EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

4 May 2011
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Human Medicines Development and Evaluation

European network of paediatric research (EnprEMA)

Recognition criteria for self assessment

The European Medicines Agency is tasked with developing a European paediatric network of existing national and European networks, investigators and centers with specific expertise in the performance of studies in the paediatric population.

Following a test pilot phase, public consultation and the outcome of the second workshop with participants of 28 networks and/or clinical trial centres in March 2010, recognition criteria have been finalised which will have to be fulfilled by existing networks to become a member of the European paediatric network. All networks wishing to become a member of EnprEMA are invited to perform self-assessment and to send the filled-in document to the European Medicines Agency.

The document should be sent to Merja.Heikkurinen@ema.europa.eu

END OF SELF-ASSESSMENT PERIOD	31 July 2010

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EnprEMA

European network of paediatric research at the European Medicines Agency

Recognition criteria for self-assessment

The European Paediatric Regulation (EC) No 1901/2006, as amended, calls for the fostering of high-quality ethical research on medicinal products for use in children. This should be achieved through efficient inter-network and stakeholder collaboration. To meet this objective, a European paediatric research network is to be formed of national and European networks, investigators and centres with specific expertise in performing drug trials in the paediatric population. General information can be found at:

http://www.emea.europa.eu/htms/human/paediatrics/network.htm

Minimum criteria that have to be fulfilled to be recognised as a member of the EnprEMA

This document defines 6 criteria with several subcategories (items) for self-assessment. The criteria and their items have been set up in a public process. Minimum criteria were defined that networks should fulfil to be recognised as a member of the EnprEMA. The defined minimum criteria are flagged with a superscript "**M**".

Irrespective of whether or not only minimum criteria / items are fulfilled, the full list of the criteria and items as well as the network identification should be completed to the extent possible.

Use of the document and application of the recognition criteria

The criteria should be reported for the highest level that the network currently attains. Networks should report on the status of the network, not on individual investigators or sites. For the purpose of this document, the highest level is called the reporting party.

The document should be filled in by the reporting party (once only per network), taking into account the guidance text provided for the various items within the respective criterion. For transparency in general and to permit public scrutiny of the self-assessment, the completed document should be made public by the reporting party, for example, on their website.

For the same purpose, the reporting party should also make publicly accessible the actual data on which the statements are based. For example, if numbers of paediatric trials are provided, references to clinical trial registration numbers could be made publicly accessible.

The self-assessment should be updated annually.

This document should be sent to the European Medicines Agency; it will be published on the EMA webpage.

Criteria for the recognition of an investigator, site* or network as a member of the EnprEMA*

* only when the investigator or the site is not part of a network

Identification [M]

Name	EBMT PD WP – European Group for Blood and Marrow Transplantation; PD WP : Paediatric Diseases Working Party EBMT registered Office: PO Boch 3151, 6202 ND Maastricht, The Netherlands	Include legal address, define acronyms

Name	EBMT PD WP – European Group for Blood and Marrow Transplantation; PD WP : Paediatric Diseases Working Party EBMT registered Office: PO Boch 3151, 6202 ND Maastricht, The Netherlands	Include legal address, define acronyms
Type	The European Group for Blood and Marrow Transplantation (EBMT) is a non-profit organisation based in Maastricht, The Netherlands, that was established in 1974 in order to allow scientists and physicians involved in clinical bone marrow transplantation to share their experience and develop co-operative studies. The EBMT aims to promote all aspects associated with the transplantation of haematopoietic stem cells from all donor sources and donor types including basic and clinical research, education, standardisation, quality control, and accreditation for transplant procedures. The Paediatric Diseases Working Party was established in 1995 to support research and education to improve the availability, safety, and efficacy of hematopoietic stem cell transplantation and other cellular therapeutics for children and adolescents. Further aims: • Increase Collaboration – With other EBMT WPs – With other Pediatric Organisations, e.g. chemotherapy front line study groups • Promote prospective clinical trials – Academic • e.g. prevention and treatment of acute and chronic GVHD, prevention of late effects – Pharmaceutical: • Registration of new drugs, e.g. PIP for Treo • Registration of off patent drugs, e.g. Etoposide, ATG („PUMA") • Help new members: training, ~~fellowships for physicians and nurses~~ • Establish paediatric standards within the Accreditation Process through JACIE to guarantee and maintain a high quality of patient	Indicate type of reporting party, e.g. national or speciality network. May include short mission statement

