Collaborative Approaches to Accelerate Better Therapies for Patients with Rare Tumors

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on behalf of MyPART/CCDI/COG/Ultra-Rare Public-Private Partnership Collaborators

NCI MyPART My Pediatric and Adult Rare Tumor Network
A program of the National Cancer Institute of the National Institutes of Health

CANCER MOONSHOT

CHILDHOOD CANCER DATA INITIATIVE
Community-driven progress through data

CHILDREN'S ONCOLOGY GROUP

FDA

FNIH
Foundation for the National Institutes of Health

NIH NATIONAL CANCER INSTITUTE

September 6, 2023

Overview

1. Definition of rare tumors and challenges
2. Landscape of rare tumor efforts
3. MyPART
4. CCDI national rare tumor effort
   - International collaboration
5. Public-private partnership to enable drug development for ultra-rare tumors

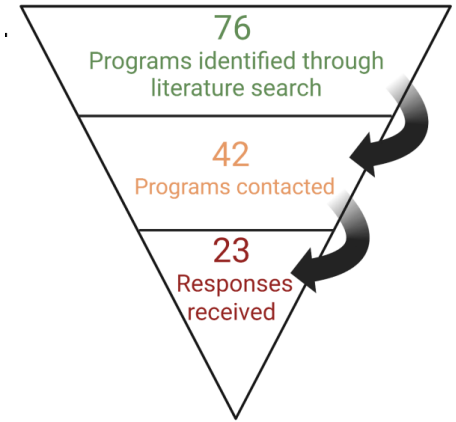
Rare Tumors Present Unique Challenges

- Rare cancer: <150 cases/million/year
  - 27% of cancer diagnoses and 25% of cancer deaths
  - All pediatric cancers, nervous system cancers, and sarcomas are rare
- Challenges for patients:
  - Long time to diagnosis
  - Limited experience at many medical centers
  - No “standard of care”
  - Limited social and advocacy support
- Challenges for researchers:
  - Long time accruing to clinical trials
  - Limited tools and diversity of models
  - Limited support from granting agencies
  - Limited financial incentives for industry to collaborate on drug development

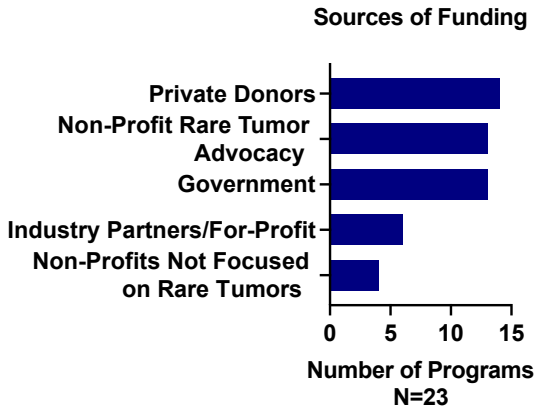
Landscape of Rare Tumor Research

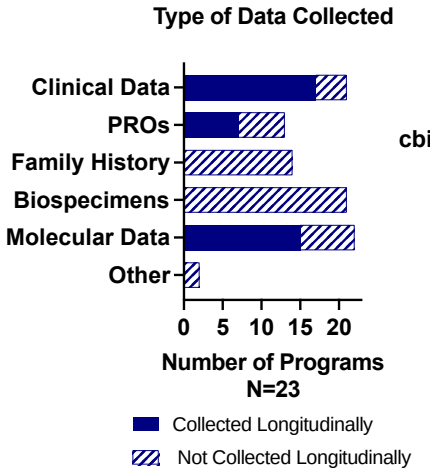
Landscape of Rare Tumor Research Programs: Funding and Data Sharing

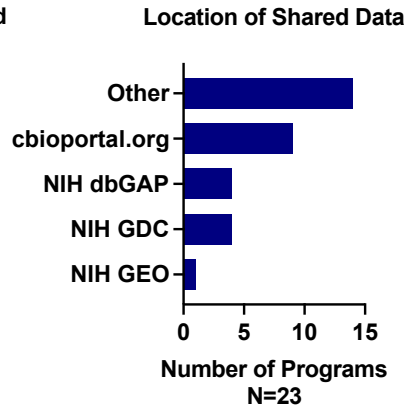
Survey of 42 Rare Tumor/Cancer Programs
Conducted **May – Nov 2021**
45 optional questions



Information Collected:
- Program Description
- Funding Mechanisms
- Patient Population
- Engagement/Outreach
- Biospecimen/Data Collection
- COVID-19 Impact







- Funded primarily by philanthropy, advocacy, and government
- Most data is not collected longitudinally
- No standardized data collection across programs*
- The rare tumor research community is inclined to share data, but more work is needed to make data **findable** and reduce burden of data sharing*

* Goal of Childhood Cancer Data Initiative est 2019

"The Landscape of US and Global Rare Tumor Research Programs: A Systematic Review" Vivelo et al, submitted

My Pediatric and Adult Rare Tumor Network (MyPART)

MyPART Mission

Mission: Increase patient and family involvement in rare tumor research to develop new therapies for rare pediatric and adult solid tumors through increased understanding of tumor biology and natural history

MyPART: My Pediatric and Adult Rare Tumor Network

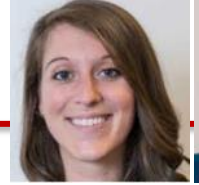
CANCER MOONSHOT

- Focusing on rare solid tumors affecting **children**, **teens**, and **young adults (≤39 yo)**
- Engaging patients, family members, advocates, clinicians, scientists, as **partners in research**
- Collecting longitudinal **molecular, clinical,** and **patient reported** outcome data through the **Natural History Study of Rare Solid Tumors** (NCT03739827)
- Holding **workshops and symposia** on rare tumors to develop expert consensus
- Hosting **multi-day clinics** for rare tumors to bring patients and nationwide experts together
- Building a **multi-institutional network** of sites to collaborate on data collection

Clinical Research
Key Accomplishments

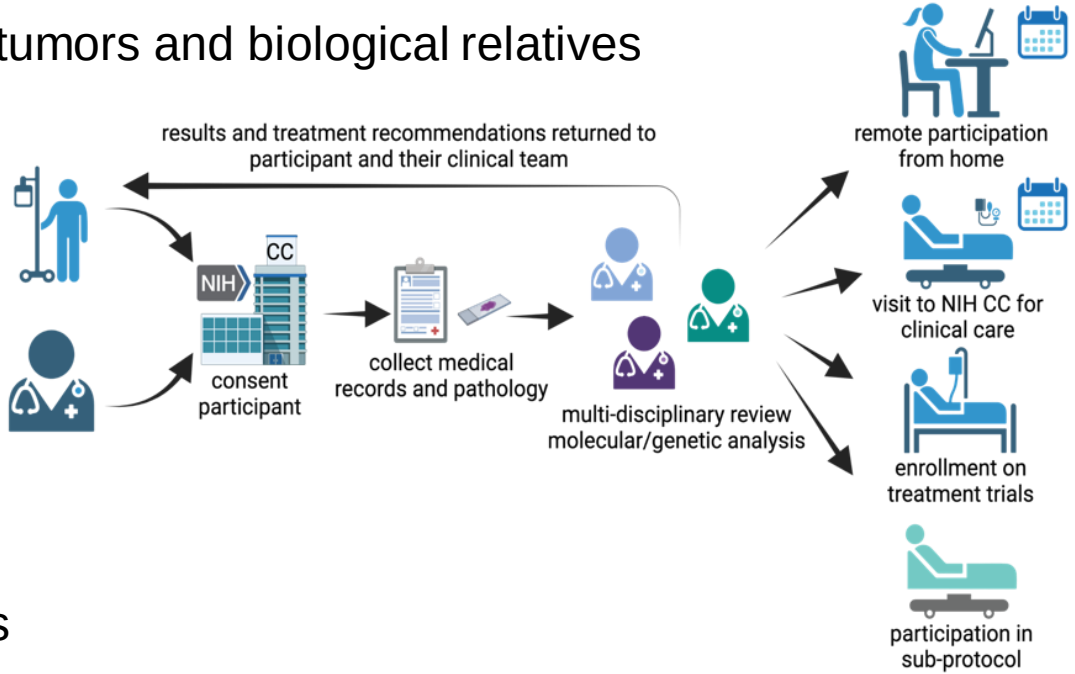
- Development of Natural History Study and tumor-specific subprotocols
- Remote enrollment during COVID pandemic
- Analysis of tumor and blood/saliva biospecimens
- Expansion of Rare Tumor Specialty Clinics at NIH
- Interventional trials for children and adults with rare tumors with NCI Developmental Therapeutics Clinic
- Clinical and biospecimen data submitted to dbGAP
  - ✓ 10662 data fields on first 500 participants
  - ✓ 57245 data fields of first 200 participants
  - ✓ Variant calls for 193 tumors
  - ✓ 2370 biospecimen annotation fields for 193 tumors
  - ✓ Public release under controlled access est Q4 2023
    (Ahmed et al Cancer Research Comm, accepted)

Natural History Study of Rare Solid Tumors (NCT03739827)


Mary Frances Wedekind Malone


Jaydira Del Rivero

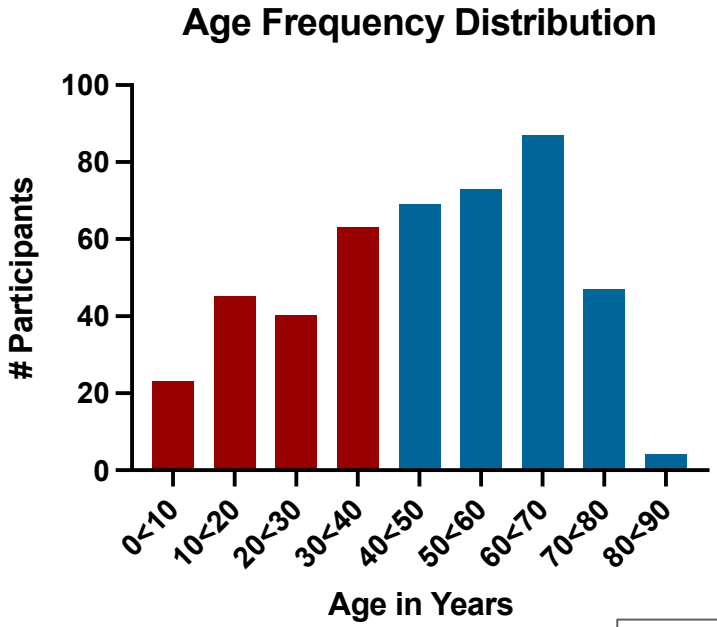
- Standardized longitudinal evaluation: Retrospective and prospective
  - Medical and family history, patient reported outcomes, clinical evaluation
  - Extensive medical record data extraction
- Children and adults with rare solid tumors and biological relatives
  - Off or on site participation
  - Treatment recommendations
- Comprehensive molecular profiling
  - Tumor tissue, blood, saliva
- Molecular tumor board
- Genetic counseling
- Annotated biospecimen repository
- Development of interventional trials

