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Proposing the study idea and submitting and selecting the study protocol for EBMT Working Party studies

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Background

EBMT has defined the following purpose for its Science mission:

Transform science on blood and marrow transplantation and advanced cellular therapy through collaborative platforms and translational evidence & innovation, to become the EU reference in the field with global impact.

One of its objectives is to implement a standardised selection process for study protocols that includes study plan(ning) & feasibility.

Currently, EBMT Working Party (WP) Study Coordinators, Data Managers and Statisticians are often required to work on many studies simultaneously and have to perform many activities in parallel. This means less time is available for each study, and, as a result, it often takes a long time for a study to be published. This delay between the submission of a study protocol and the publication of a study may create a significant risk that both the scientific community and the Principal Investigator (PI) themselves will lose interest in the study's results.

Implementing a standardised study protocol submission and selection process will lead to improved harmonization across the different EBMT WPs and make this process more transparent and comprehensible for EBMT members. Moreover, having well-defined criteria, also taking into account the number of hours available within a WP versus the estimated workload of the proposed study, will eventually lead to fewer studies being conducted in parallel, and therefore more resources being available for each study. This is expected to expedite the time to publication, which will increase a study's relevance and ideally contribute to EBMT being seen as the EU reference in the field.

Therefore, to achieve its Scientific purpose, EBMT proposes a standardised policy for protocol submission and selection across all EBMT WP

I. Proposing the study idea

There are several time windows each year during which new studies can be proposed. Ideally, this is during the WP Business Meetings. However, it is recognised that each WP has a variable number of business meetings, and for some WPs more selection episodes might be necessary to avoid clustering of data requests to centres. Therefore, each WP can independently decide how many defined selection moments per year will be implemented. This needs to be agreed upon and recorded in the business meeting minutes and, as much as is feasible, formal submissions made at these pre-agreed intervals only. This will facilitate prioritisation, comparison of relevance and correct allocation of planned workload. In the case of a highly important or very urgent study topic (e.g. as per the studies on COVID-19 at the start of the pandemic), an exception can be made and the study protocol can be submitted outside of the fixed selection windows.

Please note that only researchers affiliated with Centre Members who are full members of the EBMT can initiate studies. In case an Individual Member from a non-member centre wishes to conduct a study within EBMT, approval will need to be obtained from the WP chair and the Executive Committee of EBMT.

1. **Before a WP Business Meeting (or other fixed time points in the year as defined by the WP)**, EBMT members can propose a study idea to the WP by filling out the **very concise EBMT Study Idea Template** providing the following information succinctly:
    - Background
    - Main objective/ Endpoint(s)
    - Proposed study type
        - *(Survey / Retrospective / Non-Interventional Prospective, Registry-based / additional data collection)*
    - Study population
        - *(e.g. adults with Acute Leukaemia treated with allogeneic transplant in the last five years, children with an autoimmune disorder who experienced acute Graft versus Host Disease, etc.)*
    - Study time horizon
        - *(e.g. from the start of conditioning until three months after transplant, within one year after infection, etc.)*
2. The WP Secretary will keep track of all proposed study ideas and communicate them to the WP Chair and WP Study Coordinator/Data Manager.
3. In case the study topic fits within the scope of another WP, the Chair of the WP should evaluate the overlap with the implicated WP. It is the Chair's responsibility to carefully evaluate the possible overlaps with other WPs. In case of doubt, it is best practice to discuss the case with the other potentially implicated WP/WPs. If it is evaluated as a significant overlap, the WP chair will contact the chair of the WP/WPs to check for overlapping studies and propose a potential joint study, and in that case, agree upon who will proceed with the proposal, submission and selection process. This should be discussed between each WP chair and a mutually acceptable work plan should be agreed upon. This is similar to the process that occurs surrounding current CAR-T-orientated studies to avoid overlap and dual workload.
4. If a similar study idea has not already been accepted within EBMT and the WP has enough resources available to start a new study, the PI will be asked to ideally give a **very concise presentation (5-10 min) of their study idea** containing the points from the EBMT Study Idea Template during the WP Business Meeting or during an organised virtual WP meeting if possible. If not possible, this can be carried out through email correspondence incorporating all relevant parties for input.

