ACCELERATE
2023 Annual Conference

9-10 February 2023
Brussels Radisson Collection Hotel
& Virtual

Conference Programme

Day 1 - Thursday 9 February

Central European Time

09h00 – 10h00 Registration

10h00 – 10h15 Welcome and intro

10h15 – 12h00 Session I - Real World and/or Trial data – where are we heading to?

Chair: Pamela Kearns, *University of Birmingham*

- **NCI's Childhood Cancer Data Initiative - CCDI**
  Gregory Reaman, NIH
- **European initiatives for access to molecular and Real World Data**
  Stefan M. Pfister, *KiTZ Heidelberg*
- **ACCELERATE Long Term Follow Up initiative**
  Andy Pearson, *ACCELERATE*
- **The Data Analysis and Real World Interrogation Network - DARWIN EU**
  Daniel Morales, *European Medicines Agency*

Panel Discussion
Max Williamson, P*atient Advocate for the ACCELERATE FAIR Trials WG*
Lynley Marshall, *Royal Marsden*
Gregory Reaman, NIH
Andy Pearson, ACCELERATE
Stefan M. Pfister, KiTZ Heidelberg
Daniel Morales, European Medicines Agency

12h00 – 13h15 Lunch

13h15 – 14h45 Session II - ACCELERATE

Chair: Hubert Caron, *Roche*

13h15 – 13h45 **Accelerating Impact for Children with Cancer** - Patient Advocates-led session
Nicole Scobie, *Zoé4life*
Susan L. Weiner, *Children's Cancer Cause*
Leona Knox, *Solving Kids' Cancer UK*
Patricia Blanc, *Imagine for Margo*
Joe McDonough, *The Andrew McDonough B+ Foundation*

13h45 – 14h45 ACCELERATE at 360

- **Education**
  Teresa de Rojas, *ACCELERATE*

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- Intercontinental collaboration
  Leona Knox, *Solving Kids' Cancer UK*
- *ACCELERATE access to new medicines initiative*
  Gilles Vassal, *ACCELERATE*
- Fostering Age-Inclusive Research
  Nathalie Gaspar, *Institute Gustave Roussy*

14h45 – 15h15 Coffee break + photo

15h15 – 18h00 Session III – Parallel Breakout sessions

BkS 1 – Paediatric Patient-Reported Outcomes – can we make patient-centred research a reality?

Group A	Group B
Leona Knox *Solving Kids' Cancer UK*	Pamela Kearns *University of Birmingham*
Lia Gore *Children's Hospital Colorado*	Willemijin Plieger *Dutch Childood Cancer Org*

BkS 2 – Paediatric Strategy Forums 2.0

Group A	Group B
Susan Weiner *Children's Cancer Cause*	Nicole Scobie *Zoe4life*
Elizabeth Fox *Children's Research Hospital Colorado*	Chinyere Okpara *Eisai Co., Ltd*

BkS 3 – Implementing Mechanism of Action globally

Group A	Group B
Elly Barry *Day One Biopharamaceuticals*	Peter Adamson *Sanofi*
E. Anders Kolb *Nemours Children's Hospital*	Brenda Weigel *University of Minnesota*

20h00 – 23h00 Social dinner at the historical Belgian restaurant «Aux Armes de Bruxelles»

Day 2 - Friday 10 February

Central European Time

08h00 – 08h30 Registration

08h30 – 10h00 Session IV - Update on regulatory landscape in Europe and USA

Chair: Delphine Heenen, *KickCancer*

- The RACE for children act at two years – progress in development of MoA targeted paediatric oncology drugs
  Martha Donoghue, *U.S. Food and Drug Administration*
- The European Regulatory strategy for supporting childhood cancer therapy developments
  Dominik Karres, *European Medicines Agency*
- Update on the Revision of the EU Paediatric Regulation
  Fabio D'Atri, *European Commission*
- ACCELERATE Fit for Filing
  Pamela Kearns, *University of Birmingham*

10h00 – 10h30 Coffee break

10h30 – 12h00 Session V - Paediatric Patient-Reported Outcomes

Chair: Martha Donoghue, *U.S. Food and Drug Administration*

- Paediatric Patient-Reported Outcomes - US perspective
  Bryce B. Reeve, *Duke University School of Medicine*
- Paediatric Patient-Reported Outcomes - EU perspective
  David Riedl, *Innsbruck Medical University*
- Report from Breakout session "Paediatric Patient-Reported Outcomes measures – can we make patient-centred research a reality?"