Name	EBMT PD WP – European Group for Blood and Marrow Transplantation; PD WP : Paediatric Diseases Working Party EBMT registered Office: PO Boch 3151, 6202 ND Maastricht, The Netherlands	Include legal address, define acronyms
Street	EBMT Secretariat : C/ Rosselló 140, 1º, 1ª	
Postal code	08036	
Town	Barcelona	
Country	Spain	
Telephone 1	+ 34-93 453 8711	
Telephone 2		
Mobile phone		
Fax	: +34-93 4519583	
Web site	http://www.ebmt.org	If available (see criterion 4)
Email for general enquiries	1)info@ebmt.org 2) pdwp@ccri.at	If available (see criterion 4)
Representative (main) contact	---	Include first and second name, email, telephone, address, as far as available
First name	Christina	
Second name	Peters	
Telephone	+43 1 40 170 3106	
Mobile phone	+43 676 934 28 41	
Email	christina.peters@stanna.at	
Further contact(s)	---	Include first and second name, email, telephone, address, as far as available
First name	Adriana	
Second name	Balduzzi	
Telephone	Fax: +39-039-233.3523	
Mobile phone	Phone: +39-039-233.2442	
Email	a.balduzzi@hsgerardo.org	
The data in this document are 'current' as of	05 12 2012	Provide the date when the criteria were last updated
State how this document can be accessed by the public	http://www.ebmt.org/Contents/About-EBMT/Who-We-Are/Workingparties/Pages/Working-parties.aspx#PaediatricDiseaes	This should be a link to a webpage, but other means and formats to make public are possible

Description [M]

Year of foundation	1974, PD WP: 1995	Of the network, or of the investigator's or site's specific paediatric research activities
Paediatric age ranges of study participants covered by the network		
Preterm and / or term newborn	☒ Yes ☐ No	Newborn: from birth to less than 28 days of age
Infants from 1 month to less 24 months of age	☒ Yes ☐ No	
Children from 2 years to less than 12 years of age	☒ Yes ☐ No	
Adolescents from 12 years to less than 18 years	☒ Yes ☐ No	
Specialities / Conditions covered	Haematology: acute and chronic leukemia, bone marrow failure syndromes, red cell disorders, Oncology: solid tumors Immunology: hemophagocytic diseases, autoimmune disorders, Metabolic Disorders Osteopetrosis	ENPREMA will cover a range of different networks, from single speciality trials groups to those covering all paediatrics. If not all areas within one speciality are covered, specify conditions
Multispeciality? Specify	Haematopoietic stem cell transplantation including all complications, e.g. infections, acute and late effects including organ dysfunction secondary malignancies, growth deficiency, hormonal disorders, fertility, therefore all paediatric subspecialities are included: haematology, oncology, anaestiology, nephrology, infectious diseases, cardiology, surgery, pulmonology, immunology, gynecology, neurology, endocrinology, pharmacology	For example, oncology or infectious diseases
Speciality or disease specific? Specify		For example, cardiology only
Conditions covered? Specify	Acquired conditions: bone marrow failure syndromes, haemtological malignancies, haemophagocytic disorders, histiocytic disoders, solid tumours, and others. Congenital disorders: Immunodeficiencies, metabolic diseases, red cell disorders, e.g. haemoglobinopathies and others.	E.g. hypertension (within cardiology) or asthma (within respiratory diseases)

Year of foundation	1974, PD WP: 1995	Of the network, or of the investigator's or site's specific paediatric research activities
Procedure / intervention specific? Specify	haematopoietic stem cell transplantation: allogeneic and autologous; stem cell harvest procedures, donor treatment (including paediatric donors); intensive care measures (e.g. non invasive and mechanical ventilation); prophylaxis and treatment of early, internediate and late effects of pharmaceutical interventions and immunological reactions	For example, surgery, organ or stem cell transplantation

