The value of working together

No blame! No shame!
Generate data and propose solutions
• Implementation of 2018 action plan
• Summary of the 5 Breakout sessions held during the ACCELERATE conference
• 2019 Work Plan following the discussion held during the conference and agreed by the ACCELERATE Steering Committee
Implementation of 2018 action plan

Four Main objectives

✓ International collaboration for the implementation of the RACE4Children

• How to make the Orphan regulation work for children with cancer?

• Make proposals for new incentives

• Get HTAs on board

ACCELERATE has been reorganised as an international platform

Postponed until availability of the European Commission study results*

Postponed

So far unsuccessful

* As planned in the 10 year Pediatric Medicine Regulation Report
The five Breakout sessions

1. Further development of Paediatric Strategy Forums
   Andy Pearson, ACCELERATE
   Darshan Wariabharaj, Janssen Research & Development

2. How to implement Mechanism of Action and Biology-driven Developments of Oncology Drugs for Children and Adolescents in the new Regulatory Environment?
   Peter Adamson, Children Hospital of Philadelphia
   Patricia Blanc, Imagine for Margo

3. How to strengthen International Cooperation?
   Greg Reaman, Food and Drug Administration
   Nicole Scobie, Zoé4life

4. Readdressing the Needs for long-term Follow-up
   Mark Kieran, Bristol-Myers Squibb
   Danielle Horton Taylor, Unite2Cure

5. Designing and conducting Investigator-initiated Clinical Trials of new Drugs to meet the regulatory Requirements for Approval by Health Authorities
   Pam Kearns, University of Birmingham
   Elly Barry, Pfizer
BO1 – Future development of pediatric strategy forums

1. Develop forums alternatively in Europe and North America
2. Continue to improve the preparation and the meetings
3. Set up action plan with follow up for each forum in order to evaluate the impact of the forum and monitor its implementation
4. Define the process for deciding forum topics
5. Need for more forums

- **Next forum**
  in a class of compounds with no drugs yet approved in adults to discuss pediatric development earlier than previously – proposal BET inhibitors and epigenetic modifiers

- **Other potential forum topics**
  - Disease Focused: High Grade Glioma, Sarcomas
  - Target focused: DNA Repair, CSF1 inhibitors and other IO targets
1. Prioritize development on unmet needs, science and pre-clinical data
2. Decision on any compound should consider whether it belongs to a class of compounds with no drugs approved or with at least one drug marketed
3. Introduce flexibility and life cycle development
4. Need for better coordination between jurisdictions
5. A high-level meeting to define how to streamline and align developments
BO3 – How to strengthen International Cooperation?

WHY

- Childhood cancer is a global problem
- Small patient population(s) and getting smaller (biology, molecular subgroups)
- We all share some similar issues/problems but also have local differences
- Avoid duplication
- Advocacy → united voice is important
- To ACCELERATE!
- Widen access to treatment and information globally to save lives
- WHY NOT??

WHAT

- Lack of harmonization
- Need an entity on an international level
- Streamline and modify regulatory requirements – create the environment to harmonize?
- Be open to new and different approaches
- Focus on paediatric-specific discovery without losing our ability to leverage adult discovery
- Facilitate efficiency in innovation process
- Create and expand a pre-competitive environment wherever and whenever possible

HOW

- Think outside the box
- Get buy-in from key opinion leaders that this is a huge priority
- Learn from other disease types/models and industries
- The dream: create studies that satisfy multiple national regulatory requirements
- Define the players – identify influencers and roadblocks (legislators)
- As groups of advocates/parents/ funders DEMAND that this happens
- Ensure that stakeholders are transparent and specific about roadblocks so that advocates and campaigners have clear understanding of the issues
- Make patient level clinical and biological data available for free and open exploration – open source?
- Create systems to access publicly available data
- Capture compassionate use/n of 1 data? OR create platform trials to facilitate new drug development
- Create a position statement, white paper on importance of international collaboration
- Define priorities
- Regular communication – more opportunity for face-to-face meetings

WHO

- EVERYONE!
- Also outside of the paediatric/oncology community
- Reach out to ‘other groups’ like ethics communities, HTAs, payers, policy makers, funders (government, private groups, small charities)
- Connecting high income and low-middle income countries

RECOMMENDATION – create an ACCELERATE Working Group to deal with international collaboration which can meet outside of regular Accelerate meetings
**WHY?**

- Increasing number of survivors of targeted and immunotherapy
- Current clinical focus has been collection of acute and semi-acute toxicities
- Increasing requirement by the regulatory agencies to have a better understanding of the late effects of biologic therapies
- Needs of clinicians and families to be informed of the long-term effects of treatment in order to guide their decision making
- Opportunity to improve the survivorship care of patients with a more comprehensive understanding of late effects

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**An ACCELERATE Working Group** will be created in 2019

**Goal:** Create an international data repository with ACCELERATE to collect information on long-term health in children who have received new modalities of anticancer treatment to facilitate an open sustainable resource to allow for the evaluation and optimization of follow up care

**4 tasks:** Infrastructure / Data / Sustainability / Governance
Industry perspective: Advantages of Collaborating with Academic Groups and Utilizing Academic Study Data

- Collaboration with experts in the disease
  - Advance medicine/patient care in a meaningful way
  - Vast experience in pediatric oncology clinical research
- Access to networks of centers
  - Faster recruitment
  - Utilize existing research infrastructure
- Collaborators provide an informed perspective helpful in regulatory interactions
- Cost

Industry perspective: Challenges with Utilizing Academic Study Data

- Study design. Research objectives may not always align (e.g. endpoints, comparator arms)
- Timing. Sequencing of studies relative to regulatory submission timelines
- Less control of study time lines
- Operational/documentation challenges
  - Particularly if the use of data for regulatory submissions was not foreseen prospectively
  - May be seen as burdensome by academic centers/cooperative groups
- Operational:
  - Independent confirmation of tumor response, duration of response
  - Monitoring of data – how much?
  - Database specs (ICH part 11 compliance, audit trail)
  - Dataset transfer
- Documentation to support regulatory dossier: ICH-compliant CSR

RECOMMENDATION – create an ACCELERATE Working Group
Workplan 2019
(agreed by the Steering Committee on April 29, 2019)

1. Set up the 5th Pediatric Strategy Forum in the US and implement the BO recommendations
2. Develop the agenda of the FAIR working group and create 2 new working groups (see after)
3. Define a plan to implement BO recommendations on international cooperation
4. Explore the field of HTA evaluation across specialties and the ongoing initiatives to make a proposal at the next ACCELERATE meeting
5. Define a communication strategy
FAIR Working Group – Nathalie Gaspar and Chris Copland
Objectives: Develop the survey, implement the tool kit, new webpage, monitor implementation

LFTU Working Group – Mark Kieran and Daniele Horton
Objective: Create an international data repository

Fit For Filing Working Group – Elly Barry and Pam Kearns
Objective: Develop best principles on how to design and deliver a trial with a dataset that can be included in a package for filing
8th ACCELERATE Paediatric Oncology Conference
6-7 February 2020 | Brussels, Belgium

Save the Date!

www.accelerate-platform.eu