Connect, Share, Discover:
How Democratizing Data is Changing Research

May 18, 2022
## Agenda

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Details</th>
<th>Speakers/Participants</th>
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<tbody>
<tr>
<td>9:00am CDT</td>
<td><strong>Welcome</strong></td>
<td>Caitlin Pike, Sam Volchenboum</td>
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<td></td>
<td><strong>2021-22 at the PCDC</strong></td>
<td>Kat Blumhardt, Jamie Flerlage, Ajay Major, Kathy Pritchard-Jones, Kirk Wyatt</td>
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<td></td>
<td><strong>This year’s highlights</strong></td>
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<td></td>
<td><strong>Scientific Advisory Committee</strong></td>
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<td><strong>A global approach</strong></td>
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<td><strong>New analytics tools</strong></td>
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<tr>
<td>10:00am CDT</td>
<td><strong>A user perspective</strong></td>
<td>Raj Venkatramani</td>
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<td>10:20am CDT</td>
<td><strong>Spotlight on GEARBOx</strong></td>
<td>Sam Volchenboum</td>
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<td>10:35am CDT</td>
<td><strong>What’s next</strong></td>
<td>Sam Volchenboum</td>
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<tr>
<td>11:05am CDT</td>
<td><strong>Q&amp;A session</strong></td>
<td>PCDC team &amp; guests</td>
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Introduction

Sam Volchenboum, MD, PhD
Third Annual PCDC Public Webinar
May 18, 2022
Funding PCDC projects in 2021-22

United States Department of the Interior

The William and Evelyn Fuchs Family Foundation

The William and Evelyn Fuchs Family Foundation

"So they may live"
Connect.

Connecting data means connecting a network of pediatric cancer researchers from all over the world.

Share.

Driven by a spirit of collaboration and consensus, we work to share interoperable cancer data across research groups, institutions, and borders.

Cure.

High-quality, accessible data drives discovery.
What do we mean by democratizing data?

Accessibility
Usability
Diversity
Responsibility
Impact
Our approach to data

- **Accessibility**: lifting barriers to research
- **Usability**: high-quality, interoperable data
- **Diversity**: data that better represent the real world
- **Responsibility**: making it easy to do the right thing
- **Impact**: using data to its maximum potential
2021-22 overview

Kat Blumhardt
Ten consortia for different cancer types

The Pediatric Cancer Data Commons

- acute lymphocytic leukemia
  - INTERACT
- acute myeloid leukemia
  - NODAL
- bone tumors (OS and EWS)
  - HIBiSCus
- central nervous system tumors
  - INSPIRE
- Hodgkin lymphoma
  - INRG
- neuroblastoma
  - C3P
- predisposition
  - Global REACH
- retinoblastoma
  - INSTruct
- germ cell tumors
  - MaGIC
- soft-tissue sarcoma
  - C3P
- bone tumors (OS and EWS)
  - Global REACH
- central nervous system tumors
  - INSTruct
- germ cell tumors
  - MaGIC

The Pediatric Cancer Data Commons
US Data Use Agreements
Master agreements - 6 (including COG)
15 addenda (individual projects)

Non-US. Data Use Agreements
Master agreements - 3 institutions
5 addenda (individual projects)

Worldwide Data Contributor Agreements
US. - 4 (+6 addenda, includes St. Jude & COG)
Non-US - 13

N. America
- CBTN
- COG
- DFCI
- IDIPGR
- NRG
- PNOC
- RBTC
- St. Jude

S. America
- SOBOPE
- EpSSG

Europe
- AIEOP
- CCLG
- CRCTU
- EEC
- EpSSG
- EuPAL
- GPOH
- GEIS
- ISG
- MRC
- NCRI
- SIOPE
- SIOPEN
- SSG
- SFCE
- UNICANCER

Asia
- JCCG
- SIOPEN
- EpSSG
- COG

Oceania
- COG
- EpSSG
Recent publications from consortia


Recent publications from the PCDC

Pediatric Cancer Data Commons: Federating and Democratizing Data for Childhood Cancer Research

*JCO Clinical Cancer Informatics*

We present our experience constructing the Pediatric Cancer Data Commons to highlight the significance of developing a rich and robust data ecosystem for pediatric oncology and to provide essential information to those creating resources in other disease areas.

