

**Meeting of the “Steering Group (SG) Weesgeneesmiddelen/  
Medicaments orphelins”** Academy of Medicine, Brussels,  
October 8, 2007 10.30 – 12.30 hr

Present: Marc Abramowicz, Marc Bogaert, Marc dooms, Jean-Jacques Cassiman, Vera De Groof, Erik Tambuyzer, Klaartje Bruyninckx, Mattias Neyt, François Sumkay, Claude Sterckx

Apologies: Andre Lhoir, Vincent Bours, Yolande Avontroodt, (Thierry Velu), François Eyskens, Jeroen Keppens, Tim De Kegel (Leo Neels), Lut De Baere, Ilse Weeghman, M. Fierens

1. The agenda was approved
2. The action point of the previous meeting were approved
3. The KCE will decide on October 16 about our proposal and 2 others around the same topic, whether this cluster will be released with an open call or will be taken care of internally totally or partially. In order to be ready when the call is issued it was proposed that the Steering Group could act as an advisory group, but that a few expert groups such as those of M. Abramowicz, F. Eyskens and 1 or 2 more to be identified, should decide who will be the coordinator and how the work will be organized between these groups.

A series of sources of information on the prevalence of rare diseases in Belgium could be consulted. In preparation of a possible call by KCE the availability of numbers of patients could already be checked:

- The Orphanet prevalences published recently could be used as a first indication
- RIZIV/INAMI has numbers of reimbursed Orphan drugs; **MB** to check this
- BOKS has probably numbers also. **JJC** to check
- Pharma.be working group on Orphan drugs could contact the Pharma companies about the numbers they have. **ET** to propose this to the WG
- The genetic centers have figures. **JJC** to check
- FAPA (Familial Polyposis Coli) has figures. **JJC** to check
- The National Cancer register (M. Callens) has numbers. **FS** to check

In view of the limited time the KCE project will provide, a letter will be sent to the directions of major hospitals to inform them about the probable upcoming KCE project which will aim at determining the number of patients and the expertise of the hospital for the treatment of Rare Diseases. **JJC** to write a proposal.

4. KBS-FRB:

A contract (agreement) is being drafted by KBS-FRB for the Fund called: “Rare Diseases and Orphan drugs, Zeldzame Ziekten en Weesgeneesmiddelen, Maladies Rares et Médicaments Orphelins”. We will be informed by them when it can be signed.

5. Eurordis Belgium:

A new meeting between different Belgian patient organizations is planned for **October 26**. The members of the SC wonder whether sufficient patient organizations have been invited to this and/or whether enough of them are aware of this initiative. The Orphanet

website also has a list of a whole series of Belgian patient organizations which could be made available. **JJC** to contact Jeroen for information.

6. Since neonatal screening has an impact on diagnosis of rare diseases, contact should be sought with a representative of the screening labs. **JJC** to contact Goyens and F. Eyskens and/or Dirk Dewolf

7. ORPHANET Belgium:

The Orphanet Belgium website is coordinated by JJC. The following members were proposed to become members of the Scientific Board of Orphanet Belgium, including experts in oncology: M. Abramowicz, V. Bours, F. Eyskens, Th. Velu, M. Bogaert, M. Piccard, P. Schöffski, M. Doods, J. Longueville, somebody from ORTC. Additional expertise will be sought. **Suggestions of names to be send to JJC.**

The members of the scientific board are responsible for the quality of the information entered in the Orphanet website and should be available to respond themselves or find and appropriate expert to respond to questions posed by people (mainly patients) looking for answers on the Orphanet website.

8. Varia:

- ✓ We need to find out what happened to the proposal of Y. Avontroodt drafted for the Federal Parliament? **YA**
- ✓ Could A. Lhoir provide prevalences of Rare Diseases, as estimated by the companies when they submit their dossiers to the COMP? **A.L.**

**The next meeting will be held on**

**Friday December 14**

**10.30 – 13hrs at the Academy**