

Importance of patient reported outcomes in cardiovascular clinical trials

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Patient reported outcomes (PROs) should be comprehensively included in cardiovascular clinical trials using the best available tools, according to leading cardiologists and industry representatives in the Cardiovascular Round Table (CRT). All ESC guideline committees will now systematically consider PROs when making recommendations.

The CRT is an independent forum established by the European Society of Cardiology (ESC) and comprised of cardiologists and representatives of the pharmaceutical, device and equipment industries. The group's views are published today in *European Heart Journal*.

Professor Stefan D. Anker, lead author, said: "PROs inform about important (non-survival) aspects of life that are important to patients. They are needed for health technology assessments and reimbursement decisions. But until now PROs have not been routinely evaluated in cardiovascular [clinical trials](#)."

He added: "Our paper outlines the value of including PROs in clinical trials and how to make their assessment scientifically rigorous. The ESC Board has now committed to ensuring that PROs are considered by all ESC guideline task forces."

PROs include symptoms, health-related quality of life (HRQoL) and adherence to therapy. PRO data can be used to inform routine clinical care, particularly when weighing up the risks and benefits of alternative therapies; support a label claim that a therapy improves symptoms,

functional ability or HRQoL; and to support reimbursement decisions, which may consider the effect of a therapy on well-being, functional status or productivity, in addition to the primary clinical endpoint.

The authors argue that "integrating PRO data into comprehensive efficacy evaluations will ultimately improve the quality of care for patients across the spectrum of [cardiovascular disease](#)". PRO measures should be assessed in "at least a subset of patients enrolled in cardiovascular mega-trials", they state.

Trialists who use PROs must ensure that they apply the same scientific rigour as they do with other endpoints. The PRO hypothesis should be stated in the protocol, along with a clear statement of the key PRO that will be analysed. PRO data should be recorded by the patient whenever possible. Statistical power should be allocated to the PRO endpoint if it is a primary, co-primary, or key secondary endpoint.

Professor Anker said: "PROs are subject to patient and physician bias and therefore designing studies so that they are bias free is important. Double-blind studies are the key to achieving this."

Study procedures should be in place to minimise missing data and reasons for missing data should be captured. As the authors point out, "a treatment that prolongs life may perform worse on PRO outcomes than a treatment without a survival benefit because the patients survive to report PROs but still experience disease progression that results in recurrent events or multiple hospitalisations".

Interpretation of PRO data in cardiovascular clinical trials remains challenging because there is no consensus in many cases on what constitutes a clinically meaningful change. "Further research efforts are needed to resolve this major issue," state the authors. They add: "Until a common approach to such analyses has been adopted, we suggest that the

analysis plan for PROs should be discussed with regulatory agencies and with stakeholders (patients, PRO experts) early in the trial planning process."

The paper outlines actions the ESC will take to increase the prominence of PROs in cardiovascular research and the translation of findings to clinical practice. Some of these are: establishing PROs as a factor to be considered by all ESC guideline task forces; including well-designed studies with PRO assessments in prominent sessions of international congresses; encouraging the incorporation of appropriate PRO measures in all pivotal cardiovascular trials and registries; increasing funding opportunities for PRO methodologic research; and emphasising the publication of research on PRO instruments and PRO endpoints.

Professor Anker concluded: "The valid assessment of PROs in cardiovascular clinical trials should become usual practice and we look forward to the inclusion of PRO data in all ESC guideline discussions. These changes will undoubtedly help to improve the quality of care for patients with cardiovascular disease."

More information: Anker SD, Agewall S, Borggrefe M, Calvert M, Caro JJ, Cowie MR, Ford I, Paty JA, Riley JP, Swedberg K, Tavazzi L, Wiklund I, Kirchhof P. The importance of patient reported outcomes: a call for their comprehensive integration in cardiovascular clinical trials. *European Heart Journal*. 2014; DOI: [10.1093/eurheartj/ehu](https://doi.org/10.1093/eurheartj/ehu)

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