The role of patient reported outcomes in cardiovascular trials

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Disclosure

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Introduction

Patient-reported outcomes (PROs), such as symptoms, health-related quality of life (HRQOL), or satisfaction with care, are reported directly by the patient, without interpretation by the clinician or other caregiver.

The outcome can be measured in absolute terms (e.g., severity of a symptom, sign, or state of a disease) or as a change from a previous measure.
Introduction

Because the purpose of a PRO measure is to capture the patient’s experience, an instrument will not be a credible measure without evidence of its usefulness from the target population of patients.

PRO’s provide information that supplements clinical outcomes such as mortality, myocardial infarction, or acute heart failure. Patient-reported outcomes and clinician-reported functional outcomes measure different components of perceived patient well-being.
Health-related quality of life is composed of multiple domains that comprehensively measure the patient’s experience of symptoms, functional status, and psychosocial elements against the patient’s expectations to quantify the extent to which the burden of disease impacts a patient’s quality of life.
Instruments HRQOL

There should be qualitative evidence that the instrument comprehensively measures the concept of interest.

It should demonstrate:

**good reliability**

*reproducibility between populations and within the same subject*

**construct validity**

*instrument results are consistent with results from instruments measuring similar concepts*

**responsiveness to change**
Generic instruments may be more useful for policy-making and to quantify the burden of disease compared with other conditions. Disease-specific measures may be more useful to inform clinical decision-making and patient-centered care.
Application of PROM’s

Patients should be key stake holders in healthcare decisions.

Use of PROM’s from clinical trials to inform clinical care

This information may support discussions between a patient and healthcare provider regarding their health status and the net clinical benefit of a new therapy, or when making a choice about alternative therapies.

Use of PROM’s to support a label claim

Patient-reported outcomes data may be used to support claims that a therapy improves symptoms, functional ability, or HRQOL.

Use of PROM’s to support reimbursement decisions

To evaluate whether coverage of an intervention is warranted.
Interpretation of PROM’s

Determining an evidence-based level of meaningful change in a PRO measure is a major challenge for cardiovascular clinical trials and clinical practice, and consensus has not been reached on what constitutes a clinically meaningful change.

Further research efforts are needed to resolve this major issue.
### Potential approaches

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<thead>
<tr>
<th>Type</th>
<th>Description</th>
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<tbody>
<tr>
<td>Visual analog scale (VAS)</td>
<td>A line of fixed length (usually 100 mm) with words that anchor the scale at the extreme ends and no words describing intermediate positions. Patients are instructed to indicate the place on the line corresponding to their perceived state. The mark’s position is measured as the score.</td>
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<tr>
<td>Anchored or categorized VAS</td>
<td>A VAS that has the addition of one or more intermediate marks positioned along the line with reference terms assigned to each mark to help patients identify the locations between the scale’s ends (e.g., half-way).</td>
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<tr>
<td>Likert scale</td>
<td>An ordered set of discrete terms or statements from which patients are asked to choose the response that best describes their state or experience.</td>
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<tr>
<td>Rating scale</td>
<td>A set of numerical categories from which patients are asked to choose the category that best describes their state or experience. The ends of rating scales are anchored with words but the categories are numbered rather than labeled with words.</td>
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<tr>
<td>Recording of events as they occur</td>
<td>Specific events are recorded as they occur using an event log that can be included in a patient diary or other reporting system (e.g., interactive voice response system).</td>
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<tr>
<td>Pictorial scale</td>
<td>A set of pictures applied to any of the other response option types. Pictorial scales are often used in pediatric questionnaires but also have been used for patients with cognitive impairments and for patients who are otherwise unable to speak or write.</td>
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<tr>
<td>Checklist</td>
<td>Checklists provide a simple choice between a limited set of options, such as Yes, No, and Don’t know. Some checklists ask patients to place a mark in a space if the statement in the item is true. Checklists are reviewed for completeness and nonredundancy.</td>
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### Challenges

Incorporating PROs into composite endpoints introduces unique analytic challenges.
Conclusion

Patient-reported outcomes measure key aspects of disease burden, but they have received limited attention so far.

Cardiovascular clinical research should shift its focus to more broadly include PROs when evaluating the efficacy of therapeutic interventions. Patient-reported outcomes assessments should be scientifically rigorous so that the data can be confidently applied to evidence-based decision-making among all stakeholders.
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