Final Programme

Day 1    Thursday 4 February
3:00pm-7:30pm CET / 2:00pm-6:30pm GMT / 9:00am-1:30pm EST / 6:00am-10:30am PST /
11:pm-3:30am JST / 1:00am-5:30am AEDT

3:00pm – 3:15pm  1. Welcome

3:00pm – 3:15pm  Welcome and introduction to the Conference
Gilles Vassal, ACCELERATE Chair

3:15pm-4:30pm  2. Access to medicines outside clinical trials in the paediatric precision oncology landscape and capturing data

3:15pm – 3:30pm  International precision oncology landscape and the needs of access to medicine
Julia Glade-Bender, Memorial Sloan Kettering Cancer Center

3:30pm – 3:35pm  International legal framework for compassionate and off-label use
Michael Vranken, ACCELERATE

3:35pm – 3:40pm  Single-patient protocol (SPS)
Jim Whitlock, C17 Council on Childhood Cancer and Blood Disorders

3:40pm – 3:45pm  Securing Access to Innovative Medicines – the SACHA Project
Pablo Berlanga, Institut Gustave Roussy

3:45pm – 4:30pm  Panel discussion and Q&A
Julia Glade-Bender, MSKCC
Jim Whitlock, C17 Council
Pablo Berlanga, Gustave Roussy
Donna Ludwinski, Solving Kids’ Cancer
Dominik Karres, European Medicines Agency
Helen Kellar-Wood - Bristol-Myers Squibb
4:30pm – 4:45pm  Break

4:45pm – 5:30pm  3. Recent developments in the regulatory environment in EU and US
Chairs: Pam Kearns, SIOP Europe President, University of Birmingham
       Alberto Pappo, ODAC Paediatric sub-committee Chair, St. Jude

4:45pm – 4:55pm  Perspectives for the European Regulatory Environment
Fabio D’Atri, European Commission

4:55pm – 5:05pm  Implementation of the Race for Children ACT
Greg Reaman, Food and Drug Administration

5:05pm – 5:30pm  Discussion and Q&A

5:30pm – 5:45pm  Break

5:45pm – 7:30pm  4. Parallel Breakout sessions

BkS 1. How to facilitate pediatric development of medicines that are terminated in adult cancer development?

Co-Chairs Group A
Peter Adamson, Sanofi
Davy Chiodin, DayOne

Co-Chairs Group B
Delphine Heenen, KickCancer
Hubert Caron, Roche

Rationale. The development of many anticancer drugs in adults has been terminated for various reasons. Some of these drugs may target molecularly relevant alterations in paediatric malignancies. There are major hurdles for the paediatric development of these shelved-drugs. What are the hurdles and the solutions to this problem?

BkS 2. Tissue-agnostic evaluation of compounds in rare sub-groups

Co-Chairs Group A
Steven DuBois, Dana-Farber
Michael Cox, DayOne

Co-Chairs Group B
Lynley Marshall, The Royal Marsden
Greg Reaman, FDA

Rationale. What are the lessons learnt for recently approved medicines for agnostic indication? Can we propose the optimal, efficient, future approach agreed by stakeholders for the agnostic evaluation of innovative drugs for children and adolescents?

BkS 3. RACE for children act – early impressions and what are the best metrics to measure its success

Co-Chairs Group A
Susan Weiner, Children’s Cause
E. Anders Kolb, Nemours Al duPont Hospital for Children

Co-Chairs Group B
Cormac Owens, Children’s Health Ireland at Crumlin
Elly Barry, Pfizer

Rationale. RACE for children act has been enacted in August 2020. What are the first impressions and experiences?
**BkS 4. Incentives for drug development including first-in-child development and small biotech companies**

Co-Chairs Group A

Beth Fox, St. Jude  
Raphaël Rousseau, Gritstone Oncology

Co-Chairs Group B

Nick Bird, Solving Kids' Cancer UK  
Jeffrey Skolnik, Inovio Pharmaceuticals

**Rationale.** Major therapeutic advances have mostly failed to materialise for diseases that are unique to children or where adult development cannot address the needs of children. What is best way to incentivise ‘First-in-Child’ development and marketed authorisation of medicines for the treatment of children with life-threatening and debilitating rare diseases, such as paediatric cancers?

**BkS 5. How to prioritise developments in specific areas of pressing unmet pediatric needs?**

Co-Chairs Group A

Lia Gore, Children's Hospital Colorado  
Nicole Scobie, Zoé4life

Co-Chairs Group B

Leona Knox, Solving Kids' Cancer UK  
Dominik Karres, EMA

**Rationale.** A future revision of the EU paediatric Regulation may consider having specific rewards/incentives to direct the development of medicines in specific areas where there is a pressing need for children. How should these areas or products which would be eligible to such system be identified?

7:30pm  
End Day 1
Day 2  
Friday 5 February  
3:00pm-7:30pm CET / 2:00pm-6:30pm GMT / 9:00am-1:30pm EST / 6:00am-10:30am PST / 11:00pm-3:30am JST / 1:00am-5:30am AEDT

3:00pm – 3:05pm  5. Welcome
Welcome and introduction to the second day
Gilles Vassal, ACCELERATE Chair

3:05pm – 4:55pm  6. ACCELERATE at 360°
Chairs: Vickie Buenger, Coalition Against Childhood Cancer  
Carmelo Rizzari, SIOP Europe President-Elect, University of Milano-Bicocca

3:05pm – 3:25pm The ACCELERATE Paediatric Strategy forum initiative and further development
Andy Pearson, ACCELERATE

3:05pm – 3:25pm The International Collaboration initiative
Greg Reaman, Food and Drug Administration  
Nicole Scobie, Zoe4Life  
Teresa de Rojas, ACCELERATE

3:25pm – 3:40pm Long-term Follow-up (LTFU) platform project
Danielle Horton Taylor, PanCare & PORT  
Mark Kieran, Bristol-Myers Squibb

3:55pm – 4:10pm Break

4:10pm – 4:25pm The Fit for Filing project
Pamela Kearns, University of Birmingham  
Elly Barry, Pfizer

4:25pm – 4:40pm The Fostering Age Inclusive Research (FAIR) Trials project
Nathalie Gaspar, Gustave Roussy  
Chris Copland, University of York

4:40pm – 4:55pm Real World Evidence
Lynley Marshall, The Royal Marsden NHS Foundation Trust

4:55pm – 5:10pm Break

5:10pm – 7:10pm  7. Breakout sessions follow-up of Day 1
5:10pm – 7:10pm Reporting of the main outcomes and open discussion

7:10pm – 7:25pm  8. Wrap-up and 2021 Annual Workplan
7:10pm – 7:25pm Lessons learnt during Conference and definition of 2021 priorities

7:25pm – 7:30pm Conclusions and end of Conference