EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

EUCOPE meeting of the Orphan Medicinal Products (OMP) Working Group

23 January 2018, 11.00 – 16.00 CET

– Minutes –

Attendance

Participants to the face-to-face meeting were the following:

Surname	Name	Organisation
Albano	Claire	Alexion
Barnes	James	Vertex
Biesenbruck	Mercedes	EUCOPE
Degrief	Toon	Shire
Finnegan	Rachel	FIPRA
Fontaine	Jean-Manuel	Mithra
Gibsy	Martin	MyTomorrows
Gicquel	Erwan	Celgene
Grub (Dr.)	Thomas	Medac
Heck	Matthias	Alexion
Khraiche	Joelle	CSL Behring
Lockner	Thomas	Prothena
Maronati	Manuela	PTC Therapeutics
Roulland	Delphine	EUCOPE
Roux	Jean-Louis	BioMarin
Schmoeller	Michael	Santen
Schneider	Dominik	Ecker & Ecker
Sude	Oliver	EUCOPE

Discussions

The meeting was chaired by Joelle Khraiche (CSL Behring) and Delphine Roulland (EUCOPE). The list of participants is provided at the bottom of this document.

As an introduction, the Secretariat reminded the participants of the identified priorities of the Orphan Medicinal Products (OMP) Working Group for 2018 – i.e. incentives, HTA and pricing & access. More details

are available on slide 5 of the attached presentation. Participants were also informed of the upcoming internal meetings and external events.

1. EU Commission`s analysis of incentives and rewards

- **Policy developments**

Delphine Roulland gave a short overview about the latest policy developments, including the Commission Roadmap for the evaluation of the OMP and Paediatric Regulation as well as the planned studies and publications for 2018 and 2019. It was asked whether the evaluation will take into account technical aspects such as the recent Commission Notices on the concepts of significant benefit and of similarity.

It is considered that EUCOPE's submitted comments to the Roadmap along other submissions are unlikely to lead to a revision of the Roadmap. Nonetheless, it remains important to highlight our concerns and seek clarifications, in particular with regards to the upcoming Orphan study, which is said to feed the overall evaluation.

Action item: Secretariat to follow up with DG SANTE to enquire about the orphan study as well as the Similarity Notice and its planned publication.

The group went on to discuss the upcoming policy and political developments. The Copenhagen Economics Study Report is expected for publication by mid-February.

In this context, Claire Albano (Alexion) drew the attention to a Dutch paper that reviews existing regulations and guidelines from 35 different countries on patient access to orphan drugs. The paper points out critical differences between countries in terms of range and types of regulations and policies implemented. The paper identifies "high prices" as the main challenge in patient access to orphan medicines and offers provocative solutions such as selling on Alibaba ingredients for pharmacy to prepare treatments.

- **Incentives Steering Group's engagement plan for 2018**

Clarifying the objectives of the Incentives Steering Group (ISG), and the strategic goals, Delphine Roulland explained what success would look like and how the engagement plan (= outreach + traditional communications campaigns) will unfold in 2018. The following were stressed:

- **Outreach at a national level will be key**; priority countries have been identified to be: **Germany, France, Italy, Finland, Spain, Sweden**. Those have been selected according to their current position on the discussions (e.g. supportive of innovation and of IP protection, or not totally against the industry), or their upcoming role as EU presidency (Finland). **Members' support, via their national branches, is of crucial importance.** Any company that wishes to support such efforts can kindly inform the Secretariat.

Action item: Members to inform Secretariat of their wish to support national outreach activities.

- **SME success stories / CEO testimonials**: in order for the narrative to resonate, it is deemed important to exemplify it with individual companies' stories and business models. This was further discussed with participants when looking at the collection table.

The template, which aims at facilitating the collection of companies' case studies, will allow EUCOPE to give specific examples when meeting with decision-makers, highlighting the companies' challenges (e.g. complex & lengthy R&D; various regulatory milestones; market access and P&R). With such case studies, the Secretariat will be able to tailor its messages to the decision-maker / authority with whome it will meet.

Members' feedback was the following:

- **Pricing:** difficult to give confidential data like prices and budget impact of the drugs. One option could be to propose price ranges and clustering according to rarity of the disease (e.g. ultra-rare

diseases). In addition, it may prove difficult to get meaningful data on a drug because it varies very widely between countries.

- **OMP-specific challenges**: it was agreed that highlighting such challenges would allow us to raise awareness as the complexity and uniqueness of researching and developing orphan drugs.

Nevertheless, the OMP working group agreed that **it is important for the future direction of the way of work, to have a clearer understanding of companies' challenges**. It was concluded that a **revised version of the template will be sent to members** who will fill it in on a voluntary basis, and with information that they feel comfortable with.

The template and received responses will be discussed at the next OMP WG meeting, on 20 March.

Action item: 1. Secretariat to circulate the template to the membership. 2. Members to fill in the table and send to Delphine Roulland as they see fit.

2. Country updates

- **The new German government and perspective on orphan drugs**

Matthias Heck (Alexion) made a presentation of the political discussions over the formation of a new German government as well as the current health priorities.

