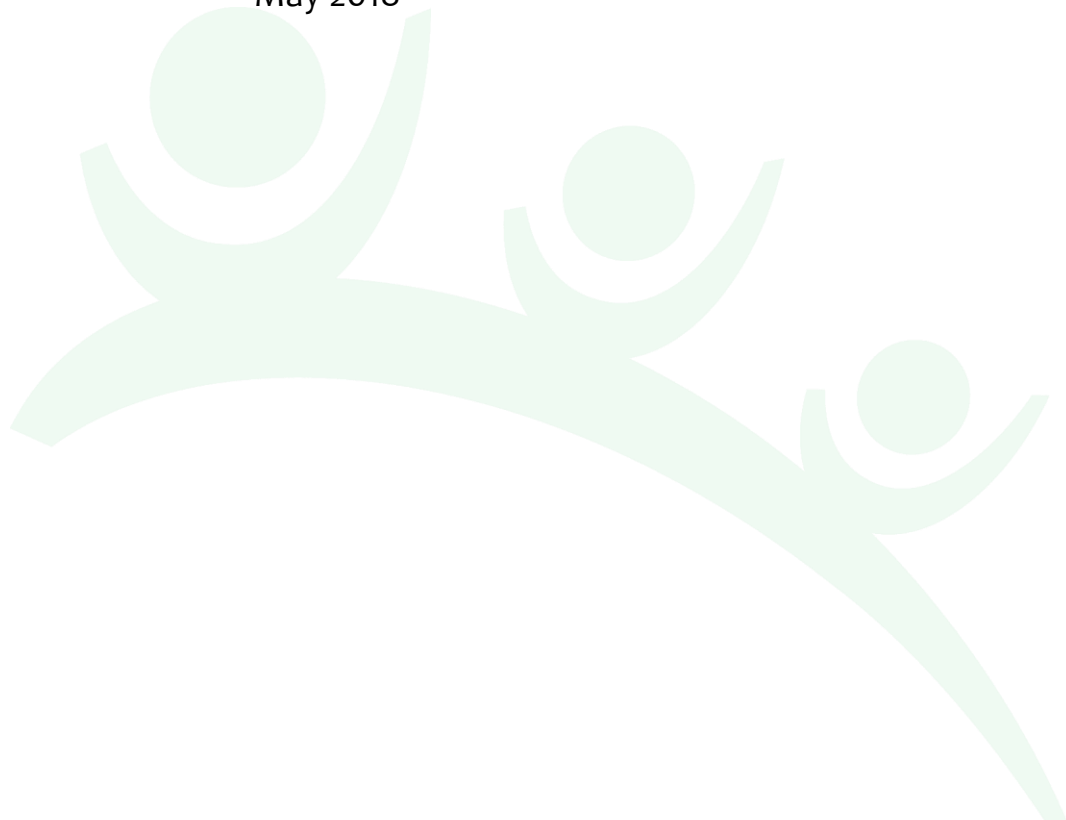


EPF Position on the European Commission's Proposal for a Regulation on Health Technology Assessment

May 2018



Contents

1.	Introduction	3
1.1	Why is an EU Regulation needed?	3
1.2	Patient involvement must be strengthened	4
1.3	Scope of the proposal	4
2.	Governance: the Coordination Group and Stakeholder Network	4
3.	Joint work on HTA – four areas of cooperation	6
3.1	Joint clinical assessment	6
3.2	Joint scientific consultation.....	8
3.3	Identification of emerging health technologies.....	9
3.4	Voluntary cooperation	9
4.	Transparency.....	10
5.	What is left out: implementing and delegated acts	11
6.	Relation of the HTA proposal to other initiatives	12
7.	Conclusion.....	12

1. Introduction

EU-level collaboration on HTA has been ongoing for years, in the form of a number of projects as well as two Joint Actions called EUnetHTA. The third EUnetHTA Joint Action is currently ongoing and will continue until 2020. HTA was one of the areas for future collaboration for which the Cross-Border Healthcare Directive (Directive 2011/24/EU) created a legal basis through Article 15. The current proposal builds further on the possibilities granted under this article. On basis of article the HTA Network was set up.

