ISPOR Panel: Patient-Centred Decision Making
Prof Katharine Barnard PhD

Key Points

- Role of PROs and critical appraisal alongside safety and medical outcomes
- How best to inform decision-making and reimbursement
- Measurement and reporting of PROs – the challenges identified in the evidence base
- A uniformed approach by academia, FDA, NIH, Industry and Non-profit funders (Helmsley & JDRF)
Role of PROs in Patient-Centred Decision Making

Expectation by patients that devices are safe, efficacious and reliable

- PROs assess the IMPACT of device/therapy/intervention on lived experience
- PROs robust assessment of acceptability and implementation in everyday life
- PROs rarely effectively evaluated to sufficient rigour for critical appraisal by regulatory approvals bodies

The Problem

PROs crucial to policy decision-making, reimbursement and patient care

BUT

- They are often poorly reported secondary outcomes in clinical trials
- There is a wide range of PROs assessing different aspects of psychosocial functioning and quality of life
- Data is often poorly reported and of poor quality, making synthesis difficult
Example of the Evidence

Systematic literature search of diabetes device studies 2016

- Qualitative research – semi-structured interviews, focus groups
- Quantitative research – questionnaires, pre / post studies, RCTs, controlled trials, observational studies

Types of Outcomes

Psychosocial aspects, from all study designs, including:

- Quality of life / Well-being / treatment satisfaction
- Diabetes distress / hypo fear / depression
- Psychosocial functioning / Change in psychosocial status
- Change in self-management activities eg SMBG, self-exam or increased clinic attendance
Results of Review

- 4554 records identified in initial search
- 723 eligible for full text assessment
- 232 met inclusion criteria and were included in review
  - 137 studies (Artificial pancreas=9; CGM=32; CSII=96)
  - 74 commentaries
  - 16 health economic articles
  - 5 policy papers

Published Literature: Clinical Relevance

- **Insufficient data** to demonstrate direct **causal link** between **psychosocial outcomes and clinical outcomes** reported in the literature

- Improved QoL associated with CSII, however **inconsistent** A1c benefit

- **Mixed psych benefits / downsides** associated with CGM

- Improved psychosocial functioning associated with AP however prototype / **early technology fraught with difficulties** but rapid development of devices means this early data is meaningless in real life
Patient-Reported Facilitators for Device Use

- Reduced mental burden / Improved QoL due to less diabetes-related distress
- Improved glycemic control, fewer highs/lows, reduced variability associated with device
- Reduced risk of long-term complications
- Less user input – less chance for human error
  - Accuracy / reliability (esp in hypo and hyper range)
  - Latest generation devices more acceptable due to technology improvements and functionality
- Size – smaller and more discreet
  - Perceived QoL benefits eg convenience, lifestyle flexibility

Patient-Reported Barriers to Device Use

- Unacceptable tasks: wearing multiple pumps/sensors/devices; too many tubes/wires; devices too large; too many tasks
- Site changes more frequently than every 3 days
- Painful insertions
- No health insurance
- Lack of accuracy and reliability
- Adolescents don't like wearing / using it / visibility of disease state
- Over-reliance on the device, potential to forget basic MDI skills
Views, Attitudes and Experiences of Patient, HCP and Payers

- Patients: tech will improve A1c & QoL, reduce diabetes burden and reduce risk of long-term complications but burden of tech includes alarms, lack of reliability, increased visibility of disease state and cost EXPERIENCE

- HCPs: believe new technologies optimize diabetes control in people with T1D however insufficient time to effectively implement and manage them MEDICAL OUTCOMES

- It is not possible [currently] to pre-judge those who will ‘do best’ on technology (REPOSE trial)

- Payers: no information on payers

Frameworks, Models or Theories Used to Explain Effect and Relevance

- None identified in the review – rarely reported!

- No direct causal links, in any literature on devices between mechanisms of psychosocial factors to clinical outcomes

- Fear of hypoglycaemia and treatment satisfaction were the only PRO measures that correlate with clinical outcomes
What PROs Add to Question of Relevance and Comparative Effectiveness?

- Contribution is mixed. Positive and negative impact on psych functioning widely published for CGM and CSII, less so for AP due to novelty of technology.
- It is widely acknowledged by regulatory approvals bodies such as FDA, NICE etc that PROs are crucial to critical appraisal of health technologies.
- Inconsistent assessment: timing, measures, outcomes and links to clinical outcomes makes it impossible to effectively make sense out of them.
- Consistent, evidence-based theory-driven psychosocial measurement is required (INSPIRE).

Harmonisation of PROs in Clinical Trials - INSPIRE

- INSPIRE patient preference measures used as basis for harmonisation across ALL clinical trials.
- Matrix of psychological constructs with all validated and reliable measures mapped to each construct.
- All clinical triallists are using harmonised measures to ensure consistent, comprehensive and robust PRO assessment.
- Regulatory approvals bodies and payers WILL be able to meaningfully critically appraise PROs alongside safety and efficacy data.
PRO benefits associated with diabetes devices but evidence is mixed for earlier and newer generations (making assessment difficult)

PRO evidence is currently insufficiently robust to be considered equally with clinical outcomes

No direct link to clinical outcomes a result of poor reporting (what is meaningful difference?). PRO often a ‘bolt on’ rather than integral to clinical outcomes assessment

Standardised measures, assessed at standardised timepoints in clinical trials crucial for effective PRO assessment in HTA TARs eg INSPIRE
For further information
contact:

katharinebarnard@bhrltd.com
Tel: 0044 (0)7590 532866
www: bhrltd.com