

**Meeting of the “Steering Group (SG) Weesgeneesmiddelen/
Medicaments orphelins” Academy of Medicine, Brussels,
February 5, 2007 10.30 – 13 hr**

Present: Marc Bogaert, Erik Tambuyzer, Tim De Kegel (Leo Neels), Marc dooms, M. Fierens,
Claude Sterckx, François Sumkay, Vera De Groof, Lut De Baere, François Eyskens,
Ilse Weeghmans, Jean-Jacques Cassiman

Apologies: Andre Lhoir, Marc Abramowicz, Vincent Bours, Yolande Avontrodt, Thierry Velu,

1. The report:

- ✓ Based on the comments received, a new version of the report and the executive summary will be produced, **JJC**, and submitted for final approval.
- ✓ As previously decided the executive summary and text will be sent to the press, politicians, ministry of health, learned societies, and to the participants of the symposium. Members of the SG should present the conclusions at their respective society meetings. Please provide addresses of societies to Iris. **ALL**
- ✓ The text will not be printed but sent as print out and will be placed on the website (see further).

2. Website:

- ✓ A website has been developed. The domain name will be: zeldzameziekten.be and maladiesrares.be giving access to the same site. **JJC**
- ✓ All info of the Symposium, report, pp presentations, pictures will be placed on the web. **JJC**
- ✓ Links are made to Pharma.be, Bio.be and to the site of the Hospital pharmacists. **JJC**
- ✓ Link to EMEA COMP <http://www.emea.eu.int/htms/human/comp/a-zcompsumop.htm>
- ✓ A report on transparency of prices, made by the consultancy firm Alcimed for the EU commission, should be available on the EU Commission website. **ET** to forward the report.

Comment [t1]: En naar de <http://pharmacos.eudra.org> website van de Europese commissie?

3. The future:

1. Report from the EU ad hoc working group: **A. Lhoir (coordinator), F. Sumkay, E. Tambuyzer, V. De Groof.**

- The following mail from F. Sumkay pertaining to this aspect was received:

Chers collègues, Il faut bien constater que, jusqu'à présent, les éléments sur lesquels nous sommes arrivés à un consensus entre nous pour définir "un volet européen" dans les objectifs de notre Groupe sont encore très limités, ce qui me semble logique au stade actuel, encore embryonnaire, de la mise en place d'un tel "Groupe de pilotage belge pour les maladies rares". Aussi, pour tenter de réussir le difficile exercice de résumer en qqes

mots les éléments sur lesquels nous sommes déjà d'accord, je vous adresse la proposition martyre suivante, qui serait de mentionner simplement que

" le volet européen des objectifs du Groupe de pilotage serait de fournir aux autorités belges des avis visant à :

- ✓ faire diffuser et connaître en Belgique :
 - les informations déjà disponibles en Europe sur les maladies rares et leurs traitements;
 - les mesures et recommandations qui sont déjà en application en Europe pour optimiser la prise en charge des maladies rares;
- ✓ inciter les intervenants belges à agir au niveau des différentes instances européennes pour développer une véritable politique européenne visant à améliorer la prise en charge des maladies rares dans tous les pays de l'Union."

F. Sumkay draws the attention of the members to the fact that the representatives of the InterMutualistic College had only a mandate for the organization of the Symposium. To continue would mean to request a further mandate and to focus on Rare Diseases rather than only on Orphan Drugs.

- The workgroup also proposes to make the orphan medicinal products (OMP) Regulations better known in Belgium and to propose to ensure that an improved link is made between the CHMP and COMP members representing Belgium in those bodies and the authorities working on the reimbursement files.
- Note: according to the OMP Regulation EC 141/2000, an orphan medicinal product is a product for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and that no satisfactory method of diagnosis, prevention or treatment of the condition in question has been authorized in the European Union, or if such method exists, the orphan medicinal product is of significant benefit to patients..

The members agree with the vision to extend the focus of the ad hoc group to Rare Diseases.

2. The ad hoc workgroup on the Belgian dimension **F. Eyskens (coordinator), L. De Baere, T De kegel, I. Weeghmans, C. Sterckx**
 - No formal meeting was held. The situation in The Netherlands and France were reviewed
 - Priorities have to be defined
 - Adequate patient information, expert centres, stimulation of research are topics to consider.
 - Role of the patient organizations in following the criteria established (expertise of the centre, correct application procedure...)
 - (Compassionate use is available in Belgium providing EMEA approves)

As a general conclusion the members suggest that JJC would put a few suggestions on paper in preparation of the next meeting.

**The next meeting will be held on Monday April 23
10.30 – 13hrs at the Academy**