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Several initiatives in the USA* have created incentives and obligations for companies resulting in product information for over 100 drugs which now includes dosing, efficacy and safety information for children. Children in Europe, particularly those affected by rare diseases, will soon benefit similar conditions.

**Paediatric Rule of 1998 enjoined in 2002, Best Pharmaceuticals for Children Act of 2002, and Paediatric Research Equity Act of 2003*



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PAEDIATRIC DRUGS AND RARE DISEASES



Sources of information on paediatric drugs:
Eurordis's website and electronic monthly newsletter
www.eurordis.org
European Medicines Agency (EMA)
<http://emea.europa.eu>



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Rare Diseases Europe

PAEDIATRIC DRUGS AND RARE DISEASES

The EU Regulation on Paediatric Drugs was adopted by the European Parliament and the Council of Ministers in 2006, after many years of advocacy work by stakeholders (academia, industry and patient groups) with Eurordis at the forefront as early as December 2000. The Regulation was published in the Commission's Official Journal in December 2006 and its implementation started in 2007.

PAEDIATRIC DRUGS: THE FACTS

- Many medicines administered to children have not been developed for them (50 to 90% according to "Diseases and Countries in Europe")
- Medicines used are those for adults. They are administered to children by decreasing quantities based on weight, which is extremely hazardous
- Serious consequences, inefficacy and side effects often stem from incorrect dosage
- The use of unlicensed and off-label medicines is widespread among children affected by rare diseases
- Children are in a de facto situation of permanent and uncontrolled clinical trial, by taking drugs that have not been trialled for them
- The US regulation on paediatrics (Best Pharmaceuticals for Children Act) was only adopted in 2002
- There was no EU regulation on paediatrics until December 2006.



MAIN MEASURES OF THE EU REGULATION

- Obligation of paediatric research (PIP - Paediatric Investigation Plan) for every new drug developed for adults and having a potential use for children
- Creation of an inventory of specific needs for paediatric medicinal products
- Creation of a Paediatric Committee including patient representatives at the European Medicines Agency (EMA)
- 6 month extension of the patent for the paediatric formula of existing "still under-protection" adult medicines
- 2 year extension of market exclusivity for orphan drugs for children (12 years in total instead of 10 years for adult orphan drugs)
- Financial support via the EU Research Framework Programme for Research on old (off-patent) drugs to study and develop paediatric use
- Implementation of a process to avoid unnecessary clinical studies on children
- Specific label for products studied in children and authorised: the Paediatric Use Marketing Authorization (PUMA)