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SUPPLEMENTARY INFORMATION

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Harnessing the patient voice in real-world evidence: the essential role of patient-reported outcomes

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Supplementary Table 1 | **Key design considerations for collecting patient-reported outcomes for real-world evidence generation**

Design element	Considerations
Objectives	Clear objectives should be determined as to why PRO data are being collected. This should be informed by existing evidence where available (for example, evidence from trials, meta-analyses or real-world data).
Patient population	Patient population should be defined by inclusion/exclusion criteria.
Instrument selection / tool box	The questionnaire(s) used to collect the data should be relevant and valid for the objectives, the population of interest and meet stakeholder needs. Questionnaires should have been developed with patient input. Language availability, patient acceptability/burden, permissions and fee for use should also be considered.
Frequency of administration	Frequency will depend on stakeholder needs and the study population. Patients with high symptom burden may require more frequent monitoring.
Mode of administration	The data collection plan should outline the permitted modes of administration (for example, paper, telephone, electronic, other).
Data collection method / source data	Consider primary or secondary collection. Feasibility and resources to support data collection, existing registries, electronic health records, requirement for bespoke collection. Specify management strategies to minimise missing data and bias. Methods to ensure quality control. IT infrastructure may be based on existing system or customised / commercial products.
Monitoring of data	Whether PRO data will be monitored and used to directly inform patient care[1,2].
Presentation of results	The data should be analysed and reported appropriately, in accordance with the prospective described objectives and the instrument recommendations, leading to robust conclusions considering potential sources of bias / confounding.
Ethics	The requirement for ethics approvals should be consider early in the proposals for data collection, following engagement with the health authorities
Data ownership and consent	Contact and agree with health authorities / registry owners.
Audit	Mechanisms for on-going audit of data quality etc should be considered.
Privacy	Safe guarding privacy and confidentiality of the data.
Clinician feedback	Consider the need for PRO alerts, mechanism to feedback concerning results with potential to integrate into patient management pathway[3].
Patient feedback	Consider if patients will be able to review PRO results and use these data to actively participate in decisions regarding their care.
Healthcare provider feedback	Potential to integrate into managed access programmes, etc.
Drug manufacturer feedback	Flag to manufacture emerging trends on tolerability and effectiveness in different populations.
Regulatory authority feedback	Support safety reporting, post authorisation marketing commitments, long-term activity data.
Resources	Determine who pays for license fees, training, data collection, clinic time, device costs etc

PRO, patient-reported outcome.

References

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3. Kyte, D., Draper, H. & Calvert, M. Patient-reported outcome alerts: ethical and logistical considerations in clinical trials. *JAMA* **310**, 1229–1230 (2013).