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Consortium for Pediatric Cellular Immunotherapy

3rd Annual Meeting
September 24, 2020
Virtual

Children's Hospital Colorado
Children's Hospital LOS ANGELES
Children's National Health System
Seattle Children's HOSPITAL • RESEARCH • FOUNDATION
UCSF Benioff Children's Hospital Oakland

Welcome!

NCATS

PJ Brooks, PhD
Program Director
National Center for Advancing Translational Sciences
National Institutes of Health

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Vice Chair for Clinical Research, Department of Pediatrics Division of Pulmonology Allergy and Immunology, School of Medicine
Vice Dean for Translational Research, School of Medicine
Principal Investigator, Clinical and Translational Science Collaborative, School of Medicine

Moving Beyond Cancer

Luigi Notarangelo, MD
Chief, Laboratory of Clinical Immunology and Microbiology
National Institute of Allergy and Infectious Disease

Karin Chen, MD
Associate Professor
Immunology
University of Washington
Seattle Children's Hospital

Michael Keller, MD
Assistant Professor
Immunology
George Washington University
Children's National Health System

U01 General Objectives

- Accelerate the development of novel cellular immunotherapies for pediatric disease, including cancer, infection, and immune tolerance

- Develop and disseminate resources to enhance the development and implementation of novel cellular immunotherapy
  - Establish collaborations across the CTSA network
  - Train clinical, manufacturing, research, and regulatory teams

- Expand patient access to novel cellular immunotherapy

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Year 2
Key Accomplishments
At-a-Glance

Training & Mentoring

Presenting at quarterly CPCI Scientific Talk series

Amy
Hont
MD
GWU

David
Nguyen
MD, PhD
UCSF

Eric
Kohler
MD
CU

Corinne
Summers
MD
UW

Hema
Dave
MD, PhD
GWU

Babak
Moghimi
MD
USC

Jessica
Lake
MD, MPH
CU

Colleen
Annesley
MD
UW

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Training and Mentoring

Contributing through Committees and Working Groups

Paibel
Aguayo-Hiraldo
MD
USC

Hema
Dave
MD, PhD
GWU

Jonathan
Esensten
MD, PhD
UCSF

Anurekha
Gollapadi
MD
UW

Kimberly
Jordan
PhD
CU

Michael
Keller
MD
GWU

Adam
Lamble
MD
UW

Jonathan
Marron
MD, MPH
Harvard

Agne
Taraseviciute
MD
USC

Lena
Winestone
MD
UCSF

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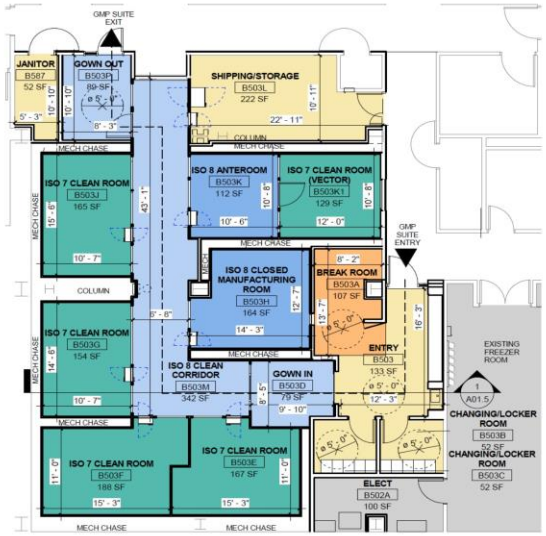
SA1: Expand Manufacturing Capabilities of Cellular Immunotherapy Products

- **Key Year 2 Outcomes**
  - **Expanded available cGMP facilities – opened new cGMP facility at SC increasing production capability 5-10fold**

- **SC is providing help to CHLA in new construction of cGMP facility scheduled to open in 2022**



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SA2: Expand the Clinical Development of Cell-Based Immunotherapy for Pediatric Disease

- **Key Year 2 Outcomes**
  - **Four ongoing multi-center trials across Consortium**
    - **PLAT-02 at SCH, CHLA, BCHO-completed**
    - **ACES at CN, SCH, CHLA, CHC-ongoing**
    - **PLAT-05 - opened at SCH, CN**
    - **PLAT-06 - opened at SCH, CHLA**

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SA3: Expedite the Assessment of Key Biologic Correlates Uniquely Associated with Cellular Immunotherapy

- **Key Year 2 Outcomes**
  - **Web-based data platform, Labkey, implemented at 4 CPCI sites (SCH,CHLA, CHMC and BCHO) for 3 ongoing multi-center trials (PLAT -02, PLAT-05 and PLAT-06) with 85 trained users.**

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SA4: Facilitate Sustainable Access to the Most Promising Cellular Immunotherapies for Children

- **Year 2 Outcomes**
  - **Model of sustainability – U-01(NOT-OD-038) supplemental grant awarded –**

Notice of Award

RESEARCH PROJECT COOPERATIVE AGREEMENT **Federal Award Date:** 09/09/2020

Department of Health and Human Services
National Institutes of Health

NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES

Grant Number: 3U01TR002487-03S1
FAIN: U01TR002487

Principal Investigator(s):
Anurag Agrawal
JULIE R PARK (contact), MD

Project Title: Prospective evaluation of barriers to patient referral and enrollment in emerging cellular therapy trials: determining methods and structure to improve equity in future trial design

  - **Congratulations to Anu Agrawal and the Patient Advocacy Committee!**

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COVID-19 Barriers

- Increased clinical demands leading to less time for manuscript drafting
- Shift in IRB focus to COVID-19 projects resulting in longer time for study/survey approval
- Conversion of in-person meetings to virtual (Aim 1Competency/Proficiency Workshop) or deferral (mock FACT audits) or cancelation (CTSA meeting)

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Agenda

Aim 1 Presentation
Aim 2 Presentation

Break (9:50 – 10:00)

Aim 3 Presentation
Aim 4 Presentation

Break (11:00 – 11:10)

Scientific Talk
Wrap-up

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Aim 1

Catherine Lindgren
Christopher Brown
Stephanie Mgebroff

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Aim Overview

Develop the infrastructure to expand manufacturing capabilities of cellular immunotherapy products developed for treatment of pediatric disease

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Sub-Aims

- Define and align best practices across cGMP facilities
- Expand distribution of cellular therapeutics to sites of patient care
- Expansion of available cGMP facilities across CTSA

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Year 2 Planned and Actual Accomplishments

- Accomplishment 1

  Paper submitted, reviewers responses completed and re-submitted to ‘Cytotherapy’.
- Accomplishment 2

  Virtual half-day working group meeting held in May
- Accomplishment 3

  Personnel training, proficiency and competency testing, and job descriptions shared with consortium members

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Current Working Group Members