Panel discussion
Leona Knox, *Solving Kids' Cancer UK*
Lia Gore, *Children's Hospital Colorado*
Pamela Kearns, *University of Birmingham*
Willemijn Plieger, *Dutch Childhood Cancer Org.*
Bryce B. Reeve, *Duke University SoM*
David Riedel, *Innsbruck Medical University*

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12h00 – 13h15 Lunch

13h00 – 14h30 Session VI - Pre-clinical prioritisation - which way to go?

Chair: Douglas S. Hawkins, *Seattle Children's Hospital, COG Chair*

- **ITCC P4 - Paediatric Preclinical Proof Of Concept Platform**
  Louis Stancato, *Eli Lilly and Company*
- **PIVOT Cancer Preclinical Drug Development Program**
  Malcolm Smith, *NIH*
- **Report from Breakout session** "Implementing Mechanism of Action globally"

Panel discussion
Louis Stancato, *Eli Lilly and Company*
Malcolm Smith, *NIH*
Elly Barry, *Day One Biopharmaceuticals*
E. Anders Kolb, *Nemours Children's Hospital*
Peter Adamson, *Sanofi*
Brenda Weigel, *University of Minnesota*
Angelika Joos, *Merck/EFPIA*
Patricia Blanc, *Imagine4Margo*

14h30 – 15h00 Coffee break

15h00 – 16h00 Session VII - Paediatric Strategy Forums: what next?

Chair: Vickie Buenger, *Coalition Against Childhood Cancer - CAC2*

- **Analysis of the impact of Paediatric Strategy Forums**
  Andy Pearson, *ACCELERATE*
- **Report from Breakout session** "Pediatric Strategy Forum 2.0"
- **Voting the topics of the next Forums**

16h30 – 16h45 Wrap-up and 2023 Work Plan

16h45 – 17h00 Conclusions and end of Conference

Breakout session pitches

BKS 1 – Paediatric Patient-Reported Outcomes (PROs) – can we make patient-centred research a reality?

Patient-reported outcomes (PROs) are the gold standard to assess the patients' subjective health status. While both the Food and Drug Administration and European Medicines Agency recommend the use of PROs as endpoints in paediatric clinical trials to support claims for medical product labelling, PRO assessment is extremely rare in paediatric oncology clinical trials. In fact, only 8.2% of childhood cancer trials conducted between 2007 and 2020 used PROs as endpoints, and only 0.6% as the primary endpoint (Riedl, EJC 2021).

Questions

- What are the hurdles to conducting patient-centred research in paediatric oncology? What is the role of PROs measures?
- What are the available tools and ongoing initiatives globally to address PROs in paediatric oncology?
- How can we facilitate implementation of PROs in paediatric trials to guarantee patient-centred research and treatments?

BKS 2 – Paediatric Strategy Forums 2.0

The tenth Paediatric Strategy Forum was held in 2022. In view this it is timely to review the objectives and format of the Forums. They were established to prioritise medicinal products in a landscape of mechanism of-action-driven drug development, where the large number of drug products available for adults exceed the small size of the eligible population of children. They have achieved this goal and some products have been prioritised and others given lower priority. Furthermore, the Forums have made conclusions generally about criteria on prioritisation and helped to frame future discussions between industry and regulators and catalysed the development of platform trials. Living prioritisation was first introduced with the second Forum on anaplastic lymphoma kinase inhibition and second meetings will be held on multi-targeted kinase inhibitors in bone sarcomas and menin inhibitors.

Questions

- How can Paediatric Strategy Forums continue to fulfil unmet needs in paediatric oncology?
- Should the scope of Paediatric Strategy Forums be broadened?
- How can Paediatric Strategy Forums be sustained

Breakout session pitches

BKS 3 – Implementing Mechanism of Action globally

In the US, the Research to Accelerate Cures and Equity (RACE) for Children Act (2020), has implemented a Mechanism of Action (MoA)-based approach, to cancer drug development for children with cancer. This requires paediatric evaluation (submission of paediatric study plan, PSP) of new molecularly targeted drugs and biologics intended for the treatment of adult cancers and directed at a molecular target substantially relevant to the growth or progression of a paediatric cancer.

The ongoing process to propose revisions of both the EU Paediatric and Orphan regulations has the objective to make these regulations and drug development for children and rare diseases more centred on patients' unmet needs. Furthermore, it is envisioned and hoped that these regulations will move to being driven by the MoA, rather than the adult indication.

Questions

- What can be learnt from the implementation of RACE?Has the implementation of mandatory MoA-driven PSPs changed the landscape for paediatric oncology research so far?
- How can we develop high-quality MoA-informed paediatric drug development programmes?
- How can the implementation of MoA be harmonised in Europe, the US, Canada, Australia and Japan?

Register today!