Mapping Pediatric Oncology Clinical Trial Collaborative Groups on the Global Stage

*JCO Global Oncology*

We describe pediatric cancer clinical trial groups on the international stage, with the goal of identifying the structure and function of these consortia, as well as the clinical data sources they collect, to reveal opportunities for collaborative efforts within these regions.
Preliminary launches of new platforms

PCDC Data Portal launched with INSTRuCT data

GEARBOx in pilot phase
Data dictionaries integrated with NCI thesaurus

5 dictionaries integrated to date
2 completed in 2022

- ALL
- NBL
- NRSTS
- RMS
- CNS
- RB
- HL
- OS
- GCT
- AML
- EWS

2020 2021 2022

To begin 2022 Future

2022
2021-22 highlight:
Scientific Advisory Committee

Jamie Flerlage, MD, MS
Two PCDC advisory bodies

Scientific Advisory Committee

**Membership**
Consortium representatives and domain experts *with knowledge of the PCDC*

**Goal**
Provide strategic and operational guidance on PCDC efforts

**First meeting**
November 2021

External Advisory Board

**Membership**
Clinical, data science, and digital health experts *not affiliated with the PCDC*

**Goal**
Share experience and offer high-level strategic advice

**First meeting**
Planned for Autumn 2022

*Charters and nomination processes* developed with feedback from consortium representatives
Purpose

- To ensure that the PCDC has an **appropriate, sustainable and ethical approach** to linking data across diseases and data types (clinical, genomics, imaging, tissues, histology, etc.)
- To **build and refine the data commons** with clinical, genomic, imaging, tissue, histology, and other data while maintaining the independence of the disease consortia for scientific direction
- To include **representative key stakeholders** in advising the PCDC
- To maintain the **autonomy and scientific direction** of the clinical disease consortia
SAC founding membership

Representatives from **ten countries and four continents**, including:

- Leaders from each PCDC disease-specific consortium
- Leaders from international oncology cooperative groups
- Statisticians
- Ethicist
- Patient advocate
- Members at large, including early career investigators

41% US, 59% non-US representation
# SAC members

<table>
<thead>
<tr>
<th>Name</th>
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<th>Country</th>
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<tbody>
<tr>
<td>Todd Alonzo</td>
<td>Statistician</td>
<td>USA</td>
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<tr>
<td>Maja Beck-Popovic</td>
<td>Global REACH</td>
<td>Switzerland</td>
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<tr>
<td>Gianni Bisogno</td>
<td>INSTRuCT</td>
<td>Italy</td>
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<td>Susan L. Cohn</td>
<td>INRG</td>
<td>USA</td>
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<tr>
<td>Jamie Flerlage</td>
<td>NODAL</td>
<td>USA</td>
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<tr>
<td>Brenda Gailie</td>
<td>Global REACH</td>
<td>Canada</td>
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<td>Ajay Gupta</td>
<td>Member at Large</td>
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<tr>
<td>Darren Hargrave</td>
<td>INSPIRE</td>
<td>UK</td>
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<tr>
<td>Doug Hawkins</td>
<td>COG</td>
<td>USA</td>
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<td>Stefanie Hecker-Nolting</td>
<td>HIBiSCus</td>
<td>Germany</td>
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<td>Katie Janeway</td>
<td>HIBiSCus</td>
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<td>Pamela Kearns</td>
<td>SIOPE</td>
<td>UK</td>
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<td>Kara Kelly</td>
<td>NODAL</td>
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<td>Sarah Leary</td>
<td>INSPIRE</td>
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<tr>
<td>Marie-Cécile Le Deley</td>
<td>Statistician</td>
<td>France</td>
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<tr>
<td>Mignon Loh</td>
<td>ALL Consortium</td>
<td>USA</td>
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<tr>
<td>Akira Nakagawara</td>
<td>JCCG</td>
<td>Japan</td>
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<tr>
<td>Andy Pearson</td>
<td>Member at Large</td>
<td>UK</td>
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<td>Christopher Porter</td>
<td>C3P</td>
<td>USA</td>
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<td>Praful Ravi</td>
<td>MaGIC</td>
<td>USA</td>
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<tr>
<td>Dirk Reinhardt</td>
<td>INTERACT</td>
<td>Germany</td>
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<tr>
<td>Lainie Ross</td>
<td>Pediatrician/Ethicist</td>
<td>USA</td>
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<tr>
<td>Gudrun Schleiermacher</td>
<td>INRG</td>
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<td>Furqan Shaikh</td>
<td>MaGIC</td>
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<tr>
<td>Monika Sparber-Sauer</td>
<td>INSTRuCT</td>
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<tr>
<td>Daisuke Tomizawa</td>
<td>INTERACT</td>
<td>Japan</td>
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<tr>
<td>Liliana Vásquez</td>
<td>Member at Large</td>
<td>Peru</td>
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<td>Anita Villani</td>
<td>C3P</td>
<td>Canada</td>
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<tr>
<td>Michaela Willi</td>
<td>Patient Advocate</td>
<td>Austria</td>
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SAC co-chairs

