HPR36

Evaluating Patient Preference Information in Regulatory Decision-Making

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Background

- Patient preference information (PPI) is useful for understanding what matters most to patients and the trade-offs they make when making a treatment decision.
- PPI can particularly help understand the benefits and risks of a treatment, what are the most important and least important benefits and risks of a treatment from a patient's perspective, and how a patient thinks about the trade-offs of a treatment when compared to another.
- More recently, there is a growing interest to integrate patient perspectives, including PPI, to inform regulatory decision-making, including benefit-risk assessment (Bywall et al., 2019; FDA, 2021).
- Currently PPI is not required to be included in marketing authorization applications to regulatory bodies, and there is no agreed methodology for PPI. Work is continuing to evaluate the role of PPI data within regulatory review and which PPI methods are most promising at different decision points in the drug lifecycle (Postmus et al., 2016; IMI PREFER, 2022).
- Although there is increased interest in PPI for regulatory decision-making, the scope and type of PPI included in regulatory submissions for medicinal products, particularly drugs and biological treatments, and considered in regulatory reviews and label claims has not previously been synthesized.

Objectives

- The overall objective of this study was to understand the scope and type of PPI that is currently included for regulatory submissions and label claims.
- Key questions included:
  1. What type of PPI data are typically included in regulatory submissions?
  2. What are the methods that are used to capture PPI that are included for regulatory submissions?
  3. How is the PPI data typically analyzed?
  4. What proportion of PPI submitted to regulators are included for a label claim?

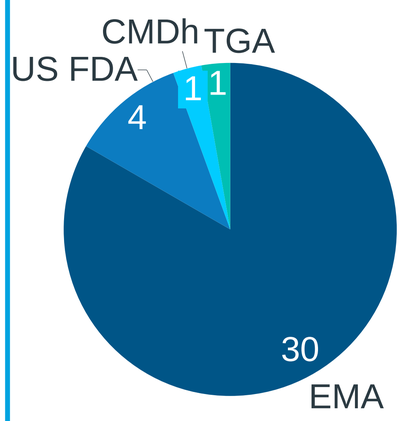
Methods

- Tarius, a global regulatory intelligence database, was used to identify regulatory documents where 'patient preference' was included in the text.
- Drug databases were used to supplement additional information (e.g., drugs@FDAlabel, EMA).
- Documents included regulatory submissions, advisory board meeting reports, dossiers, annual reports and label claims.
- The search was limited to drugs or biological treatments in the past 10 years. Medical devices were excluded. No regulatory body or therapy area was specified.
- Documents were reviewed by 4 IQVIA members, and relevant documents were included for extraction, where reference to 'patient preference' data and results were identified.

Discussion

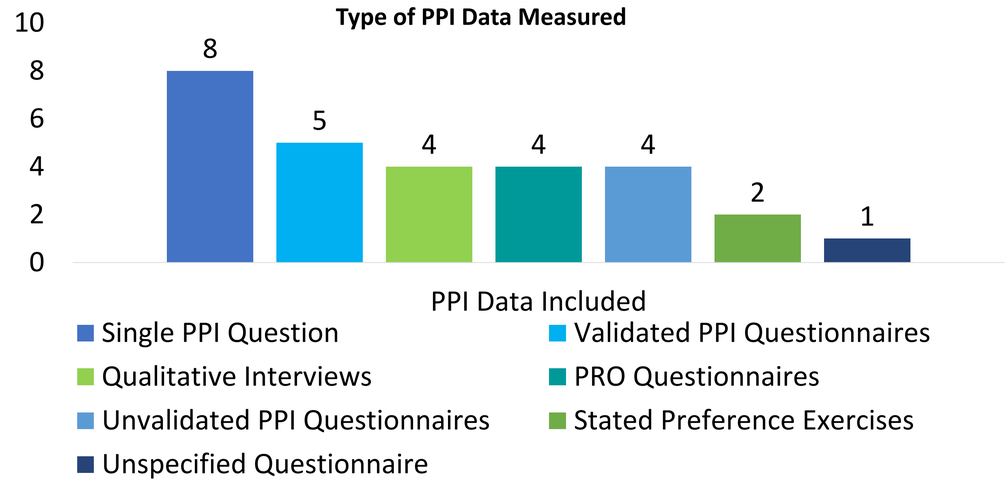
- PPI was captured using various revealed preference methods for regulatory decision-making, with growing support from regulatory bodies highlighting the importance of PPI.
- However, there was minimal stated PPI data for regulatory submissions, with only two documents referencing stated PPI data (DCE and MCDA).
- Regarding evidence for label claims and drug approvals, use of PPI was limited, with all 6 label claims including revealed preference data.
- Key limitations may include limits to the selection of the exclusion criteria and databases, as this could have excluded potentially relevant documents.
- Given the continued interest in PPI from regulatory bodies and the recent positive qualification opinion on the IMI PREFER framework, considerations on including PPI approaches for future submissions and label claims that would be of interest to regulators should be further explored.

