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The logo for Day One Biopharmaceuticals, featuring the text 'Day One' in a large, white, sans-serif font, with 'BIOPHARMACEUTICALS' in a smaller font below it. To the right of the text is a stylized sunburst graphic composed of white lines radiating from a central point.



CURESEARCH

FOR CHILDREN'S CANCER

PEDIATRIC EARLY DEVELOPMENT SYMPOSIUM

*PEDIATRIC ONCOLOGY CLINICAL TRIAL
DEVELOPMENT: NEW APPROACHES FOR
A CHANGING WORLD*

OCTOBER 6-8, 2021

THE PEDIATRIC CANCER LANDSCAPE TODAY

Every year, over

17,000 children in the US

and **300,000 globally** are diagnosed with cancer.^{2,3}

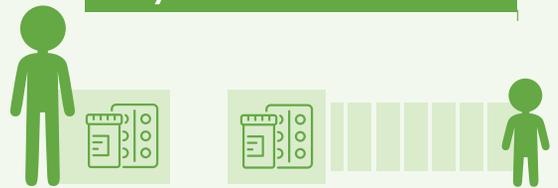


Depending on tumor type,

survival rates can range from **less than 1% to over 90%.**²

On average, clinical trials for children begin

6.5 years after adult trials.¹



Cancer is the **#1 cause of death** by disease in children in this country.⁴



4



Most current **standard treatments** for pediatric cancer were **approved before 1990;** half before the **mid-1980's.**⁵

Even as more children defy the odds, the treatments used to save their lives continue to have toxic side effects:



Due to the toxicity of treatments, **2 out of every 3 survivors** will still develop at least one **chronic health condition,** including musculoskeletal problems and second cancers.⁶



Nearly 60% of childhood cancer survivors experience severe or life-threatening **complications in adulthood.**⁷

Visit curesearch.org/Childhood-Cancer-Statistics for a complete list of references.



We're laser focused on driving new treatments to patients in an accelerated timeframe. We only fund projects with commercial potential, anticipated to reach patients in the clinic or marketplace within three to five years.

Funding Priorities:

Projects with commercial potential, anticipated to reach the clinic in an accelerated timeframe or already in clinical trials.

Fund research from U.S., Canada, EU and Australia.

Address barriers in areas of high unmet need, including high-risk, relapsed, or metastatic disease, cancers with limited or toxic treatment options, and adolescent and young adult patient populations.

Focus on innovative treatment modalities such as novel targeted therapeutics, immunotherapy, and combination therapies.

With the expert leadership of our Scientific and Industry Advisory Councils, we identify and fund only the strongest research projects that address areas of unmet need and are most likely to quickly reach patients in the clinic or marketplace via our three distinct programs.

- CureSearch Young Investigator awards combat the loss of promising scientists from the field by providing significant financial support to investigators early in their research careers. These grants are limited to truly transformational science designed to deliver the next generation of cancer treatment to the clinic in three to five years.
- CureSearch Acceleration Initiative projects are highly innovative, address a significant challenge in pediatric cancer drug development, and have a strong probability of clinical application — ready to reach patients within three years.
- Catapult Awards propel high-potential research out of the lab into the clinic and ultimately, to the kids who need it most. Our Catapult Awards provide funding for Phase 1 or Phase 2 clinical trials that advance promising therapies for pediatric cancer. The Catapult Award uniquely funds cutting-edge clinical trials that begin enrolling patients in less than a year, and every applicant must be partnered with a for-profit company to ensure they have developed the necessary infrastructure to ultimately move the therapy from clinical trials into the marketplace.

Since we began funding pre-clinical projects through our Acceleration Initiative in 2013, multiple projects have directly led to the launch of phase one clinical trials offering new treatment options, including trials that are now enrolling Ewing sarcoma and medulloblastoma patients across the country. We're impacting patients today while working to change outcomes for all children in the future.

