

Fund Rare Diseases and Orphan Drugs

managed by the King Baudouin Foundation

REPORT OF THE MANAGEMENT COMMITTEE MEETING AND FIRST MEETING OF INFORMATION EXCHANGE WITH THE UNIVERSITY HOSPITALS 22 FEBRUARY 2008

		Present	Excused
Chairman	Jean-Jacques Cassiman	X	
Members	Marc Abramovicz	X	
	Yolande Avontroodt		X
	Marc Bogaert		X
	Vincent Bours	X	
	Lut De Baere	X	
	Vera De Groof	X	
	Marc Doods	X	
	Tim De Kegel	X	
	François Eyskens	X	
	André Lhoir	X	
	Herwig Jansen	X	
	Leo Neels		X
	Mattias Neyt	X	
	Claude Sterckx	X	
	François Sumkay	X	
	Erik Tambuyzer	X	
	Thierry Vélú		X
	Ilse Weeghmans	X	
	Gerrit Rauws		X
	Invitees	David Cassiman, UZ Leuven	X
Linda De Meirleir, VUB		X	
Paul Misson, ULg		X	
Marie-Cécile Nassogne, UCL		X	
Bruce Poppe, UGent		X	
Gauthier Remiche, Erasme		X	
Alain Denis, Yellow Window		X	
Elfriede Swinnen, UZ Leuven		X	
Secretary	Tinne Vandensande	X	

The dates for the following two meetings are:

- Regular Management Committee meeting: **Wednesday 16 April 2008**, in the offices of the King Baudouin Foundation, from 10:30 am to 1:00 pm.
- Second information exchange meeting with representatives of the university medical centres re specific activities on rare diseases: **Friday 19 September 2008**, KBF offices, 10:30 – 1:00 pm.



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1. Welcome address; approval of the agenda and minutes

Issue	Welcome address to invitees and members of Management Committee (MC); aim of the meeting
Info/ Comments	<p>Documents:</p> <ul style="list-style-type: none"> - Minutes MC, 14 December 2007 - List of the participants <p>Tinne Vandensande apologizes for the errors in the attendee list of the previous MC meeting; will be rectified.</p> <p>Erik Tambuyzer refers to the work of the High Level Pharmaceutical Forum. Erik has been actively involved in the temporary subgroup of the working group Pricing of the Pharmaceutical Forum. This subgroup has recently produced a paper on 'Improving Access to orphan drugs for all affected EU citizens'. This paper will be presented and discussed on the 17th & 18th of April with the whole Pricing working group. It is only one of the items dealt with by the Pricing group (tendering is another one etc...). The document cannot be distributed yet. [Erik is also member of the HLPF working group on Relative Effectiveness].</p>
Decisions	<p>Jean-Jacques Cassiman welcomes Herwig Jansen, coordinator BMR-RBM, Scientific Institute of Public Health, as new member of the Management Committee; he gives a hearty welcome to the representatives of the university medical centers: Bruce Poppe, Marie-Cécile Nassogne, Linda Meirleir, Gauthier Remiche, David Cassiman and Jean-Paul Misson and welcomes also Alain Denis, Yellow Window consultant. He introduces Elfriede Swinnen of the Leuven University who will be involved in the follow-up of the exchanges of experiences on rare diseases in the university medical centres.</p> <p>Jean-Jacques confirms that the composition of the Steering Group is similar to the Management Committee.</p> <p>The aim of the meeting is two-fold: to collect information on the rare diseases management in the university hospitals, with the University Hospitals of Leuven as a first case, and to reflect on the gathering of common data for further analysis. It is without doubt an historical meeting, being the start of a challenging trajectory involving many diverse actors.</p> <p>Minutes and agenda are approved.</p>

2. The activities of the multidisciplinary working group on rare diseases of the University Hospitals Leuven

Issue	Casus UZ Leuven – presentation and discussion
Comments	<p>Documents:</p> <ul style="list-style-type: none"> - PowerPoint presentation David Cassiman and Marc Dooms - Survey Orphan Diseases-Drugs; <i>slides & survey attached as appendices</i> <p>The Task Force (TF) Orphan Diseases was created in 2006. Besides regular consultation meetings and quality improving projects, like e.g. the central BSF/FSS submission and tracking, the TF recently invested in a hospital-wide survey on rare diseases and orphan drugs. Mid 2007 the TF launched a survey, via an intranet questionnaire, to all staff members and head of</p>

departments. Aim of the survey was: to collect data on which rare diseases occur, number of patients in registry, which orphan medicines are used, info about the reimbursement procedure, the use of medical material, including cost for patients and for hospital, how the care is organised – convention, nurse specialist- and if there is clinical or fundamental research on specific diseases.

The first results are presented; the full report will be discussed shortly with the hospital management.

Marc Dooms presents some web pages on the internet site of the Vlaamse vereniging van Ziekenhuisapothekers. The VZA website is only available for members and provides info on orphan drugs and shares experiences of hospital pharmacists on orphan drug preparation, via the working group 'weesgrondstoffen' (see slide on the metabolic box, where the comment was made that OH-Vitamin B12 must be added). Several links to other sites for professionals are available. French speaking pharmacists can also register. There is a direct link to the site of the 'association francophone des pharmaciens hospitaliers' www.afphb.be

The discussion between the participants focuses on one of the first conclusions re the (hidden) cost for hospital and patient. For respondents to the survey it was apparently not possible to say 'what the costs are', due to the heterogeneity of the orphan patients and of their treatment. It would be interesting to have more data on the cost differences between an ordinary and an orphan patient.