EPF has been an active member of the EUnetHTA Joint Action 2 Stakeholder Forum, set up as part of the governance of the EU Joint Action. We are currently member of the HTA Network Patient and Consumer Stakeholder Pool, where the HTA Network is the high-level entity bringing together Member States representatives. EPF also collaborates with Health Technology Assessment International (HTAi), a global society and forum for collaboration, particularly through its Interest Group on Patient and Citizen Involvement to promote meaningful patient involvement.^{1,2}

In 2010-2013, EPF conducted research on patient involvement in HTA to promote meaningful patient involvement in HTA processes. We published a report of our survey (2013) as well as a good practice toolkit, which was shared with HTA agencies, patient organisations and decision-making bodies in the EU.³

In 2017, based on the views captured within the membership, EPF responded to the European Commission's public consultation about the future of the EU cooperation on HTA, which explored different policy options and their acceptability to different stakeholders. EPF's response called for mandatory participation with mandatory uptake of joint work.⁴

Below, we provide EPF's response to various aspects of the Commission's proposal.

1.1 Why is an EU Regulation needed?

Based on our previous engagement with the topic and views gathered from our membership, **the European Patients' Forum warmly welcomed** the publication of the European Commission's legislative proposal ([COM\(2018\) 51 final](#)) on 31 January 2018 as an important step towards improving patients' equitable access to high-quality healthcare.⁵

After more than 10 years of collaboration on HTA at EU level, the European Patients' Forum (EPF) believes it is now time for Member States to commit to a more integrated framework on HTA. EPF welcomes the proposal for a Regulation, as that will be directly applicable rather than first being transposed into national legislations as a Directive would be. The Commission's proposal is ambitious: it wants Europe to advance towards a permanent cooperation on HTA, including mandatory uptake of joint clinical assessments by Member States. Although medicines are increasingly authorised at

¹ <https://www.htai.org/interest-groups/patient-and-citizen-involvement/>

² <https://www.eupati.eu/category/health-technology-assessment/>

³ <http://www.eu-patient.eu/whatwedo/Policy/HTA/>

⁴ <http://www.eu-patient.eu/globalassets/policy/hta/epf-response-to-hta-consultation.pdf>

⁵ EU language versions of key documents are available at

https://ec.europa.eu/health/technology_assessment/eu_cooperation_en

European level by the European Medicines Agency (EMA) and marketing authorisation is granted for all EU Member States, the HTA process is in the hands of individual Member States. Essentially, the same process is repeated 28 times across the EU, and the timeframes in which this is done vary, often taking place quite some time after the granting of marketing authorisation.

EPF has drawn attention to the problems of divergent HTA assessment by Member States: from a patient's perspective, the fact that HTA bodies in different Member States reach divergent decisions on the same medicines leaves patients in an unequal situation and is confusing.⁶ We believe **mandatory uptake is needed** to overcome the current fragmentation and low uptake of joint EU-level work. But the Commission's proposal also mitigates for potential obstacles and bottlenecks, depending on country specificities, by applying the principle of proportionality and phased implementation with a transition period.

1.2 Patient involvement must be strengthened

Mandatory and meaningful involvement of the patient community in the HTA process, including the selection of technologies to be assessed, is needed to **ensure HTAs are conducted in the interest of patients**. In line with the EU strategy for health technology assessment,⁷ the proposal envisages the involvement of patients throughout the process, including in horizon-scanning, joint scientific advice and joint clinical assessment. EPF welcomes this principle, although the provisions are not fully adequate at the moment. Our position paper makes a number of specific recommendations to strengthen the Regulation to ensure patient involvement is meaningful, structured and resourced.

1.3 Scope of the proposal

The proposal focuses on **clinical aspects of HTA only**, because it considers that they are “typically based on global evidence (e.g. worldwide clinical trials in the case of medicinal products and multi-national clinical trials for medical devices)”, whereas assessing the non-clinical aspects of HTA are more dependent on the national context: non-clinical assessments focus on economic, organisational, ethical and legal domains which are more specific to the national context and “closer to the final decisions on pricing and reimbursement which remain strictly in the hands of Member States.” The Commission also considers that formal cooperation on clinical assessments provides the most “EU added value” while respecting the principles of subsidiarity and proportionality.⁸ EPF supports this overall position.

