9th Meeting of the Working Group on Pricing of Pharmaceuticals

Brussels, 12 of December 2007,

Draft Minutes

1. WELCOME AND PRACTICAL POINTS

The chair has been apologised for not being able to preside this meeting. The vice-chair, chaired the meeting. 25 Member States and 10 stakeholders were present (a list of participants is attached in annex). We welcomed new representatives from the Czech Republic, Finland and Sweden. Representatives of Eurordis and of Genzyme were welcomed for the session on orphan medicines (point 4.)

The proposed agenda of the meeting was adopted.

2. ADOPTION OF THE MINUTES MEETING 01/10/2007

The minutes of the last meeting had been distributed. One written comment came in from GIRP, i.e. to add their name as interested parties in one of the pilot projects. During the meeting, EGA also added its interest to one of the pilots. Listing of stakeholders for next steps in the trade and distribution (point 7.) was corrected.

The minutes were adopted after inclusion of these few remarks. A finalised version will be send.

3. FROM ASSESSING INNOVATION TO PRICING AND REIMBURSEMENT

This workstream aims to address a central question "How do assessments of innovative medicines translate into concrete economic rewards, mainly through pricing and reimbursement decisions?"

Although not always so easy to understand, some Member States have pricing and reimbursement decisions that take in specific ways account of the assessments of new medicines. Some of these approaches were presented to the entire group for debate.

- Overview - Introduction (EASP)

The experts of the Andalusian School of Public Health have studied 6 Member States where value assessments are being taken into account for pricing and reimbursement decision making (SE, UK, GE, FR, NL, BE). At hand of information collected in their previous study, literature and direct Member State contacts they have brought a high-level and structured overview of these Member State systems and how they translate value assessments into economic decisions. The key economic decisions studied were (1) pricing, (2) reimbursement level, (3) utilisation and (4) timing/uptake. In addition it was illustrated how several of these 6 Member State with (partial) value-based pricing are often referred to by other Member States in cross-border reference pricing.

- Country perspective (BE)

Belgium has presented in more detail how Belgium makes assessments of new medicines and how these assessments are used to make pricing and reimbursement decisions. In addition. some statistics were presented on the outcomes of these assessments made (level of innovation, nr of "breakthroughs", etc) and a comparison was made with outcomes of added therapeutic value assessments in other countries. The BE system seems in particular to be much in line with the methods and outcomes in NL and FR. Several clarifications were given on the Belgian Committee procedures.

- Discussion – Next steps

Several interesting discussion points came up, in particular:

- Value assessments of new medicines usually rely on cost-effectiveness which is more feasible than assessing cost-utility for which it is very hard to collect the necessary data. At moment of initial evaluation, several Member States look to confirm some elements (efficacy, safety) with the data brought forward, other elements (applicability, effectiveness, convenience) are assessed in an exploratory way, requiring a later confirmation.
- Assessments could normally be expected to lead to the same results in all EU Member States given similarity of patient profiles. Nevertheless, it was clarified that different systems and requirements applied per Member State. Companies therefore have to adapt their approaches towards each individual Member States. In addition, there are many local differences like health needs or the level of resources available, which each can explain different final pricing and reimbursement decisions.
- The current price levels Europe are often considered too high for those Member States with a lower GDP/capita and therefore creating affordability issues. Although individual setting of prices for each Member State would be most beneficial for both patient access and company turnover, the current free flow of goods does not allow this today within the Single Market.
- The presented data (from BE and FR) showed that only in less than 10% of evaluations a major added clinical benefit was identified in the assessments. Though it needed to be clarified that many evaluations cover minor changes/line extensions of existing products/molecules. Where it really concerns new molecular entities (NMEs) the level of major added clinical benefit is significantly higher.
- A debate followed on cost-based pricing versus value-based pricing. Cost-based pricing would bring and require more transparency of R&D costs for each medicine, which in practice is difficult to obtain. A key feature of cost-based pricing is the fact that it rewards efforts rather than the guarantee for result. Therefore this might lead to costly but ineffective funding. Companies usually apply value-based pricing. EFPIA's representative will bring an overview of how this works in one of the coming sessions.
- Some interest was raised by the French assessment system, which leads to graded outcomes in 5 different levels (ASMR I-V) and therefore allows recognition of different levels of incremental innovation.

è As next steps, it was agreed to develop a short descriptive report of the ways in which Member States translate value assessments into economic decisions (pricing, reimbursement, utilisation, timing/uptake). The 6 studied Member States will provide further inputs. Other Member States and stakeholders can provide additional inputs; A draft report will be presented to a future session of the Working Group Pricing for comments/suggestions. The finalised version might provide new inputs for a new version of the Guiding Principles and/or for the Final Report.

4. ORPHANS

The field of orphans highlights well many of the previous debates in the Working Group, as Orphan medicines are usually (1) very innovative with high added value ("breakthrough"), (2) there are often difficulties of equitable access for patients over Europe and (3) often there is significant stress on the Member State budgets. Objective of this session was therefore to bring forward the "main issues/barriers to come to an effective and equitable use of orphan medicines over Europe ". The session contained 3 speakers, each bringing a different perspective on orphan medicines (patients, payors and industry).