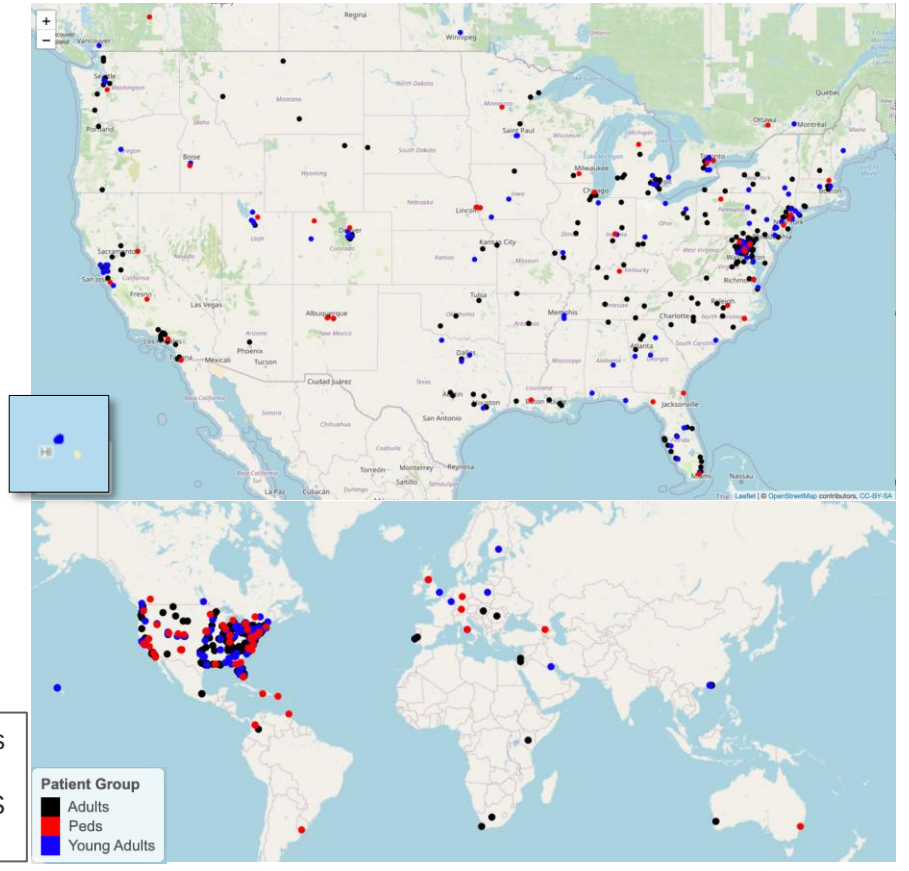


Wedekind et al 2023 Pediatric Blood and Cancer

Natural History Study Enrollment

- Protocol started enrolling January 28, 2019
- As of Sept 1, 2023, **571** participants have enrolled





- >68 different histologies
- 6 continents
- 27 countries outside US
- 46 US states, DC, PR

Data as of 9/1/23

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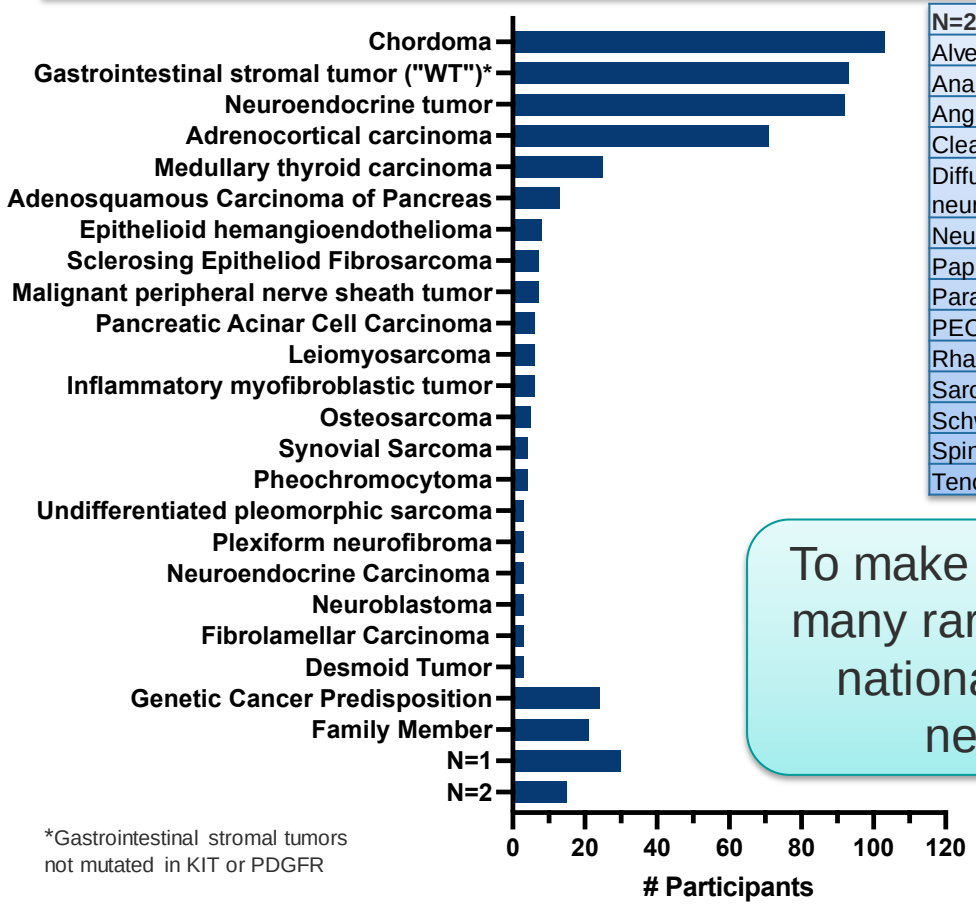
Natural History Study Enrollment



*Gastrointestinal stromal tumors not mutated in KIT or PDGFR

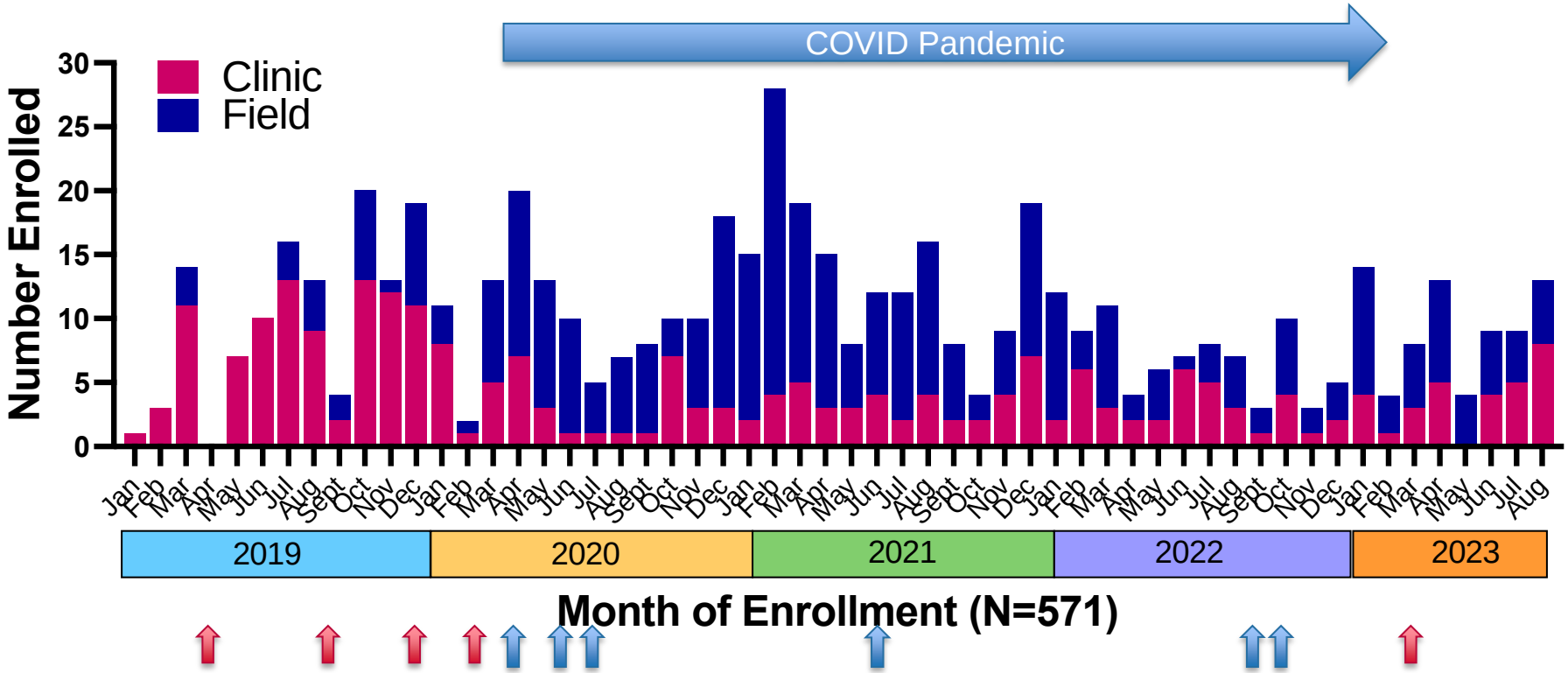
N=2 Participants
Alveolar soft part sarcoma
Anaplastic thyroid cancer
Angiosarcoma
Clear cell sarcoma
Diffuse idiopathic pulmonary neuroendocrine cell hyperplasia
Neurofibroma
Papillary thyroid carcinoma
Parathyroid carcinoma
PEComa
Rhabdomyosarcoma
Sarcoma
Schwannoma
Spindle cell carcoma
Tenosynovial giant cell tumor

