Expert Panel on Effective Ways of Investing in Health (EXPH)

Opinion on
Application of the ERN model in European cross border healthcare cooperation outside the rare diseases area

The Expert Panel has been requested to analyse the following:

(a)What are the areas that can benefit most from the ERN model of cross-border cooperation?

(b) Should the ERN model be used as a whole (covering diagnosis and treatment, research, training, knowledge generation…) or only include specific modules of collaboration?

(c) How would national health systems integrate such networks into their national framework?

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Ever since the Treaty of Maastricht, the European Union has recognized the contribution that cooperation in health services can bring to the people of Europe, while respecting the rights of Member States to decide how they will organize health services within their own national borders.

The 2011 Directive on the Application of Patient's Rights in cross-border care (cross-border directive) sets out the conditions under which a patient may travel to another EU country to receive medical care and reimbursement.

It covers healthcare costs, as well as the prescription and delivery of medications and medical devices.

In addition, the directive established the principle of voluntary cooperation between healthcare units in member states in highlight specialized healthcare.

In accordance with the article 12 of the cross-border directive ERNs were established in 2017 as cross-Europe virtual health-provider networks, aiming to facilitate discussion on complex or rare diseases that require highly specialized knowledge or treatment.

The expectation of ERNs is that they will offer the following benefits:

- access to a much larger, cross-border pool of expertise and knowledge, increasing the chances of receiving the best advice to diagnose and treat their diseases, particularly for small member states;

- bringing together patients and resources, enabling economies of scale for accessing treatment for rare diseases;

Starting from March 2017, 24 ERNs have been established, bringing together over 300 hospitals from 26 member states (MS): the number of MSs participating in each ERN varies between 8 and 19, and the number of reference centres in each MS varies between 1 and 21 (with many reference centres taking part in more than one ERN)
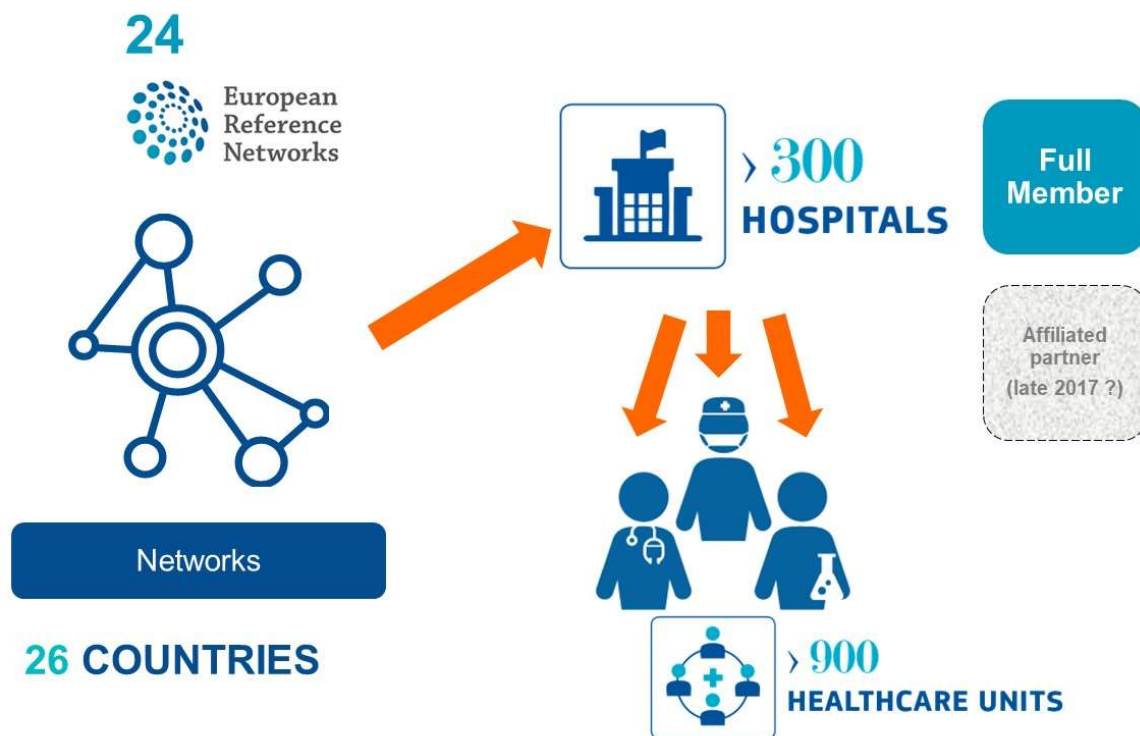
BOND ERN	Bone Diseases	**EuroBloodNet**	Onco-Hematological Diseases
CRANIO	Craniofacial anomalies and ENT disorders	**EUROGEN**	Urogenital Diseases
Endo-ERN	Endocrine Conditions	**EURO-NMD**	Neuromuscular Diseases
EpiCARE	Rare and Complex Epilepsies	**GUARD-HEART**	Diseases of the Heart
ERKNet	Kidney Diseases	**ITHACA**	Congenital Malformations and Intellectual Disability
ERN GENTURIS	Genetic Tumour Risk Syndromes	**MetabERN**	Hereditary metabolic diseases
ERN-EYE	Eye Diseases	**PaedCan-ERN**	Paediatric Cancer
ERNICA	inherited and congenital anomalies	**RARE-LIVER**	Hepatological Diseases
ERN-LUNG	Respiratory Diseases	**ReCONNET**	Connective Tissue and Musculoskeletal Diseases
ERN-RND	Neurological Diseases	**RITA**	Immunodeficiency, AutoInflammatory and Auto Immune Diseases
ERN-Skin	Skin Disorders	**TRANSPLANT-CHILD**	Transplantation in Children
EURACAN	Solid Adult Cancers	**VASCERN**	Multisystemic Vascular Diseases