Jamie Flerlage, MD, MS
St. Jude Children’s Research Hospital
Memphis, Tennessee, USA
*PCDC Consortium Representative (NODAL)*

Daisuke Tomizawa, MD, PhD
National Center for Child Health and Development
Tokyo, Japan
*PCDC Consortium Representative (INTERACT)*
SAC meetings

Most recent meeting: March 2022

- Reviewed the nominees for the PCDC External Advisory Board
- Discussed protecting the privacy of patients with data in the PCDC, focused on preventing potential re-identification of data by implementing minimum sample size reporting thresholds for PCDC data exploration

Next meeting: June 2022

- We will welcome our first patient advocate to the committee
SAC meeting, March 2022
2021-22 highlight: A global approach
Pediatric cancer research on the global stage

Ajay Major, MD, MBA
Databases → Data Commons → Data Platform
There is an exceptional variety in pediatric oncology collaborative groups across continents with respect to clinical trial design and execution.

This review suggests that a one-size-fits-all approach to increasing collaboration between international pediatric cancer clinical trial groups is not possible, with a nuanced understanding of local stakeholders and needs necessary to form partnerships.
THE NEED FOR A GLOBAL APPROACH

Kathy Pritchard-Jones

SIOP President
Professor of Paediatric Oncology,
University College London, UK

No child should die of cancer: cure for more, care for all
Key messages

• Value of clinical trials

• Added value of secondary use of clinical trial data

• Importance of global collaboration that embraces LMICs

• Importance of Parent and Patient involvement in research
Population survival from childhood cancer in Britain during 1978–2005 by eras of entry to clinical trials

C. A. Stiller¹*, M. E. Kroll¹,² & K. Pritchard-Jones³

¹Childhood Cancer Research Group, Department of Paediatrics; ²Cancer Epidemiology Unit; Nuffield Department of Clinical Medicine, University of Oxford, Oxford; ³Department of Paediatric Oncology; Institute of Child Health, University College London, Great Ormond Street Hospital for Children NHS Trust, London, UK


> 25,000 children
Multi-centre clinical trials of first line treatment open over >10yr period
Majority of children enrolled
Trial portfolio coordinated by a National Children’s Cancer professional society
Population survival from childhood cancer in Britain during 1978–2005 by eras of entry to clinical trials

C. A. Stiller¹*, M. E. Kroll¹,² & K. Pritchard-Jones³

¹Childhood Cancer Research Group, Department of Paediatrics; ²Cancer Epidemiology Unit; Nuffield Department of Clinical Medicine, University of Oxford, Oxford; ³Department of Paediatric Oncology; Institute of Child Health, University College London, Great Ormond Street Hospital for Children NHS Trust, London, UK


• The key message from this study is that clinical trials are good for you if you are a cancer patient. Taking part in clinical trials is considered best practice for most newly diagnosed childhood cancers now. This study shows how the increase in the proportion of children taking part in trials has gone hand in hand with improvements in survival.
Outcomes for Children and Adolescents With Cancer: Challenges for the Twenty-First Century