2. Governance: the Coordination Group and Stakeholder Network

The Regulation establishes a **Member State Coordination Group** on Health Technology Assessment which oversees the work. Member States would designate their HTA bodies as members of the group, and its sub-groups which would do the actual assessments. Sub-groups may be established, for

⁶ EPF (2016) “Core Principles from the Patients’ Perspective on the Value and Pricing of Innovative Medicines.” Available at http://www.eu-patient.eu/globalassets/policy/epf_pricing_statement_160616.pdf

⁷ EU HTA Network: “Strategy for EU Cooperation on Health Technology Assessment”, 2014, p. 8. Available at http://ec.europa.eu/health/technology_assessment/docs/2014_strategy_eucooperation_hta_en.pdf

⁸ Impact assessment report accompanying the legislative proposal (SWD(2018) 41 final), available at: https://ec.europa.eu/health/sites/health/files/technology_assessment/docs/2018_ia_final_en.pdf

example, for medicines, devices or other health technologies. The Coordination Group would “ensure appropriate involvement of stakeholders in its work”.

A **Stakeholder network** is established in Article 26. This refers to organisations, not individuals, and will be established by an open call for application with selection criteria (to be defined). The commission will organise *ad hoc* meetings between the Stakeholder Network and The Coordination Group in order to “update stakeholders on the work of the Group and provide an exchange of information on the work of the Coordination Group. If the Coordination Group requests, the Commission can invite patients ... nominated by the Stakeholder Network to attend meetings as observers”.

EPF believes adequate patient involvement is essential for the Regulation to succeed. A distinction needs to be made between patients’ participation in specific assessments of technologies and patient representation in the HTA governance.

Patient representatives should be members of the Coordination Group. Patients should be represented in the main governing body of the HTA framework, that is, the coordination group, given their special position as key stakeholders and end beneficiaries of therapies. Patient representation is already enshrined in the European legislation governing pharmaceutical regulation: the European Medicines Agency includes patient representatives in nearly all of its official bodies, including the scientific committees in the management board, and are increasingly invited to contribute to the deliberations of the CHMP; the added value of this is well recognised.

Nominating patients as full members of the Coordination Group satisfies the need for adequate involvement of civil society and reinforces the democratic nature of the decision-making process. It is also consistent with a key principle of meaningful patient involvement, whereby patient advocates should be involved at every level of decision-making for all decisions that affect the lives of patients. This is the only way to ensure mutual trust and create necessary partnerships and a true demonstration that HTA activities are patient-centred.

The patient representatives on the Coordination Group should be **representing European-level patient organisations** that participate in the stakeholder network. A public call for expressions of interest is recommended, similar to the current procedure for selecting patient representatives to the EMA committees and governance body. The **criteria for selection** of stakeholder organisations for the network should be aligned with other existing criteria, such as the EMA eligibility criteria for patient and consumer organisations.

EPF calls for the role of the Stakeholder network to be strengthened.

The Stakeholder network should not be only a passive recipient of information about the work of the Coordination Group, but a platform for genuine dialogue; its views should be taken on board by the Coordination Group. We recommend **regular meetings and exchange of information** between the network and the Coordination Group.

While we welcome the obligation on the Coordination Group to ensure appropriate involvement of stakeholders, it is not mentioned which stakeholders this refers to. It should be clarified which stakeholders would be included in the different processes and bodies. For example, as industry is

already involved in HTA being the developers of the products that are being assessed, we do not see any pressing rationale for their presence also in the Stakeholder network.

EU level Guidelines are needed to ensure meaningful, systematic and appropriately-resourced patient involvement in HTA. These should be developed at EU level, in collaboration with patient organisations, and these should address all aspects of the HTA framework where patient input is relevant. Building on initiatives such as the HTAi Patient and Citizens' Sub-Group and existing best practices in HTA agencies across the world, appropriate methodologies and structures for patient involvement should be developed and applied throughout.

