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Foreword

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I welcome this new series on *"Health Technology Assessments and Rare Diseases Therapies"*. As the use of health technology assessment increases, this is an important opportunity to reflect on the progress that can be made at European level to help the many millions of sufferers of rare diseases in Europe.

Currently, between 5 000 and 8 000 different rare diseases affect around 6–8% of the total EU population, or around 29–36 million Europeans. The relatively limited number of patients affected by specific rare diseases, and the fragmentation of knowledge about them, makes this a prime example where working at European level is not only beneficial, but even necessary.

The European Commission is playing an active role by helping patients to receive appropriate and timely diagnosis, information and care by supporting Member States in their efforts to work together and pool their knowledge, expertise and resources in this important area.

Support for EU-wide cooperation on health technology assessment has been ongoing for over five years. For example, EU funding has helped to establish a European network linking HTA agencies. Within this network, a common methodological approach to HTA has been developed. This model simplifies the re-use of HTA information made in one country and offers the potential for task-sharing between Member States. This is clearly more efficient than each Member State working in isolation.

European Reference Networks

We hope to build on this valuable work with the newly adopted **EU Directive on the application of patients' rights in cross-border healthcare** (**Directive 2011/24/EU**).

The Directive foresees the development of **European Reference Networks** and the adoption of criteria and conditions that healthcare providers should fulfil in order to be part of those networks.

Such networks should have the capacity to provide complex or highly technological diagnostic or treatment services for medical conditions requiring a particular concentration of expertise, resources or technology, as is the case for many rare diseases.

This would improve access to high quality and highly specialised expertise across Europe enabling the mobility of medical expertise, particularly when patient or professional mobility is not possible.

EU cooperation on HTA

Currently, the EU co-finances formal intergovernmental co-operation on HTA involving almost all Member States. Through this cooperation, HTA agencies will use the "HTA core model" they have developed to assess different kinds of health technologies. By testing this model through a significant number of pilots, we will gain important insights into how Member States can cooperate most effectively in this field.

From 2013 onwards, HTA co-operation will move from *a project-driven* to a *regular* network. The EU Directive on patients' rights in cross-border healthcare defines HTA as one of the key areas for co-operation between Member States. The European Commission's ambition is that the future HTA network should establish both costeffective and time-effective ways to cooperate on HTA. This could result in less duplication of work between Member States when assessing the clinical effectiveness of health technologies. It could also benefit companies as it establishes more common standards for industry submissions for reimbursements in Member States. And most importantly, it could benefit the patients by providing broad and timely access to valuable health technologies.

National Plans and strategies on rare diseases

In parallel, Member States are elaborating – and some have already adopted – national plans or strategies on rare diseases. The European Commission-funded EUROPLAN Project (European Project for Rare Diseases National Plans Development) provides valuable support to Member States to set out clear guidelines, indicators and content for their national plans. The project will be further developed through formal intergovernmental cooperation which is expected to be launched later this year, with the clear objective of providing technical assistance and training for Member States.

Investment for the future

The visibility and inventorying of rare diseases still need to be improved. This is why the European Commission launched another Joint Action last year to support and broaden the ORPHANET database, the world reference database on rare diseases. The European Commission has so far funded this database with €6 million so that rare diseases are adequately coded and traceable in all health information systems. Any patient or health professional can receive information on all the clinics, hospitals and specialists able to treat the disease in all EU Member States. It also provides information on orphan medicines currently available, ongoing clinical trials, best practices, information on patients' organisations and registers. Information on 5 868 rare diseases are already available on this database.

The European Union is also a major funder of research on rare diseases. The main financing instrument for research at EU level is the 7th Research Framework Programme covering the period 2007–2013. It has so far invested €75 million in 20 research projects on rare diseases across the EU.

Empowerment

To succeed in any policy on rare diseases we need to listen and learn from patients themselves. Member States are encouraged to closely consult patients' representatives on policy development; facilitate patients' access to updated information on rare diseases; and promote the important activities of patients' organisations. For its part, the European Commission has been working closely for many years with organisations such as EURORDIS, the European organisation representing patients of rare diseases, and will continue to do so.

Solutions

The area of rare diseases is clearly one where assessing new treatments is particularly challenging. The low number of patients at Member State level makes it difficult for manufacturers of new medicines to make optimal clinical studies. HTA agencies similarly straggle to assess new treatments where few or no alternatives exist. As a consequence, it often takes time before Member States make decisions on the use and reimbursement of new rare disease treatments.

Rare diseases is therefore an area where increased EU cooperation is essential. Cooperation may increase the total number of patients available to participate in research and development of new treatments. This equally applies to HTA as joint approaches to assessing treatments for rare disease patients can allow for better access to patients' data.

To explore these possibilities, rare disease medicines could be among those piloted in further formal inter-governmental cooperation on HTA.

Earlier this year, the European Commission commissioned a study by Ernst & Young to explore mechanisms for a more effective introduction of orphan medicines onto the markets of EU Member States. This study will look at the different actors involved in the process leading to an orphan medicine being given to a patient – such as the European Medicines Agency, HTA agencies and national health insurers.

It will suggest alternative and/or improved mechanisms for better co-ordination between all actors involved in the process. Ibis is particularly pertinent considering that today, it often takes too much time at national level to decide on the actual use or non-use of a new orphan medicine following the marketing authorisation given by the European Commission. The study will be finalised later this year. On the basis of this study, the Commission will consider if further action is required.

EU initiatives on rare diseases can support increased EU-wide cooperation on HTA and improved treatment and hence a better quality of life for rare disease patients all over Europe.