8th Workshop
Eurordis Round Table of Companies

"Impact of the EU Paediatric Regulation on Orphan Drug Development"

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Concept Paper

The European Regulation on Medicinal Products for Paediatric Use (Regulation (EC) No 1901/2006), which entered into force on 26 January 2007, was adopted with the clear intention to remedy the inequitable situation faced by children of the EU when they need a prescription for medications.

Prior to this Regulation, it was estimated that 90% of existing medicines were prescribed to children in the absence of paediatric-use studies. When treating their paediatric patients, doctors had no alternative to prescribing unlicensed or off-label medicines, thus risking inefficacy and/or immediate or long-term adverse reactions. Children living with rare diseases are particularly exposed to such risks. It is for this reason that Eurordis has played a frontline advocacy role, over the last four years, in getting this new EU Regulation adopted.

Today, clear and specific responsibilities must be taken on by the different actors involved in development of new medicinal products with a potential paediatric indication:

- The pharmaceutical industry when developing a new drug has to make a clear decision whether or not to develop a paediatric form of the product,
- The regulators when assessing Paediatric Investigation Plans, deciding on waivers and deferrals or evaluating clinical development results,

- The health professionals when designing specifically adapted clinical studies for paediatric drugs and when making any decisions concerning the involvement of children in clinical trials,
- The families when deciding on whether or not to let their children participate in a clinical trial.

Ten percent (10%) of designated orphan medicines are for conditions exclusively affecting children. Forty-five percent (45%) of designated orphan medicines are developed for conditions potentially affecting both children and adults. To stimulate timely paediatric use clinical studies for concerned orphan drugs, Eurordis has advocated for an additional 2-year market exclusivity.

One year after the adoption of the Paediatric Regulation, the 8th Eurordis Round Table of Companies Workshop will be the occasion to hear about the first lessons learnt since its implementation and to assess the impact that the new provisions may have on orphan drug development.

The morning session of this meeting will provide attendees with an overview of:

- the experience of the new Paediatric Committee (PDCO) at the EMEA,
- how the Agency has integrated the work of this committee into the activities of the previously existing EMEA Committees for Human Medicines (COMP, CHMP) and
- the work programme of the PDCO for the remaining years of its first mandate.

A member of the Office for Orphan Product Development (OOPD) of the FDA, with former experience at the Paediatric Office, will report on the 7-year experience since the adoption of the "Best Pharmaceuticals for Children Act" in the US and on its potential impact on the development of Orphan Drugs.

More than 300 dossiers have already been processed by the PDCO in the last 8 months. Two pharmaceutical companies that have experienced the new paediatric procedures for an orphan product will conclude the morning session of the workshop and offer a critical evaluation of the new legislation from the "users" point of view.

The Eurordis' candidate to the PDCO as patient representative will open the discussion on the expectations of parents regarding paediatric drugs, and what role patients can play in such a scientific committee which, more than all other existing committees, will have to face very sensitive ethical issues in the framework of its activities.
Ethical issues that are also shared by those who in their everyday work have to evaluate the risk and the benefit of involving their young patients in clinical trials, as well as explain to parents what these risks are and what benefits could be realistically be expected when their child is involved in a trial.
The "Industry Experience in Partnering with Patient Groups for International Paediatric Clinical Trials" will be the starting point for a discussion session on how all stakeholders involved can contribute to facilitating the development of paediatric drugs in particular in orphan indications.

As with all ERTC Workshops, the final discussion will be dedicated to exploring how to facilitate the development of paediatric drugs in orphan indications, where we face the additional burden of limited knowledge of the disease and the small number of patients.