

Guidance and Procedure: Gene Transfer Therapy/Recombinant DNA (June 15, 2010)

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General Overview

Human Gene Therapy Research is defined by federal regulations as “any deliberate transfer of recombinant DNA, or DNA or RNA derived from DNA into human research subjects”. Specific provisions are necessary for conducting human gene transfer research at UCLA. Principal Investigators must complete a series of different reviews and approvals at the institutional level as well as the federal level (see [NIH Guidelines for Research Involving Recombinant DNA](#)). The approvals listed below are also required for the transfer of human cells (autologous or allogeneic) transformed with recombinant DNA administered to human subjects.

Coordination of Approvals

This guidance is intended to assist investigators in obtaining all required reviews as efficiently as possible.

Application to the following four committees will be required and can be submitted concurrently:

- RAC – Recombinant DNA Advisory Committee (NIH)
- FDA – Food and Drug Administration (DHHS)
- ISPRC – Internal Scientific Peer Review Committee (UCLA)
- UCLA IBC – Institutional Biosafety Committee

All of the above approvals are required prior to final UCLA IRB approval, but not review. Access to each of the above applications can be accessed under each individual section.

Federal Guidelines

NIH Recombinant DNA Advisory Committee

- In accordance with the National Institutes of Health (NIH) guidelines, [NIH Recombinant DNA Advisory Committee](#) (RAC) approval is required at initial review of all gene therapy transfer studies, regardless of funding, including the clinical trial site that is added after the RAC review process. The RAC is within the [NIH Office of Biotechnology Activities](#) (OBA).
- The RAC review must precede institutional review. Any recommendations resulting from the RAC review, including the public RAC review, must be included in the applications to the UCLA IBC and the IRB.
- Protocols and consent forms should be prepared in accord with NIH Guidelines, Appendix M. For additional information see [RAC Frequently Asked Questions](#) FAQs
- UCLA IRB approval of all research involving human gene transfer is contingent upon UCLA IRB receipt and acknowledgment of letter of approval from the NIH Recombinant DNA Advisory Committee.

Food and Drug Administration (FDA) Review

The study sponsor or investigator sponsor will also need to submit an [Investigational New Drug](#) (IND) application to the FDA. The IND number will be required before final IRB approval.

Institutional Guidelines

UCLA Internal Scientific Peer Review Committee (ISPRC)

- All human gene therapy transfer trials at UCLA must have ***UCLA scientific review and approval***.
- The UCLA [Internal Scientific Peer Review Committee](#) (ISPRC) conducts a review of scientific merit of all human gene therapy transfer trials conducted at UCLA.
- The [ISPRC Application](#) and review and approval of all studies which fall within all therapeutic, diagnostic and non-cancer gene therapy clinical research studies is required at initial IRB review and when modifications to the protocol are made.

UCLA Institutional Biosafety Committee (IBC)

- In accordance with the NIH Guidelines for Research Involving Recombinant DNA Molecules, the IBC reviews and approves recombinant DNA experiments in compliance with the [NIH Guidelines](#).
- UCLA [IBC review](#) and approval is required at initial review for studies involving recombinant DNA.
- For additional information about IBC submissions, contact the EH&S Biosafety Program at 310-825-3323 or email biosafety@ehs.ucla.edu.

UCLA Institutional Review Board (IRB)

- The UCLA IRB Reviews the study and gives final approval. Although, other external and institutional reviews may be submitted concurrently (e.g., RAC, IBC, ISPRC), they are required **prior to final IRB approval**. These can be submitted within the webIRB application.
- As these external and institutional board reviews will contribute to the information reviewed by the IRB, all correspondence and approvals from external and other institutional boards must be submitted to the IRB upon receipt by the investigator.
- For additional information regarding IRB gene therapy submissions contact MIRB 2 Committee Administrator at 310-825-5344.

Reporting of Adverse Events

PIs at all sites must report qualifying serious adverse events (SAEs) to the NIH Office of Biotechnology Activities according to the guidance provided in [Appendix M-I-C-3 and M-I-C-4 of the NIH Guidelines](#). PIs may delegate this task to another party (e.g., the sponsor), provided a letter of delegation signed by the PI is on file with the OBA. Any OBA reports concerning UCLA-enrolled participants should also be submitted to the UCLA IRB and IBC. **Investigators must also follow [UCLA Post Approval Reporting Requirements](#) for reporting adverse as well as other post approval events.**

References

- [Appendix M-I-C-3 and M-I-C-4 of the NIH Guidelines](#)
- [FDA Investigational New Drug Application](#)
- [Gene Therapy Legislation in the United States](#)
- [NIH Office of Biotechnology Activities \(OBA\)](#)
- [NIH Recombinant DNA Advisory Committee \(RAC\)](#)