

## **MEDICINES FOR CHILDREN IN THE EUROPEAN UNION**

### **The patient perspective**

**By Y.S. Poortman**

#### **Seventy million children at risk**

There are seventy-five million children in the European Union all under the age of sixteen, who are at risk when taking medicines. With the enlargement of the EU this will soon be over hundred million children.

Fifty to ninety (in neonates) percent of the available medicines have not been tested for safe use in children and consequently lack information on safe and effective use. "Children do at least deserve as much attention and carefulness as do adults" (Byrne)

Children represent a truly vulnerable population with developmental, metabolic, pharmacodynamic and kinetic, physiological and psychological differences from adults. There is a high level of concern among Patient Organisations in the Europe Union about the safety and efficacy issues, the limited amount of age-appropriate research and the lack of regulation accorded to medications that are currently being used in the treatment of children. This situation results in significant and unnecessary risks, lack of efficacy, unavailability of therapeutic advances, unexpected adverse effects (chloramphenicol, thalidimide, diethylstilbestrol, tetra cycline, valproate, asperin &) and even death among this age group.

The importance of the subject "children and medicines" will considerably grow in the near future because of increasing insights from genetic and molecular research and the increasing possibilities for early detection of disease . Consequently medicines will play a more vital role for children in prevention, suppression and/or delay of disease expression.

#### **A large joint and coherent effort**

A large coherent effort of all parties - clinical, research/academia, industry, regulatory and financial community and last but not least the patient organisations - is necessary to address the present unacceptable situation for existing medicines as well as for applying new knowledge and technologies for the development and production of new effective and safe drugs.

Patient Organisations greatly welcome the EC-DG Enterprise action on "Better medicines for children" and the invitation for comments and input. They highly favour the proposed incentives, regulatory requirements, research funding suggestions and the attention for greater transparency and expert regulatory, ethical and clinical supervision.

Patient Organisations are supportive of resolutions, statements and activities of those various stakeholders in this proposal - the Council of the European Union, the European Federation of Pharmaceutical Industries (EFPIA), the European Forum for Good Clinical Practice (EFGCP) and the Confederation of European Specialists in Paediatrics.

#### **Special Initiatives**

Patient Organisations would like to see specific initiatives developed in the areas of:

1. **Rare Genetic Disorders** that require a special and separate supportive approach and have been adopted as a special field of interest by Patient Organisations. New technologies using molecular diagnostic tests will be used more and more to detect genetic mutations linked to various diseases. Sixty five percent of all of the opinions of the Committee for Orphan Medicinal Products (COMP) concern those medical conditions (usually Rare Diseases) affecting children. In respect to rare disorders special attention is asked for the communication on the consultation paper by the European Organisation for Rare Disorders (EURORDIS) which is fully supported.

## 2. Collaboration Beyond Europe:

- By the use of information and research findings from countries such as USA and Japan. This is of special importance for the proposed central database on information about medicinal products for children that will include information on newly approved indications and negative trials.
- By obtaining more statistically relevant data more easily In the field of Rare Diseases.
- By shortening the time period between now and the effective implementation of the proposed new regulation (approval by 2004 with implementation in 2006?).  
“Importing” the already existing data from the United States could be a contribution. President Bush signed in January this year the “Best Pharmaceuticals for Children Act . Over 400 studies are registered and for over 20 medicines information regarding the use in children changed. (<http://pharmacos.eudra.org/F2>)

3. **Improvements in the ethical and scientific requirements** for conducting research in children (also including clinical trials with children on pharmaco genetic properties of new medicines). A strong and adequate European framework specialised in the ethical aspects of trials with children is necessary in order to protect the dignity and the rights of children. The European Forum for Good Clinical Practice (EFGCP) is working, and since recently in partnership with patient organisations, on these issues also to see that paediatric patients are not overexposed to research and not over or under treated.

4. **Increased awareness and education** concerning the required activities for the availability and prescription of safe and effective medicines for children. Dialogue on the benefits and risks of paediatric research for individual children participating in trials as well as for public health in general, is important. Awareness of the great potential pressure and burden which is laid on parents and children participating in trials. Government officials, researchers and the pharmaceutical industry must be motivated towards a synergistic approach with investments at national levels and the European level.

## 5. Priority for (preventive) treatment of serious and life threatening diseases

6. **The active role that Patient Organisations** play and could play in this field. Many Patient Organisations have excellent relationships with paediatric specialist groups at both national and European levels. The involvement of patient organisations on the European policy and regulatory level as well on the projectbound and individual level should be recognised by making available the required financial resources. Moreover patient organisations could play an important role in the set up of clinical trials, in recruitment of participants and in communication.

## 7. Long term follow up of the effects of paediatric medicines.

### Patient Organisations favour:

1. The **creation of a paediatric fund** at the European level that would support additional research on existing medications regarding their use in children. Due to the backlog this fund would initially require an amount of 200 million Euros with investment opportunities for both the public and private sectors. This amount is equivalent to what the United States Congress provided for this purpose. It would be advisable that the proposed regulation more clearly indicates and defines other possible resources for the fund.
2. Extension of **Market exclusivity** for suitable pediatric programmes and “Kid Marketing Authorisation” that gives a period of data protection for studies on medicinal products where no protection of intellectual property exists. These are

necessary incentives for industry whose efforts in generating paediatric scientific studies deserve recognition and which are of special relevance in the field of rare disorders. Unnecessary hurdles in an already complex field should be avoided.

3. The development of a **central database**, the formation of an **EMA pediatric expert advisory group** and the building of a **pan-European network of clinical paediatric and pharmacological excellence** and specific expertise in the performance of trials on paediatric populations and that could link existing national and European initiatives. These initiatives can avoid unnecessary trials and contribute to efficient and adequate use of already available expertise, experience and data.

Patient organisations are worried about the considerable backlog and the gap between the present and required situation. Many existing medicines have to be re-tested which means a large research investment. Regulation and incentives to test new medicines are lacking so far. Therefore, it is essential that measures be taken in the short term to address this urgent and unacceptable situation.

Europe's children will greatly benefit from a well co-ordinated, well supported, and a well-funded approach.

Finally it should be realised that the subject "better medicines for children" is part of a huge research effort and worldwide process running from diagnosis to treatment and from gene to cure (see "pathways to treatment"). This effort greatly benefit from new genetic and biotech knowledge and suffers from fragmentation, lack of coherence and of structural funding. Patient organisations play an increasing role by providing data, motivating patients/families, facilitating and supporting research, communication with media and politicians, influencing health policy on the national and EU-level. For this reasons they have united in European Alliances and started the European Patient Forum. In the European Platform Patient Organisations Science and Industry (EPPOSI) patient organisations work together with scientific groups and industries to influence healthcare policy.

Although this paper may be considered as representative for the opinion of many parents and patient organisations, it does reflect explicitly the opinion of 7 European patient alliances who discussed the subject extensively during a meeting in Brussels (Gamian, EAGS, EAMDA, ECFPN, IDF, EPV, EAIH).

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