

## ERN ITHACA Patient Advisory Board (PAB)

### Terms of References ToR (2023-2025)

#### European Patient Advocacy Groups (ePAGs)

##### *About ERN ITHACA*

*“ERN-ITHACA is a patient centred network which meets the needs for highly specialized, multidisciplinary healthcare for patients with rare (multiple) malformation syndromes and rare intellectual and other neurodevelopmental disorders of genetic, genomic/chromosomal or environmental origin, both diagnosed and undiagnosed. ITHACA stands for Intellectual disability, TeleHealth, Autism and Congenital Anomalies.*

*The name ITHACA also refers to the Odyssey that for many families the diagnostic journey of a child with a rare developmental disease represents.”*

**Terms of reference (TOR)** define the rules of ePAGs' engagement in the ERN ITHACA: aims, objectives, and the activities agreed to work together in order to accomplish the main goal, to represent the voice of their community in the ERN ITHACA.

These recommendations for patient engagement aim at facilitating the effective and meaningful engagement of patient representatives as a partner in the activities of European Reference Network on congenital malformations and rare intellectual disability (short [ERN ITHACA](#)). They are based on the [governance framework](#) developed by EURORDIS for patient engagement consistent with the rules of ERN, and contain specific provisions to adapt them to the governance structure of [ERN ITHACA](#).

#### **Introduction**

According to the European Commission Expert Group (EUCERD) patients and patient representatives should play an active role in the decision and opinion making process of the European Reference Networks (ERNs) and be involved in structural and clinical network activities. The Expert Group recommended that ERNs demonstrate meaningful patient involvement, patient-centeredness, and empowerment through recognition of the role of patients, as experts by experience and co-producers of knowledge, in the ERNs structural and clinical activities and therefore demonstrate meeting the legal requirements in the Delegated Acts.

The position of ePAG advocate within the ERN ITHACA Patient Council is a voluntary position and does not involve any financial compensation. Travel and accommodation expenses will be reimbursed according to the ERN ITHACA policy on reimbursement for travel expenses.

ePags act as facilitators and interface between ERN ITHACA and their wider community.

#### **Constitution**

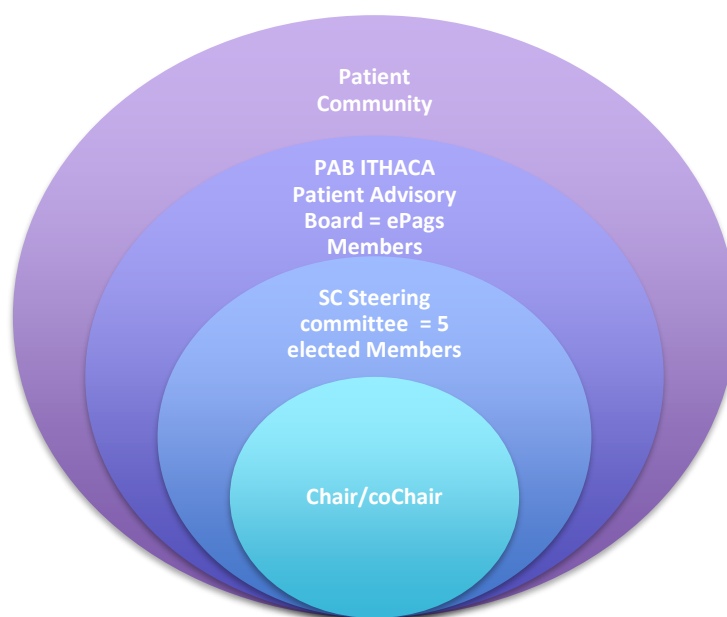
The **ERN ITHACA Patient Advisory Board (PAB)** is established under the direction of ERN ITHACA European Reference Network on congenital malformations and rare intellectual disability.

The role of **European Patient Advocacy Groups (ePAGs)** is to represent the voice and interests of the patient community and act as a bridge between this community and the ERN.

ePAG have an official permanent mandate to represent PO member organisations.

They liaise with these organisations to ensure a real and fair representation of the patient voice by participating in the ERN ITHACA Board, working groups and task forces.