N=1 Participant
Acute myelogenous leukemia
Adamantinoma-like EWS
Atypical myofibroblastic neoplasm
Carcinoma of unknown primary
Cervical cancer
Colon adenocarcinoma
Epitheliod MPNST
Ewing Sarcoma, cutaneous
Ganglioneuroblastoma
Paraganglioma
Glomangiosarcoma
Heptatoblastoma
Hereditary Leiomyomatosis, Renal cell carcinoma
Malignant spindle cell neoplasm
Malignant triton tumor
Mesenchymal chondrosarcoma
Mixed adeno-neuroendocrine carcinoma
Mucinous adenocarcinoma of the appendix
Mucinous cystoadenocarcinoma
Myoepithelial carcinoma
Myxofibrosarcoma
NUT carcinoma
NUTM1 rearranged sarcoma
Optic glioma
Pancreatic cancer, NOS
Pancreatoblastoma
Mucoepidermoid carcinoma
Sarcomatoid squamous cell carcinoma
Spindle cell fibrous tumor

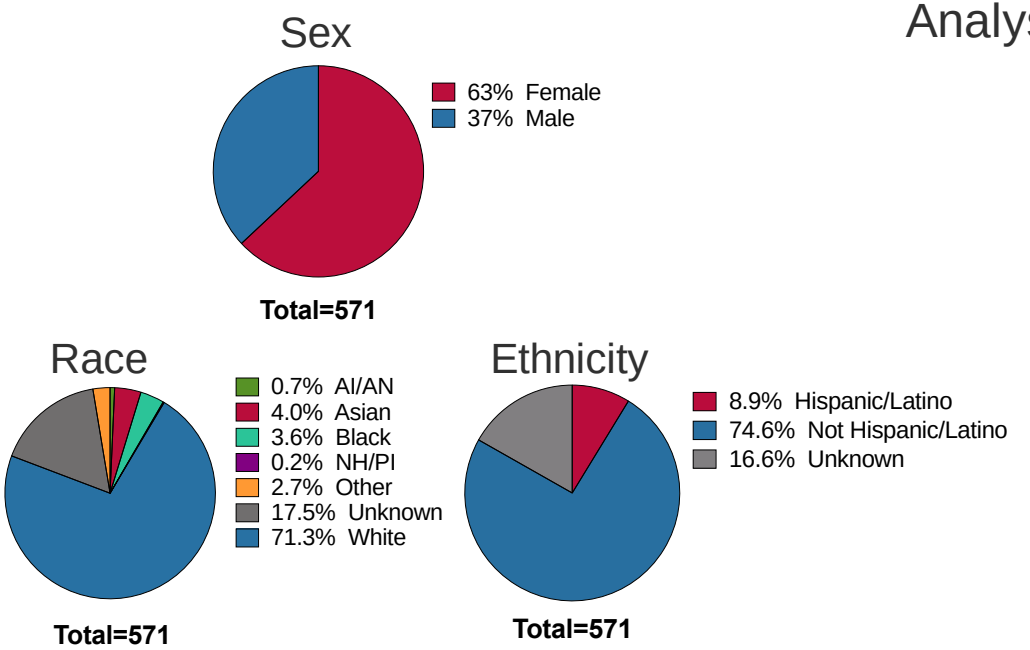
To make progress in many rare tumors, a national effort is needed

Data as of 9/1/23

Effect of COVID Pandemic on Patient Enrollment



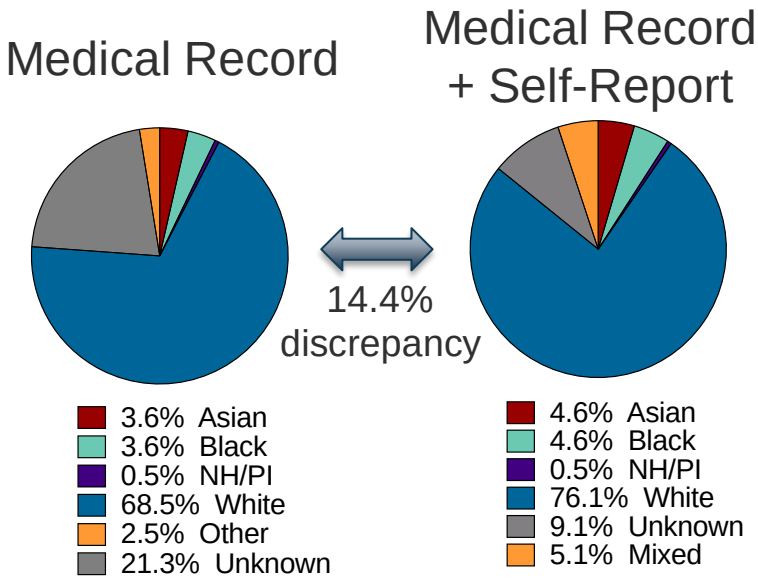
Sex, Race, Ethnicity



Enrollment as of Sept 1, 2023

- ✓ More females than males due to tumor types
- ✓ Working on improving outreach to underrepresented populations

Analysis of Race Data in First 200 Enrollees



- ✓ Including self-reported race improves assignment of race to participants
- ✓ 14.4% discrepancy between self-reported race and race in medical record

Ahmed et al Cancer Research Communications, in press 2023

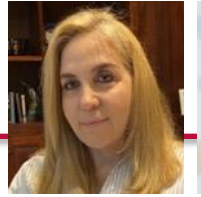
Patient Reported Outcomes (PROs) for Rare Tumors


Robin Lockridge

- MyPART uses 25 instruments to gather data
  - Adults, children, guardians
  - Anxiety, cognitive functioning, depression, fatigue, mobility, pain, peer relations, emotional support, upper extremity function
- Preliminary analysis demonstrates differences in PROs for particular tumor types (e.g. medullary thyroid cancer*)
  - Ongoing projects to analyze baseline PRO data for Chordoma, ACC, and NET
- Data has led to expanded tumor-specific PRO collection in sub-protocols

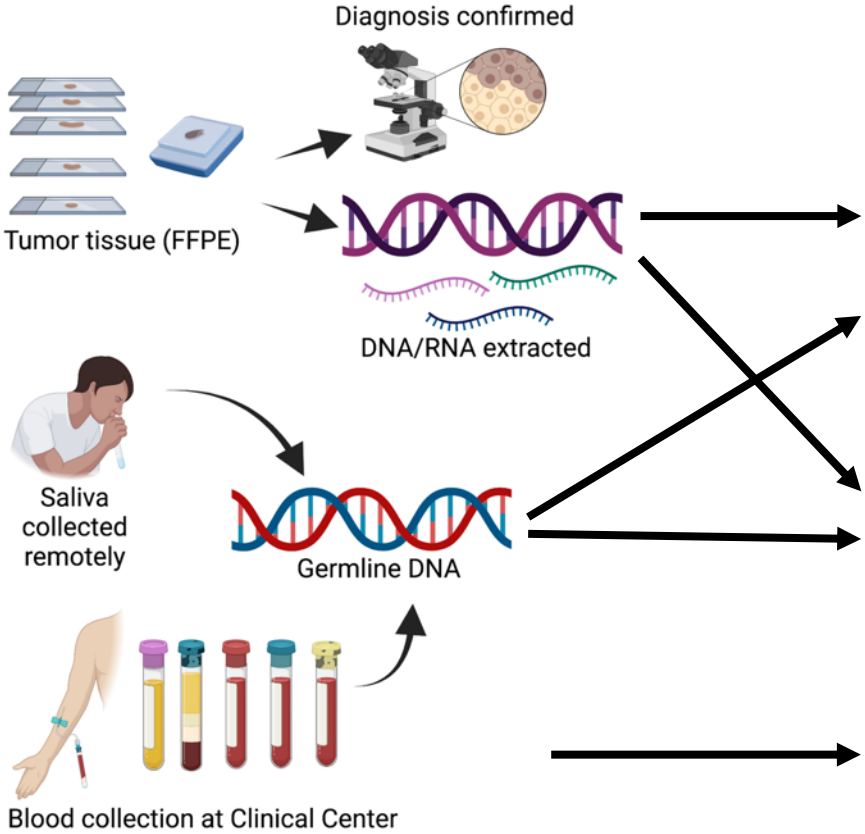
*Lockridge et al Children (Basel) 2022

Molecular Analysis of Biospecimens


Margarita Raygada


Ken Aldape
LP, CCR


Mark Raffeld
LP, CCR



Results returned to patients (CLIA):

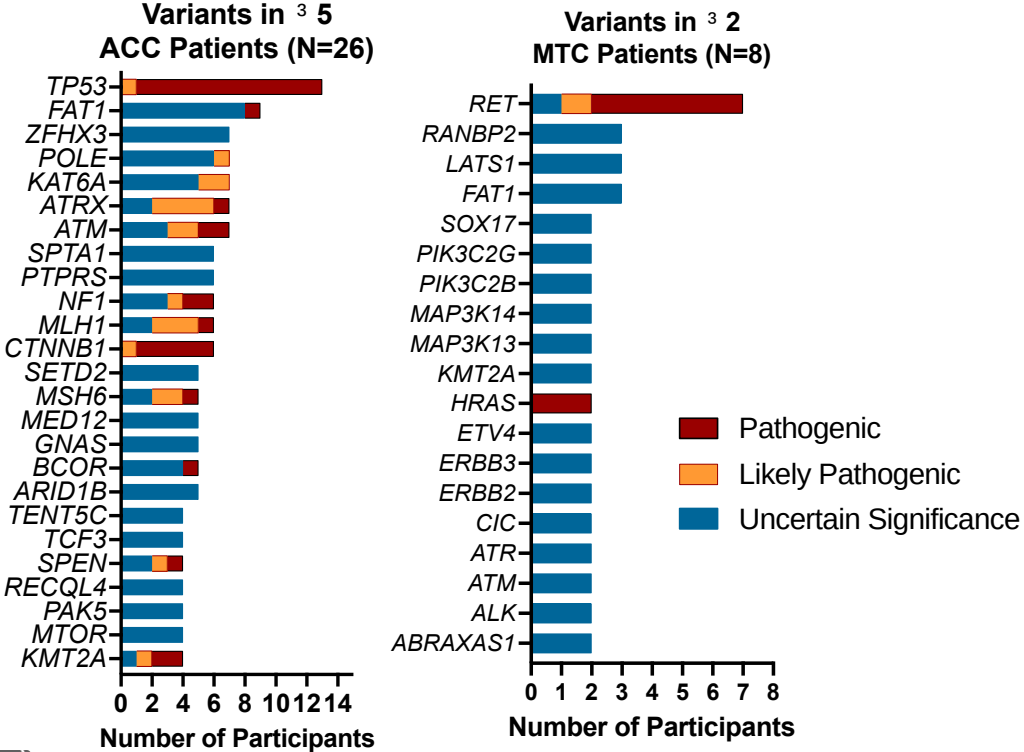
- Consensus diagnosis (Molecular Tumor Board)
- Actionable gene mutations (TSO500)
- Matched tumor-germline WES (FY21)
- Germline DNA sequencing (if clinically indicated)
- RNAseq (FY22)