• Julie Annis	*Supervisor, BMT Laboratory – CHLA*
• Christopher Brown	*Director Manufacturing/ Process Dev. SCRI*
• Jonathan Esensten, MD, PhD	*Medical Director, Regulatory T Cell Manufacturing Group - UCSF*
• Chris Garbe	*Directory of Quality Charles C. Gates Biomanufacturing Facility – CU*
• Roger Giller	*Medical Director Charles C. Gates Biomanufacturing Facility – CU*
• Patrick Hanley, PhD	*Director, GMP for Immunotherapy - CNHS*
• Ashley Leinbach	*Project Manager, Regulatory T Cell Group – UCSF*
• Catherine Lindgren	*Senior Director, Therapeutic Cell Production & Quality Assurance – SCRI*
• Stephanie Mgebroff	*Director Quality Control SCRI*
• Matt Seefeldt	*Director of Cell Therapy Charles C. Gates Biomanufacturing Facility – CU*
• Jay Tanna	*Quality Assurance Lead – CNHS*
• Nan Zhang	*GMP Cellular Therapy Lead - CNHS*

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Year 3 Goals

- Disseminate best practices for annual competency/proficiency training of cGMP personnel
- Ongoing collaboration related to staff development pathways and retention by holding ad hoc discussions / meetings over the course of year 3.
- Initiate discussion related to supply chain challenges with the intention of generating a future goal in this area. (initially, supply chain risk assessment tool has been shared in year 2)
- GMP facility audits between 2 consortium sites (COVID-19 dependent)

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Metrics for Year 3 Goals

- Deliverable related to best practices in competency/proficiency (whitepaper, abstract, learning tool, TBD as discussions progress)
- # of ad hoc discussions between consortium group members
- # of GMP audits between consortium group sites

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Year 4 - 5 Goals

- In person working group meeting in year 4
- Continue to collaborate to support CHLA GMP facility start-up
- Continue to share learnings with Colorado
- Expanding distribution of cellular therapeutics- continue to develop operational structures/management
- Support business continuity through the next 1-2yrs in spite of unforeseen challenge(s) in the field – i.e. COVID-19

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Discussion Points

- Barriers
  - Working group bandwidth (due to COVID-19)
  - Travel restrictions

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Aim 2

Julie Park

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Aim Overview

Expand the clinical development of cell-based immunotherapy for pediatric disease

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Sub-Aims

- Establish the training and infrastructure to promote development and implementation of clinical immunotherapy trials in pediatric patients
- Utilize clinical trial designs that account for the unique constraints of rare disease-focused clinical trials in pediatric populations
- Ensure equitable access for all participants who may directly or indirectly benefit from cellular immunotherapies clinical trials

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Year 2 Accomplishments

- CPCI share point website developed to enhance collaboration
- Continued to share learnings from Immunotherapy Coordinating Center
  - Shared Project Management Tools for IND submission/trial development across CPCI sites
- Clinical Trial Collaboration ongoing
  - Completed PLAT-02 Phase 2 for pediatric ALL
  - ACES ongoing
  - Activated PLAT-05 and PLAT-06 at 2 or more sites (virtual training)
- Implemented sIRB for PLAT trials (SCH, CHLA)

