EUCOPE
European Confederation of Pharmaceutical Entrepreneurs AISBL

15 February 2019

OMP WORKING GROUP MEETING

6 FEBRUARY 2019

Meeting minutes

ATTENDANCE

Surname	Name	Organisation
Anzévui	Jean-Pierre	Santen
Bahr	Volker	Medac
Balz-Johnson	Matt	EURORDIS
Bolt	Tresja	Bluebird Bio
Carmeliet	Tine	Allen Overy
Causey	Neil	FIPRA
Corazza	Andrea	FTI Consulting
Dupraz	Anne	Orchard Therapeutics
Gicquel	Erwan	Celgene
Gracia	Alfredo	Ferrer
Godfrey	Josie	JG Zebra Consulting
Heck	Matthias	Alexion
Hein	Julian	Chiesi
Hughes-Wilson	Wills	Mereo
Hutchings	Adam	Dolon
Jaggs	Tom	PTC Therapeutics
Jenewein	Joerg	Alcon
Khraiche	Joelle	CSL Behring
Macchia	Flaminia	Vertex
Maronati	Manuela	Atara Bio
Meulenbelt	Maarten	Sidley
Piazza	Annarita	EUCOPE
Roulland	Delphine	EUCOPE

Roux	Jean-Louis	BioMarin
Rübesan	Tim	Biotest
Schmidt	Fabian	Orphan Europe
Schneider	Dominik	Ecker & Ecker
Schnorpfeil	Willi	Value & Dossier
van den Bos	Hein	Hogan Lovells
Van Vooren	Bart	Covington
Wawrzyniak	Anna	Covington

DISCUSSIONS

1. WELCOME / INTRODUCTION / MEETING AGENDA AND OBJECTIVES

Delphine presented the agenda items of the meeting and welcomed Jean-Louis Roux from BioMarin as new chair of the EUCOPE OMG WG for 2019. The meeting objectives were two-fold:

- Information-sharing and updates on EU projects national developments related to the OMPs
- Group discussion on the issue of industry involvement in European Reference Networks (ERNs) and EUCOPE's participation to the EURODIS symposium (13-14 February 2019)

2. OMP WG 2019 PRIORITIES AND ACTION PLAN

- **2019 priorities**

The **three main areas of action** for EUCOPE in 2019 (EC analysis of the EU Regulation on OMPs; market access for OMPs and patient engagement on improved access) can be found on slide 10 of the attached presentation.

- **2019-2020 timelines**

cf. slide 12 of the attached presentation.

3. ASSESSMENT OF THE ORPH-VAL PRINCIPLES: PRESENTATION OF THE FINAL REPORT

Adam Hutchings (Dolon) provided an overview of the **assessment of the ORPH-VAL principles v. country systems.**

[Refresher] Launched in 2015 in a multi-stakeholder format **the ORPH-VAL initiative sets out nine principles to reduce inequalities in accessing OMPs in the P&R process in Europe** (full list on slide 15).

The final assessment report was published in December 2018. This report shows to which extent the big five EU Member States are aligned with or diverging from these principles and which differences exist among the national health systems with regards to patient access to OMPs.

Group discussions revolved around the **methodology used for this assessment report and whether this can be strengthened.** Adam Hutchings stressed that, to move the conversation forward, **pharmaceutical companies and national associations need to organise accordingly and ensure local dialogues with decision-makers** take place on the implementation of the principles at national level.

Adam also informed the group of the organisation of an ORPH-VAL workshop with the paper contributors, with the view to discuss possible areas of interest for future activities.

Next steps

- EUCOPE to make use of the assessment report in future national meetings.
- Corporate members to discuss with their national associations the possibility to engage the discussion with decision-makers on the basis of the report.

4. 2016 NOTICE ON THE APPLICATION OF ARTICLES 3, 5 AND 7 OF REGULATION N°141/2000

Jean-Pierre Anzévui (Santen) presented **a case study on the maintenance of the orphan designation of one of their medicinal products** – Verkazia – following the application of the 2016 Notice on the application of articles 3, 5 and 7 of Regulation n° 141/2000.

