NCI Childhood Cancer Data Initiative

Update from Year 1 and Framework for Years 2-10

Joint Meeting of the NCI National Cancer Advisory Board and the NCI Board of Scientific Counselors

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Outline of Presentation

• Goals of CCDI and update of progress from Year 1

• Program structure and goals for future years

• Proposed Governance
The goal of the CCDI is to build a community of pediatric cancer researchers, advocates, families, hospitals, and networks committed to sharing data to improve treatments, quality of life, and survivorship of every child with cancer.
Year 1 Accomplishments
CCDI Working Group Report

Landscape of Pediatric/AYA Cancer Research Data & Needs Analysis

Types of Data for Collection and Aggregation

Potential Barriers to Progress

Generating New Data

Distinction Between Research & Clinical Data

Engaging Diverse Array of Stakeholders for Input

Potential Opportunities for Transformative Discoveries

24 specific recommendations
Year 1 – Fiscal Year 2020 Portfolio

• Develop a **catalog** of all available childhood cancer data registries and data repositories; landscape analysis complete, starting to build a pediatric data catalog prototype – $2M

• Develop a Federated Pediatric Cancer Data Ecosystem of research repositories & patient registries (initial efforts to lay foundation):
  ✓ Establish **National Childhood Cancer Registry** (NCCR) to link clinical patient data – $7M
  ✓ Build a data infrastructure for preclinical data models that can inform/validate FDA Relevant Molecular Targets List; establishing **Preclinical Pediatric Data Commons** (PPDC) & creating pediatric-specific instance of Open Targets analytic platform – $3M
Year 1 – Fiscal Year 2020 Portfolio (2)

- Aggregate existing data through transfer of patient-linked clinical (phenomics, treatment) and molecular data (genomics, proteomics, imaging, preclinical) and analytic tools to NCI resources – $8M
  - Cancer Center Supplements (registries and data repositories)
  - Childhood Cancer Survivor Study (CCSS) clinical data

- Generate new cancer models and sequence data to fill gaps for key NCI initiatives; data will be submitted to NCI databases – $9M
  - Diagnostic tumors and germline samples from Pediatric MATCH
  - Up to 1,400 PDX/cell lines
  - Secondary cancers from CCSS
  - Organoids and cell lines, including CNS tumors
Year 1 – Fiscal Year 2020 Portfolio (3)

• Develop or adapt **analytic tools & computational methods** using grants and contracts for use in childhood and AYA cancer research – $7M
  ✓ Automated curation of data (e.g., natural language processing) for refining, scaling & real-world data capture
  ✓ Interpreting pathology images & patient reports
  ✓ Pediatric data model & terminology harmonization
• Establish a **Rare Pediatric Tumor Cell Atlas** from tissues obtained through the NCI Pediatric Rare Tumor Network – $4M
Year 1 – Fiscal Year 2020 Portfolio (4)

• Supplement **intramural** and **extramural grants, contracts** – $10M
  ✓ Etiological and clinical risk prediction and genetic susceptibility for the development of childhood malignancies
  ✓ Patient-reported outcomes and toxicities in pediatric and AYA patients and survivors
  ✓ Improvements to childhood clinical trials data reporting
  ✓ Molecular pathogenesis of pediatric and AYA cancer development
Vision for Years 2 - 10

Goals, program structure, and governance for future years
Foundational Goals for Years 2-10

• Gather data from every child/AYA diagnosed with cancer, regardless of where they receive their care
• Develop core data from consented patients, including tumor and germline molecular characteristics, to enable research using patient-level data in a secure and de-identified way
• Create a system that can bring data of different types, from different sources together in a way that incentivizes researchers to query the available data in new ways
CCDI Implementation for Years 2-10

4 working groups co-chaired by NCI and extramural experts and made up of NCI staff, external experts and advocates
Identifying and Filling Gaps: Adding to the Existing Landscape

- Project: EveryChild
- Kids First
- Childhood Cancer Data Platform
- St. Jude Cloud
- Ped cBioPortal
- Treehouse
- TARGET
- National Childhood Cancer Cohort
- Childhood Molecular Characterization Protocol

