

# Medical Devices

## Patient-reported outcomes for medical devices

The good news first: Patient-reported outcomes (PRO) are basically the same in both the medical device and the pharmaceutical sectors. There are the general quality of life tools such as the EQ-5D<sup>1</sup> or the Short-Form Survey questionnaires (e.g. SF-12, SF-36),<sup>2</sup> and there are tools that target specific disease areas, such as the Kansas City Cardiomyopathy Questionnaire (KCCQ)<sup>3</sup> and the Oswestry Disability Index (ODI).<sup>4</sup>

The most commonly used PRO in the medical device sector is the EQ-5D questionnaire because it is easy to use, comprising five questions and a visual analogue scale. It is one of the cheapest questionnaires (yes, cost plays a role in medical devices, particularly if you want to convince somebody to use PRO) and can be used to determine quality-adjusted life years (QALY).

### Use of patient-reported outcomes

PRO are important tools to measure patients' experience with medical devices and the effects of treatment. PRO complement clinical and physiological information and can be used as clinical and physiological endpoints. For instance, in trials with rare complications, quantifying a patient's health status may be a critical requirement for assessing treatment benefits.<sup>5,6</sup> Furthermore, PRO are relevant as they represent the patient's voice.<sup>6</sup>

It goes without saying that the quality of life after a procedure is important; it is not enough to survive a treatment only to be left with intolerable symptoms. For example, it is undesirable to have a successful procedure only to suffer a disabling stroke. On the other hand, a therapy might not increase survival but might improve the quality of life. For instance, in trials in peripheral artery disease or of orthopaedic devices, pain reduction is highly relevant and can be measured with PRO, e.g. Wong-Baker Faces® Pain Rating Scale<sup>7</sup> or the Visual Analogue Scale (VAS).<sup>6</sup>

Measuring quality of life in combination with survival is the concept of QALYs. QALYs are relevant when calculating the incremental cost-effectiveness ratio (ICER).<sup>8</sup> The ICER is

calculated as the difference in costs between two possible interventions divided by the difference in their effect. A common application is in cost-utility analysis. Having set a threshold for cost-per-QALY, it can be used to determine which interventions to adopt. Thresholds vary among countries, but in the UK, NICE typically has a threshold of between £20,000 and £30,000 per QALY.<sup>9,10</sup>

### Relevance of patient-reported outcomes

PRO have been used increasingly over the years<sup>6</sup> and it is expected that their relevance will continue to rise. New consensus documents on clinical trial endpoints now recommend use of quality of life endpoints: endpoint definitions now include PRO in coronary intervention trials<sup>5</sup> and transcatheter mitral valve trials, where improvement in quality-of-life, e.g. KCCQ improvement by  $\geq 10$ , is part of the patient success endpoint.<sup>11</sup>

This summary is intended to give a broad overview of the use of PRO in the medical device sector. In a nutshell, PRO are relevant and are used in the same way in both the medical device and pharmaceutical industries; therefore medical device writers are encouraged to read the full issue of *Medical Writing* on PRO. Happy reading!

### References

1. EQ-5D [cited 2018 Aug 04]. Available from <https://euroqol.org/>
2. 36-Item Short Form Survey (SF-36) [cited 2018 Aug 04]. Available from <http://cvoutcomes.org/pages/3214> [https://www.rand.org/health/surveys\\_tools/mos/36-item-short-form.html](https://www.rand.org/health/surveys_tools/mos/36-item-short-form.html).
3. CVO Cardiovascular Outcomes Inc. The Kansas City Cardiomyopathy Questionnaire [cited 2018 Aug 04]. Available from <https://cvoutcomes.org/pages/3214>.
4. Fairbank JC1, Pynsent PB. The Oswestry Disability Index. *Spine (Phila Pa 1976)*. 2000 Nov 15;25(22):2940-52; discussion 2952.
5. Garcia-Garcia HM, McFadden EP, Farb A,

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et al. Standardized End Point Definitions for Coronary Intervention Trials: The Academic Research Consortium-2 Consensus Document. *Circulation*. 2018 Jun 12;137(24):2635--50. doi: 10.1161/CIRCULATIONAHA.117.029289.

6. Guidance for Industry. Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labelling Claims [cited 2018 Aug 04]. Available from <https://www.fda.gov/downloads/drugs/guidances/ucm193282.pdf>.
7. Wong Baker Faces [cited 2018 Aug 04]. Available from <http://wongbakerfaces.org/>.
8. Wikipedia. Incremental cost-effectiveness ratio [cited 2018 Aug 04]. Available from [https://en.wikipedia.org/wiki/Incremental\\_cost-effectiveness\\_ratio](https://en.wikipedia.org/wiki/Incremental_cost-effectiveness_ratio).
9. Brecker S, Mealing S, Padhiar A, et al. Cost-utility of transcatheter aortic valve implantation for inoperable patients with severe aortic stenosis treated by medical management: a UK cost-utility analysis based on patient-level data from the ADVANCE study. *Open Heart*. 2014 Oct 23;1(1):e000155. doi: 10.1136/openhrt-2014-000155. eCollection 2014.
10. NICE blog. Carrying NICE over the threshold [cited 2018 Aug 04]. Available at <https://www.nice.org.uk/news/blog/carrying-nice-over-the-threshold>.
11. Stone GW, Adams DH, Abraham WT, et al. Clinical Trial design principles and endpoint definitions for transcatheter mitral valve repair and replacement: Part 2: Endpoint definitions: A consensus document from the Mitral Valve Academic Research Consortium. *J Am Coll Cardiol*. 2015 Jul 21;66(3):308-21.

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