

Tree of hope, Germany

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:	National level organisation
Please indicate for what the administration is responsible:	
1.1.1. Other (please specify):	We participate in a 2012 launched informal international NBIA ALLIANCE, the international NBIA patient advocacy, consistent of HoBa, Germany / AISNAF, Italy / AIDNAI, France / ENACH Association, Spain, NBIA Disorders Association, USA / and an UK-group under development, we are partner in TIRCON, an EU-funded research project
1.2 Please indicate the name of your organisation or centre:	Hoffnungsbaum e.V. Verein zur Förderung der Erforschung und Behandlung von NBIA (vormals: Hallervorden-Spatz-Syndrom), in English: Tree of hope, Association dedicated to foster research and treatment in NBIA (formerly: Hallervorden-Spatz-Syndrome), ACRONYM: HoBa
1.3 Please indicate the country where your organisation/centre is located/has its headquarters or main representative office in Europe:	DE
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:	1
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:	hoffnungsbaum@aol.com
1.6 Please provide us with a contact person (incl jobtitle and daytime phone number):	Angelika Klucken chairwoman 0049-2051-68075
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:	We're working on this topic "Centers of Excellence/Expertise" for years, partly on behalf of NBIA, partly on behalf of Rare Disorders in general, as part of the German Rare Diseases umbrella ACHSE e.V
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)	<ul style="list-style-type: none"> Priorities, description and characteristics of CoE and HSHC Ethical analysis Social aspects Professional performance, clinical practice, quality and safety of specialized healthcare Assessment/evaluation/certification of clinical practice and healthcare providers
2.2.1. Space for further comments:	Of course our expertise, knowledge and experiences are limited by our status as lay advocacy. We're no professionals but much committed to this purpose of implementing NBIA and other RD-CE's
2.3 Is highly specialised healthcare a priority in your organisation's strategies and work plans?	High

2.3.1 Space for further comments:	In progressed disease stages many NBIA-patients can't be transported to distant centers. Travel of expertise/info instead of patients would be a requirement as well as telemedicine support
2.4. What specific field of healthcare services/specialities are most relevant for your centre/organisation's field of work?	High tech diagnostic and treatment services (radiology, nuclear medicine, electrophysiology, radiotherapy etc.)
Please specify:	labs, surgical speciality (DBS, orthoped.) as well
2.5. Has your organisation/centre been directly involved in the design or assessment of professional standards and criteria related with highly specialised healthcare?	Occasionally
2.5.1 Please describe your role in such actions/projects:	We participated in the consultations of ACHSE-members for the development of a RD National plan for Germany, HoBa's mission is the implementation of NBIA CE's, our clinical network is TIRCON (FP7)
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?	Non applicable
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)?	No
2.7.1 Space for further comments:	HoBa developed an internal list of criteria / a draft structure for clinical NBIA Ce's network. TIRCON-partner OHSU (USA) currently develops PKAN guidelines, jointly funded by HoBa, AISNAF and NBIA DA

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	4
3.1.1.1. Complexity of the diagnosis and treatment	4
3.1.1.2. High cost of treatment and resources	2
3.1.1.3. Need of advanced/highly specialised medical equipment or infrastructures	4
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)	4
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:	In case of rarity a joint approach of research and care is needed. By medical care you get data for research which might improve the care. Transition from pediatric to adult care should be addressed.
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:	Rare Neurodegenerative Diseases (ND), as they frequently affect children and medical care is a special challenge. They might become "orphan" again, if Health policy focuses only on geriatric ND.

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

3.2.1. Organisation and management	3
3.2.2. Patients empowerment and centered care	5
3.2.3. Patient care, clinical tools and health technology assessment	5
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	4
3.2.7. Overall framework and capacity for research and training	4
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:	willingness, abilities and facilities of the center to contribute to psychosocial and/or palliative care for 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	4
3.3.2.3. Structural conditions	4
3.3.2.4. Specific equipment	4
3.3.2.5. Presence and coordination with other required complementary units or services	3
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	4
3.3.5. Research, training, health technology assessment in the field of expertise	5
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:	numbers of diagnosed/treated/advised patients per year or since a date at a CE, related to the prevalence of the disease