The Regulation should include **specific provisions for ensuring the meaningful involvement of patient representatives as well as individual patients**, as appropriate, in HTA assessments, scientific consultations and horizon-scanning activities. This should include allocation of sufficient financial resources. The proposal foresees allowances to HTA bodies for carrying out joint work, as well as to national experts for participation in the Coordination Group and its sub-groups. Patients are also experts, and their input in the process is crucial, so appropriate remuneration for patients' expertise and resources to ensure participation of patient organisations needs to be ensured in the budget foreseen for the HTA framework. Not to do so would relegate patient involvement to a tokenistic level.

Finally, we would like clarification on the following: The Regulation does not specify in what situations the Coordination Group would adopt a report by simple majority vote, rather than consensus. This should be defined. Secondly, HTA bodies that are not part of the sub-group in charge of preparing the joint clinical assessment do not have the possibility to comment on the draft report. Allowing them to do so might help reach consensus on the final report.

3. Joint work on HTA – four areas of cooperation

The proposal sets out four main pillars of cooperation: (1) joint clinical assessments; (2) joint scientific consultations (early dialogue); (3) identification of emerging health technologies (horizon-scanning); and (4) voluntary cooperation. EPF supports this approach while we call for certain aspects to be strengthened and clarified. (See below, and also our comments under section 5 on implementing and delegated acts.)

3.1 Joint clinical assessment

Joint clinical assessments⁹ are the main new proposition of the Commission. Following a transitional period, Member States are expected to participate and obliged to apply the joint assessment reports in their own work. Member States are not obliged to assess a technology that is being or has been jointly assessed, but if they do so they must use the joint assessment report and must not repeat the

⁹ The Regulation defines '*clinical assessment*' as compiling and evaluating available scientific evidence in comparison with one or more other technologies, in the following domains: the health problem addressed by the technology and current use of other technologies for that health problem; description and technical characterisation of the technology; relative clinical effectiveness; and relative safety. '*Non-clinical assessment*' is defined as including "the cost and economic evaluation of a health technology, and ethical, organisational, social, and legal aspects related to its use".

clinical assessment. They are meant to draw conclusions on the overall added value of a technology based on both the joint clinical assessment and their own non-clinical assessment.

EPF supports joint clinical assessment. Joint clinical assessments have significant potential for improving equitable access to therapies since they focus solely on clinical data. Member States will still conduct their own assessment of economic, social, legal etc. aspects and make decisions regarding pricing and reimbursement accordingly. Reducing duplication and fragmentation of work through joint assessment will lead to more effective and efficient use of scarce resources in national health systems – a significant benefit especially with the current economic constraints on most Member States. These resources could be used for critical investments elsewhere.

The added value of joint assessments can only be realised if they are fully implemented by Member States, rather than adding an extra element into the national process. **EPF thus supports the principle of mandatory uptake** of the joint assessment results into national full HTA, with no repetition of the same analyses.

The Commission proposes that joint assessments would only be conducted for new medicines, active substances and new indications, with the possibility of assessing certain medical devices and *in vitro* diagnostics. **EPF considers that joint assessments should not be limited to new technologies.** It should be possible to identify also existing technologies that would benefit from an assessment (for example to identify those that are ineffective and should no longer be used). In addition, **patient representatives**, for example through the Stakeholder Network, should be able to propose technologies for joint assessment. This would be consistent with the centrality of patients' unmet needs.

Criteria for selection

The Regulation proposes a number of criteria for selecting medical devices. These criteria would also be used during the transition period to select medicines for assessment, but after the transition period all new medicines would be assessed. The criteria are unmet medical need; potential impact on patients, public health, healthcare systems; significant cross-border dimension; EU-wide added value; and available resources.

EPF supports these criteria as long as it is clarified that **“unmet medical need” and “impact on patients” must be developed with the involvement of patients and patient organisations.** This is necessary for the assessment to be accurate. Further, in line with our comment above, “evidence of obsolescence/lack of effectiveness” of existing technologies should be added as a criterion.

