# *Regulation on Health Technology Assessment*
# *Draft Report of the European Parliament's ENVI committee*
# *Rapporteur: MEP Soledad Cabezón Ruiz*

## Statement of the
## European Social Insurance Platform (ESIP)

**8 June 2018**

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## About the European Social Insurance Platform (ESIP)

The ***European Social Insurance Platform*** **(ESIP)** represents over **50 national statutory social insurance organisations** (covering approximately **250 million citizens**) in **16 EU Member States and Switzerland**, active in the field of health insurance, pensions, occupational disease and accident insurance, disability and rehabilitation, family benefits and unemployment insurance. The aims of ESIP and its members are to preserve high profile social security for Europe, to reinforce solidarity-based social insurance systems and to maintain European social protection quality. ESIP builds strategic alliances for developing common positions to influence the European debate and is a consultation forum for the European institutions and other multinational bodies active in the field of social security.

**Statement regarding positions submitted by ESIP:** *ESIP members support this position in so far as the subject matter lies within their field of competence.*

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## General Remarks

On 31 January 2018 the European Commission published a proposal for a Regulation on health technology assessment.[1] On 23 April 2018 ESIP adopted a common position regarding the Commission proposal expressing its general support for establishing a permanent structure for EU cooperation on HTA and at the same time highlighting a number of critical aspects. Bearing in mind these concerns **ESIP warmly welcomes and applauds** the draft report of rapporteur MEP Cabezón Ruiz on behalf of the ENVI committee. ESIP is pleased to see that numerous concerns from the side of statutory health insurance and health care payers have been taken on board. In particular**, we welcome the following amendments:**

- Am. 22, 89-92 **addressing uptake of joint assessments**: Member State institutions will have more flexibilities in using the joint assessment reports in their respective health technology assessments. While Member States shall not unnecessarily duplicate the work done at EU level, they are not prevented from carrying out complementary assessments as part of their own appraisal processes.
- Am. 39, 51, 73, 77, 84, 87, 145, 163 **regarding the role of the European Commission**: Cooperation on HTA at European level has to be Member States driven. The amendments clarify that the Coordination Group has the final say on the publication of joint assessments. The European Commission provides administrative and organisational support for the joint work and has the right to speak but not to vote.
- Am. 50, 78 **regarding the decision-making process**: The Coordination Group will take its decisions on the basis of a two-thirds majority, if not by consensus. This decision-making process is necessary in order to build trust and avoid outcomes that would negatively impact individual healthcare systems. However, while guarding the principle of "one Member State, one vote", it may be necessary by virtue of the national organisation of HTA to appoint more than one national representative to the Coordination Group.
- Am. 139 **regarding methodology and quality**: The Coordination Group shall draw up the methodology to be used for clinical assessments and consultations.
- Am. 38, 54, 57, 68, 76, 83, 88, 109, 160 **regarding transparency**: The highest possible level of transparency throughout the entire process of EU cooperation on HTA is crucial to ensure the necessary trust and acceptance. This will be achieved by making public the work and decisions of the Coordination Group, , including negative results of the assessments, comments of stakeholders, as well as giving full public access to all the information contained in the IT platform.
- Am. 68, 96, 138 **regarding compliance and participation of technology developers**: An implementing act shall establish a sanction mechanism in the event of non-compliance by the technology developer, with the obligation to provide allavailable information.

[1] COM(2018) 51 final.

## Additional Amendments

Notwithstanding our general support for Ms. Cabezón Ruiz report, we would like to propose the following amendments that reflect concerns of statutory health insurance institutions and health care payers in more detail and focus on the following aspects:

- **Clarifications regarding uptake of joint assessments** allowing Member States to draw their own conclusions in the context of their appraisal decisions (Am 17, 22)
- **Membership in the Coordination Group** (Am 49)
- Clarifications guaranteeing **quality, timeliness, transparency** and **stakeholder involvement** with regard to **joint assessments** (Am 71, 74, 75, 87, 108, 139)
- Guaranteeing **compliance of health technology developers** (Am 96)
- **Avoiding harmonisation of health technology assessments** carried out on **national level** (Am 133)