## Patients Representative in ERN ITHACA



## Aims

The aims of the **ERN ITHACA Patient Advisory Board (PAB)** are to:

- Represent the voice of patients within ERN ITHACA to ensure that the needs of people living with congenital malformation or rare intellectual disability are included in the discussions of the Executive Committee and in the strategic and operational delivery of the network.
- Create a bridge between the ERN ITHACA Executive Committee and the ERN ITHACA patient community, ultimately to improve access to high quality diagnosis, care and treatment.

The PAB will deliver these aims through undertaking the following activities:

- Contribute to the development of patient information, treatment policies, good practice guidelines and care pathways.
- Support the network in the dissemination of information and communication to the wider patient community.
- Contribute to the development of research priorities and ensure that they are informed by the needs of patients and families.
- Provide advice on ethical issues in relation to areas such as the application of personal data rules, compliance with information consent and management of complaints.
- Engage with the appropriate patient communities for disease specific activities and projects.

- Contribute to regular teleconferences and other communications so that all patient representatives are involved and up to date with Patient Council and [ERN ITHACA](#) Executive Committee activities and developments.
- Work to grow the representation and engagement of patients in the Working Groups.

## Mandate

The ERN ITHACA PAB has an important role and missions in Governance, Ethical issues, Care, Research, Evaluation, and as expert patients in education of patients and families. In order to ensure that the ERN ITHACA PAB provides quality recommendations and feedback that genuinely reflects patient's viewpoints, members commit to:

- Represent the voice of the ITHACA community in network meetings on behalf of the community.
- Conduct proper internal consultation with their respective ePAG patient organisations to the best of their ability.
- Share important news and send a brief report of any ERN meeting attended to ERN ITHACA, their respective ePAG affiliated members and EURORDIS.
- Inform ERN ITHACA and EURORDIS when representing the ERN ITHACA PAB at a conference, workshop, or any meeting of public importance.
- Declare their potential Conflicts of Interests in formal meetings or activities.
- Comply with the ERN ITHACA conflict of interest policy.
- Adhere to the terms of the mediation agreement in the case of a mediation process described in the [the EURORDIS ePAG Constitution and Rules of Procedure](#)

## Composition and membership

The ERN ITHACA PAB membership is voluntary. It comprises all rare disease patient organisations based in Europe (members and non-members of EURORDIS) which represent a specific rare disease who falls under the scope of the ERN ITHACA and that have expressed their interest and have registered with EURORDIS to be engaged with this ERN.

This ERN ITHACA PAB is composed by the [ePAG Community](#) and the [ePAG Advocates](#) who are willing to be involved (at different levels described below, either as member of the PAB, Elected member of the Patient Board with Chair and co-chair of the Board, as representatives of the working groups, etc.) and to participate in any other capacity or activity related to the ERN ITHACA.

- [The ePAG Community](#) is known as a group of Patient Organisations, whether members and non-members of EURORDIS, that have registered with EURORDIS to be engaged with the ERN ITHACA, to collaborate on specific tasks (e.g. respond to surveys), to disseminate information about the ERN across their wider patient community, be consulted occasionally for feedback, and be kept informed on the development of the ERN. These Patient Organisations won't be involved actively in any of the working groups as an advocate.

[To join the ERN ITHACA ePAG Community](#), it is best that a Patient Organisation be in contact with EURORDIS to register and become a member, but it's not obligatory. The EURORDIS ERN team will inform the ERN ITHACA PAB and the ERN management team of any Patient Organisations who have registered their interest to engage with the ERN on a continuous basis.

- [ePAG Advocates](#) are patient representatives officially endorsed by a Patient Organisation based in Europe who have knowledge of, or experience of, living with a rare disease that is included in the

scope of ERN ITHACA. They provide support on a voluntary basis and represent the interests of the wider patient community. They have to meet the conditions and roles for being an ePAG Advocate and agree to adhere to the set of core values outlined in the [EURORDIS ePAG Constitution and Rules of Procedure](#) (Section 4).