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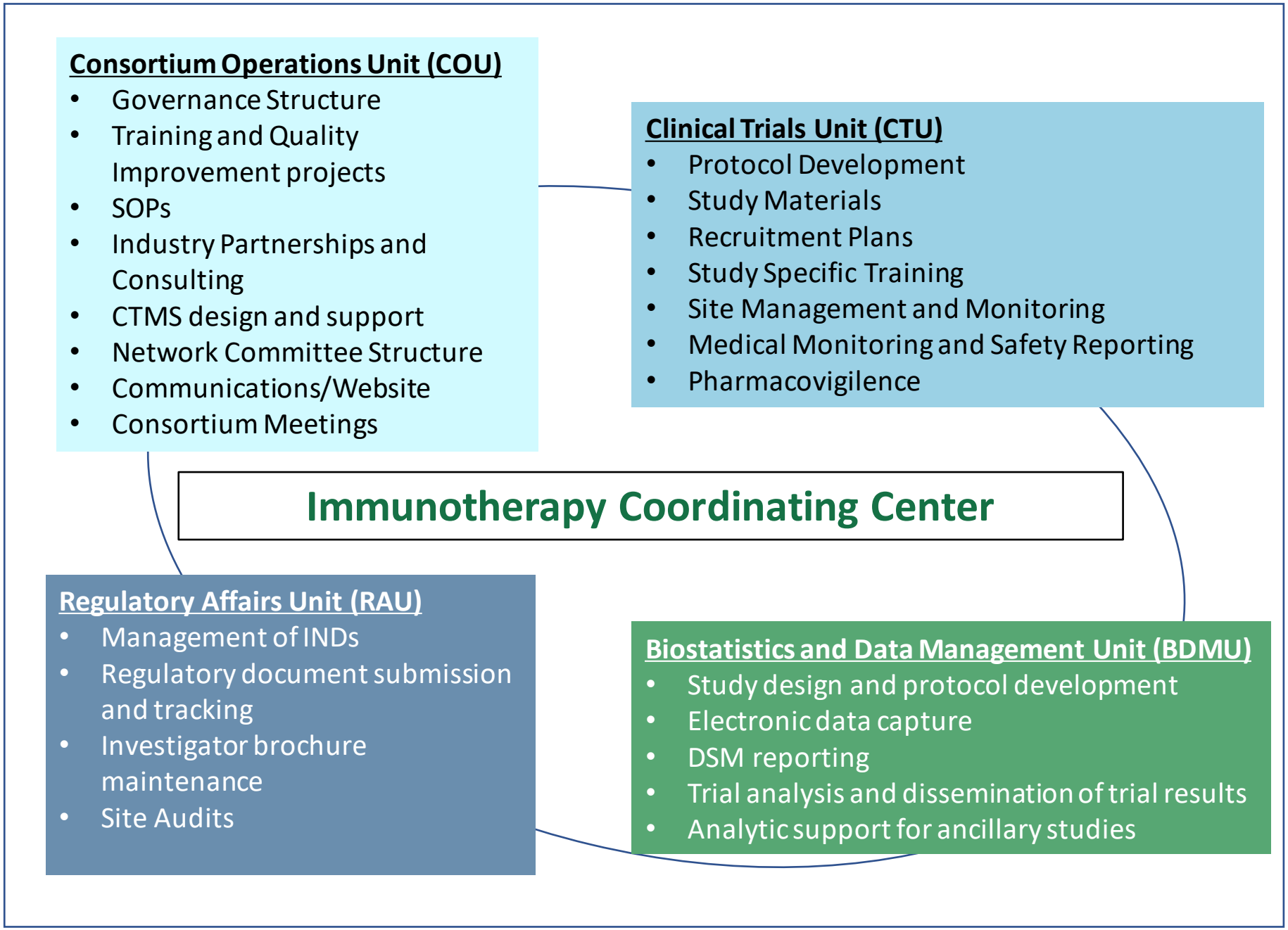
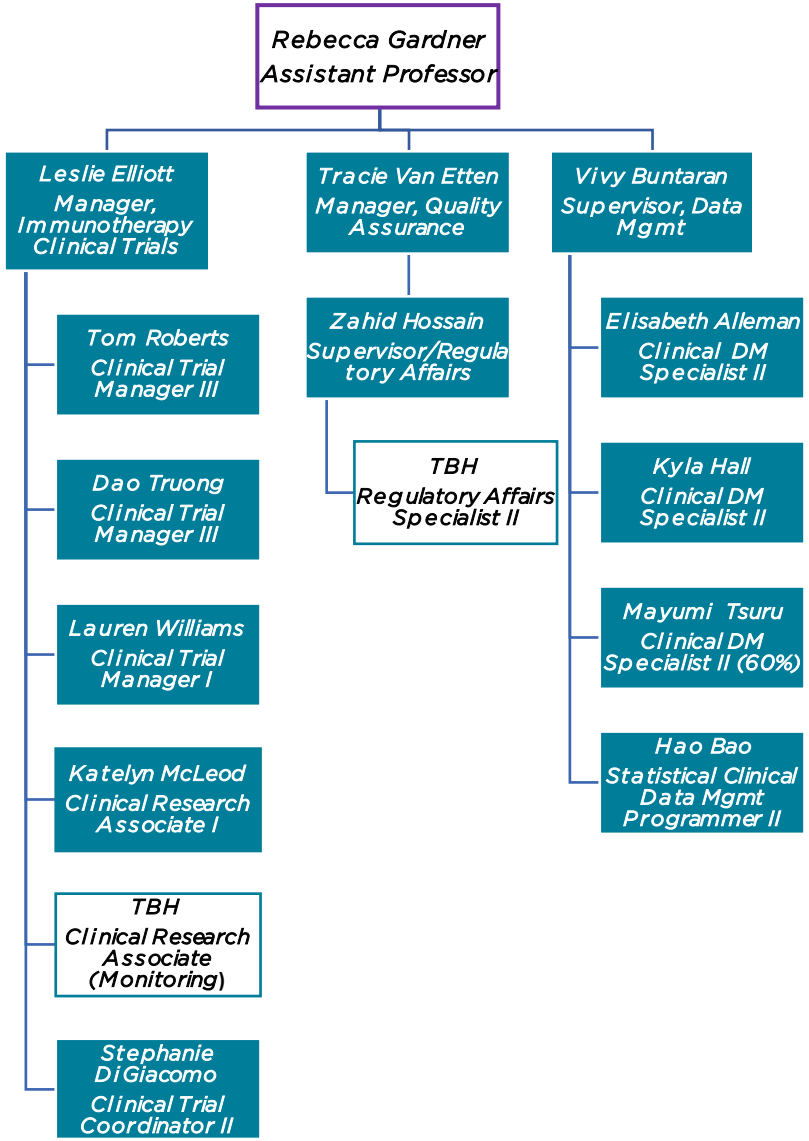
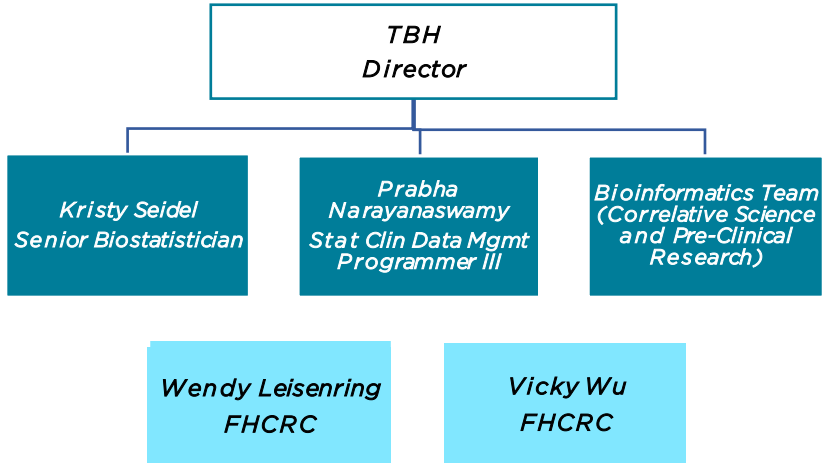

Consortium Operations Unit (COU)
• Governance Structure
• Training and Quality Improvement projects
• SOPs
• Industry Partnerships and Consulting
• CTMS design and support
• Network Committee Structure
• Communications/Website
• Consortium Meetings
Clinical Trials Unit (CTU)
• Protocol Development
• Study Materials
• Recruitment Plans
• Study Specific Training
• Site Management and Monitoring
• Medical Monitoring and Safety Reporting
• Pharmacovigilence
Immunotherapy Coordinating Center
Regulatory Affairs Unit (RAU)
• Management of INDs
• Regulatory document submission and tracking
• Investigator brochure maintenance
• Site Audits
Biostatistics and Data Management Unit (BDMU)
• Study design and protocol development
• Electronic data capture
• DSM reporting
• Trial analysis and dissemination of trial results
• Analytic support for ancillary studies

Clinical Development and Integrated Data Science Organizational Chart





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Clinical Trials

Name	NCT.gov	Site	Status
PLAT-02	NCT02028455	CHLA, SC, U-BCHO	Phase 2 leukemia completed 12/2019, open for lymphoma
ACES	NCT03475212	CHC, CHLA, CNMC, SC, UCSF	Open to accrual
PLAT-05 (mCD19xCD22)	NCT03330691	CNMC, SC	Amendment activated (revised CD22 vector)
PLAT-06 (human CD19)	NCT03684889	CHLA, SC, (U-BCHO pending)	Amendment activated (revised manufacturing)

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Year 3 Goals (beyond PRC and PAC)

- Establish tools needed to efficiently and effectively develop and implement clinical trials
  - CRF Global Library (delayed from Year 2 due to staff turn-over)
    - Export Cancer Data Standards Registry and Repository (caDSR) directly into RAVE approved August 2020!
  - Standardized toxicity grading for patients with CNS tumors (ICANS)
- Establish standards for monitoring and share clinical trial monitoring plans (delayed from Year 2 due to requirement for staff hire)
  - Monitoring for FDA Long Term F/u requirements
- Initiate Salesforce manufacturing

