Patient Global Impression of Benefit–Risk (PGI-BR): Incorporating Patients' Views of Clinical Benefit–Risk Into Assessment of New Medicines

D Eek • K Halling • E Flood • M Blowfield • O Meyers • M Venerus • J Paty • R Hermann

Background

- Understanding how patients weigh the 'good' (benefit) against the 'bad' (risk) when assessing their treatment experiences has value for patients and those who make decisions about developing, approving, reimbursing and prescribing treatments

Aims

- Learn what patients think about when evaluating their experiences of receiving new medicines
- Develop a patient-reported outcome (PRO) questionnaire to evaluate patients' overall views of the benefit–risk of a new medicine in a clinical trial

Methods

- Trained researchers interviewed 47 patients: 20 patients had cancer, 12 had respiratory conditions, 10 had metabolic conditions and 5 had cardiovascular conditions
- Interviews were grouped to allow researchers to review results and incorporate patient feedback between groups

Concept elicitation interviews (interview groups 1–4)

- **Concept elicitation** uses open-ended questions to allow patients to talk freely, with follow-up probing questions if needed
- Patients' views on benefit–risk of treatment were elicited

Literature review

- Guidance from regulatory (e.g. the FDA) and research bodies
- Existing questionnaires

Interview group 1 (5 patients) → Interview group 2 (8 patients) → Interview group 3 (9 patients) → Interview group 4 (10 patients) → Interview group 5 (5 patients) → Interview group 6 (10 patients)

Next steps

- Further testing
- Determine scoring

Cognitive debriefing interviews (interview groups 2–6)

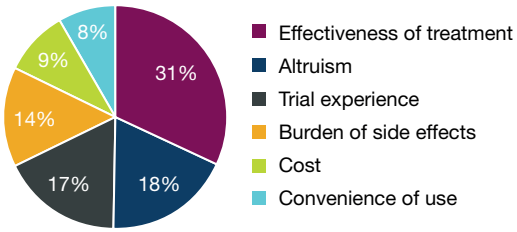
- **Cognitive debriefing** involves pilot-testing the draft questionnaire in the target patient population to assess clarity, relevance and appropriateness of response options
- Patients provided input on draft questions, instructions and response options, and patients' feedback was incorporated into the questionnaire

FDA, Food and Drug Administration.

Key findings

- Patients considered effectiveness of treatment, burden of side effects, convenience of use and overall acceptance of and satisfaction with the treatment when weighing up the benefits against the risks of a new medicine
- Cost, trial experience and helping others ('altruism') were aspects that patients considered that went beyond the medication itself

Relative importance that patients attached to their experiences
Overall acceptance/satisfaction was not part of this assessment



What did patients say?

"I like more definitive, not how I feel, but what the tests show. Does the CT scan show that I'm losing lung tissue at a normal rate?"

"Is it once a day or is it four times a day? It's nearly impossible to remember to take a pill three or four times a day. Frequency is important"

"If the drug helps, great. As long as it doesn't hurt, even better"

"There aren't any good options for my type of cancer…"

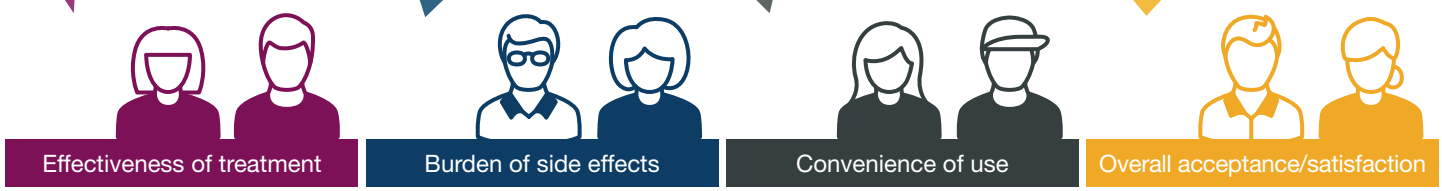
"It has not reduced tumor burden but it has stabilized me and I'm very happy with being stable"

"I think the positive point was getting pain relief"

"The bowel changes could be disruptive to daily life, but never in such a way that it made me question the value of the treatment"

"It's not convenient to take an injection. I've got gel packs that keep everything cold. I can't go anywhere or travel anywhere"

"It's also something that has little side effects…And that is a much better option than taking a pill that causes you to be stable but also takes away your quality of life"



- The final PRO questionnaire, called the Patient Global Impression of Benefit–Risk (PGI-BR), can be applied across medical conditions to assess patients' views of the benefit–risk trade-off of a new medicine in the clinical trial setting
- The PGI-BR will provide the patient voice on whether benefits of treatment outweigh risks, ensuring that patients' views on medicines are considered when making critical decisions about access to medicines

The infographic represents the opinions of the authors. For a full list of declarations, including funding and author disclosure statements, please see the full text online (https://doi.org/10.1007/s40264-021-01079-7)

Adis