Key Features:

- The process and criteria for establishing an ERN and for determining eligibility of Centres of Expertise and healthcare providers to join are established in the Commission Delegated Decision (2014/286/EU)

- ERNs must fulfil criteria for implementation and evaluation, including being patient-centred and clinically led, with a minimum of 10 members in at least 8 countries, subject to robust independent third party assessment, and endorsed and approved by National Authorities.

All Networks and their Members should be periodically evaluated, at the latest every five years after their approval or last evaluation.

Key Functions:

- To review a patient's diagnosis and treatment, ERN coordinators convene **'virtual' advisory panels** of medical specialists across different disciplines, using a dedicated **IT platform** and telemedicine tool called the Clinical Patient Management system (CPMS). This allows expertise travel to the patient, rather than vice versa.

- It allows sharing of expertise to diagnose, follow-up, and manage patients, and facilitates a multidisciplinary approach, as well as collaboration and learning across centres of expertise and networks at national and international level

- The focus is first on diagnosis and treatment by providing tools for collaboration and virtual consultations. However, there are clear potential research applications.

- These functions support the development of good practice guidelines and outcome measures for quality control, as well as contributing to research

What do we know about existing ERNs?

ERNs are a very new concept and experience in their operation is very limited.

The existing ERNs have only been operational since 2017 and a relatively small number of patients have been referred, so it is too early to determine how effective they have been.

To inform our work we have undertaken interviews with two ERN co-ordinators, Franz Schaefer, coordinator of ERKNET: European Reference Network for Rare Kidney Diseases, a consortium of 38 expert paediatric and adult nephrology centres in 12 Member States, and Marissa Tejedor Botello, project manager for TransplantChild, the ERN for Paediatric Transplantation, including both Solid Organ Transplantation (SOT) and Hematopoietic Stem Cell Transplantation (HSCT).

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How do networks benefit patients?

1. the pooling of expertise

achieved by convening specialists in member centres (which in theory improves management of all patients being treated in the centre due to knowledge sharing and development), and through uploading individual cases to an online Clinical Patient Management System (CPMS). Once a case is referred and uploaded, a panel of experts is selected, depending on the specific expertise needed. Clinical details are reviewed, and an online chat facility enables communication between the primary clinician and the expert panel. A report is then produced, providing the primary clinician advice for patient management.