2020 PEDS IMPACT STATEMENT

The CureSearch Pediatric Early Development Symposium was held virtually on October 1-2, 2020. This inaugural event convened representatives from all areas of pediatric oncology: pharmaceutical and biotechnology companies, clinical research organizations, academic leaders, parents and patient advocates, venture capitalists, regulators, and like-minded partners. Attendees and speakers participated from across the United States, Canada, the United Kingdom, the Netherlands, Italy, Germany, Switzerland and France.

The two-day meeting featured interactive sessions exploring key, timely topics in iPSP and PSP development: The Evolution of Cooperative and Collaborative Groups; Formulation Consideration for Pediatrics; Advancing Promising Agents Into The Clinic: What Is An “Acceptable” Preclinical Data Package?; Clinical Development Considerations: Lessons Learned from PIPs and PSPs; Nuts and Bolts of Writing and Negotiating Successful PIPs and PSPs; Global P(a)ediatric Development: How To Engage Internally and Enable External Alignment; and PIP/PSP Development and Implementation.

The recurring themes through the Symposium were the urgent need for **early and frequent collaboration**, and expanded opportunity for **CureSearch to drive this collaboration as a connector and resource** for academic and industry leaders.

2020 Pediatric Early Development Symposium Action Plan

Based on robust panel discussions and audience participation, CureSearch has identified three key opportunities below. Over the next 12 months, working collaboratively with global stakeholder and volunteer leaders, CureSearch will execute on the following strategies:

Strategy	Promote global collaboration across all roles in pediatric drug development	Provide pediatric drug development resources to stakeholders	Address areas of unmet need in clinical development for children with cancer
Actions	<p>Develop a comprehensive list of current industry leaders who trained as pediatric oncologists and are interested in collaboration to advance pediatric drug development</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Consult with CureSearch Industry and Scientific Advisory Councils to begin creation of a global roster <input checked="" type="checkbox"/> Invite greater pediatric oncology stakeholders to participate and contribute 	<p>Bridge the educational gap for academic leaders on the drug development process</p> <ul style="list-style-type: none"> <input type="checkbox"/> Explore providing in collaboration with a like-minded partner, a Continuing Medical Education (CME) training <input type="checkbox"/> Develop a mentorship program in drug development to connect Young Investigators to pediatric oncology champions in the industry 	<p>Drive collaborative discussion around optimal clinical trial designs</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Create a venue of discussion for engaging with consortia globally <input type="checkbox"/> Collaborate with like-minded partners to explore increasing industry-sponsored master protocols
	<p>Design and launch a virtual open-forum for Industry and Academic leaders to ask questions, request assistance, connect, and collaborate</p> <ul style="list-style-type: none"> <input type="checkbox"/> Identify core members to actively engage on platform <input type="checkbox"/> Invite academic institutions and industry leaders to participate 	<p>Create a Pediatric Oncology Drug Development Resource page on curesearch.org to include:</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Global regulatory processes and links to regulation <input checked="" type="checkbox"/> Global patient advocacy groups and consortiums <input checked="" type="checkbox"/> Case Studies, White Papers & Studies 	<p>Discuss the current state of research-only biopsies from children</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Host the 2021 CureSearch Virtual Summit Series <input type="checkbox"/> Create comprehensive list of pediatric oncology biorepositories to list on CureSearch Drug Development Resource page <p>Create a working group to explore the unique issues related to regulatory submissions for cellular and immunotherapies for pediatric cancer</p> <ul style="list-style-type: none"> <input type="checkbox"/> Identify Chair and members <input type="checkbox"/> Establish the preclinical data needs for regulatory filings

FROM THE PEDS CO-CHAIRS

Welcome to our colleagues!

