UNIAMO
FEDERAZIONE ITALIANA
MALATTIE RARE
ONLUS
Rare Diseases Italy

EU4Health Programme

Suggested amendments and additional remarks for the EP Report on the proposal for a Regulation on the new Health Programme

EURORDIS Rare Disease Europe with the support of UNIAMO as National Alliance for Italy, welcomes the proposal of the Commission for a Regulation establishing the new Health Programme 2021-2027 that is endowed with the financial means required to address the challenges of today. EU4Health, as proposed by the Commission, has the potential to support greater leadership of the EU in an area where 70% of European citizens want the EU to do more. The ongoing COVID-19 crisis has intensified the need for stronger cooperation and greater solidarity, underpinned by adequate resources and a clear mandate to act.

Together with #EU4Health, a coalition of civil society organisations in the public health sector, EURORDIS called for a more ambitious, standalone programme in support of EU actions in the field of health.

We commend therefore the bold response of the European Commission and call on the European legislator to maintain and even increase the ambition set by the Commission. We call on you to endorse the proposed amendments below that we trust will help improve the programme.

This proposal is structured around the pillars we consider the most important for the community EURORDIS represents at European level. Firstly, there is need for a **clear and meaningful civil society role in the governance of the programme**. Following the rapporteur's line of thought in the amendments specified below, we call for the introduction of a structure that will allow the participation of NGOs and other actors in the public health sphere to give their input to the needs the programme needs to meet on an annual basis. Only by giving a voice to civil society in an institutional structure, will we ensure that the programme addresses the expectation of European citizens.

Of course, to be able to fulfil this role and continue to provide the digested opinion of our respective communities in an unbiased way, public funds should be invested in organisations who are operational at European level. We therefore enclose a number of recommendations that will allow for civil society to be sufficiently funded.

Issues such as **pricing and reimbursement** are also addressed in our suggestions, as well as a link to the **Health Technology Assessment** file. The material and information we have gathered on those topics go much further that the enclosed, and we are happy to provide you with any additional information for a more concrete discussion on any of those topics.

When it comes to **rare diseases specifically**, we have three main topics that we would like to see addressed in this legislation. Please note that the points below derive both from the needs identified by our membership, but are also explicitly called for by the European Court of Auditors Special report on EU actions for cross-border healthcare. This is the opportunity to integrate those recommendations and action points identified in a legally binding text.

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1. ***There is an urgent need for introducing or updating a general rare disease framework.*** The **Communication from the European Commission on rare diseases (2008) and the Recommendation from the Council of the European Union (2009)** constitute **two key milestones in establishing a comprehensive strategy** to support EU Member States on issues including diagnosis, treatment and care for rare disease patients throughout Europe, **integrating EU regulations and recommendations relevant to rare diseases.** Most importantly, the Council Recommendation called on EU Member States to adopt a national plan or strategy for rare diseases. All those documents (both at national and European level) need to be updated to reflect the current reality. With the support of the European Parliament, EURORDIS is conducting the Rare 2030 foresight study. Rare 2030 gathers the input of a large group of patients, practitioners and key opinion leaders to propose policy recommendations that will lead us to improved policy and a better future for people living with a rare disease in Europe. At the beginning of next year, it will result in recommendations on the most critical areas needing sound policy.

What can be done at this stage, is the inclusion of a call for action in the binding text of the legislation, so that more concrete actions can follow later on.

2. **European Reference Networks** have shown to have a great potential with the results that they have produced in the time that they have already been operational. Nevertheless, this has also allowed for many points of improvement to be identified.
3. **Digital health** is an area that is being developed increasingly more at both national and European level. A coherent approach should be taken from the start to avoid hurdles later down the line. We therefore suggest that the Electronic Healthcare Records are connected to patient registries, and in the field of rare diseases linked to the ORPHAcodes.

A) Civil Society in decision-making on work programmes

Suggestions for new amendments:

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Proposal for a regulation Recital 15 a (new) Text proposed by the Commission	*Amendment* (15a) The rights of patients also extend to their right to be involved in any decision that affects their lives. Furthermore, the participation of patients and citizens is key for civil society to witness how decisions are made, as a matter of transparency and trust. In that sense, the role of patients is clearly stated in most Union legislation on pharmaceuticals. The Union legislator decided that patients are members with full rights in the Committee for Orphan Medicinal Products established by Article 4 of Regulation (EC) No 141/2000 of the European Parliament and of the Council a, in the Paediatric Committee established by Article 4 of Regulation (EC) No 1901/2006 of the European Parliament and of the Council b ,in the Committee for Advanced Therapies established by Article 21 of Regulation (EC) No 1394/2007 of the European Parliament and of the Council1c, in the Pharmacovigilance Committee established by Article 61a of Regulation (EC) No 726/2004 of the European Parliament and of the Council1d , and in the Management Board established by Article 65 of Regulation (EC) No 726/2004. _____ 1a Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products (OJ L 18, 22.1.2000, p. 1). 1b Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004 (OJ L 378, 27.12.2006, p. 1). 30.4.2004, p. 1). Or. en