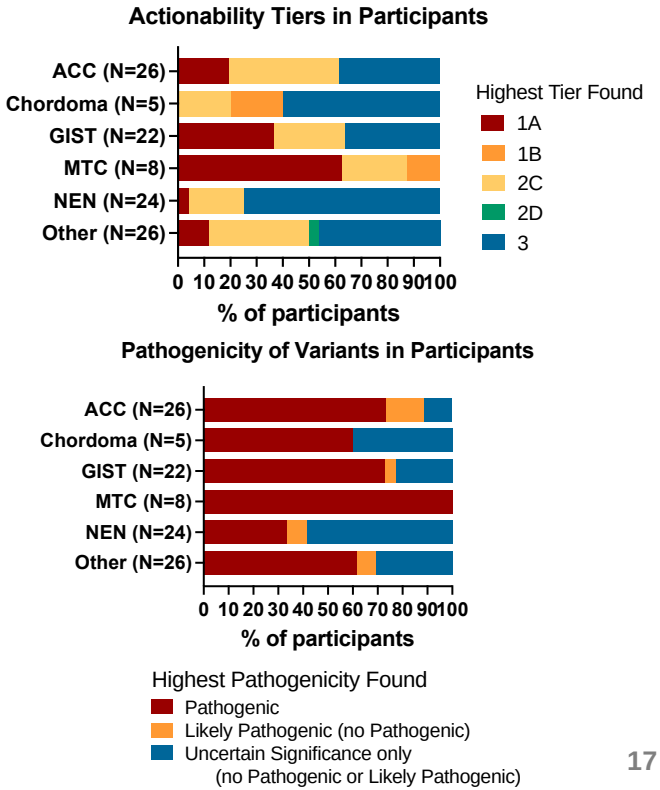
Data collected for research:

- Matched tumor-germline WES
- Shallow WGS
- DNA methylation arrays (EPIC)
- Matched tumor-germline WGS (pilot)
- Tumor RNAseq
- Blood RNAseq
- PBMC immune phenotypes
  - lymphoid and myeloid cells
  - activation and maturation states
  - immune checkpoint markers
- Cytokine levels in plasma

Analysis of Tumor Biospecimens From First 200 Participants Using Trusight Oncology 500 Gene Panel Sequencing

- Out of ~500 genes tested, expected pathogenic mutations were identified (e.g. ACC and MTC)
- Proportion of pathogenic and actionable mutations varied by tumor





Ahmed et al, Cancer Research Communications, in press 2023

New Rare Tumor Clinics Using the wt-GIST Clinic Model

CHORDOMA FOUNDATION

- Clinics bring 10-15 patients with select very rare tumors to the NIH CC
  - Disease experts (intra- and extramural) and advocates
  - Detailed clinical and biospecimen evaluations
  - Patient reported outcomes, focus groups
  - Patients meet with each other and with experts
  - Communication of expert opinion
- Established new rare tumor clinics
  - WT-GIST; planning Sept 2023 clinic
  - Medullary Thyroid Carcinoma
  - Chordoma; 4th yearly clinic held May 2023
- Remote and in-person participation
- Planning new clinics

Inaugural pediatric chordoma clinic

John et al 2023 Pediatric Blood and Cancer

Pediatric Chordoma: Paradigm for Very Rare Tumor Research

Interest within IRP

CCR/DCEG Chordoma Working Group 27 members

Workshop of national leaders in research and advocacy

June 15, 2018

Formalized partnership with Chordoma Foundation

NCI MyPART My Pediatric and Adult Rare Tumors Network

A program of the National Cancer Institute of the National Institutes of Health

Natural history study Chordoma subprotocol

First pediatric chordoma clinic at the NIH Clinical Center

April 16-18, 2019

Multi-Institution virtual tumor boards

Pediatric chordoma working group

45 extramural members

July 2020 to present

Grant applications and new treatment trials for chordoma patients

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Phase II trial of Tiragolumab + Atezolizumab

Expression of PD-L1 and TIGIT in SMARCB1 deficient tumors

- Tiragolumab: Monoclonal antibody against TIGIT
- Atezolizumab: Monoclonal antibody against PD-L1

Eligibility:

- SMARCB1 or SMARCA4 deficient tumors
- Ages ≥ 12 months
- **Cohorts:**
  - Poorly differentiated chordoma
  - Renal medullary carcinoma
  - Malignant rhabdoid tumor (extra-CNS)
  - Atypical teratoid rhabdoid tumor (CNS)
  - Epithelioid sarcoma
  - Other SMARCB1 or SMARCA4 deficient tumors

NCI-CTEP: Multi-site, PEP-CTN coordinated

- Collaboration with NCI DTC, UOB, POB
- Correlative studies supported by MyPART

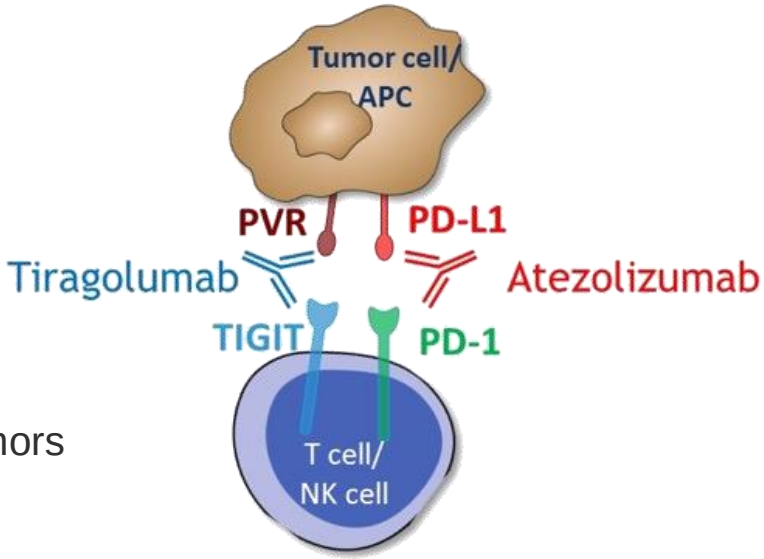
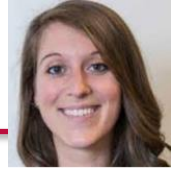


Figure adapted from Manieri et al.
Trends Immunology 2017


Mary Frances Wedekind Malone


Srivandana Akshintala


Alice Chen, DTC


James Doroshow, DTC

Preclinical Research
Key Accomplishments

- Development of new PDX and organoid models of rare tumors
- Generation of *Sdhb*-deficient and *BRaf*^{V600E} mouse models of “wt” GIST

Preclinical Models


Francesco Tomassoni Ardori


John Glod


Lino Tessarollo MCGP


Arnulfo Mendoza


Carol Thiele

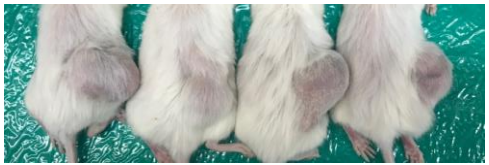

Rosa Nguyen


Jaydira Del Rivero

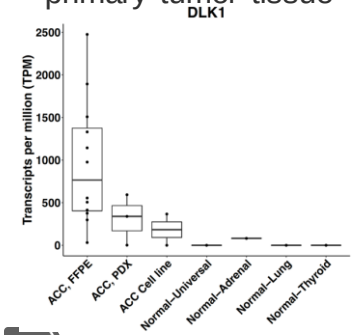
Orthotopic Patient-Derived Xenografts (PDX):

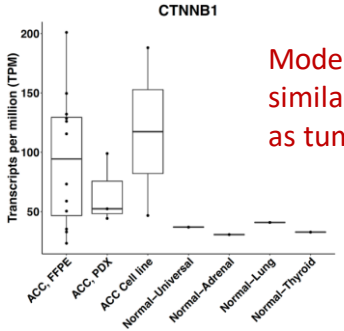
- Medullary thyroid carcinoma (1 patient)
- Anaplastic thyroid carcinoma (1 patient)
- Adrenocortical carcinoma (5 patients)
- SDH-def Gastrointestinal stromal tumor (2 patients)
- Chordoma (1 patient)
- Synovial sarcoma (1 patient)
- Rhabdoid tumor (1 patient)

Orthotopic metastatic ACC PDX-3 (P2)



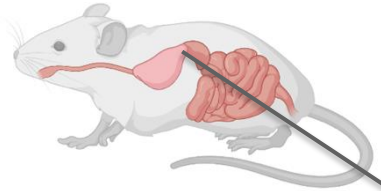
- DNA and RNA from models are being sequenced with primary tumor tissue

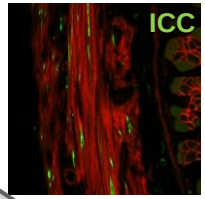




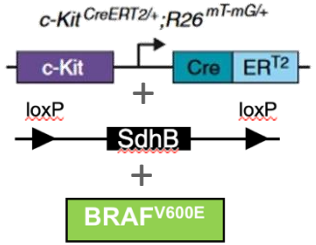
Models overexpress similar ACC markers as tumors

