

Capturing the Real-World Patient Perspective in HTA: Expert Patient Insights Lead to Robust Healthcare Cost and Resource Use Inputs for Cost-Effectiveness Model in SMA

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Objective

To elicit realistic healthcare resource use inputs to inform a cost-effectiveness model for a therapy in spinal muscular atrophy by engaging with patient experts throughout the study design and interpretation.

Background

- Spinal muscular atrophy (SMA) is a rare, neuromuscular condition that requires management from a variety of specialists, as well as equipment to support daily living, incurring high levels of healthcare cost and resource use (HCRU).¹
- Realistic estimates of HCRU are needed to inform cost-effectiveness modelling for health technology assessments (HTA) of therapies in this condition; however, previous studies have underestimated the full extent of HCRU by patients with SMA in the UK, due to potential issues including recall bias, small sample sizes and limited consideration of costs outside of secondary care.²
- In order to develop robust HCRU inputs to inform an HTA submission to the National Institute for Health and Care Excellence (NICE; TA11386³), the perspective of patient experts in SMA was sought in the design and interpretation of a modified Delphi panel aiming to gain consensus on these data among specialist healthcare professionals (HCPs) in the UK.

Methods

Summary of Delphi Panel Process

- Two rounds of questionnaires were administered to 34 HCPs, including specialist consultants, nurses and allied health professionals actively managing patients with SMA in the UK (Figure 1).
- In Round 1, participants anonymously completed a questionnaire soliciting estimates of quantities and frequencies of HCRU in UK patients with SMA according to disease severity. Categories of HCRU included healthcare visits, tests and procedures, and specialist equipment.
- The level of consensus for each item was then assessed based on pre-defined thresholds for the spread of individual responses around the median.
- In Round 2, participants were presented with the aggregate results from Round 1; for any items that had failed to reach consensus, they were asked to rate their level of agreement with the median value obtained using a six-point Likert scale.

Scope of Patient Engagement in the Study

- Two patient experts (AT and PT) from the patient advocacy groups SMAUK and TreatSMA were invited to join the steering committee for the Delphi panel; AT is living with SMA, and PT is a parent and caregiver for a child with SMA.
- The patient experts provided direct input on the Round 1 questionnaire to ensure relevance and validity of the HCRU items included, as well as the interpretability of the question wording.
- In addition, they reviewed the results obtained in the Delphi panel to comment on their face validity.

Results

Impact of the Patient Perspective on Study Design

- Input from patient experts in the questionnaire design enabled: a) comprehensive identification of relevant HCRU items; b) recognition of the need to stratify questions by SMA type and age group; and c) insight into complexity of care pathways beyond secondary care (Figure 2).

Impact of the Patient Perspective on Interpretation of Results

- After the two Delphi rounds, consensus between the participating HCPs was reached on >90% of HCRU items included in the Delphi panel (data not shown), allowing comprehensive population of HCRU inputs in the economic model for submission to NICE.
- Validation of the study results from a real-world patient perspective provided confirmatory support for the values obtained.
- The patient experts' review nonetheless revealed a potential underestimation by HCPs of HCRU items commissioned in the community care setting, e.g. through community physiotherapists, providing greater insight into the wider economic burden of SMA.

Conclusion

Involvement of patient experts in the design of the modified Delphi panel enabled HCRU by patients with SMA in the UK to be comprehensively captured for inclusion in the cost-effectiveness model, with feedback from the NICE External Assessment Group noting the "rigorous" questionnaire design and "valuable insights into the economic impact of SMA" provided by the study.³

Inclusion of the patient perspective in real-world studies from start to finish facilitates the generation of robust evidence to inform HTA decision-making.

