



[Press release]

Rare disease community welcomes proposal for future European cooperation on Health Technology Assessment

31 January 2018, Paris – EURORDIS-Rare Diseases Europe, an alliance of 779 rare disease patient organisations from 69 countries, calls on the European Parliament and Member States to adopt the European Commission's (EC) proposal, published today, for a future European cooperation on Health Technology Assessment (HTA).

- *Only a permanent structure can guarantee long-term cooperation and organise sharing of scientific expertise and methods across all EU HTA agencies.*
- *Joint HTA reports represent a major progress towards high quality, transparent and timely information necessary for the subsequent coverage/ reimbursement decisions made on a national level.*
- *With this proposal, patients will be better equipped to understand the scientific rationale behind the assessment of the added value of health technologies, everywhere in the EU and for the first time in all Member States.*
- *In 2018, 24 years after the first EU project on HTA, it is time to create a more efficient process, with no replication in 28 Member States of the assessment of the added value when diseases and patients are the same across the EU.*
- *This is particularly needed when diseases are rare or technologies complex.*

The EC proposal calls for the **mandatory uptake** of joint HTA assessments. EURORDIS welcomes this as the only guarantee that the future cooperation will achieve its goals (no duplication, high quality assessment, timely production of the reports).

When a joint report will be formally adopted at the European level, with the possibility for each Member State to comment before its final adoption, national authorities should not re-assess the clinical domains already assessed (efficacy, safety, relative effectiveness).

François Houÿez, EURORDIS Treatment Information and Access Director, commented, "The concern that the use of the joint report will become mandatory with the perceived impression that a report done by others could be imposed on a Member State is not valid, as each agency will have opportunities to contribute, exactly as they successfully do today in the European Medicines Agency's decentralised process."

He continued, "Patients have high expectations regarding the new cooperation and a lot to gain in consistency of the assessment and in transparency of the information needed for the decision-making downstream. Patients are ready to engage at every stage, from the development of guidelines to the assessment itself."

Currently, agencies use different methods and sources of data for HTA assessments, and do not have the resources to assess all new technologies or obsolete ones. Every year, more than one hundred innovative

pharmaceuticals are authorised, and high numbers of invasive medical devices or in vitro diagnostic tools are proposed, and complex surgery need to be validated.

There is no reason why 28 Member States should conduct their assessment separately; this unnecessary duplication of efforts introduces disparities in the quality of the assessment and in the timing of the decision-making. With at least 20 Member States having limited capacities, optimising them and pooling of their resources, makes sense.

The legislative proposal will enable HTA agencies to share and transfer the highest scientific standards, applying common methods and guidelines, build mutual trust and share expertise and know-how. It will focus on clinical aspects, while costs and economic ones will remain a national-level responsibility.

Assessing new health technologies jointly is the only way to move away from the current status quo. For this, clear procedures by which all agencies can contribute with enough due time to comment on the joint work need to be in place. No Member State should feel a joint report to be imposed to them, all scientific contributions should be considered.

The new scientific secretariat hosted by the European Commission will ensure independent and efficient work. It will support Member States, with a stepwise approach, in the initial joint assessments.

As well as mandatory uptake of joint assessments, EURORDIS welcomes the activities envisaged for this future cooperation: from joint scientific consultations (early dialogues) to horizon scanning, development of guidelines and methodologies, evidence generation and research activities on a voluntary basis.

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EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 700 rare disease patient organisations from more than 60 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 6000 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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