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PGEU Comments

RARE DISEASES: EUROPE’S CHALLANGES

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1. Introduction

The Pharmaceutical Group of the European Union (PGEU) is the European association representing community pharmacists in 30 European countries including EU Member States, EEA countries and EU applicant countries. Within the enlarged EU, over 400.000 community pharmacists provide services throughout a network of more than 160.000 pharmacies, to an estimated 46 million European citizens daily.

PGEU's objective is to promote the role of pharmacists as key players in healthcare systems throughout Europe and to ensure that the views of the pharmacy profession are taken into account in the EU decision-making process.

PGEU welcomes the European Commission's initiative to further develop EU action in the important area of rare diseases and the opportunity to submit comments to the consultation paper. In this submission, PGEU wishes to address only the questions where the intervention of community pharmacists can be valuable and significant.

2. Improving prevention, diagnoses and care of patients with rare diseases

Since rare diseases affect a small portion of society many health professionals do not have the necessary knowledge about diagnostics, treatments and counselling of these patients. They may easily not realise that they are facing a rare disease or if they do, what should be the best action to take. Therefore the gathering and dissemination of such knowledge is most relevant and urgent.

PGEU considers that a key element for improving prevention, diagnoses and care in the field of rare diseases is to provide accurate information in a format adapted to the needs of professionals and of affected persons.

It is often the case that in order to have proper treatment or follow up of their rare disease, patients need to travel long distances or even to change their living places. This causes additional stress, inconvenience and moreover requires financial investments. Again, disseminating knowledge and providing incentives to specialised training would allow a bigger range of health professionals, including pharmacists, to engage in providing better information and care to people suffering from a rare disease while raising awareness about rare diseases overall.

As identified in the consultation paper, there are few rare diseases for which primary intervention is possible. Nonetheless, pharmacists, due to their unique position of proximity and regular contact with the community can play an important role in raising awareness about environmental factors that may cause a wider range of rare congenital malformations, as well as childhood cancers. This is particularly interesting if we consider the fact that mothers, and women in general, are those who most often visit a pharmacy on a daily basis looking for advice regarding nutrition, obesity, alcohol intake, smoking, and use of medicines.

Furthermore, because pharmacies are the healthcare setting that can be most easily accessed throughout national territories all over Europe, awareness policies should take into account the impact of involving community pharmacies in specific campaigns or dedicated days to certain rare diseases. This can help building the necessary environment for people affected by rare diseases to be better integrated in the society.

3. Comments to specific questions

Question 4: Should the European Reference Networks privilege the transfer of knowledge? The mobility of patients? Both? How?

In PGEU's opinion the ERN should privilege the transfer of knowledge over mobility of patients as patients should, by principle, have access to care in their own country and in their own language. Travelling may be a health problem in itself for many patients and therefore it should be the knowledge to be mobile and not the patient as a general principle.

We believe the EC approach to prioritise cooperation and knowledge sharing between the national/regional centres of reference is the most appropriate. We would like to underline the importance of transferring the information not only within the network but to all healthcare professionals. To concentrate the information only within the centres of reference may limit the dissemination of relevant information which may help frontline healthcare professionals in early identification of people at risk of or already suffering from a rare disease as well as signposting the patient to these centres.

Question 5: Should on-line and electronic tools be implemented in this area?

PGEU fully supports the development of on-line and electronic tools to support action in the field of rare diseases. In order to make the most out of such tools, its development and implementation should take due account of patients and health professionals needs and investigate how they could be best integrated in existing IT solutions at the different healthcare settings level.

Question 6: What can be done to further improve access to quality testing for Rare Diseases?

A step to improving access to quality testing for Rare Diseases which is not mentioned in the consultation paper is the need for information on the available tests and screening strategies. Community pharmacists can once again have an important role in disseminating this type of information and signposting patients to the relevant healthcare professionals. To do so, the necessary information tools need to be in place and should be easily included in existing information systems in the pharmacy.

Question 8: Do you envisage the solution to the orphan drugs accessibility on a national scale or on an EU scale?

Patients should not be left with the concern of getting access to treatments and, in particular, fair treatments. This is the reason why PGEU considers that an EU scale solution should be envisaged because it widens the net and the use of these orphan drugs. However, national realities and competences should be respected in terms of access to medicines, namely with regards to pricing and reimbursement.

Notwithstanding the respect for the national medicines distribution systems it would be important to note how to have access to orphan drugs approved by EMEA but which are only marketed in some countries. Exceptions to national mechanisms of distribution should be considered for these specific medicines.

Question 9: Should the EU have an orphan regulation on medical devices and diagnostics?

In PGEU's opinion, the same arguments that led to an EU policy for orphan drugs, apply, in principle, to medical devices and diagnostics.

4. Final remarks

PGEU acknowledges the importance of shaping the future of rare disease policy at the European and national levels and would like to restate that community pharmacists are prepared to contribute to developing and implementing that policy.

We applaud the EU initiative for orphan drugs and look forward to see it further taken into account under the framework programmes for research and technological development, including what concerns advanced therapies strategies. We would also expect it to be expanded in the sense of covering as well medical devises and diagnostics.

PGEU also supports the discussion of initiatives envisaging the promotion of equal access to orphan drugs throughout the EU as well as the dissemination of knowledge about rare diseases.

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