ACCELERATE 2023 Annual Conference

9-10 February 2023

Brussels Radisson Collection Hotel & virtual

Highlights of the upcoming edition

Day 1

1. Real World and/or Trial data - Where are we heading to?

Presentation and discussion of international research initiatives, including ACCELERATE Long Term Followup (LTFU) project.

2. ACCELERATE at 360°

Reporting on the core ACCELERATE work in 2022: Education, Intercontinental collaboration and Fit For Filing Working Groups, HTA initiative, and more.

3. Patient Advocates-led session

More information will follow.

4. Breakout sessions (run in separate on-site and virtual settings)

- BKS 1 – Paediatric Patient-Reported Outcomes (PROs) and Quality of Life (QoL) measures – can we make patient-centred research a reality?
- BKS 2 – Paediatric Strategy Forums 2.0
- BKS 3 – Implementing Mechanism of Action globally

Day 2

5. Update on regulatory landscape in Europe and USA

Presentation and discussion of regulatory initiatives in Europe and in the US.

6. Pre-clinical prioritisation - Which way to go?

Presentation of international initiatives for pre-clinical evaluation and report from Breakout session "Implementing Mechanism of Action (MoA) globally".

7. Paediatric Patient-Reported Outcomes and Quality of Life

Introductory talks and reporting from Breakout Session "Paediatric PROs and QoL measures – can we make patient-centred research a reality?"

8. Paediatric Strategy Forums - What next?

Analysis of the impact of Paediatric Strategy Forums and report from Breakout Session "Pediatric Strategy Forum 2.0".

Breakout session pitches

BKS 1 – Paediatric PROs and QOL measures – 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 and QOL measures?
- What are the available tools and ongoing initiatives globally to address QOL and 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!