

PATIENTS' INVOLVEMENT IN THE ENDO ERN REGISTRY PROJECT (EuRRECa)

ENDO ERN

May 2019

Activity	Involvement in the ENDO ERN registry project
Area	<i>Research and registries</i>
Duration	Spring 2018- Spring 2021 (3 years)
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1. SUMMARY

i The landscape of endocrine registries in Europe is scattered, they are not interconnected and patient engagement has been very limited. The EuRRECa project (European Registries for Rare Endocrine Conditions), funded by the EU Health Programme, overall objective is to ensure that the European Reference Network on Rare Endocrine Conditions (Endo-ERN) achieves its mission of driving up standards of clinical care and patient-centred research through maximizing participation in disease registries.

EuRRECa ultimate goal is to maximise the opportunity for all patients, health care professionals and researchers to participate and use high quality, patient-centred registries for rare endocrine conditions covered within Endo-ERN. EuRRECa is closely linked to Endo-ERN but is open to all professionals providing endocrine care and who would like to participate.

The project, launched in February 2018, will develop the following activities:

1. set up an electronic reporting system which captures activity and allows for a better understanding of the occurrence of rare endocrine conditions;
2. develop a new core endocrine registry that collects a core dataset – a very small amount of data -; that also includes objective markers of clinical outcome;
3. signpost participants to high-quality, detailed, disease-specific and patient-centred registries that have been evaluated by EuRRECa.

2. INITIAL SITUATION

i There are more than 100 endocrine (or hormonal) disorders. As a result, a large number of separate registries have been set up. Every country, even every hospital has set up their own registry for a particular endocrine condition. This is the reason why the landscape of registries is scattered and very fragmented, just as it happens with other rare disease areas. A paper published earlier this year, identified the cross-border international registries for rare endocrine conditions that are led from Europe and concluded that, although there was a need to develop new registries, there was a more immediate need to improve the awareness and participation in existing registries¹.

It is broadly acknowledge that patient-focus registries can improve clinical care² Additionally, patient registries may also play an important role in monitoring the safety of medicines³. However, setting up good registries has proven to be challenging; the quality of existing registries for endocrine conditions is variable and interoperability is poor.

EuRRECa (European Registries for Rare Endocrine Conditions) aims to address the gaps raised in a survey amongst health care providers by developing a core registry and an e-reporting programme for all rare endocrine conditions that are covered within Endo-ERN. EuRRECa will not only increase awareness of registries amongst patients and professionals by facilitating interaction between existing and new disease registries within Endo-ERN, it will also assess the quality of existing new registries using agreed guidance.

The involvement of patients in the development of existing registries has been variable. The EuRRECa project provides for the first time the opportunity for patients to participate and convey their views and needs regarding registry data capture (what type of data) and use (how it will be used and shared).

3. CONTRIBUTION TO PATIENT ENGAGEMENT AND/OR IMPROVEMENT OF CARE

i The project plan states: EuRRECa is aimed at maximising the opportunity for all patients, health care professionals and researchers to participate and use high quality, patient-centred registries for rare endocrine conditions covered within Endo-ERN.

Patients are not only the “subjects” of the registries, but are also actively involved in the project. Not only ePAG patient advocates, but also ‘regular’ patients and parents are participating and contributing to all project areas and working groups, functioning at different levels, ranging from patients’ representatives to parents or patients who are experts on their condition or the condition of the one they care for.

Specifically, the **Patients, Parents and Ethics Working Group** is composed of 7 persons: 3 patient representatives, 2 clinicians, 1 parent and 1 ethicist (from Belgium, Netherlands, Italy, UK and Serbia).

¹ The current landscape of European registries for rare endocrine conditions (European Journal of Endocrinology (2019) 180, 89–98)

² Patient focused registries can improve health, care, and science (BMJ 2016; 354: i3319)

³ See EMA discussion paper on the use of patient disease registries for regulatory purposes [here](#)

The first task of this working group is to understand the needs and expectations for patients, parents and families in registries, ensuring that the patient focus is maintained in the development of core outcomes in the core endocrine registry; the development of clear ethical guidance for the operation of registries; the appraisal of the extent of patient involvement in existing disease registries and in the dissemination and evaluation process.