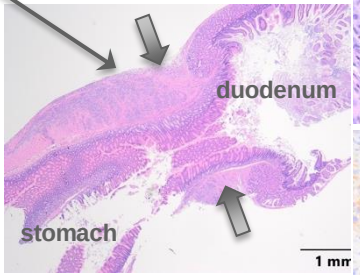
Genetically engineered mouse model of "wild-type" GIST

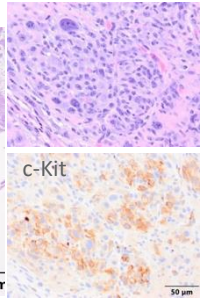




Sdhb is knocked out and *Braf*^{V600E} is activated in interstitial cells of Cajal in 21 day old neonates





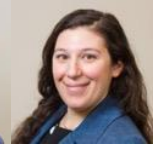


Engagement
Key Accomplishments

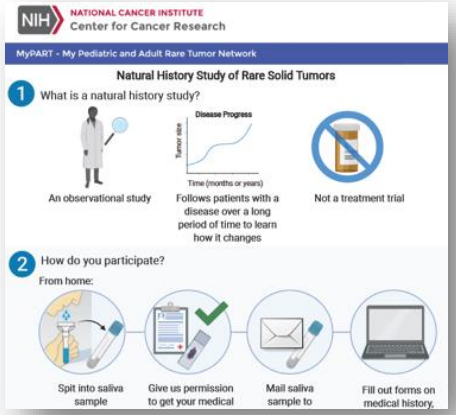
- Multiple communications platforms maintained
  - ✓ Website, Newsletter, Twitter
  - ✓ Multimedia
- Advocacy partnerships/outreach:
  - Advocacy network of 29 partners
  - Targeted 1-on-1 outreach

Advocacy Engagement and Communications


Abby Sandler


Christina Vivelo

- Established 29 advocacy partnerships
- Website with information and support resources for patients and caregivers
  - English and Spanish version
  - Approximately 70,000 unique visitors per month
- The MyPART Minute (Newsletter): 13,000 subscribers
- POB Twitter (@NCI_CCR_PedOnc): 1,700 followers



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The Life Raft Group
Cure Alveolar Soft Part Sarcoma International (iCureASPS)
FCF Fibrolamellar Cancer Foundation
SARC
WHO CARES TEEN CANCER AMERICA
RUCKER COLLIER FOUNDATION
MiB AGENTS
cure MEC the myoepithelial carcinoma project
LeiomyoSarcoma
LMS
SDH-RA
NORD RARE CANCER COALITION
CLEAR CELL SARCOMA FOUNDATION
stupid cancer
RCRF THE RARE CANCER RESEARCH FOUNDATION
CHORDOMA FOUNDATION
ACPMP APPENDIX CANCER PSEUDOMYXOMA PERITONEI RESEARCH FOUNDATION
Sarcoma Foundation of America SFA
ThyCa: Thyroid Cancer Survivors Association Inc. www.thyca.org
ECAN Esophageal Cancer Action Network
TargetCancer Foundation
ANGIOSARCOMA AWARENESS, INC.
JOEY'S WINGS
THE KEME FOUNDATION
THE DESMOID TUMOR RESEARCH FOUNDATION
the paula takacs FOUNDATION for SARCOMA RESEARCH
LET'S CURE ACC
SUMMER'S WAY be strong be positive be better

MyPART Experience/Future

- **Advocacy** and clinical/scientific **rare tumor expertise** are critical
- **NIH Rare Tumor Clinics** provide insight one could not get through evaluation of single patients at multiple sites
- Building meaningful cohorts is **resource and time intensive**
- **Focus on select tumor types** is needed to accrue sufficient patient numbers
- **Partnership** with consortia / COG / community hospitals / advocacy and national experts will be critical to **accelerate** rare tumor progress

NCI CCDI vision for rare tumors

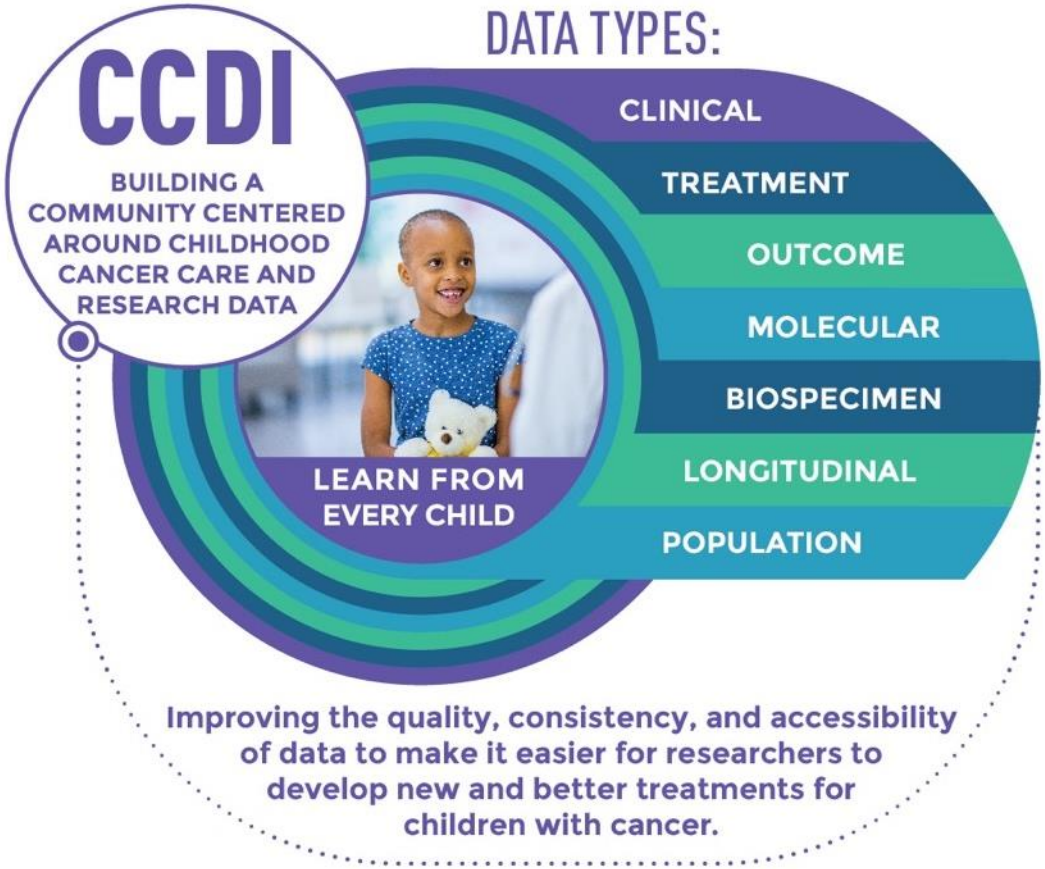
- Develop a collaborative national strategy for very rare pediatric and AYA cancers coordinated by the NCI CCDI

➢ Goal to efficiently study and characterize rare tumors and advance therapies

CCDI national rare tumor effort

NCI Childhood Cancer Data Initiative (CCDI)

Gregory Reaman



Learn from and use data

- EHR pilots
- Cohorts
- Survivorship
- Data catalog

Aggregate and generate data

- Preclinical models
- **Molecular characterization initiative**
- **National rare tumor initiative**

Build foundational infrastructure

- Data ecosystem
- CCDI participant index
- Computable consent
- Tools interoperability
- Federated infrastructure
- Clinical data commons

https://www.cancer.gov/research/areas/childhood/childhood-cancer-data-initiative

CCDI Molecular Characterization Initiative (MCI)

