

Genome Editing Competency Hub at UCSF Alpha Clinic & Innovative Genomics Institute for the California Institute for Regenerative Medicine (CIRM) Alpha Clinics Network

To broaden the scope of therapeutic gene editing in hereditary disorders, we have expanded our collaboration with the **Innovative Genomics Institute (IGI) at UC Berkeley** in clinical translation of CRISPR technology to precision medicine-guided targeting of selected hereditary disorders.

This collaboration is best exemplified by the lead clinical trial of CRISPR modification of autologous hematopoietic stem progenitor cells for correction of the sickle mutation, which is an active clinical trial under IND, supported by a CLIN2 and matching NHLBI Cure Sickle Cell Initiative funding, brought to the clinic through a disease-team collaboration of teams from UCSF, IGI, and UCLA.

KEY SERVICES PROVIDED

Product Classification and Regulatory Pathway Evaluation

Determine product classification (e.g., drug, device, combination product, biologic) and assess the need for an Investigational New Drug (IND) or Investigational Device Exemption (IDE)

IND/IDE Application Support

Assist with IND or IDE applications and subsequent submissions, including pre-submissions (e.g., INTERACT, pre-IND/IDE meetings), amendments, safety/annual/final reports, and special designations such as Orphan Drug Designation (ODD), Regenerative Medicine Advanced Therapy (RMAT)

Protocol Development

Clinical protocol development in support of FDA submissions

FDA Meeting Support

Preparation, coordination, and attendance at FDA meetings including pre- and post-meeting documentation and correspondence with the FDA and research team

FDA Inspection Support

Preparation for and regulatory support during FDA inspections of investigator-sponsored clinical trials

Regulatory Consultation & Education

Provide consultation and presentations to investigators, study teams and community regarding drug, device, and biologic products and technology

ResearchGo

UCLA [ResearchGo](#) is an online portal of tools, templates, guidance, go-to, and resources for investigators, study staff, and partners/affiliates.

To discuss how we can provide services for your CIRM Alpha Clinic Network trials, please contact us at FDAConsults@mednet.ucla.edu