**Amendment 17**
**Proposal for a Regulation**
**Recital 11**

| Text proposed by the EC | Amendment by the Rapporteur | New Amendment |
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| (11) In accordance with Article 168(7) of the Treaty on the Functioning of the European Union (TFEU), the Member States remain responsible for the organisation and delivery of their healthcare. As such, it is appropriate to limit the scope of Union rules to those aspects of HTA that relate to the clinical assessment of a health technology, and in particular, to ensure that the assessment conclusions are confined to findings relating to the comparative effectiveness of a health technology. The outcome of such assessments should not therefore affect the discretion of Member States in relation to subsequent decisions on pricing and reimbursement of health technologies, including the fixing of criteria for such pricing and reimbursement which may depend on both clinical and non-clinical considerations, and which remain solely a matter of national competence. | (11) In accordance with Article 168(7) of the Treaty on the Functioning of the European Union (TFEU), the Member States remain responsible for the organisation and delivery of their healthcare. As such, it is appropriate to limit the scope of Union rules to those aspects of HTA that relate to the clinical assessment of a health technology. ***In this connection, the joint clinical*** assessment ***provided for by this Regulation, which will be mandatory for Member States, constitutes a scientific analysis of the relative effects of*** health technology ***on clinical outcomes, evaluated in relation to the chosen comparative indicators and chosen groups or subgroups of patients, taking into account the HTA Core Model criteria. This will include consideration of the degree of certainty on the relative outcomes, based on the available evidence.*** The outcome of such ***joint clinical*** assessments should not therefore affect the discretion of Member States in relation to subsequent decisions on pricing and reimbursement of health technologies, including | (11) In accordance with Article 168(7) of the Treaty on the Functioning of the European Union (TFEU), the Member States remain responsible for the organisation and delivery of their healthcare. As such, it is appropriate to limit the scope of Union rules to those aspects of HTA that relate to the clinical assessment of a health technology. ***In this connection, the joint clinical*** assessment ***provided for by this Regulation~~, which will be mandatory for Member States~~, constitutes a scientific analysis of the relative effects of*** health technology ***on clinical outcomes, evaluated in relation to the chosen comparative indicators and chosen groups or subgroups of patients, taking into account the HTA Core Model criteria. This will include consideration of the degree of certainty on the relative outcomes, based on the available evidence.*** The outcome of such ***joint clinical*** assessments should not therefore affect the discretion of Member States in relation to subsequent decisions on pricing and reimbursement of health technologies, including |

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| | the fixing of criteria for such pricing and reimbursement which may depend on both clinical and non-clinical considerations, and which remain solely a matter of national competence. ***The assessment conducted by each Member State as part of its national appraisal therefore falls outside the scope of this Regulation. Such appraisals must include: (1) the joint clinical assessment; (2) the data specific to each Member State (suitable comparative indicators and their reimbursement status); the medical need within their health system; information on a national early-access programme, if available; the target group, therapeutic strategy, clinical use); (3) context-specific analyses (suitable comparative indicators, relevant patient subgroups, target population, cost of the health-care system, guaranteed high-quality use); (4) additional context-specific considerations for each Member State (number of patients affected in the Member State, current treatment received by patients in the health system, costs).*** | the fixing of criteria for such pricing and reimbursement which may depend on both clinical and non-clinical considerations, and which remain solely a matter of national competence. ***The assessment conducted by each Member State as part of its national appraisal therefore falls outside the scope of this Regulation. ~~Such appraisals must include: (1) the joint clinical assessment; (2) the data specific to each Member State (suitable comparative indicators and their reimbursement status); the medical need within their health system; information on a national early-access programme, if available; the target group, therapeutic strategy, clinical use); (3) context-specific analyses (suitable comparative indicators, relevant patient subgroups, target population, cost of the health-care system, guaranteed high-quality use); (4) additional context-specific considerations for each Member State (number of patients affected in the Member State, current treatment received by patients in the health system, costs).~~*** |
| *Justification:*<br>Member States obligations concerning uptake are set out in the revised Article 8 and further explained in the revised Recital 16. In order to avoid confusion, the phrase referring to the "mandatory" nature of joint assessments should be removed from Recital 17.<br>Appraisal decisions remain an exclusive competence of Member States. The proposed criteria to be included in national appraisals are therefore beyond the scope of the Regulation and must not be included. | | |

