SOP TITLE: Human Gene Therapy Administration at ACTRI

1 SCOPE

1.1 This guideline covers the UCSD ACTRI Center for Clinical Research (CCR) staff as they conduct scheduled events on human gene therapy clinical trials.

2 REVISIONS FROM PREVIOUS VERSION

2.1 N/A

3 PURPOSE

3.1 To provide guidance for the safe care of research participants receiving human gene therapy, including the administration of investigational products, and to minimize the risk of transmission of infection to healthcare providers, patients, and visitors.

4 RESPONSIBILITIES

4.1 Unit Leadership is responsible for coordinating and revising this guideline with guidance from the UCSD Institutional Biosafety Committee.

5 DEFINITIONS

5.1 **Human Gene Therapy**

5.1.1 Insertion of normal DNA directly into cells to correct a genetic defect. The treatment of disease by replacing, altering, or supplementing a gene that is absent or abnormal and whose absence or abnormality is responsible for a disease.

5.2 **Institutional Biosafety Committee (IBC)**

5.2.1 The UCSD Institutional Biosafety Committee (IBC) is the local review body responsible for oversight of all research and teaching activities involving biohazardous materials and recombinant/synthetic nucleic acids. The IBC is responsible for reviewing and approving Biological Use Authorization (BUA) applications and enforcing applicable policies.

5.3 **Viral In Vivo Gene Therapy**

5.3.1 Therapy intended to treat disease by delivering genes into non-reproductive (somatic) cells using biological agents, typically via wild-type or modified viruses. Common viral vectors include retroviruses, adenoviruses, adeno-associated viruses, poxviruses, bacteriophages, and picornaviruses.

5.4 **Non-Viral In Vivo Gene Therapy**

5.4.1 Utilizes physical or chemical methods (e.g., lipid nanoparticles) to deliver genetic material into cells. These vectors do not require the same infection prevention protocols as viral vectors.

6 PROCEDURE

6.1 Infection Prevention

6.1.1 Only ACTRI staff trained for the study may prepare, transport, and administer gene therapy agents. This includes nursing staff, laboratory staff, pharmacy staff, clinical coordinators, PI's and any staff at the ACTRI who may be designated to perform these steps.

6.1.2 Staff must complete required training:

6.1.2.1 ***Biosafety: Clinical Use of Recombinant DNAs***

6.1.2.2 ***Biosafety: Recombinant and Synthetic Nucleic Acids***

6.1.3 Staff must review the protocol summary prior to administration.

6.1.4 In-person in-service with study team and nursing staff is a requirement.

6.1.5 All body fluid spills must be treated as biohazardous. Personnel must wear PPE for hazardous spill cleanup.

6.1.5.1 Refer to:

6.1.5.1.1 ***Research Safety: How to Handle Chemical Spills in Laboratories***

6.1.5.1.2 ***Hazardous Drugs Spill Clean-Up Procedure.pdf***

6.1.5.2 ACTRI nurses must immediately report any incidents, accidents, spills, or inadvertent exposures to the PI.

6.1.5.2.1 Refer to: ***Hazard Control Plan – Blood*.**

6.2 Preparation and Administration

6.2.1 The UCSD Investigational Pharmacy or the Stem Cell Lab will thaw or prepare the study agent in the appropriate space using IBC recommended room including appropriate pressure and hood. The agent must be transported in a rigid, non-porous, leak-resistant container fitted with lids and biohazard labels.

6.2.2 ACTRI staff must:

6.2.2.1 Place a **"Biohazard"** placard, IBC approval, and BSL level sign on the patient's room door.

6.2.2.2 Ensure only trained personnel involved in the study enter the room (except in emergencies).

6.2.2.3 The PI must be present for all first time gene therapy administration.

6.2.2.4 Include the PI's name and contact info on the placard.

6.2.3 Two ACTRI nurses must verify the orders per ACTRI policy.

6.2.4 Don PPE as required by the BSL level and IBC recommendations.

6.2.5 Administer the study agent via subcutaneous, intramuscular, or intravenous injection according to protocol requirements and nursing orders.

6.2.6 Flush the agent with **a minimum of 30 mL normal saline**, using a closed system adapter. The adapter will be pre-attached by the pharmacy.

