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On 19 November 2016, the CPME Board adopted the 'CPME policy on access to medicines and pharmaceutical pricing' (CPME 2016/063 FINAL)

CPME policy on access to medicines and pharmaceutical pricing

The Standing Committee of European Doctors (CPME) represents national medical associations across Europe. We are committed to contributing the medical profession's point of view to EU and European policy-making through pro-active cooperation on a wide range of health and healthcare related issues[1].

The best possible quality of health and access to healthcare, which include access to the best medicinal treatments available, should be a reality for everyone. However, the availability of medicinal products does not necessarily mean that patients have access to them. The affordability of medicinal products, especially innovative medicines, is therefore crucial. Otherwise, equal access for all in need is in jeopardy. Lack of research in some areas (e.g. chronic conditions such as diabetes) and drug shortages due to parallel import or drug withdrawal can also impair patients' access to essential medicines.

Affordability of innovative medicines

Nowadays, the principle of equal access to medicines is called into question at a time when soaring drug prices can be observed across Europe and beyond. Over the last decade, more targeted treatments, in particular in the field of oncology and hematology, have been introduced in clinical practices but they have come with an ever increasing and unsustainable price[2].

An alarming example is the price of drugs for certain types of cancer, such as chronic myeloid leukemia. In ten years, the price of new cancer drugs has more than doubled in some countries[3].

Impact on sustainability of healthcare systems and clinical practice

While European countries are facing significant constraints or even cuts in their health budgets coupled with a growing demand for healthcare, the increasing burden of health expenditure related to medicinal products could threaten the overall sustainability of healthcare systems. In order to

[1] CPME is registered in the Transparency Register with the ID number 9276943405-41. More information about CPME's activities can be found under www.cpme.eu

[2] Experts in Chronic Myeloid Leukemia, The price of drugs for chronic myeloid leukemia (CML) is a reflection of the unsustainable prices of cancer drugs: from the perspective of a large group of CML experts. *Blood.* 2013 May 30; 121(22):4439-42.

[3] WHO Regional office for Europe, Access to new medicines in Europe: technical review of policy initiatives and opportunities for collaboration and research, March 2015.

contain this surge in prices, national health systems have adopted various approaches when it comes to their pricing and reimbursement (P&R) policies.

For instance, some national authorities have decided not to reimburse some costly treatments on the basis of socio-economic indicators whereas other countries have chosen to ensure access only for those patients who are the most severely affected by a disease, i.e. hepatitis C treatment which has been restricted to patients presenting the most advanced stages of hepatitis fibrosis or HIV co-infection[4]. This illustrates the impact of prices on clinical practice and ultimately on health inequalities between patients not only across Europe but also within national healthcare systems.

In this respect, the fact that doctors may have to wait patient's health status to deteriorate before providing necessary and medically indicated treatments raise severe ethical questions in terms of nonmaleficence and equity. CPME believes that patients have the right to healthcare and to receive the best treatment available, regardless socio-economic conditions. It is the medical profession's obligation under professional ethics to serve the patient's best interest.

Recommendations

CPME welcomes the recent Council Conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States from 17 June 2016, adopted under the Dutch presidency, which draw up an accurate inventory of the current challenges related to unsustainable drug prices and which identifies a number of areas of work, such as the investigation by the Commission of the existing incentives under the current EU legislation and further collaboration within the HTA network. These findings are rightly reflected in the draft report of the European Parliament on EU options for improving access to medicines, prepared by MEP Soledad Cabezon-Ruiz.

Indeed, CPME recognises the need to have a comprehensive overview and revision of the existing regulatory framework to tackle the issue of affordability of medicinal products, starting with the revision of the current model of incentives to an enhanced transparency.

When conducting this work, CPME recommends including further considerations on:

- Revision of the orphan drug regulation

Over the last decade, the number of orphan drug designations increased steadily. While the orphan drug regulation has provided incentives to develop medicines for rare diseases, experience has often shown that this increase is related to the stratification of diseases where a patient population is divided into subcategories, which individually meet the orphan designation criteria[5] [6]. This approach further caused extensions to new indications and/or other subcategories of patients to maximise profits. CPME doubts this situation is in accordance with the intention of the law.

CPME calls for the revision of the orphan drug regulation to: a) apply stricter rules for the orphan drug designation, b) ensure the reassessment of the orphan designation to take into account

[4] NICE, Sofosbuvir for treating chronic hepatitis C, Published date: 25 February 2015. https://www.nice.org.uk/guidance/ta330/, consulted on 22 September 2016

[5] Steven Simoens, Pricing and reimbursement of orphan drugs: the need for more transparency. Orphanet J Rare Dis. 2011; 6: 42.

[6] Denis A, Mergaert L, Fostier C, Cleemput I, Simoens S. Issues surrounding orphan disease and orphan drug policies in Europe. Applied Health Economics and Health Policy. 2010;8(5):343-50.

extensions and additional authorisations granted for the same medicinal product and c) revise the market exclusivity system.

- Early access should be restricted

The European Medicines Agency (EMA) launched in 2014 its first initiative on 'adaptive pathways' aiming at improving timely access to medicines that address patients' unmet medical needs[7]. The adaptive pathways approach has been developed to provide an early approval to a medicine for a restricted patient population, relying on a smaller evidence base. The market authorisation will then be further expanded as soon as more evidence has been gathered in the post-marketing phase.

CPME remains cautious with regards to this initiative as patient safety aspects may not be adequately taken into consideration. On the contrary, CPME believes that a high level of clinical evidence, based on extensive clinical data gathered under controlled circumstances, should remain the rule. Therefore, the adaptive pathways approach should be limited to situations where no other alternative is available and a strictly regulated framework will be indispensable to safeguard patient safety and health systems. Such regulatory framework, which should be based on quality, safety and efficacy standards, should foresee specific obligations and restrictions that should be carefully monitored and sanctioned in case of infringement.

- Transparency on costs and incentives

CPME considers transparency as a prerequisite for pharmaceutical pricing. The transparency should encompass not only R&D and manufacturing costs but also incentives, such as granted market exclusivity and received public investment at the stages of research and clinical trials. Given that a significant percentage of industry R&D is paid by taxpayers, governments should be encouraged to systematically request more transparency from manufacturers.

Whereas the drug regulatory system is highly complex and fragmented, there is a need to take into account and reflect in the final prices prior incentives that companies benefited from as part of the drug development. In this respect, the final price of a drug should be reasonable and not entirely disconnected from the public investment and the manufacturing costs.

- Multidimensional approach to pharmaceutical pricing

National approaches differ widely when it comes to define prices and evaluate different criteria such as the therapeutic added value or the value for society. However, sensitive national differences could still be taken into consideration when member states would be invited to participate in a voluntary cooperation on pricing.

Consequently, CPME favours a multidimensional approach to pharmaceutical pricing, based on health technology assessment (HTA), where various factors are taken into account from the therapeutic added value and the value for society to the ability to pay and the cost of drugs. Additionally, the priorities required in pharmaceutical pricing must first concern patient's rights, then be applied to solidarity in care determined by need, and finally concern cost efficiency. Finally, a better integration between HTA and scientific approaches right from the start is needed in order to address real unmet medical needs of the patients, ensure a better positioning of medicinal products in the therapeutic strategy but also ensure the sustainability of healthcare systems.

[7] European Medicines agency (EMA), Final report on the adaptive pathways pilot. London: EMA, 28 July 2016.