Results



Out of 223 documents, 36 referenced PPI in a meaningful way.

- 30 were from EMA, mostly from a European Public Assessment Report (EPAR) (n=17; 57%)
- Others included US FDA, the Coordination Group for Mutual Recognition and De-centralised Procedures - human (CMDh) and the Therapeutic Goods Administration (TGA)
- 28 documents included PPI data (78%). 8 documents did not discuss PPI data but discussed benefits of PPI (22%).

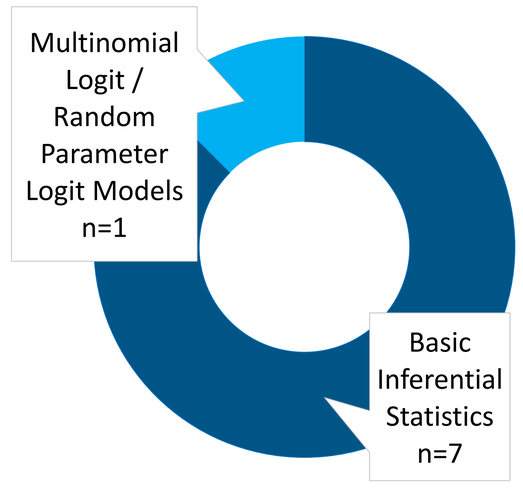


Of the documents that included PPI data (n=28, 78%), 26 studies included revealed preference data. This included:

- A single PPI question (n=8, 28%)
- Validated PPI questionnaires (n=5, 17%)
- Qualitative interviews (n=4, 14%)
- PRO questionnaires (n=4, 14%)
- Unvalidated PPI questionnaires (n=4, 14%)
- An 'unspecified' questionnaire (n=1, 6%)

Two documents referenced stated PPI data (discrete choice experiment (DCE) & multicriteria decision analysis (MCDA); 7%)

Where PPI data was analysed and included / referenced in a regulatory document (n=8, 31%), the majority of these findings were analysed using basic inferential statistics (n=7, 87%). Other analyses included multinomial logit / random parameter logit models (for analysis of stated PPI data) (n=1, 13%)



Of the 28 documents with referenced PPI data, 19 (68%) documents referenced PPI data for regulatory submissions. Of the 19, 6 (32%) referenced PPI data in label claims

Of the 6 label claims, these all included revealed preference data, including interviews (n=2, 33%), validated PPI questionnaires (n=2, 33%), an unvalidated PPI questionnaire (n=1, 17%) and a PRO questionnaire (n=1, 17%)

References

1. Bywall, K. S., Veldwijk, J., Hansson, M. G., & Kihlbom, U. (2019). Patient perspectives on the value of patient preference information in regulatory decision making: a qualitative study in Swedish patients with rheumatoid arthritis. The Patient-Patient-Centered Outcomes Research, 12(3), 297-305.
2. Center for Biologics Evaluation and Research & Center for Drug Evaluation and Research, Guidance for Industry: Benefit-Risk Assessment for New Drug and Biological Products (2021), https://www.fda.gov/media/152544/download; see also 86 Fed. Reg. 54,211 (Notice of Availability).[cited 2022 Oct 18].
3. Postmus, D., Mavris, M., Hillege, H. L., Salmonson, T., Ryll, B., Plate, A., ... & Pignatti, F. (2016). Incorporating patient preferences into drug development and regulatory decision making: results from a quantitative pilot study with cancer patients, carers, and regulators. Clinical Pharmacology & Therapeutics, 99(5), 548-554.
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