Year of foundation	1974, PD WP: 1995	Of the network, or of the investigator's or site's specific paediatric research activities
Number of collaborating countries	31 European, 14 Non-European Countries List all collaborating countries: Austria Belarus Belgium Bulgaria Croatia Chechia Denkmark Estonia Finland France Germany Greece Hungary Ireland Italy Latvia Lithunia Netherlands Norway Poland Portugal Romenia Russia Slowakia Slovenia Spain Sweden Switzerland Turkey Ukraine United Kingdom Israel Australia Iran Saudi Arabia South Africa New Zealand Argentina Brazil China Algeria Lebanon Jordan Canada Egypt Tunisia	State the number of collaborating countries. Indicate "1" if national; Indicate if Europe, outside of Europe, other..... (describe)

Year of foundation	1974, PD WP: 1995	Of the network, or of the investigator's or site's specific paediatric research activities
Number of collaborating centres	483: 449 European, 34 Non-European List all collaborating centres: find a list of all paediatric centers; the whole EBMT membership list is posted on the EBMT website. In principle, all registered EBMT centers are collaborating centres as many activities are not restricted to paediatric issues.	State the number of collaborating centres and provide a list of all collaborating centres (attachment or link possible)
Type of activity/studies		
Clinical studies	☒ Yes ☐ No	
Experimental research	☒ Yes ☐ No	
Other activity	1) .) Observational audits: non interventional studies, e.g. on outcome after autoHSCT for Wilms-Tumour .) "Epidemiological studies": e.g. which patients received Anti-Thymocyte Globuline before HSCT .) Outcome studies, e.g. "Late effects after Reduced Intensity Conditioning before Allogeneic HSCT in Children" .) Surveys: e.g.: What is the current practice for Graft-versus Host-Disease Prophylaxis and therapy in Children?, "Infection Prophylaxis for Patients with Chronic Graft versus Host Disease" .) Evidence Based Recommendations: "Vaccination after allo HSCT in Children" .) Consensus Based Reports: e.g. "Classification of acute Graft versus Host Disease in Children" 2) JACIE: Joint Accreditation Committee IBMTR/EBMT: Accreditation Program for Stem Cell Transplantation Centers incuding all issues of a Quality Management Program. 3) Education & Training: Specific meetings, training courses for all topics of paediatric stem cell transplantation are performed by the PD WP to guarantee updated knowledge and help new members with establishing high level care for children and adolescents.	Describe type of activities other than clinical and/or non-clinical studies

Evidence for each criterion

How to provide evidence

1. The evidence for this self-assessment document should be based only on the activity of the network during in the last 5 years.
2. Evidence used in this document should have a reference (e.g., publication, annual or periodic report or internal network document).
3. The self-assessment document is to cover a range of different network types. It is recognised that some networks may not be able to accurately respond to every item. In such circumstances, state why it is not possible to respond.
4. The network is referred to as the "reporting party".

Criterion 1: Research experience and ability

Do not include planned trials, but only ongoing and completed trials.

addendum!

1.1 Number of completed trials [M] Number of ongoing trials [M]	> completed 20, e.g. 1.Prospective Study of the Incidence and Outcome of Veno-occlusive Disease (VOD) with the Prophylactic Use of Defibrotide (DF, Gentium, Italy) in Pediatric Stem Cell Transplantation S Corbacioglu 2.Treosulfan-based preparative regimen for advanced haematological malignancies in children demonstrating high risk of conventional regimen related toxicity Jacek Wachowiak PDWP Results and factors influencing outcome after fully haploidentical hematopoietic stem cell transplantat in children with very-high risk acute lymphoblastic leukemia - impact of center size: an analysis on behalf of the Acute Leukemia and Pediatric Diseses Working Parties of the european Blood and Marrow Transplant group T Klingebiel Blood 20040760 PDWP January 2009 Granulocyte transfusions in neutropenic patients: beneficial effects proven? C Peters Vox sanguinis 19207168 PDWP November 2009 Allogeneic transplantation for children and adolescents with Hodgkin lymphoma. Lymphoma and Paediatric Diseases EBMT WP N Schmitz Blood 19965711 PDWP September 2009 Allogeneic hematopoietic stem cell transplantation in children and adolescents with recurrent and refractory Hodgkin lymphoma: an analysis of the European Group for Blood and Marrow Transplantation. A Claviez Blood 19498021 PDWP September 2009 Cardiac and pulmonary late effects do not negatively influence performance status and non-relapse mortality of children surviving five yr after autologous hematopoietic cell transplantation: report from the EBMT Paediatric Diseases and Late Effects	Any interventional clinical trial, whether non-commercial, investigator-initiated, industry-sponsored or commercial, in which the reporting party actively took part. Minimum requirement ([M]): one ongoing or one completed trial.