**Amendment 22**
**Proposal for a Regulation**
**Recital 16**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| (16) In order that the harmonised procedures fulfil their internal market objective, Member States ***should be required to*** take ***full*** account of the results of joint clinical assessments and not ***repeat those assessments.*** Compliance with this obligation does not prevent Member States from carrying out non-clinical assessments on the same health technology, or from drawing conclusions on the added value of the technologies concerned as part of national appraisal processes which may consider clinical as well as non-clinical data and criteria. It also does not prevent Member States from forming their own recommendations or decisions on pricing or reimbursement. | **(**16) In order that the harmonised procedures fulfil their internal market objective, ***and their aim of improving innovation and the quality of clinical evidence,*** Member States ***must take*** account of the results of joint clinical assessments and not ***repeat them unnecessarily***. Compliance with this obligation does not prevent Member States from carrying out non-clinical assessments on the same health technology, or from drawing conclusions on the ***clinical*** added value of the technologies concerned as part of national appraisal processes which may consider clinical as well as ***the*** non-clinical data and criteria ***specific to the Member State concerned, at national and/or regional level.*** It also does not prevent Member States from forming their own recommendations or decisions on pricing or reimbursement. | **(**16) In order that the harmonised procedures fulfil their internal market objective, ***and their aim of improving innovation and the quality of clinical evidence,*** Member States ***must take*** account of the results of joint clinical assessments and not ***repeat them unnecessarily***. Compliance with this obligation does not prevent Member States **from taking into account other clinical and non-clinical data and evidence which did not form part of the joint clinical assessment, from** carrying out non-clinical assessments on the same health technology, or from drawing **different** conclusions on the ***clinical*** added value of the technologies concerned as part of national appraisal processes which may consider clinical as well as ***the*** non-clinical data and criteria ***specific to the Member State concerned, at national and/or regional level.*** It also does not prevent Member States from forming their own recommendations or decisions on pricing or reimbursement. |

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| *Justification*:<br>The suggested amendments clarify the meaning of the obligations of "using" and "not repeating" joint assessments at national level included in Article 8 (1) and the new Article 8 (1a) (Amendment 92) and anticipate the possibility of different conclusions being drawn and different outcomes at national level. |

**Amendment 49**
**Proposal for a Regulation**
**Article 3 – paragraph 2**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| 2. Member States shall designate ***their*** national ***authorities and bodies*** responsible for health technology assessment as ***members*** of the Coordination Group and its sub-groups ***and inform the Commission thereof and of any subsequent changes. Member States may designate more than one authority or body responsible for health technology assessment as members of the Coordination Group and one or more of its sub-groups.*** | 2. Member States shall designate ***one*** national ***or regional*** authority or body responsible for health technology assessment as ***a member*** of the Coordination Group and its sub-groups. | 2. Member States shall designate **their** national **or regional authorities** or **bodies** responsible for health technology assessment as **members** of the Coordination Group and its sub-groups. |
| *Justification*: Responsibility for HTA in Member States may be shared between different national bodies and/or organised on a regional basis. While guarding the principle of "one Member State, one vote", Member States need to be able to designate more than one authority or body as members. | | |

**Amendment 71**
**Proposal for a Regulation**
**Article 6 – paragraph 5 – point a**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| (a) an analysis of the relative effects of the health technology being assessed on the patient-relevant health outcomes chosen for the assessment; | (a) an analysis of the relative ***efficacy and safety*** of the health technology being assessed ***in terms of the clinical criteria relevant to the clinical entity and patient group*** chosen for the assessment; | (a) an analysis of the relative ***efficacy and safety*** of the health technology being assessed **in terms of the clinical criteria relevant to the clinical entity and patient group** chosen for the assessment; t**he analysis should be based on the patient-relevant health outcomes and adhere to the international standards of evidence-based medicine;** |
| *Justification:*<br>The assessment must focus on patient-relevant health outcomes. Surrogate end-points can only be accepted in exceptional cases and if qualified using scientifically validated criteria. Evidence-based medicine is the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients. The practice of evidence-based medicine means integrating individual clinical expertise with the best available evidence from systematic research. As such it is internationally recognised and its standards should be enshrined in the European HTA Regulation. | | |