Coordination of experts on rare diseases has resulted in the development of guidelines, with 39 new ones published by (ERKNET), as well as knowledge sharing and benchmarking.

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2. the pooling of patients.

creating a critical mass of patients: collecting and coordinating

experience in treating patients with rare conditions requiring

complex treatment allows the development of registries (ERKNET)

and provides a platform for research. Indeed, since the sample size

required for studies can vary greatly, even the pooling of relatively

few cases can result in new opportunities for research ;

What issues should be addressed to ensure proper functioning of ERNs?

1. robust referral mechanisms within national health systems in order to ensure equitable access for patients

2. adequate funding

3. clear management system and mechanism of governance

4. cost effectiveness

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Dimensions of the operations of reference networks

Governance	Objectives	Function	Material scope	Geographical scope
• Formal	• Efficiency	• Referral of patients	• Prevalence	• EU-wide
• Informal	• Quality		• Cost	• Transnational
• Peer structure	• Safety	• Transferring knowledge	• Complexity	• National
• Hub-and-spokes	• (Equity)		• Rare	• Interregional
• Organic	• Market position		• Critical	• Regional
			• Chronic	
			• Common	

Source: W. Palm, I. Glinos, B. Rechel, and P. Garel, "Building European Reference Networks in Health Care," *European Observatory on Health Systems and Policies,* 2013.

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In order to define what is an effective network, the first step is to define its mission

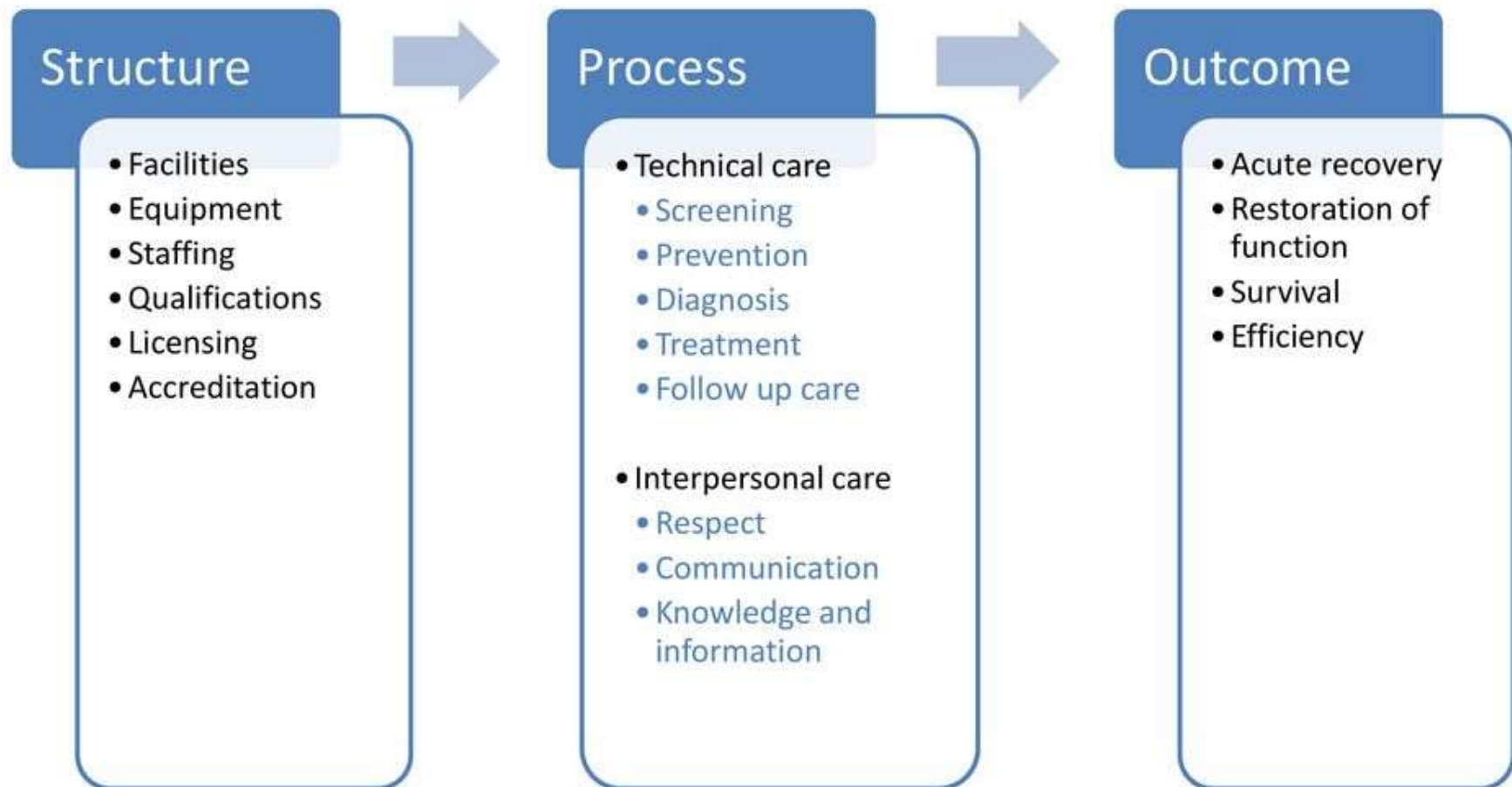the ability for networks to become integrated in the national health care systems

