ACCELERATE MULTISTAKEHOLDER PLATFORM

6TH ACCELERATE Paediatric Oncology Conference

Brussels, Belgium
8 - 9 February 2018

Wrap Up & WorkPlan
No blame! No shame!
Generate data and propose solutions
This Document contains:

• Summary of the break out sessions held during the ACCELERATE conference
• Work Plan in the form of Working Groups as agreed during the wrap up session of the conference
Break out sessions:

How to accelerate paediatric oncology new drug development in the new regulatory environment?

4 groups, same question
Group 1

• In the blue sky: a global innovation consortium that would cover the topic down to access to medicines

• Facilitate repurposing

• Install FAIR trials as a way of life, including in-trial adaptation

• Overarching thinking to elevate dialog beyond PIPs

• Get ethics committees and competent authorities on board of the platform

• More science to drive drug development
Group 2

- Expand precision medicine
- Prioritize targets and combinations
- Facilitate communication and multi-stakeholder collaboration
- Set up and communicate on follow up of the Pediatric Strategy forums
- Incentivize biootech companies
- Better connection between Pediatric and Orphan regulations
Group 3

• A meeting to Agree on the Problem
• Leverage Adolescent participation which does not substitute to pediatric development
• Guidance on initiation of pediatric phase 1/2 investigation before submitting a PIP
• Better incentives because currently mis-aligned with the goals
• Explore the Buy-Out model*

Making Better Drugs for Children with Cancer
http://www.nap.edu/catalog/11259.html

*Needle M, Ped Blood Cancer 2012, 59:3
Group 4

• Move from a competitive to a cooperative system
• Set up Innovative designs
• Improved incentives
• Facilitate access to trials
• Better coordination on targets between academia and industry
WORK PLAN 2018

Agreed on February 9th, 2018
Four Main objectives

- International collaboration for the implementation of the RACE4Children act
- How to make the Orphan regulation work for children with cancer?
- Make proposals for new incentives
- Get Ethic Committees, Competent Authorities and HTAs on board
Working Groups

• WG1: Development of Pediatric Strategy Forums
• WG2: Fostering Age Inclusive Research
• WG4: New models for paediatric oncology drug development
• New working Groups
  ▪ Incentives
  ▪ Orphan Drugs
• WG3: Long term Follow up (suspended in 2016)
WG1 : Development of Pediatric Strategy Forums*

• Forum 3 : Immune checkpoints in combination Septembre 5 & 6, 2018
• Forum 4 : acute myeloïd leukemia (to be planned)
• Call for expression of interest on EMA and ACCELERATE websites
• Publish forums in peer-review journals
• Set up and communicate on follow up of forums

*implemented by ACCELERATE and EMA
WG2 : Fostering Age Inclusive Research FAIR trials

• Survey issues with opening adult trials to adolescents in European countries and monitor
• Develop tool kit for sponsors
• Inform ethic committees and competent authorities
• Expand communication and awareness

https://doi.org/10.1093/annonc/mdy002
WG4 : New models for pediatric oncology drug development

• Proposal - The Accelerator a program to develop new paediatric oncology drugs - a dedicated, integrated paediatric platform, company beyond current isolated initiatives

• Next steps
  ▪ Describe Accelerator in a white paper
  ▪ Identify a drug as a proof-of-concept-test
New WG : New incentives

• Work was done in ACCELERATE in 2015 – 2016 and drafted as an article

• Now the EU Commission report is released

  « further evaluation will aim at providing results by 2019 so to allow the next Commission to take informed decision about possible policy options”

• ACCELERATE will make proposals to better incentivize specific pediatric drug development, repurposing and accelerated developments
New WG: How to make Orphan regulation work for children with cancer?

- The EU Commission will look at the combined effects of the Orphan and Paediatric Regulation (PMR report)
- Survey of pediatric cancers in the Orphan regulation is done and available in: *Orphan Drug Regulation: A missed opportunity for children and adolescents with cancer. Eur J Cancer 2017, 84: 149-158*
- ACCELERATE will contribute to the EU Commission initiative
- ACCELERATE will identify the reasons why Industry does not use the Orphan regulation to develop their anticancer drugs in children
WG3 : Long-term follow up measures

• From 2013 to 2016, WG3 prepared a white paper to make recommendations to address long term follow up of children and adolescents receiving new medicines

• The creation of a public-private initiative to warrant adequate and high quality follow up was not considered as a priority since most PIPs asked for a 5 years follow up only

• The situation may change, in particular with CART cell therapy and immune therapies with a longer follow up needed

• ACCELERATE steering committee will discussed the need to reactivate WG3
7 th ACCELERATE Conference

February 2019

www.accelerate-platform.eu