

This survey is intended for individuals holding leadership or decision-making roles within patient organisations (e.g., board members, directors, coordinators).

Please ensure that the questionnaire is completed by **one person representing the organisation**.

Dear Sir or Madam,

My name is Jan Domaradzki, and I am a researcher at Poznan University of Medical Sciences, where I conduct research on the functioning of patient organisations operating in the field of rare diseases in Poland.

This study is a continuation of a previous project examining strategies for building and sustaining communities of caregivers of people with rare diseases, as well as the role these communities play in providing emotional, informational, and practical support. That study was conducted in collaboration with patient associations and foundations.

In response to the suggestions and needs expressed by patient organisations, I am currently conducting a new study aimed at examining the involvement of rare disease patient organisations in research activities, including their participation in scientific projects, collaboration with researchers and healthcare professionals, contributions to patient registries and clinical studies, as well as the opportunities and challenges associated with research engagement.

I would like to kindly invite you to participate in this study by completing the questionnaire, which takes approximately 10-15 minutes.

Participation is entirely voluntary, and the survey is fully anonymous. No personal data is collected, and all information will be used solely for scientific purposes.

The project has no commercial purpose.

If you are unsure how to answer a particular question, please select the response that you consider most appropriate.

Thank you for your time and valuable contribution to this study, which we hope will contribute to meaningful improvements in policy and practice.

Jan Domaradzki, PhD

Health Sociology and Social Pathology Research Unit

Department of Social Sciences and Humanities, Faculty of Health Sciences

Poznan University of Medical Sciences

7 Rokietnicka Street, 60-806 Poznań, Poland

Tel.: +48 61 854 27 70

E-mail: jandomar@ump.edu.pl

STATEMENT OF THE RESEARCH PARTICIPANT:

I hereby declare that:

1. I was informed by the principal investigator and research coordinator, dr. hab. Jan Domaradzki, about the aims, assumptions, course and method of conducting this study.
2. I know that participation in the study is entirely voluntary, and refusal or withdrawal from participation carries no consequences.
3. I have been informed that the study is anonymous and does not collect any personal or sensitive data.
4. I know that data collected in the study will be processed exclusively for scientific purposes, in accordance with GDPR principles.
5. I have been informed about the manner and rules of data storage, and that access to it will be held solely by the project director.
6. I understand that I may withdraw my consent to participate in the study at any stage, without giving a reason and without any consequences.
7. I know that participation in the study is unpaid and involves no remuneration.
8. I have had the opportunity to ask questions and obtain additional information about the study.
9. I understand all the information presented to me and voluntarily consent to participate in the study.

☐ Yes, I consent to participate in the study

☐ No, I do not consent

QUESTIONNAIRE

1. Name of organisation (*confidential information, used solely for return control; will not be linked to responses. The organisation's name will only be included in a collective acknowledgements list covering all participating organisations.*)

2. What is your role in the organisation? (*Please select one answer that best describes your role.*)

- ☐ President / Chair
- ☐ Vice-President / Vice-Chair
- ☐ Board member
- ☐ Director / Manager of the organisation
- ☐ Lead coordinator (e.g. operational, programmatic)
- ☐ Person delegated by the board to complete the survey
- ☐ Other managerial role (which?): _____

3. Year the organisation was founded: _____

4. Legal status of the organisation:

- ☐ Foundation
- ☐ Association (unregistered)
- ☐ Registered association (KRS)
- ☐ Umbrella organisation
- ☐ Other

5. Type of diseases covered by the organisation's activities by frequency of occurrence (*more than one answer may be selected*)

- ☐ Rare (< 1:2,000)
- ☐ Ultra-rare (< 1:50,000)
- ☐ Hyper-rare (< 1:1,000,000)

6. Geographical scope of the organisation's activities:

- ☐ Local
- ☐ National
- ☐ International

7. Number of formally enrolled members (*i.e. persons holding statutory membership; does not apply to observers, supporters or members of social media groups*)

