AGENDA

Topic

1. **UCSF Alpha Stem Cell Clinic (ASCC)**
   - Dr. Mark Walters
   - Christine Razler
   - Christina Chun
   - 8:30 – 9:00

2. **Member Roundtable Discussion**
   - All
   - 9:00 – 9:30

Meetings are on the 2nd Tuesday of each month.

2020 Meetings:
- January 14
- February 11
- March 10
- April 14
- May 12
- June 9
- July 14 - canceled
- August 11
- September 8
- October 13
- November 10
- December 8
The mission of the UCSF Alpha Stem Cell Clinic is to support and advance stem cell-based clinical research in regenerative medicine, with a goal of bringing new disease treatments to the people of California. We do this by providing high-level infrastructure support to investigators at any point in their research, from pre-clinical studies to active clinical trials.
Challenges to Accelerating Stem Cell Therapies

- **Challenge:** Moving stem cell treatments through IND-enabling studies to clinical trials takes disproportionally more time when compared to non-cellular treatments.

- **Solution:** As integral components of CIRM’s Strategic Plan, CIRM’s infrastructure programs are designed to overcome the obstacles that currently slow the progress of translating stem cell research to high quality clinical trials.
UCSF ASCC Overview

- UCSF one of five Alpha Clinics (UCLA/UCI, UCD, UCSD & City of Hope)
- Funded by CIRM – study start-up assistance at no cost to UCSF investigator
- Accelerate & advance stem cell research
- Power of the ASCC Network - multi-center reliance attractive to sponsors & more
- Community education & engagement
UCSF Alpha Clinic Organization

EXECUTIVE LEADERSHIP
Program Director, Mark Walters, MD
Co-Director, Michael Matthay, MD
Fellowship Director- Kathleen D. Liu, MD, PhD
cGMP Medical Director - Jonathan Esensten, MD, PhD

STAFF
Program Manager - Christine Razler
Patient Care Coordinator - Marci Moriarty, RN, MSN
Research Compliance Manager - Christina Chun, MPH
Allyson Beaulieu – Clinical Regulatory Specialist

STEERING/ADVISORY COMMITTEE
Christopher Dvorak, MD, Chief, Pediatric Allergy, Immunology & BMT
Michelle Hermiston, MD, Associate Professor, Pediatrics
Tippi MacKenzie, MD, Pediatric Surgeon, Fetal Treatment Center
Tang Qizhi, PhD, Associate Professor, Diabetes Center
UCSF ASCC Partners

CTSI
- Subject Recruitment
- EHR Data
- Team Science
- Clinical Research Svcs
- Biostat

IRB
- Reliance
- SMART IRB
- GESCR

OSR
- Contract Negotiation
- Master Agreements

OCTA
- Coverage Analysis
- Budgeting
Power of the ASCC Network

- Opportunities for multi-site clinical trials with ASCC Network
- Set up to activate multi-center trials quicker
- Shared templates/master contracts
- Central IRB
- Shared regulatory SOPs
Opportunities for expansion of cell therapies

### Public Inquiries
- Arthritis 21%
- Immunology 5%
- Nephrology 5%
- Neurological 16%
- Ortho 27%
- Other 5%
- Pulmonology 11%
- Retinopathy 5%
- Diabetes 5%

### ASCC Studies
- Cancer 39%
- Diabetes 5%
- HIV 5%
- Immunology 11%
- Neurological 6%
- Pulmonology 6%
- Sickle Cell Disease 17%
- Thalassemia 11%
- Other 5%
FDA IND Preparation and Submission
- Pancreatic Islet and PARAthryoid Co-Transplantation for Treatment of Diabetes in Intra-Muscular Site: PARADIGM - Peter Stock, MD
- An Expanded Access Study of using the CLINIMACS Device for Alpha/Beta T-Cell Depletion in Stem Cell Transplant Recipients – Christopher Dvorak, MD
- Developing Engineered Autologous Leukemia Vaccines to Target Residual Leukemic Stem Cells – Karin Gaensler, MD
UCSF ASCC Filling the Gaps

Facilitating Cell Therapy trials with CTSI/CRS

DOM ZSFG Investigator – **Steven Deeks, MD**

- A Comparative Study of Autologous CD4+ T Cells Genetically Modified at the CCR5 Gene by Zinc Finger Nucleases SB-728 versus ex vivo Expanded Unmodified Autologous CD4+ T Cells in Treated HIV-1 Infected Subjects “TRAILBLAZER”