Proposal for a regulation **Recital 34 a (new)**	**(34a) To achieve a coherent implementation of the actions included in the EU4Health programme, a EU4Health Steering Board should be established. That independent stakeholder group should be responsible, inter alia, for coordination, cooperation in the implementation of the actions, and for creating synergies between the Programme and other programmes which comprise a health dimension.**

Proposal for a regulation **Article 16 – paragraph 1** *Text proposed by the Commission* The Commission shall consult the health authorities of the Member States in the Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable Diseases on the work plans established for the Programme and its priorities and strategic orientations and its implementation.	*Amendment* The Commission shall consult the health authorities of the Member States in the Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable Diseases, **as well as relevant Union decentralised agencies, the EU4Health Steering Board and other relevant stakeholders, such as representatives of civil society organisations, in particular patients' organisations**, on the work plans established for the Programme and its priorities and strategic orientations and its implementation.
Justification *The adequate involvement of civil society representatives, including patient representatives, in decisions on the Health Programme would reinforce the democratic nature of the decision-making process, as evoked in the EU Treaty (Declaration 17 of the Annex) "(…) Transparency of the decision-making process strengthens the democratic nature of the institutions and the public's confidence in the administration."* *The participation of civil society will also reinforce the public acceptance and ensure and better dissemination of decisions taken by the Steering Group.* *Moreover, as far as patient representatives are concerned, this is consistent with a key principle whereby* "patients' advocates should be involved at every level of decision-making for all decisions that affect the lives of the patients, and they should be included in all forums with equal credibility as other participants*".*	

Proposal for a regulation	*Amendment*
Article 16 a (new) *Text proposed by the Commission*	**Article 16a EU4Health Steering Board** **1. The Commission shall establish a EU4Health Steering Board ('the Steering Board') to advise it, in a consultative capacity, in steering the implementation of the Programme, as well as its monitoring and evaluation.** **2. The Steering Board shall focus on creating synergies between the Programme and other Programmes which comprise a health dimension , through coordination, cooperation and synergies, promoting engagement with patients and society , and providing scientific advice and recommendations to the Commission. In exercising its role, the Steering Board shall provide value oriented health actions, sustainability, better health solutions, and shall foster access and reduce health inequalities.** **3. The Steering Board shall be an independent stakeholder group, composed of actors from relevant sectors in the field of public health, wellbeing and social protection, with participation of representatives of regions and local health authorities, patient representatives and citizens.** **4. The Steering Board shall be composed of 15 to 20 highly qualified individuals drawn from the fields referred to in paragraph 3. The members of the Steering Board shall be appointed by the Commission in consultation with the Parliament, following an open call for nominations or for expression of interests or both.** **5. The members of the Steering Board shall be appointed for the period referred to in the second paragraph of Article 1.** **6. The Steering Board shall have a chair who shall be appointed by the Commission from among its members. The Steering Board shall meet at least four times per year.** **7. The Steering Board shall:** **i. provide input, in the form of a comprehensive strategy, for developing annual work plans for the Programme,** **following a proposal from the Commission; ii. elaborate a plan for steering coordination, cooperation and synergies between the Programme and other Programmes which comprise a health dimension; iii. advise the Commission with regard to monitoring and evaluating the Programme, as set out in Articles 19 and 20 respectively.** **The plan for steering coordination, cooperation and synergies shall facilitate action or efforts to ensure that all the existing financial mechanisms relevant to health are visible and coordinated, and shall help to steer coordination and cooperation.**

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	8. The Commission may consult the Steering Board on matters other than those referred to in paragraph 7.

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B) <u>Funding for civil society</u>

Article 9a new	Article 9a Beneficiaries eligible for grants 1. Grants for the functioning of patient organisations at Union level may be awarded to European patient organisations which comply with all of the following conditions: (a) they are non-governmental, non-profit-making, and have as their primary objectives and activities the promotion and protection of the health, and representation of the community they represent in the European-level policy processes; (b) they are mandated to represent the interests of patients at Union level by organisations in at three fourths of the Member States that are representative, in accordance with national rules or practice, of patient organisations, and that are active at regional or national level.
Justification *There is a large number of arguments why civil society organisation should be funded with public funds. The European Economic and Social Committee has given an overview of many of them and calls for more EU funding for civil society organisations.* https://www.eesc.europa.eu/en/our-work/opinions-information-reports/opinions/financing-csos-eu-own-initiative-opinion	