1.2 Total number of participants actually recruited each year Proportion of eligible participants actually recruited each year Describe way of screening and participant recruitment	1999-2011: 34.211 (approx. 3.000 patients per year) nearly all patients are eligible at least for non commercial studies A letter of interest and a study synopsis is mailed to the PD WP members; studies will be posted on the new Website (work in progress); central data management in the statistical office in paris; announcement of new studies during the WP meetings and training courses	Relevant to speciality specific networks. State total recruitment capacity for any interventional clinical trial, whether non-commercial, investigator-initiated, industry-sponsored or commercial, in which the reporting party actively took part. Which strategies or pathways are used to screen and recruit participants?
1.3 Total number of collaborating centres	studies are open for all EBMT centers - that depends on the topic; number of centers who perform HSCT only in children/adolescents: 165	For completed and ongoing (open) paediatric trials. Do not include sites in set-up.
Academic (investigator) initiated studies	---	Studies conducted independently from pharmaceutical companies (no sponsorship and no funding). There is a separate category (below) for industry-funded studies.
1.4 Number of ongoing and completed clinical trials	Absolute number: > 5, during the last years, e.g. 1., 2., 3., 4., from 1.1.; Suttorp M, Claviez A, Bader P, Peters C, Gadner H, et al: Allogeneic stem cell transplantation for pediatric and adolescent patients with CML: results from the prospective trial CML-paed I. Klin Padiatr. 2009 Nov-Dec;221(6):351-7. Epub 2009 Nov 4. Proportion of all studies: about 20%	Paediatric interventional trials of any phase of the pharmaceutical development (phase I to IV, including therapy optimising trials if requiring authorisation by regulatory authority) (for other Paediatric trials unrelated to drug development see below)
1.5 Number of paediatric	nearly all paediatric specialities are included in the different trials,	Count specialities, without repetition, across all

specialities covered by paediatric trials	e.g. haematology, cardiology, neonatology, endocrinology, nephrology, infection, etc: depends on study topic	ongoing or completed paediatric trials
1.6 Number of paediatric conditions covered by paediatric trials	Haematology: acute and chronic leukemia, bone marrow failure syndromes, red cell disorders, Oncology: solid tumors Immunology: hemophagocytic diseases, autoimmune disorders, Metabolic Disorders Osteopetrosis, other rare conditions with indication for HSCT (e.g. Pearson syndrome)	If not all areas within one speciality covered count conditions, without repetition, across all ongoing or completed paediatric trials
1.7 Number of other ongoing research studies / programs	> 10; e.g. addendum .) Survey on 2nd CR ALL HSCT Registry Study G. Dini .)Salvage high-dose chemotherapy for children with extragonadal germ cell tunors: long term results from the registry of the european group for blood marrow transplantation (in co-operation with ST WP). De Giorgi .)European Survey of current practise in growth ormone treatment after HSCT (in co-operation with LE WP). A Cohen, A Rovelli, A.lawitschka .) Family haploidentical allogeneic SCT compared with cord blood T for AL (in co-operation with AL WP). R.Hough	For example, epidemiological studies, outcome studies, translational research in which the reporting party is participating Include cohort studies but not audits. Research is defined as a project with a specific research question in which the participant/family provides formal consent.
1.8 Indicate the proportion of public funding	Proportion of academic initiated studies: about 80% Proportion of budget: Cannot provide you precise information as some studies are sponsored by universities, national programs, e.g. Deutsche Krebshilfe, FP6-programs (Allo-Stem)	Indicate the proportion of the budget handled for completed and ongoing paediatric trials that is derived from public funding sources such as governmental programs, competitive public grants, university contributions
1.9 Number of registered study participants (all studies)	it is impossible to give precice numbers - from 2001 - 2010 25268 paediatric patients were registered in the EBMT database; cannot comment on the total number of registered study patients	
Industry-sponsored trials	1 (Meta analysis on Treosulfan in children and adolescents before HSCT)	

