EAHAD
European Association for Haemophilia and Allied Disorders

Cours Saint Michel, 30B
1040 Brussels, Belgium
info@eahad.org | www.eahad.org
Registered Number (AISBL): 0718.594.509

EAHAD Real World Evidence Working Group

Terms of Reference

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1. Purpose and goals

The purpose of this Working Group is to define and standardize shared criteria and methodological standards for the collection of real-world evidence (RWE) data on inherited bleeding disorders across European Haemophilia Treatment Centres. While randomized clinical trials remain the cornerstone for evaluating efficacy and safety of therapies, regulatory authorities and health technology assessment bodies increasingly require real-world data to complement trial results and to support decision-making in routine clinical practice and reimbursement policies. This need is particularly relevant in rare diseases such as haemophilia and allied disorders, where clinical trials often involve limited patient numbers and may not fully capture long-term outcomes, treatment patterns, and heterogeneity of real-life care. The Working Group will engage with registries as all relevant stakeholders must be involved. It will prepare outcomes, which will be in line and in accord with existing data owners, like national and/or international registries. The goal is not to build brand new registries, but to optimize utilisation of all RWE available to date and to standardise approach where feasible, enabling the pooling of data across current and future RWE data sources in Europe. Ethical considerations, including ethical committee requirements and country-specific regulatory approvals, will be carefully addressed as part of this work, recognising the complexity and variability across European jurisdictions. In addition, collaboration with European Reference Networks will be actively pursued. In particular, the ERN initiative and its EpiBlood registry represent an important and emerging effort in this space, and there is a clear opportunity for alignment and mutual support, with EAHAD contributing its experience in real-world evidence generation. The Working Group will also benefit from the established collaboration between EAHAD and ISTH. The Working Group will have a completely different remit than the Accreditation and Audit Working Group but will work together with the EUHASS Steering Committee as appropriate.

The main goals of the Working Group are:

- To establish a harmonised framework for RWE data collection across haemophilia centres. The Working Group will determine if the data collection would be based on data from patients or centres, once the relevant parameters are defined.
- To define a minimum core dataset of parameters and key outcome measures suitable for routine clinical practice.
- To promote data quality, comparability, and interoperability across centres and national registries in Europe.
- To support the generation of robust RWE that can be used alongside clinical trial data for regulatory, clinical, and health policy purposes.

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2. Membership

The Working Group will include 6 to 8 members, with flexibility depending on expertise and geographical representation. Membership will include:

- Haematologists with recognised expertise in haemophilia care.
- Experts in clinical epidemiology, registries, and outcomes research.
- Specialists with experience in regulatory science and real-world data.
- Up to 1–2 patient representatives, to ensure patient-relevant outcomes are adequately considered.
- The Chair will be appointed by the EAHAD Executive Committee. A Vice-Chair can be nominated by the Working Group to provide assistance to the Chair and replace the Chair in his/her absence.
- The original composition of the Working Group will be decided by the Executive Committee and the Chair of the Working Group.
- The Working Group will include at least one EAHAD Executive Committee member.
- When a vacancy arises, or a new need in one or another specific field is identified, a call for applications will be published on the EAHAD website and circulated via EAHAD's networks. Current members will review the applications. Majority agreement within the Working Group is desired for the appointment of a new member. In case of a tie, the Chair has the deciding vote.

3. Duration of the mandate

The Working Group will have a fixed term mandate of 2 years. After that period, the mandate term will be re-evaluated by the Executive Committee, based on goals, objectives, and deliverables.

4. Frequency of Meetings

Maximum of 3 meetings per year. The meetings will be in-person or virtual.

In-person meetings should, when possible, be organised to coincide with other meetings (e.g. the annual conferences).

All members are required to actively participate in the work of the Working Group and attend at least 50% of the meetings organised

At least one staff representative from the EAHAD Office will attend the meetings and help with the preparatory work, the minutes and reporting.

5. Funding

Meetings and support by the EAHAD Office will be funded by EAHAD with possible external support according to the EAHAD Sponsorship Policy.

The annual budget is 15,000 EUR.

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The funding will cover in-person meetings, teleconferences, publication fees, IT support, etc.

6. Reporting requirements

The Working Group will produce reports outlining its progress on agreed objectives. The reports will be submitted to the Executive Committee. A summary will be included in the EAHAD annual report.

Minutes will be drafted and distributed to Working Group members after the meeting date.

7. Publication acknowledgement

Publications and other projects should acknowledge that they are produced by the Working Group, listing all members unless a different arrangement has been decided.

8. Review of the terms of reference

The Working Group will review the terms of reference every two (2) years.

September 2026