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Owner	Terra-Noel Hughes: Rsch Cmplnc Mgr 1
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Human Gene and Cell Therapy Policy

	Human Gene and Cell Therapy Policy
Issuing Officer:	**Chief Medical Officer for Clinical Research (CMO-CR), UCLA Health**
Responsible Department:	**Office of Clinical Research**
Effective Date:	TBD
Supersedes:	New

I.	**PURPOSE & SCOPE**
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I. PURPOSE & SCOPE

The UCLA Human Gene and Cell Therapy Program (HGCTP) is a collaborative 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 provides scientific, safety, and quality assurance oversight of all Human Gene Transfer Clinical Trials conducted at UCLA and supports a manufacturing facility for cell and gene transfer products. Attachment A is a schematic representing the essential components of the program and UCLA Oversight Committees.

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 Study 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 in handling private information related to study participants during the course of a research project. Key Study Personnel also include Faculty Sponsors who oversee 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 UCLA Human Gene Transfer research must be approved by the following institutional bodies before work can begin:
    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) (See UCLA Policy 992 and IBC Requirements for Human Gene Transfer (HGT) Studies)
    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 the 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 regarding scientific review, DSMB services and quality assurance oversight, please see the JCCC ISPRC website, the JCCC DSMB website and the CTSI ORA website.

C. **Training**

1. **Required Training**

All UCLA Key Personnel involved with Human Gene Transfer Clinical Trials must have completed and be up to date with the following courses offered through the Collaborative Institutional Training Initiative (CITI) program:
- Biomedical Good Clinical Practice Training (See UCLA Policy 917);
- NIH Recombinant DNA Guidelines*; and
- Human Gene Transfer*.

*Training is valid for 3 years.

2. **Training Responsibility**
Principal Investigators are responsible for assuring that all Key Study Personnel associated with Human Gene Transfer research projects for which they are responsible have completed 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 Study Personnel have completed the three required courses. All Key Study Personnel must complete the required trainings before Scientific Review Committee approval can be issu

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 Trials in which a cell or gene therapy product is manufactured under an FDA IND. For more information, see the HGCTF website: https://medschool.ucla.edu/research/human-gene-and-cell-therapy-facility

IV.CONTACT INFORMATION

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

For non-oncology studies, contact the Clinical and Translational Science Institute (CTSI) Office of Regulatory Affairs (ORA): 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

For questions about Institutional Review Board (IRB) review and approval, contact the MIRB at mirb@research.ucla.edu.

For questions about Institutional Biosafety Committee (IBC) review and approval, contact the IBC administrative staff: ibc@research.ucla.edu

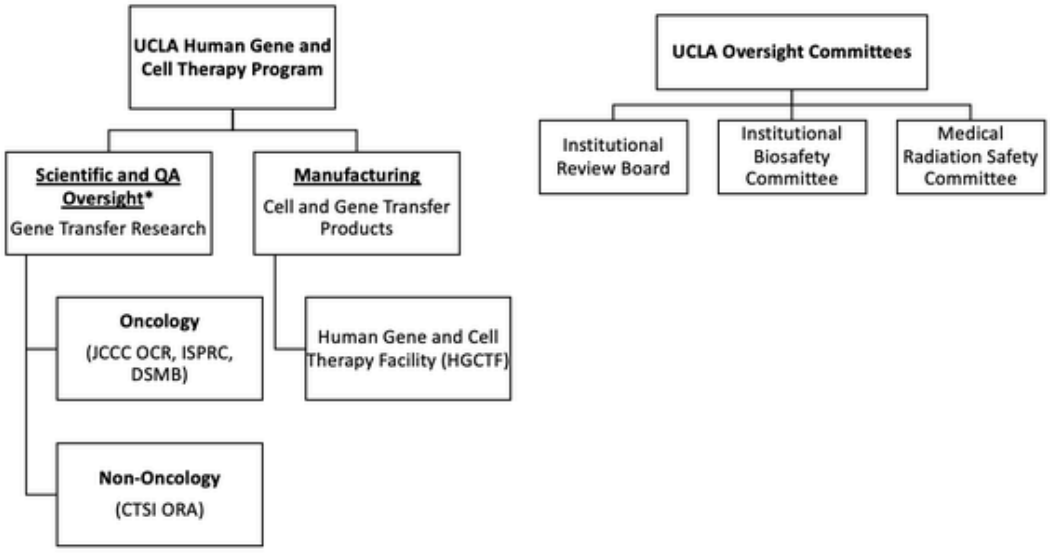
For questions about the Medical Radiation Safety Committee, contact the RSC administrative staff: rsc@research.ucla.edu

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)
4. UCLA Policy 915
5. UCLA Policy 992
6. UCLA Policy 994: Radiation Safety

VI.ATTACHMENTS

A. UCLA Human Gene and Cell Therapy Program and UCLA Oversight Committees
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

Approval Signatures

Step Description	Approver	Date
Chief Operations Officer Approval	Richard Azar: Coo Med Ctr [JB]	04/2024
Hospital System Policy Committee	Jeffrey Bergen: Regl And Cmplnc Hc Mgr 2 [KK]	04/2024
	Terra-Noel Hughes: Rsch Cmplnc Mgr 1	02/2024

Applicability

Ambulatory Care - UCLA, Resnick Neuropsychiatric Hospital, Ronald Reagan UCLA Medical Center, Santa Monica UCLA Medical & Orthopaedic, UCLA Health

History

Created by Hughes, Terra-Noel: Rsch Cmplnc Mgr 1 on 2/28/2024, 7:48PM EST

> This is a new policy that applies to Human Gene and Cell Therapy Research conducted at UCLA. The requirements are not new, they are just now being included in a formal policy.

Approved Date by Hughes, Terra-Noel: Rsch Cmplnc Mgr 1 on 2/28/2024, 7:48PM EST

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Approved Date by Azar, Richard: Coo Med Ctr on 4/2/2024, 7PM EDT

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