The inaugural CureSearch Pediatric Early Development Symposium (PEDS), held in 2020, established a new platform for collaboration between academia, industry, advocacy, and regulatory experts in order to accelerate the pace of pediatric drug development. Over the past year, CureSearch has worked to fulfill the next steps identified at the initial symposium, outlined in the 2020 PEDS Action Plan, and has planned our Second Annual Pediatric Early Development Symposium, a meeting that we are excited that you will be participating in. While the first symposium was focused on best practices for the development of pediatric study plans, this meeting will focus on innovating for greater efficiency in pediatric clinical trial execution. We hope that the broad array of topics covered during this meeting will help us all share learnings and strategies to ensure success for the next generation of pediatric oncology clinical trials.

On August 18, 2020, Title V of the FDA Reauthorization Act (FDARA), commonly known as the RACE for Children Act, took effect. While this legislation has increased the opportunities for new therapy development for children with cancer, now a year later, we are learning about the unique pressures that it – as well as the ongoing pandemic - have placed on our existing clinical research infrastructure. As the number of anti-cancer agents available to children increase, innovative adjustments to all parts of the clinical trial process will be required to enhance efficiency, increase access to and for patients, and ensure that the most beneficial therapies reach children faster. Collaboration and forethought within, and across, the pediatric oncology drug development ecosystem are pivotal for the promotion of innovation. At this meeting, we aim to explore new strategies to better enable pediatric oncology drug development by biopharmaceutical drug developers, academic investigators and clinical trial sites, and importantly, patients. Similarly, we hope to openly identify and address challenges and lessons learned as these strategies have been implemented.

With even more pediatric oncology clinical trials in development, as a community we must all be strategic and creative in how we plan and implement this work, as well as in how we continue to build collaborations between academia and industry in order to most efficiently develop new cancer therapies for children.

We are in an era of immense promise for the development of new cancer therapies for children, but with this promise there are also inherent challenges. Working together to gain a greater understanding of how to address these challenges and sharing information will allow us to develop, evolve, and implement strategies that not only meet regulatory requirements, but truly advance the care of children with cancer.

Innovating within the clinical trial ecosystem to benefit children with cancer is going to require enduring and inventive contributions of all stakeholders. Convening at this event is only the first step in improving the system. Thank you in advance for your active participation in this virtual meeting.

Warmly,



Samuel C. Blackman, MD, PhD
Chief Medical Officer and Co-Founder
Day One Biopharmaceuticals



Brenda Weigel, MSc, MD
Director of the Division of Pediatric
Hematology/Oncology
University of Minnesota's Masonic Cancer Center

MESSAGE FROM OUR CEO

Dear Pediatric Oncology Stakeholders,

I'm thrilled to welcome you to the 2021 CureSearch Pediatric Early Development Symposium (PEDS) and continue the collaborative conversations that we began during last year's inaugural symposium.

At CureSearch, we're working to bring children diagnosed with cancer to the forefront of drug development, and that requires strategic collaboration across the pediatric cancer ecosystem. This year's symposium serves as a platform for leaders in academia, industry and regulatory agencies to address the unique aspects and challenges of pediatric cancer clinical trial design.

In the months following the 2020 PEDS symposium, we worked together to execute the strategies developed from meeting insights, including:

Building a comprehensive list of current Industry leaders who trained as pediatric oncologists and are interested in collaboration to advance pediatric drug development

Creating a Pediatric Oncology Drug Development Resource page on [curesearch.org](https://www.curesearch.org) to include: global regulatory processes and links to regulation; global patient advocacy groups and consortiums; case studies and white papers

Discussing the current state of research-only biopsies from children via the 2021 CureSearch Summit

As we continue this collaborative work, thank you for your participation in this year's event. We look forward to developing a 2021 action plan that will drive greater efficiency and collaboration around pediatric oncology clinical trials, so that we can improve therapies for the children who are counting on us.



Kay Koehler
President & CEO
CureSearch for Children's Cancer

INDUSTRY ADVISORY COUNCIL



The Industry Advisory Council includes leaders from the largest global oncology and biotechnology companies, and clinical research organizations who champion CureSearch and pediatric cancer programs within industry. The group reviews potential CureSearch-funded research projects for clinical and regulatory feasibility and provides objective counsel to researchers to assess clinical translation and drug development when evaluating each project.

