ISPOR Panel: Patient-Centred Decision Making

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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)

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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

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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

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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

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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

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Results of Review

- 4554 records identified in initial search
- 723 eligible for full text asssessment
- 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

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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

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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

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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

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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

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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

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What PROs Add to Question of Relevance and Comparative Effectiveness?

NOT ENOUGH!

- 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)

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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

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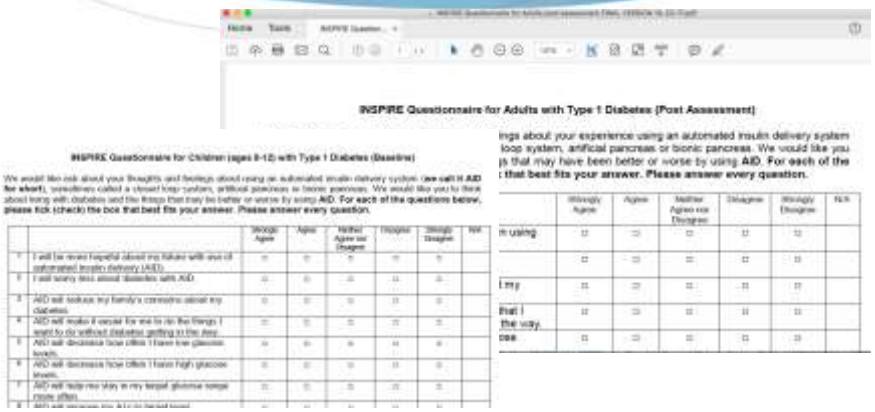
INSPIRE Examples



INSPIRE Questionnaire for Adults with Type 1 Diabetes (Post Assessment)

INSPIRE Questionnaire for Children (ages 8-12) with Type 1 Diabetes (Baseline)

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Conclusions

- 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

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Thanks for Listening

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