C. C)

C) <u>European Cooperation on pricing and reimbursement of medicines</u>

Suggestions for new amendments:

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ANNEX I	ANNEX I
LIST OF POSSIBLE ELIGIBLE ACTIONS PROVIDED FOR IN ARTICLE 13 (i) Actions on medicines, vaccines and medical devices: (i) Support to initiatives to improve vaccination coverage rates in the Member States; (ii) Support actions to fight vaccine hesitancy; (iii) Support clinical trials to speed up the development, authorisation and access to innovative, safe and effective medicines and vaccines; (v) Support action to encourage the development of innovative products and of less commercially interesting products such as antimicrobials; …	LIST OF POSSIBLE ELIGIBLE ACTIONS PROVIDED FOR IN ARTICLE 13 (i) Actions on medicines, vaccines and medical devices: (i) Support to initiatives to improve vaccination coverage rates in the Member States; (ii) Support actions to fight vaccine hesitancy; (iii) Support clinical trials to speed up the development, authorisation and access to innovative, safe and effective medicines and vaccines; ***(iv)b Support action to ensure greater availability in the Union of medicines and medical devices and contribute to their affordability for patients and health systems;*** (v) Support action to encourage the development of innovative products and of less commercially interesting products such as antimicrobials; …

Justification

Strengthening European cooperation in selected areas can bring better outcomes for patients and health care professionals, whilst increasing the efficiency of health systems (Council Conclusions on Encouraging Member States-driven Voluntary Cooperation between Health Systems (16 June 2017).

Mechanisms of voluntary cooperation between EU Member States have been gaining in strength over the last few years with the emergence of multi-country platforms of discussions on negotiations of pricing and reimbursement of medicines. As recently as 13 June 2020, an alliance of four countries have reached a deal to pre-purchase and make available COVID19 vaccine, once approved (cfr. Euronews and other media outlets). Such efforts need consolidating and unifying while respecting current treaty competences.

Cooperation should rely upon the principles of volume and evidence generation. The Programme should also fund initiatives that can favour the reduction of uncertainties on efficacy and effectiveness of medicines, an essential need, not only for national healthcare systems but also for patients and clinicians. In particular, medicines for rare diseases pose many different challenges to competent national authorities for pricing and reimbursement, not least as they increasingly tend to arrive at the

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time-point of marketing authorisation with higher levels of uncertainty on efficacy and effectiveness. The level of evidence available is often insufficient for health technology assessors to perform a stringent assessment on effectiveness and for national competent authorities on pricing and reimbursement to make a well-informed decision.

The Programme should also include financing to facilitate access to treatment across borders for people for rare diseases to approved treatment when not available in their country of origin, as indicated in existing legislation on patients' rights in cross border healthcare.

D) Health Technology Assessment (HTA)

The Commission adopted a proposal on Health Technology Assessment (HTA) to support cooperation on health technology assessment at Union level. Even before the momentum created by the COVID 19 pandemic for increased EU cooperation in health, it emerged that a strengthened HTA cooperation between Member States is pivotal to ensure access to high quality, timely and reliable assessments of the best available evidence to support decision-makers. In addition, the HTA proposal has been recognised as an important component for achieving the objectives of the President's Mission Letter to Commissioner Kyriakides. Therefore, the EU4Health Programme should clearly spell out the support for the implementation of the future EU framework for HTA cooperation.

Support amendments: 10, 23, 75

Suggestions for new amendments:

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Recital (11) As in the time of health crisis emergency health technology assessment as well as clinical trials can contribute to the rapid development of medical countermeasures the Programme should provide support to facilitate such actions. The Commission has adopted a proposal11 on Health Technology Assessment (HTA) to support cooperation on health technology assessment at Union level.	Recital (11) ***In*** the time of health crisis emergency Clinical Trials and Health Technology Assessment (HTA) can contribute to the rapid development, identification and availability of medical countermeasures. The Commission has ***presented, in January 2018, a Proposal for a Regulation to support cooperation on Health Technology Assessment (HTA) at Union level. The European Parliament already provided its position expressing a large support for the Proposal, which is awaiting the position and approval of the European Council.*** ***A cooperation among Member States has been running for more than 20 years on a voluntary basis. The Commission Proposal aims at making this coordination fully operational and sustainable under the EU budget.*** ***The Proposal profiles the creation of a permanent secretariat (also known as Coordination Group) to support Member States cooperation in the timely identification and assessment of relevant health technologies, so as to ensure the necessary transparency and consistency on the medical and scientific information to be made available to Member States, with the due involvement of experts and patients.*** ***The programme should provide support to facilitate such actions, including the creation of a scientific committee to advice the HTA Coordination Group of Member States on the technologies which are meant to benefit the most from a common assessment. Medical and scientific evidence must override any national administrations' political or industrial interests.***

