

## International Patient Organisation for Primary Immunodeficiencies (IPOPI), UK

1. Respondent Profile	
1.1 Please indicate the type of organisation on behalf of which you are responding to this consultation:	Patient organisation/association
Please indicate level:	European Union umbrella organisation
Please indicate Member States representation:	Pan European
Please indicate for what the administration is responsible:	
1.1.1. Other (please specify):	IPOPI represents the following countries in the EEA area: Austria, Belgium, Bulgaria, Cyprus, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Netherlands, Norway, Poland, Portugal, Romania, Spain, Sweden, United Kingdom
1.2 Please indicate the name of your organisation or centre:	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
1.3 Please indicate the country where your organisation/centre is located/has its headquarters or main representative office in Europe:	UK
1.4 Please indicate the number of EU Member States and EEA countries (Norway, Iceland, Lichtenstein) and accessing country (Croatia) in which your organisation conducts business/is represented:	22
1.5 If need be, can we contact you by e-mail to obtain further information on your submission?	Yes
1.5.1 Please provide an e-mail address where we can contact you:	johan@ipopi.org

1.6 Please provide us with a contact person (incl jobtitle and daytime phone number): Johan Prevot, Executive Director

1.7 Please provide additional contact details if needed:

## 2. Involvement of your organisation in the matter of centres of excellence/reference (COE) and healthcare networks in highly specialised healthcare (HSHC).

2.1 How would you describe your organisation's knowledge of CoE and HSHC? High

2.1.1 Space for further comments: IPOPI has recently organised a PID Forum Meeting at the European Parliament on the Implementation of National Plans for Rare Diseases: Centres of reference and patients' registries.

2.2. What aspects or domains related to the topic of CoE and HSHC would correspond to your organisation's key knowledge? (cross any that applies) Highly specialised healthcare provision  
Priorities, description and characteristics of CoE and HSHC

2.2.1. Space for further comments:

2.3 Is highly specialised healthcare a priority in your organisation's strategies and work plans? Very high

2.3.1 Space for further comments: The implementation of specialised reference centres, where patients can benefit from the collaboration of different specialists in order to treat all chronic manifestations of each patient

2.4. What specific field of healthcare services/specialities are most relevant for your centre/organisation's field of work? Medical speciality

Please specify: Clinical immunology, PID reference centres.

2.5. Has your organisation/centre been directly involved in the design or assessment of professional standards and criteria related with highly specialised healthcare?	Never
2.5.1 Please describe your role in such actions/projects:	
2.6. Has your organisation been involved in projects/activities supported by the Commission in relation with HSHC or professional and technical criteria/standards in highly specialised healthcare?	Yes
2.7. Do you have concrete examples based on your own organisation's experience or could you provide us with references or links to documents related with professional criteria and standards in highly specialised healthcare/CoE or HSHC (e.g. quality criteria, guidelines, consensus documents)?	Yes
2.7.1 Space for further comments:	IPOPI is frequently involved in Rare Diseases consultations. The CEREDIH in France provides gold standard example of what can be achieved through specialized PID reference centres.
2.13. What is the scope of the network?	
2.14. Which kind of network?	
2.14.1 Space for further comments:	
2.15. Would you be interested in applying to the process to be considered Centre of Excellence of the future European Reference Network? (1 = not interested at all, 5 = very interested)	
2.15.1 Space for further comments:	

### 3. Proposed criteria for ERN (scope, general and specific criteria)

#### 3.1 Criteria related with diseases or conditions in order to be considered under the scope of the ERN

3.1.1. Need of highly specialised healthcare	5
3.1.1.1. Complexity of the diagnosis and treatment	5
3.1.1.2. High cost of treatment and resources	5
3.1.1.3. Need of advanced/highly specialised medical equipment or infrastructures	5
3.1.2. Need of particular concentration of expertise and resources	5
3.1.2.1. Rare expertise/need of concentration of cases	5
3.1.2.2. Low prevalence/incidence/number of cases	5
3.1.2.3. Evaluated experiences of Member States	4
3.1.3. Based on high-quality, accessible and cost-effective healthcare	4
3.1.3.1. Evidence of the safety and favourable risk-benefit analysis	4
3.1.3.2. Feasibility and evidence of the value and potential positive outcome (clinical)	5
3.1.4. Do you recommend any additional criteria or option that would effectively address the issue?	Yes
3.1.4.1 Explain your proposal in free text:	Focus should be given to rare diseases for which expertise is scarce. Treatable rare diseases (ie PIDs) should be a priority area. If diagnosed and treated early most PID patients lead normal lives

3.1.5. Would you prioritise or suggest any concrete disease or group of diseases to be addressed by the future ERN according to the above criteria? Yes

3.1.5.1 Explain your proposal in free text:

Primary Immunodeficiencies represents a significant subgroup of rare disorders (around 250) which would benefit from enhanced specialist centres and network in the EU.

### 3.2. General criteria of the centres wishing to join a European Reference Network

3.2.1. Organisation and management 5

3.2.2. Patients empowerment and centered care 5

3.2.3. Patient care, clinical tools and health technology assessment 3

3.2.4. Quality, patient safety and evaluation framework policies 5

3.2.5. Business continuity, contingency planning and response capacity 4

3.2.6. Information systems, technology and e-health tools and applications 5

3.2.7. Overall framework and capacity for research and training 5

3.2.8. Specific commitment of the management/direction of the centre/hospital to ensure a full and active participation in the ERN 5

3.2.9. Do you recommend any additional option that would effectively address the issue? Yes

3.2.9.1. Space for further comments:

HTA assessment for RD such as PIDs should take into account the significant macro-economic and societal benefits which early diagnosis and appropriate treatment will bring to patients

### 3.3. Specific criteria regarding the areas of expertise

3.3.1. Competence, experience and good outcomes and care	5
3.3.2. Specific resources and organisation:	5
3.3.2.1. Human resources	5
3.3.2.2. Team/centre organisation	5
3.3.2.3. Structural conditions	5
3.3.2.4. Specific equipment	5
3.3.2.5. Presence and coordination with other required complementary units or services	5
3.3.3. Patient care pathways, protocols and clinical guidelines in the field of expertise	5
3.3.4. External coordination, care management and follow-up of patients	5
3.3.5. Research, training, health technology assessment in the field of expertise	4
3.3.6. Specific information systems	4
3.3.7. Do you recommend any additional criteria or option that would effectively address the issue?	Yes

3.3.7.1. Space for further comments:

Effective patient registry. ESID registry provides a gold standard example of an EU wide PID registry to improve knowledge about the conditions, facilitate research and information exchange.