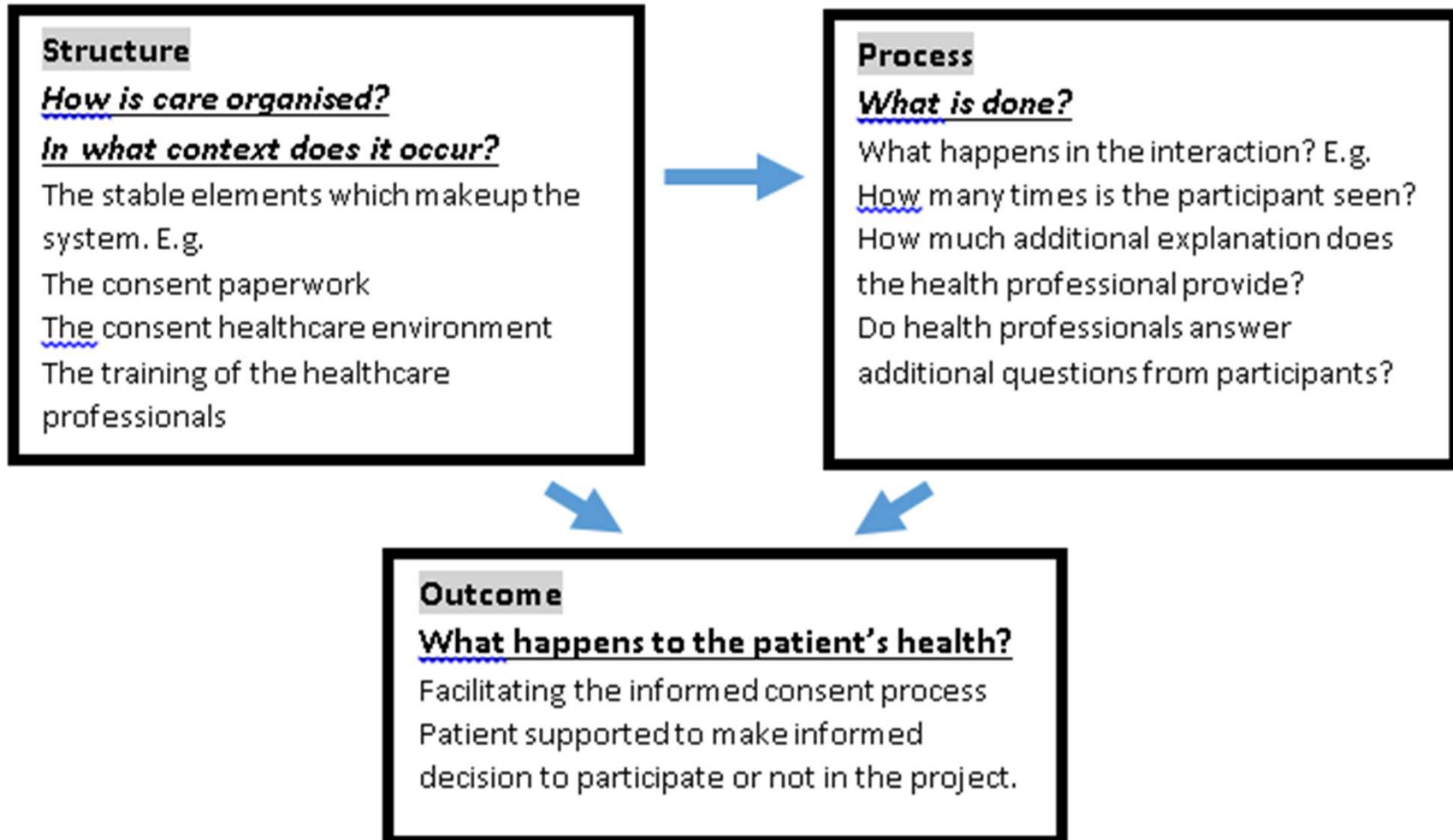to make this process of improving treatment and integration cost-effective