1.10 Number of ongoing and completed trials	about 5 during the last 5 years, e.g. i.v. busulfan, defibrotide, rituximab, G-CSF for SAA, ATG for SAA, etc.	Paediatric interventional trials of any phase of the pharmaceutical development (phase I to IV, including therapy optimising trials if requiring authorisation)
1.11 Number of paediatric specialities covered by paediatric trials	see above	Count specialities, without repetition, across all ongoing or completed paediatric trials
1.12 Number of paediatric conditions covered by paediatric trials	Haematology: acute and chronic leukemia, bone marrow failure syndromes, red cell disorders, Oncology: solid tumors Immunology: hemophagocytic diseases, autoimmune disorders, Metabolic Disorders Osteopetrosis	If not all areas within one speciality covered count conditions, without repetition, across all ongoing or completed paediatric trials
1.13 Number of registered study participants (all studies)	i.v. Bu: > 60; Defibrotide > 350, G-CSF > 100; ATG: > 100;	

Criterion 2: Network organisation and processes

2.1 Existence of an identified contact person for external enquiries [M]	☒ Yes ☐ No Comments: the chair of the PD WP is elected by the EBMT members during the EBMT annual meeting for 3 years. Current Chair person: Christina Peters, elected 2008 and is nominated for re-election for another 3 years (until 2014).	Enquiries from patients, parents, organisations, researchers, pharma-ceutical companies or regulatory authorities are co-ordinated or answered by a nominated contact person. Provide contact details in section "Identification" above.
2.2 Existence of an internal steering committee [M]	☒ Yes ☐ No Comments: The PD WP board covers all subspecialities of HSCT: e.g. leukemia, Graft versus host diseases, infectious complications, stem cell sources, solid tumors, graft processiing. The PD WP steering committee consists of following members: see attachment	Minimum requirement ([M]): either an internal steering committee (2.2) or an external advisory / steering committee (2.3).
2.3 Existence of an external advisory / steering committee directing the reporting party [M]	☒ Yes ☐ No Comments: EBMT-Board (President, 10 other WP members, treasurer, executive Director, statistic director etc	Minimum requirement ([M]): either an internal steering committee (2.2) or an external advisory / steering committee (2.3).
2.4 Existence of a website	☒ Yes ☐ No Comments: http://www.ebmt.org/Contents/About-EBMT/Who-We-Are/Workingparties/Pages/Working-parties.aspx#PaediatricDieseaes	If available, mention in "identification" above
2.5 Existence of newsletter	☒ Yes ☐ No Comments: EBMT-newsletter - see attachment PD WP newsletter - see attachment	Newsletter of any format (electronic, surface mail), distributed actively to selected recipients.
2.6 Existence of an internal database(s) for disease, condition, treatment and / or outcome [M] If yes, please describe	☒ Yes ☐ No Comments / description: > 53000 patients are registered in the EBMT registry. Every EBMT member center must register all patients undergoing any kind of HSCT	For example, data base or disease registry to facilitate planning or conducting future trials (may or may not contain individual patient data)

2.1 Existence of an identified contact person for external enquiries [M]	☒ Yes ☐ No Comments: the chair of the PD WP is elected by the EBMT members during the EBMT annual meeting for 3 years. Current Chair person: Christina Peters, elected 2008 and is nominated for re-election for another 3 years (until 2014).	Enquiries from patients, parents, organisations, researchers, pharmaceutical companies or regulatory authorities are co-ordinated or answered by a nominated contact person. Provide contact details in section "Identification" above.
2.7 Provisions to ascertain data protection and data security [M]	☒ Yes ☐ No Comments: The registration of the patient details are handle via ProMISe (Project Manager Internet Server) is the central data management system used by the EBMT. Users are able to enter and retrieve data directly over a secure Internet connection. Passwords to access ProMISe: MED-AB Project are issued by the Central Registry Office in London. Personal passwords are secure and each user should have their own individual username and password which are non transferable. (NB: ProMISe passwords are separate from team passwords used to access protected areas of the web site). There are two levels of access: • Data Entry: access to all functions: Data Entry; Statistical Reports; Patient Reports and Downloading Centre Data. Personal, non-transferable usernames and passwords will be assigned to those entering data on behalf of their team. All Data Entry passwords must be authorised by the Principal Investigator of that particular member centre. • Data Download: as above but with Data Entry disabled. Individuals should apply for either data entry or data download;	Are provisions in place to ascertain patients' /study participants' data protection and data safety within network