- Introduction (EC)

To open the session, the Commission brought a short introduction on orphans, including economic and regulatory aspects.

- Patients perspective (Eurordis)

The presentation started with a general overview of definitions, the EU regulation and numbers and types of developed orphan medicines. The presentation focused then on high variation in access to orphan medicines between individual EU Member States. Centralisation on the EU-level was highlighted as driver for increased development and registration of orphan medicines. De-centralised processes, like value assessments and pricing and reimbursement, were indicated as main drivers for the variety in access. Increased collaboration between Member States was suggested as a key way forward, in particular to come to joint value assessments and thus collect the small number of patients of multiple Member States. Eurordis suggests that a European reference price could be negotiated, as basis for the national pricing and reimbursement decisions.

- Payer perspective (ESIP)

This presentation highlighted the main problems with orphan medicines from a payer perspective. In particular the lack of good evidence on the value of orphan medicines, the very high prices and consequent budget impact. The importance of the varying levels of national GDP in the EU Member States was also highlighted. ESIP called to all parties to contribute to ways forward, e.g. by increasing transparency and get better data/use data better or by organising controlled funding (risk-sharing agreements, centers of excellence, …). In addition, some first long-term ideas were put forward covering different areas like regulation, funding and in particular and increased collaboration between industry, payers and health professionals in the development and use of or phan medicines. This should lead to an earlier and better understanding of the value of new orphan medicines.

- Company perspective (Genzyme)

The presentation reminded the spirit of the EU orphan regulation, i.e. to provide timely and equitable access to therapies for rare disease patients, and to balance the development risk by providing sufficient economic incentives to companies. The presentation illustrated the risks and difficulties to develop orphan medicines, largely attributable to the very small numbers of potential patients. These risks go farther than development, as in contrast to other drug areas, companies need to take the responsibility to keep orphan medicines on the market while there is no alternative for the patient. In particular SME's are very vulnerable to these risks. It is also the low number of patients that requires higher prices for orphan medicines so that a sufficient market is created. Without sufficient market no company is able to develop orphan medicines.

- Discussion – Next steps

Several interesting discussion points came up, in particular:

- It was clarified that one of the main reasons for delays in access to orphan medicines in some Member States lies in the complexity and variety in procedures of registration, evaluation, pricing and reimbursement. In particular SME's do not have the capacity to introduce their newly developed orphan medicine to all EU Member States and are therefore forced to focus on the most important markets.
- It became clear that it might be very efficient to collaborate between Member States to do value assessments for a medicine which treats only some hundreds of patients in the entire EU. There was interest in exploring the idea of collaboration amongst Member States in the field of orphan medicines.
- Although pricing and reimbursement discussion are to be taken by each Member State individually, Eurordis suggested there could be value in joining the small national volumes of orphan medicines in order to negotiate a EU reference price basis.
- Although the cost of clinical trials is normally driven by the high number of patients, trials with very few orphan patients also seem to be very expensive. The costs are in this case driven by the need to screen and identify rare patients and then bring the few identified patients together in global clinical trial centers.
- The orphan designation of some 'blockbuster' drugs was questioned. While it is exactly the objective to incentivize and protect the development and sales of medicines with a small market potential, offering these incentives and protection for medicines with more than 1 billion$ annual sales seems rather to put at risk the sustainability of the entire orphan idea and related incentive programmes.

è As next steps it was agreed to focus on 3 areas: (1) the cost of developing orphan medicines, (2) the issues with assessing them and (3) the existing problems creating an inequal market entry/access in the EU. A short descriptive report will be created in order to understand these issues and put some potential solutions forward. Eurordis, EuropaBio, FR, Sl, IE will prepare a first draft of this document together with the secretariat.

5. PILOTS ON GUIDING PRINCIPLES – RISK SHARING

Risk sharing practices are those pricing and reimbursement practices where the cost of new treatments is shared between companies and authorities, in function of the benefits realised by the treatment.

Risk Sharing practices are increasingly explored by Member States who see them as potential ways in order to at the same time ensure (1) access to potentially high-added value medicines and (2) control the total spending of these medicines. Companies see them as an opportunity to get (3) a return on investment where the existing proof of value can not yet be fully demonstrated.

- Introduction - overview of some practices identified (EC)

In preparation of this meeting, several Member States have sent more information to the secretariat on their specific experiences with cost-sharing and conditional approval. The secretariat presented a short overview of 4 experiences (in BE, NL and UK (2x)) on how to deal with uncertainty of the potential high value of new medicines. In all experiences the set up of a risk-sharing practice was triggered by (a) a doubt about (cost-) effectiveness of a new medicine plus (b) an expected significant benefit and/or a severe disease without a satisfactory existing treatment. Further commonalities lie in controlled settings of utilisation, pre-agreed timings, pre-agreed output-expectations and pre-agreed consequences.