Survival is not equitable….. yet

Figure 6. Estimated childhood cancer 5-year net survival by country (2015–2019)

Source: Adapted from Ward et al. 2019 (26).
Childhood cancer cases globally

Major et al (2022)
Co-operative groups in LMICs  
Carlos Rodríguez-Galindo et al, JCO (2015)
Clinical trials

• Clinical trial costs increasing
• Smaller subgroups for therapeutic questions
• Need to:
  • increase ‘value’ of data collected
  • new methods for prospective clinical research
  • increase diversity of populations included
Society expects that data is shared for good purpose with appropriate safeguarding of privacy.
Treatment of Orbital Rhabdomyosarcoma: Survival and Late Effects of Treatment—Results of an International Workshop

By Odile Oberlin, Annie Rey, James Anderson, Modesto Carli, R. Beverley Raney, Joern Treuner, and Michael C.G. Stevens for the International Society of Paediatric Oncology Sarcoma Committee, the Intergroup Rhabdomyosarcoma Study Group, the Italian Cooperative Soft Tissue Sarcoma Group, and the German Collaborative Soft Tissue Sarcoma Group

**TREATMENT OF ORBITAL Rhabdomyosarcoma**

**Fig 3.** EFS according to initial radiotherapy: radiotherapy, 82% (range, 76% to 87%); no radiotherapy, 53% (range, 76% to 87%) \(P < .001\).

**Fig 4.** OS according to initial radiotherapy: radiotherapy, 86% (range, 82% to 93%); no radiotherapy, 86% (range, 77% to 95%).
Personalised medicine – the data perspective

- Understanding variation within and between populations
- Identifying rare subgroups with different outcomes
- Matching patients to therapies
- Clinical trial datasets are a goldmine
Personalised medicine – the data perspective

- Understanding variation within and between populations
- Identifying rare subgroups with different outcomes
- Matching patients to therapies
- Clinical trial datasets are a goldmine

All require ability to share patient-level data
Building trust with parents/patients is key
Overall survival rates vary across Europe

- Largest differences (> 20%) between East and West European countries
- Smaller differences (~3-5%) between UK and France/Germany

- How much is explained by differences in:
  - Tumour stage at diagnosis?
  - Tumour biology?
  - Treatment delivery?
Understanding data sharing for children’s and young people’s cancer
Types of data

Spectrum of identifiability

- Personally identifiable
- De-personalised
- Anonymous

More identifiable  Less identifiable
Under special laws don’t need consent to collect your data.
Best interests of patients and the public.
Follow strict rules on how the data is stored and used.
Including who can see it.
Checked annually.
Strong support for data sharing for research from childhood cancer survivors and their families, but

Keep us involved and informed!
Together we can help improve outcomes for children with cancer wherever they may live in the world.
THANK YOU – QUESTIONS?

k.pritchard-jones@ucl.ac.uk

No child should die of cancer: cure for more, care for all
2021-22 highlight: New analytics tools

Kirk Wyatt, MD, MAS
Responsible data access

- Data access
- Promoting research
- Protecting patient-level data
- Responsible use

Project request process
How can we decrease the barrier to entry and increase engagement while protecting patient-level data?

Facilitate **controlled data access** prior to project request stage

- Explore available data
- Determine sample size/feasibility
Cohort explorer

- Accessible to anyone
- Easy to use
- Provide results instantly
- Does not expose patient-level data
Kaplan-Meier survival analysis tool

- Facilitates early hypothesis exploration
- Accessible via pilot launch
- Launching early June 2022
Safeguards & pilot

- Safeguards to prevent misuse developed with input from consortia
- Pilot to assess effectiveness of safeguards
- Users of survival analysis tool agree to abide by pilot terms
Explore the data commons!

portal.pedscommons.org

Kaplan-Meier survival analysis tool
coming early June 2022

Thank you: member consortia and Scientific Advisory Committee
A user perspective

Raj Venkatramani, MD, MS, MBA
USING INSTRuCT TO IMPROVE SARCOMA OUTCOMES
(INternational Soft Tissue saRcoma ConsorTium)