- ☐ 1–2 persons
- ☐ 3–5 persons
- ☐ 6–10 persons
- ☐ 11–20 persons
- ☐ 21–50 persons
- ☐ Over 50 persons
- ☐ Not applicable (organisation without statutory membership)

8. Number of persons actively participating in the organisation's activities (e.g. regular support of activities, volunteering, participation in projects, event organisation)

- ☐ 1–2 persons
- ☐ 3–5 persons
- ☐ 6–10 persons
- ☐ 11–20 persons
- ☐ 21–50 persons
- ☐ Over 50 persons

9. Is the organisation a member of, or does it collaborate with, international patient networks? (more than one answer may be selected)

- ☐ We do not collaborate with international networks
- ☐ International patient organisation dedicated to the same disease/disease group (e.g. global or European organisation for a given disease)
- ☐ EURORDIS – Rare Diseases Europe (organisational membership)
- ☐ Participation of an organisation representative in an ePAG (European Patient Advocacy Group)
- ☐ Collaboration with European Reference Networks (ERNs)
- ☐ Collaboration with ORPHANET / Orphanet Poland
- ☐ Rare Disease International (RDI)
- ☐ Global Genes
- ☐ NORD – National Organisation for Rare Disorders (USA)
- ☐ Other international network (which?): _____

10. Annual budget of the organisation:

- ☐ PLN 0 / no budget
- ☐ Below PLN 5,000
- ☐ PLN 5,001–10,000
- ☐ PLN 10,001–50,000
- ☐ PLN 50,001–200,000
- ☐ PLN 200,001–500,000
- ☐ Over PLN 501,000

11. What types of educational activities does your organisation provide? (Please select all that apply.)

- ☐ Educational materials (brochures, guides, online content)
- ☐ Educational workshops/webinars (for patients, families, or other target groups)
- ☐ Individual information and counselling sessions
- ☐ Educational media campaigns
- ☐ Education in schools (for students and/or teaching staff)
- ☐ Education of healthcare professionals
- ☐ Education of public administration and institutional stakeholders
- ☐ Other: _____

12. Who are the main target audiences of your organisation's educational activities? (Please select all that apply.)

- ☐ Patients
- ☐ Parents and caregivers

- ☐ Siblings and other family members
- ☐ Physicians and clinicians
- ☐ Nurses, physiotherapists, and other healthcare professionals
- ☐ Staff of diagnostic centres/centres of expertise
- ☐ Teachers, educators, and school/preschool staff
- ☐ Students/peers
- ☐ Psychologists, educators, and psychosocial support professionals
- ☐ Employees of public institutions (e.g., social welfare centres, family support centres, local government offices)
- ☐ Media professionals and journalists
- ☐ Policymakers and decision-makers
- ☐ General public
- ☐ Other groups: _____

13. Does your organisation collaborate with physicians? (*e.g., through expert consultations, educational activities, patient care, or research projects*)

- ☐ Yes, regularly
- ☐ Yes, occasionally
- ☐ No
- ☐ No, but we would like to

14. How would you rate physicians' collaboration with your organisation overall?

- ☐ Not applicable / no collaboration
- ☐ Very poor
- ☐ Poor
- ☐ Moderate
- ☐ Good
- ☐ Very good

15. Does your organisation collaborate with researchers or academics? (*e.g., through research projects, scientific consultations, data collection, or participant recruitment for studies*)

- ☐ Yes, regularly
- ☐ Yes, occasionally
- ☐ No
- ☐ No, but we would like to

16. How would you rate researchers'/academics' collaboration with your organisation overall?

- ☐ Not applicable / no collaboration
- ☐ Very poor
- ☐ Poor
- ☐ Moderate
- ☐ Good
- ☐ Very good

17. Does your organisation engage in research activities? (*e.g., initiating, co-designing, conducting, or supporting research projects*)