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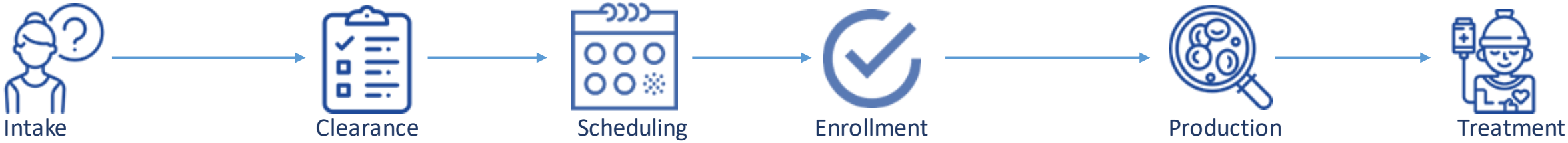
Immunotherapy Production Scheduling



System requirements:

- ✓ Track clinical trial inquiries and referrals at SCH through the screening and clearance process
- ✓ Allocate TCPC cell therapy production slots to SCTx clinical trial programs and sites
- ✓ Manage utilization of production slots among clinical trial programs and sites
- ✓ Reserve production slots for prospective participants prior to enrollment
- ✓ Flexible and scalable to accommodate growth

Salesforce: Intelligent Customer Success Platform

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User Profiles and Key Activities

SCH INTAKE

- Creates Contacts and Inquiries for clinical trials at SCH
- Tracks pre-screening process

SCH SITE RESEARCH

- Creates Contacts, Trial Encounters and Trial Products
- Manages clearance process
- Requests Production Slot reservations

EXT SITE RESEARCH

- Creates Contacts, Trial Encounters and Trial Products
- Manages clearance process
- Requests Production Slot reservations

ICC RESEARCH

- Allocates Production Slots to clinical trial Programs
- Reserves Production Slots for all Trial Products

PRODUCTION (TCPC)

- Creates Production Slots
- Confirms Reservations for all Trial Products
- Tracks Product and Production Slot Status

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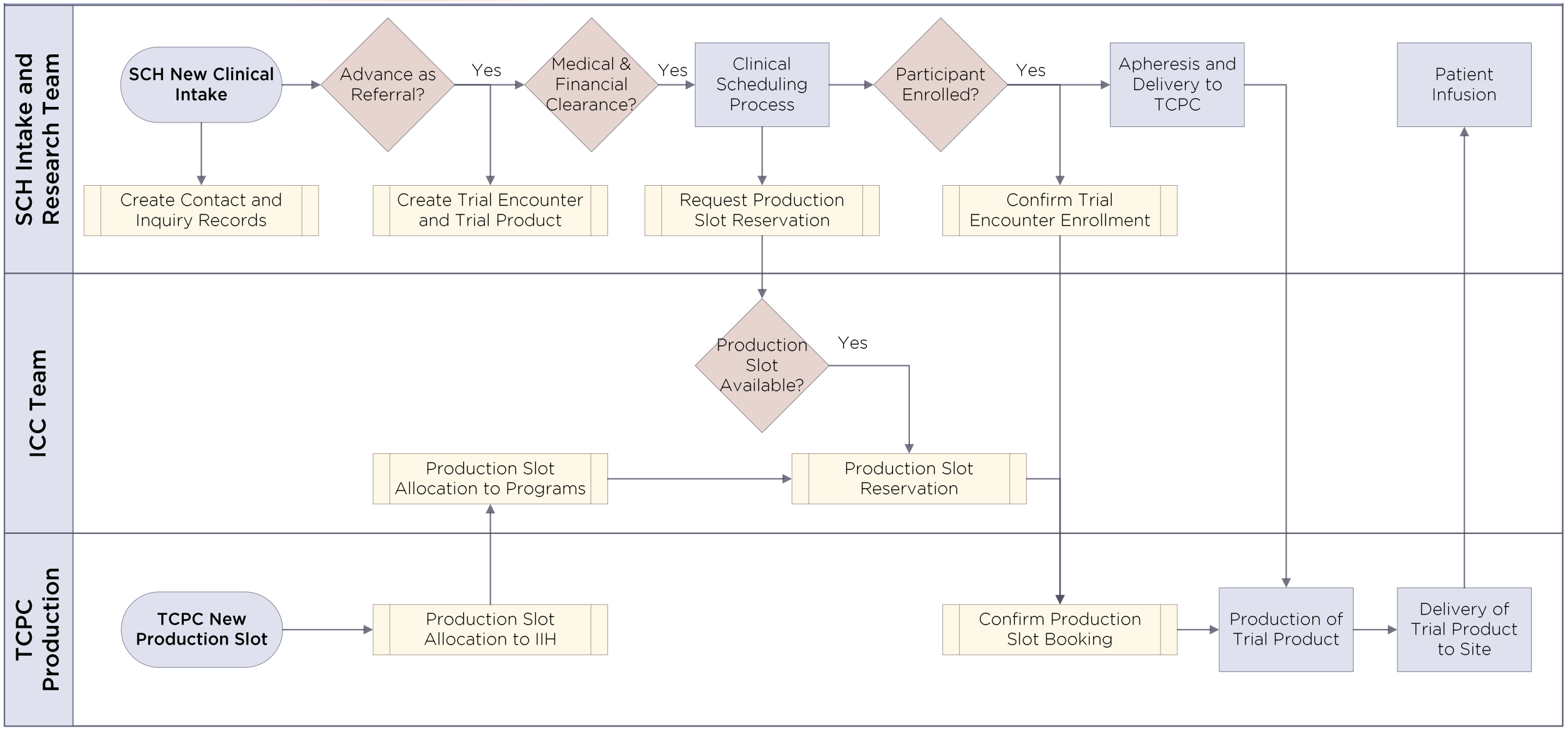
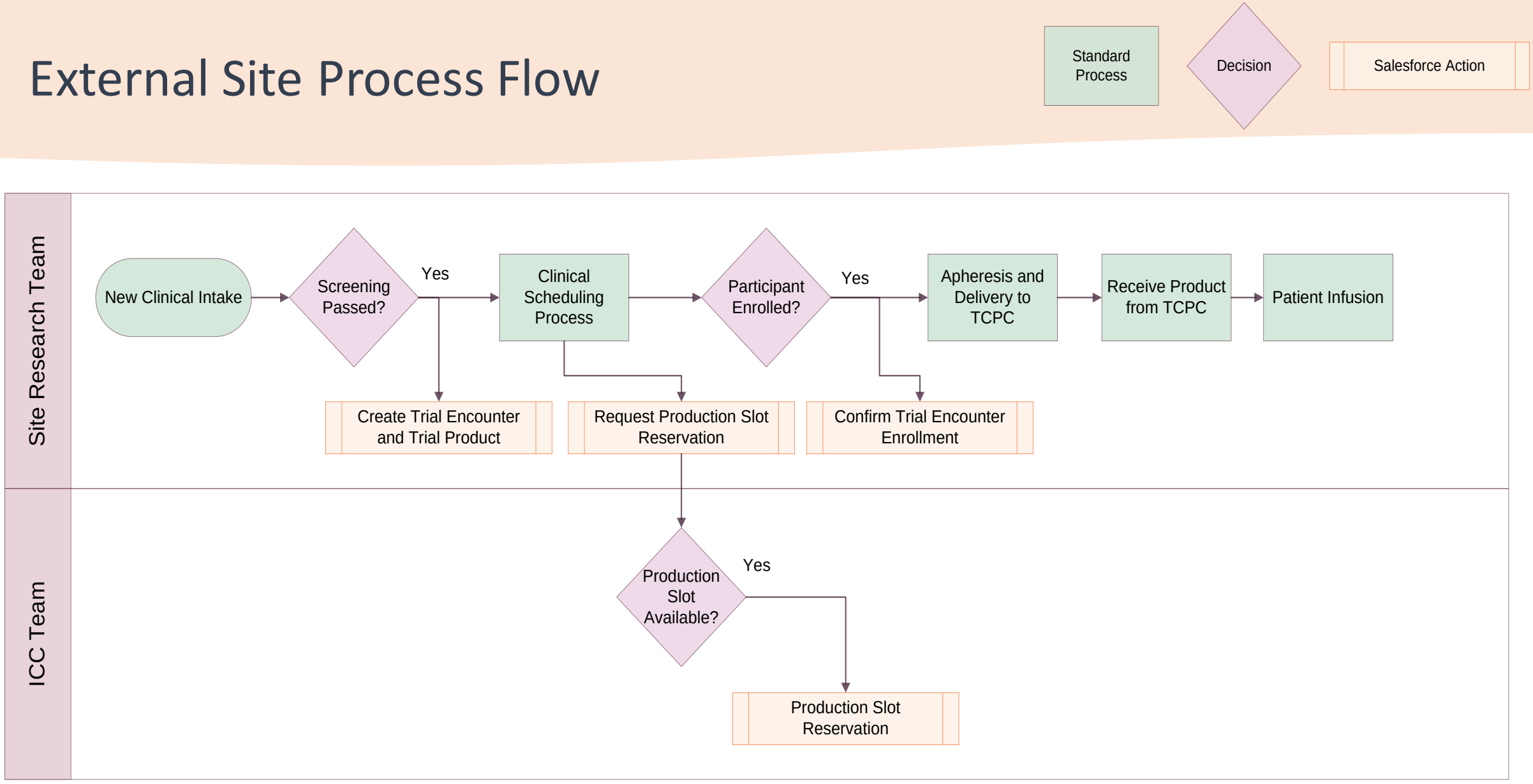
SCH Process Flow