- Country example (NL)

The Dutch representative has brought an illustration of a specific conditional pricing mechanism that has been set-up in the Netherlands. It includes temporary funding to use very-innovative medicines in hospitals, on the condition of controlled use which is restricted to some expert centres and on condition of developing convincing proof of the value of these medicines. These hospital expert centres have 3 years time to do research in order to collect the additional data needed to develop this proof of the value of innovative medicines. This proof of value is the condition to continue the further funding of these medicines.

- Discussion - Next steps

Several interesting discussion points came up, in particular:

- There was some concern on the fact that the increasing use of these practices would take away the obligation of companies to deliver full proof of value/evidence for new medicines. It was made clear that these practices should apply in very restricted situations where significant value is expected but needs further proof, while some patients should be allowed to use the medicine because of lack of alternative. The discussion made a distinction between a situation in which (1) there is lack of evidence versus a situation in which (2) there is evidence, but no proven cost-effectiveness. The real value of these deals lies in the collection of additional information that allows us to work with a potentially valuable medicine.
- Some concerns were expressed regarding the consequences of the pre-agreed future (re-)evaluations. Companies fear that the only practical option after a future evaluation will be directed downwards, i.e. price reductions or restrictions in utilisation, but never upwards. Payers fear that strict actions like de-listing might not

be feasible in practice given that patients are relying on these medicines. Though this last fear seems to be relative as a de-listing would apply on products with insufficient benefit which therefore anyhow will be less used.

è As next steps it was agreed to develop a fact-finding report bringing an overview of how different risk-sharing/conditional approval practices are set up and experienced. The presented mechanisms will be included. Member States and companies with additional experiences are also invited to provide their experiences. A draft report will be presented to a future session of the Working Group Pricing for comments/suggestions. The finalised version might provide new inputs for a new version of the Guiding Principles and/or for the Final Report.

6. PILOTS ON GUIDING PRINCIPLES – OTHER

During the last Working Group some participants expressed interest to develop some additional pilot projects. These participants have sent the secretariat their proposals in written, which were forwarded to the participants of the Working Group. The proposals have been distributed and are presented to the Working Group.

It was made clear that the proposals do not envisage to build complete studies but rather to bring together different existing sources/materials to organise a discussion in one of the future sessions of the Working Group Pricing to build common understanding. Also, it needs to be clear that while a proposal can be presented and taken forward by one participant of the Working Group, the final findings/conclusions will be of the entire Working Group and can/will therefore be different from the views of the coordinating participant.

- Proposal generics and free price competition (EGA)

EGA has proposed to deepen common knowledge on 2 of the Guiding Principles (use of free price competition, alignment of demand side actors) as generics offer a good field to do so. A first analysis would compare how different Member State organise price-control/free pricing of generic medicines and what this means for the price-levels of medicines. A second analysis would compare how different Member State organise/influence demand-side behaviour of patients, doctors and pharmacists and what this means for the uptake of generic medicines. These analyses will build on existing studies/materials and consider different situations in the EU Member State. EGA will take the coordinating role together with the WG secretariat. EFPIA, MT, BE and PL agreed to join in the effort.

- Proposal Tendering (ESIP)

ESIP has proposed to develop further knowledge on the use of tendering procedures and the experiences with the potential benefits and risks. While in ~15 Member States hospitals are expected to apply tendering, only few Member States use tendering for non-hospital medicines. BE and NL use tendering to define prices for the retail-distributed medicines. CY and MT rely on tendering for non-hospital medicines supplied by the public authorities. This proposal will try to cover both the hospital sector and the retail sector. ESIP will check possibilities with an external expert (ÖBIG) to collect a first overview of existing tendering practices and their characteristics. This should allow to

build a map on where/who is applying tendering in the EU. A questionnaire might be developed to collect further info from the participants of the Working Group.

7. OTHER WORKSTREAMS

- Proposal on Trade and Distribution (ESIP-AIM-PGEU-GIRP)

AIM, ESIP, GIRP and PGEU have jointly proposed to build some better common understanding of the distribution landscape and expected impact of some ongoing changes in distribution like e.g. direct-to-pharmacy distribution or short-line vs full-line wholesaling. The common underlying objective of distribution systems is to ensure that universal access to medicines is organised in a cost-effective manner. Some ongoing or expected changes might significantly impact this objective. The 4 stakeholders will further prepare for a future session building some more common understanding and list the different hot topics of interest they respectively want to elaborate In preparation of this session a questionnaire might be sent to Member States to collect further information.

8. ANY OTHER BUSINESS

An e-mail including practical follow-up and next steps will be distributed in the coming days after the session.

EPF drew attention to an outstanding consultation by the United Nations on Human Rights Guidelines for Pharmaceutical Companies in relation to Access to Medicines. The link with information will be forwarded by the Secretariat. Comments can be sent until 31 December 2007

The next meeting will take place on 13-14/2/2008.

All the best for the next year

9. CLOSING