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Justification *The importance of HTA for a European integrated health system obliges us to remind the importance of the Commission Proposal, approved by the Parliament and blocked in the Council.* *The Amendment reminds the terms and the conditions of the Proposal, including transparency, evidence-based medicine, patient engagement, and calls for support by this programme.*

Recital (15a) new	*Amendment* ***The rights of patients also extend to their right to be involved in any decision that affect their lives. Furthermore, the participation of patients and citizens is key for civil society to witness how decisions are made, as a matter of transparency and trust.*** ***In that sense, the role of patients is clearly stated in most EU legislations on pharmaceuticals. At the European Medicines Agency (EMA), the European legislator decided patients are members with full rights in the Committee for Orphan Medicinal Products (Regulation EC 141/2000 Art.4), in the Paediatric Committee (Regulation EC 1901/2006 Art.4), in the Committee for Advanced Therapies (Regulation EC 1394/2007 Art.21), in the Pharmacovigilance Committee (Regulation (EU) No 1235/2010 art.61), and in the Management Board (Regulation EC 726/2004 Art.65).***
Justification *Patients' rights include their engagement in any decision-making that affect their lives.*	

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Proposal for a regulation **Recital (21)** *Text proposed by the Commission* In accordance with Article 114 TFEU, a high level of health protection should be ensured in the legislation adopted by the Union for the establishment and the functioning of the internal market. On the basis of Article 114 TFEU and point (c) of Article 168(4) TFEU, a considerable body of Union acquis was developed which guarantees the high standards of quality and safety for medicinal products and medical devices. Given the rising healthcare demand, Member States' healthcare systems face challenges in the availability and affordability of medicines and medical devices. To ensure a better public health protection as well as the safety and empowerment of patients in the Union, it is essential that patients and health systems have access to high quality healthcare products and can fully benefit from them	*Amendment* In accordance with Article 114 TFEU, a high level of health protection should be ensured in the legislation adopted by the Union for the establishment and the functioning of the internal market. On the basis of Article 114 TFEU and point (c) of Article 168(4) TFEU, a considerable body of Union acquis was developed which guarantees the high standards of quality and safety for medicinal products and medical devices. Given the rising healthcare demand, Member States' healthcare systems face challenges in the availability and affordability of medicines and medical devices. To ensure a better public health protection as well as the safety and empowerment of patients in the Union, it is essential that patients and health systems have access to high quality healthcare products and can fully benefit from them***, based on a transparent, consistent patient-oriented medical information. That is expected to be ensured by a stable and permanent cooperation on Health Technology Assessment, in support of the Member State decision-making.***

Article 4.9 support integrated work among Member States, and in particular their health systems, including the implementation of high-impact prevention practices, and scaling up networking through the European Reference Networks and other transnational networks;	Article 4.9 support integrated work among Member States, and in particular their health systems, including the implementation of high-impact prevention practices, ***the identification of health technologies meant to benefit from a European assessment***, and scaling up networking through the European Reference Networks and other transnational networks;
The Amendment translates into specific objectives the point on HTA explained in Whereas 11 and 21, giving a more concrete idea of the what has to be achieved as ultimate goal.	

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Proposal for a regulation **ANNEX I** *Text proposed by the Commission* (xiii) Support an Union framework and the respective interoperable digital tools for cooperation among Member States and in networks, including those needed to enable Member States to deliver joint clinical assessments and joint scientific consultations to exchange outcomes of HTA cooperation.	*Amendment* (xiii) Support an Union framework ~~and the respective interoperable digital tools for~~ to ***strengthen health technology assessment*** cooperation among Member States ~~and in networks, including those needed~~ ***in order to*** enable Member States to deliver ***and exchange timely, reliable and high quality*** joint clinical assessments, ~~and~~ joint scientific consultations ***and other relevant activities to support decision-makers***. ~~to exchange outcomes of HTA cooperation~~. ***(xiv) Support an Union framework and the respective interoperable digital tools to facilitate cooperation among Member States.***
Justification *See above*	

E) European Reference Networks (ERNs) - General points

ERNs Networks are a ground-breaking new structure that represent a unique opportunity that, based on the innovative use and sharing of knowledge and health data across borders, aim to improve diagnosis and care for people living with a rare or complex disease.

To fulfil this goal, ERNs need to be supported by a budget that matches the ambition.

As highlighted by the European court of Auditors, in their "*Special report no 07/2019: EU actions for cross-border healthcare",* the "ERNs face significant challenges to ensure they are financially sustainable and are able to operate effectively within and across national healthcare systems".

The Programme shall support with direct grants and adequate funding the coordination of both existing and future ERNs. It will scale up current funding to ensure that that the Networks fulfill the objectives set out in their mission.

