Dreams

DREAMS project

[D8.4] Creation of the Patient Advisory Board (PAB)

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TERM	DEFINITION
DMD	Duchenne Muscular Dystrophy
EDMD2	Emery-dreyfuss muscular dystrophy
EU	European Union
IPR	Intellectual Property Rights
NDA	Non-Disclosure Agreement
PAB	Patient Advisory Board
PRO	Patient-Reported Outcomes

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1. EXECUTIVE SUMMARY

The present document represents Deliverable 8.4 – Patient Advisory Board of the DREAMS project. It has been developed as part of Work Package 8.

Set to span five years, the DREAMS consortium is paving the way for a novel approach to treating neuromuscular diseases. This transformative project merges the power of artificial intelligence, pluripotent stem cells, and cutting-edge pharmacological screening techniques to seek out therapeutic solutions for five debilitating conditions affecting more than 400 000 people worldwide.

The DREAMS consortium unites a highly skilled and complementary consortium of nine European partners, encompassing clinicians, patients, academic researchers, and private stakeholders. These organizations include: I-Stem (France), Kantify (Belgium), The Institute of Myology (France), Center for Neuroscience and Cell Biology - University of Coimbra (Portugal), The Technion - Israel Institute of Technology (Israel), Samsara Therapeutics (UK), Assistance Publique - Hôpitaux de Paris (France), AFM-Telethon (France) and Zabala Innovation (Spain).

DREAMS Patient Advisory Board (PAB) will be created to ensure that Patients will be involved in all activities of the project. The PAB enables patient voices to be heard throughout the research and development process of the DREAMS PROJECT. It is designed to contribute to defining the project's priorities by facilitating the exchange of information between representatives of the DREAMS consortium and patients, as well as their relatives.

2. Organisation of the Patient Advisory Board (PAB)

2.1 Chairmanship & membership

The Patient Advisory Board (PAB) operates under the coordination of AFM-Téléthon. Wael Khazen, AFM's Clinical Project Manager, assumes the role of PAB chairman, he will serve as the primary point of contact. In this capacity, he oversees communication, coordination, meeting organization, and the drafting of meeting minutes. The PAB comprises 10 patients or patient representatives from various European countries, with two representatives designated for each of the initially selected five neuromuscular diseases: Duchenne Muscular Dystrophy (DMD), Emery-Dreyfuss Muscular Dystrophy (EDMD2), Centronuclear Myopathy linked to DNM2 gene mutations, Danon Disease, and Pompe Disease.

2.2 Members

AFM has actively collaborated with multiple patient networks and associations across Europe to establish the PAB, which includes representatives from its existing internal peer-support groups and the ERN Euro NMD Patient Advisory Board, currently led by AFM-Telethon. DREAMS's primary focus has been on engaging patients or their representatives with backgrounds in patient networks and associations, as well as individuals possessing expertise in scientific, medical, sociology or clinical fields. The patients recruited bring a rich diversity of skills to the table; some are seasoned in medical and clinical research, while others excel in patient networks, associations, or legal realms. The overarching goal has been to integrate various forms of expertise to strengthen the development and management of the Dreams project.

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The DREAMS consortium has aimed to incorporate 2 patients per pathology (duchenne muscular dystrophy (DMD), emery-dreyfuss muscular dystrophy (EDMD2), centronuclear myopathy linked to DNM2 gene mutations, danon disease, pompe disease) into the PAB. Presently, 2 patient representatives per pathology have been secured, except for DNM2, for which currently only one patient representative has been identified. To meet the 10-member target for the PAB, 3 patient representatives for Duchenne have been enlisted, given it's the pathology with highest incidence among the 5. Consequently, all 10 patient members of the PAB have already consented to participate.

Table 1. DREAMS PAB members

PAB member	Pathology/ Country
#1	Duchenne Muscular Dystrophy (DMD)/ Greece
#2	Duchenne Muscular Dystrophy (DMD)/ Portugal
#3	Duchenne Muscular Dystrophy (DMD)/ Romania
#4	Danon/ France
#5	Danon/ England
#6	Centronuclear Myopathy linked to DNM2/ Ireland
#7	Emery-Dreifuss Muscular Dystrophy (EDMD)/ France
#8	Emery-Dreifuss Muscular Dystrophy (EDMD)/ Italy
#9	Pompe/ France
#10	Pompe/ France

Note: The biographies of each member are not available in the public version of this deliverable.

