

STATEMENT

Patient organisations on

“BETTER MEDICINES FOR CHILDREN”

Patient organisations are very concerned about the situation in the EU where 50 to 90 (in neonates) percent of the have not been tested for use in children. This means that existing medicines have no information on safe and effective use in children leading to the use of unauthorised medicines which may result in significant risks, lack of efficacy, unexpected adverse effects and even death. This also means non-availability of therapeutic advances for children.

"Children deserve as much attention as do adults" Children represent a vulnerable population with developmental, metabolic, physiological and psychological differences from adults.

There are no regulations for testing new drugs for children.

A large coherent effort of all parties concerned (clinical, research, industry, regulatory and financial community and of course patient organisations) will be necessary to address the present, unacceptable situation for existing medicines as well as to address the situation for new medicines.

The target group is seventy-five million children in the EU, all aged under sixteen.

Patient organisations are supportive of resolutions, statements and activities of various stakeholders such as 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 (and its ethical and operational guidelines for good clinical practice in paediatric research).

Patient organisations welcome the EC- consultation document from DG Enterprise on “Better medicines for children” and the invitation for comments.

Patient organisations 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 request attention for

1. Specific issues as the rare disorders, which have been adopted as a special field of interest and require a special and separate supportive approach, but also since many of the future diseases are genetic (65% of all COMP opinions concern medical conditions affecting children).

2. Collaboration beyond Europe

- a. on 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 on medicinal products for children including information on newly approved indications and negative trials
- b. in the field of rare diseases to obtain more statistical relevant data more easily.
- c. the time before the effective implementation of the new regulation, which will probably only be in 2004 with likely results in 2006. Could the existing US data already be brought to Europe ?

3. The high ethical and scientific requirements for conducting research in children. We refer to the great work of the European Forum for Good Clinical Practice. This forum works, since

January this year, together now with patient organisations. A strong and adequate European ethical framework specialised in the ethical aspects of trials with children is desirable. This also in order to protect the dignity and rights of children and to see that paediatric patients are not overexposed to research

4. The need for increasing awareness and education on the subject to contribute to alertness in the prescription of medicines for children as well as to motivate governmental officials, researchers and industry towards a synergistic approach and investment on the national as well as on the European level. 5. The role patient organisations play and can play in this field. Many patient organisations have good relationships with (paediatric) specialist groups at national and European levels, which would facilitate trials.

Patient organisations favour

1. The creation of an EU-level fund that would support additional research on existing medications regarding their use in children.

Due to the backlog this fund would need an amount of 200 million euros which is about the same as the US- Congress provided for this purpose. Public and private sectors could invest in this fund. It would be advisable that the proposed regulation more clearly indicates and defines possible resources for the fund.

2. Market exclusivity /one year patent extension for suitable pediatric programmes; and “kid marketing authorisation” or period of data protection for studies on medicinal products where no protection of intellectual property exists will be necessary; these are all providing the necessary incentives for industry.

Especially in the field of rare disorders, caution should be paid to the “pediatric rule”, which sets the system as an obligation. One should not add hurdles to an already complex field.

3 The creation of a central database, the formation of an EMEA paediatric expert advisory group and the building of a pan-European network of clinical excellence that would link existing national initiatives and develop European capacity and knowledge

Patient organisations are worried about the considerable backlog. Many medicines have to be re-tested and a large research investment will be needed. Therefore action and measures have to be taken in the short term to address this unacceptable, urgent situation.

Broadly speaking there is a common view on this urgent and vital subject.

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