Coordination Center
CCDI National Childhood Cancer Cohort

• Gather data from every child diagnosed with cancer in the United States
• It will:
  ✓ Capture the cancer care trajectory of children and AYAs, including care provided outside of COG and other networks, to identify gaps and disparities in care and outcome
  ✓ Track biospecimen availability
  ✓ Provide access to data from underserved patients
  ✓ Provide for consistent research consent
  ✓ Allow for long-term follow up of childhood cancer patients
• A critical component of this effort will be the National Childhood Cancer Registry (NCCR)

Working group chaired by NCI and an extramural expert
A national strategy, building on efforts including COG’s Project:EveryChild, to offer appropriate clinical and molecular characterization to every child with cancer that:

- Enables discovery when these and other data are connected
- Defines a minimum set of molecular diagnostics to be collected for every pediatric and AYA cancer patient
- Is accessible to all children with cancer, including those treated at community-based institutions; provide access to underserved pediatric cancer patients
- Clinical sequencing of ~3,000 patients
- Align with Rare Pediatric Tumor Cell Atlas

*Working group chaired by NCI and an extramural expert*
Childhood Molecular Characterization Protocol

• Expand access to comprehensive molecular sequencing as a step towards the goal of reaching all children with pediatric cancer
• Develop NCI-recommended guidelines for clinical and molecular data collection as part of standard of care
• Create a comprehensive, harmonized, and integrated database of clinical, genomic, and phenomic data for research
• RFI this month to define an initial pilot

**Clinical Service:** Diagnostic clinical molecular characterization services for patients who might not otherwise have access to them

**Data to be collected (CLIA certified)**
- DNA: CLIA WES or NGS targeted panel
- RNA: CLIA RNA-seq
- Methylation: CLIA DNA Methylation array
- Clinical annotation

**Research Discovery:** Molecular characterization to learn more about disease subtypes and rare cancers

**Data to be collected** (in additional to clinical/seq data on selected populations)
- WGS/deep molecular (DNA) profiling
- Longitudinal data
CDDI Childhood Cancer Data Platform

- Designed to federate data from multiple children’s cancer institutions and community-based and NCI-supported childhood/AYA data resources, featuring:
  - Patient-level data from all available sources
  - Easy access to data to enable deep analytics
  - Supports interoperability among existing data resources and with tools and other resources for use by researchers
  - Provide a central portal to find and analyze childhood/AYA cancer data

*Working group chaired by NCI and an extramural expert*
CCDI Coordination Center

- Develop guidelines and approaches to address cross-cutting needs and implement activities that align CCDI priorities, such as:
  - Develop common data elements (CDE)
  - Data harmonization
  - Systems interoperability
  - Data utility
  - Integrate CCDI-wide biobanking in coordination with the STAR Act
  - Consent and assent
  - Obtain scientific input from extramural communities
  - Data governance system to ensure long-term sustainability and function

*Working group chaired by NCI and an extramural expert*
CCDI Governance Structure

CCDI Steering Committee

National Childhood Cancer Cohort

Coordination Center

Childhood Molecular Characterization Protocol

Childhood Cancer Data Platform

CCDI Engagement Committee

4 working groups co-chaired by NCI and extramural experts and made up of NCI staff, external experts and advocates
CCDI Steering Committee

**Membership:** Working group chairs, advocates, and NCI CCDI leadership

**Goal:** To address high-level, strategic and cross-cutting issues that will inform all CCDI activities and priorities

**Topics to address:**
- Strategic direction
- Scientific Priorities
- Identifying gaps in childhood cancer data and infrastructure that can be filled by CCDI
CCDI Engagement Committee

**Membership:** NCI staff, advocates, and extramural researchers

**Goal:** To engage the broader childhood cancer community in CCDI, in order to meet the data, education, and analytic needs of researchers, care providers, advocates, and patients and their families.

**Topics to address:**
- Identifying issues of importance to advocates, caregivers, and patients, survivors, and their families
- Identify opportunities for engagement with researchers
- Extending the reach of CCDI to other initiatives
Questions?