After the national election in September last year, the new government is still not in place. The coalition negotiations between the Conservative and the Social Democrat Parties are currently still in process. Therefore, the future development of the health care system is difficult to predict. Still, it is expected that the Federal Ministry of Health, currently held by the conservative Hermann Gröhe, will go to the S&D Party.

In terms of possible changes in the German healthcare system, the HTA process and the structure of the insurance are two main elements:

- Some, including Prof. Eckert (GBA) consider the system has been "flooded" with orphan drugs and requires a stricter HTA process. This **could entail the introduction of a category of "non quantifiable additional benefits",** though this would considerably weaken companies' position with payers.
- Insurance system: the SPD is working towards the abolition of the private health insurance. Timelines are unclear.

- **Introduction to the access issues in Ireland and the specific case of orphan drugs**

Jean-Louis Roux (BioMarin) presented an overview of the status of healthcare in Ireland and then looked more specifically at the challenges of access to orphan drugs.

At present, there is still a strong anti-industry sentiment seen in politicians and authorities' decisions and public statements. Despite the interesting 5-year Health Service Plan of the Government, initiated by the former Prime Minister, it appears that the focus for all drugs, and for orphan drugs in particular, remains on lowering prices – rather than looking at value - and encouraging generics' uptake. This was made clear in the Saintecare Report of the Committee on the Future of Healthcare, published in May 2017.

Jean-Louis Roux then spoke of the new **Rare Disease Technology Review Working Group**, chaired by Prof. Michael Berny. He deplored the absence of patients at the table yet, and warned against the major stumble block towards the industry.

From 2017 onwards, Irish MPs have started voicing their concerns over access to orphan drugs. The October 2017 event around the Iceberg Report, which highlights the access challenges in Ireland, enabled to gather parliamentarians' support to raise their concerns. At this event organised by IPPOSI Ireland, both industry and patients were aligned on criticising a dysfunctional healthcare system. Since then, a

second public hearing was held in the Parliament (but to which industry was not invited) and another is expected to take place in 2018.

At the next meeting, the OMP Working Group will have the opportunity to pursue the discussion on the Irish market with the head of IPPOSI Ireland, who will present their Iceberg 2.0 Report.

3. EURORDIS` paper on options to improve patient access to orphan drugs, in response to which we deem important to develop a EUCOPE position

Joelle Khraiche gave a short presentation of the paper`s main conclusions (slides 27 to 40).

For each pillar, participants shared their views and made suggestions for a possible EUCOPE positioning. It was clarified that:

- The audience for such a Paper is not only industry, but also their own membership, decision-makers in Brussels and across Europe, etc. That explains why some statements, meant to be aspirational, can be perceived as idealistic or dangerous (e.g. EURORDIS` ambition: "3 to 5 times more new rare disease therapies approved per year, 3 to 5 times cheaper than today by 2025" was criticised).
- EUCOPE's position should be structured in a similar way as EURORDIS' paper in order to best address their proposals.
- EURORDIS would welcome EUCOPE's honest opinion of the paper (with red flags), even if not made publicly available and only shared in informal discussions.
- Neither EFPIA nor EuropaBio have currently respond to the paper.

In addition, there was a shared feeling that many ideas are theoretically interested but hardly implementable and that the devil is in the details of the recommendations (e.g. misunderstandings and contradicting statements on pricing).

It was concluded that a **detailed summary of the discussions** will be shared by the Secretariat with the membership, as a starting point. Members are then invited to provide further feedback to finalise what will remain for now an **internal EUCOPE position**.

4. The Commission Notice on significant benefit and the particular issue of pharmacy preparations as comparators

Oliver presented on the Commission Notice on Significant Benefit (introduced Nov 2016) and reminded the participants of EUCOPE's comments submitted in the March 2016 public consultation, with regards to the risk related to pharmacy preparations as comparators. He further noted that the EMA had taken a similar view on the matter. He then presented the first application of the pharmacy preparation rule by the EMA Committee of OMPs (COMP) in general terms, calling for participants' comments on possible EUCOPE advocacy.

It was made clear that, though this is the first application case, the likelihood that such rulings be repeated for other orphan drugs is important. Furthermore, the **impact on orphan drugs**, for which the Orphan Designation has not been renewed, would be considerable in the **P&R negotiations**. **Potential economic off-label use** could also be feared.

Members tried to assess the potential damages of a common EUCOPE action for the wider industry, and requested some more information on the product (is this an old indication that was "recycled?").

In light of the high risks around this topic and in the absence of further information, it was concluded that **this topic will be monitored and discussed again at some future OMP working group meeting**.

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5. EMA reports on decision-making for orphan medicines

Oliver Sude presented on the recently-announced decision of the EMA to publish additional reports on its decision-making for orphan medicines. The reports will aim at summarising the reasoning of the COMP on whether or not an orphan drug still fulfils the designation criteria at the time of its authorisation. Alongside the announcement, EMA released its first orphan maintenance assessment for Merck's antiviral drug Prevymys (letermovir).

The Secretariat will monitor the publication of future reports and the group may discuss it again at a future meeting.

Next meetings

- **20 March, 10.30 – 15.30 CET**