2.3 Meeting frequency

PAB will meet at least every 6 months to follow the project development and dedicated meetings will be organized every time it is useful to collect patients' opinions, meetings can be adjusted depending on the progress of the project (the provisional total duration of the project is 5 years). Meetings are primarily conducted via video conference.
Patients participating in the PAB may also receive invitations to join the consortium's annual meeting.
The solicitation and meetings of the PAB vary depending on the stage of advancement of the project.

However, PAB members actively participate in the three key stages of the project:

- Development of the operating charter of the Patient Advisory Board, which outlines the rules, objectives, and missions of this board.
- Participation in the design phase of the study, including protocol development, evaluation criteria, Patient-Reported Outcomes (PRO), and eligibility criteria.
- Once the candidate drug(s) have been selected, involvement in determining various parameters related to these medications, such as formulation, mode of administration, toxicity, and tolerance.

2.4 Agenda

The PAB chairman (AFM) is required to compile and distribute the meeting agenda no later than 7 days prior to the scheduled meeting. The agenda should detail the topics to be discussed and the individuals responsible for each item. Additionally, any PAB member may propose additional agenda items by providing written notice to all other members at least 3 days before the meeting.

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2.5 Minutes

Minutes of the PAB meetings, once accepted, shall be sent by the PAB chairman to the different Members of PAB for information and/or validation.

3. Missions, obligations, and rights of the PAB members

3.1 Missions

The patient advisory board enables patient voices to be heard throughout the research and development process of the DREAMS project. It is designed to contribute to defining the project's priorities by facilitating the exchange of information between representatives of the DREAMS consortium and patients, as well as their relatives.

The PAB will be structured around active patient involvement, aiming to identify patient needs, provide clinical research with a patient perspective on their lives and the impact of treatments, and ensure patient engagement at various levels of the project. The PAB will enable the DREAMS consortium to align itself with the realities as perceived by patients.

Two patients from the Patient Advisory Board (PAB) will be incorporated into the clinical expert committee that will design a clinical trial adapted to rare neuromuscular diseases (Deliverable D6.2 – Innovative clinical trial design), to facilitate dialogue between clinical experts and patient representatives. These two patients will be encouraged to actively engage in discussions within the clinical expert committee, incorporating the valuable insights and experiences of individuals affected by neuromuscular diseases into the development of clinical trial designs.

3.2 Tasks

The tasks assigned to the PAB are as follows:

- Represent patients in dealings with other stakeholders and facilitate exchanges.
- Enabling the patient voice to be taken into account in the design and conduct of clinical trials.
- Working closely with clinical expert committee.
- Be an active member of the project and the decision-making process.
- Establish priorities and projects to improve the patient experience.
- Feedback on practices and guidelines that patients find significant and useful.
- Review all the documents.

In the context of future clinical studies, PAB is supposed to participate in:

- Definition of eligibility criteria in clinical studies (criteria for inclusion and exclusion of participating patients).
- Assist in the development of participant recruitment strategies and engagement initiatives.
- Study design and methodology.
- The identification of pertinent PRO Measures to be included in the clinical trial protocol.
- Review of essential documents (protocols, patient information and consent, case report form, etc.)

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- To collaborate with researchers, clinicians, and study sponsors to prioritize patient-relevant outcomes and endpoints.
- To ensure that study findings are communicated in a clear, accessible manner to participants and the broader patient community.

PAB can be called upon by all the General Assembly or any of the 9 partners of the Dreams consortium to provide patient advice.

3.3 Protection of Intellectual Property

PAB members recognize the importance of protecting the intellectual property rights (IPR) arising from the project's research and innovation activities. All PAB members commit to refrain from publicly disclosing any information that could compromise the protection of intellectual property rights (either existing or new) until appropriate protective measures are taken, by signing a Non-Disclosure Agreement (see section 3.7). This information includes but is not limited to promising or validated therapeutic targets, promising or validated molecules and their properties or mode of action, promising or validated therapeutic indications.

3.4 Personal Data Protection

The personal data collected for this project is processed and stored in a secure computer file. The data collected will be communicated only to authorized persons who have a link with the PAB and who are subject to the duty of confidentiality. The data will be kept for the duration of the project (5 years), but also for 5 years afterwards for monitoring purposes.
In compliance with current regulations on the protection of personal data, all patients and their relatives have the right to access, rectify and delete their data, subject to the Board's management requirements.
To exercise these rights, patients may contact the person responsible for data processing (Wael Khazen).