how they can be achieved and,
therefore, which aspects need to
be evaluated to define a network
as successful

Donabedian's well-known
structure-process-outcome
model

Donabedian's Model

Structure →

- Facilities
- Equipment
- Staffing
- Qualifications
- Licensing
- Accreditation

Process →

- Technical care
  - Screening
  - Prevention
  - Diagnosis
  - Treatment
  - Follow up care

- Interpersonal care
  - Respect
  - Communication
  - Knowledge and information

Outcome

- Acute recovery
- Restoration of function
- Survival
- Efficiency

Structure

How is care organised?

In what context does it occur?

The stable elements which make up the system. E.g.

The consent paperwork

The consent healthcare environment

The training of the healthcare professionals

Process

What is done?

What happens in the interaction? E.g. How many times is the participant seen? How much additional explanation does the health professional provide? Do health professionals answer additional questions from participants?

Outcome

What happens to the patient's health?

Facilitating the informed consent process Patient supported to make informed decision to participate or not in the project.

Are there other areas where European Reference networks might be appropriate?

One element of ERNs is the exchange of knowledge on how to treat certain conditions.

Without a structured system, such as that created by the ERNs, it is difficult to know whether different centres are providing care that is consistent with best practice, to the extent that this is known.

In the absence of such a system, it is likely that there will be variation in models of care.

This variation is not confined to rare diseases.

It is widespread in all sectors of healthcare with unwarranted variation.

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The approach adopted by ERNs to address the issues related to care for rare diseases is knowledge sharing and benchmarking.

This implies that performance of the healthcare system should be evaluated in a transparent and systematic manner in order to identify areas of best practice and those which need to be improved on.

Then the results must be disclosed to the public: making the results available offers the opportunity to share best practices and increases the physicians' awareness of the quality of their performance

The question then is whether the ERN model might be appropriate as a means of reducing unwarranted variation, offering a forum in which those managing different conditions could share experience.

We do not find this argument persuasive.

Except in small countries, there is no obvious need to create an international network to function: on the contrary, there are reports of implementation of these measures at a local and regional level with excellent results even for common conditions.

In other words, once there is a sufficient number of cases, the methods used by ERNs can be easily applied at a national, regional or even local level, since the focus is not on gathering information per se, but rather on using the information to improve healthcare, and increasing the amount of information available is useful only if it can change decisions on treatment.

Indeed, the necessity for international cooperation has only arisen for rare diseases because the volumes are too small to gather a reliable amount of data and identify differences in quality of care.

Moreover, conditions with a higher prevalence can implement the same actions as ERNs without having to face all the challenges linked to international cooperation.

Even if there is a desire for international collaboration, for example to develop guidelines for complex conditions or indications for new treatments, this can often be achieved equally well by networks developed within other frameworks, for example by European scientific and professional organisations, many of which are already engaged in such mechanisms.