Rajkumar Venkatramani, MD
Texas Children’s Hospital, Baylor College of Medicine
INSTRuCT Executive Committee

Chairs
Gianni Bisogno (EpSSG), Doug Hawkins (COG), Ewa Koscielniak (CWS)

Members

<table>
<thead>
<tr>
<th>COG</th>
<th>CWS</th>
<th>EpSSG</th>
<th>Statisticians</th>
<th>CIO / Data Commons</th>
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<tr>
<td>David Rodeberg</td>
<td>Martin Ebinger</td>
<td>Hans Merks</td>
<td>Wei Xue</td>
<td>Sam Volchenboum</td>
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<td>Suzanne Wolden</td>
<td>Monika Sparber-Sauer</td>
<td>Veronique Minard-Colin</td>
<td>Gian Luca De Salvo</td>
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Work Groups

- 2018 Work Groups
  - Pathology/Biology
  - NRSTS
  - Surgery
  - Statistics
- July 2019
  - Radiation Oncology
- March 2020
  - Imaging
4 Groups
- COG
- EpSSG
- CWS
- MMT

4600 RMS Patients
UPFRONT SURGERY

DELAYED SURGERY
3 RISK GROUPS

4 RISK GROUPS
Main Goal

- International risk stratification for RMS
- Use clinical trial data from mid-1990s onward
- Aim to finish analysis by summer 2022
AREAS OF UNCERTAINTY

- Favorable vs. unfavorable sites
- Tumor size cut-point
- PAX3 vs. PAX7 fusion
CHALLENGES

• Missing data (especially FOXO1)
• Evolving histology classification
• Different treatment philosophies
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<th>Data source</th>
<th>Requestor</th>
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<tr>
<td>Pooled analysis of outcome after first recurrence of metastatic RMS</td>
<td>COG, CWS, MMT</td>
<td>Julia Chisholm</td>
<td>Approved</td>
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<td>Clinical features and outcome for extremity RMS</td>
<td>INSTRuCT</td>
<td>Sapna Oberoi</td>
<td>Approved</td>
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<td>Pooled analysis of biliary rhabdomyosarcoma</td>
<td>INSTRuCT</td>
<td>Jamie Aye</td>
<td>Approved</td>
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<td>Outcome for orbital rhabdomyosarcoma</td>
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<td>Jonathan Metts</td>
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<td>Infants with rhabdomyosarcoma</td>
<td>INSTRuCT</td>
<td>Sapna Oberoi</td>
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<tr>
<td>Isolated distant nodal metastatic rhabdomyosarcoma</td>
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<td>Federico Mercolini</td>
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## Consensus projects under development

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<tr>
<th>Topic</th>
<th>Work Group</th>
<th>COG Lead</th>
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<tr>
<td>Molecular testing of childhood RMS in clinical trials to improve risk stratification and outcome</td>
<td>Pathology/ Biology</td>
<td>Corinne Linardic</td>
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<td>Surgical approach, local control for biliary/liver RMS</td>
<td>Surgery</td>
<td>Todd Heaton</td>
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<td>Surgical approach, local control for perineal/perianal RMS</td>
<td>Surgery</td>
<td>Dave Rodeberg</td>
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<td>Histologic grading in high risk NRSTS</td>
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<td>Nodal spread in NRSTS</td>
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<td>Radiotherapy for parameningeal RMS</td>
<td>Radiation Oncology</td>
<td>Dana Casey</td>
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<td>Radiotherapy for orbital RMS</td>
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<td>Ralph Ermoian</td>
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<td>Radiotherapy for metastatic RMS</td>
<td>Radiation Oncology</td>
<td>Stephanie Terezakis</td>
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</table>
Publications – collaboration even before data

- Introducing INSTRuCT: An international effort to promote cooperation and data sharing
  DOI: 10.1002/pbc.28701

- Local treatment of rhabdomyosarcoma of the female genital tract: Expert consensus from the Children’s Oncology Group, the European Soft-Tissue Sarcoma Group, and the Cooperative Weichteilsarkom Studiengruppe
  DOI: 10.1002/pbc.28601

