

EAHAD Gene Therapy Working Group

Terms of Reference

1. Purpose and goals

With gene therapy, haemophilia patients receive a one-time treatment and have factor levels that last for years or decades, rather than being dependent on repeated administration of factor concentrates or non-factor replacement therapy at short intervals.

Clinical studies have provided the proof of concept for gene transfer in haemophilia with various vectors and coagulation factor genes. Gene therapy is thus increasingly regarded as a realistic treatment option for haemophilia. The introduction and approval of gene therapy for haemophilia present a fundamental paradigm shift in the treatment of haemophilia with many potential consequences for patients and the haemophilia centres.

In this context EAHAD established a Gene Therapy Working Group. The Working Group will strive to be at the forefront of providing critical information to health professionals and patients about the practicality and safety of gene therapy.

The Working Group will continuously work towards a set of objectives agreed in discussion with the EAHAD Executive Committee.

2. Membership

- The Working Group will include at least four (4) members from at least three (3) different European countries and a maximum of ten (10) members.
- The Working Group could invite co-opted member as observers based on their expertise and needs of the different projects.
- The Working Group members must be experts in the field of gene therapy and able to substantially contribute to support EAHAD.
- 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 discussed with the Executive Committee and the Chair of the Working Group.
- The Working Group will include at least one EAHAD Executive Committee member and one patient's representative designated by the EHC.
- When a vacancy arises or a new need in one or another specific field is identified in the Working Group, a call for new members will be published on the EAHAD website and circulated via EAHAD's social networks. Existing members will review the applications. Agreement within the Working Group is desired and a majority of members of the Working Group must agree on the appointment of a new member. In case of a tie, the Chair has the deciding vote.

- Members of the Working Group will have a term of two (2) years with possibility of one (1) year extension and can be re-elected.

3. Duration of the mandate

- The Working Group will have a fixed term mandate of three (3) 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 five (5) 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 congress)
- All Working Group Members are required to actively participate in the work of the Working Group
- 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 10,000 EUR.
- 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. Dissemination activities

- The Working Group will support the dissemination of its activities by providing content to the EAHAD staff to share via the EAHAD website and social media channels
- Publications and presentations by the Working Group are encouraged
- Any publications should acknowledge the Working Group and all its members

8. Review of the terms of reference

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

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