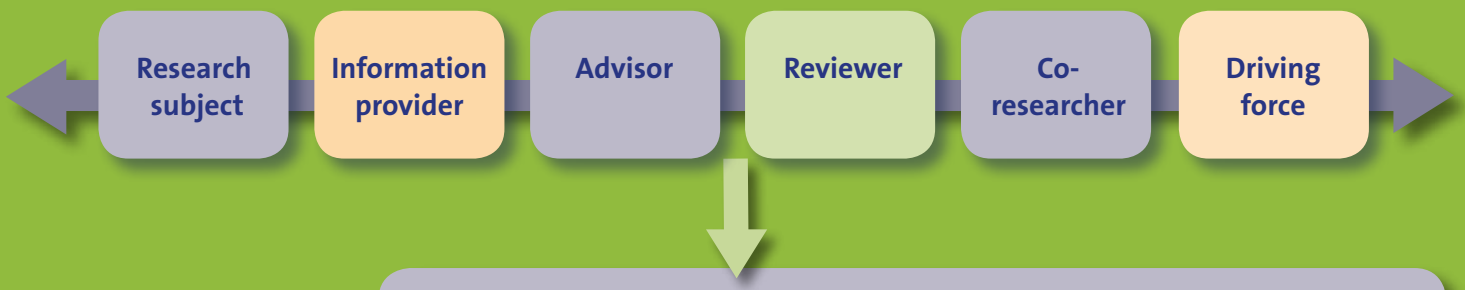
Patient Involvement
in Rare Disease Clinical Research

asterix

Why should patients be involved?

Patients and parents of a child with a rare or ultra-rare disease can make an important contribution to the trial becoming a success. As patients manage their disease on a day to day basis, they have more knowledge on which aspects of their disease should have priority for further investigation by researchers. Patients are able to offer a unique perspective based on this experiential knowledge. Patients should be involved in the choice of outcome measures in a trial and the composition of patient information. They better understand the burden of trial participation for them and their families, and can help researchers design a more accessible trial.
In this leaflet, we will show how patients can be involved in research, and which tools may be used.



Roles of Patients in clinical research

This diagram represents the different roles that patient representatives play in the clinical trial process: a research subject, an information provider, an advisor, a reviewer, a co-researcher and a driving force. This diagram was developed in the EU project patient partner, based on the Participation ladder of Arnstein, a vertical ladder. **All roles are necessary and important and there is no hierarchy of one above the other**, thus the ladder was turned. Patients can be involved in clinical trials in various ways: setting the research agenda, design of clinical trials, recruitment and dissemination of the results. The Asterix project studies the design of better clinical trials for small populations.

European collaborations on patient involvement in research

EURORDIS

Eurordis is the alliance of rare disease patient organizations in Europe. Over 700 patient organisations are members of Eurordis, and they represent over 4000 rare diseases. The organization is non-governmental and driven by patients. Eurordis represents the patients' voice on a European level. Eurordis strengthens the capacity of patient advocates and provides training on clinical trials, drug development and regulatory processes in their Summer School. Trained patients are partner of researchers, and are members of various committees at the EMA, such as the Patients' and Consumers' Working Party (PCWP).

EUPATI

EUPATI, short for 'European Patients' Academy on Therapeutic Innovation' offers information and training to patients about healthcare research. EUPATI organises Patient Expert Training Courses for patients. The EUPATI toolbox on Medicines R&D is an information tool in several languages, which patients can use to contribute to Research & Development of new treatments with researchers, policy makers, and the pharmaceutical industry.

The EMA framework for interaction with Patients

The European Medicines agency involves patients in several of her activities. Patients are members of the Management Board and scientific committees; are consulted on disease-specific requests by the committees and working parties; take part in discussions on the development and authorisation of medicines and in the preparation of guidelines. The EMA has developed Training & Resources for patients, like video's or workshops.

Asterix methods: the POWER model

In the ASTERIX project, we have developed a model where patient representatives are involved in the choice of outcomes in the design stage of a trial, the POWER model (Patient participation in Outcome measure WEighing for Rare diseases). Patients and researchers decide together which aspects of a disease are relevant to patients and which outcomes we will measure to evaluate an experimental treatment.

The POWER model has four steps ▶

1. Create the right circumstances for communication between researchers and patient representatives
2. Prepare a consultation, for example with training on research methods or communication skills
3. Consultation, where patient representatives and researchers interact, for example trough focus groups or interviews and where they make decisions on how to progress
4. Follow-up, for example an evaluation with the patient representatives and researchers and agreements how to keep each other up-to-date on the progress of the trial

Possible benefits for patients

When scientists involve patients in the early stages of a trial, the trial may:

- investigate topics that are more relevant to patients
- become more appealing for patients to take part in

When the trial is tailored more towards patients, the efficacy of a drug may be demonstrated in endpoints that are relevant to patients.

Possible downsides

- It may be possible that patient representatives who have been involved in the design stage of a trial cannot take part in the trial themselves, because they do not fit the inclusion criteria. It is important to manage these expectations, as patient representatives often put in a lot of time and effort and hope to find a cure.
- The dedication and time that patient representatives put in research projects is not always acknowledged.

More information

EMA/Partners&Networks/Patients&Consumers
EUPATI: https://www.eupati.eu/
Eurordis: http://www.eurordis.org/content/patient-advocates-involvement
PatientPartner: Guide for Patient Representatives
Participation ladder: Arnstein SR. A ladder of citizen participation. Journal of the American Institute of planners. 1969;35(4):216-24.

There are many more national initiatives on patient involvement in research. However, in this leaflet, we focus only on the European collaborations.

Contact details

ASTERIX - Advances in Small Trials dEsign for Regulatory Innovation and eXcellence *This leaflet is developed together with the Patient Think Tank and is part of the Asterix project, funded by the EU FP-7 program. For more information, see our website: www.asterix-fp7.eu*