The process of preparing joint assessment reports

Member States will lead the process through their HTA bodies. The timing will be coordinated with the marketing authorisation process to minimise delay in completing the HTA assessment once marketing authorisation is granted. The sub-group doing the joint assessment would need to “ensure that stakeholders, including patients and clinical experts, are given the opportunity to provide comments [...] and set a time-frame in which they may submit comments.”

EPF supports this approach in principle, but we believe some clarifications need to be made.

1. We welcome the mention of “patient-relevant health outcomes” in the clinical assessment. The integration of patient-relevant outcome measures in HTA is necessary in order to arrive at an accurate assessment of the added value of an intervention. **Patient-relevant outcomes – whether clinical or relating to quality of life – must be defined by patients themselves**, and measures used for assessment must be validated on this basis, as currently many so-called “patient reported outcomes” were not developed with patients.¹⁰
2. We welcome the obligation on HTA bodies to provide patients with an opportunity to comment on the joint assessment. A **workable and fair process must be developed to enable patient organisations to give input**, including a time-frame that is feasible for membership-based organisations to consult with their patient communities. The mechanism to give input should be easy and accessible.
3. We are concerned that the proposal does not specify any obligations on developers of technologies to provide the comprehensive evidence needed for the joint assessment, so it is unclear how this would be ensured and what would happen if the evidence is considered insufficient even after the additional request. We propose that **sharing all evidence and data in their possession, including confidential information, should be mandatory for companies** in order to allow a comprehensive assessment and meaningful patient input.
4. In addition, the subgroup preparing the report should request **additional data from relevant sources**, such as patient registries, databases, or European reference networks, where this is deemed necessary to complement the information provided by the developer and to perform a more accurate clinical assessment of the technology.

3.2 Joint scientific consultation

Industry could request a joint scientific consultation – sometimes referred to as early dialogue – on its product from the Coordination Group during the development phase to get advice on what evidence and data would likely be required as part of a future joint clinical assessment. The process would work similarly to the joint clinical assessment, except the reports of these consultations would not be published nor would they be binding to anyone. The Coordination Group would include anonymised summary information on such consultations in its annual reports.

The Coordination Group would take into account certain criteria when deciding on scientific consultations, similar to the selection criteria. The sub-group doing a joint scientific consultation must ensure that stakeholders, including patients, “are given an opportunity to provide comments during the preparation... and the timeframe in which they may submit comments.”

EPF supports early dialogue between all relevant stakeholders at EU level from the earliest stages of medicines research and development, building on existing examples such as the SEED and MOCA initiatives.¹¹ This dialogue, together with horizon-scanning, can help overall in making research and

¹⁰ Wiering B, De Boer D, Delnoij D. “Patient involvement in the development of patient-reported outcome measures: The developers’ perspective” *BMC Health Services Research* (2017) 17:635; Wiering B, Boer D, Delnoij D. “Patient involvement in the development of patient-reported outcome measures: a scoping review”. *Health Expect.* 2017;20(1):11–23.

¹¹ See EPF (2016) “Core Principles from the Patients’ Perspective on the Value and Pricing of Innovative Medicines”, available at http://www.eu-patient.eu/globalassets/policy/epf_pricing_statement_160616.pdf

development more predictable for the industry and can enable Member States and patients to give input on their priorities to companies.

We **welcome the obligation to consult with patients**, with the same comments as above to ensure the input is meaningful. Mechanisms for ensuring input from patients should be expanded and strengthened also at national level; this will help in identifying anticipated technologies that promise added benefit for patients.

To avoid conflicts of interest, we believe **clinical assessments and scientific advice must remain separate functions**. Separate experts should be responsible for each. In addition, the coordination between joint scientific consultations and scientific advice given by the EMA should be reinforced to avoid risks of duplication. **Transparency** should remain the overarching principle. Reports on the scientific consultations should thus be published together with the joint clinical assessment.

