



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/EC multi-stakeholder workshop to further improve the implementation of the PMR

20 March 2018

- *Which ongoing initiatives to identify paediatric medical needs are you working on or aware of?*
- *Which criteria and methodology would you suggest to prioritise diseases/conditions of unmet paediatric needs?*



Initiatives to identify paediatric medical needs



Unite2Cure : European network of parents and patient representatives united for better cures for childhood cancer



Determine needs of children with cancer based on own experience



Raise public awareness & advocate on national and EU levels about unmet needs
(campaigning, open letter, public consultations, ...)



Open and transparent dialogue with all stakeholders (Accelerate Platform, Paediatric Strategy Forums, EMA workshop, ...)

Goals: better treatments for children & young people with cancer and swifter access to innovation

Urgent needs for children with cancer

IMPROVE THE CURE RATE

High risk cases:
cure rate below 50%

IMPROVE SURVIVORS' quality of life

Severe long-term side
affecting **2/3 survivors**
50% impacting daily life

- 6.000 deaths / year in Europe
- 1st cause of death by disease
- Cure rate stalled for last 15 y
- 2011-2015: 70 new anti-cancer adult drugs vs. only 2 for children
- PMR and ODR not working for childhood cancer



CALL FOR CHANGE! NOW!

Criteria and methodology to prioritise diseases/conditions of unmet needs?



- Continue public awareness
Call of parents and patients organizations in Europe on the urgent need to access innovation
- Paediatric Strategy Forums
- Breaking 18 years dogma
- Speed-up molecular understanding of paediatric cancers
- FDA and EMA alignment on regulation (RACE for children Act on MoA and list of unmet needs)
- Improved implementation of PMR: earlier submission PIPs (Recital 10, Article 16)
- Evaluate impact of the class waiver list review (applicable mid 2018)

**Children should not have to wait
to access life-saving drugs!**