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Proposal for a regulation **Recital (26)** *Text proposed by the Commission* Cross-border cooperation in the provision of healthcare to patients moving between Member States, collaboration on health technology assessments (HTA), and European Reference Networks (ERNs) are examples of areas where integrated work among Member States has shown to have strong added value and great potential to increase the efficiency of health systems and thus health in general. The Programme should therefore support activities to enable such integrated and coordinated work, which also serves to foster the implementation of high-impact practices that are aimed at distributing in the most effective way the available resources to the concerned population and areas so as to maximise their impact	*Amendment* **Recital (26)** ***The*** Cross-border cooperation in the provision of healthcare to patients moving between Member States***, as established by Directive 2011/25, included the*** collaboration on health technology assessments (HTA), and European Reference Networks (ERNs)***. Those*** are examples of areas where integrated work among Member States has ***already*** shown to have strong added value and great potential to increase the efficiency of health systems and thus health in general***. Nevertheless, those areas are not yet developed, both in terms of European legislation, implementation and resources.*** The Programme should therefore ***aim at completing the full development and implementation of*** such integrated and coordinated work ***in those areas***, which also serves to foster the implementation of high-impact practices that are aimed at distributing in the most effective way the available resources to the concerned population and areas so as to maximise their impact.
Justification *The Amendment provides corrections and precision about the mentioned Cross Border Healthcare Directive, together with the link to the HTA and ERNs legislations, state of play, and necessary future developments under the support of this Programme.*	

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Proposal for a regulation **Recital (27)** *Text proposed by the Commission* The ERNs, established pursuant to Directive 2011/24/EU of the European Parliament and the Council are virtual networks involving healthcare providers across Europe. They aim to facilitate discussion on complex or rare diseases and conditions that require highly specialised treatment, and concentrated knowledge and resources. As the Networks can improve the access to diagnosis and the provision of high-quality healthcare to patients with rare conditions and can be focal points for medical training and research and dissemination of information, the Programme should contribute to the upscaling of networking through the ERNs, and other transnational networks. It should consider the extension of ERNs beyond rare diseases to communicable and non-communicable diseases such as cancer.	*Amendment* **Recital (27)** The ERNs, established pursuant to Directive 2011/24/EU of the European Parliament and the Council16 are virtual networks involving healthcare providers across Europe. They aim to facilitate discussion on complex or rare diseases and conditions that require highly specialised treatment, and concentrated knowledge and resources. ***ERNs are a ground-breaking new structure that represent a unique opportunity that, based on the innovative use and sharing of knowledge and health data across borders, aim to improve diagnosis and care for people living with a rare or complex disease. Therefore, the Programme shall support with adequate funding the coordination and collaborative activities of both existing and future ERNs through grants or other instruments that are fit for purpose. It will scale up current funding to ensure that that the Networks fulfill the objectives set out in their mission.*** As the Networks can improve the access to diagnosis and the provision of high-quality healthcare to patients with rare conditions and can be focal points for medical training and research and dissemination of information, ***the Programme*** should ***also*** contribute to the upscaling of networking through the ERNs, and other transnational networks. It should consider ~~***the extension***~~ supporting the creation of new ERNs to cover ~~***beyond rare diseases to communicable***~~ ***infectious*** diseases***, rare and complex pregnancies and rare and complex mental health diseases***, *and* non-communicable diseases, such as cancer.

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Justification *ERNs are a ground-breaking initiative that make cross-border healthcare a more tangible reality for people with rare and complex conditions in Europe, while relying upon knowledge and data sharing across experts in EU countries. To fulfil their goals and be true to their mission, ERNs need to be supported by a budget that matches the ambition. As highlighted by the European court of Auditors, in their "Special report no 07/2019: EU actions for cross-border healthcare", the "ERNs face significant challenges to ensure they are financially sustainable and are able to operate effectively within and across national healthcare systems".* *The Special Report recalls that each ERN coordinator currently receives €1 million over a period of five years in EU funding for administrative costs. A Commission survey of ERN coordinators in January 2018, to which 20 ERNs responded, showed that sustainability of financing is one of the top two challenges facing the ERNs. 17 of the 24 ERNs have included identification of other funding sources within their objectives or risk-mitigation strategies.* *The public funds to sustain the ERNs operations should have a direct link and proportionate between size and scale of ambition and activities, through a 'cost and volume' model, combining a fixed payment for all Networks of the same amount regardless of size (structural costs, such as the staff of the project management team), complemented with an additional payment proportionately based to the size of the Networks (networking activities and logistics).* *It is therefore of primary importance that each ERN is supported with increased funding that secure the Networks' sustainability in the long-term. Adequate funding is needed for coordination (structural costs) and also to support the development of networking/collaborative activities in the areas of care, training, knowledge sharing and research. The instruments used to channel this funding could be grants or any other instrument, but the important aspect is that the financial reporting is simplified to reduce the bureaucratic burden for the ERNs.* *As for the scope of future ERNs, advanced discussions are taking place on the creation of ERNs on rare and complex mental health diseases, rare and complex pregnancies and infectious diseases. Finally, four existing ERNs are already covering rare cancers.*