The first discussions in the Working Group have led to the following initial considerations to assess registries patient-centredness:

- Data collected in disease registries should contain patient-centred outcome measures (PCOM)
- As overall quality of life is considered the most important PCOM for all conditions, each registry should contain at least 1-2 generic (i.e. non condition-specific) question(s) on QoL
- Participants should have access to their personal data stored in a disease registry
- Participants should be able to download a personal health status record extracted from their personal data, e.g. to be shared with their professional caregivers
- A registry should provide formal possibilities for patient-initiated research questions or project proposals
- Research conclusions and study summaries should be made available for participants through the registry platform and/or other communication channels (e.g. newsletter)
- Registries should use clear and simple language, jargon and acronyms need to be avoided
- The registry platform should contain objective and up-to-date information about the condition(s) under scope
- Participants should have access to anonymized aggregated data or meta-data, e.g. about epidemiology, quality of life
- Registries, especially those on rare diseases, should offer possibilities for patients to interact with each other

Later this year, the Patients Parents and Ethics Working Group, in collaboration with the Quality Assurance and Evaluation Working Group, will start a pilot to develop assessment tools using 4 established international registries (managed by participants within the EuRRECa project).

4. SUCCESS FACTORS

- i** • **Having a good representation of patients in the ERN overall governance structure facilitates patient involvement in this kind of strategic projects.** Endo-ERN governance structure already involved patients' representatives in the different working groups. This governance model was copied into EuRRECa so patient involvement in this project was built in the project from the outset. The structure of EuRRECa (see annex) consists of 8 Expert working groups (analogue to the MTGs of Endo-ERN) and 6 working groups (Endo-ERN has 5). In every WG patients are included in the project structure and within the Patients, Parents and Ethics WG, both patients and parents are involved. Due to the matrix governance structure patients and parents interact with every part of the project.