- <u>The following data will be processed and stored for this project:</u>
  - First name, name.
  - Age.
  - Disease (including genetic mutations)
  - Patient's general condition.
  - Contact details (e-mail address and telephone number)
  - Professional experience (expertise)
  - patient biography

- <u>This data is processed and stored for the following purposes:</u>
  - Drawing up a patient file for selection by the Board.
  - Taking into account the pathology for the needs of the project.
  - Anonymized transmission of data to the appropriate project teams.
  - Retention of patient files for follow-up.
  - Relations with regulatory authorities and patient associations.
  - Communication (including intra-group communication)

- <u>Data retention period: 10 years</u>

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3.5 Integrity, independence, and conflicts of interest

To fulfill the missions outlined in this document, each member of the PAB must declare any potential links (whether familial, financial, organizational, or occupational) with the DREAMS consortium and its 9 partners. It is important to note that the presence of such potential conflict of interest does not automatically disqualify participation in the Board's work. However, these links must be carefully examined and evaluated on a case-by-case basis to determine if they could lead to a conflict of interest within the project.
The objective is to prevent conflicts of interest and situations where patients may feel compelled to refrain from participation due to perceived risks. In such cases, patients are encouraged to refer the matter to the AFM-Téléthon Scientific Department (which will consult the Risk and Compliance Department). This department will then provide written confirmation if a conflict of interest exists. If a conflict of interest is confirmed by the AFM-Téléthon Scientific Department, the patient must immediately cease all activities related to the concerned protocol.
Examples of potential conflicts of interest include:

- Attempting to influence the selection of eligibility criteria for inclusion in the clinical study to promote one's own or a family member's participation.
- Attempting to influence the choice of investigators participating in the study (e.g., by encouraging acquaintances to participate as coordinating or principal investigators).
- Attempting to influence the selection of investigator centers (e.g., by favoring centers close to one's residence).
- Attempting to influence the selection of the study's primary or secondary endpoints to prioritize the treatment of one's own pathology.
- Disclosing confidential information about the study to personal networks.

In cases of exceptional circumstances, such as extreme behavior or blatant conflicts of interest, the Project coordinator, in collaboration with the Ethics committee of the project will adjudicate on the situation.

3.6 Operating charter of the patient advisory board

The various members of the PAB must sign an operating charter before participating in the PAB.
The purpose of this Charter for the organization and operation of the Patients' Advisory Board is, on the one hand, to facilitate collaboration between the stakeholders in clinical research and, on the other hand, to specify the rules and procedures of this Board, both upstream and downstream of the project.
The primary concern is to establish principles that foster relationships among stakeholders and while upholding the value of transparency. However, this Charter's objectives extend beyond this, as it must also ensure that all parties involved are aware of patients' rights, particularly the right to participate in clinical studies within the framework of the Dreams project. The Patient Advisory Board will enable the DREAMS consortium to align itself with the realities as perceived by patients. This Charter will outline the AFM's guiding principles on this matter, including:

- **Transparency**: Adherence to strict standards throughout the project, from inception to completion.
- **Independence:** Ensuring independence of opinions and recommendations.
- **Privacy:** Upholding confidentiality for all information shared with the Board, including discussions, reports, and summaries, unless otherwise agreed by the Board.

This Charter will initially introduce the PAB by outlining its objectives, missions, and composition, followed by detailing its establishment and organization.

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3.7 Non-Disclosure Agreement (NDA)

The tasks performed by the PAB are carried out in strict compliance with the rules of integrity and confidentiality. The PAB's opinions, advice and analyses remain free and independent.
The signature of the Non-Disclosure Agreement (NDA) by the members of PAB signifies their commitment to maintaining confidentiality regarding sensitive information related to the study. This agreement ensures that all members of the Board understand their responsibility to protect proprietary data, research findings, and any other confidential information disclosed during the Dreams project.

3.8 Meeting Deliberations

The discussions, deliberations, and materials presented during PAB meetings are confidential and should be treated as such by all members. This includes, but is not limited to, discussions on:

- Potential intellectual property and patent strategies.
- Specifics of commercialization and market entry strategies.
- Identification and analysis of potential stakeholders and partners.

3.9 Responsibility of Consortium Members

By appointing a representative to the PAB, each DREAMS partner ensures that its representative will respect and adhere to the confidentiality obligations applicable to all members of the consortium. These obligations, as described in the projects Consortium Agreement include:

- Maintaining the confidentiality of discussions, decisions, and documents related to the PAB meetings.
- Ensuring that any confidential information obtained through participation in the PAB is not disclosed to unauthorized individuals or used for any purpose outside the scope of the project.

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