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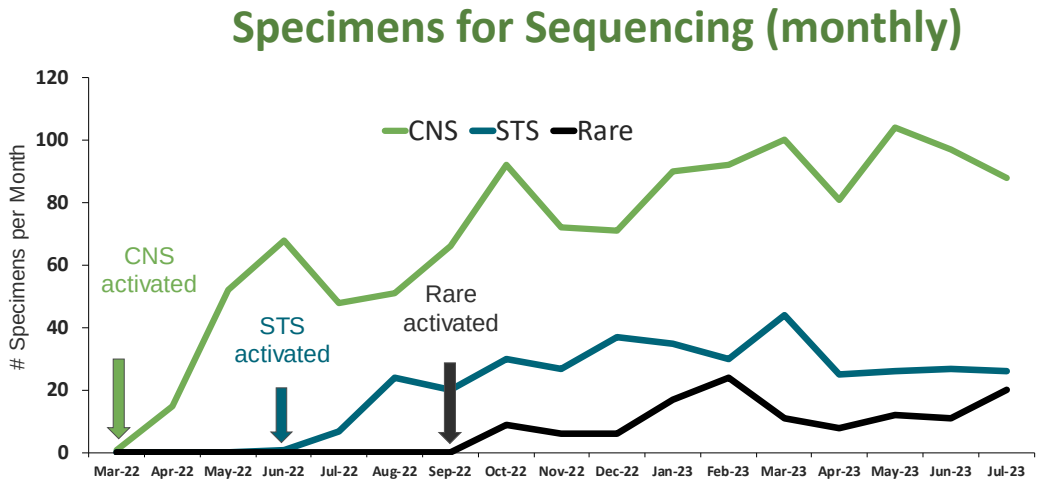
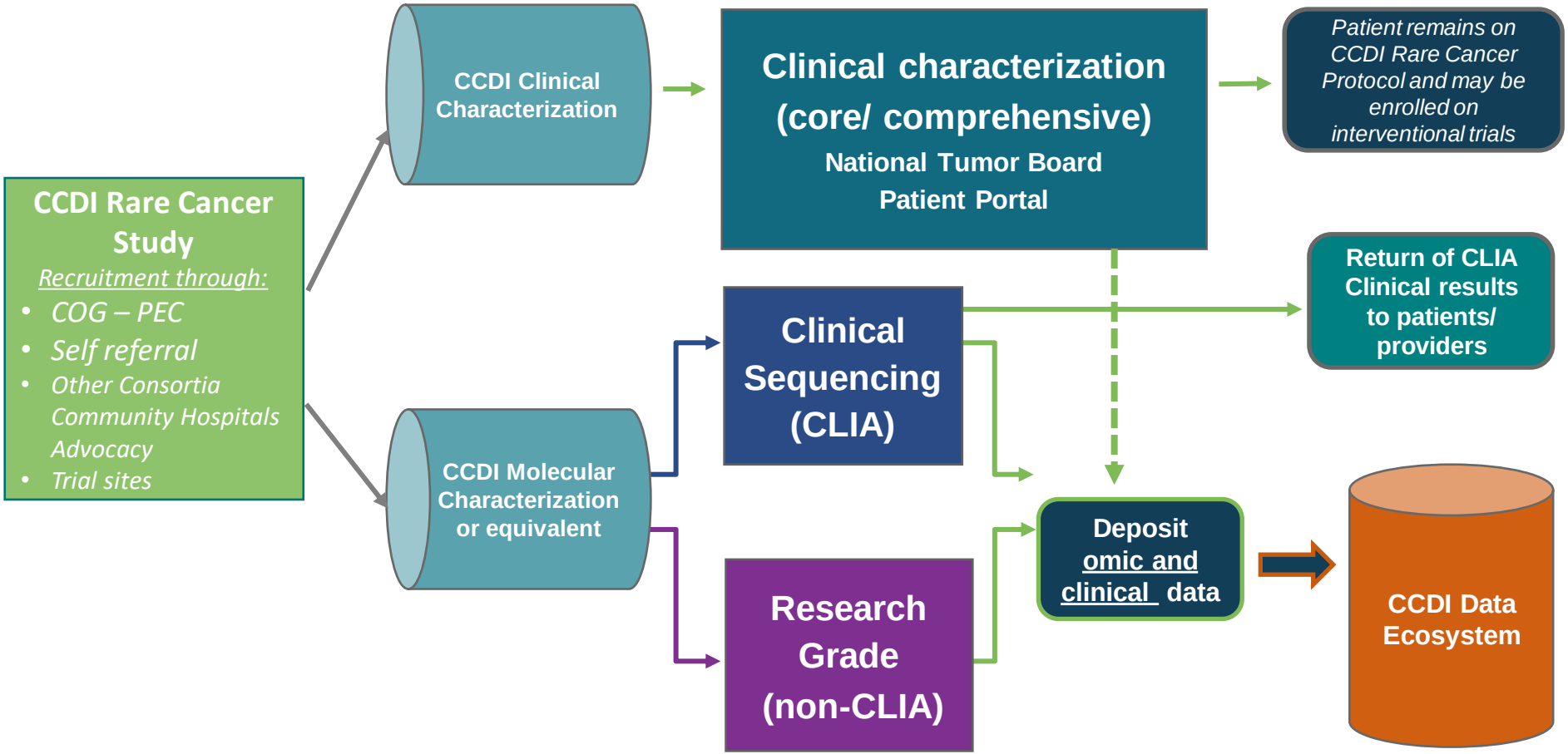
- Partnership between NCI and COG Project:EveryChild
- State-of-the-art molecular characterization at diagnosis (WES, fusions, methylation)
- Results returned to participants and treating physicians within 21 days
- Identification of molecular tumor subtypes
- In its first year, MCI enrolled more than 1,000 participants from 47 states, Canada, Australia, and New Zealand

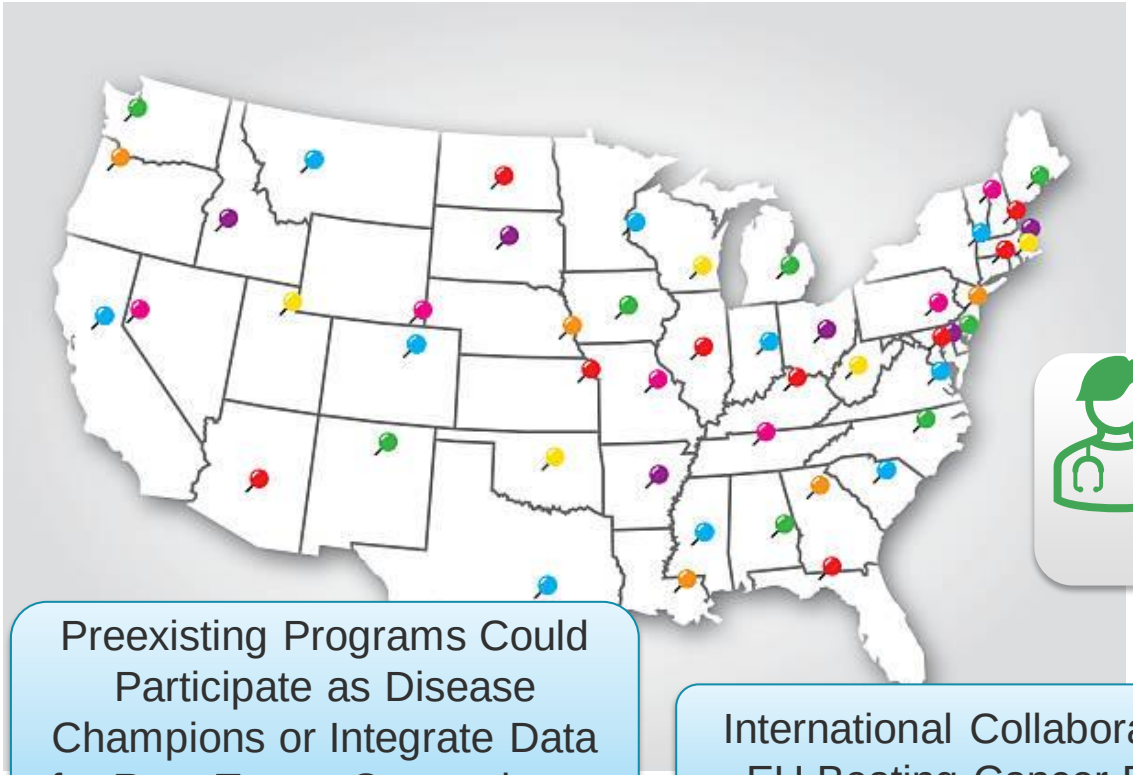
Enrollment as of Aug 2023 is 3035 patients



CCDI-Coordinated Rare Pediatric/AYA Cancer Study



Centralized Coordination with Distributed “Champions”

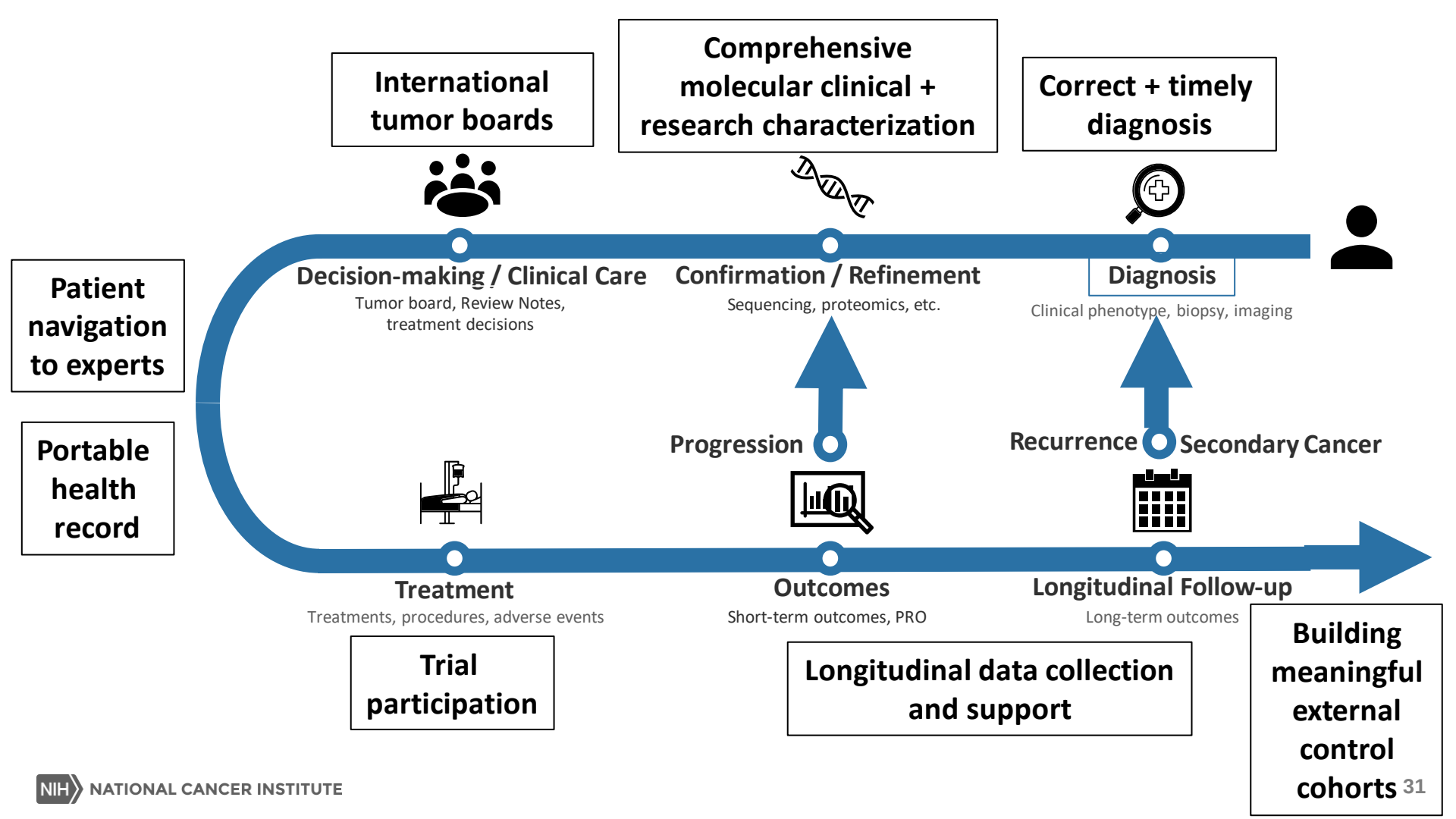


Disease Champion PIs
Advocacy/Research Partners
Disease-Specific Data Collection

Centralized Overall Study PI
Remote Enrollment
Core Data Collection

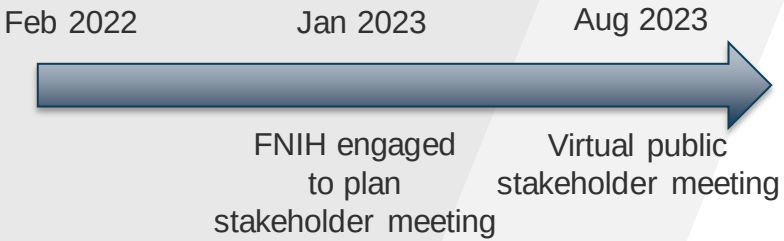
Preexisting Programs Could Participate as Disease Champions or Integrate Data for Rare Tumor Comparisons