Article 14 6. Under the Programme, direct grants may be awarded without a call for proposals to European Reference Networks. Direct grants may also be awarded to other transnational networks set out in accordance with EU rules.	Article 14 6. Under the Programme, direct grants ~~may~~ ***shall*** be awarded without a call for proposals to European Reference Networks ***and with a simplified financial and technical reporting system***. Direct grants may also be awarded to other transnational networks set out in accordance with EU rules.

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Justification

As reported in the European Court of Auditors Special Report n° 07/2019: EU actions for cross-border healthcare, "ERN coordinators consider that participating in the numerous calls for proposals run by the Commission imposed significant administrative burden". In order to support the ERN initiative, whose EU added value is recognised in the Programme as well as their great potential to increase the efficiency of health system, it is important that the resources devoted to the functioning of ERNs are focused on core rather than purely administrative tasks.

I. *ANNEX*

II. *Additional points about European Reference Networks (ERNs)*

European Reference Networks are a still at their infancy, as recalled in the Opinion by the Expert Panel on Effective Ways of Investing in Health, and they need to be strengthened in order to fulfil their ambition, as emphasised by the European Court of Auditors in their 2019 Special Report n°7. In order to achieve their objectives, ERNs require financial support to support not only their coordination, but also the numerous collaborative activities that the Networks are expected to perform. Moreover, their integration into national healthcare systems is still far from being achieved, yet it is essential to their success. Last but not least, all the above should be supported by a preliminary need-based assessment of the performance of the Networks in their first years of life.

Details of the proposed actions are provided below, with additional points that could be added to the report and related justifications:

1. Rare disease populations needs assessment and ERNs quality improvement

- ***fund a methodologically robust needs assessment study to capture the current needs and expectations of the rare disease population.***

Justification: To be effective and useful in assessing the impact of the Networks over time, the ERNs evaluation framework should be mapped against the relevant population needs that the Networks aim to address. Today our understanding of these needs is outdated and based on surveys that were conducted more than 10 years ago (EurordisCare2 and EurordisCare3).

- ***Adequate funding to sustain the implementation of the ERNs continuous assessment, monitoring, evaluation and quality improvement system (AMEQUIS)***

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Justification: The Assessment, Monitoring, Evaluation and Quality Improvement System (AMEQUIS) will facilitate political and technical accountability by showing to what extent the ERNs deliver on their mandate, objectives and ambition to tackle the public health needs of rare diseases and reduce inequalities for EU citizens within and between Member States.

At the same time, developing and implementing a fully fledge and integrated quality improvement system that builds on different domains (assessment, monitoring and evaluation) is now critical to shift the ERN system from deployment to full operations. The system will facilitate a continuous learning and quality improvement cycle within the Networks day-to-day operational activities, while at the same time guaranteeing a high level of quality assurance of the Networks and its individual members.

2. A renewed push of national Rare Disease strategies and integration of ERNs into national health systems

- ***Support Member States in the revision of their rare disease national plans to enact the necessary financial and organisational arrangements to integrate effectively the European Reference Networks system into the national health systems.***

Justification: For the ERN system to deliver on its full potential, MS need to implement the necessary financial and organisational arrangements to effectively integrate this new system into their own rare disease healthcare systems. A new national planning process will strengthen MS individual approaches to RD and will force them to have an approach to rare diseases taking into consideration the lessons-learnt and the renewed focus on facilitating cross-border healthcare via the ERNs and beyond.

- ***Support Member States in strengthening their Centres of Expertise for rare diseases to build the national health systems competencies to diagnose, treat and manage these diseases and at the same time increase the collective capacity and knowledge of the ERNs.***

Justification : Member States must strengthen the capacities of their Centres of Expertise to enable them to undertake the core functions set out in the EUCERD Recommendations. Enhancing the capacity of Centres of expertise is a win-win for both the Member States and the Networks; it will build the health systems competencies to address the needs of people living with a rare disease locally, while increasing at the same time the ERNs collective capacity and knowledge.

- ***Support Member States in developing well-organised national networks of Centres of Expertise to pool resources and expertise at national level, provide a structure for patients and clinicians to***

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work more closely across administrative boundaries and facilitate the integration and connection with the European Reference Networks.

Justification : Ultimately, the same rationale that underpins the ERN system is also valid at national level: pooling together resources and expertise on rare diseases at national level, could contribute to improve the delivery of healthcare and reduce the disparities in access to care for RD patients that we see in some Member States. At the same time, well-organised national networks for rare diseases complement, streamline support and connect national healthcare systems and the efficient operations of ERNs (which should complement but never unnecessarily replace national pathways for patients). National networks of Centres of Expertise would also reduce the weight of membership under the Networks, making them more streamlined and manageable.

- ***To advance the integration of ERNs into national health systems, the Programme will support the organisation of national multi-stakeholders workshops on integration to stimulate local discussions, as well as the development and implementation of the set of policies, rules and procedures required to anchor the ERN system to the national level.***

Justification : The ERN Board of Member States Statement on Integration and EURORDIS Recommendations on the Integration of European Reference Networks into National Health Systems, set out clear, concrete actions in five areas – i. Member State support; ii. Development of national networking between ERNs members and local healthcare providers; iii. Defining the referral pathways to HCP Members and to the ERNs; iv. Using the Networks´ activities and products – guidelines and consensus statements, training webinars, virtual consultations etc.; and v. Development of national care pathways for rare diseases. Each Member State needs to define its action plan and allocate resources to progress in these 5 areas.

3. Cross-ERN collaboration and multi-disciplinarity at ERN level

- ***Earmark funding to create effective and permanent mechanisms to build cross-ERNs collaboration to address the multi-systemic needs of rare diseases and to facilitate diagonal networking between different specialities and disciplines.***

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Justification : Regular cross-ERNs collaboration is not yet well established. A main priority over the five next years should be to create effective and permanent mechanisms to build cross-talk between the different Networks and cross-ERN working to address the multi-systemic needs of most rare diseases.

Likewise, multi-disciplinary collaboration at Network level is practically inexistent and this is preventing the Networks to deliver on their full potential. The multidisciplinary collaboration that exists at the level of the expert centres must be transferred to the ERNs discussions and activities through the active participation of other specialists and professionals (social workers, physiatrists, psychologists, physiotherapists etc,) in the collaborative activities and meetings. All Networks should have in place the mechanisms to integrate health professionals from other disciplines and should be running multidisciplinary collaborative activities on a regular basis, including developing decision support tools, research, education and training.

4. ERNs Collaborative Activities: Care, Knowledge Sharing, Training and Education and Research.

Care

- ***Adequate funding to consolidate and expand the ERN model of cross-border healthcare by securing the provision of a range of clinical services through different channels, including online second opinion and specialist advice for patients on treatment and management and virtual « online out-patient » clinics.***

Justification : Consolidating the provision of cross-border healthcare services by the ERNs will require financial resources to develop the organisational aspects (clinical governance, pricing and reimbursement models), provide the technical means and address the privacy and ethical aspects linked to the provision of routine cross-border healthcare by online means.

Knowledge Sharing

- **The Networks need additional direct funding for a common Clinical Decision competence centre to support further the revision and development of clinical practice guidelines and consensus statements.**

Justification: The ERNs should be supported to develop a library of evidence-based guidelines and expert opinions that cut across the whole care pathway from diagnosis to long-term follow up for all rare diseases included in their operational scope. A common dedicated 'in-house' Guideline office should provide support on selected functions such as literature review and methodological training in the

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assessment and endorsement of existing guidelines and consensus statements and the development of new clinical decision support tools.

- ***Funding to promote the use of digital technologies in clinical practice to assist clinicians in making diagnosis and treatment decisions, risk-adjust the care pathway and help to optimise care coordination.***

Justification : Adoption and uptake of clinical decision support tools and clinical pathways by clinicians across all MS will be one of the measures for success of the ERNs. In this way, the full collective knowledge and expertise of the ERNs will translate into the delivery of quality clinical care for all patients living with a rare or complex condition regardless of where they live. However, developing scientifically accurate, evidence-based clinical decision support tools is the first step. There are different barriers for the uptake of clinical guidelines by clinicians including the way the information is transmitted. Digital tools can help bring that information to frontline clinicians and to assist them in their decisions. Additionally digital technologies can also use the data collected on health outcomes to improve care delivery by risk adjusting the care pathway and of course by facilitating the communication and coordination of care.

- ***Additional investment to improve and further develop the ERN registries.***

Justification : The quality of the ERNs registries is the cornerstone of the ERNs structure that will unleash the potential of knowledge sharing to improve the quality of care for people living with a rare disease. An integrated ERN registry infrastructure essential (i.e. common rules on data collection, standards, access policies, etc.) is also to build the integrated quality improvement system envisaged in the ERNs Assessment, Monitoring and Evaluation Quality Improvement System (AMEQUIS).

- ***Support for the ERNs in identifying and collecting patient-centered outcome measures, including clinician-reported outcomes, performance outcomes, patient-reported outcomes, patient-reported experience and biomarkers as well as the refinement or development of new measurement instruments to fill the gaps where needed.***

Justification : Over the next years all Networks should be collecting on a regular basis outcome measures. Benchmarking these outcome measures, is critical to facilitate continuous improvement and learning from differences in clinical practice of Members within the Network. Expert centres should increasingly share their outcomes and analyse the differences in clinical practice to further develop the evidence base for care and management of each rare disease. At the same time, as part of their collaborative research activities

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ERNs will need to identify and collect outcome measures to include in clinical trials and other research activities to assess the effectiveness of treatments.

Education and Training

- ***Additional financial, administrative, and technological support to develop integrated educational programmes, combining online and face to face trainings and including trainings in areas that have so far been neglected such as:***

- ***training activities for patients and their families (empowering patients and families for self-care and management)***
- ***clinical governance (emerging clinical best practice, evidence-based case, clinical audit and medical errors)***
- ***supra-specialised healtchare training – such as innovative surgical techniques, etc.***
- ***training for GPs and other specialists on rare diseases***

Justification: With the weight of ERN Members coming from the 5 largest Western European Member States, these centres have the opportunity and responsibility to secure increased knowledge and share their expertise with health professionals throughout all EU Member States. These activities can significantly contribute to build the EU health systems capabilities and position the EU as a global leader on rare diseases. For the Networks' experts to share meaningfully their knowledge and support the development of the local health care systems capabilities additional funding and support is required in this area. Currently funding opportunities remain restricted to grants for short-term exchange of medical professionals. The ambition (and potential) for the Networks to promote and deliver medical training and education activities **along the chain of care in** all Member States remains yet to be fulfilled. Significant increased support, resources and e-training tools are needed to unlock this potential and support the development of local healthcare systems competencies in rare and complex diseases.

ERN Common Data Management Center

- ***Development of a common data management center to support ERNs in all aspects related to data collection, curation and data management as well as providing technical and project management support for all the Networks.***

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Justification: The data needs of the ERNs are bound to grow over the next years. Adequate support and governance in this area is required to match the ambition of the Networks. Specifically, this common infrastructure would provide data services such as common data collection protocols; data curation services; data management and data analytics tools and services; cloud computing services and engineering support; health data governance framework and policies and ethics oversight. Additionally, to streamline the implementation of data-related projects and aggregate demand, a dedicated project team will provide technical expertise and expert advice to the ERNs (for example on data collection and analysis, statistics, epidemiology, etc.). This team could also include project managers with experience in health IT projects that would implement common methodologies for change management and user engagement and liaise with the ERNs HCPs IT teams to be the bridge between these and the service providers of the projects in which 2 or more ERNs will be involved.

Research

- ***Funding to support individual Networks to study a minimum of five diseases to develop natural history studies to get ready for clinical testing and improve their chances to secure funding to develop clinical trials.***

Justification: To secure progress of ERN collaborative research activities in the short term, each ERN should agree on very specific commitments around the development of strong natural history studies that provide insights into the causes and progression of the diseases, ways to measure outcomes of treatments and biomarker studies.

- ***Funding to support the creation of Clinical Research Networks for collaborative research and excellence within the ERNs, the Programme should fund the deployment of services in 4 core areas: clinical research services, data management, engagement and dissemination services and administrative support.***

Justification: The Coordination and Support Action funded by the H2020 programme to support the creation of Clinical Research Networks, should lay the ground to set up the core services in the 4 areas: Additional funding is required to gradually deploy the core services so that they will be fully operational in 2025. Specifically, each core service will include:

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- Clinical research services: protocol development, guidance on the design and implementation of natural history studies, biostatistics and study design support, support in establishing ethics committees, coordination of training activities, support and guidance on the translational research process and support and guidance in identifying patient-centred outcome measures and tools to find existing measurement instruments (library of common outcome measures and disease-specific items).

- Data Management services to be provided through the ERN Common Data Management Centre.

- Engagement and dissemination services – delivery of a broad outreach plan that extends to basic and clinical researchers, academic and practicing physicians, patients, and the general public and an engagement strategy to facilitate the interactions of ERNs researchers with research infrastructures (BBMRI, EATRICs, EORTC, RD Connect, Biobanks, etc.) and IRDiRC Clinical Research Network TF and maintenance of an internet-based web-portal to serve as a central access point to information generated by the ERNs clinical research networks.

- Administrative support. Overall coordination for the ERN Clinical Research Networks and management of activities, including ERN research working group meetings, oversight and coordination of all services; support for European Patient Advocacy Group meetings on research-related activities; draft of the ERN CRNs Strategic Research Agenda (horizon scanning to identify common areas of strategic interest) and preparation of annual reports

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