Council Chair — Samuel C. Blackman, MD, PhD

Chief Medical Officer and Co-Founder
Day One Biopharmaceuticals

Oncoming Chair — Jeffrey Skolnik, MD

Vice President, Clinical Development
Inovio Pharmaceuticals Inc.

Elly Barry, MD, MMSc

Senior Vice President, Clinical Development
Day One Biopharmaceuticals

Anne Borgman, MD

Vice President & Head of Hematology-Oncology
Clinical Development
Jazz Pharmaceuticals

Hubert Caron, MD, PhD

Senior Medical Director
Hoffman-La Roche AG

Davy Chiodin, PharmD

Senior Vice President, Product Development
Day One Biopharmaceuticals

John Chung, MD

Executive Clinical Development Leader
Pediatric Oncology
Bayer Pharmaceuticals

Ruchi Gupta, MS

Program Director
Regulatory Affairs
Genentech

Kevin Heller, MD

Executive Vice President, Research
and Development
Jasper Therapeutics

Geoffrey Kannan, PhD, MD

Senior Medical Director
Global Clinical Development
LabCorp Drug Development Inc.

Mark Kieran, MD, PhD

Vice President, Clinical Development
Day One Biopharmaceuticals

Katherine Minson, MD

Medical Director
Parexel International

Jaiver A. Otero, MD, PhD

Senior Clinical Program Leader
Novartis Institute of Biomedical Research

Mark Sorrentino, MD, MS

Vice President
Centre for Pediatric Clinical Development &
The Centre for Vaccine Research and Emerging
Infectious Diseases
ICON PLC

Louis F. Stancato, PhD

Research Fellow,
Pediatric Capabilities
Eli Lilly and Company

Lori Styles, MD

Senior Medical Director
Pharmacyclics, an AbbVie Company

Jaszianna Tolbert, MD

Director, Clinical Development, Oncology
Janssen Research and Development, LLC

SCIENTIFIC ADVISORY COUNCIL

The Scientific Advisory Council (SAC) includes best-in-class pediatric oncologists who set the academic priorities for CureSearch research initiatives and evaluate projects on scientific merit. With the support of the SAC, CureSearch funds laboratory research aimed at transcending barriers and developing innovative approaches to solve the field's most challenging problems.

Council Chair — Brenda Weigel, MSc, MD

Professor and Lehman Family Chair in Pediatrics
Division Director, Division of Pediatric Hematology
and Oncology
University of Minnesota
Masonic Cancer Center

Scott A. Armstrong, MD, PhD

Chair, Department of Pediatric Oncology
Dana-Farber Cancer Institute
David G. Nathan Professor of Pediatrics
Harvard Medical School

Malcolm Brenner, MD, PhD

Professor, Department of Pediatrics
Section of Hematology-Oncology
Baylor College of Medicine
Director, Center for Cell and Gene Therapy
Texas Children's Hospital

Elizabeth Fox, MD

Senior Vice President, Clinical Trials Administration
Comprehensive Cancer Center
Associate Director of Clinical Research
St. Jude Children's Research Hospital

Lee J. Helman, MD

Professor of Pediatrics, Keck School of Medicine
University of Southern California
Head, Basic and Translational Research
Children's Center for Cancer and Blood Diseases
Children's Hospital of Los Angeles

Stephen L. Lessnick, MD, PhD

Director, Center for Childhood Cancer & Blood
Diseases for The Research Institute at Nationwide
Children's Hospital
Physician, Division of Hematology and Oncology at
Nationwide Children's Hospital
Professor of pediatrics, The Ohio State University
College of Medicine

Maureen O'Brien, MD, MS

Medical Director of the Leukemia/
Lymphoma Program
Cincinnati Children's Hospital Medical Center
Associate Professor of Clinical Pediatrics University of
Cincinnati College of Medicine

