Farmacologia pediatrica: implementazione e ruolo alla luce del regolamento pediatrico

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Regulating paediatric research means searching for the balance between two goals: protecting children while making sure they benefit from safe and efficient medicines. To this aim different legal instruments were adopted in the EU: Directive 2001/20/EC, the Paediatric Regulation, ICH Topic E11 and the EU Ethical Recommendations (EC, 2008). Those instruments were closely working together, but the European framework surrounding clinical research is going to change with the release of the new EU clinical trials Regulation (EU) 536/2014 aimed at repealing Directive 2001/20/EC. One of the major challenge is to overcome the lack of harmonisation of trial procedures among countries, and hence its attractiveness especially for multinational trials. This Regulation was adopted in May 2014 and was supposed to enter into application by May 2016. Due to delay in the development of the portal and database, it will come into application in 2019.

How the new Regulation will affect paediatric research? Does it represent a step forward to better protect children and foster high quality research?

Analysing the new Regulation provisions, we have found that the involvement of minors in the informed consent procedure according their age and mental maturity, and the need for paediatric expertise or advice in Ethics Committees, as stated by the above mentioned EC Paediatric Recommendations, have been introduced. Interestingly, in the first draft of the Regulation released by the EC, these provisions were lacking. In this achievement, the efforts and the participation in the consultation process of paediatric networks, such as TEDDY and GRiP, resulted incisive.

Furthermore, some issues, such as the centralisation of trial assessment, a well-defined list of documents to be submitted and their contents, will favour the conduction of paediatric trials, that are generally multi-centre and multi-national.

However, many clarifications are still needed e.g. on the definition of “benefit/risk”, “minimal additional risk or burden”, “low-intervention trials” as well as to the role and functioning of Ethics Committees. These clarifications will be particularly important to guarantee transparency and legal certainty and to foster multinational ethically sounded paediatric trials. In fact, groups at EU level, such as at the European Network of Paediatric Research at the European Medicines Agency (Enpr-EMA), are working to provide pragmatic responses on these aspects.

In conclusion, a stronger rule, such as a Regulation, will harmonise and coordinate the clinical research in the EU, affecting positively the paediatric one and will make mandatory some important preconditions to start a paediatric trial. In this way, it is expected to keep up well-conducted paediatric trials and to harmonise practices. However, only the implementation of the new Regulation will learn us if it holds its promises and improve the quantity and quality of trials in children without bending the ethical principles. Unfortunately, the expected advantages for paediatric research will be delayed to 2019.