

EMA/EC multi-stakeholder workshop to further improve the implementation of the paediatric regulation 20 March 2018



- Which ongoing initiatives to identify paediatric medical needs are you working on or aware of?

PEDIATRIC STRATEGY FORUMS

jointly implemented by ACCELERATE and EMA

- Which criteria and methodology would you suggest to prioritise diseases/conditions of unmet paediatric needs?

**Gilles Vassal,
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Pediatric Strategy Forum

Goals

- Share information and advance learning, in a pre-competitive setting, to inform paediatric drug development strategies and subsequent decisions
- Define the needs
- Facilitate prioritization

Principle

- dialogue and constructive interactions between ALL relevant stakeholders :
- clinicians, academics, patient representatives, pharmaceutical companies and regulators

Content

- Biology of disease
- Therapeutic needs including epidemiology, clinical features, standard therapy current needs and future therapeutic plans – Europe and North America
- Non-clinical and clinical data on compounds

Setting

- 6 months preparation including TCs with speakers and pharma
- 2 days meeting at EMA
- On invitation following expression of interest (pharma, academia, parents)

Output

- Summary on websites
- Article in a peer-reviewed journal

Proof of concept

- Forum n°1: ALK Inhibition in Paediatric Malignancies Pilot Forum 30 & 31 January 2017
- Forum n°2 : mature B-cell malignancies in children 13 & 14 November 2017

Paediatric Strategy Forum: Mature B cell malignancies in children

13 & 14 November 2017

Participants

- 73 Participants
- European and North American academic experts
- Patient representatives from Unite2Cure (Europe) and Children's Cause for Cancer Advocacy (US)
- Regulators from EU national competent authorities, EMA & US FDA; and PDCO members
- 15 Pharmaceutical companies
- 20 Medicinal products discussed

- Highly effective current therapy for high-risk mature B cell malignancies with EFS~ 95% and no salvage therapy in relapse
- Acute toxicity – significant, but most survivors - no or mild long term toxicity

Unmet therapeutic needs

- i) develop innovative treatments for patients remaining incurable
- ii) reduce high acute toxicity of current therapy

Conclusion

- Most malignancies in children differ from those in adults
- Successful de-escalation at low risk in front line therapy can only be undertaken with an effective salvage regimen
- Priority = developing treatment for relapse
 - Very small number of patients = global strategy
 - Combination approach rather than monotherapy
 - Correlative biology studies should be integrated

Consensus of clinicians on priorities

- Antibody drug conjugates
- CAR-T cells (not products for initial use but only for consolidation)
- T-cell Engagers

Pediatric Strategy Forum (next)

- **Third Forum** - Checkpoint inhibitors used in combination
5 & 6 September 2018 at the EMA
 - **Fourth Forum** - Acute Myeloid Leukaemia
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- **Expand beyond pediatric oncology**

Which Diseases?

Based on the EC and EMA activity reports:

- Diseases with many drugs and PIPs that do not deliver
- Disease with very few or no medicine in development

DO not duplicate the 2007 – 2009 Priority List process

The ACCELERATE-EMA Paediatric Strategy Forums

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