3.3 Identification of emerging health technologies

Joint work by the Coordination Group would also encompass an annual study to identify important emerging health technologies. This is usually called “horizon-scanning”. The report would contribute to the annual work programmes of the HTA Coordination Group. The coordination group is obliged to consult with patient organisations, amongst other stakeholders, in the preparation of this report.

EPF supports efforts for horizon scanning and forecasting, which will facilitate planning and allocation of resources. We welcome the requirement to consult fully with all relevant stakeholder groups, including patient organisations. Assessments of patients’ needs would be helpful in the prioritisation process of emerging technologies.

3.4 Voluntary cooperation

The proposal provides for the possibility for Member States to cooperate voluntarily at EU level going beyond the joint clinical assessment reports whilst benefiting from the support framework. This could be HTA on other technologies, non-clinical assessments, collaboration on gathering additional evidence to support HTA such as real-world data, or evaluation of eHealth technologies and personalised medicine, or the impact of a technology on the organisation of care.

Patients’ needs go beyond medicines and include other therapeutic options, social and community services and peer support. Developing common work in these areas can **add value and facilitate innovation, including social and organisational innovation**, that brings benefits to patients and supports more effective, efficient and sustainable health systems. The scope of supporting voluntary cooperation could include for example finding better ways of structuring and delivering integrated care; evaluating the impact of patient-centred practices and patient involvement; social innovation; and the development and effective use of user-driven technologies.

EPF believes this aspect of the Regulation needs to be strengthened. Article 19 is currently too weak and does not provide any specific resourcing for this work. It should be explicitly mentioned that also in this area, the **criterion of stakeholder involvement – particularly patient involvement** – should be met; and **sufficient budgetary resources** should be ensured for such collaboration in the EU budget post-2020 with full participation of patients and patient organisations.

Overall, we believe there is a need for capacity-building to bring greater alignment in the quality of HTA across Member States and would recommend that Regulation should be supplemented by a **European “best practice” guide to HTA**, which the Member States should be encouraged and supported to implement. This could also incorporate guidance for transparency, managing divergent interests, and patient involvement.

4. Transparency

The joint assessment reports would be communicated to the Commission, which would maintain a “list of technologies having undergone joint clinical assessment”. Approved assessment reports and “summary reports” would be published on an IT platform to be developed, but published reports would not include “information of a commercially sensitive nature”. On joint scientific consultations, only summary information would be included in the annual reports of the Coordination Group and the IT platform. Article 27 states that “The Commission shall ensure appropriate levels of access to the information contained in the IT platform for Member State bodies, members of the stakeholder network, and the general public.” However, it is not specified what appropriate access means.

EPF supports the principle of maximum transparency. We call for more robust transparency provisions in the Regulation. Transparency is vital for generating and maintaining trust of patients and the wider public in the frameworks governing healthcare policy. Patients are increasingly becoming active participants in their own care. Patient involvement is a vital element to ensure the future high quality and sustainability of European healthcare systems. In order to empower patients to make informed decisions in partnership with health professionals, it is vital that patients have access to all the relevant information needed to make those decisions.

- 1. All reports emanating from HTA assessments** – both EU level joint clinical assessment and national assessment – must be made available in a lay-friendly format, similar to the “lay summaries” of clinical trial results provided for in the EU Clinical Trials Regulation.¹² These reports should be available in all EU languages.
- 2. Guidance for the preparation of summary reports** should be developed at EU level through an inclusive process. EPF recommends a similar process to that which led to the existing guidance on clinical trials results.¹³
- 3. The IT platform should be in principle fully public** unless specific reasons require that a piece of information should not be published. This is the principle in other EU legislation such as the Clinical Trials Regulation. As with results of clinical trials, redacting “commercially confidential information” must balance the legitimate economic interests of a company against the public interest in favour of disclosure. EPF considers that “commercially confidential” should be defined as narrowly as possible, and requests to redact information should be fully justified. A

¹² http://www.eu-patient.eu/globalassets/policy/clinicaltrials/epf-lay-summary-position-final_external.pdf

¹³ https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017_01_26_summaries_of_ct_results_for_laypersons.pdf

transparency policy should be developed with public and stakeholder consultation to ensure maximum transparency while protecting legitimate business interests.

