EuroBloodNet
European Reference Network in Rare Hematological Diseases

Criteria defined by the Network for Bone marrow failure (BMF) and rare haematopoietic disorders

The following information defines the specific criteria for our proposal for a European Reference Network (ERN) for Rare Hematological Diseases, EuroBloodNet. Each health care provider willing to be member of our ERN has to fulfil these criteria. These evidence based criteria intend to be realistic while ensuring a high level patient care.

The following information corresponds to the following points in the HCP application form:

1) Point 7_Table in Page 6 Diseases, conditions and highly specialized interventions: it includes not only diseases but also highly specialized interventions, treatments eg bone marrow transplantation

2) Point 11_Table in Page 9 10 of the 16 "Diseases, conditions and highly specialized interventions" defined in point 7 needs to be quantified

3) Point 12_Table in page 11 "Multidisciplinary team"Up to 16 Health care professionals have to be defined: position, training and number of patients/procedures by year (for assure expertise)

4) Point 13_Table in page 12 "Specialised equipment, infrastructure and IT" Up to 16 Specialised equipment, infrastructure and IT used by the HCP to support diagnosis, care and treatment

5) Point 21_Table in page 18 "Clinical outcome data" Up to 22 relevant clinical outcomes related to the Rare or complex disease, condition or highly specialized interventions defined

- Subthematic area of expertise:
  - Rare red blood cell disorders and allied defects
  - X **Bone marrow failure (BMF) and rare haematopoietic disorders**
  - Hemochromatosis and other rare genetic disorders of iron metabolism and heme synthesis
  - Rare bleeding-coagulation disorders
  - Myeloid hemopathy
  - Lymphoid hemopathy

1) Point 7_Table in Page 6 Diseases, conditions and highly specialized interventions: it includes not only diseases but also highly specialized interventions, treatments eg bone marrow transplantation

Subthematic area of expertise	Rare or complex disease, condition or highly specialized interventions	ICD / Orphanet Code
Bone marrow (BM) failure and rare haematopoietic disorders	Congenital dyserythropoietic anemia	ICD10 D64.4/ORPHA85
Bone marrow (BM) failure and rare haematopoietic disorders	Blackfan-Diamond anemia	ICD10 D61.0/ORPHA124
Bone marrow (BM) failure and rare haematopoietic disorders	BMF- Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	D61- ORPHA 164823 / D59-0RPHA447
Bone marrow (BM) failure and rare haematopoietic disorders	BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others	D61-0RPHA84/0RPHA 1775082 / ORPHA 274222 I/061-0RPHA 3319
Bone marrow (BM) failure and rare haematopoietic disorders	Immunosuppressive treatments	
Bone marrow (BM) failure and rare haematopoietic disorders	Bone-marrow transplant for Bone marrow failures and rejection management	
Bone marrow (BM) failure and rare haematopoietic disorders	Diagnosis	

2) Point 11_Table in Page 9 10 of the 16 "Diseases, conditions and highly specialized interventions" defined in point 7 needs to be quantified

Specific diseases, conditions and highly specialized interventions	Measure	Evidence
Congenital dyserythropoietic anemia	Number of patients per year	5
Blackfan-Diamond anemia	Number of patients per year	3
BMF- Acquired (Aplastic Anaemia,Paroxysmal Nocturnal Hemoglobinuria)	Number of patients per year	25
BMF- Acquired (Aplastic Anaemia,Paroxysmal Nocturnal Hemoglobinuria)	Number of new patient per year	5
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others	Number of patients per year	10
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others	Number of new patient per year	2

3) Point 12_Table in page 11 "Multidisciplinary team"

Healthcare professional	Training and qualifications	Nº procedures/patients per year
Hematologist	Expertise in BM failures > 3 years	50
Pediatrician	Expertise in BM failures > 3 years	50
Pediatrician/Haematologist	Expertise in Bone marrow transplant in BM failures > 3 years	2
Nurse	Expertise in BM failures > 3 years	30
Laboratory specialist (expertise in cytogenetics, genomics and morphology)	Expertise in BM failures diagnosis > 3 years	20

4) Point 13_Table in page 12 "Specialised equipment, infrastructure and IT"

Specific diseases, conditions and highly specialized interventions	Specific equipment, infrastructure and information technology (IT)
Bone-marrow transplant for Bone marrow failures and rejection management	Bone Marrow Transplant Unit
Diagnosis	Laboratory tests including morphology , cytogenetics and molecular analysis
BMF- Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	Hematology Intensive care unit Daycare facility and outpatient clinic Home care full time hospitalization / Emergency room
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others	Hematology Intensive care unit Daycare facility and outpatient clinic Home care full time hospitalization / Emergency room

5) Point 21_Table in page 18 "Clinical outcome data"

Rare or complex disease, condition or highly specialized interventions	Clinical outcome	Evidence (quantifier)
BMF- Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	Aplastic Anemia (pediatric and adult) - allograft - acquired - Overall Survival	91 %
BMF- Acquired (Aplastic Anaemia and Paroxysmal Nocturnal Hemoglobinuria)	Aplastic Anemia (pediatric and adult) - IST - acquired - Overall Survival	90 %
BMF Inherited (Fanconi anemia, Dyskeratosis congenital, GATA2 syndrome,Congenital amegakaryocytic thrombocytopenia and others	Aplastic Anemia (pediatric and adult) - allograft - inherited - Overall Survival	67%