External Site Process Flow



Year 4 - 5 Goals

- Evaluate accuracy of standardized timelines
- Further expand trials beyond cancer
- Evaluate use of training tools and compliance to protocol
- Expand utilization of sIRB

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Protocol Review Committee

Michael Verneris

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Year 2 Planned and Actual Accomplishments

- Well attended, monthly interactive meetings

✓ Drafted a protocol to test the delivery of Prevnar13 prior CD19 or CD22 CAR T

❑ Share a biospecimen collection protocol

❑ Develop “modular” protocol templates

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Current Committee Members

- Dana Dornsife
  - *Founder and Chair - Lazarex Cancer Foundation*
- Leslie Elliott
  - *Manager, Clinical Trial Management Unit, ICC - SCRI*
- Rebecca Gardner, MD
  - *Pediatric Hematologist-Oncologist - SCRI*
- Michael Keller, MD
  - *Pediatric Immunologist – CNHS*
- Jennifer Michlitsch, MD
  - *Pediatric Hematologist-Oncologist - BCHO*

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Current Committee Members (cont)

- Julie Park, MD
  - *Bushnell, Towne and Wilkerson Endowed Chair in Pediatric Neuroblastoma; Medical Director, ICC - SCRI*
- Bonnie Ramsey, MD
  - *Director, Center for Clinical and Translational Research; Associate Director, Pediatric Clinical Research Center - SCRI*
- Agne Taraseviciute, MD
  - *Pediatric Hematologist-Oncologist – CHLA*
- Michael Verneris, MD
  - *Pediatric Hematologist-Oncologist – CHC*
- Vicky Wu, PhD
  - *Assistant Member, Clinical Research Division; Assistant Member, Public Health Services Division - FHCRC*

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Year 3 Goals

- Complete Prevnar13 protocol
- Sharing biobanking protocols
  - Seattle
  - UC Denver
  - ? Other
- Protocol Templates, with global library of sections
- IND Templates

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Metrics for Year 3 Goals

- Final submission of Prevnar13 protocol
  - Smart IRB approval
  - Creation of REDCap database
  - Open and enrolling
- Completion of the “modular” protocol template
- Completion of biorepository template
  - Where to post these templates?

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Year 4 - 5 Goals

- Use consortium for another joint clinical trial/project
  - T APC at a multicenter?
  - Novel reinfusion protocol?
  - Collection of samples for joint lab studies?
- Consider making a template IND submission
- Other?

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Discussion Points

- What are we missing and where are the gaps?
- Desire to do something meaningful with years 4-5
- The above will help w/future grants and sustainability

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Patient Advocacy Committee

Anurag Agrawal

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Year 2 Planned and Actual Accomplishments

- Further development and expansion of Patient Advocacy Committee members and goals
- ASPHO abstract
- Awarded OD-20-038 supplemental grant to U01
- Ongoing work on multiple projects

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Barriers to Year 2 Goals

- Multiple barriers in Year 2
  - Delay in IRB approval due to COVID restrictions
  - Difficulty finding help for data collection
  - Lack of funding to sites
- Most of these have been addressed going into Year 3

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Mission Statement

Ensure cellular therapy trial development includes discussion and strategies to ensure equitable access, with input by families about what is important to them

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Current Committee Members

- Anurag Agrawal, MD
  - *Pediatric Hematologist-Oncologist - BHCO*
- Paibel Aguayo-Hiraldo, MD
  - *Pediatric Hematologist-Oncologist - CHLA*
- Lourdes Baezconde-Garbanati, PhD
  - *Director, Community Outreach and Engagement; Associate Dean, Community Initiatives, Keck SOM - USC*
- Tumaini Coker, MD, MBA
  - *Research Director, Center for Diversity and Health Equity - SCRI*
- Hema Dave, MD, MPH
  - *Pediatric Hematologist-Oncologist – CNH*

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Current Committee Members (cont)

- Dana Dornsife
  - *Founder and Chair - Lazarex Cancer Foundation*
- Anurekha Gollapudi, MD
  - *Pediatric Hematology-Oncology Fellow - SCRI*
- Amy Keating, MD
  - *Pediatric Hematologist-Oncologist - CHC*
- Adam Lamble, MD
  - *Pediatric Hematologist-Oncologist – SCRI*
- Jonathan Marron, MD, MPH
  - *Pediatric Hematologist-Oncologist, Clinical Ethicist - BCH*

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Current Committee Members (cont)