2.1 Existence of an identified contact person for external enquiries [M]	☒ Yes ☐ No Comments: the chair of the PD WP is elected by the EBMT members during the EBMT annual meeting for 3 years. Current Chair person: Christina Peters, elected 2008 and is nominated for re-election for another 3 years (until 2014).	Enquiries from patients, parents, organisations, researchers, pharma-ceutical companies or regulatory authorities are co-ordinated or answered by a nominated contact person. Provide contact details in section "Identification" above.
2.8 Procedure(s) to access the database by third parties	☒ Yes ☐ No Comments: .) a written contract is necessary if data a shared with third parties. .) direct access to the data base is impossible outside the data base management. .) Request for statistical analysis is handeled by the EBMT statisticians and data managers.	Are provisions in place that data can be shared for planning, conducting or analysing a trial(s)?
2.9 Access to external databases /registries	☒ Yes ☐ No Comments: Cooperation with other national and international transplant organisations, e.g. .) cooperation with American data base for HSCT = IBMTR. .)Donor data base .)International unrelated donor registries .) EUROCORD	For example, national databases that are not publicly accessible but to which the reporting party has open or privileged access; database(s) immediately relevant to area and / or scope
2.10 Standardised process to access an external database(s)	☒ Yes ☐ No Comments: by written contracts	Is a standardised process in place to access external/ national databases?

Criterion 3: Scientific competencies and capacity to provide expert advice

3.1 Number of peer-reviewed publications in the last 5 years Provide exact reference(s) Describe the network's contribution to publication(s)	addendum >25, e.g.: Herr AL, Kabbara N, Bonfim CM, Teira P, Locatelli F, Tiedemann K, Lankester A, Jouet JP, Messina C, Bertrand Y, Díaz de Heredia C, Peters C, Chaves W, Nabhan SK, Ionescu I, Gluckman E, Rocha V. Long-term follow-up and factors influencing outcomes after related HLA-identical cord blood transplantation for atients with malignancies: an analysis on behalf of Eurocord-EBMT. Blood. 2010 Sep 16;116(11):1849-56. Epub 2010 Jun 10. PubMed PMID: 20538797. Peters C, Cornish JM, Parikh SH, Kurtzberg J. Stem cell source and outcome after hematopoietic stem cell transplantation (HSCT) in children and adolescents with acute leukemia. Pediatr Clin North Am. 2010 Feb;57(1):27-46. Review. PubMed PMID: 20307710. lingebiel T, Cornish J, Labopin M, Locatelli F, Darbyshire P, Handgretinger R, Balduzzi A, Owoc-Lempach J, Fagioli F, Or R, Peters C, Aversa F, Polge E, Dini G, Rocha V; Pediatric Diseases and Acute Leukemia Working Parties of the European Group for Blood and Marrow Transplantation (EBMT). Results and factors influencing outcome after fully haploidentical hematopoietic stem cell transplantation in children with very high-risk acute lymphoblastic leukemia: impact of center size: an analysis on behalf of the Acute Leukemia and Pediatric Disease Working Parties of the European Blood and Marrow Transplant group. Blood. 2010 Apr 29;115(17):3437-46. Epub 2009 Dec 29. PubMed PMID: 20040760. Ljungman P, Bregni M, Brune M, Cornelissen J, de Witte T, Dini G, Einsele H, Gaspar HB, Gratwohl A, Passweg J, Peters C, Rocha V, Saccardi R,	The publications should indicate that they are related to and reference the reporting party.