A second issue is whether the ERN model can promote access to healthcare for vulnerable communities (such as cross-border communities that are currently not adequately covered by national health services), small countries, remote areas, and/or EU border regions, all of which may suffer from inadequate access to high-quality healthcare that is not limited to rare diseases.

Again, there are many other mechanisms that can address these issues, including the now numerous examples of cross-border collaboration and it is not clear what the ERN model would add to them.

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With other challenges (e.g. refugees, homeless people, disaster interventions) the needs of those affected should be addressed directly by offering appropriate service provision.

That being said, the panel urges member states and the European Union to optimise the care for these groups and the preparedness to provide acute responses, through the creation of platforms and "Learning Communities" in Europe with participation of multiple member states and experts in the different domains, in order to improve access in quality of these vulnerable people.

the current criteria for establishing a ERN, as a means of improving the management of patients with rare and complex diseases is appropriate. The ERN concept does not address other issues raised for consideration, such as remote areas and border regions, or the development of new medicines or interventions, for which there are alternative and more appropriate mechanisms.

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The application of the ERN model as a whole or part of the model will depend on the needs to which the model is expected to respond.

The ERN model can tackle one, several or all the main fields for action currently identified for rare diseases: research and innovation, generating and sharing evidence, developing guidelines, and training.

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In summary

while it is not possible to come to definitive conclusions about the role of ERNs we examined the scope of the ERNs research and guideline development, there would seem to be considerable scope for incorporating these additional roles beyond the immediate objective of providing advice on individual patients. This will, however, require dedicated resources, some of which will have to come from other sources.

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How should national health systems integrate such networks into their national framework?

Properly implemented ERNs are not an obstacle to the overall functioning of the national healthcare systems; indeed, the added value of ERNs is evident as long as the focus is on information and knowledge exchange and as long as the patient is only treated in the "foreign" system when it is appropriate and justified.

At this point it is important to consider the financial sustainability of the ERN model in the light of constraints facing some national health systems.

At present, individual cross-border patient mobility takes place on a small scale. According to the May 2015 Eurobarometer, entitled "Patients' rights in cross-border healthcare in the European Union", only 5% of EU citizens obtained treatment abroad.

The optimal way to finance ERNs in the medium-long term remains unresolved: as highlighted above, further elaboration will be needed to achieve long-term sustainability.

This issue may be a problem for all EU member states, but especially so for some of them.