- Diana Merino Vega, PhD
  - *Science Policy Analyst - Friends of Cancer Research*
- Mark Walters, MD
  - *Director, Blood and Marrow Transplantation Program – BHCO*
- Ben Wilfond, MD
  - *Director, Treuman Katz Center for Pediatric Bioethics - SCH*
- Lena Winestone, MD, MSHP
  - *Pediatric Hematologist-Oncologist - UCSF*

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Year 3 Goals

- Completion of ongoing projects
  - Retrospective review of ALL patients treated at consortium institutions
    - Currently all sites are in data collection phase (to be completed end of November with data analysis and manuscript to follow—goal end of year)
  - Retrospective patient/family survey with subsequent semi-structured interviews
    - Currently pending Seattle IRB approval
    - To then be submitted to additional consortium sites
    - Development of semi-structured interview
      - To be done in parallel with U01 supplemental grant aims
    - Goal completion April 2021

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Year 3 Goals

- Planning and accomplishment of NOT-OD-20-038 supplemental grant aims
  - Prospective evaluation of families enrolling on CAR-T trials at consortium sites through semi-structured interviews
  - Provider survey and follow up semi-structured interviews to determine referral decision-making
  - Upcoming planning meeting
    - Sites with funding to assist with IRB submission, prospective patient consenting and identification/communication with referring providers
    - Development of survey tools
    - Development/implementation of semi-structured interviews
  - Completion July 2021

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Year 3 Goals

- Further understanding of the insurance coverage landscape for FDA-approved and CAR-T trials
  - Currently working with Seattle financial analysts to determine what data we have
  - Contacting regional Novartis reps to assist with Kymriah coverage
  - Goal completion pending additional projects

Metrics for Year 3 Goals

- Manuscript for retrospective review of ALL patients accessing cellular therapy trials across consortium sites
- Manuscript for retrospective patient/family survey
- Completion of provider surveys and retrospective and prospective semi-structured interviews

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Year 4 - 5 Goals

- Development of focus groups to further understand the barriers based on surveys and interviews
- Furthering work regarding coverage landscape for FDA-approved and cellular therapy trials
  - This will inform further advocacy/policy work to improve equitable access
- Further grant exploration
  - TR-20-001 (Ethical Issues in Translational Science Research)
  - Others
- Develop collaboration with local CTSI/CCHE

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Planned Outputs

Manuscripts for the retrospective review and family survey

Completion of aims for supplemental U01 grant

Completion of semi-structure interviews for families who have completed CAR-T trials

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Discussion Points

- How to access families who do not participate in trials
- Is there overlap between other committees (especially in regard to information dissemination, education regarding trials)?
- How best to collaborate with local CTSI/CCHE?
- Additional grant opportunities

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Aim 3

Ashley Wilson

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Aim Overview

Enhance rigorous assessment of key biologic correlates uniquely associated with cellular immunotherapy mechanism(s) of action in conjunction with safety and outcome metrics

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Sub-Aims

- Develop reproducible sample collection and process standards for use across Consortium trials
- Apply a web-based data platform for the integration, analysis visualization and sharing of data across sites
- Establish outcome measures to assess safety, efficacy and promote rapid translation of findings

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Year 2 Planned and Actual Accomplishments

- Develop collection, shipping, and processing standards for correlative specimens for use across sites.
  - Results/recommendations from gap analyses in manuscript preparation
  - Defined minimum assays to analyze cellular immunotherapy correlatives (flow and cytokine)
- Expand use of LabKey (LK) to share data with participating study sites.
  - New versions of SOPs created: 1) basic use of LK and 2) LK administration to classify user groups, permissions & access, and folder configuration to ensure high data integrity/security. Includes access request form/approval (R&U to document training by user s)
  - Uni-directional data reporting (CAR persistence and RCL) to **5 sites**: SCH (Seattle), CHLA (Los Angeles), BCHO (Oakland), CNMC (DC) & BCCH (British Columbia). 3 multi-site trials tracked in LK (PLAT-02, -05, -06)
  - Multi-directional use implemented for a separate clinical lab to upload their datasets for reporting to **3 sites** (PLAT-05).
- Initiate biobanking efforts to retain leftover CAR T specimens.
  - Established working group and began building tools/infrastructure
- Accomplishments provide robust data to inform future research, and consistency in correlative sample processing and basic use of LK leads to greater confidence in data generated and reported.

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Current Working Group Members

- Hisham Abdel-Azim, MD, MS
  - *Pediatric Hematologist-Oncologist - CHLA*
- Hema Dave, MD, MPH
  - *Pediatric Oncologist - CNHS*
- Kimberly Jordan, PhD
  - *Assistant Director, Human Immunology & Immunotherapy Initiative - CU*
- Ashley Wilson, PhD
  - *Manager, Human Immunotherapy Correlative Studies – SCRI*
- Wenjun Huang, PhD (LabKey)
  - *Lead Data Scientist, SCRI CSL*
- Silvia Yu (LabKey)
  - *LabKey web developer*

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Year 3 Goals

- **Goal 1:** Manuscript accepted summarizing best practices for collection, shipping and processing of clinical correlates.
- **Goal 2:** Create processing standards for cerebrospinal fluid (CSF) and define key analytes for cellular immunotherapy trials.
- **Goal 3:** Develop additional LK SOPs and training tools/resources for multi-directional use between sites.
- **Goal 4:** Develop template biobank consent language, new processes, and SOPs to share with sites.
- **Goal 5:** Develop and share a statistical analysis plan for correlative studies with sites.

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Metrics for Year 3 Goals

- Manuscript published and shared with NIH-NCATS
- New LabKey SOPs and training tools developed, document training at sites (track R&U forms)
- Extend multi-directional LabKey usage to a multi-site trial
- Biobank consent template language developed and shared with sites
- New biobank workflow processes are developed, documented in SOPs and shared with sites
- SAP created, statistical methods and analytical approaches for biologic correlates are documented

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Planned Outputs

Manuscript highlighting correlative sample collection, shipping and processing for flow & cytokine analysis.

Statistical analysis plan created and documented

SOPs and **training documentation forms** for extending LabKey usage

Template biobank consent language to share with sites

(language IRB approved with PLAT-07!)