**Amendment 74**
**Proposal for a Regulation**
**Article 6 – paragraph 8**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| 8. The assessor shall provide the draft joint clinical assessment report and the summary report to the ***submitting*** health technology developer ***and set a time-frame in which the developer may submit*** comments. | 8. The assessor shall provide the draft joint clinical assessment report and the summary report to the health technology developer ***for*** comments. | 8. The assessor shall provide the draft joint clinical assessment report and the summary report to the submitting health technology developer and set a time-frame **of maximum14 days** in which the developer may submit comments. |
| *Justification*:<br>As the developer has already provided the assessors with all relevant information available to him at the beginning of the process, it should be ensured that any "clock-stop" in the clinical assessment process remains reasonably short and should not lead to an inappropriate delay. Further, complete transparency regarding the involvement of the developer in the process is essential, as highlighted in amendments to Art. 6 paragraph 10. | | |

**Amendment 75**
**Proposal for a Regulation**
**Article 6 – paragraph 9**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
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| 9. ***The designated sub-group shall ensure that stakeholders, including patients*** and clinical experts***, are given an opportunity to provide*** comments during the ***preparation of the draft*** joint clinical assessment ***report and the summary report and set a time-frame in which they may submit comments***. | 9. ***Patients, consumer organisations, healthcare professionals*** and clinical experts ***may submit*** comments during the joint clinical assessment. | **9. The assessor shall provide the draft joint clinical assessment report and the summary report to stakeholders, including patients and clinical experts and set a time-frame of maximum 14 days in which the stakeholders may submit comments.** |
| *Justification*:<br>The list of stakeholders that might be asked to give comments should not exclude other interest groups, e.g. payers' organisations. To guarantee the necessary transparency and independence of the assessment, all stakeholders should be subject to similar rules regarding submission of comments (see amendment 74 above). | | |

**Amendment 87**
**Proposal for a Regulation**
**Article 7 – paragraph 5**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| 5. If the Commission concludes that the modified approved joint ***clinical*** assessment report and summary report do not comply with the ***substantive and*** procedural requirements laid down in this Regulation, ***it shall decline to include*** the ***name*** of the ***health technology*** in the List. The Commission shall inform the Coordination Group thereof, setting out the reasons for the ***non-inclusion***. The obligations laid down in Article 8 shall not apply with respect to the health technology concerned. The Coordination Group shall inform the submitting health technology developer accordingly and include summary information on those reports in its annual report. | 5. If the Commission concludes that the modified approved joint assessment report and summary report do not comply with the procedural requirements laid down in this Regulation, ***the health technology which is*** the ***subject*** of the ***assessment shall be included*** in the List***, together with the summary report of the assessment and the Commission's comments, and all published on the IT platform referred to in Article 27***. The Commission shall inform the Coordination Group thereof, setting out the reasons for the ***negative report***. The obligations laid down in Article 8 shall not apply with respect to the health technology concerned. The Coordination Group shall inform the submitting health technology developer accordingly and include summary information on those reports in its annual report. | 5. If the Commission concludes that the modified approved joint assessment report and summary report do not comply with the procedural requirements laid down in this Regulation, the health technology which is the subject of the assessment shall be included in the List, together with the summary report of the assessment and the Commission's comments, and all published on the IT platform referred to in Article 27. The Commission shall inform the Coordination Group thereof, setting out the reasons for the ~~**negative report**~~ **determined noncompliance with procedural requirements**. The obligations laid down in Article 8 shall not apply with respect to the health technology concerned. The Coordination Group shall inform the submitting health technology developer accordingly and include summary information on those reports in its annual report. |
| *Justification*:<br>Clarification; the wording "negative report" is misleading and could be misunderstood as a negative result. | | |

**Amendment 96**
**Proposal for a Regulation**
**Article 9 – paragraph 1 – subparagraph 1 (new)**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
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| | ***In the cases referred to under points (a) and (b), the technology developer shall submit the additional information. In the event of a failure to do so, the earlier joint assessment would no longer fall within the scope of Article 8.*** | In the cases referred to under points (a) and (b), the technology developer shall submit the additional information. In the event of a failure to do so, the **sanctions mechanism according to Article 22 (1) b applies**. |
| *Justification*:<br>The sanction mechanism established in the new Article 22 (1) b (Amendment 138) is a more appropriate way to guarantee compliance of health technology providers. | | |

