



ACCELERATE MULTISTAKEHOLDER PLATFORM

6TH ACCELERATE Paediatric Oncology Conference

Brussels, Belgium
8 - 9 February 2018



**WRAP UP
&
WORKPLAN**



The value of working together



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



ACCELERATE Multistakeholder Platform

6th ACCELERATE Paediatric Oncology Conference

08-09 February 2018
Brussels, BE

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 biotech 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

Joint Adolescent - Adult Early Phase Clinical Trials to Improve
Access to New Drugs for Adolescents with Cancer Proposals from
the Multi-stakeholder Platform - ACCELERATE 

<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