Biorepository process SOPs to share with sites

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LabKey Biobanking – Live Demo

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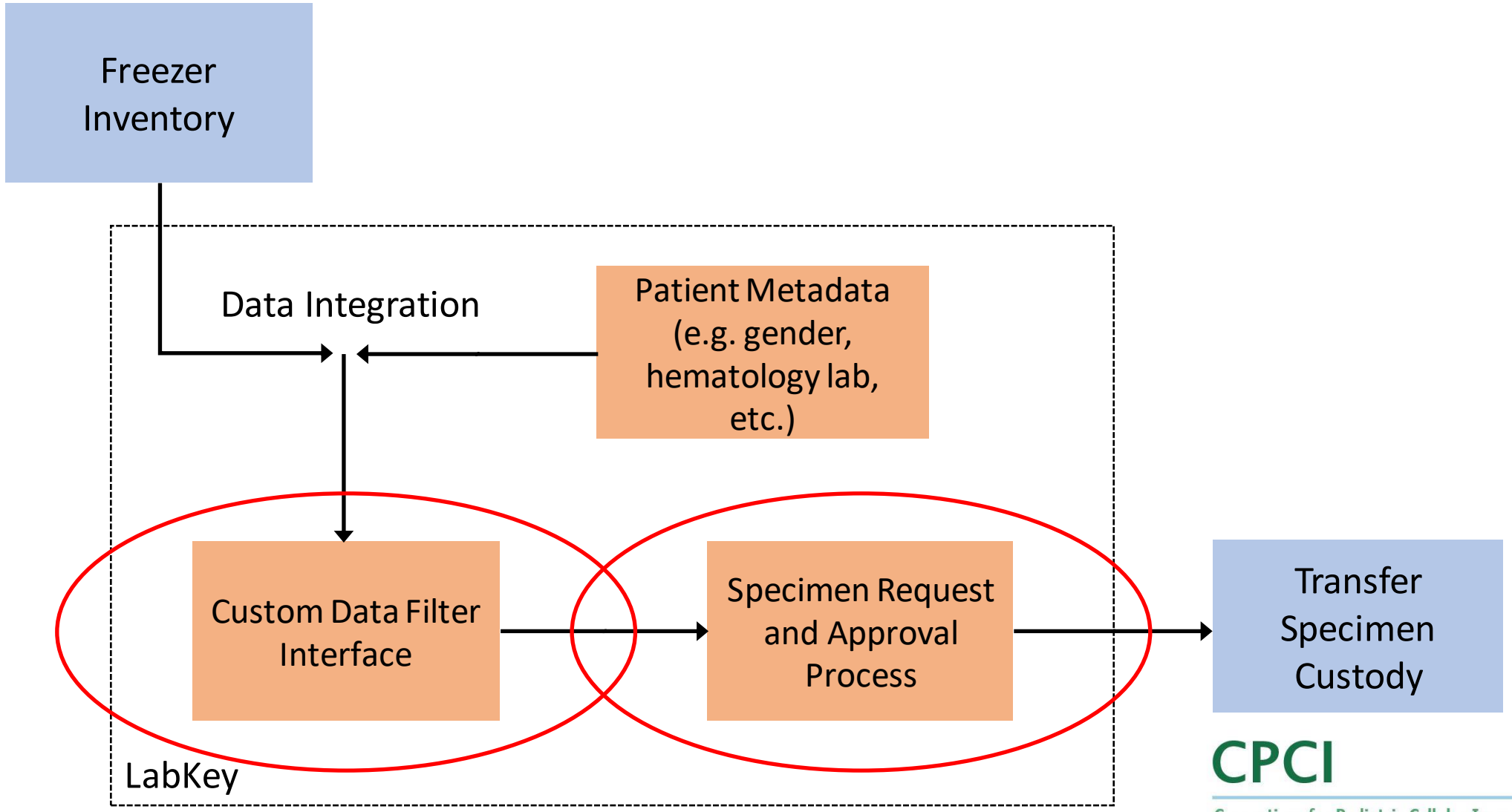
Proposed Biorepository Workflow in LK



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Custom Data Filter Interface

