Roles and responsibilities of
EURORDIS Patient representatives (members, alternates and observers/experts)
in the Committee for Orphan Medicinal Products (COMP)

Legislation (Regulation (EC) Nº 141/2000 and Regulation (EC) 726/2004) provides for patient membership in the Committee for Orphan Medicinal Products (COMP) amongst other scientific committees.

EURORDIS, who actively participated in the development and implementation of the Orphan Regulation, would like to ensure that rare disease patients are represented by full EURORDIS members at the COMP. These full EURORDIS members may be proposed by EURORDIS in answering a Call for Expression of Interest by the European Commission.

Alternatively, EURORDIS may also endorse the application of rare disease representatives pertaining to its Membership who may want to apply on their own.

The following job description clarifies the roles and responsibilities of the rare diseases patient representatives proposed or endorsed by EURORDIS within the European Medicines Agency's Committee for Orphan Medicinal Products.

General role:

In addition to the general work performed by each COMP member, the rare disease patient representative (in collaboration and with the support of EURORDIS) is expected to:

Role:

- Represent patients interests and provide a "patient or parent of patient perspective" view, on behalf of those directly affected by regulatory decisions
- To be involved in the activities of the TAG (Therapeutic Action Group) and to liaise with EURORDIS on a monthly basis at the minimum by providing feedback from the COMP meetings

Scope of the work:

- Team up is crucial between the representatives (members, alternates and observers/experts) from the various EMA Committees (COMP, PDCO, CAT) and EURORDIS representatives on the PCWP (Patients and Consumers Working Party)
- Liaise with EURORDIS by:
  - Participating actively in the EURORDIS Therapeutic Action Group (TAG) monthly conference call and annual meetings.
  - Sending a brief report after each Committee for Orphan Medicinal Products meeting (or sharing orally during TAG conference calls) and sending a report when attending to conferences (to TAG or to EPAC depending on the topic of the conference)
  - Being closely involved in EURORDIS activities related to therapeutic development and patient engagement in the medicinal product lifecycle
- In the review of applications and contribution to policy and guidelines, you are expected to:
  - Bring experience of the disease and/or identify patients with experience of the disease when necessary
  - Raise ethical issues during the discussion; identify ethical risk factors, propose risk prevention and minimisation measures
  - Identify potential topics which may require or benefit from additional patient consultation

- Actively contribute to patient information and communication material related to medicines, disseminate Committees' outcomes when they become public; pass on information to patients and patients' organisations
- Be prepared to participate or speak in other meetings and conferences
- To promote the European Regulation and the results of its implementation

Mandate

- 3 years, renewable

Time Commitment / Workload

7-8 days/month (3 in London, 4-5 homework) plus about 8 additional days of travel per year.

- Time in COMP meetings:
  - 3 days/month in London
  - Additional meetings in London may be organised.
- Homework: we estimate about 4-5 days/month of work to prepare and follow up the meetings. EMA estimates 250-300 applications for orphan designation per year, over 100 applications for protocol assistance per year and around 10 applications for reassessment of the orphan status at the time of marketing authorisation. Patients' representatives are expected to have an opinion on all dossiers, but not necessarily a scientific opinion as their main role is to introduce the patients' perspective.
- **Capacity-building opportunities**: approximately 8 days/year
  - Participation in the EURORDIS Membership Meeting, the European Conference on Rare Diseases and the EURORDIS Round Table of Companies Workshops (depending on the topic of the workshop).
  - Participation in national meetings (e.g. national rare disease alliances) or international meetings (e.g. DIA Euromeetings and forums, TOPRA…)
  - Participation in training and capacity-building opportunities (e.g. DIA tutorials, University seminars)

Confidentiality and Conflicts of Interest

- All patients' representatives are expected to comply with the EMA for full confidentiality and declaration of potential conflict of interest
- Patients' representatives may be representing EURORDIS in the committee, if they are identified, selected and proposed by EURORDIS and appointed by the European Commission on behalf of EURORDIS
- Alternatively, patients' representatives may be representing their own organisations and will be appointed by the European Commission in that capacity. Nevertheless, EURORDIS can endorse patient representatives' applications through a letter of support.
- In the first case, it is EURORDIS policy that patients' representatives maintain their double affiliation when presenting themselves (i.e. EURORDIS and their own patients' organisation)
- As a consequence patients' representatives must declare their potential Conflicts of Interests at three levels:
  - i) those of EURORDIS (provided by EURORDIS)
  - ii) those of their own patient association provided by the President of the association and
  - iii) their own personal Conflicts of Interests as individuals.

  All Declarations of Conflict of Interest should be submitted concurrently to EMA and EURORDIS.

Conditions

- All EMA meetings are on week days, travels and meetings organized by Eurordis can be on weekends
- Member of an EMA Committee:
  - The travel and accommodation expenses are covered by the EMA

    - The member receives a daily allowance (or a double daily allowance) to cover his/her extra expenses in London, when travelling for the Committee for Orphan Medicinal Products.
- Alternate of an EMA Committee: not applicable for COMP)
- Observers/Experts
    - EMA may invite patient representatives on an ad hoc basis and cover their travel and accommodation
    - Alternatively, the travel and accommodation expenses are covered by EURORDIS upon prior approval by the Therapeutic Development Director (following EURORDIS rules)
- Your travel and accommodation expenses are covered by EURORDIS or the conference organiser, when travelling for EURORDIS on agreed assignments. The rules of EURORDIS must be respected (i.e. agreement on cost of travel and accommodation) and EURORDIS reimbursement claim forms must be used. Prior agreement has to be obtained from EURORDIS Therapeutic Development Director.

Profile

- Must belong to a rare disease patient organisation that is a full member of EURORDIS (preference is given to patients or parents of patients)
- Based in the European Union
- Available to travel to London 3 days/month plus a few other travel assignments and to dedicate enough time for the preparatory work
- Fluent English is compulsory (spoken and written)
- Although a medical background is not a requirement, a broad knowledge of medical and to a certain extent regulatory issues related to the research, approval and use of medicines is recommended and will be needed to effectively contribute to the scientific discussions of the Committee.