

A patients' view on rare disease research

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LESSONS LEARNED

1

Patient representatives prefer equal collaboration with researchers

2

In an equal collaboration, researchers are guided towards designing more relevant trials with useful outcome measures

3

Also, patients may feel more involved in research, might be more willing to participate in it and can be the driving factor behind new research topics



BACKGROUND

In rare diseases, involving patients in health decisions is seen as more and more important. When it comes to health research, patient involvement is not yet standard practice, even though patients may have a strong opinion on what research in their specific disease should look like.

OBJECTIVES

Our main aim was to discover what aspects patients perceive as most important in rare disease research, and what aspects they want to be involved in.

METHODS

We have interviewed 10 rare disease patient representatives in a semi-structured way. All the patients take part in the Patient Think Tank of the FP-7 program ASTERIX.

RESULTS

THEME 1: Communication

- Patients want to be involved in the choice of outcome measures and want the outcome measures to be more disease-specific.
- Patients want researchers to communicate the results of a study to the patients in a way that all patients understand.
- Patients want the results of a trial to be applicable to them.

THEME 2: Patient role

- Patients can be initiators of a research, but they can also be involved in a trial merely for recruitment purposes.
- Patients want to be informed about the trial, and could also be involved in the trial design.
- Even within a patient organization for a particular rare disease, there may be different opinions depending on for instance age, severity or stage of the disease.

- Patients prefer the placebo group in a trial to be as small as possible.
- Patients have experienced that finance is often a problem.
- The transition from experiments in animals to trials in humans in terms of dose and length of the trial is also perceived as problematic.
- Research in children is often difficult, even though the treatment window can for some diseases only be seen in younger age.

- The position of the pharmaceutical industry can become problematic.
- Companies sometimes don't want to share information on the progress of a trial, as they fear that this information may be shared with other companies. However, this is often vital information for patients.
- Patients feel that companies sometimes promise results that they cannot substantiate in the end.

THEME 3: Trial issues

THEME 4: Industry