- **ERN ITHACA ePAG Advocates** are involved in the network activities and they are active in the ERN governance structure including its working groups as listed below. They collaborate with the ERN clinicians and the research leads on a set of different activities such as the development of patient information, treatment policies, good practice guidelines, care pathways, research priority setting and monitoring and assessment.
- **The enrolment of new ePAG Advocates** is done through a written application. Once a year on a Specific ePAGS Call (generally from June to September). The new patient representative should submit an endorsement letter signed by the legal representative of his or her Patient Organization as well as a motivation letter (one page) and a short CV to the EURORDIS ERN team. (Documents are prepared for this purpose).  
[Approval of the application will be granted or refused by the ERN ITHACA Network Coordinator represented by the Project manager in charge Patient Program and the existing ERN ITHACA PAB, in consultation with the SC and EURORDIS when needed.](#)  
Each year ePags are asked to renew their membership to the PAB and to notify us of any change in their situation.
- **ePAG advocates also commit to adhering to the following set of core values**
  - Respecting the mission of the ERN ITHACA and its governance structure.
  - Listening to the opinions and requests of others.
  - Showing solidarity, mutual respect and support.
  - Adhering to the principles of equity and social justice.
  - Conducting themselves with professionalism in engaging with the clinical, research leads and fellow patient advocates.
- **ePAG alternates**  
PAB ITHACA may nominate an alternate from his/ her own patient organisation to attend specific meetings. The ERN Coordinator and or the Chair of the ERN ITHACA PAB shall validate the participation of such alternates in the meetings, respectively. Alternates must comply with the ERN ITHACA conflict of interest policy and with the core principles when attending ERN or ePAG meetings.

### **Steering Committee (SC): Elected Members of the PAB**

The Patient Board of the PAB consists of five representatives of patient organisations elected by the PAB based in Europe who sit on the ERN ITHACA Board. Among this group of five members, two persons are designated as Chair and Co-chair and represent Patient Council in the ERN ITHACA Executive Committee (short ExCom). In addition to the role and responsibilities of the members of the PAB, the Board has the following responsibilities:

- Represent the voice of patients in the [ERN ITHACA Board](#) and [ERN ITHACA Executive Committee](#) to ensure that the needs of people living with congenital malformation or rare intellectual disability are included in the discussions and in the strategic and operational delivery of the network.
- Ensure that the [ERN ITHACA Board](#) and [ERN ITHACA Executive Committee](#) consider and prioritise the patient, carer, and family perspective.

- Contribute to regular teleconferences and other communications so that all ERN ITHACA patient advocates are involved and up to date with the network activities and developments.
- Liaise with members of the ERN ITHACA PAB prior to ERN ITHACA Executive Committee and ERN ITHACA Board meetings, or an ad-hoc basis if needed, to address a specific request, to collect their input on any relevant items of the agenda and inform the group on a regular basis about the outcome of the Executive Committee and ERN Board discussions.
- Share important news and update the members of the ERN ITHACA PAB and the wider patient community on the network activities as appropriate.
- Ensure the sustainability of the ERN ITHACA PAB group.
- Monitor ePAG advocates activities in the Work Packages.
- In collaboration with ERN Coordinator coordinate the activities of the whole group, and report shared vision.

**The Chair and the Co-chair of the Board** are also responsible for sharing important information and for updating the ERN ITHACA PAB activities as appropriate. They consult with the ERN ITHACA PAB on relevant issues and feedback information to the ERN ITHACA Network Coordinator and to the Executive Committee. The ERN ITHACA Patient Board has the right to co-opt an ePAG member with a special expertise required by the tasks of the board.

**An ePAG volunteer Secretary** may be appointed by the SC to help the PAB, in supporting the administrative tasks of the ePAG group, including welcoming the new ePAG advocates or the new members of the ePAG Community; setting up the regular ePAG meetings (agenda, minutes, follow-up actions) and disseminating any relevant information to the ePAG group.

Each decision in the ERN ITHACA PAB is taken by consensus, but vote is possible if necessary. In case of equality, the voice of the Chair is predominant.

### Election Process Steering Committee (SC)

The ERN ITHACA PAB has elected 5 ePAG Advocates, known as **the Steering Committee (SC)**, who sit on the ERN ITHACA Board and will represent the voice of the members of the ERN ITHACA PAB. Among this group, 2 persons have been designated as chair and co-chair (see table below) to represent the Patient Council in the ERN ITHACA Executive committee. The Board of the Patient Council has voting power (if voting is necessary) and is enrolled for a 2-years term renewable thereafter. Be aware that some Positions in the PAB or SC means time commitment.

