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

NIH NATIONAL CANCER INSTITUTE

Warren Kibbe, Ph.D.
James Doroshow, M.D.

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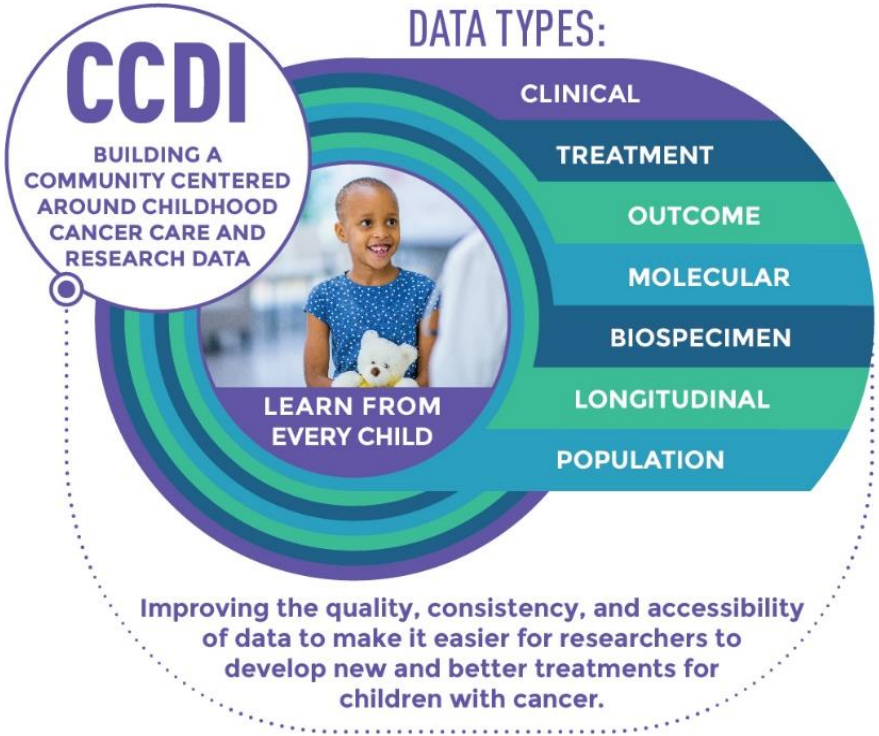
Outline of Presentation