In particular, the specific challenges experienced by Santen concerned **Article 3 of the Notice**, which stipulates that in certain cases, **when assessing whether a product fulfils the requirements for an ODD, a medicinal product prepared for an individual patient in a pharmacy according to a medical prescription, may be considered as satisfactory treatment if they are well known and safe.**

The timeline and reasoning adopted by Santen and their legal counsel is specified on slides 36 to 41.

Following convincing clinical and legal arguments put forward by Santen, COMP decided after nearly a

year to revise its initial negative opinion of the maintenance of the OMP designation – which had been granted in 2006 and to grant an OMP marketing authorisation for the medicinal product.

This case, **which represents a precedent of an overturn of an EMA COMP negative opinion based on magistral/officinal preparation,** showed, according to Jean-Pierre:

- The importance for pharmaceutical companies to take into account also the political aspects which are related to the EU market regulatory framework,
- the importance of the support of a European industry association in providing support, guidance and legal assistance throughout the entire process.

5. EUROPEAN REFERENCE NETWORKS: WHAT ROLE FOR THE INDUSTRY?

A. EURORDIS' VIEWS ON THE FUTURE OF ERNS AND CHALLENGES AHEAD

Matt Bolz-Johnson, ERN & Healthcare Advisor for EURORDIS, introduced the topic with a presentation on the European Reference Networks, their structure and functioning (cf. slides 44 to 51).

With regards to industry interactions with ERNs, the following remarks were made:

- I**t is legally required that there is not only cooperation between Member States**, **but also an effective involvement of the pharmaceutical industry**.
- The ERN Coordinators of the Ethnical Working Group are currently developing a Code of Conduct Policy which includes guidance on how ERN should engage with companies. **A new statement on ERN & Industry from the Board of Member States is expected to be available in early 2019.**
- In its 2017 ERTC, EURORDIS facilitated the development of a "draft industry wish-list on the top 10 areas" where collaboration with companies should be explored (cf. slide 58).
- For the ERN on cancers, there is existing collaboration with the EORTC, an independent research hub, which acts as an honest broker between the industry and the ERN.

B. GROUP DISCUSSION

Members contributed their respective experiences and recommendations:

- Oftentimes, the EMA is asking how the companies are working together with the relevant ERNs
- MS' main concerns are with **ERNs' independence and the trust issues**
- **Companies already do interact with ERNs** (or centres of expertise which compose the ERNs) to discuss specific rare diseases, questions of prevalence, etc.
- The problem may be that **those interactions are on *ad-hoc* basis and unsystematic** from one ERN to another, un-transparent.
- Industry would gain in **understanding the strategic roadmap for ERNs**, in order to understand possible industry funding would go.

- EURORDIS already called for a **data strategy** to be put in place to guide the work of the ERNs.

Next steps

- Members to provide case studies or examples of their indirect contact with ERNs at present
- Interest members to volunteer in providing support to the Secretariat in the development of EUCOPE messages and asks.
- EUCOPE Secretariat to gain insights into and organise association's preparedness for the 25 February workshop planned by the EC on the topic of industry involvement
- EUCOPE to make a political statement on the essential of industry with ERNs at the EURORDIS Symposium.

6. COMPOUNDING OF ORPHAN MEDICINAL PRODUCTS IN THE NETHERLANDS, AN UPDATE

Maarten Meulenbelt (Sidley) introduced his presentation by reminding the group of the recent developments (cf. slide 64):

- 10 December 2018: **Health and Youth Care Inspectorate (IGJ) Decision*,** which allows pharmacists to use the technique of compounding in "**small scale**" production of medicinal products. The Dutch **Minister of Health Bruno Bruins expressed satisfaction with the IGJ decision** and announced his intention **to define "small scale":** the definition is expected to be **released in the first quarter of 2019**, and there are concerns regarding how this new definition will impact the OMP market in the Netherlands. The problem may well be that if a threshold is set up in an absolute manner, this may be particularly problematic for OMPs.
- 19 December 2018: Dutch Authority NZA decision to **allow reimbursement of compounded products even if authorised alternatives exist.**
- 1 February 2019: introduction of the **'pharmacist exception'** into patent law: even if pharmaceutical companies possess the patent on a particular formulation, **this exclusivity cannot be evoked against the pharmacists** who develop the same kind of product through compounding techniques.
- 7 February 2019: **general debate took place in the Dutch Parliament** on SPC, the legal framework for compounding and the compulsory licensing.

