Working Group 2

New incentives for specific paediatric drug development and drug repositioning

Led by Pam Kearns and Patricia Blanc

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Executive Summary:

Greater investment is needed in the development of innovative therapies to improve the treatments available to children and adolescents with cancer. In spite of the explosion in therapeutic innovation within the oncology arena, the benefits to the paediatric population are limited and have not impacted on cancer’s premier position as the leading cause of death from disease in childhood.

The current European legislative environment, including the Paediatric Regulation and Orphan Drug Designation, aims to promote drug development for paediatrics and for rare diseases, however children and adolescents with cancer have not derived the expected benefit from these important initiatives. Cancer drug development programmes remain inextricably linked to the market potential for adult cancer indications, with independent paediatric cancer drug development remaining commercially unviable. Incentives are needed to motivate paediatric cancer drugs development uncoupled from adult cancer indications and that proportionately reward the investment in paediatric specific drug development.

We propose pragmatic changes in the timing (segmented reward approach) and nature (for example; a transferable exclusivity voucher) of the rewards associated with delivery of a Paediatric Investigation Plan (PIP).

A critical gap in the current framework is the lack of sufficient incentive to develop a medical product exclusively for a paediatric cancer, due to the lack of marketing potential. We propose that a variant on the US Creating Hope ACT that is more aligned to the current European legislations could drive this type of drug development.

We also seek a collaborative approach to PIPs with industry forming partnerships in the early stages of development of new classes of drugs with potential applications on childhood cancers, facilitated by EMA and clinical academia.
Together, these incentives could substantially increase interest from both big industry and small SMEs to invest in developing innovative therapies for the rare paediatric diseases, to the significant benefit of children and adolescents with cancer.

ACCELERATE publication and Key papers: