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Summary of Proposal (Draft for discussion)
"Expectations and Eligibility Criteria for European Reference Network of Centres of Expertise for Rare Diseases – ERNCoE"

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European Reference Network of Centres of Expertise

1. **Two essential pre-conditions:**
   1. **Professional qualification. Both clinical and scientific experience. Proven qualification documented by publications & grants & already existing certification or accreditation.**
   2. **Commitment to cooperate & share information.**

Importance of the general « atmosphere & attitude»: trust rather than competition among experts to ensure effective cooperation.

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2. Patient's access to a multidisciplinary team of experts:
- not only at CoE level, also at ERN level
- full competence do not exist in single Member state
- cross-disciplinary approach needed to tackle RD patients needs
- for diagnosis, care, treatment, therapeutic research

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3. Importance of coordination between professionals:
- coordination intra-CoE, between CoE within ERN, between CoE and proximity/primary level of care
- coordination between care and research activities
- an intelligent way of circulating the information, organising the continuum of activities (care, follow up care, research) placing the patient at the centre, making better use of existing expertise and resources
- improve quality of care & reduce patient psy. burden (feeling of being lost in the system, lack of support, language, etc)

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4. Importance of global approach (holistic, comprehensive) between medical and social levels

- both at CoE level and ERN level; not only a local service

- social support often overlooked; patient unsatisfaction twice higher than for medical services;

- need to develop common European approach to social services for given rare diseases and methods to support patients, increased quality of life,

- specific administrative tasks for ERN to support patient mobility for cross border care & to get reimbursement when travelling, reimbursement of medical services etc.

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5. Capacity to pool together patients = the critical mass of patients will enhance knowledge on the disease and allow for meaningful conclusions related to care in a field where scarcity undermines knowledge/quality; identification of unknown disease cause; management of complex or rare disease situation; power of patient number in clinical trials

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6. General expectation:

- Elaboration of agreed best parcatice, standards and guidelines for diagnosis and treatment of rare diseases at international level.

- Dissemination of European reference diagnostic and therapeutic protocols will ensure equity at EU level by reducing the impact of the "postal code lottery" and will increase trust in local services

- Provision of expert opinion, confirmation of diagnostic or therapeutic options

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7. Participation into research activities at European & international levels:
- linking excellence of care with excellence of research, where the patients are, where is the multidisciplinary expertise on the disease,
- multi-centre clinical studies; facilitate partnership with pharmaceutical companies
- shared databases, shared biological resources (DNA, RNA, Tissue, Cells), registries (harmonization of procedures), international epidemiological surveillance, pharmaco-vigilance
- participation into EU-funded research projects

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8. **Perform education, information, communication** outreach activities towards the public & primary health care professionals (to improve referrals & follow up)

9. **Perform training** activities for health professionals, including exchange of staff, meetings and conferences to exchange best practices, harmonised processes, disseminate standards & guidelines

10. **Perform activities to empower patients** at different levels: information, education, training, so to build patient and families capacities in managing the medical & social aspects of their rare disease, enhance their autonomy, increase their compliance, help improving their quality of life. A patient outcome.

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11. **Collaborate with patient organisations at different levels:**
    - patient organisation contribution into the management and evaluation of the Networks
    - facilitate creation of patient groups
    - improve links and exchanges between professionals (care & research) and patients
    - could be link to information, communication, educational and training activities of ERN
    - need to link more broadly ERN, Research network and patient organisations

12. General agreement that ERN should be:

- initially evaluated and accredited at EU level via agreed set of criteria (minimum set of standardised criteria and objectives + additional ones)

- regularly assessed on common indicators with soft values and hard values, and « patient outcome measures ».

Need to develop methods and tools to:

- perform regular self-evaluation by ERN

- measure patient outcomes, long term & across ERN.

13. Financial and management aspects of ERN:

- are perceived to be cost-effective,

- need proper funding for their specific European & international activities

- need long term sustainable public funding

- share good governance practice (organisation, leadership, regulate, steering committee) and coordination practice between ERN

- ability to disclose procedures and results and to benchmark

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14. Importance of flexibility when selecting the types of centres belonging to the networks and flexibility for geography of the networks.

- Not all Member states; 1 country = 0, 1, 2 or more CoE; to be adapted to the 3 type size of countries; according to reality of the local healthcare organisation; ERN role in accreditation of CoE.
- Patient acceptance to travel (to be confirmed and detailed).
- Different suggestions were made such as two level of CoE with identification of "leading centres" and "associated centres"; and possible "sub-national networks" with "centres of competence".
- Different typology of centres to be grouped within a European network e.g. CoE embedded in different medical specialities, children/adult CoE, specialised health care professionals.
- Large networks complemented by "sub-networks" with different very rare diseases to be grouped within the same EU network, etc.
- Excellent contribution of new Member states to ERN.

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Further comments by EU patients

Overall positive comment: the ENCR will generate greater awareness of differences between Member State healthcare systems (benchmark) and will put pressure on Member States to close gaps in access to and quality of care for rare disease patients.

Risk identified: create networks may generate disproportionate bureaucracy for accreditation and evaluation mechanisms. Adaptation & sustainability of mechanisms for expenditures and reimbursement will have to be agreed at EU level. Political commitment of Member states to financially support CoE. This will need Member states political willingness to defer national autonomy in pursuit of pan-European equity.