Considerations for Maximising the Research Value of Patient-Reported Outcome (PRO) Data Collected in Clinical Care

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Background

- Real-world evidence (RWE) and PROs continue to play an ever-increasing role in drug development, regulatory review, and reimbursement[1–5]
- RWE can be used by decision-makers to better understand the burden of disease and the effectiveness and tolerability of drugs and health technologies in real-world (RW) settings[3–5]
- PROs can provide evidence directly from the patient's perspective and across a wide variety of health concepts,[6] addressing the potential for healthcare providers to underdetect or underestimate symptoms or the severity of outcomes experienced by patients[7]
- PROs are commonly collected in clinical trials[8] and increasingly collected in RW settings (RW-PROs)[9]
  - RW-PROs can be collected prospectively in noninterventional (observational) studies[9]
  - RW-PROs can also be implemented in clinical care settings with data sets from electronic medical records systems that are repurposed to meet a multitude of goals, ranging from enhancing individual patient care to informing value-based reimbursement[10]
- Guidance has recently been established to support the use of PROs in patient care[10]
  - Well-established guidelines are also available for optimising the use of PROs in clinical trials,[11] and similarly, for the collection and interpretation of non-PRO RW data (eg, treatment effectiveness, treatment persistence, healthcare resource utilization)[11–13]
- However, best practices to ensure that RW-PRO data sets are optimally collected for research purposes and as rigorously interpreted as RWE are currently underdeveloped

Objective

- In this conceptual research, we explored the challenges and opportunities for collecting research-quality RW-PRO data and the scope for best-practice guidance

Methods

- A previously published literature review,[9] conducted in January 2021, first identified and examined published recommendations for generation of RW-PRO–specific evidence to support regulation, reimbursement, and health policy
  - The search strategy did not have any restrictions applied on language or publication date
  - The knowledge gained from the literature review informed ongoing discussions between the authors, other academic researchers, and industry experts with interest in RWE and patient-centric research
  - The discussions were convened via virtual meetings and emails and focused on the aspects of best practice and scope for developing further guidance on RW-PRO generation
- Initial discussions enabled the generation of an initial list of considerations, while further discussions allowed iterative refinements for classification within overarching concepts, previously established by the PROTEUS Consortium,[10] for generating research-quality RW-PRO data

Results

- The literature review revealed that the available guidance on RW-PRO generation is fragmented, providing researchers with a limited understanding on how to optimally collect and use RW-PRO[9]
  - Two additional papers were identified after the initial search,[14,15] which confirmed the conclusions from the initial literature review. The main themes from the limited guidance centred around RW-PRO data collection, analysis, and stakeholder collaboration (**Figure 1**)
- The participants of the discussions included 5 academic researchers and 2 industry experts
- These discussions first identified goals for the use of RW-PROs with significant potential for successful secondary use (**Figure 2**)
- Use cases and important considerations for generating valid and reliable research data were also identified (**Figure 3**)

Figure 1. Categories of Recommendations for RW-PROs from the Published Literature[9]

HCP, healthcare provider; PRO, patient-reported outcome; PROM, PRO measure; RW, real-world.

Conclusions

- Setting standards for the secondary use of PRO data collected in routine healthcare settings (ie, RW-PROs) would maximise the benefit of these types of RW data sets
- However, methods for collecting and using RW-PROs are currently underoptimised, and there is no international guidance specific to the use of these RW-PRO data sets in the research context
- This conceptual research, built on a review of the limited existing guidance, has identified some key considerations for the design, implementation, and systems/data management of RW-PROs