- Safety and anti-HIV activity of autologous CD4+ and CD8+ T cells transduced with a Lentiviral vector encoding bi-specific anti-gp120 CAR molecules (LVgp120duoCAR-T) in anti-retroviral drug-treated HIV-1 infection

Transplant Nephrologist – **Deborah Adey, MD**

- Phase 2 Multicenter, Randomized, Double-Blind, Placebo-controlled, Multiple dosing interval, 3-period study of the safety, tolerability and effectiveness of adoptively transferred Viralym-M multi-virus-specific T Cells in kidney transplant recipients with either high or low levels of BK viremia
Study Activation for UCSF ASCC Lead Studies

- A Single Center, Non Randomized Study of the Safety and Efficacy of In Utero Hematopoietic Stem Cell Transplantation for the Treatment of Fetuses with Alpha Thalassemia Major – Tippi MacKenzie, MD

- A Phase 1 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin BB305 Lentiviral Vector in Subjects with Severe Sickle Cell Disease – Mark Walters, MD

- Study to Assess the Safety, Tolerability, and Efficacy of ST-400 for Treatment of Transfusion-Dependent Beta-thalassemia (TDT) – Mark Walters, MD

- Mesenchymal Stem/Stromal Cells: Testing for ARDS after Trauma in a Phase 2B Trial – Michael Matthay, MD
Study Activation for CIP- Cancer Center

- **B-Cell Malignancies (PI: Charalambos Andreadis, MD)**
  - A Phase 1 Clinical Trial of Anti-CD19 Chimeric Antigen Receptor T Cells for the Treatment of B-Cell Malignancies - (Homegrown CAR-T)
  - A Phase 1, dose-escalation and cohort expansion study of the safety and efficacy of anti-CD19 allogeneic CRISPR-Cas9-Engineered T cells (CTX110) in subjects with relapsed or refractory B Cell Malignancies

- **Relapsed/Refractory Multiple Myeloma (PI: Nina Shah, MD)**
  - Open-Label, Multicenter, Single Ascending Dose Study to Assess the Safety of P-BCMA-101 in Subjects with Relapsed/Refractory Multiple Myeloma Followed by a Phase 2 Assessment of Response and Safety (PRIME) - (Poseida)
  - A Phase 1, open-label dose-escalation study to evaluate the safety, expansion, persistence and clinical activity of UCARTCS1A (allogeneic engineered T-cells expressing anti-CS1 chimeric antigen receptor), administered in patients with Relapsed/Refractory Multiple Myeloma (Cellectis)

- **Solids Tumors (Basket Trial) and metastatic castration-resistant prostate cancer (PI: David Oh, MD)**
  - Personalized tumor mutation-targeted adoptive TCR-T cell therapies for solid tumors (PACT Pharma)
  - A Phase 1 Dose Escalation and Expanded Cohort Study of P-PSMA-101 in Subjects with Metastatic Castration-Resistant Prostate Cancer (mCRPC) (Poseida)
Getting you to the *starting* line quicker
UCSF ASCC Services

ASCC Team

- Pre-IND/IND Regulatory
- IRB
- Project Management
- Bio-specimen handling
- Trial Coordination
- OnCore Management
- Clinical Protocol Development
- Data collection
- SOPs
Liaison with existing UCSF infrastructure

UCSF Partners

- Coverage Analysis
- CT Budgeting
- CT Agreements
- Study/Research Design
- Regulatory & Ethics
- Subject recruitment
- EHR/Data extraction
- Data management
- Biostatistics
Living Therapeutics Initiative
Clinical Delivery Taskforce

Executive Summary:

Develop new therapies and manufacturing for testing in clinical trials, which will study novel cellular therapies in persons with diseases difficult to treat

- Sustained and new investment in cellular therapy development and clinical delivery programs, with infrastructure to facilitate rapid review and approval of these clinical trials.

- Facilitate productive scientific collaborations/project teams, investing in full-spectrum services and expansion of GMP capacity, and centralizing effective regulatory/quality/study activation systems.
How to Access ASCC services

• Contact Us: alphascc@ucsf.edu 415-502-2558

• Complete our study intake & feasibility assessment
Questions?