As a consequence, it can be noted that in the Netherlands the regulatory framework has changed to favour compounding. Nevertheless, it might be possible that the reimbursement of compounded drugs and the definition of small scale will be referred to the Court of Justice, which will have the final word on those issues.

However, it should be borne in mind that in the Dutch law, appeals have no suspensive effect unless there is an injunction procedure, even though it seems quite difficult that this will happen in this case. At the same time, there is strong public pressure in the country against high prices for drugs.

7. GERMANY: CABINET POSITION ON THE DRAFT LAW FOR MORE SAFETY IN THE SUPPLY OF MEDICINAL PRODUCTS (GSAV)

Willi Schnorpfeil (Value & Dossier) presented a thorough overview of the potential implications of the draft Law GSAV in the German pharmaceutical market, giving some insights about how the orphan drug market and the pharmaceuticals market might be affected.

[Refresher] The German Ministry of Health presented **a draft Bill in late November 2018** (cf. Members email of Alexander Natz, dated 19 November 2018), which was slightly amended into a **Government position adopted on 31 January** (cf. Members email of Oliver Sude, dated 30 January 2019). If the legislative process goes as expected, the **Bill is expected to come into force in July 2019.**

Main changes include:

- **OMPs**:. Once a new OMP introduced in the German market exceeds the 50 Mio threshold, a new benefit assessment has to be initiated by the pharmaceutical company itself, regardless of the fact that the drug in question is used in hospital or in an outpatient context. Therefore, **with this proposal, hospital products are integrated in this procedure which previously only concerned outpatient sales.**
- **Registries:** the Cabinet agreement foresees **the establishment of new registers for OMPs and drugs with conditional approval and with approval under exceptional circumstances**. These registries have to be set up and financed by manufacturers. They will serve to **gather new data** and to **perform recurring benefit assessments** to prove the effects which are associated with these drugs. In case it is not possible to prove the benefits associated with the medicinal products, a renegotiation of the price will take place. **The G-BA will be responsible for the definition of such registries.**
- **Other important implications**: through the establishment of these registries, the Cabinet agreement foresees to make the country able to gather more long-term data and new evidence for patients getting G&C therapy and at the same time to combine this information with payment models. **However, what is currently missing in the law is the link to hospital reimbursement**. Furthermore, the whole calculation for reimbursement is based on annual therapy cost, which is not appropriate for G&C Therapy. **As a consequence, there is still room to modify the law before its final approval, and the industry should take action in order to influence the latter**.
- **Biosimilars Market in Germany:** So far, the exchangeability of biosimilars by pharmacies has not been allowed, while this is possible for generic products. **The new law would allow pharmacies to exchange those products too, and the G-BA is going to define the exchangeability of biosimilars**. Criteria for this procedure will be developed, and the entire process will probably take three years to be set up.
- **Parallel Import:** a new incentive scheme has been defined, with the introduction, for medicinal products that cost less than 300 €, of a price difference to the original product of at least 5%. This will allow health insurances to have more additional savings for expensive drugs.

8. MARKET ACCESS FOR ORPHAN MEDICINAL PRODUCTS IN THE UK

Josie Godfrey (JG Zebra Consulting) shared an update on the current situation in the UK on the healthcare reimbursement system, focusing on reimbursement for OMPs in England and Scotland (cf. slides 77 to 102).

The Office for Health Economics (OHE) in its 2017 report underlined the fact that the UK is falling behind in both numbers of OMPs and time to reimbursement. Indeed, both STA and HTA are long procedures and, as a consequence, some OMPs are stuck in the process or are facing challenging negotiations. One of the most challenging aspect of the current situation is that for NICE there is no case for taking rarity alone into account. Therefore, NICE does not recognize evidential challenges for most rare diseases.

In addition, some changes might take place in 2019, which include: charging fees for appraisals, new more proactive commercial team and a possible revision of the methodology, in particularly for PPRS and HST, which is likely to start this year.

Concerning Scotland, in 2018 a new pathway for ultra-orphan medicines was announced. This implies that if a new medicine meets the criteria of the new definition, it will be made available on the NHS for at least **three years** while information on its effectiveness is gathered. Although this action might be interpreted as a clear indication of intent to make more treatments for ultra-rare conditions available in Scotland, **it is yet too early to foresee the scale of the real implications**.