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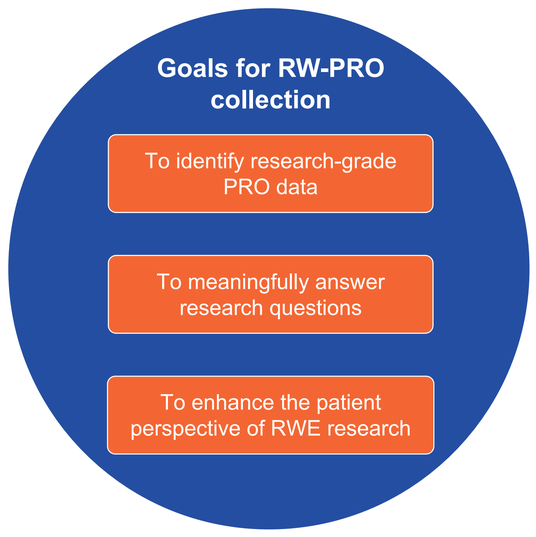
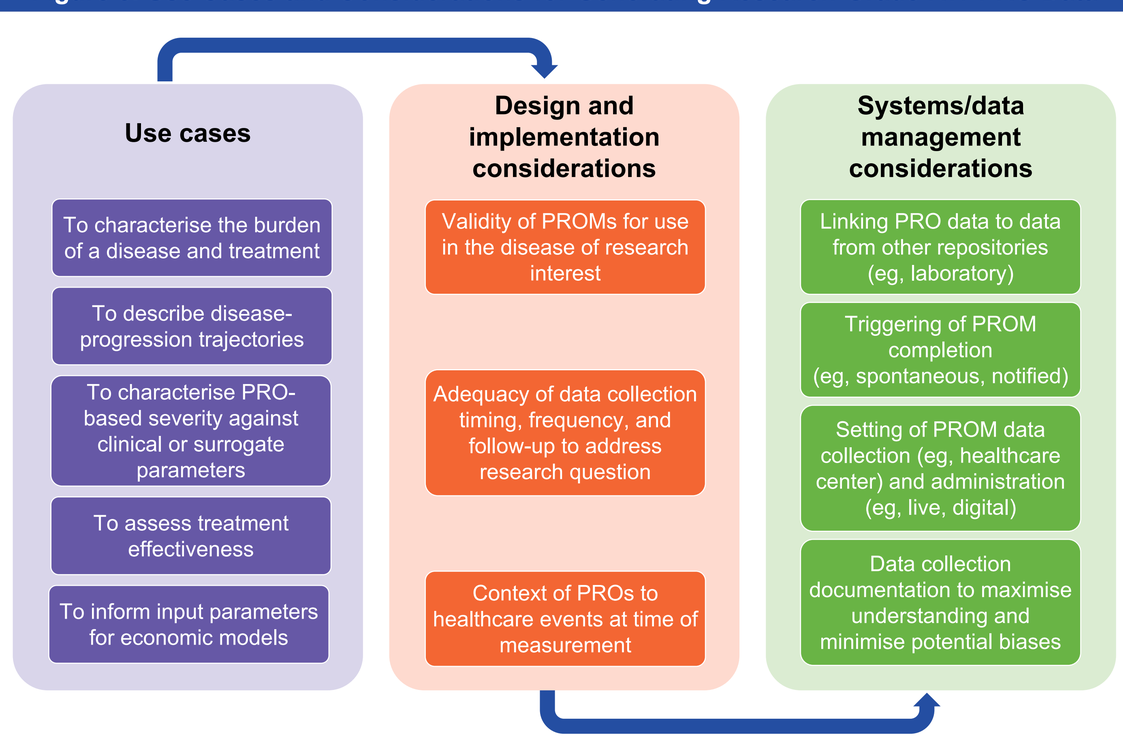
Results *(cont'd)*

Figure 2. Goals for the Use of RW-PROs with Significant Potential for Successful Secondary Use



PRO, patient-reported outcome; RWE, real-world evidence.

Figure 3. Use Cases and Considerations for Generating Research-Grade RW-PRO Data



PRO, patient-reported outcome; PROM, PRO measure; RW, real-world.

Discussion

- Collected in routine healthcare settings, RW-PROs can provide outcomes data from the patient's perspective across a wide variety of health concepts[6]
- With the growth in both PROs and RWE,[1–5] there is increasing need to ensure that RW-PRO data sets are optimised for research
- Setting standards for the secondary use of RW-PRO data would maximise the benefits of these data sets
- The needs of various stakeholder groups (eg, researchers, PRO measure administrators, clinicians) should be considered when developing these standards
  - Moreover, close cooperation with the individuals responsible for implementation of PROs and document data collection, curation, and manipulation is needed to maximise the rigor of collected RW-PRO data sets in order to support research into patient outcomes of healthcare
- In this early research, the discussions with the range of PRO experts helped build a shared understanding on the value of RW-PRO data and the valid and reliable generation of data sets, allowing accurate and meaningful interpretation

Limitations

- A limitation of the present phase of the research is that the discussions were not systematically conducted; however, the open-ended discussions were a starting point on which to build further dialogue and systematically derived guidance

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Conflicts of Interest

Konrad Maruszczyk is the holder of a GSK-funded doctoral research grant.
Linda Nelsen is an employee and shareholder of GSK.
Norah Crossnohere is a Sr. Project Manager (currently paid directly; previously paid through institution) for the PROTEUS Corporation.
Thomas Keeley is an employee and shareholder of GSK.
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