



8th ACCELERATE PAEDIATRIC ONCOLOGY CONFERENCE

6-7 FEBRUARY 2020

BRUSSELS

Short report and Work Plan 2020

The **8th ACCELERATE Paediatric Oncology Conference** took place on 6-7 February 2020 in Brussels, Belgium.

Successfully co-organised by SIOP Europe and ITCC, the 8th edition of the ACCELERATE Paediatric Oncology Conference was attended by almost 200 participants from 18 countries worldwide.

The participants included experts from academia, industry, patient/parent/survivor advocacy groups, foundations and regulatory authorities interested in accelerating new oncology drug development for children and adolescents. The diverse and high turnout, together with the organisation of the first Paediatric Strategy Forum in the US clearly showed that ACCELERATE has become a truly 'international' organisation. This is a key development in our effort towards accelerating the process of evaluating innovative therapies and introducing them in standard cancer care in children and adolescents.

This year's edition focused on a number of important topics such as:

- New regulatory environment with a focus on the implementation of the Race for Children act
- Building the assets for preclinical evaluation
- Latest developments in ACCELERATE: Multi-stakeholder Paediatric Strategy Forums, Fostering Age Inclusive Research (FAIR) trials, Fit-For-Filing academic trials, Long-Term Follow-Up (LTFU), international cooperation
- New initiatives and business models for accelerating paediatric oncology drug development



- Recent successes in paediatric oncology drug development

The ACCELERATE Chair Professor Gilles Vassal (ACCELERATE Chair) kicked off the event with an introduction highlighting the goals, projects and updates on the main work carried out in 2019.

The second session on the ACCELERATE Paediatric Strategy Forum initiative and its development was co-chaired by Elizabeth Fox (St Jude Children's Research Hospital) and Greg Reaman (FDA). After an introduction on the initiative made by Prof. Andy Pearson, the session covered two themes which were discussed during the third and fourth Forum (AML and B-Cell malignancies) and presented a number of initiatives already in place such as the PedAL/EUPAL consortium and the International ITCC European intergroup for Childhood NHL COG platform trial. Successively, participants were asked to provide via live polling their preferences for the topic of the 6th Forum planned in November 2020 at EMA premises in Amsterdam.

The third session was conceived in the form of a lecture and Prof. Michela Casanova from Fondazione IRCCS Istituto Nazionale dei Tumori presented lessons learnt and next steps in the field of NTRK inhibitors.

The fourth session, co-chaired by Patricia Blanc (Imagine for Margo) and Ruth Ladenstein (Children Cancer's Research Institute), reported on the activities of the four ACCELERATE Working Groups that were set up to speed up drug development in paediatric oncology. These working groups are: Fostering Age Inclusive Research (FAIR), Fit For Filing, Long-Term Follow-Up and International Collaboration.

The fifth session, co-chaired by Brenda Weigel (University of Minnesota) and Delphine Heenen (KickCancer), was dedicated to new and innovative initiatives to accelerate paediatric oncology drug development. Representatives of Day One Therapeutics, OncoHeroes and Y-mAbs were invited and interacted with the audience.

The sixth session looked to a more global perspective in paediatric oncology drug development and included presentations by the Children's Cancer Institute of Australia (Peter Wejborra) and Health Canada (Alysha Crocker).

The seventh session, co-chaired by Guillaume Bergthold (Roche) and Susanne Gatz (University of Birmingham), targeted the new regulatory landscape and included some relevant initiatives for ACCELERATE such as the Implementation of the Race for Children ACT in the US and the



evaluation process of the Orphan and Paediatric Regulation in Europe. The session also included two examples of assets to generate biological and preclinical data, ITCC-P4 and FNIH.

The last part of the event was dedicated to breakout sessions on four central topics ACCELERATE identified as key 2020:

1. Challenges in prioritisation – The way forward
2. Beyond randomized controlled trials: ‘non-conventional’ data sources for regulatory decision-making
3. Preclinical testing in order to benefit children
4. Implementing the Race for Children Act

All delegates gathered at the end of the conference to report on the main outcomes for each breakout session and a final discussion was held on the definition of the 2020 ACCELERATE Work Plan, available below:

Objective 1	Consolidate and expand ACCELERATE Paediatric Strategy Forums because they will be key to facilitate prioritisation in the new environment: set up impact assessment, follow up meetings (when required) and strategy update; reduce time to publication and enlarge dissemination.
Objective 2	Explore the relevance and feasibility of using data registries and real word data for evaluation of anticancer medicines for children: a pilot project will be proposed.
Objective 3	Make recommendations on metrics to monitor the impact of the RACE act.
Objective 4	Facilitate further the EMA/FDA collaboration in evaluating pediatric oncology development plans: make the current level of cooperation better known and advertise/ educate people about the current mechanisms; explore and pilot how concomitant and “interactive” evaluation by both agencies could be performed.
Objective 5	Engage with Health Technology Assessment bodies: initiate contacts, explore the topic taking into account what is ongoing more largely in pediatrics, generate data and prepare a session for the 2021 ACCELERATE annual conference.



Objective 6	Propose measures to facilitate and incentivize repositioning and specific paediatric anticancer drug development: the European Commission is about to release the results of the evaluation of the EU Paediatric and Orphan Regulations and is preparing a new Pharmaceutical Industry Strategy. which may lead to the opening of a current regulation window, with the option of modifying the current Paediatric regulation or proposing a new regulatory measure.
Regarding the Break-out session on Preclinical evaluation. More/better/together/scale up and Explore/better define role of patient advocates, ACCELERATE will not have specific actions but will monitor of the outcomes of initiatives such as FNIH in the US and ITCC-P4 project. One of the unsolved points refers to the lack of clarity of the role of patient advocates in such preclinical initiatives. ACCELERATE will not produce any concrete actions on this point.	

The 9th edition of the Annual Conference will take place on **4-5 February 2021** in Brussels.

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