**Please note that these timelines serve to give both the PI and the WP team an indication of what can be expected in terms of duration. However, in certain circumstances, more time can be needed (e.g. heavy workload/sickness/annual leave), and upon request, the timelines can be extended, provided that there is clear mutual communication.*

5. During the meeting, the other WP members are given the opportunity for questions and comments, providing input to the PI to further refine their research idea.
6. If applicable, after the WP Business Meeting or other virtual meeting, the WP Study Coordinator/Data Manager will perform a feasibility check (i.e. a general check on whether the required data is sufficiently available within the database).
7. If the study idea is feasible and seems sufficiently relevant, the WP Study Coordinator/Data Manager will provide the PI with the EBMT Study Protocol Template.

Please note that the preliminary concise proposal and presentation of the study idea serve to prevent receiving multiple study protocols on a similar topic and to confirm the study is indeed feasible before the PI dedicates their time and effort to preparing the full study protocol. Plus, it will allow the EBMT scientific community to provide input beforehand, and discuss, and potentially refine, the study idea before it is finalised.

There is, however, **no guarantee** that the study protocol will eventually be accepted, as this decision will be based upon the full protocol, including the input of the WP Statistician, and the resources needed versus the resources available within the WP.

II. Preparing the study protocol

The following sections of the EBMT Study Protocol Template will be filled in by the PI as best they can **within four weeks after the WP Business Meeting or other virtual meeting or, if applicable, after receiving the results from the feasibility check***:

1. Title of the study
2. Principal investigator (PI)
3. Rationale & background
4. Main objective and research questions:
    - The primary research question
    - Secondary research questions
5. Study design (in collaboration with the WP Statistician)
6. Study population:
    - Inclusion criteria
    - Exclusion criteria
    - Sample size (in collaboration with the WP Study Coordinator/Data Manager and Statistician)
    - Data collection & statistical analysis
    - Study variables
    - Endpoint(s) (in collaboration with the WP Statistician)
    - References
    - Data Sharing
7. Estimation of the required budget including the WP Study Coordinator/Data Manager and Statistician hours calculated in conjunction with the WP study team

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8. Upon completion of these sections, the PI will send the preliminary study protocol to the WP email address and put in a copy to the WP Chair and Secretary.
9. If necessary, the WP Study Coordinator/Data Manager and Statistician can make adjustments to these sections in consultation with the PI.
10. The following section of the EBMT Study Protocol Template will be filled in by the WP Statistician, in consultation with the PI, ideally within two weeks after the PI submits their sections*:
    - Study design
    - Study population
    - Sample size (if applicable, power calculation)
11. Data collection & statistical analysis:
    - Endpoint(s)
    - Statistical method
12. The following sections of the EBMT Study Protocol Template will be filled in by the WP Study Coordinator/Data Manager, within two weeks after the PI submits their sections*:
    - Working Party (WP)
    - Study population
    - Sample size (if applicable, a feasibility check, number expected to be included)
13. Authorship:
    - Writing Committee
14. The WP Study Coordinator/Data Manager and Statistician will also prepare the Time investment estimation (indication of the hours the WP Study Coordinator/Data Manager and Statistician are expected to have to invest in the study) and the Study timeline estimation (indication of how many months are expected between the start of study preparation and study completion) in a separate document. If this is felt to be excessive or disproportionate to the impact of the study, it should be discussed with the WP Chair on time for a decision on whether the study should proceed or not.

III. Assessing the study protocol

Within four weeks after completion of the EBMT Study Protocol Template*, the study protocol will be reviewed by a reviewing committee consisting of:

- WP Chair (optionally, the WP Secretary can also aid in reviewing the study protocol)
- WP Study Coordinator
- WP Statistician
- Additional members as decided by the WP (in case a WP has subcommittees, this could, for instance, be the Subcommittee Chairs, but the WP Chair could also request an EBMT member affiliated with the topic, but not directly involved in the study, to review the relevance of the study protocol)
- The reviewers will decide upon either ‘Accepted’, ‘Revision needed’ or ‘Rejected’ based upon the following criteria:

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Scientific impact:

These aspects can be graded as follows: 1, 2 or 3 with 3 being optimal and 1 being unclear/not specific/low impact/not novel/ill-fitted to the data:

- Clarity: clear objectives
- Focus: The variables to be collected are essential for answering research questions
- Relevance (to be determined by the scientific community, i.e. does not need to be assessed by WP Study Coordinator and Statistician): potential impact on patient care
- Novelty (to be determined by the scientific community, i.e. does not need to be assessed by WP Study Coordinator and Statistician): distinct from studies already being conducted on this topic
- Positioning (to be determined by the scientific community, i.e. does not need to be assessed by WP Study Coordinator and Statistician): good fit within the research field and a good addition to studies already being conducted on this topic

Feasibility:

These aspects can be graded as follows: 1, 2 or 3 with 3 being optimal and 1 being unavailable/inexecutable/statistically non-viable/insufficient return on resources spent:

- Resources: required resources are available within the WP (e.g. time/budget/people)
- It is recommended that a new study protocol be accepted when the study team concerning WP has an estimated current workload of less than 9 months. This means that a WP can **add a new study to the study list** when **the total estimated hours of work its ongoing studies require** does **not exceed the working hours** the Study Coordinator(s) and Data Manager(s) for that WP combined have **available in a period of 9 months**, or the working hours the Statistician has available in that period.
- However, this is not absolute and needs discussion within each WP as prioritisations will change to ensure agility and contemporary academic output.
- This allows the WP Study Coordinator/Data Manager Statistician and PI to have the availability and commitment to bring the study to publication in the short term. In case a study is scientifically relevant, but cannot be performed at that time due to lack of resources, the protocol can be reassessed at the next selection moment.
- *Executability*: data expected to be obtained and processed without major challenges and in line with EBMT's Data Sharing policy
- *Statistical viability (to be determined by the WP Statistician)*: study design and intended outcome data allow for statistically sound analyses
- *Return on investment*: The benefits of the intended output are worth the investment in the study

The PI will make potential revisions in close collaboration with the WP team **within two weeks after the initial assessment*.**

The WP Chair and Study Coordinator, who may consult with the other reviewers in case of doubt, will reassess the revised study protocol **within two weeks after resubmission***, and, if all previous issues have been satisfactorily resolved, the study protocol will be accepted and the final protocol will be signed. **It is imperative at this stage that all have agreed upon the main authorship list as regards**

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the PI(s), WP(s) Chair, Secretary members and potential other collaborators, in particular as regards the first author and senior author position.

Please note:

- Try to avoid making any amendments to the final, accepted study protocol. If amendments are necessary after the study protocol's acceptance, these amendments will first need to be approved by the WP Chair, Secretary, Study Coordinator and Statistician.
- If there are unreasonable delays with the planned PI in completing the required aspects on time, the WP Chair will have the right to reassign the study to another PI as deemed appropriate to the aim of moving the study forward.
- An audit of each WP, completed by members of the WP, should be considered yearly to review the study protocol selection process and identify delays and areas of potential improvement/ best practice.
- Each WP should also gauge the best time for the presentation of the project and at which meeting it should be presented – this should be when full analysis has occurred and sufficient scientific merit will be gained by sharing the results at that stage.

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IV. Responsibility assignment

R = **Responsible:** Those responsible for completing the task

A = **Accountable**: Those with ultimate responsibility for the task, who also assess whether the end product is satisfactory

S = **Supporting**: Those who assist the person responsible for the task

C = **Consulted**: Those whose advice, approval or input is sought by the person responsible for the task

I = **Informed:** Those who need to be informed about decisions, progress and results regarding the task

R = Responsible, **A** = Accountable, **S** = Supporting, **C** = Consulted, **I** = Informed

Task	PI	Chair	Secr	Stat	SC/DM
1. Preparation phase:					
Feasibility check	C	A	I	R	R
Protocol writing	R A	C	C	S	S
Protocol review	I	A	R	R	R
Data collection form creation	R A	C	C	S	R
2. Active data collection phase:					
Invite sending	I	I	I		R
Data entry					R
Additional query sending					R

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Data check	**C**			**C**	**R**
3. Analysing phase:					
Data file preparation	**I**	**I**	**I**	**C**	**R**
Statistical analyses	**C**	**I**	**I**	**R**	**S**
4. Manuscript phase:					
Manuscript writing	**R** **A**	**C**	**C**	**S** **C**	
Manuscript check	**I**	**R** **A**	**R**	**R**	**R**
Author list preparation	**C**	**A** **C**		**S**	**R**
Manuscript submission	**R**	**I**	**I**	**I**	**I**
If appl. Manuscript revision	**R** **A**	**C**	**C**	**R** **S**	**S**

PI: Principal Investigator, **Chair**: Working Party Chair, **Sec**: Working Party Secretary, **Stat**: Working Party Statistician, **SC/DM**: Working Party Study Coordinator and/or Data Manager

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Links

Please note: links are only correct at time of printing

Controlled Document links:

- **Document: CR 257: EBMT Study Idea Template** v1.0 (Pending Release (29-Feb-2024))
- **Document: CR 258: EBMT Study Protocol Template** v1.0 (Pending Release (29-Feb-2024))
- **Document: CR 259: EBMT Study Time Estimation** v1.0 (Pending Release (29-Feb-2024))

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