Meeting of the National Authorities on Pricing and Reimbursement and Public Healthcare Payers
12 December 2022, 9H30-15H00 CET
(Hybrid: Brussels/Webex)

OUTCOMES

During the opening, the *Commission* underlined the importance of the NCAPR as a forum for collective thought and action to promote affordability and assist national authorities in respect to their national competences. In line with this, a progress update on the NCAPR 2021-2023 workplan was shared, providing an update on the important developments on biosimilar competition and the negotiations workstream.

Session 1 - Working together: outcome-based networking

The physical meeting of the NCAPR aimed to promote a more participatory approach and reinforce better interaction and networking opportunities among the members as well as identify further opportunities for cooperation in the area of orphan medicines. Using participatory leadership techniques, and with the support of the Commission (DG.HR.A3), the group identified their learning objectives for the day, the challenges encountered for orphan medicines pricing and reimbursement and the areas where greater cooperation could make the biggest difference. Participants regarded the **vicious circle of low evidence (high uncertainty) and high prices** as the (current) greatest challenge for orphan medicines. Consequently, the NCAPR saw strong **collaboration potential** in establishing a more **demand-driven approach** where payers identify unmet medical needs and define their willingness to pay upfront to become active buyers. The group also highlighted the potential for cooperation on **data sharing** (on clinical data, but also on willingness to pay and P&R status), and on exploring joint negotiations, joint horizon scanning and joint procurement.

Session 2 - Key figures on access to medicines for rare diseases in the EU

To provide some background on the current trends and challenges for orphan medicines in the EU, *the Commission* first presented some initial data demonstrating that the share of orphan medicines in the EU's pharmaceutical expenditure has grown persistently and substantially. This upward trend is the result of the growing number of orphan designations and authorisations and their associated high R&D and production costs. The revision of the pharmaceutical regulation, therefore, serves several policy objectives, and aims to find a balance between innovation, access and affordability.

The countries' experiences underscored the observed trends in the EU, with Czechia, France, Austria, Portugal, and Germany reporting a substantial growth in total expenditure and delay in timely access, patent- and SPC-enabled extensions of the protection period for the majority of high price products, and that competition typically brings sales up and prices down but also increases access for patients. However, there is a growing number of orphans that do not see generic competition at protection expiry (considered as a 'market failure').

From the discussion, the NCAPR identified **key data gaps** (i.e. clinical data, launch dates, volumes, defining value) and **lack of alignment** (i.e. orphan definition/ status) were found to hinder effective cooperation. Greater horizon scanning (reactive to proactive) and (timely) information sharing are expected to address these gaps and will form the basis for further discussions.

Session 3 - Revision of the EU Regulatory Framework for orphan medicines

The Commission briefly introduced the main objectives of the revision of the regulatory framework on medicines for rare diseases, namely: stimulate innovation and developments in areas of (high) unmet medical needs and improve affordability and patient access.

ESIP/MEDEV followed with a presentation of suggested amendments to the regulation, namely a downward adjustment of the prevalence criteria (5->1/10,000) to focus incentives on development

into truly rare diseases, clarifying the definition of 'significant benefit'**,** maintaining the profitability criterion in the context of the re-evaluation, and strengthening the criteria for maintaining market exclusivity (including submission of P&R applications in EU27 within 2 years).

During the discussion, several NCAPR members confirmed their support for the ESIP/MEDEV proposals. *The Commission* explained changes such as early dialogue that will support downstream decision makers and other measures for access and affordability (including incentives and earlier generic entry). However, some NCAPR members were calling for a more restrictive application by limiting the scope of the legislation to ultra-rare diseases (prevalence criteria 1 in 10.000 compared to the current 5 in 10.000) and by maintaining the profitability criterion. During the meeting, the Commission explained that those changes were not supported by the evidence and the consultation activities, in particular the views of the patients.

Session 4 - National pricing and reimbursement pathways for orphan medicines

The *Czech Presidency* thanked those who participated in the EU survey ahead of the meeting. Of the twelve replies, only Belgium and Czech reported different processes for orphans and non-orphans, while six MS (AT, FR, SE, BE, NL, and CZ) further mentioned other pathways aimed at facilitating access for groups of products other than orphan medicines (such as ATMPs and inpatient-only medicines). Interestingly five out twelve MS (AT, BE, SE, NO, DE) reported to have collaborated with other MSs in either joint assessments or in joint negotiations for orphans.

Individual countries then briefly presented their national procedures for orphans with *Czechia* presenting their new P&R system for orphans which introduced an appraisal committee, increased patient involvement, and adopts a societal perspective for health economic analyses of orphan medicines. Similarly, *France* introduced a new P&R system consisting of a framework agreement between the economic committee for health products (*CEPS*) and the pharmaceutical industry (*LEEM*) aiming to increase the attractiveness of orphan medicines. Germany also aims to maintain incentives for the development of new orphan medicines, adapting their P&R conditions to uphold good and fast access to orphan medicines. Lastly, Austria and Denmark, who do not have any dedicated procedures, shared tools and mechanisms, such as conditional reimbursement and managed entry agreements respectively, that can be used to handle uncertainties when assessing orphan medicines.

Session 5 - Key take-aways and next actions

In conclusion, participants called to **action** on developing a common data set on market status in MS and willingness to pay and planning further workshops to exchange information on willingness to pay for specific molecules in the pipeline (building on horizon scanning) and on becoming 'active buyers' (promoting a more forward looking, strategic approach).

Conclusions – Next steps

The co-chairs thanked the 37 participants from 21 MS and Norway and noted the next meetings in 2023

- 29-30th March NCAPR Plenary meeting in Stockholm under the Swedish Presidency
- May/June, Dissemination event on PPM study