Andy DJ Pearson, MD

Retired Professor of Pediatric Oncology
Institute of Cancer Research
Royal Marsden Hospital, England

Gregory H. Reaman, MD

Associate Director, Oncology Sciences
Office of Hematology and Oncology Products
OND Center for Drug Evaluation and Research
U.S. Food and Drug Administration

Donald Small, MD, PhD

Kyle Haydock Professor of Oncology
Professor of Oncology, Pediatrics, Cellular
Molecular Medicine, Human Genetics
Director, Pediatric Oncology
Sidney Kimmel Comprehensive
Cancer Center at Johns Hopkins

Loren D. Walensky, MD, PhD

Associate Professor, Department of Pediatrics
Attending Physician in Pediatric Oncology
Principal Investigator, Linde Program in Cancer
Chemical Biology
Director, Harvard/MIT MD-PhD Program
Dana-Farber Cancer Institute

THE BRIDGE INITIATIVE



Many promising medicines, despite strong safety data and pharmaceutical characteristics, are deprioritized by pharmaceutical companies for non-technical reasons including strategy, organization, and financial changes. Unlocking these drugs — for intended or new indications — is challenging due to informational, operational, and cultural obstacles within those companies. Meanwhile, patients and families suffer from waiting for new therapies for their conditions.

FasterCures, Children's Tumor Foundation, and CureSearch for Children's Cancer have created a non-profit initiative that will identify promising but discontinued drugs, approach biopharmaceutical companies, develop an externalization framework, and match validated drugs to new development and investment partners.

The organizations will be advised on a voluntary basis by leaders in the pediatric space, including:

Promising drugs can be deprioritized for many reasons:

- Low commercial potential
- High development hurdles
- Shift in strategic priorities
- Change in competitive environment
- Resource constraints

- **D. Wade Clapp, MD**, Richard L. Schreiner Professor and Chairman, Department of Paediatric at the Indiana University School of Medicine and Physician-in-Chief for Riley Hospital for Children
- **Elizabeth Fox, MD**, Senior Vice President, Clinical Trials Research at St. Jude Children's Research Hospital
- **Kevin Grimes, MD**, Co-director of the SPARK Translational Research Program and Professor of Chemical and Systems Biology, Stanford University School of Medicine
- **Andrew Kung, MD, PhD**, Chair, Department of Pediatrics, at Memorial Sloan Kettering Cancer Center
- **Freda Lewis-Hall, MD**, Board Member of FasterCures and former Chief Patient Officer and Chief Medical Officer at Pfizer
- **Chris Lee**, Managing Director, Strategic Growth at Palladium and member of CureSearch Strategic Oversight Committee
- **Baiju Shah**, Senior Advisor to FasterCures and former CEO of BioMotiv
- **Craig Thomas, PhD**, Leader, Division of Preclinical Innovation, Chemistry Technologies at National Center for Advancing Translational Sciences
- **Kathy Wanner**, Board Member of CureSearch and a Partner at Fairway Capital Management
- **Brigitte Widemann, MD**, Chief of the Pediatric Oncology Branch at the National Cancer Institute.

For more information, contact:

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FasterCures
schoe@milkeninstitute.org

Kay Koehler | President & CEO
CureSearch for Children's Cancer
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Annette Bakker, PhD | President
Children's Tumor Foundation
abakker@ctf.org

AGENDA: WEDNESDAY, OCTOBER 6

All times listed are Eastern Time Zone.