International Collaboration: EU Beating Cancer Plan


International tumor boards
Comprehensive molecular clinical + research characterization
Correct + timely diagnosis
Patient navigation to experts
Portable health record
Decision-making / Clinical Care
Tumor board, Review Notes, treatment decisions
Confirmation / Refinement
Sequencing, proteomics, etc.
Diagnosis
Clinical phenotype, biopsy, imaging
Progression
Recurrence
Secondary Cancer
Treatment
Treatments, procedures, adverse events
Outcomes
Short-term outcomes, PRO
Longitudinal Follow-up
Long-term outcomes
Trial participation
Longitudinal data collection and support
Building meaningful external control cohorts

Proposal for a Public-Private Partnership to Develop Drugs for Ultra-Rare Cancers

Project Design and Planning Committee



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- Alice Chen, Head, Developmental Therapeutics Clinic, DCTD
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- Liz Ottinger, Acting Director, Therapeutic Development Branch
- Joni Rutter, Director, NCATS

FNIH:

- Stacey Adam, Associate Vice President, Science Partnerships - Translational Science
- Dana Connors, Director, Translational Science – Cancer
- Kat Lambertson, Project Manager, Translational Science

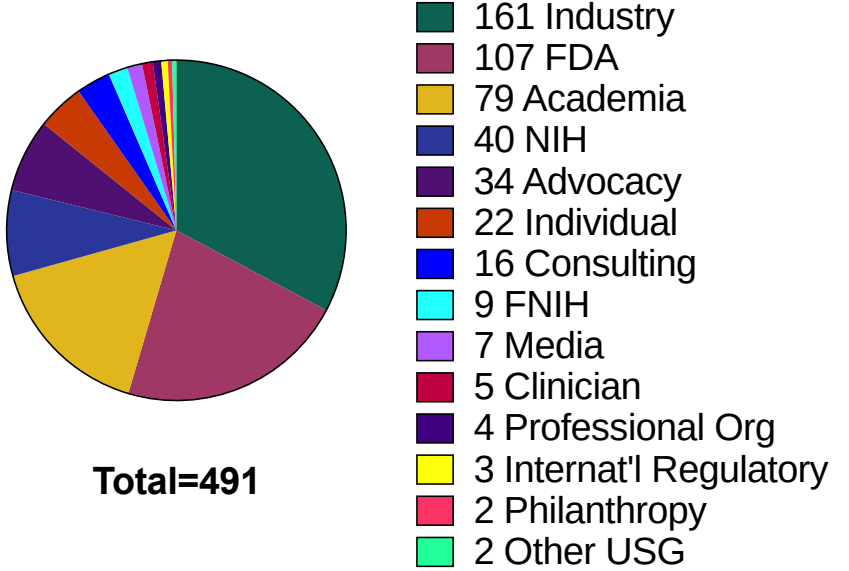
Project Design and Planning Committee

Feb 2022 — Jan 2023 — Aug 2023

FNIH engaged to plan stakeholder meeting

Virtual public stakeholder meeting

Registrants at Aug 2023 Stakeholder Meeting



Ultra-Rare Tumors

- The Connective Tissue Oncology Society (CTOS) defines “ultra-rare” sarcomas as those with an annual incidence of <1 in 1,000,000

  “entities whose rarity makes it extremely difficult to conduct well powered prospective clinical studies”

  -Stacchiotti et al 2021

- Orphanet ranks rare diseases according to 6 prevalence bins, with the rarest being <1 in 1,000,000 (European-based)
  - 1 in 1,000,000 is ~340 cases in the current US population
- Review of data from Orphanet and CTOS analysis suggests there are ≥ 222 ultra-rare tumors, of which ≥ 60 have characteristic molecular alterations
  - ~75,000 people affected by ultra-rare tumors each year
  - ≥ 29 ultra-rare tumors with fusions
  - ≥ 38 with disease-causing germline or somatic mutations
- ~43% of participants on the MyPART study have an ultra-rare tumor

Examples of Disease-Causing Gene Mutations in Ultra-Rare Tumors

APC	CSF3R	IDH1	NPM1	SMARCA4
ASXL1	CTNNB1	IDH2	PRKAR1A	TERT
ATP4A	DICER1	KIT	SDHB	TET2
CDC73	DNMT3A	MET	SDHC	TP53
CDKN2A	FLT3	NF1	SDHD	ZNRF3

Guiding Principles and Assumptions For Ultra-Rare Tumors

- Patients with ultra-rare tumors are as deserving of curative therapies as patients with common cancers
- Due to the high cost of drug development and very limited commercial market, drugs are unlikely to be economically sustainable under current supply-demand paradigms

CHORDOMA FOUNDATION Labs

- Many of the critical steps of drug discovery/development fall to advocacy organizations and academic researchers
- Existing, potentially effective drugs may be shelved or otherwise unavailable due to lack of use in more common cancers → lack of economic incentive to continue development

- Drivers of ultra-rare tumors exist, but have not been fully exploited because they are specific to ultra-rare tumors

NIH and FDA are well-poised to reduce hurdles in drug development for ultra-rare tumors by establishing public-private partnerships to incentivize drug development and clinical trials, with the aid of FNIH

Public-Private Partnership for Ultra-Rare Tumor Drug Development

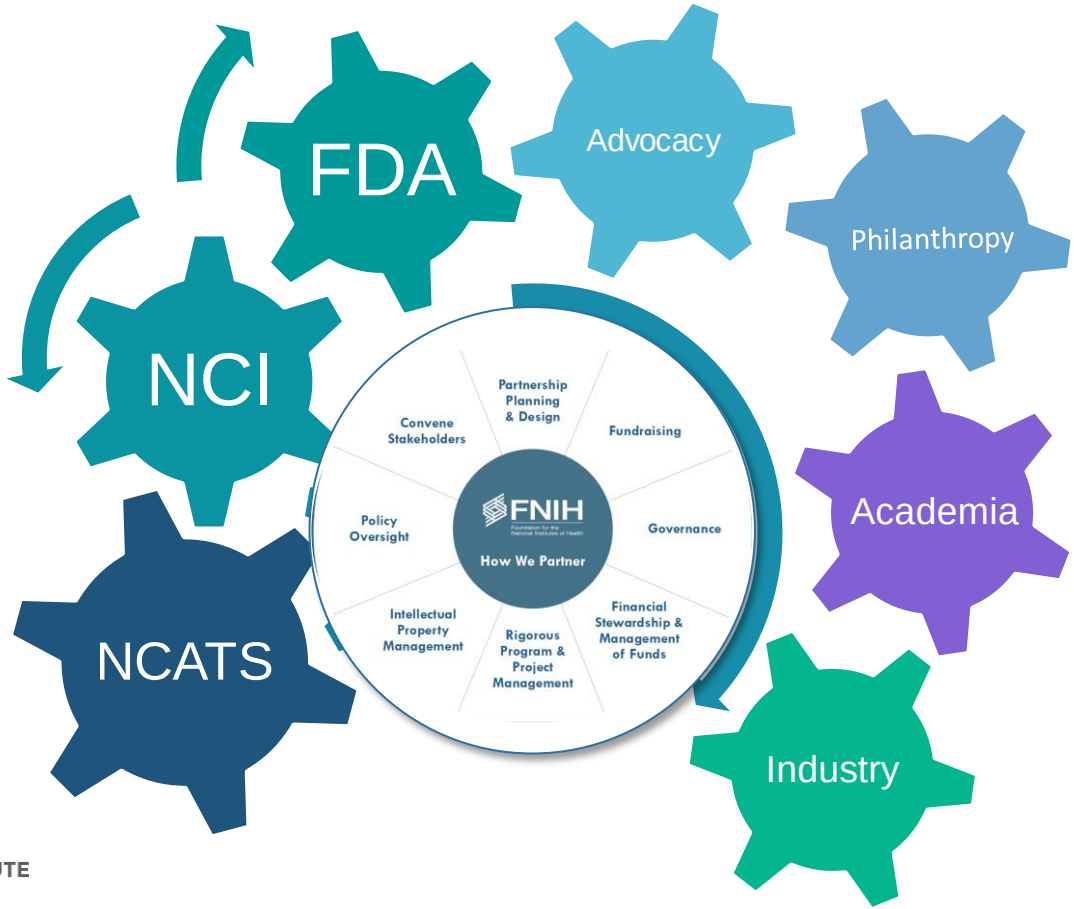
Aim

To harness **state-of-the art technologies** to target **well-established but previously undruggable biologic vulnerabilities** of ultra-rare cancers that **lack commercial incentive** for drug development through an **open science, multistakeholder public/private partnership**.

Objectives

- Explore in-depth the pathognomonic biology of select ultra-rare cancers to identify and characterize molecular vulnerabilities that confer potential druggable targets.
- Evaluate the feasibility and expediency of various drug development platforms to target the identified aberrant biology.
- Develop an open-science process across government, academia, and industry to leverage and coordinate resources in developing drugs for ultra-rare cancer indications.
- Coordinate and champion the development, from concept to clinical trial, of a drug targeting the aberrant biology of an ultra-rare cancer.