- Surgical management of extremity rhabdomyosarcoma: A consensus opinion from the Children’s Oncology Group, the European Pediatric Soft-Tissue Sarcoma Study Group, and the Cooperative Weichteilsarkom Studiengruppe
  DOI: 10.1002/pbc.28608

- Pathology of childhood rhabdomyosarcoma: A consensus opinion document from the Children’s Oncology Group, European Paediatric Soft Tissue Sarcoma Study Group, and the Cooperative Weichteilsarkom Studiengruppe
  DOI: 10.1002/pbc.28798

- Surgical Management of Paratesticular Rhabdomyosarcoma: A Consensus Opinion from the Children's Oncology Group, European Pediatric Soft Tissue Sarcoma Group, and the Cooperative Weichteilsarkom Studiengruppe
  DOI: 10.1002/pbc.28938
We gratefully acknowledge and thank...

The International Soft Tissue Sarcoma Consortium and the Pediatric Cancer Data Commons are supported in part by Cancer Research Foundation, Children's Research Foundation, Comer Development Board, KickCancer, King Baudouin Foundation, Rally Foundation for Childhood Cancer Research, Seattle Children's Foundation from Kat's Crew Guild through the Sarcoma Research Fund, St Baldrick's Foundation, and The Andrew McDonough B+ Foundation. This work is made possible through the efforts of Children's Oncology Group, Cooperative Weichteilsarkom Studiengruppe der GPOH (CWS), The European paediatric Soft tissue sarcoma Study Group, MMT Malignant Mesenchymal Tumour Committee, STSC AIEOP Italian Soft Tissue Sarcoma Committee
Relapsed patients struggle to find therapies

- Traditional therapy
- Additional testing
- Phase I/II/III clinical trial

Child with relapsed AML

*Weeks/months*
Relapsed patients struggle to find therapies

Child with relapsed AML

Traditional therapy
Additional testing

Weeks/months

Phase I/II/III clinical trial

Tumor genetics
Clinical information
Germline testing

Trial 1
Trial 2
Trial 3
Trial 4
ClinicalTrials.gov is difficult to use

Partial inclusion criteria for PEPN2113

“Highest Dose of Uproleselan in Combination With Fludarabine and Cytarabine for Patients With Acute Myeloid Leukemia, Myelodysplastic Syndrome, or Mixed Phenotype Acute Leukemia Relapsed or Refractory and That Expresses E-selectin Ligand on the Cell Membrane”
Relapsed patients struggle to find therapies

- Traditional therapy
- Additional testing
- Phase I/II/III clinical trial

48-72 hours

Genomic Eligibility Algorithm at Relapse for Better Outcomes
Find clinical trials for your patients. Instantly.

GEARBOx Generic Eligibility Algorithm at Relapse for Better Outcomes helps you rapidly match patients with relapsed or refractory disease to appropriate clinical trials.

GET STARTED
Patient characteristics
Disease characteristics
Lab tests
Genomic testing

Clinical trials
Information about enrollment
Study locations
Third Annual PCDC Public Webinar
May 18, 2022

@PedsDataCommons
commons.uchicago.edu
This site is intended for pilot use only at this time and matching results should not be used for eligibility assessment of actual patients.

**OPEN TRIALS**

**Matched (2)**

**APAL2020SC**

**Title**
A Study to Test Bone Marrow and Blood in Children With Leukemia That Has Come Back After Treatment or Is Difficult to Treat

**Description**
This study aims to use clinical and biological characteristics of acute leukemias to screen for patient eligibility for available pediatric leukemia sub-trials. Testing bone marrow and blood from patients with leukemia that has come back after treatment or is difficult to treat may provide information about the patient’s leukemia that is important when deciding how to best treat it, and may help doctors find better ways to diagnose and treat leukemia in children, adolescents, and young adults.

**Link**
- [ClinicalTrials.gov](https://clinicaltrials.gov)

Pediatric Clinical Trial Nurse Navigator One-on-One Support
To connect with a Pediatric Clinical Trial Nurse Navigator at the Leukemia & Lymphoma Society who will personally assist your patient throughout the entire clinical trial process, click this link to fill out a Clinical Trial Support Center referral form. One of our pediatric oncology nurses will call your patient within 1 business day and provide you with a copy of the individualized trial search results.
GEARBOx - Future state

Global trials
All tumor types

Access controls

GEARBOx

List of trials

Centralized pan-cancer navigator

Global trials
All tumor types

List of trials

GEARBOx

Access controls

TEMPPUS

Hematologies, Inc.

Global trials
All tumor types

List of trials

Centralized pan-cancer navigator

Global trials
All tumor types

List of trials

Centralized pan-cancer navigator
GEARBOx expansion

- GEARBOx will be expanded to include additional cancers
  - Neuroblastoma
  - Rhabdomyosarcoma
  - Osteosarcoma
  - Acute lymphoblastic leukemia
  - Germ cell tumors

- Anyone interested in helping should contact the PCDC
What's next for the PCDC?

Sam Volchenboum, MD, PhD
Integration with national projects
NCI Childhood Cancer Data Initiative (CCDI)

$50M per year for 10 years

Maximize every opportunity to improve treatment and outcomes for children with cancer

Build a connected data infrastructure to enable sharing of childhood cancer data from multiple sources

Identify opportunities to make data work better for patients, clinicians, and researchers

Develop and enhance tools and methods to extract knowledge from data

Credit: Jaime M. Guidry Auvil
CCDI: connecting pediatric cancer data

Credit: Jaime M. Guidry Auvil
Supporting the Childhood Cancer Clinical Data Commons (C3DC)

**PCDC will be:**

- Providing data dictionary services
- Harmonizing clinical data
- Assisting the NCI in building and deploying data models
PCDC plans
PCDC Data Portal

Cohort discovery and visualization

Direct connection to other data commons (Genomic Data Commons, COG Tissue Bank)
Cancer cases integrated to date

- INSTRuCT
- INRG

PCDC formed

2005 2010 2015 2020

0 10,000 20,000 30,000
### Cases integrated to date

#### INRG

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#### INSTRuCT

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**29,262 cases to date**

Retrospective data
Integrated portal browser
#PCDC100K

Current | Projected (Estimates)
--- | ---

Total Cases

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100,000
PCDC is already working on most cancer types

Brain tumors (28%)
Acute lymphocytic leukemia (17%)
Other rare tumors (renal, NPC, etc.) (~8%)
Acute myelogenous leukemia (4%)
Non-Hodgkin lymphoma (7%)
Germ cell tumors (6%)
Hodgkin lymphoma (6%)
Thyroid (5%)

- Neuroblastoma (4%)
- Wilms tumor (4%)
- Rhabdomyosarcoma (2.5%)
- Osteosarcoma (2.5%)
- Retinoblastoma (1.5%)
- Ewing sarcoma (1.4%)
- Melanoma (2%)
- Liver tumors (1.4%)

Incidence in US as a percent of all cancers, ages 0-19
Total ~16,000 cases/year in the US

Adapted from https://acsjournals.onlinelibrary.wiley.com/doi/epdf/10.3322/caac.21654

Current PCDC project
Not yet a PCDC project
Working to add even more cancer types

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- **Current PCDC project**
- Not yet a PCDC project
- Work in progress

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Connections to other commons

Use USI to query clinical / genomic data and refer researcher to data source
Connections to other commons

- Stephen Skapek, MD, Lin Xu, PhD, Susan Cohn, MD, Mark Applebaum, MD
  *Identifying neuroblastoma drivers and bringing them to the clinic*

- Riyue Bao, PhD, Ami V. Desai, MD, Susan Cohn, MD
  *Validation of a T-cell inflammatory signature and outcomes in patients with neuroblastoma*
Survivorship / long-term follow-up

- Most children with cancer in the US survive
- **Long-term follow-up** is important to monitor the effects of therapy
- 30 years after treatment, 75% have at least one chronic health condition
- Patients’ ongoing access to their data has been a challenge
- As newer therapies emerge, follow-up is even more critical
- There are many social and technical issues with LTFU
- Long-term follow-up data in the PCDC can be linked to clinical trial information
Beyond pediatric cancer
We want to maximize the power of high-quality, accessible data to drive discovery and improve human health.
Our approach to data

