Press release

Rare disease community calls for decisive steps for an agile European HTA framework

EURORDIS welcomes German Presidency's compromise on HTA, but urges further review to ensure it does not fall short for patients

14 December 2020, Brussels – As the Working Party on Pharmaceutical and Medical Devices meet today to discuss the *Proposal for a Regulation on European Cooperation on Health Technology Assessment*, EURORDIS-Rare Diseases Europe asks them to take this opportunity to progress this legislation further.

EURORDIS - Rare Diseases Europe welcomes efforts so far by the German Presidency to significantly improve the Proposal for a Regulation on European Cooperation on Health Technology Assessment (HTA). In particular, the greater clarification of the scope of the Proposal, the creation of a single centralised "data stop" and the recognition of the integration of Joint Clinical Assessment in the national assessments are recognised as significant steps forward.

Nevertheless, EURORDIS sees opportunities to refine the text further for the sake of a rapid launch of the last phase of negotiations in the Trilogue, between Commission, Parliament and Council, and in order to deliver for the 30 million people living with a rare disease in Europe, for whom the HTA process is critical in improving access to treatments.

> **François Houÿez, Information & Access to Therapies Director & Health Policy Advisor, EURORDIS-Rare Diseases Europe** commented:
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> "Thanks to the work of the German Presidency we are now a step closer to the approval of this Regulation. We want this to succeed. However, that will only be possible if it creates a framework for an agile and effective cooperation, in the spirit of mutual trust and learning-by-doing.
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> "The Regulation should avoid too rigid and complex procedural constraints. Once fixed by law, those may risk to undermine the implementation of the text. What has already been achieved by the European Cooperation in EUnetHTA Joint Action 3 must serve as a basis for further improvements and not for a regression."

In the final stage of discussions within the Working Party on Pharmaceutical and Medical Devices today, EURORDIS encourages due consideration to the following outstanding points:

- **Rare Diseases should have their place in the scope from the start,** as the field where European cooperation in HTA reaches its highest value, both for patients and Member States, due to the challenge that rare diseases therapies may represent to national HTA and the need to pool expertise and information.
- **The future Coordination Group should be free to define the procedural steps and timing, ready to adapt and to fit with its evolving capability and expectations**. These aspects should then be kept for delegated and implementing acts, outside of the text of the law.
- **Aspects already evaluated by the European Medicines Agency for marketing authorisation should not undergo any re-assessment.** EU taxpayers' money cannot be spent twice on the same work with risk of inconsistency.
- **The possibility left-open to Member States to re-assess large parts of the EU reports should be limited to what is strictly necessary, to avoid any duplication.**

- **The European Commission should be given the means and the legal power to ensure the correct working of this cooperation, as they are accountable to the European citizens and the European Court of Justice for this.**

Now that we are closer than ever to this Regulation being adopted, EURORDIS hopes to see these contributions taken into account to ensure this legislation works to improve patients' access to the treatments they need the most.

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EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 944 rare disease patient organisations from 73 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit eurordis.org.

Rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date, affecting an estimated 30 million people in Europe and 300 million worldwide. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

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