Proposed Structure of Public-Private Partnership



Cycle of Drug Development for Public-Private Partnership

Identify **1-2** ultra-rare tumors with known driver mutations

Workshop with disease experts and stakeholders to analyze tumor biology and determine promising approaches

✓Develop collaboration/IP agreements
✓Establish Scientific Advisory Board/Steering Committee
✓Build infrastructure for open notebook platform/data sharing

Finalize targeting modalities and research plan for drug development

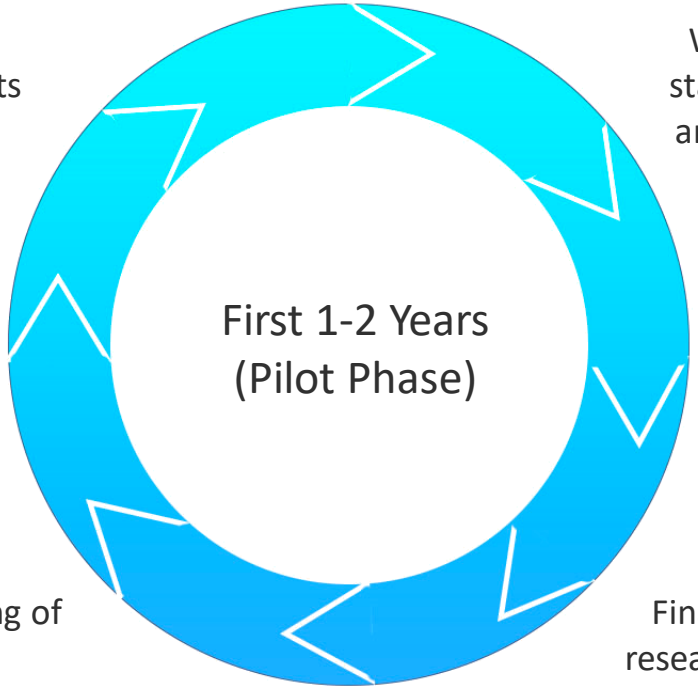
Compound identification and optimization

Depending on target and modality chosen

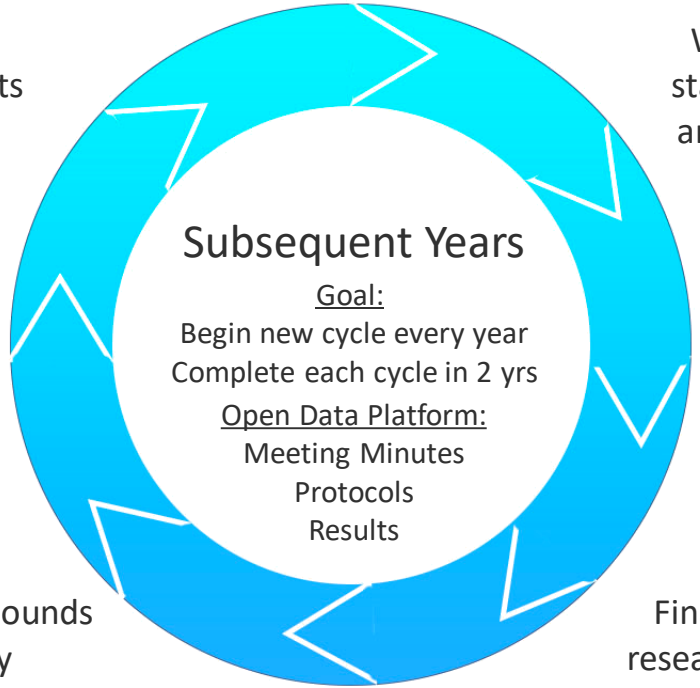
Preclinical testing of compounds
PK/PD and toxicology

Initiate clinical testing for regulatory approval

Develop sustainability plan to ensure supply of drug to patients
For-profit vs Not-for-profit

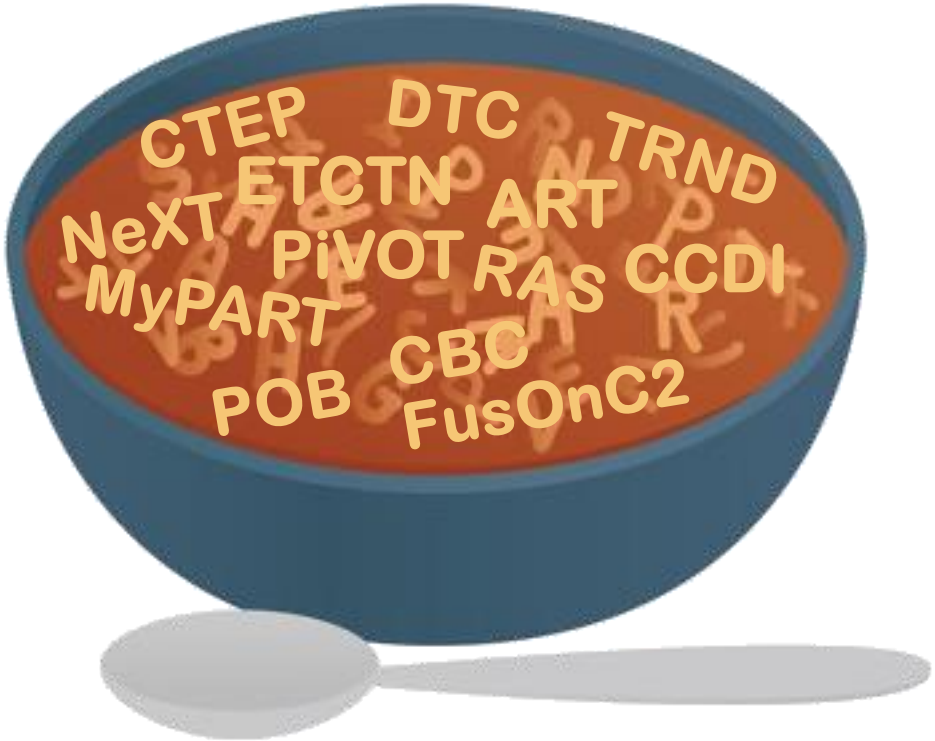


Cycle of Drug Development for Public-Private Partnership



Many NIH Programs Exist That Could Be Harnessed for Drug Development in Ultra-Rare Tumors

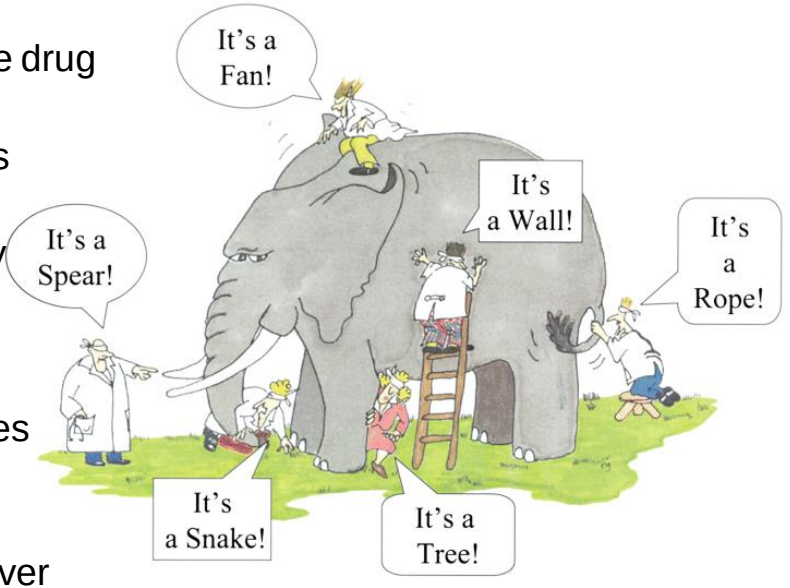
- Compound Identification and Molecular Optimization (SAR)
- Preclinical Optimization and Testing
- Natural History of Rare Tumors
- Molecular Characterization
- Early Phase Clinical Trials
- Clinical Trial Networks



Making sure we're all talking about the same thing!

What the proposed PPP is:

- Addressing **unmet** need in **ultra**-rare tumors (<1 per million or ≤300 cases in US)
- Focusing on **well-established, undrugged** vulnerabilities
- Establishing **collaboration and IP agreements** to promote drug approval and sustained supply for patients
- Developing a transparent, **open-science paradigm** across government, academia, and industry
- Bringing together **champions** for ultra-rare tumors, biology mechanisms, and technology to rapidly develop drugs



What the proposed PPP is not:

- Competing with other efforts to develop rare tumor therapies
- Focusing on therapeutic approaches not directly related to characteristic molecular alterations
- Focusing on ultra-rare tumors without a clear molecular driver
- Trying to cure all ultra-rare tumors at once!

Elements for Success

- Strong governance empowered to make decisions quickly
- Focus on a small number of ultra-rare tumors at a time
- Infrastructure to move through projects quickly
- Teams of “champions” for each project
  - Disease
  - Biological mechanism
  - Technology/drug
- Robust collaboration/IP agreements
  - Preserve commercialization strategies
  - Take advantage of government march-in rights
- “Open notebook” strategies that facilitate drug development, while protecting trade secrets and intellectual property

Elements for Success (continued)

- Harnessing government and private sector resources to move quickly
  - Compound optimization
  - *In vitro* and *in vivo* testing
  - PK/PD
  - First in human early phase clinical trials
- Incentives (e.g. vouchers, Orphan Drug designation)
- Innovative strategies for compounds sharing in high-risk projects without jeopardizing main indications
- Developing sustainability strategies for continued drug development and availability

What Makes a “Ideal” Ultra-Rare Tumor to Pursue?

Prioritization:

- ✓ Low incidence (<1 in 1,000,000) and well-defined diagnostic criteria
- ✓ High mortality/morbidity with no effective therapies (unmet need)
- ✓ Untargeted, well-defined molecular drivers of tumorigenesis
- ✓ Well-characterized experimental model systems
- ✓ Committed advocates
- ✓ Clinician champions willing to lead early phase clinical trials
- ✓ Champions for targets and technological approaches willing to commit to rapid drug optimization

Value of Rare Tumor Research to All Cancer Patients

- Rare tumor researchers must collaborate to be successful
  - ➢ Impetus to develop creative solutions to IP and collaboration barriers
- Rare tumor research has informed many of the hallmarks of cancer

  - ✓ Retinoblastoma → cell cycle mechanisms (RB1)
  - ✓ Von Hippel Lindau → hypoxia (VHL/HIF1α)
  - ✓ Glioblastoma → cellular metabolism (IDH)
- Pathways to drug development and regulatory approval in rare tumors will be relevant to drug development in subtypes of common cancers

➢ This proposed public-private partnership will address an unmet need in ultra-rare tumors and develop new paradigms and incentives for drug development

Summary

- MyPART has made substantial progress in engaging researcher and advocacy groups for the study of rare tumors
- We have established a strong foundation for the collection and analysis of linked clinical and molecular data on rare tumors
- Our studies have resulted in the development of new, collaborative, pediatric-adult interventional trials
- MyPART is playing a key role in establishing national programs for rare tumor research, particularly in collaboration with CCDI, COG, and FDA

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- Joni Rutter
- Stacey Adam
- Dana Connors
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