



Calvary Centre for
Palliative Care Research



Patient Reported Outcomes for CDG

Dr Liz Forbat

Professor of Palliative Care
Australian Catholic University



CONGENITAL
DISORDERS OF
GLYCOSYLATION
WORLD CONFERENCE

FAMILIES AND PROFESSIONALS

Context

Understanding symptoms and their impact is critical in supporting those affected.



What are Patient Reported Outcome Measures? (PROMs)

- Few for use in paediatrics
- Fewer still for children with rare (non cancer, non diabetic) conditions
- Have to be reliable, valid, easy to complete
- Hard to design something which quantifies accurately

What do you want it to do?

- What do we want from measures:
 - Outcomes? (what effect does x have?)
 - Process? (what is x like?)
 - Communication? (help me summarise x)

Why might families want PROMs?

- To help monitor treatment/ care
- To identify problems
- To describe the severity/frequency of problems

Why might clinicians want PROMs?

- To see if treatment/care is working well
- To adjust treatment/care if outcomes are worsening or not improving
- To compare care with other services (benchmarking)
- Clinical trials are impaired by the lack of specific patient-reported outcome measures (PROMS)

Why is measurement important?

“Only by measuring and understanding self-reported symptoms and function in children and adolescents with incurable cancer can we **adequately address threats to their quality of life and improve symptom control** and supportive care,” Hinds and co-authors conclude. “**By giving children a voice in the process, clinicians will be able to better anticipate and manage symptoms and thereby improve life for patients and their families.**” Hinds et al. (2017)

All the PROMs for CDG



How do you make a PROM?

- Develop candidate items (interviews, cognitive interviews, long-list, short-list)
- Test the items (with a lot of people)
- Refine the sub-scales / constructs; complex statistics
- Translation
- \$ € £

Clinicians and parents prioritise different things

- There's a need for co-production with clinicians, parents, and the child to working together

What is unique about children?

Developmental age =

- A need for different self-report tools
- Different social expectations
- Shifts in existential insights/concerns
- Changes in cognitive ability

Symptoms

- Different to most conditions where tools exist



What might the future hold?

- Online, mobile phone apps, paper-based?
- Condition-specific, tailored to your child
- Real-time data collection and synthesis
- For the family, shared with clinicians
- Global database of symptoms and management strategies



What next?

- Tell us what you want in a PROM
- Say you'll be involved!
- Keep in touch