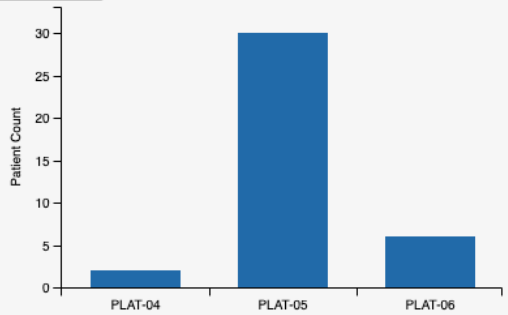
Patient Metadata Filter

Step 1. Specimen Filters

Total Patient Match: 38

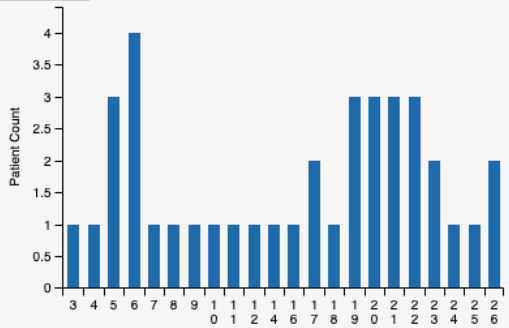
Study

Select Study



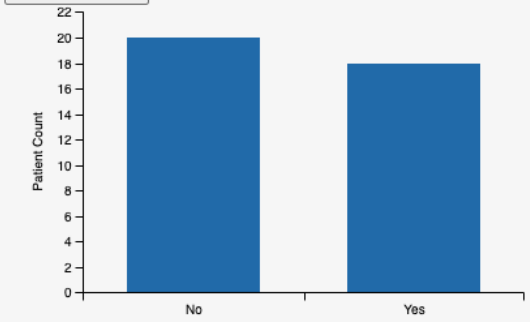
Age

Select Age



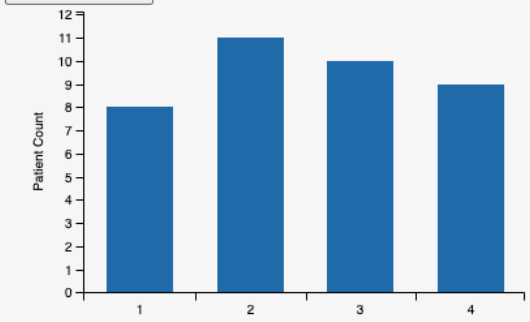
Luminex

Select Luminex



Max CRS

Select Max CRS



Render Search Result

Freezer Inventory Filter

Step 2. Vial Selection

Design | Check Inventory | 1 - 14 of 14

Product Registry

Participant ID	Product Id Appendage	TCPC Lot ID	Product Type	Cell Type	Generation	Spacer	Inventory Summary	Total Cells in All Vials (x10^6)
00961_CS_S003	N/A		Final Product	CD4/CD8	2G/3G	LS	1 obtained date(s) totaling 5 vials.	760
00961_CS_S005	N/A		Final Product	CD4/CD8	2G/3G	LS	1 obtained date(s) totaling 2 vials.	488
00961_CS_S005	N/A		Negative Fraction	N/A	2G/3G	LS	1 obtained date(s) totaling 18 vials.	990
00961_CS_S005	N/A		Starting Product	CD4	2G/3G	LS	1 obtained date(s) totaling 9 vials.	95.4
00961_CS_S005	N/A		Starting Product	CD8	2G/3G	LS	1 obtained date(s) totaling 18 vials.	190.8
00961_CS_S010	N/A		Negative Fraction	N/A	2G/3G	LS	1 obtained date(s) totaling 18 vials.	345.6
00961_CS_S010	N/A		Starting Product	CD8	2G/3G	LS	1 obtained date(s) totaling 11 vials.	114.4
00961_CS_S010	N/A		Starting Product	CD4	2G/3G	LS	1 obtained date(s) totaling 9 vials.	95.4
00961_CS_S011	N/A		Starting Product	CD4	2G/3G	LS	1 obtained date(s) totaling 5 vials.	50
00961_CS_S011	N/A		Starting Product	CD8	2G/3G	LS	1 obtained date(s) totaling 5 vials.	50
00961_CS_S012	N/A		Starting Product	CD4	2G/3G	LS	1 obtained date(s) totaling 5 vials.	50
00961_CS_S012	N/A		Starting Product	CD8	2G/3G	LS	1 obtained date(s) totaling 3 vials.	30
00961_CS_S030	N/A		Final Product	CD4/CD8	2G/CD8alpha	LS		
00961_CS_S030	N/A		Starting Product	CD4/CD8	2G/3G	LS	0 obtained date(s) totaling 5 vials.	50

Sample Request & Approval Process Highlights

- Role-based permission groups ensure proper data access for different user groups.
  - Requestor, Reviewer, Coordinator, etc.
- Customizable request status and review checkpoints facilitate flexible workflow control.
  - Submitted, Under Review, IRB Approval, Approved, etc.
- Fully supported comments, file attachments, email notifications allow request details to be captured and stored in one place.
- Changes to the request are tracked and can be accessed in audit history.

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Request & Approval Interface

Specimen Request 3 DNA

Request Information

Requester	Wenjun Huang
Requesting Location	CSL
Request Date	2020-06-15 15:20:23
Description	Assay Plan: Plan to run some assay on LTFU 18yr old patient samples Shipping Information: shipping from CSL Comments: [Not provided] Additional Comments: [Not provided]
Status	Request Submitted

Attachments
- Screen Shot 2020-06-16 at 7.56.02 AM.png
- LabKey User Account Request - Remote.docx

Document Attachments

Request Details

VIEW HISTORY | UPDATE REQUEST | ORIGINATING LOCATION SPECIMEN LISTS | PROVIDING LOCATION SPECIMEN LISTS

Current Requirements

Actor	Location	Description	Status	
Specimen Requestor	unknown	test requirement	Incomplete	DETAILS
Reviewer	N/A	need review	Incomplete	DETAILS

Add Requirement

Approval Requirements (Checkpoints)

Associated Specimens

Export | Specimen Search | Upload Specimen Ids | Remove Selected

Requested Vial List

	Participant Id	Global Unique Id	Location	Sample Comments	Description	Freeze Thaw Cycle	Sample Source	Cell Source	Name	Name3	Name4	Draw Date	DMAGE
[history]	27924	1027924	CS-FRZ80-01→Drawer 1→Shelf 2→Rack 11→Column 2→3					Whole specimen	14602_CS_S1013	4M	PB	2017-05-02	18

DNA: Specimen Request Notification

wenjun.huang@seattlechildrens.org <wenjun.huang@seattlechildrens.org>
To: Huang, Wenjun

Specimen request #2 was updated in DNA.

Request Details

Specimen Request	2
Destination	CSL
Status	Medical Director Review
Modified by	wenjun.huang@seattlechildrens.org
Action	Comment added.
Attachments	CSL External Read and Understood Remote Signoff.docx Book3.xlsx

Current Comments
Please fill out this form

Assay Plan:
I need these two vials for some assays

Shipping Information:
ship to me directly

Comments:
Passing on to CSL internal review

Specimen List (Request Link)

	Global Unique Id	Location	Sample Comments	Name	Description	Freeze Thaw Cycle	Sample Source	Cell Source
1	1027885	CS-FRZ80-01→Drawer 1→Shelf 2→Rack 11→Column 2→3		14602_CS_S1031 PreA.12 PB				Whole specimen

Email Communication

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Discussion Points

- Is there value in reducing the complexity of clinical protocols by standardizing correlative practices (e.g. serum vs. plasma isolation)?
- What are ways that we can better collaborate with the PRC (Aim 2) to share biobanking consent language and/or protocol design?
- Advantages and/or risks to generating a “virtual” biobank?
- How can we extend our reach within the greater CTSA community (abstract presentations available during COVID)?

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Aim 4

Julie Park
Mike Jensen

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Aim Overview

Facilitate sustainable access to the most promising cellular immunotherapies for children

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Sub-Aims

- Sustain through extramural grant funding and industry co-development

- Establish an organizational and financial model to develop a sustainable infrastructure through hospital-centric support

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Year 2 Accomplishments

- CTSA/UO1 supplemental R21 grant award

- Formalized philanthropic support for PLAT series clinical trial
  - Funding coordinating center regulatory/clinical trials work
  - Funding manufacturing
  - Funding site costs (per patient reimbursement)

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Year 2 Accomplishments

- Formalized industry collaboration for protocol development
  - BlueBird Bio – AML
  - Umoja – Solid Tumor

- CureWorks
  - Standardization of General Member Agreement & Clinical Trial Participation Agreement
  - Regularly scheduled membership meetings
  - Expansion of membership

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CureWorks Members

CureWorks
A NEW WAY TO BETTER CURES

Children's National

Seattle Children's
HOSPITAL · RESEARCH · FOUNDATION

BC Children's Hospital
An agency of the Provincial Health Services Authority

Riley Children's Health
Indiana University Health

Children's Hospital LOS ANGELES

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Year 3 Goals – Grants/Biotech Collaborations

- Identify additional grant opportunities
  - NIH Diversity Supplement Connections Program [seattlechildrens.org] Tumani Coker at Seattle Children's, Lisa Manhart at UW School of Public Health, Chris Li at Fred Hutch—plan to create a centralized site through ITHS where PIs and trainees can go to access diversity supplement opportunities.
  - Submit SPORE for CNS tumors (CNMC, SCH)
  - Dedicated Steering Committee meetings for grant planning
- Collaborative work to identify additional philanthropic support
- Initiate one trial with industry collaboration

Year 3 Goals - CureWorks

- Seek optimum size to be transformative in speed and development of trials
- Stabilize finances and business operations to provide maximum financial resilience
- Manage diversity of CW members and its patients to enhance scientific progress
- CureWorks:CPCI collaboration will seek CTSA resources to leverage trial implementation at University of Indiana/Riley Children's Hospital

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Discussion Points

- What areas can be most successfully leveraged for grant funding?
- How do we engage with biotech to foster pediatric applications of key technologies?
- How do we activate our respective hospital foundations to collaborate to more effectively compete in the philanthropy marketplace?

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