POWER model

Patient participation in Outcome measure WEighing for Rare diseases

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Background

In drug trials, it is relevant to ask patients and their caregivers what they hope the effects are of a new medication. The instruments used to measure these effects are of pivotal importance for the result of the trial and the subsequent decision-making. In rare diseases the choice of outcomes is even more important, due to the small numbers and heterogeneity of the patients who are included.

Goal:

We want to develop a model to involve patients in the decisions about relative weights of outcomes in the trial design stage.

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What do we want from you?

Please give us your input and ideas about general methods for involving patients and their caregivers in the choice and appraisal of outcomes.

STAKEHOLDERS THAT NEED TO BE INVOLVED

- Patients/ caregivers
- Academic Researchers
- Regulators

STEPS THAT NEED TO BE TAKEN

- STEP 1: Find relevant outcome measures
  How do we obtain this information?
- STEP 2: Contact patient representatives
  How & when should patients be approached?
- STEP 3: Ask patients which outcomes are most important to them
  How should patients be involved?
- STEP 4: Use the chosen outcome measures in research
  What should the follow-up of the study look like?

What have we done so far?

Based on literature and meetings with clinical researchers and researchers on patient involvement, we have developed a draft method to involve patients in the determination and weighing of outcome measures. Subsequently, the Dutch Genetic Alliance (VSOP) have organized a meeting with a patient think tank, consisting of a group of patient representatives, to gather feedback on our model and improve it.