### Steering Committee (2023- 2025)

ERN ITHACA (Governance Excom,Board)	Name	Role
ERN Executive Committee & ERN Board	. Dorica Dan	Chair
ERN Executive Committee & ERN Board	. Tanja Zdolsek	Co-Chair
ERN Board	. Sue Routledge	Elected
ERN Board	. Tomasz Grybek	Elected
ERN Board	. Katarzyna Swieczkowska	Elected

*Nb: In the event of departure during the term of a mandate, a replacement shall be appointed in agreement with the PAB.*

### **Collaboration with other (external) patient organisations registered in Europe**

External patient organisations registered in Europe that do not have a formal representative in the ePAG may wish to engage with the ERN ITHACA. This wider community of patient organisations may be willing to collaborate on specific tasks (e.g. respond to surveys), help to disseminate information about the ERN across their wider patient community, be consulted occasionally for feedback, and be kept informed on the development of the ERN activities.

### **Collaboration with individual patients and family members**

Individual patients or family members may want to volunteer their time to collaborate with the on specific tasks or projects. However, when engaging in the ePAG or ERN activities and projects, they must comply with the ERN ITHACA conflict of interest policy and the core principles.

### **Decision-making and disputes**

Each decision in the ERN ITHACA Patient Council is taken by consensus, but vote is possible if necessary. In case of equality, the voice of the Chair is predominant.

If a dispute arises between patient representatives in the ePAGs, they commit in good faith to try to take steps to resolve the dispute together and supported by EURORDIS. We will refer to the support of Eurordis PAG Constitution and Rules of Procedure. However the ERN ITHACA Coordinator will be informed on a confidential basis in all cases

### **Authority of the ERN ITHACA Board**

Nothing in this document removes the rights of the ERN ITHACA Network Board to interpret and/or amend its content in the best interest of the Network or to comply with relevant directives or guidance from the European Commission.

### **Participation in Working groups**

The activity in ERN ITHACA is organized in Working Group and Task Forces and are evolving over time according to all projects and needs of ITHACA. <https://ern-ithaca.eu/about-us/workgroups/>

The nominated ePAG advocates (see table below) represent the voice of the patients within ERN ITHACA in each Working group (WG) and they contribute actively to the regular activities and meetings with their specific WG. They collaborate with the ERN clinicians and the research leads on a set of different topics defined in the work plan and they will provide feedback on the progress of the activities to the members of the ERN ITHACA Patient Council during the bi-monthly calls and to their wider patient community.

It is the policy of the ERN ITHACA PAB to have no more than two ePAG Advocates representing each of the ERN ITHACA Working group. This policy does not preclude additional patient representatives participating in particular working groups or, by invitation of the ERN Coordinator, to have access to the ERN ITHACA information and/or to attend the ERN ITHACA Annual Meeting

In the appendix **(ANNEX 2024-1)** you will find the different activities and workgroups in which the patients representatives are involved.

## Attendance to meetings and frequency of the calls

The ERN ITHACA PAB will meet regularly by video conference call. The meeting dates will be decided on a case-by-case basis and the meetings will be held in English.

- **PAB** - meet on a bi-monthly basis with the ERN PM to discuss on the operational activities of the ERN ITHACA and any other relevant topics (internal facing group).
- **SC** - meet on a bi-monthly basis with all the members to engage them and report back on the discussion with the ERN Coordinator team
- **Patient Workshop** - All ePAG Advocates will be invited to attend 1 annual meeting called "Patient Workshop" join to the ERN ITHACA Board per year. ITHACA will cover the costs of attending (unless there are financial constraints). Each of the five people representing the PAB, Steering Committee can nominate another person from the ERN ITHACA PAB if he/she is unable to attend a meeting/call.