7:30 am – 10 am **Networking Lounges and One-on-One Networking**

10 am – 10:15 am **Welcome to the 2020 Pediatric Early Development Symposium**

Samuel C. Blackman, MD, PhD

PEDS Co-Chair & Chief Medical Officer & Co-Founder
Day One Biopharmaceuticals

Brenda Weigel, MD

PEDS Co-Chair & Director of the Division of Pediatric Hematology/
Oncology, University of Minnesota's Masonic Cancer Center

10:15am – 12:30 pm **Expanding the Global Footprint of Pediatric Oncology Clinical Trials**

This session will address the unique issues relating to expansion of clinical trials into other areas of the world. Panelists will discuss the potential and challenges for global pediatric oncology trials and regulatory challenges that differ amongst different countries and how companies and institutions are working to bridge the gaps

Session Chair: Sabine Mueller, MD, PhD

Professor of Clinical Neurology, UCSF; Co-Leader, Pediatric
Malignancies Program, UCSF Helen Diller Family Comprehensive
Cancer Center

12:30 pm – 1:15 pm **Break — Grab Lunch, Networking Lounges and One-on-One Networking**

THANK YOU TO OUR WORKING GROUP MEMBERS



CureSearch would like to extend our immense gratitude to the members of the Pediatric Early Development Symposium Working Group. This group of academic, industry and regulatory champions for pediatric oncology spent countless hours working on the PEDS agenda that we all get to enjoy. They also called friends and colleagues to identify chairs and build a team of speakers with appropriate expertise and passion for the innovation of pediatric oncology clinical trials. Without this team, the 2021 PEDS meeting would not be possible.

PEDS Co-Chair – Samuel Blackman, MD, PhD

Co-Founder and Chief Medical Officer
Day One Biopharmaceuticals

PEDS Co-Chair – Brenda Weigel, MD, MSc

Professor and Lehman Family Chair in Pediatrics
Division Director, Division of Pediatric Hematology
and Oncology
University of Minnesota
Masonic Cancer Center

Davy Chiodin, PharmD

Chief Development Officer
Day One Biopharmaceuticals

Elizabeth Fox, MD

Senior Vice President
Clinical Trials Administration
Comprehensive Cancer Center
Associate Director of Clinical Research
St. Jude Children's Research Hospital

Mark Kieran, MD, PhD

Vice President, Clinical Development
Day One Biopharmaceuticals

Daniel Morgenstern, MB BChir, PhD

Staff Physician - Solid Tumour Program
Medical Director, Oncology/BMTCT Clinical Trials
Support Unit
Director, New Agent and Innovative Therapy
Program
Director, Therapeutic MIBG Program
Associate Scientist, Translational Medicine, SickKids
Research Institute
Assistant Professor, Department of Paediatrics,
University of Toronto
Division of Haematology/Oncology
The Hospital for Sick Children

Sabine Mueller, MD, PhD

Professor, Neurology
Weill Institute for Neurosciences
University of California, San Francisco School of
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Clinical Lead, Diffuse Midline Glioma Center
University Children's Hospital of Zurich

Gregory H. Reaman, MD

Associate Director Oncology Sciences
Office of Hematology and Oncology Products, OND,
CDER
Associate Director for Pediatric Oncology
Oncology Center of Excellence, OMPT, OC
U.S. Food and Drug Administration

Louis F. Stancato, PhD

Research Fellow, Pediatric Capabilities
Eli Lilly and Company

AGENDA: FRIDAY, OCTOBER 8

All times listed are Eastern Time Zone.

7:30 am – 10 am Networking Lounges and One-on-One Networking

11:00 am – 12:45 pm Decentralized Clinical Trials

The session Decentralized Clinical Trials will explore the options for remote evaluation and use of satellite sites for clinical trials. Not only will the panel consider how decentralized trials are best conducted and the infrastructure required, but also how decentralization of trials and virtual platforms make trial participation easier for families.

