Patient-Reported Outcomes and Continuous Glucose Monitoring: Can We Do Better With Artificial Pancreas Devices?

Katharine D. Barnard,[1] Thomas Kubiak,[2] Norbert Hermanns,[3] and Lutz Heinemann[4]

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Patient-reported outcomes (PROs) assess a person's experience, feelings, and thoughts about both their condition and its treatment. PROs are able to contribute to a benefit assessment of new medical products by introducing the patients' subjective evaluation of medical products into the evaluation process. Thus, PROs are also the cornerstone of medical product development for understanding patients' perceptions on medical products and/or its benefit assessment.

In the past 15 years, PROs were also evaluated in most clinical trials performed with continuous glucose monitoring systems. However, in its recent evaluation of such trials, the German Institute for Quality and Efficiency in Health Care (IQWiG)—which has to provide an evidence-based review of the benefits and risks that is key for reimbursement—regarded the quality of PROs in the publications evaluated as quite low. Major criticisms were the huge heterogeneity of PROs used, making comparisons across clinical trials almost impossible. Generic health-related quality-of-life instruments were used with diabetes-specific and device-specific questionnaires. Instruments assessing PROs on these different levels have different sensitivities for assessing the benefits or adverse effects of medical products, which translates into a different statistical power to measure the impact on the subjective evaluation of medical products (1). As clinical trials are usually powered to reach a certain medical outcome (e.g., glycemic control as primary end point), which usually has greater effect sizes than the impact of medical products on PROs, the results on PROs were mostly statistically insignificant. Although acknowledging these issues regarding PROs, the IQWiG disregarded the information based on PROs for their benefit assessment. Thus, all the efforts to evaluate PROs in such trials were more or less useless when it came to such an important aspect such as a reimbursement decision.

As we cannot change the past, we would like to draw attention to the fact that we should not repeat this mistake in another area of research: the development of artificial pancreas (AP) systems. This has made tremendous progress in the past years. A number of scientists and companies are performing clinical trials—also under at-home conditions for longer periods of time—with different AP systems to assess treatment benefits and risks of their usage. Evaluation of PROs is critical to develop AP systems that are used in the real world by the patients. Therefore, we believe that clarity and a certain amount of harmonization are required about which PROs are used in AP trials, by considering the above-mentioned problems.

We suggest establishing a working group in which members of the respective research teams work collaboratively to achieve consensus about suitable PRO measures. These have to be adjusted to the relevant domains associated with AP use. This will hopefully assist regulatory authorities in their decision making about market approval and reimbursement. The data provided by PROs will also help clinicians to address barriers of AP uptake and to support continued use of such devices.

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[1]*Human Development and Health Academic Unit and Clinical and Experimental Sciences Academic Unit, Faculty of Medicine, University of Southampton, Southampton, U.K.*
[2]*Health Psychology, Johannes Gutenberg University, Mainz, Germany*
[3]*Diabetes Center Mergentheim, Research Institute Diabetes (FIDAM), Bad Mergentheim, Germany*
[4]*Science & Co., Düsseldorf, Germany*

Corresponding author: Katharine D. Barnard, k.barnard@soton.ac.uk.