Average annual growth in health spending in real terms

Source: OECD Health Data 2013
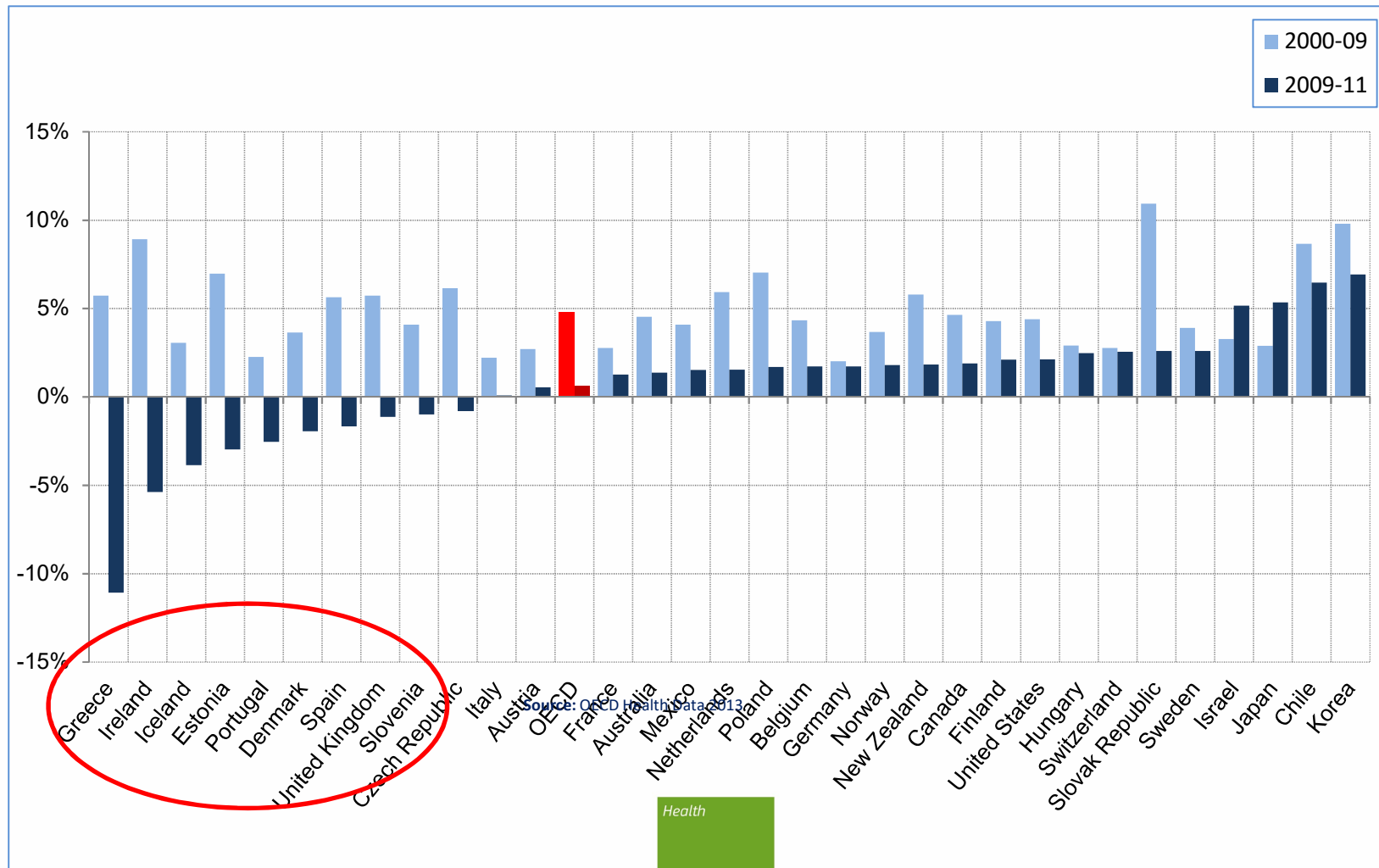
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Finally, the ERN model could usefully be applied within a national health system, or even a regional section of a health system, when dealing with common diseases.

Indeed, in some cases the application of the ERN model does not need an EU dimension: it might be translated with equal or higher effectiveness to a national or regional dimension.

EXPH proposes a set criteria for creating a European network.

1. The condition/ intervention must be rare (however defined).

2. The condition must occur throughout large parts of the EU.

3. The condition must be complex and there must be incomplete knowledge, benefitting from consultation among specialists.

4. The establishment of a ERN should only be undertaken when there is no existing alternative mechanism to obtain access to relevant expertise or other forms of collaboration or where a simpler solution is not possible.

CONCLUSIONS AND RECOMMENDATIONS

- the establishment of ERNs appears to have been, at least potentially, among the most important European cooperative initiative for decades.

- the extension of the ERN model to diseases other than rare diseases is currently considered premature.

- the application of the ERN model as a whole or part of the model will depend on the needs to which the model is expected to respond in fields other than rare diseases..

- EXPH recommends that well-designed, independent research is carried out on the impact of ERN on healthcare of rare diseases including the clinical (e.g., improved diagnosis), scientific (e.g., added value of shared knowledge) and social (e.g., benefits for patient's life quality, sustainability) aspects; the adaptations that may be needed in order to fit the ERN model to diseases other than rare diseases

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Finally, the implementation of ERN, for rare diseases as well as for other conditions, requires the availability of trained professionals at all levels of healthcare; it is, therefore, recommended that the lessons learned from ERNs should be reported and disseminated in ways that can feed into in the undergraduate through to post-graduate and professional education of the healthcare professions.

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Thanks for your attention

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