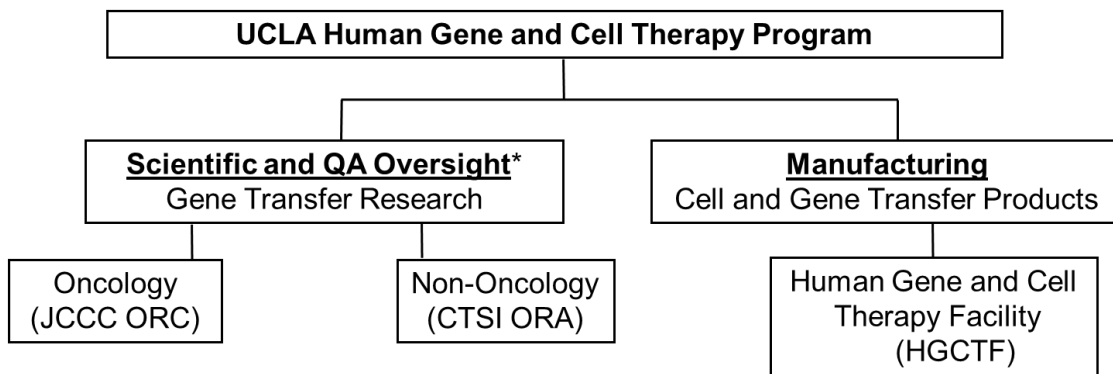
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**Questions concerning this policy or procedure should be referred to  
the Responsible Department listed at the top of this document.**

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## ATTACHMENT A



\*Oversight includes

1. Scientific review of protocols
2. Data and safety monitoring
3. Quality assurance (monitoring and auditing of trial conduct)
4. Training