Session Chair: Mark Turner, BSc, PhD, MBChB, MRCP(UK), MRCPCH, DRCOG, FFPM(Hon) – Professor of Neonatology and Research Delivery, University of Liverpool, Co-coordinator, Conect4Children

12:45 pm - 1:00 pm Break – Grab lunch in preparation for “Regulators Dish” Session

1:00 pm - 2:00 pm Lunch Plenary — Regulators Dish on the Future of Pediatric Oncology Clinical Trials

In this fireside chat, hear from global regulators on the next two decades in pediatric oncology clinical trials

Session Chair: PEDS Co-Chairs

2:00 pm - 2:30 pm Wrap Up

Co-Chairs: Samuel C. Blackman, MD, PhD - Chief Medical Officer & Co-Founder, Day One Biopharmaceuticals

Brenda Weigel, MD - Director of the Division of Pediatric Hematology/Oncology, University of Minnesota's Masonic Cancer Center



1:15pm – 3:00 pm

Real-world Data and Synthetic Control Arms

The session Real-world Data and Synthetic Control Arms will contextualize the current state of the emerging use of real-world data and synthetic control arms. There are very real challenges to the use of real-world data, from defining to collecting to archiving and utilizing them, but there is also a great deal of hope around the potential of real-world data to fulfill needs in postmarket safety monitoring, support clinical trial design and generate innovative treatment approaches. This session will balance the challenges and promise of real-world data. Synthetic control arms are another emerging field that offer potential to improve clinical trials. Discussion will center on the validity of synthetic control arms and the use of modeling to minimize the number of patients needed for clinical trials.

Session Chair: Peter Adamson, MD

Global Head, Oncology Development & Pediatric Innovation, Sanofi

3:00 pm –10:00 pm

Networking Lounges and One:One Networking

AGENDA: THURSDAY, OCTOBER 7

All times listed are Eastern Time Zone.

7:30 am – 10:00 am **Networking Lounges and One-on-One Networking**

10:00 am – 1:45 pm **Pharmacokinetic Modeling and Extrapolation from Adult Drug Dosing to Pediatrics**

This session addresses pharmacokinetic modeling and extrapolation from adult drug dosing to pediatrics, where the paucity of data in children and limited pool of patients demand the process be as safe, cost- and time-efficient as possible. The session will offer perspectives from academic, industry and regulatory leaders who will attempt to answer the following questions:

- As children are not small adults, what assumptions are appropriate to include in extrapolation of adult data in PK modeling?
- With adult PK data in hand, what parameters inform pediatric dosing model and decisions for size-based dosing (mg/kg or mg/m²) or flat or fixed dose?
- PBPK concepts vs allometric scaling (and how they can inform the starting dose)? How are PBPK and PopPK models qualified/validated?
- Given limited pediatric population sizes and the necessity of offering a prospect of direct benefit, when single or limited dose levels are evaluated in children is there sufficient data to validate a population PK model? What is the impact on evaluation of dose-response, dose-toxicity relationships in pediatric tumors?
- What is the current regulatory guidance on PK modeling and extrapolation in pediatric oncology? Do FDA and EMA take a similar stance?

Session Chairs:

Elizabeth Fox, MD, MS

Member, St. Jude Faculty, Senior Vice President, Clinical Trials Research, Associate Director for Clinical Research, Comprehensive Cancer Center, St. Jude Children's Research Hospital

Louis Stancato, PhD

Research Fellow, Pediatric Capabilities, Eli Lilly and Company



11:45 am – 12:15 pm **Break – Grab Lunch, Networking Lounges and One:One Networking**

12:15 pm – 1:15 pm **Keynote: The Path of a Pediatric Targeted Therapy Through Drug Development**

Session Chair: Elly Barry, MD, MMSc, Vice President of Clinical Development, Day One Biopharmaceuticals

1:15 pm – 3:00 pm **Clinical Trials with Combination Therapies**

Now that there is an avenue to test single agents early through the RACE Act and we strongly suspect that single agents will not be as effective as those used in combination, it is important to explore avenues to pursue combination clinical trials for pediatric cancer. This session will focus on the development of clinical trials with combination therapies.

Session Chair: Mark Kieran, MD, PhD –Vice President, Clinical Development, Day One Biopharmaceutical

3:00 pm – 10:00 pm **Networking Lounges and One:One Networking**