- ☐ Yes, regularly
- ☐ Yes, occasionally
- ☐ No
- ☐ No, but we would like to

18. In what ways does your organisation support the development of research? *(Please select all that apply.)*

- ☐ Not applicable (the organisation does not support research activities)
- ☐ Providing grants or funding for research (e.g., mini-grants, scholarships, funding scientific projects)
- ☐ Advocacy for research (e.g., lobbying for research funding, supporting policies that promote research)
- ☐ Participation in research-related decision-making processes (e.g., strategic consultations, advisory committees, scientific boards)
- ☐ Supporting participants in clinical research (e.g., logistical, psychological, or informational support)
- ☐ Disseminating information about research and research findings (e.g., announcements, webinars, newsletters, translation of materials)
- ☐ Collaboration with research consortia and scientific institutions (e.g., ERNs, universities, research institutes, laboratories)
- ☐ Other research-support activities: _____

19. How would you rate your organisation's overall involvement in research activities?

- ☐ Not applicable – the organisation is not involved in research activities
- ☐ Very low – the organisation is rarely involved in research; only occasional, isolated activities
- ☐ Low – the organisation participates in research sporadically and to a limited extent
- ☐ Moderate – the organisation supports or conducts selected research activities, but not systematically
- ☐ High – the organisation actively supports or co-creates different types of research on an ongoing basis
- ☐ Very high – the organisation leads or co-creates numerous research initiatives and plays a strategic role in research

20. What are the main reasons for your organisation's lack of involvement in research? *(Please select all that apply.)*

- ☐ Not applicable
- ☐ Research is conducted by another organisation or institution
- ☐ Lack of interest from researchers or clinicians
- ☐ The organisation is newly established or still developing its structures
- ☐ Insufficient financial resources
- ☐ Lack of personnel or research-related expertise
- ☐ Research is not currently a priority for the organisation
- ☐ Formal or regulatory barriers (e.g., GDPR requirements, ethics procedures)
- ☐ Too few patients/recruitment difficulties
- ☐ Lack of infrastructure (e.g., IT tools, patient registry, technical support)
- ☐ Lack of time or excessive workload among staff/volunteers
- ☐ Other reasons: _____

21. In which types of research has your organisation been involved? *(Please select all types of research that your organisation initiates, co-designs, supports, or participates in.)*

☐ Not applicable – the organisation has never been involved in any research

A. NON-CLINICAL RESEARCH

☐ Patient registry (maintained by the organisation or in collaboration with other entities)

☐ Biobank (involvement in its establishment or collaboration in biological sample storage)

☐ Natural history studies

☐ Social research (e.g., assessment of patient and family needs, experiences, quality of life of patients and/or caregivers)

☐ Collection of patient experience and outcome data (PREMs/PROMs)

☐ Epidemiological research (e.g., population size, disease prevalence)

☐ Academic research (e.g., in collaboration with universities or research institutes)

☐ Other non-clinical research

B. CLINICAL RESEARCH

☐ Support for recruitment into clinical trials

☐ Support for clinical trial participants (e.g., logistical or informational support)

☐ Participation in clinical trial design (e.g., consultations, selection of endpoints)

☐ Participation in advisory committees related to clinical trials

☐ Initiation of clinical research projects

☐ Collaboration with pharmaceutical companies in clinical research

☐ Other clinical research activities

C. TRANSLATIONAL / LABORATORY RESEARCH

☐ Translational research (e.g., cellular, molecular, or animal model studies)

☐ Preclinical research projects

☐ Support for research laboratories/funding of basic science research

☐ Other translational research projects

22. Which of the following types of research does your organisation initiate (i.e., acts as the originator of the idea, project leader, or formally launches the study)? *(Please select all that apply.)*

☐ Not applicable (the organisation does not initiate research)

☐ Patient registry

☐ Biobank

☐ Natural history studies

☐ Social research (e.g., assessment of patient and family needs, experiences, quality of life of patients and/or caregivers)

☐ Clinical research

☐ Translational research projects

☐ Preclinical research projects

☐ Other: _____

23. Which of the following research-related activities does your organisation support or co-create? (i.e., participates in research implementation but is not the initiator)? *(Please select all that apply.)*

☐ Not applicable (the organisation does not support or co-create research)

☐ Recruitment of participants for clinical studies/trials

☐ Support for research participants (e.g., informational or logistical support)

