

## PROTOCOL FOR MANAGING DISEASE AREAS WITHIN CURRENT ERN HEALTHCARE PROVIDERS

EUROPEAN REFERENCE NETWORKS  
FOR RARE, LOW PREVALENCE AND COMPLEX DISEASES

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## DOCUMENT INFORMATION

### PROTOCOL FOR MANAGING DISEASE AREAS WITHIN CURRENT ERN HCPs

Approved by ERN Board of the Member States in written procedure on 02/07/2021

#### Short Description

This document describes the procedures for the extension of diseases covered by the existing members of the ERNs (HCPs). The final goal is that this procedure would contribute to reaching as comprehensive coverage of all known rare or low prevalence and complex diseases by the ERN system as possible.

This procedure is inspired by the process carried out in 2019 to extend the coverage of existing ERNs with new diseases in addition to those established in the initial call for ERNs in 2016 (this exercise took place in the context of the call for new members of existing ERNs launched in 2019).

A number of ERNs decided to add new diseases to their coverage in 2019 and the Commission included those new diseases under the eligible areas of expertise for the new applicants who were able to apply for these additional disease areas under the 2019 call.

In this context it is important:

- to set up a mechanism for the validation of the expertise of the already existing ERN members not previously assessed or validated for the new diseases included by their ERNs in 2019.
- to set up a mechanism for existing ERN members to expand into pre-existing disease areas of their ERNs for which they did not initially apply in 2016.



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## INTRODUCTION

In 2019, a number of ERNs have updated the disease areas that they cover and added new diseases. It has been possible for applicants to the call opened on 30 September 2019 by the European Commission (EC) for new ERN members to apply for these new disease areas. However there is no mechanism for existing members of ERNs to do so. In addition, existing members of ERNs also need an opportunity to expand into pre-existing disease areas for which they did not apply in 2016.

This document is limited to the extension of expertise of an HCP for new diseases that are already recognised as being within the scope of the ERN by the ERN Board of Member States representatives (ERN BoMS).

This document is addressing the procedure to assess the expertise of an HCP for the extension of its disease-area coverage to those areas:

- already within the scope of the ERN when the HCP joined the network but for which they did not apply.
- that have subsequently been included within the scope of an ERN since the HCP joined the network (in the 2019 call for new members) .

**Timing** - It is envisaged that ERNs will implement an annual process to expand their disease areas and this is likely to begin once the new members have joined the ERNs.

## BACKGROUND

- The disease coverage of the 24 ERNs was analysed and discussed in 2015-2016 following a bottom-up approach in consultation with the broad rare diseases community, including Patient Associations, Scientific Societies, Member States and consultation bodies (Joint Action on Rare Diseases and Commission Expert Group on Rare Diseases).
- 24 ERNs applied to the 2016 call for ERNs, each proposing a defined list of thematic and sub-thematic areas of diseases, alongside the corresponding applicable criteria and thresholds that the individual healthcare providers wishing to join a network would be required to fulfil.
- Although the initial 24 Network proposals were quite exhaustive in terms of the diseases coverage, it has been deemed appropriate to consider in 2019, two years after the launch of the ERNs, whether any major gaps exist in the disease coverage of the Networks that need to be addressed.
- Several Networks have been raising this issue and, in order to better understand the perceived needs and proposals of the 24 ERNs, a survey was conducted in May 2019 on the potential disease expansion of the current 24 ERNs.
- Following the discussion held on this issue in both the ERN CG and the ERN BoMS meetings on the 24-25 June 2019, a comprehensive consultation with the ERNs was carried out with those who wished to extend their disease-area coverage. Based on this consultation, an



update of the disease coverage (including, when needed, new thresholds and criteria) was published on the Commission's website<sup>1 2</sup>.

- As a result of this, applicants of the 2019 call for new ERN members were able to apply to cover these new disease areas.

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<sup>1</sup> In June 2019, 16 ERNs replied to the 'Survey on the expansion of diseases to be covered by the ERNs'. 8 of them proposed the expansion. In September 2019, 8 ERNs included changes in the specific criteria and thresholds to add new diseases and areas of expertise.

<sup>2</sup> For any future extension of the disease coverage of the existing ERNs, the trigger for expansion should be either a needs analysis regarding new thematic ERNs or an analysis of existing disease covering gaps between ERNs. A procedure should always be in place to cross-check the opinion of other ERNs or their possible own wish to expand thematic coverage in the same direction. Finally, before entering the assessment process, the proposals should be presented to the group of coordinators, patient representatives, the EC ERN Team and the Board of Member States.



## 1. PROPOSAL

In order to update the disease coverage of the existing members (i.e. a HCP which may include different teams who are experts in different disease groups within the same ERN) and to validate their fulfilment of the new threshold and ERN specific criteria, an application procedure, supported by a peer review by the Board of Network of the ERN in question and further supplemented by the National authorities / ERN BoMS validation, is required.

In order to avoid an overly onerous process for current HCP members (units of expertise) a lighter assessment process can be applied: the HCP have already demonstrated compliance with the General Criteria, under the initial call for application and during the initial assessment of ERNs and their members in 2016. The additional team proposed within this HCP will now have to be evaluated only with regard to the new rare disease(s) for which they are applying. This assessment procedure is proposed to be structured under three steps, as described below.