4. **HTA decision-making, must become more transparent for patients and citizens**, including the criteria used for taking decisions and how these are evaluated. This is needed both at EU level and in the national processes, which include joint clinical assessments as well as the member state-specific non-clinical assessments. Member States (governments and HTA bodies) should communicate more effectively with patients and the public regarding their processes and procedures. They should engage in dialogue about patient's needs, societal needs and values, in order to create a more nuanced understanding among all about the socio-economic context in which policy decisions are made.

5. What is left out: implementing and delegated acts

Significant parts of the proposal are left vague, to be defined later through implementing¹⁴ and delegated acts¹⁵. They are to be based on the procedures, documents and methodologies developed in the EUnetHTA Joint Action 3.

Chapter III of the draft Regulation only contains an overview of the HTA process. *Common procedural rules and methodology* and *detailed procedural rules* for the preparation of the joint clinical assessments will be developed through implementing acts. *The contents of submission documents and reports, and the rules for selecting stakeholders*, will be defined through delegated acts. Procedural rules for joint scientific consultations, including rules for the consultation of patients and other stakeholders, will be developed by implementing acts.

EPF believes that the rules and procedures to be applied should be defined in the Regulation so as to ensure the highest standard of joint assessments. The quality of the result is vital as it will be applicable across the EU. Sufficient – though not excessive – time must be insured to conduct a high-quality assessment including consultation with patients. We believe the Regulation should be more precise on these aspects and they should not be left entirely to implementing legislation.

Further, as mentioned above, it should be specified that a **framework and rules for the consultation of patients and patient organisations** will be developed for the whole HTA framework to ensure consistency and meaningful patient input at all relevant stages of the process.

¹⁴ Although primary responsibility for implementing EU law lies with Member States, there are certain cases in which the Commission (or exceptionally the Council) can adopt an implementing act. This often happens in areas where uniform conditions for implementation are needed (taxation, agriculture, the internal market, health and food safety, etc.).

¹⁵ The Treaty allows the EU legislators (the European Parliament and Council) to delegate power to the Commission to adopt non-legislative acts that supplement or amend certain non-essential elements of a legislative act. This can include for example updates to reflect technological developments. There are strict limits to this and a delegation can be revoked.

6. Relation of the HTA proposal to other initiatives

As we have stated above, EU-level joint clinical assessment does not interfere with the Member States' right to conduct full HTA taking into account the nonclinical factors, such as economic, social and legal ones. Currently a number of initiatives are ongoing in the area of pricing and reimbursement, such as the BENELUXA plus Ireland, and other collaborations. **These initiatives on pricing and reimbursement should not be used to undermine progress towards EU-level joint Health Technology Assessment.** They can be seen as complementary, but separate.

Orphan medicines¹⁶ constitute a particular category of health technologies, due to the small number of patients dispersed across the EU and the often high price of these medicines. This is an area where in our view decisions should be taken at EU level regarding pricing and reimbursement and it would add value for patients and for Member States. Certain other products could be considered for this approach also. In May 2015 EPF and EURORDIS published a joint letter calling on the EU's pricing and reimbursement authorities to support the scaling-up of pilots on early dialogue and to establish a "table for price negotiation" with a group of Member States, i.e., to take a collaborative European approach to negotiating the prices of medicines with pharmaceutical companies, rather than one that is fragmented. We believe this would lead to better collaboration between industry and payers and, ultimately, to better access to medicines and improved health outcomes.

7. Conclusion

The European Patients' Forum supports the Commission's legislative proposal. We call for certain aspects of the proposal to be clarified and/or strengthened. We believe this will help ensure the adoption of a Regulation that will bring real progress in advancing equitable access to high-value medical technologies across the EU. As the European cross-disease patient umbrella organisation, EPF is committed to working closely with the European legislators and in dialogue with our membership towards a sustainable European framework for HTA where patients are actively involved as partners and which ensures timely and equitable access for patients to medical technologies that add value and improve their lives.
