Preliminary Programme

Day 1 – Thursday, 10 February 2022

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 – 3:15pm	**1. Welcome**
3:00pm – 3:15pm	**Welcome and major 2021 hallmarks/achievements** *Gilles Vassal, ACCELERATE Chair*
3:15pm – 3:45pm	**2. Scientific Breakthrough** **Chair:** *TBC*
3:15pm – 3:35pm	**Targeting fusion proteins in pediatric malignancies** Speaker TBC
3:35pm – 3:45pm	**Discussion and Q&A**
3:45pm – 4:45pm	**3. Updating the regulatory landscape** **Chairs:** TBC
3:45pm – 4:00pm	**More than 18 months o`f Race for Children ACT** Greg Reaman, *Food and Drug Administration*
4:00pm – 4:15pm	**Revision of the EU pediatric and orphan regulation** Speaker TBC
4:15pm – 4:30pm	**FDA and EMA cooperation and new initiatives** Dominik Karres, *European Medicines Agency*
4:30pm – 4:45pm	**The 'Innovation Sandbox' initiative in Canada** Speaker TBC

4:45pm – 5:00pm	***Break***
5:00pm – 6:00pm 5:00pm – 5:15pm 5:15pm – 5:30pm 5:30pm – 5:45pm 5:45pm – 6:00pm	**4. Disparities in accessing innovative medicines** **Chairs:** *TBC* **The ACCELERATE out of trial access initiative** Teresa de Rojas*, ACCELERATE* **Reducing inequalities in access to clinical trial in Europe** Pamela Kearns*, University of Birmingham* **Economic and racial disparities in the US** Speaker TBC **Discussion / Q&A**
6:00pm – 7:30pm	**5. Parallel Breakout sessions (details in the last page)** BkS 1. Optimising industry / Academia partnerships BkS 2. Accelerating the development of combination regimens in frontline therapy BkS 3. Innovation after a first pediatric regulatory approval BkS 4. When is a Randomized Clinical Trial not required for registration?
7:30pm	***End Day 1***

Day 2 – Friday, 11 February 2022

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	**6. Welcome** **Welcome and introduction to the day** Gilles Vassal, *ACCELERATE Chair*
3:05pm – 5:00pm	**7. ACCELERATE at 360°** **Chairs:** *TBC*
3:05pm – 3:30pm	**Pediatric Strategy Forums including survey and vote** Andy Pearson, *ACCELERATE*
3:30pm – 3:45pm	**The International Collaboration initiative - Survey** Teresa de Rojas, *ACCELERATE*
3:45pm – 4:00pm	**Fostering Age Inclusive Research (FAIR) Survey** Nathalie Gaspar, *Gustave Roussy* Chris Copland, *York University*
4:00pm – 4:15pm	**ACCELERATE educational programme, including Fit For Filing** Pamela Kearns, *University of Birmingham* Andrea Demadonna, *ACCELERATE*
4:15pm – 4:30pm	**ACCELERATE-ing patient and market-access** Speakers TBC
4:30pm – 5:00pm	**Advocacy in ACCELERATE** Speakers TBC

5:00pm – 5:15pm	***Break***
5:15pm – 6:50pm 5:10pm – 6:50pm	**8. Report from Breakout sessions Day 1** **Chair:** Gilles Vassal, *ACCELERATE* **Reporting of the main outcomes and open discussion**
6:50pm – 7:25pm 6:50pm – 7:25pm	**9. Wrap-up and 2022 Annual Workplan** **Lessons learnt during Conference and definition of priorities for 2022**
7:25pm – 7:30pm	***Conclusions and end of Conference***

Parallel Breakout sessions

BkS 1. Optimising Industry / Academia partnerships

The rapid availability of new drugs to cure pediatric malignancies still suffers from the lack of expertise and resources by academics in developing new drugs, whereas industry usually lacks the expertise in pediatric oncology in general, and in pediatric preclinical and clinical research capabilities in particular. Significant progresses have been made in the recent years. However, academia could benefit more from industry's know-how in developing and manufacturing drugs while industry could benefit more from academia's preclinical research and clinical trial network, resources and expertise.

This session will build on the results of the ACCELERATE Fit for Filing working group to discuss how to further improve industry/academia partnerships and seek for proposals.

1. Why optimizing industry/academia partnerships in the field of drug development for children and adolescents with cancer?
2. What could be the new/optimized partnership models of industry and academia working together?

BkS 2. Accelerating the development of combination regimens in frontline therapy

Curing more children and adolescents with cancer will be achieved by introducing new safe and effective anticancer agent(s), best in combination, in frontline therapy of patients with high-risk malignancies, a treatment that would be best biomarker- and risk driven.

1. What are the hurdles in the design of relevant combinations?
2. What are the current limitations in rapid clinical evaluation of combinations in the relapse and frontline setting?
3. What solutions could accelerate the development of combinations?

BkS 3. Innovation after a first pediatric regulatory approval

For many medicinal products, there is a need for continued pediatric development after a first pediatric approval. Indeed, Innovation to optimize the utility of new drugs and maximise their overall benefit for children may need to continue after a first approved indication. This poses significant problems especially for high-cost therapies, such as adoptive cell therapies (e.g., CD19 CAR T cells), and antibody-driven therapies (e.g., anti-GD2 directed therapy).

1. What are the barriers to post marketing investments dedicated to support line extension in pediatric oncology indications?
2. How can this essential continued development best be supported?
3. Should new regulatory requirements and/or incentives be proposed? If so, what would be appropriate?

BkS 4. When is a Randomized Clinical Trial not required for registration?

Randomised clinical trials are acknowledged globally to be the gold standard to generate evidence on efficacy of new therapies or therapeutic approaches. However, recently certain innovative drugs have been approved without randomised clinical trials under specific circumstances. This has been predominantly when there was an exceptionally rare population with outstanding activity of the new agent. In addition, there are ongoing initiatives to define how to introduce big data and real world data in the regulatory decision-making processes.

1. In the current landscape, what are the parameters to define the pediatric cancer indications for drugs to be licensed without evidence from randomized trials?
2. What are the pros and cons of this approach?
3. How can academia, industry, regulators, payers and patient advocates work together on this topic?