- **Accessibility**: lifting barriers to research
- **Usability**: high-quality, interoperable data
- **Diversity**: data that better represent the real world
- **Responsibility**: making it easy to do the right thing
- **Impact**: using data to its maximum potential
Accessibility

● Making data easily **accessible** lifts barriers to research
● Manual processes and siloed data collection and storage have held back progress
Usability

- PCDC takes a “data first” approach.
- Data must be FAIR:
  - Findable
  - Accessible
  - Interoperable
  - Reusable

Adapted from Mark A. Musen, MD, PhD
Diversity

- **Diversity** and representation in data are important for both equity and scientific knowledge
  - Geographic diversity
  - Demographic diversity
  - Genetic diversity

- **Data that “speak the same language”** and are collected in interoperable ways can be combined across sources and borders to create diverse datasets more representative of the real world

Responsibility

- As stewards of data, we want to make it easy to do the right thing.

- Increasing data accessibility makes it even more important to respect:
  - Patient privacy
  - Security
  - Varied international legal requirements
  - Good scientific practices
    - Promoting data literacy in the scientific community
Impact

Accessibility, usability, diversity, and responsibility make it possible for data to be used and reused to its maximum potential for knowledge and impact.

Applying our approach beyond pediatric cancer

Other diseases

- Rare diseases - benefit from larger study cohorts
- Rarer subtypes of common diseases
- Diseases associated with **specific genetic markers**

The sociome

- Studying the **social determinants of health**
- Combining medical data with other types of information to make new connections
Rare disease: monogenic diabetes

Why create a monogenic diabetes data commons?

- An atypical form of the disease
  - Results from changes to a single gene
  - Represents **1-4% of cases** in the US
- Need to increase the number of cases studied by **10-fold or more** to understand the full scope of the syndromes
- Data-generating research initiatives already exist, but are not coordinated

Source: [www.niddk.nih.gov/health-information/diabetes](http://www.niddk.nih.gov/health-information/diabetes)
Monogenic diabetes data commons

To include:
- Clinical data
- Patient-reported outcomes
- Data from wearable devices such as continuous glucose monitors

Process
- Working closely with the UChicago Kovler Diabetes Center
- Applying PCDC methods to building data dictionaries, creating consortia, and developing governance structures
What is the sociome?

Social determinants of health (SDOH)

Source: CDC
Does environment influence response to cancer immunotherapy?
Environment and blood pressure

1. Does moving away from high-crime areas result in lower blood pressures?
2. What are the biological and sociome predictors of who actually benefits most?
Environment matters
Data distribution and missingness
Datasets under investigation

- Traffic
- Crime
- Energy Benchmarking
- Beach Weather Data
- Parks
- Land use
- Grocery Stores
- Graffiti
- Pedestrians
- Tree canopy

- Air quality
- After school programs
- Preschool Programs
- Foreclosures
- Vacant and abandoned buildings
- Flu Shot Locations
- WIC Clinic Locations
- Primary Care Clinic Locations
- Asbestos Notifications
- Neighborhood vulnerability measures
Get involved
Join the conversation

● Join us for **The Common Good**, our guest speaker series about big ideas in cancer research, clinical trials, and data sharing
  ○ **Next talk**: July 21 at 11am CDT, Dr. Carlos Rodriguez-Galindo on the global landscape of pediatric oncology clinical trials
  ○ **Watch videos of past talks**: sam.am/commongood

● Sign up for newsletters at **sam.am/PCDCnews**

● Join the PCDC team, or share our job postings with your networks: commons.cri.uchicago.edu/careers/
Do research with the PCDC

**portal.pedscommons.org**
Explore the PCDC Data Portal

**sam.am/datadictionaries**
Access data dictionaries on the PCDC website

**kblumhardt@bsd.uchicago.edu**
Email Kat Blumhardt if you are interested in getting involved with a consortium
Help sustain our work

- Contact Bobbi Nease (bnease@mbsd.uchicago.edu) to learn more about opportunities to partner with or support the PCDC
- Integrate expectations for data sharing and/or data standards plans into grant requirements and requests for proposals
- Help raise funds with your local groups and networks
## Our funders

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