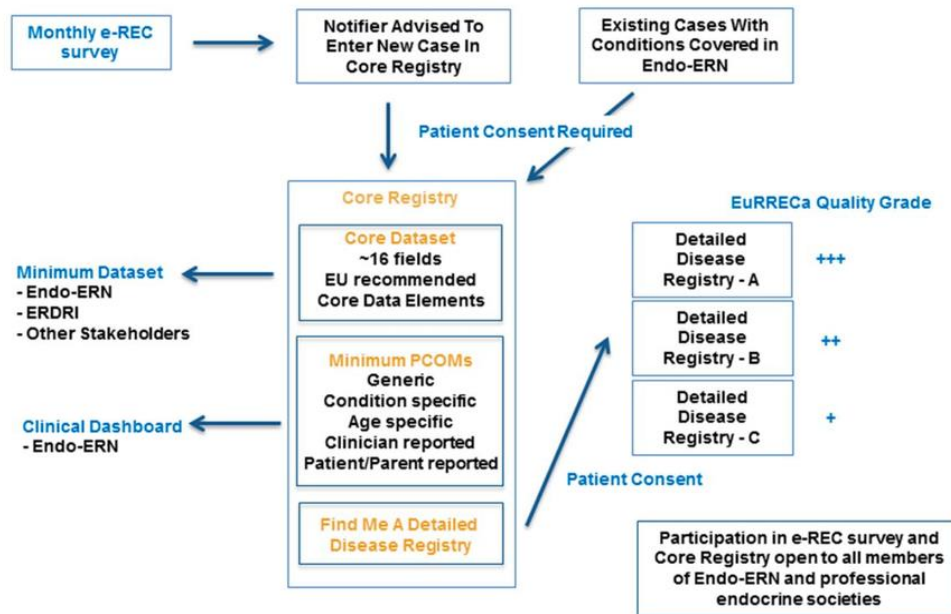
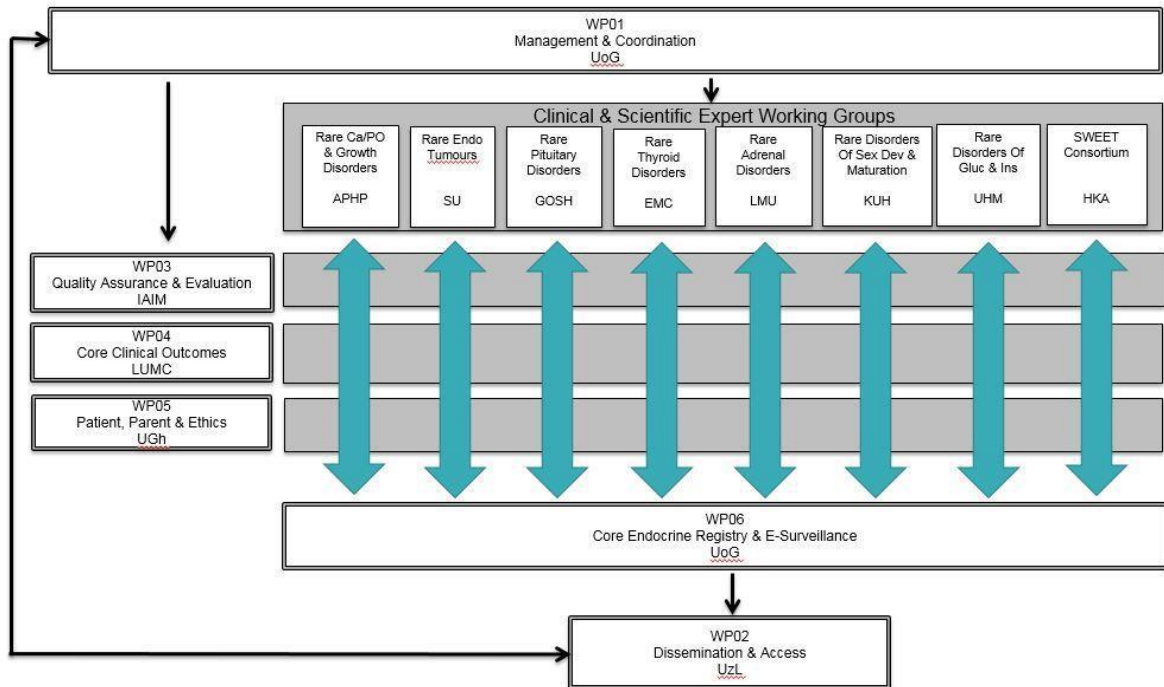
- **A practical approach to patient recruitment for this project helped to address the scarcity of patient representatives who could volunteer and had experience in registries.** Endo-ERN ePAG patient advocates were invited to join the project, but clinicians also brought in other patients with whom they had a relationship and who had experience in registries development. This might have not been the most democratic recruitment approach, but in practical terms is working in EuRRECa, partly because the governance of the project is clearly defined and everyone understands their role.
- **Clinical leads experience with patient engagement has helped to set up a work structure in which we collaborate as equal partners.** Clinicians within Endo-ERN have endorsed a governance model in which patients are involved at the same level as they are. This is the reason why patients have a prominent place in the EuRRECa project. Clinicians actively approached potential patient representatives they already knew within Endo-ERN, but also from their own networks.

5. LESSONS LEARNED

- i** The project will last for a period of three years and at this stage is difficult to share a comprehensive list of lessons learned. At this early stage of the project we can share two considerations:
 - Governance is a powerful means to guarantee good patient engagement throughout the project. Getting right the governance structure from the outset, where the patient voice is well represented and roles are clearly defined, is crucial to enable meaningful patient engagement.
 - In the initial stage of the project we have not engaged with our broader patient community to capture their feedback, partly because we believe that registries are a difficult subject for patients. The Patients and Parents Ethics Working Group does plan to reach out to patients at large, since one of the aims of the EuRRECa project is to improve awareness and patients' participation in existing registries (and, by the way, also to increase clinicians' awareness and participation). Outreach to better represent the views of the broader community is an area for improvement. We know that we will need to set up the mechanisms to get better at this.

Annex

EURRECA governance and project outline



Endo-ERN - European Reference Network on Rare Endocrine Conditions
 ERDRI - European Rare Disease Registry Infrastructure