Table

Extract from G. Vassal et al., 'Creating a unique, multi-stakeholder Paediatric Oncology Platform to improve drug development for children and adolescents with cancer', European Journal of Cancer, Volume 51 , Issue 2 , 218 - 224

Improving early access to new anticancer drugs for children and adolescents in Europe

1. Improve access to compounds for preclinical testing and biological studies
2. Increase the number of drugs in early phase trials significantly
3. Consider running early phase trials before submitting a Paediatric Investigation Plan (PIP)
4. Consider accrual of adolescents in adult phase 1 and 2 trials when scientifically and medically relevant
5. Emulate the National Cancer Institute (NCI) clinical trial funding and programming model in Europe
6. Work to de-risk the perception of paediatric studies and create value in paediatric oncology
7. Simplify the process for initiation of a PIP proposal and enforce academic participation in this

Prioritising oncology compounds for development in children/adolescents with cancer

1. Develop a strategy for selection and prioritisation of drugs for paediatric development based on biology and mechanism of action rather than the current process based on adult cancer indications
2. Increase our understanding of molecular pathways and key drivers which are relevant for paediatric tumours
3. Set up disease focus groups: academic tumour groups must identify key contacts, review existing data on tumour biology and preclinical work, and define a strategy for each disease based on current treatment options
4. Consider paediatric oncology drug development as pre-competitive research
5. Set up early cross-portfolio evaluation, including academic investigators and paediatric oncology networks
6. Implement cross-pharmaceutical company discussion that will facilitate drug selection and prioritisation
7. Develop multi-compound, multi-company trials to speed up evaluation and spread risk and cost
8. Set up better incentives tailored to risks taken and commitments made by pharmaceutical companies, as well as for development of specific paediatric drugs

Facilitating cooperation and collaboration between all stakeholders

1. Achieve better academia-industry communication with improved trust and confidence. Academic groups to identify global leaders who will link with industry and have global harmonised opinions

2. Encourage four-party discussions and drug-prioritisation meetings

3. Broaden collaborative links between clinicians, scientists, European Medicines Agency (EMA)/Paediatric Committee (PDCO) and parent/patient organisations: the Cancer Drug Development Forum (CDDF)-Innovative Therapy for Children with Cancer Consortium (ITCC)-European Network for Cancer Research in Children and Adolescents (ENCCA)- European Society for Paediatric Oncology (SIOPE) Paediatric Oncology Platform

4. Set up an annual international working meeting with all stakeholders to update and share, address issues, propose solutions and elaborate action plans

5. Link with initiatives in North America and worldwide

6. Include the European Commission

7. Prepare the proposals for revision of the Paediatric Regulation in 2017

Setting-up long-term follow-up (LTFU) of children and adolescents exposed to new drugs

1. Set up LTFU that is patient-centred and performed in academic centres

2. Define LTFU so that data can be shared with regulatory authorities for continuous monitoring of benefit-risk

3. Build a joint programme and partnership between academia and industry

4. Use the concept of Survivorship Passport and empower survivors as partners of LTFU research

5. Implement LTFU on extension studies, with post-marketing surveillance and risk management plans

6. Perform large, joint – academic and industry – randomised trials and transfer LTFU to sustainable academic platforms

7. Consider cross-pharmaceutical company initiatives