- 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

24 specific recommendations

- 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

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

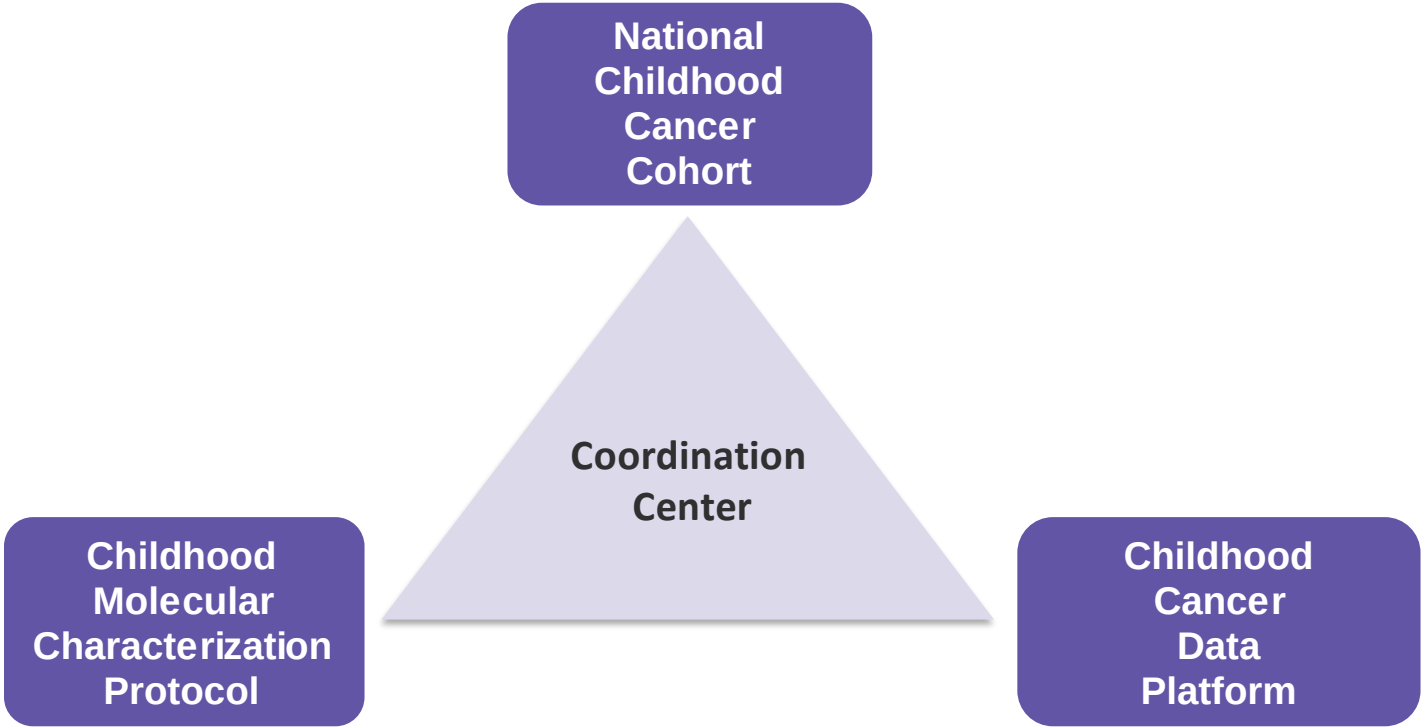
Vision for Years 2 - 10

Goals, program structure, and governance for future years

Foundational Goals for Years 2-10

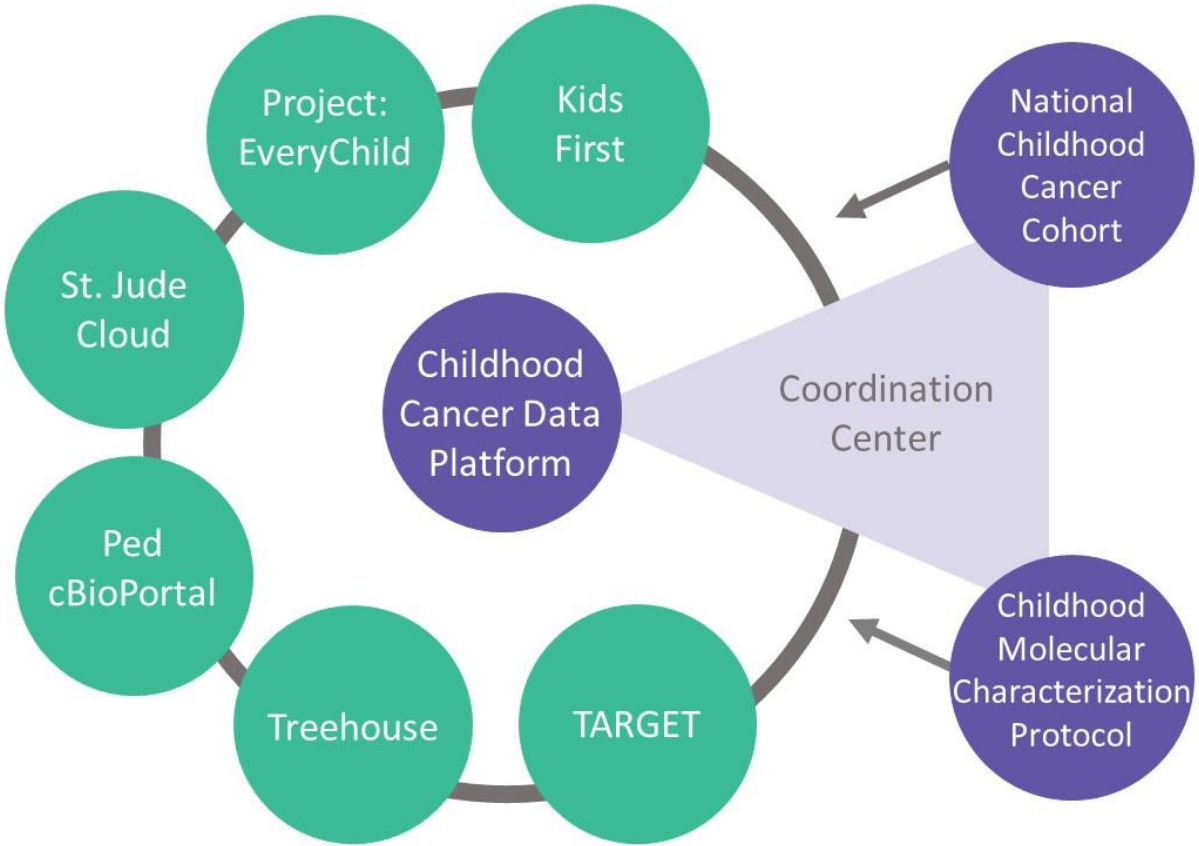
- Gather data from every child/AYA diagnosed with cancer, regardless of where they receive their care
- Develop core data from consented patients, including tumor and germline molecular characteristics, to enable research using patient-level data in a secure and de-identified way
- Create a system that 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



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

CCDI Childhood Molecular Characterization Protocol

- 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

CCDI 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

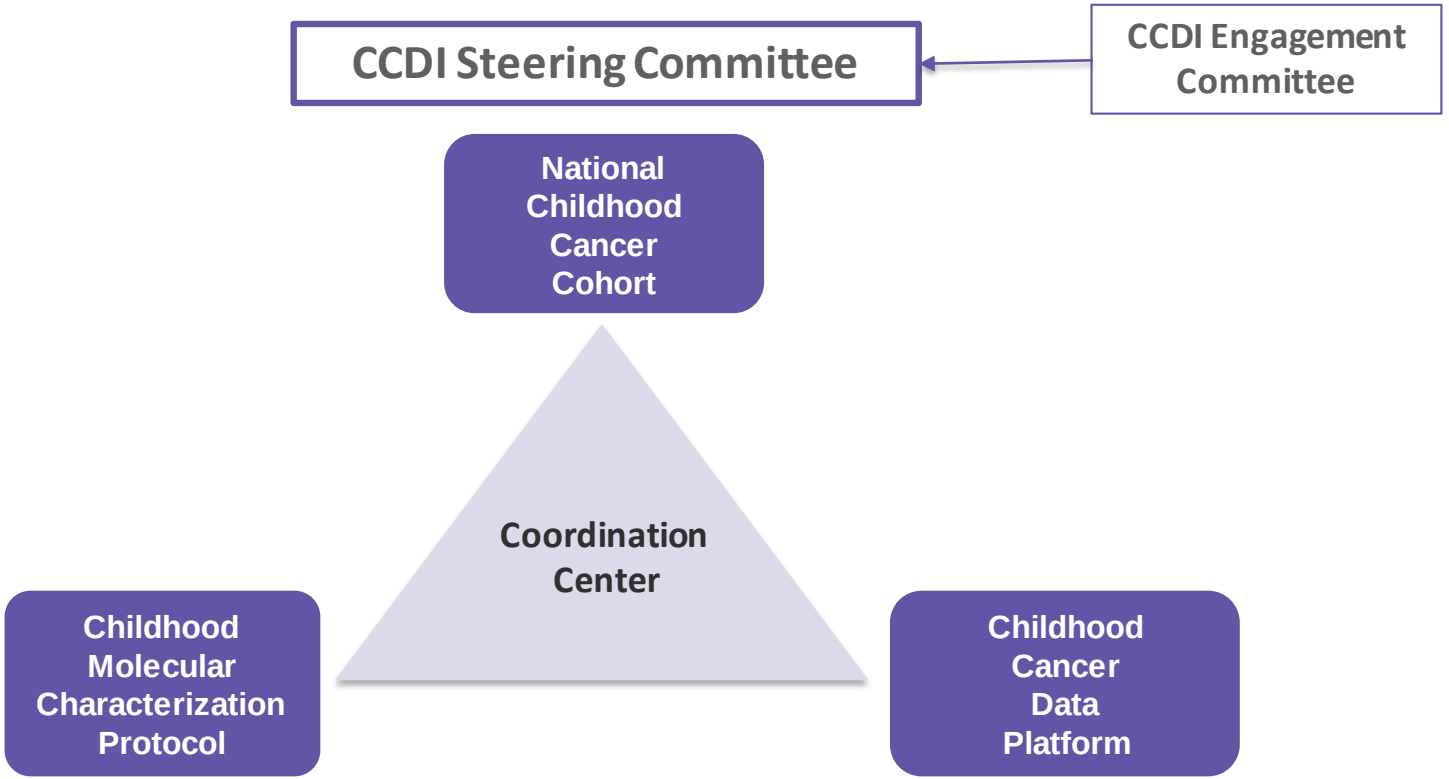
Working group chaired by NCI and an extramural expert

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



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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?

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