**Amendment 108**
**Proposal for a Regulation**
**Article 13 – paragraph 8**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| 8. ***The designated sub-group shall ensure that stakeholders, including patients*** and clinical experts ***are given an opportunity to provide*** comments during the ***preparation of the draft*** joint scientific consultation ***report and set a time-frame in which they may submit comments***. | 8. ***Patients, consumer organisations, healthcare professionals*** and clinical experts ***shall submit*** comments during the joint scientific consultation. | **No change to COM proposal.** |
| *Justification*:<br>The list of stakeholders should not exclude other interest groups, e.g. payers' organisations. The Commission proposal is clear about the procedure and should be maintained. | | |

**Amendment 133**
**Proposal for a Regulation**
**Article 20 – paragraph 1 – point b**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
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| b) clinical assessments of medicinal products and medical devices ***carried out by Member States***. | b) clinical assessments of medicinal products and medical devices ***falling within the scope of this Regulation and not included in the annual work programme***. | **Point b is deleted.** |
| *Justification*:<br>In order to acknowledge the existing uncertainties with regard to methodology and quality the proposed harmonisation should be limited to joint assessments at EU level in accordance with Chapter II of the proposed regulation. Clinical assessments of medicinal products and medical devices carried out by Member States (Article 20 [b]) must not be harmonised at this stage. | | |

**Amendment 139**
**Proposal for a Regulation**
**Article 22 – paragraph 1 a (new)**

| *Text proposed by the EC* | *Amendment by the Rapporteur* | *New Amendment* |
|---|---|---|
| | ***(1a) The coordination group shall draw up the methodologies to be used to carry out joint clinical assessments and consultations and shall define the content of these assessments and consultations. In any case:***<br>***(a) the methodologies shall be based on high standards of quality, the best available scientific evidence, stemming primarily from double-blind randomised clinical trials, meta-analysis and systematic reviews;***<br>***(b) the assessment of relative effectiveness shall be based on end-points which are relevant to the patient with useful, relevant, tangible and specific criteria suited to the clinical situation concerned;***<br>***c) the comparators shall be the reference comparators for the clinical entity concerned and be the best and/or most commonly used technological or process based comparator;***<br>***d) the technology developers shall for the purpose of its clinical assessment provide the coordination group with the complete dossier in eCTD format submitted to the European Medicines Agency for centralised authorisation. This package shall include the Clinical Study*** | (1a) The coordination group shall draw up the methodologies to be used to carry out joint clinical assessments and consultations and shall define the content of these assessments and consultations. In any case:<br>(a) the methodologies shall be based on high standards of quality, the best available scientific evidence, stemming primarily from double-blind randomised clinical trials, meta-analysis and systematic reviews;<br>(b) the assessment of relative effectiveness shall **in accordance with international standards of evidence-based medicine** be based on end-points which are relevant to the patient with useful, relevant, tangible and specific criteria suited to the clinical situation concerned **and shall display the specific outcomes for different subgroups**;<br>c) the comparators shall be the reference comparators for the clinical entity concerned and be the best and/or most commonly used technological or **process-based** comparator, **taking into account differences between Member States**;<br>d) the technology developers shall for the |

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| | ***Report and the data of individual patients in all clinical trials;***<br>***e) the information to be provided by the health technology developer shall relate to the most up-to-date and public research. Failure to comply with this requirement may trigger a sanctions mechanism.*** | purpose of its clinical assessment provide the coordination group with the complete dossier in eCTD format submitted to the European Medicines Agency for centralised authorisation. This package shall include the Clinical Study Report and the data of individual patients in all clinical trials;<br>e) the information to be provided by the health technology developer shall relate to the most up-to-date and public research. Failure to comply with this requirement may trigger a sanctions mechanism. |
| *Justification*:<br>b) It is important to refer to internationals standards of evidence-based medicine within a regulation on HTA; in addition, assessments need to be fit for purpose, taking into account differences within the more general authorised populations.<br>c) Regarding comparators, differences in standard care between Member States have to be taken into account to guarantee that joint assessments can be used by all Member States. | | |