9. ANALYSIS OF THE EU OMP REGULATION: AN UPDATE

A. EU DEVELOPMENTS

Delphine updated the group of the current developments of the EU Regulation concerning OMPs.

The Technopolis Study, commissioned by the EU Commission to provide an analysis of the OMP Regulation and the EU orphan drug market, is expected for finalisation by April 2019. More information on the potential timeline for the next year can be found on slide 107.

According to EUCOPE's Incentives Steering Group (ISG), it is expected that a revision of the EU Regulation n° 141/2000 on OMPs will formally be done in 2020.

Post-meeting addendum/revision: According to a 14/02 discussion with Commission officials,

- The study is unlikely to be released immediately after finalisation and as a standalone document.
- The EU Commission plans to organise a conference with stakeholders around June 2019, the outcomes of which will feed into the evaluation of the combined effects of the EU OMP and Paediatric Regulations.
- As a consequence, EUCOPE expects that this EC evaluation will be published in Autumn 2019.

B. EUCOPE STUDY

Against this background and the risk that the Technopolis Study on OMPs will not investigate some key aspects of the OMP Regulation, **EUCOPE** – in cooperation with the Office of Health Economics (OHE) - **has decided to conduct its own study on the matter**, in order to complement the evaluations which are and will be done by the EU authorities.

The EUCOPE study will be composed of three main pillars:

1. The profitability of developing OMPs
2. The value of OMPs to patients & society
3. Competition in orphan indications

More information on each of the pillar can be found on slides 111 to 114. A **first draft** of the study will be available for EUCOPE members **in May 2019,** and in **early June 2019** the **final version** will be released.

C. EUCOPE ENGAGEMENT

Besides its own Study, **there are four key activity areas where EUCOPE can make a difference in the future EU developments on the OMPs:**

1. **Broaden the scope of the debate –** (EUCOPE should not focus only on the OMP regulatory framework but it should also - for instance - understand how it can address the 4 pillars of EURORDIS' 2018 WhitePaper "Breaking the Access Deadlock to Leave No-one Behind')
2. **Build partnership with EURORDIS.**
3. **Engage with 'swing' Member States –** (i.e. those which are more likely to be in favor of the industry position, such as Belgium, France, Sweden and Germany).
4. **Build simple and comprehensive messages.**

10. EURORDIS MULTI-STAKEHOLDER SYMPOSIUM, 13-14 FEBRUARY 2019

A. PROGRAMME AND OBJECTIVES

Interestingly, **for the 3rd multi-stakeholder symposium** *on improving patients' access to rare disease therapies* **organised by EURORDIS,** industry participation will not be particularly strong in this event. Nevertheless, **EUCOPE** was **represented by Alexander Natz** at

- a panel discussion on constituencies' perspectives on how they see *the development and access to OMPs unfolding.*
- **Breakfast Session 3** on *A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impacts.*

Overall, **EUCOPE had to ensure that it has a clear strategy on the topics covered during the symposium.** A few members volunteered to support in the drafting of EUCOPE's speaking points, laying out which elements EUCOPE could agree with EURORDIS and on which ones it should have a more critical stance.

For more information, please refer to the speaking points and EUCOPE arguments herewith attached.

B. DRAFT ROADMAP

EURORDIS has formulated a draft roadmap which will serve to guide European and national institutions alike to improve the development of, and access to, therapies at all levels. **The document presents four main pillars,** which correspond to those of the EURORDIS 2018 White Paper (cf slides 133 to 141 and attached roadmap). On this roadmap too, **EUCOPE started developing a coherent strategy and the outline of a clear position**. Members interested to support EUCOPE with feedback and opinions on the way to proceed were invited to inform Delphine.

Next steps

1. EUCOPE to develop an effective strategy and coherent position on the EURORDIS multi-stakeholder symposium.
2. Members interested to support EUCOPE to inform Delphine Roulland.

NEXT MEETINGS

- 15 May 2019, 11.00 - 16.00, Brussels
- 3 July, 14.00 - 17.00 CET, teleconference (tbc)
- 9 October, 11.00 - 17.00, Brussels
- 10 December, 14.00 - 17.00 CET, teleconference (tbc)