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## List of Acronyms

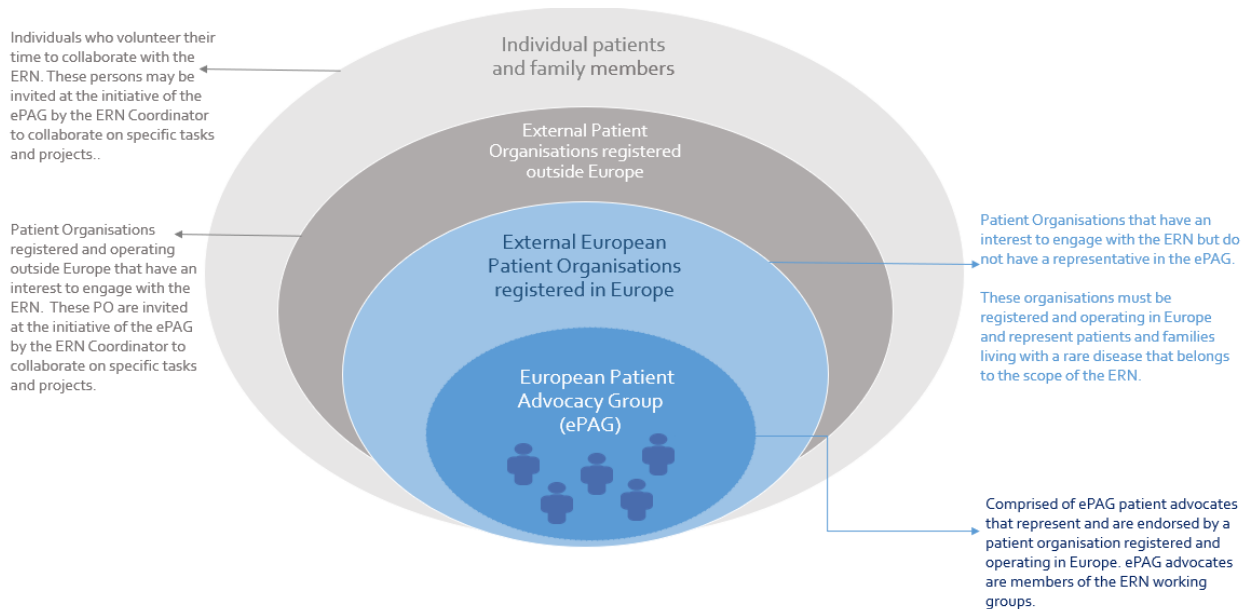
- CPMS - Clinical Patient Management System
- EC - European Commission
- ePAG - European Patient Advocacy group
- ERN - European Reference Network
- ERN ITHACA - European Reference Network on congenital malformations and rare intellectual disability
- EURORDIS - European Organisation of Rares Diseases
- HCPs - Health Care Providers
- ILIAD - International Library of Intellectual and Anomalies of Development - Ithaca registry
- ITHACA - Intellectual disability, TeleHealth, Autism and Congenital Anomalies
- MS - Member States
- NDDs - NeuroDevelopmental Disorders
- PAB - Patients Advisory Board is known also as Patients' Council
- SC - Steering Committee (5 elected ePAGs from the PAB)
- PC - Patient's community - All ePAGs , including those not selected but involved in some specific activities (ex: Guidelines, Registry, Research) Partnership
- PO - Patient Organisation
- QoL - Quality of Life
- ToR - Terms of Reference -Statute defining the rules for patient participation in ITHACA
- WG - Working Group
- WP - Work Package

## ePags Information on ERN ITHACA WEB SITE

- <https://ern-ithaca.eu/for-patients-and-families/ern-ithaca-patients-representatives/>
- <https://ern-ithaca.eu/wp-content/uploads/2022/07/Flyer-PC.pdf>

## EURORDIS Rare diseases Europe, ePAG Resources

European Patient Advocacy Group and relationships with the wider European and International Patient Community and individuals patients and family members.



<https://www.eurordis.org/epag-resources>

- . Guide to developing a Patient Journey (March 2024)
- . Patient Partnership Framework for ERNs (November 2023)
- . Together4RD position statement on collaboration between European reference networks and industry
- . Together4RD position statement on collaboration between European reference networks and industry (September 2023)
- . Patient-clinician reflection sessions (February 2023)