Following an application submitted by an existing member to be recognised as an expert in a new rare disease area within the scope of its Network, the assessment process will be conducted under the following steps:

- Step 1: The initial step in the process will be for member HCPs to identify any changes to disease areas they wish to cover and provide details of these, along with the appropriate volumes and evidence in an application submitted directly to the Board of the Network of the relevant ERN. A standardised template for the application should be developed for this purpose. HCPs should be advised that they will need a letter of endorsement from the national Ministry of Health or similar certification at national level confirming that the existing member is recognised as an expert centre for this rare disease or complex condition under their respective national legislation.
- Step 2: The member HCP needs to notify a copy of the application to the Member State's competent authority. Upon this notification, the Member State's competent authority may either: (i) endorse the application by issuing a letter of endorsement or similar certification at national level confirming that the existing member is recognised as an expert centre for this rare disease(s) or complex condition(s) in its Member State under their respective national legislation; or (ii) take note of the application without any further action within 30 days after the notification; or (iii) reject the application. This evidence should be submitted by the applicant to the Board of the Network. In cases described under (i) and (ii) the assessment continues as explained under Step 3 below. In case described under (iii) the procedure is terminated at this stage.
- Step 3: The application will be peer reviewed by the Board of the Network of the ERN (see internal ERN review process section below for how this can be managed), and an assessment report issued. Internal review by the Board of the Network (or delegated by the Board of the Network to a dedicated Task Force or Working Group) should include engagement with patient organisations as required by the governance of that network. The Board of the Network will give a favourable or non-favourable opinion. Where this assessment is negative, the member will not be further assessed and therefore not



validated for the coverage of that disease or group of diseases. If the assessment is positive only for some of the diseases that have been assessed, it will be a decision of the Board of the Network whether to ask the ERN BoMS for the validation of only those diseases which have been positively assessed by them or to terminate the process.

- Step 4: Where the assessment by the Board of the Network is positive, the ERN BoMS and the EC will be informed of the changes by a letter from the ERN Coordinator. Applications that secure both an endorsement by the Member State's Ministry of Health and a positive opinion by the Board of the Network will be validated by the ERN BoMS. All the applications with positive assessment from the same network should be sent to ERN BoMS (for decision) and to the EC (for information) jointly on an annual basis (once a year). The ERN BoMS should consider jointly all applications received within the same period of time (annually) and validate all of them at the same (annual) meeting.
- Step 5: After the decision of BoMS the EC will inform the applicants and the ERNs concerned about the decision. The EC and the ERNs will ensure that any changes to the coverage of disease areas are reflected in appropriate information systems and website material.
- Step 6: Follow-up of the extension of the disease coverage should be made a mandatory and integral part of the annual monitoring and 5-year re-evaluation by the IAB.

## 2. INTERNAL ERN REVIEW PROCESS

Each ERN will need to decide how to organize the internal review process of the applicant healthcare providers (HCPs) within the necessary timeframes, according to the governance rules of each ERN.

The internal review process should reflect, as far as is possible, other internal assessments undertaken by ERNs. The internal ERN review process, for example, will not involve the overall checking of all aspects included in application forms for the calls for new members to join existing ERNs. But the two processes can reflect the same methodology for how the assessments are undertaken across the members of the ERN. The internal ERN review process would need to focus on the specific criteria and check there is evidence that the existing members meet the Network's specific criteria with regard to the specific disease applied for. In the case of a consortium proposal (for e.g. a new partner will join the original application), a specific endorsement shall be issued by the Member State's competent authority.

The ERN Board of the Network is required by the legislation to ensure that every HCP in the network has its representative on the Board of the Network. In addition, all of the ERNs have patient representatives on the Board of the Networks, many of whom have voting rights, depending on the governance rules of the individual ERN. Therefore, the role of the patient representatives in the assessment of the applicant HCP expertise teams will be determined by the Board of the Networks according to any applicable governance procedures of the network in question, including ensuring that any appropriate conflict of interest rules are followed.



The members of the Board of the Network or any member of the ERN involved in the peer review process must ensure that they maintain the highest professional standards of integrity, transparency and independence. All the participants of the Board of the Network shall communicate and notify immediately if they have any conflict of interest. Peer review of existing members should not be undertaken by other members from the same country. Additionally, the members of the ERN Board of the Network have the obligation to respect European laws on the protection of personal data and confidential data and the applications cannot be shared with the third parties. In addition, they shall respect in particular:

- the rules on the protection of personal privacy and personal data,
- the rules and obligations of professional secrecy laid down by the treaties and their implementing rules.

To that end, it is suggested that each ERN will need to decide how to organise the internal review process of the healthcare provider full members wishing to extend their disease areas within the necessary timeframes, according to the governance rules of each ERN.

The following are all possible options that could be implemented:

- A. The ERN Board of the Network could delegate the decision making responsibility to a smaller decision making group in the ERN (an evaluation team, for example) and they will take the decision on behalf of the ERN. If the decision making is delegated to a smaller group, it is recommended that the evaluation team members are not from the same country as the applicant being assessed, in order to prevent any conflict of interest.
- B. The Coordinator or the evaluation team could disseminate a list of the results of their evaluation (favourable or non-favourable) for all applications for extension of disease coverage with a request for comments within two weeks to the Board of the Network. If no comments are received the list will be accepted as agreed with no changes
- C. All ERN Board of the Network members could vote on every application,

In any case, the ERNs should keep a record of their delegated decision process according to their internal governance rules. ERN experts working in the same field as the applicants are not considered as having a conflict of interest.

### 3. RECOMMENDATIONS FOR ERN ACTION

It is recommended that the ERNs begin immediate preparations internally for their role in assessing their current full member healthcare providers who wish to expand the coverage of their disease areas, specifically by organising any voting that might be needed if delegated decision making is needed which should follow the ERN's governance rules.







## European Reference Networks

[https://ec.europa.eu/health/ern\\_en](https://ec.europa.eu/health/ern_en)

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