3.2 Number of competitive grants obtained in the last 5 years	The PD WP did not apply for competitive grants by its own but many active members did: eg. FP6 programs, other national grants, e.g. for the "GVHD evaluation program"	Grants obtained by reporting party (exclusively or not).
3.3 Access to expert groups [M]	☒ Yes ☐ No Comments: the PDWP chair person and steering committee are chairs and members of numerous expert groups, e.g. Ruth Ladenstein is chair of SIOPE and ENCA, Thomas Klingebiel is head of GPOH, Peters Bader is WG chair of IBFM, etc.	Indicate if the reporting party has specific access to established expert groups, such as learned societies
3.4 Capacity to answer external scientific questions [M]	☒ Yes ☐ No Comments: All PD WP steering committee members are dedicated scientific experts for paediatric stem cell transplantation and experienced in conducting clinical trials including advice for trial design and conduction	Indicate if coordinated capacity (staff, process) is available to answer external scientific questions in relation to clinical trials during daily business.
Standardized procedures for assessment of:	---	
3.5 Site feasibility	☒ Yes ☐ No Comments: data management office in Paris; one data manager for the PD WP	This concerns the suitability of a site for conducting a given trial
3.6 Participant recruitment	☒ Yes ☐ No Comments: data management office in Paris	This concerns provisions to regularly monitor recruitment progress for a trial.
3.7 Budget calculation for studies	☒ Yes ☐ No Comments: if not done by the principle investigator at the EBMT Prospective Trial Offices: London, Paris, Leiden	This concerns, for example, quotes and prospective financial planning for a trial.

Criterion 4: Quality management

4.1 Documented adherence to Good Clinical Practice (GCP) guideline [M]	☒ Yes ☐ No Comments: the EBMT has a specific accreditation process for conducting haematopoietic stem cell transplantation: all details are available on the EBMT Website - what is EBMT: operational management JACIE (joint accreditation committee ISH- EBMT) Quality Management and GCP is an essential part: all relevant information: EBMT Website: transplant guidelines: JACIE	Declare whether studies conducted comply with the EU Directive 2001/20/EC on Clinical Trials.
4.2 Documented adherence to the ethical considerations for clinical trials in children [M]	☒ Yes ☐ No Comments: All clinical trials adhere to the ethical considerations for clinicla trials - we will specifically post it at our new website as soon it is available	Indicate if documented data / information are publicly available on implementation of / provisions for special ethical requirements for the paediatric trial(s) according to the document "Ethical considerations for clinical trials on medicinal products conducted with the paediatric population".
4.3 Documented adherence to ethical considerations	☒ Yes ☐ No Comments: As all studies follow the GCP guidelines all clinical trials get approval by ethics commitees with paediatric expertise	Declare whether reporting party requests approval by an independent ethics committee with paediatric expertise for all studies conducted.
4.4 Availability of Standard Operation Procedures (SOP)	☒ Yes ☐ No If yes, provide reference to available SOPs JACIE accreditation handbook - see website of EBMT	Indicate existence of SOP e.g. for study management, adverse events reporting etc.
4.5 Capacity to monitor studies (academic trials, industry sponsored trials) [M]	☒ Yes ☐ No Comments: if sufficient financial support is available the PD WP or the EBMT prospective clinical trial office implents the monitoring, otherwise it is obligatory according to GCP by the study sponsor	Indicate if the reporting party implements the monitoring of paediatric trials according to ICH 6 Good Clinical Practice Guideline.

4.1 Documented adherence to Good Clinical Practice (GCP) guideline [M]	☒ Yes ☐ No Comments: the EBMT has a specific accreditation process for conducting haematopoietic stem cell transplantation: all details are available on the EBMT Website - what is EBMT: operational management JACIE (joint accreditation committee ISH- EBMT) Quality Management and GCP is an essential part: all relevant information: EBMT Website: transplant guidelines: JACIE	Declare whether studies conducted comply with the EU Directive 2001/20/EC on Clinical Trials.
4.6 Capacity to monitor performance of collaborating centres	☒ Yes ☐ No Comments: Depends on financial funding of the study by the sponsor or principal investigator	Indicate if the reporting party implements the monitoring of performance of collaborating centres.
4.7 Quality control and quality assurance, traceability and data safety [M]	☒ Yes ☐ No Comments: centrally organized by the EBMT registry, head quarter London	Indicate if this is implemented in the reporting party's remit.