FIGURE 1

Delphi panel process

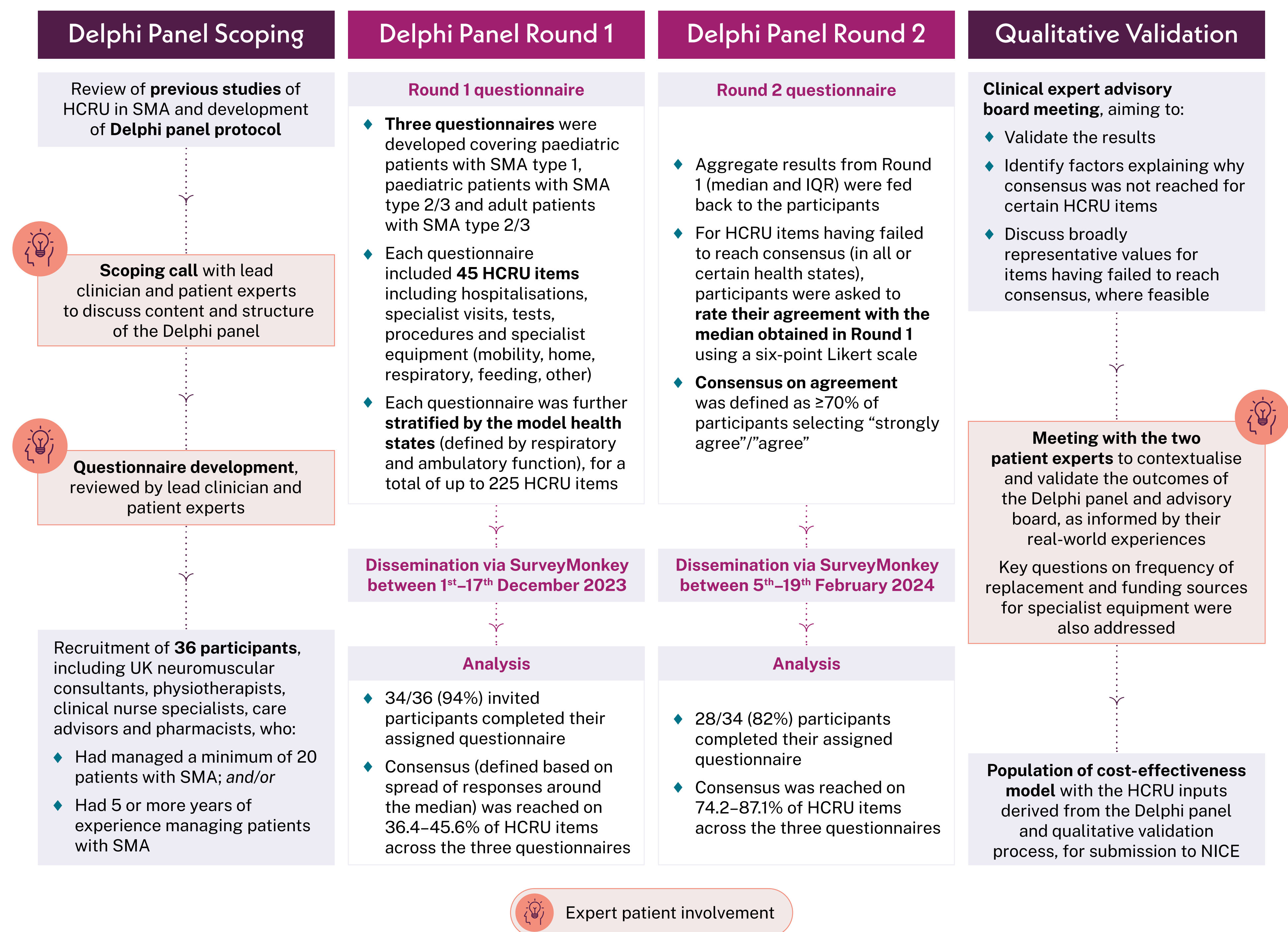
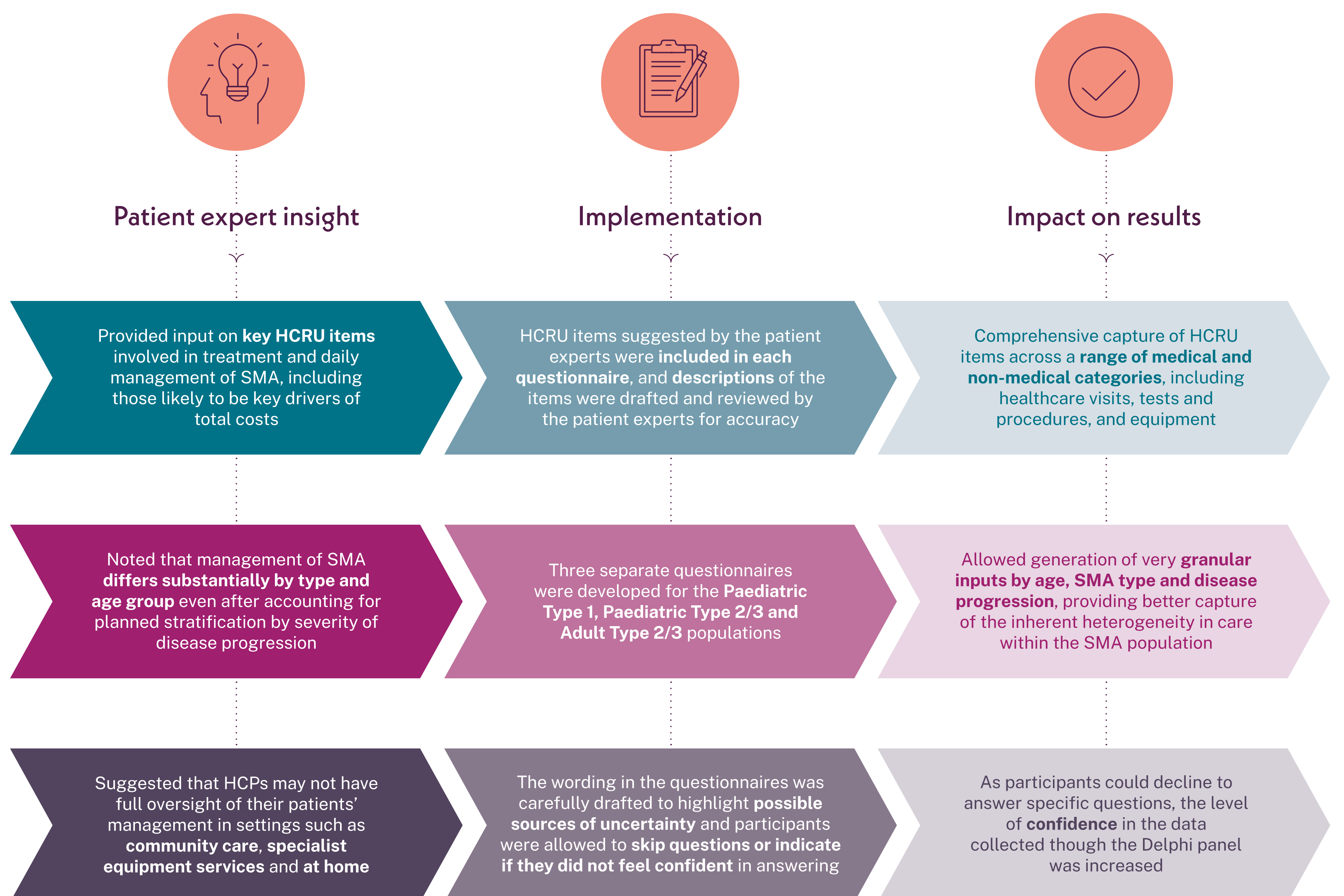


FIGURE 2

Summary of patient expert impact in the design and outcome of the Delphi panel



Abbreviations: HCP: healthcare professional; HCRU: healthcare resource use; IQR: interquartile range; NICE: National Institute for Health and Care Excellence; SMA: spinal muscular atrophy.

References: ¹Paracha N, et al. *Pharmacoeconomics*. 2022;1-28; ²NICE. MAA ID1631. Available at: <https://www.nice.org.uk/guidance/ta755/evidence/appraisal-consultation-committee-papers-pdf-10895910013> [Last accessed: 21 Oct 24]; ³NICE. MTA ID11386. Available at: <https://www.nice.org.uk/guidance/indevelopment/gid-ta11386> [Last accessed: 02 Oct 24]. Acknowledgements: The authors thank the healthcare professionals who took part in this study, as well as Davneet Judge, from Roche, and Jamie Doorbar and Eesha Dinkar, from Costello Medical, for their contributions to the design and analysis of the study. The authors also thank Courtney Gray, Costello Medical, for graphic design assistance. This study was funded by Roche Products Ltd. Disclosures: RB, AA: Employees of Costello Medical, whose support in conducting the study was funded by Roche Products Ltd; JM: Employee of Roche Products Ltd, who funded the study. PT, AT: None.