6.2.7 The subject must remain under observation in ACTRI post-administration, as dictated by the protocol.

6.2.8 ACTRI staff must be aware of potential adverse events outlined in the protocol and be prepared to respond.

6.2.9 After the subject's departure, authorized staff must request **terminal cleaning** of the room if required by the IBC

6.2.10 All waste products including IP, PPE and other disposable equipment will be disposed of per IBC approval and BSL requirements.

7 FORMS

7.1 None

8 REFERENCES

8.1 UCLA Gene Therapy SOP

8.2 UCSD Research Safety Website: https://blink.ucsd.edu/safety/research-lab/index.html

8.3 UCSD Lab Safety website/policies: https://blink.ucsd.edu/safety/research-lab/ucop-labsafety.html

9 ATTACHMENTS

9.1 **CCR-116 ACTRI Human Gene Therapy Administration Checklist**

FORM: ACTRI HUMAN GENE THERAPY ADMINISTRATION CHECKLIST

MATERIAL REQUIREMENTS:

☐ Transport container (rigid, non-porous, leak-resistant with tight-fitting lid and biohazard labels for transporting materials from IP Pharmacy to CTRC exam room)

☐ Solid Biohazard waste container (rigid, non-porous, leak-resistant with a tight-fitting lid, and labeled with the biohazard symbol on all sides and the lid, lined with two red biohazard bags that are ASTM D1922 and ASTM D1709 compliant, before use).

☐ Biohazard sharps container

☐ Biohazard Spill kit contains:

- ☐ 2 Z-packs of absorbent paper towels
- ☐ Tongs or forceps for picking up items
- ☐ Signage and hazard tape (to block off areas during spill response to prevent non-authorized personnel from exposure risk)
- ☐ Adhesive tape for signage
- ☐ Red biohazard bags
- ☐ Household bleach (~5% Sodium hypochlorite, undiluted). Marked with date of purchase and replaced annually
- ☐ Squirt bottle (empty) - pre-mark the fill level for 10% bleach with sharpie
- ☐ 2 disposable face mask/surgical masks
- ☐ 2 pairs nitrile gloves (S, M, L, XL)
- ☐ Disposable lab coats (X2)
- ☐ Shoe covers
- ☐ Disposable gown

☐ Eye coverings

☐ Splash resistant eye coverings

☐ Nitrile gloves

☐ Surgical mask

☐ Bouffant (optional)

☐ Shoe Covers (optional)

FORM: ACTRI HUMAN GENE THERAPY ADMINISTRATION CHECKLIST

INTRAVENOUS ADMINISTRATION OF HUMAN GENE THERAPY TO A RESEARCH SUBJECT:

☐ Scheduled to Large Room

☐ Room Preparation:

- ☐ Sharps container
- ☐ Empty biohazard solid waste container
- ☐ Hazard warning sign with the following taped to the door:
  - ☐ Investigational agent
  - ☐ Name and number of the PI
  - ☐ Special requirements for entering the room
  - ☐ Biosafety Level; biohazard symbol

☐ Explain to the patient that during administration, only RN and study subject are allowed in the room, and universal blood and tissue precautions should be followed.

☐ Confirm no eating, drinking, or applying cosmetics for clinic staff while IP is in room and or being administered to patient

☐ Apply PPE prior to entering the room and handling the IND

- ☐ Disposable gown
- ☐ Splash resistant face mask and protective eyewear
- ☐ Nitrile gloves
- ☐ Other PPE as listed above

☐ Administer at the prescribed rate on an approved infusion pump

☐ Upon completion of IP administration

- ☐ The syringe/needle is placed into a biohazard puncture resistant sharps container.
- ☐ All disposable contaminated materials should be placed in a red biohazard waste bag in the room.
- ☐ The biohazard sharps container and red biohazard waste bag must be placed inside the biohazard labeled secondary, non-porous leak-resistant, puncture-resistant container for transport.
- ☐ PPE to be removed before leaving the room and disposed of in a red biohazard waste bag in the room.

FORM: ACTRI HUMAN GENE THERAPY ADMINISTRATION CHECKLIST

☐ Biohazard waste bag should be closed prior to its removal from room, and placed inside a secondary non-porous leak resistant, puncture-resistant lidded biohazard container for transport. Once all waste has been placed in the closed puncture-resistant biohazard container, place it in the dirty utility room.

☐ Wash hands with soap and water for 30 seconds immediately after removing gloves and before leaving the room.

☐ All work surfaces should be decontaminated with disinfectant immediately after administration of the investigational product to research subjects and after any spill of viable material. Confirm appropriate disinfectant contact time to inactivate.