Mattias Neyt refers to the databases of IMA/AIM (Agence Intermutualiste - Intermutualistisch Agentschap) and RIZIV/INAMI, where a lot of clinical and financial data can be consulted.

Erik Tambuyzer reflects on the list of diseases that came out the survey: is 'life-threatening'/severe not the main criterion? A minority of RDs isn't that critical. Herwig Jansen doesn't agree with that limitation: all RDs need to be treated, and orphan medicinal products need to be developed for all RDs.

André Lhoir expands on the EC regulation for orphan medicinal products. This regulation laid down a procedure for the designation of medicinal products as orphan medicinal products and it provides incentives for the research, development and placing on the market of designated orphan medicinal products. André is member of COMP, the Committee for Orphan Medicinal Products of EMEA. COMP is responsible for reviewing applications from persons or companies seeking orphan medicinal product designation for products they intend to develop for the diagnosis, prevention or treatment of life-threatening or very serious conditions that affect not more than 5 in 10.000 persons in the European Union.

The representatives of the other university hospitals admit that they are less organised than UZ Leuven; there are several initiatives, informal working groups, but the collaboration on the hospital level could be substantially improved. What is needed? The idea of a common task force per hospital seems a good format; the collection of information, based on the Leuven form has proven to be very useful. How to convince the staff to participate? It is a win-win situation for patients, doctors and hospital management.

Gathering more knowledge on the 'hidden cost' for care of patients will be a convincing argument to get the hospital management on board. But better coverage for less money looks contradictory at the first sight. Success stories could be very helpful too, to demonstrate the need for a better global

	management. The ‘convention metabolic diseases’ seems to be a good example. Erik refers to an article in the Lancet on the cost effectiveness of screening for Cystic Fibrosis. Working together for the benefit of the patient and by extension for the whole society is what this steering group is trying to achieve.
Decisions	The chairman emphasizes the importance of data; each of us has already been confronted with requests from politicians for more concrete data. On the request of the representatives of the other university hospitals the questionnaire of the TF UZ Leuven will be used as a model. A period of six months is needed for launching (including reminders) and analysing the input. Elfriede Swinnen will provide a concept for common survey form.

3. The KCE call on Orphan Drugs

Issue	Status
Comments	<p>KCE has started negotiations with Yellow Window, but there are still some items to be discussed. KCE insists, among others, on assessing the evidence of EMEA decisions, including an evaluation of EMEA files (applications, process of designation...). They want a critical appraisal of the EMEA decisions: how do they reach positive/negative opinions.</p> <p>Ilse Weeghmans asks if patients’ knowledge will be integrated. Mattias Neyt confirms that a diverse group of stakeholders, including patients, will be involved in an expert group.</p> <p>As mentioned in the application, Yellow Window – most of the consultants are economists - searches collaboration with the Steering Group to include the necessary expertise. There will also be a close collaboration with staff members of KCE.</p> <p>Erik refers to the work of the Pricing group of the HLPF (see above).</p>
Decisions	Alain Denis will communicate more in-depth on the project during the next meeting of the MC (<i>to be confirmed</i>).

4. 29 February 2008 – 1st European Rare Disease Day

Issue	Activities of Radiorg.be
Comments	<p>Lut De Baere is proud to announce that Radiorg, the Rare Disease Organisation Belgium, is recognised by Eurordis as the Belgian ‘coupole’.</p> <p>Due to the united efforts of the 6 patient organisations, the creation of Radiorg has been made possible in only 4 months time.</p> <p>Radiorg wants to become a real platform for the rare diseases patients and their family. The board will undertake further initiatives to connect with very small organisations.</p>
Decisions	All members of the Steering Group are invited to participate at the special event in the House of the Federal Parliamentarians on the first European Rare Disease Day. Marc Abramowicz will be the expert spokesman at the symposium.

5. Projects VPP and BOKS

Issue	Research initiatives taken by BOKS and PPP
Comments	<p>Documents in Dutch available:</p> <ul style="list-style-type: none"> - BOKS: Problematiek en Strategisch Plan - VPP: Welke knelpunten ondervinden patiënten met een zeldzame aandoening. <p>BOKS works together with the UA to conduct a study on the prevalence and incidence of metabolic diseases. It is suggested to extend the current study to all rare diseases.</p> <p>VPP wants to deliver at the end of 2008 a paper that lists the problems experienced by orphan patients. Since May 2007 they carried out a number of in-depth interviews in association with the 12 member organisations of VPP. The next months VPP will try to reach all other patient organisations/self-help groups involved and invite them to participate in the study.</p> <p>A first analysis of the interviews will be shared with all interviewees and used to build up common statements. Final stage of the consultation process will be the producing of policy recommendations.</p> <p>This study is an initiative of VPP, but there is an agreement with LUSS for completing the intermediary results with points of interests of associations that are only active within the French community.</p> <p>Next the discussion goes further on the (hidden) interests of patient organisations: aren't they (obliged) allies of industry; aren't they too dependent of the financial support of industry? Is it a stereotype or does it contain elements of truth? It is an issue that is frequently taken into consideration during discussions on improving patient participation in health-care policy. Both industry and patient organisations are aware of the critics; there are in the meantime several ethical codes in use.</p>
Decision	BOKS and VPP are invited to report progress of their research activities during the next meetings.

6. Others

- On behalf of Orphanet Belgium Jean-Jacques calls for cooperation; Orphanet needs a network of antennae to answer the questions posted by patients.
- The next meeting will be held on Wednesday 16 April 2008 in the 'Raadzaal/ Salle du Conseil' of the KBF. Starting hour: 10.30 am.