Confédération Européenne des Spécialistes en Pédiatrie
Section Monospécialisée de Pédiatrie de l'U.E.M.S.

Confederation of European Specialists in Paediatrics
Monospecialist Section of Paediatrics of U.E.M.S

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MINUTES MEETING WORKING GROUP MEDICINES FOR CHILDREN SIGTUNA, 20 MAY 2004

Attendance list:

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Chair: José Ramet

Key issue: European Commission has requested CESP advice regarding "Medicines for Children" initiative and need for appropriate paediatric research into use of medicine in children.

Background information:

- **Jan 8** : workshop organised by the EC on use of medicines for children : active participation CESP (R. Kurz and J. Ramet)
- **Mar 29** : meeting on "Medicines for Children". Creation of the paediatric working party of the EFGCP. CESP represented by José Ramet.
  The working party included representatives from CESP, the World Medical Association (president), EFGCP (president), EMEA (European Medicines Evaluation Agency), Swiss Medical Society, pharmaceutical industry representatives and others.
- **April 1**: symposium organised by CESP and EFGCP in Brussels on Medicines for children and paediatric research.

European Commission Proposals on Medicines for Paediatric Use

Aim: To stimulate the pharmaceutical industry to support research in children

Paediatric Board
Paediatric Investigational Plan
Paediatric Study Programme: Medicines Investigation for the Children of Europe (MICE)

Objectives:

- To stimulate more high quality research in medicines for children
- To increase availability of authorised medicinal products for children
- To improve information available on medicines administered to children

Paediatric Board (proposed)

- Powerful advisory group under umbrella of EMEA
- 28 appointed delegates – core group.
- Delegates should have expertise in areas relevant to the objectives
- The Board will carry out tasks related to objectives, and in particular to examine the "Paediatric Investigation Plans"(PIP) , including methodology/design, ethics, and expected benefits for children leading to an overall positive or negative opinion.
- Provide incentives and support to the pharmaceutical industry to encourage compliance with EMEA requirements.
- Make decisions on where the research encompassed by a PIP will be carried out (i.e. appropriate study groups)
- May become a "voice" to advocate for children and their interests to the national/EC regulatory authorities.

Paediatric Investigation Plans (PIP):

- Mandatory requirement, which must precede any application to EMEA by a pharmaceutical company for marketing authorisation of:
    - a new product
    - a new route of administration for a previously approved drug
    - a new formulation of a previously approved drug.
- Must be formulated and submitted to the Paediatric Board
- Must seek to obtain data on the use of the new product/route of administration/formulation in patients throughout the paediatric age spectrum from neonates to young adults, recognising that neonates, children and adolescents have specific needs.
- On the basis of the Paediatric Investigation Plan (PIP), the Paediatric Board will make a decision to:
    - Approve the PIP, which the pharmaceutical company would then implement
    - Require the pharmaceutical company to modify the PIP to overcome ethical or methodological/design objections
    - Defer a decision
    - Waive the requirement for a PIP if the product/route/formulation is very unlikely to have paediatric application/benefit.
- The benefits for the company in successfully completing a PIP include a 6 – 12 months extension (duration yet to be decided) of the EU patent life (i.e. very significant financial incentive for pharmaceutical company). It will be important to ensure that the incentives will be sufficient to induce the pharmaceutical companies to remain in the EC for product research and licensing. An important incentive for EC based research is that research participants/subjects are voluntary (ethical code) i.e. not paid – therefore cheaper to do research.

MICE (Medicines Investigation for the Children of Europe)

- Aims to support studies for medicinal products not benefiting from patents or supplementary protection certificates (i.e. the 70% of drugs currently used for children)
- Managed by EMEA and the Paediatric Board (probably)
- Source of funding unresolved, but possibly partnership between pharmaceutical industry and EC
- This group might also support research into "orphan" drugs in which primary (often only) use is for the treatment of rare paediatric diseases. One possibility is that existing "patent-expired" drugs could be investigated by being used as "bench-marks" against which new products are measured.

Proposed resolutions by "Medicines for Children" subgroup

- The EC should take immediate action on its proposal
- A monitoring group should be created to assess the success of the legislation in fostering research, enhancing information about medicines in children and increasing the number of medicines available for children.
- CESP should be proactive in determining the composition and structure of the Paediatric Board, by:
    - Maintaining good contacts with EC commissioners
    - Maintaining good contacts with EMEA – possibly to be invited to the December CESP meeting
    - Becoming a "voice" to the incoming "new" members of the EC and European Parliament.
    - Organising a **½ day event before the CESP meeting in December** dedicated to "Medicines for Children".
    - Set methodology parameters / recruit study design expertise
    - Survey current the "national readiness" of member countries for undertaking clinical trials in children (pharmacy, ethics applications, trial design expertise)
    - Foster robust communication links
    - Consider "accreditation" or visitation of clinical trials centres / networks
    - Publication (by CESP) to support these proposals and to emphasise the importance of the "Medicines for Children" group, and produce guidelines (measurement instruments in children, setting standards, measuring burden of illness, quality of life, health economic evaluation)
    - Advocate for Clinical Trial Infrastructure support
    - Recruit CESP subgroup of 2-3 persons to carry tasks forward and present the update at the December 2004 meeting
    - Look into links between EC and US FDA
    - EU Orphan project and publication of complete set of information designated for treatment of rare diseases in children

Next meeting:

Friday morning December 10, 2004 in Brussels with all CESP delegates and representatives from the European Parliament, the European commission, EMEA, the pharmaceutical industry, the patients' representatives, the FDA.

Tauny Southwood and José Ramet