Criterion 5: Training and educational capacity to build competences

5.1 Evidence of collaboration with regulatory authorities [M]	☒ Yes ☐ No Comments: e.g. with the Austrian Ministry of health to elaborate Guidelines for Tissue Transplantation & Storage	Indicate awareness of regulatory requirements for developing medicines; for example, implementation of guidelines from regulatory authorities.
5.2 Capacity to provide competent consultation to regulatory authorities	☒ Yes ☐ No Comments: e.g. the chair person is member of the Transplantation Board of the Austrian Ministry of Health; member of the Paediatric Advisory Board of the Austrian Ministry of Health; member of the "Austrian Health Advisory Board"	Indicate the capacity of the reporting party to provide expert advice to regulatory authorities. For example, nominations into standing scientific committees to regulatory authorities, registration(s) as authorities' external expert(s).
5.3 Formal meetings for clinical trials If yes, provide number	☒ Yes ☐ No Comments: Annual EBMT Meeting once per year, Working Party meeting: at least 1/year, Study Committee meetings for ongoing trials according to study size.	For example, investigator meetings, trainings specific to a given ongoing or planned trial.
5.4 Training courses given over the last 2 years [M] If yes, provide number	☒ Yes ☐ No Comments: 4 PD WP meetings (2 of them were training courses for young physicians: Helsinki 2010, Venice 2010 (in cooperation with the EBMT inborn Error WP), Genoa 2011, Prague 2012, Bukarest 2013	For example, training specific to a trial or in general for trial(s), with external participants or from the reporting party. Minimum requirement (M): training courses either given (5.4) or received (5.5).
5.5 Training courses received over the last 2 years [M] If yes, provide number	☒ Yes ☐ No Comments: The PD WP steering committee members take part in numerous training courses, either as faculty or as participants, e.g. JACIE-inspector training courses, CIMBT-meetings, ASH, IBMTR-annual meeting etc.	For example, training specific to a trial or in general for trial(s), with external participants or from the reporting party. Minimum requirement (M): training courses either given (5.4) or received (5.5).

5.1 Evidence of collaboration with regulatory authorities M	☒ Yes ☐ No Comments: e.g. with the Austrian Ministry of health to elaborate Guidelines for Tissue Transplantation & Storage	Indicate awareness of regulatory requirements for developing medicines; for example, implementation of guidelines from regulatory authorities.
5.6 Promotion of participation in clinical trials in countries with limited resources Provide list of countries	☒ Yes ☐ No Comments: The EBMT PD WP is providing specific study support (training, advice, fellowship) for emerging centers and countries; coordinator: Jacek Wachowiak (PL). Special fellow programs for doctors and nurses from Eastern countries (e.g. Tatsikistan, Georgia, Armenia, Kazakhstan, Ukraine) at St. Anna Children's Hospital, Vienna and other EBMT-PD WP member centres	Indicate if support for such trials is provided by the reporting party.

Criterion 6: Public involvement [M]

Minimum requirement (M): involvement in at least one of the below items.

6.1 Involvement of patients, parents or their organisations in the protocol design	☒ Yes ☐ No Comments: e.g. Austrian Society of Paediatrics	Indicate if public stakeholders are /have been involved
6.2 Involvement of patients, parents or their organisations in creating the protocol information package	☒ Yes ☐ No Comments: e.g. The Austrian Children Cancer Foundation (Parents organisation). As the EBMT PD WP conducts pan-European trials with different principle investigators, national public stakeholders are involved in the specific study designs.	Indicate if public stakeholders are /have been involved
6.3 Involvement of patients, parents or their organisations in the prioritisation of needs for clinical trials in children	☐ Yes ☒ No Comments: During the Annual EBMT meeting a specific "Patient and Family Day" covers all issues for paediatric needs in the treatment course of haematopoietic stem cell transplantation.	Indicate if public stakeholders are /have been involved