- ☐ Consultations with researchers (e.g., patient perspectives, feedback)
- ☐ Participation in advisory committees or expert groups
- ☐ Collaboration with pharmaceutical or biotechnology companies in the context of research
- ☐ Promotion of research (e.g., informing patients, recruitment campaigns)
- ☐ Data collection for studies conducted by other organisations or institutions
- ☐ Surveys or questionnaire-based studies conducted in collaboration with researchers
- ☐ Other forms of research support or co-creation: _____

24. What challenges does your organisation face in providing information and education about the disease? *(Please select all that apply.)*

- ☐ Lack of access to subject-matter experts
- ☐ Lack of source materials available in Polish
- ☐ Difficulty translating complex medical information into language understandable to patients and families
- ☐ Conflicting information provided by different specialists
- ☐ Difficulty keeping information up to date (rapid developments in diagnosis and treatment)
- ☐ Lack of standards and guidelines for communicating about the disease
- ☐ Insufficient financial resources to develop educational materials
- ☐ Limited access to media or low visibility of educational activities
- ☐ Lack of time and overload among those involved in educational activities
- ☐ Insufficient human resources (lack of staff/volunteers to implement activities)
- ☐ Lack of interest from educational institutions in collaboration
- ☐ Concerns of schools and institutions regarding legal liability
- ☐ Reluctance among some parents of unaffected children to discuss the disease
- ☐ Limited digital literacy among some target groups
- ☐ Competition with fundraising campaigns and social media influencers
- ☐ Misinformation on the internet and social media
- ☐ Other barriers: _____

25. What challenges does your organisation face in collaborating with physicians? *(Please select all that apply.)*

- ☐ Lack of physicians' knowledge about the disease
- ☐ Lack of time for physicians to collaborate with patient organisations
- ☐ Physicians' workload and staff shortages
- ☐ Lack of clearly defined pathways for contacting physicians
- ☐ Limited awareness among physicians of the role and competencies of patient organisations
- ☐ Low willingness among physicians to engage with organisations
- ☐ System-level problems (e.g., lack of care coordination)
- ☐ Physicians' concerns regarding legal or administrative issues (e.g., GDPR, liability)
- ☐ Organisations' concerns regarding GDPR and patient data processing
- ☐ Lack of trust or concerns about collaboration among physicians
- ☐ Cultural barriers within the medical profession (e.g., hierarchy, lack of partnership)
- ☐ Concerns about conflicts of interest
- ☐ Lack of formalised collaboration procedures
- ☐ Organisational difficulties (e.g., scheduling, overloaded calendars)

- ☐ Differences in communication styles
- ☐ Other challenges: _____

26. What challenges does your organisation face in collaborating with researchers or academics? (Please select all that apply.)

- ☐ Lack of researchers' knowledge about the rare disease
- ☐ Lack of interest among researchers in collaborating with patient organisations
- ☐ Lack of time for researchers to collaborate outside funded research projects
- ☐ Lack of clearly defined models and principles of collaboration
- ☐ Limited awareness among researchers of the role and potential of patient organisations
- ☐ Asymmetrical relationships (the organisation is treated as a "data source" rather than a partner)
- ☐ Communication difficulties (scientific language vs. practical/patient-oriented language)
- ☐ Limited influence of the organisation on research goals and study design
- ☐ Lack of financial resources to support organisational involvement in research projects
- ☐ Expectations of financial compensation from researchers for participating in collaborative activities
- ☐ Formal and ethical barriers (procedures, approvals, ethics committees)
- ☐ Researchers' concerns regarding conflicts of interest
- ☐ Organisations' concerns regarding the use of patient data
- ☐ Lack of feedback about research findings
- ☐ Differences in expectations regarding the outcomes of collaboration
- ☐ Other challenges: _____

Comments and additional remarks (Optional response)

If you would like to share any additional reflections, comments regarding the questionnaire, or other important observations concerning the involvement of patient organisations in research and scientific collaboration, please use the space below.

Thank you for your time and participation in this study.

Your responses are extremely valuable and will contribute to a better understanding of the role and involvement of rare disease patient organisations in research activities in Poland.