CALIFORNIA KIDS CANCER COMPARISON

A DEMONSTRATION PROJECT OF THE CALIFORNIA INITIATIVE TO ADVANCE PRECISION MEDICINE
OTHER PARTNERS AND COLLABORATORS

BC Cancer Agency
CARE + RESEARCH

STANFORD
SCHOOL OF MEDICINE
Stanford University Medical Center

UCSF Benioff Children's Hospital

UCIrvine

UC Braid
University of California Biomedical Research Acceleration, Integration & Development

UC Davis

Hyundai Cancer Institute

CHOC Children's

BC Children's Hospital

Jacob's Heart
Children's Cancer Support Services

KIDS vs CANCER

NuMedii

Cisco

Unravel pediatric cancer

UCSD

tgen

CIRM

CARIS Life Sciences

NEW YORK GENOME CENTER®

USC

Alex's Lemonade Stand Foundation for Childhood Cancer
PROBLEM: GENETIC DATA ON CHILDHOOD CANCERS ARE ISOLATED IN MEDICAL AND RESEARCH SILOS

No one institute has enough on its own to make progress

Every doctor needs to be able to compare their patient’s cancer to all the other cancers
WE ARE BUILDING A GLOBAL DATA SHARING PLATFORM

We hope to at least double the yield of clinical genomic trials using genome comparisons
Clinical Genomics Trials
-- UCSF, PNO (15 pts)
-- UCI, CHOC (40 pts)
-- Stanford (100pts)

Genomic characterization data; Clinical data

Clinical leads

Tumor Boards

CLIA validation

Outcome measures:
• New clinical leads
• New evidence for clinical leads
• New/refined molecular diagnoses
Patient 1: 8 year-old boy, dural-based sarcoma
Treated with aggressive chemotherapy, autologous stem cell transplant, and local radiation

Two years later—metastases to lungs
• No standard treatment options, so Patient 1 enrolled in a personalized genomics clinical trial
• Tumor genome sequencing revealed an EWSR1-ATF1 gene fusion, which has no obvious indicated therapies
• Now what?
Tumors arranged by similarity of gene expression profiles.

Patient 1 in the context of other cancers.
JAK/STAT PATHWAY IS A TARGET FOR PATIENT 1

- JAK/STAT is downstream of both RTKs (ALK) and cytokines
- Both IL6 and ALK are elevated in Patient 1

RUXOLITINIB AND CRIZOTINIB ARE APPROVED FOR ADULTS AND IN PEDIATRIC CANCER CLINICAL TRIALS
“I restarted him on ruxolitinib again to inhibit JAK and he feels amazing again! eating, increased energy and feels like a million bucks again.”

—Treating Oncologist, August 17, 2015
OPPORTUNITIES FOR COLLABORATION

• Unclear regulatory landscape
  • Each CKCC trial has own IRB protocol. Does UCSC require separate IRB protocol for its component?
  • IRB Authorization Agreements with each institutional IRB (CHOC, UCSF and Stanford)?
  • A framework for how to develop trials that involve several IRBs is needed
• Rapidly changing regulatory landscape
  • Even if not today, this work may be regulated by CLIA/FDA/Board of Medicine
  • A watchdog to keep track of the changing regulatory landscape in clinical genomic trial space is needed
• Unclear compliance needs
  • What controls should we use when handling genetic data? FISMA versus HIPAA versus “best practices”
  • Are genetic data considered health information? Different institutions and IRBs can disagree on this.
  • Unified standards across the UCs and beyond are needed
• Global Alliance for Genomics and Health is doing work in this area, but institutional IRBs are often not aware of it
PARTNERS AND COLLABORATORS