

The sectors' asks - what
role for pharma?



4 changes to the PMR

#1: Mandatory paediatric investigation of drugs based on 'mechanism of action' rather than adult disease

#2: Prioritisation of drugs in order to match the best available therapies to children with rare or refractory malignancies

#3: Reduction in the delays in starting paediatric development of potentially life-saving innovative drugs

#4: More effective and flexible rewards to better incentivise the development of new and specific paediatric medicines

#1: Mandatory paediatric investigation of drugs based on 'mechanism of action' rather than adult disease

* Is there a paediatric cancer that could benefit from this drug?

* Regardless of its intended indication for an adult cancer

If the answer is YES

Development plan for the drug must include the investigation of the potential benefit in paediatric cancers

Paediatric cancer drug development not dependent on the adult cancer market
but dependent on how the drug works

#2: Prioritisation of drugs in order to match the best available therapies to children with rare or refractory malignancies

- * **Nobody wins** if drugs are developed in competition for a small number of patients
- * Pharmaceutical companies need **to co-operate** with each other and with disease experts **to prioritise** which drugs are developed

Paediatric cancer drug development needs pre-competitive dialogue between companies

#3: Reduction in the delays in starting paediatric development of potentially life-saving innovative drugs

- * Procrastination does not save lives
- * Studies in paediatric cancer patients must not depend on establishing the drug has a market for an adult cancer

Paediatric cancer drug development needs to become independent from a successful adult cancer drug development

#4: More effective and flexible rewards to better incentivise the development of new and specific paediatric medicines

- * Research and development costs need timely and proportionate reward
- * Award a proportion of the reward at an earlier time point based on delivery of set milestones in the Paediatric Investigation Plan
- * Develop a **new incentive** that rewards development of a drug for specific to a paediatric cancer indication

paediatric cancer drug development needs effective incentives to drive progress



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