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Informing the NHS Outcomes Framework: evaluating meaningful health outcomes for children with neurodisability using multiple methods including systematic review, qualitative research, Delphi survey and consensus meeting

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Scientific summary

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Scientific summary

Background

Estimates of the prevalence of childhood disability in the UK vary from 5% to 18%, depending on the definition or indicator of disability. Most commonly, an estimate of 1 in 20 children is cited. Neurodisability is an umbrella term for conditions associated with impairment of the nervous system and includes conditions such as cerebral palsy, autism and epilepsy; it is not uncommon for neurological impairments to co-occur. Aside from asthma, neurodisability is thought to represent the largest proportion of significant childhood disability.

Although neurodisability comprises a heterogeneous group of conditions, these conditions have much in common in terms of health-care needs. Children and young people affected by neurodisability have a range of impairments; some of these are relatively minor, but many give rise to complex health-care needs. As a consequence, children with a neurodisability are among the most frequent and intensive users of the NHS, requiring care and support from health services across primary and community care, hospital services and specialist centres.

Outcomes of a health condition or injury can be considered within the bio-psychosocial framework expressed through the World Health Organization's (WHO) *International Classification of Functioning, Disability and Health* (ICF). Patient-reported outcome measures (PROMs) assess the quality of care delivered to NHS patients from the patient perspective. PROMs measure a patient's health at a single point in time, and are collected through short, self-completed questionnaires. PROMs aim to assess components of health which are largely the components of the ICF under the rubric of health status or health-related quality of life. A wide range of generic and condition-specific PROMs has been developed for children and young people. Identifying PROMs for neurodisability requires, first, identification of the precise constructs to be measured and, then, the gathering of evidence of psychometric performance of available measures.

The NHS Outcomes Framework is part of a strategy that aims to deliver 'the outcomes that matter most to people'. Domain 2 of this framework will detail indicators of the 'quality of life of people with long-term conditions'. Proposed indicators include PROMs.

The identification of suitable outcome measures will improve the evaluation of integrated NHS care for the large number of children affected by neurodisability, and has the potential to encourage the provision of more appropriate and effective health care. This research sought to contribute to improving children's health outcomes by identifying a common purpose for NHS services for children and young people with neurodisability, and appraising appropriate outcome measures.

Aims and objectives

This research aimed to determine (a) which outcomes of NHS care should be assessed for children and young people affected by neurodisability, and (b) the extent to which they can be measured by existing PROMs.

To address these aims, the study had the following objectives:

- i. to identify key health-care outcomes, beyond measures of morbidity and mortality, that are regarded as important by children with neurodisability, and parents

- ii. to ascertain what outcomes of services health professionals think are important for this group and to assess the extent to which they agree with families' views
- iii. to seek agreement between families and professionals on important health outcomes, and assess the usefulness of candidate generic PROMs for use in the NHS
- iv. to identify relevant generic PROMs that have been used with children with neurodisability, and identify which best map onto the outcomes identified as most important by families and professionals
- v. to evaluate evidence of the psychometric performance of these PROMs when used with children with neurodisability
- vi. to make recommendations about the use of generic PROMs to measure health-care outcomes for children with neurodisability.

As part of this research, the serendipitous opportunity arose to develop and determine agreement on a definition of 'neurodisability'. Hence, the following objective was in addition to those specified in the protocol:

- vii. to develop and test agreement with a definition of neurodisability that would be acceptable and meaningful to both families and health professionals.

Methods

The research design comprised three main work streams to address the objectives:

1. a systematic review of the psychometric properties of generic multidimensional PROMs used to measure the health of children and young people
2. focus groups and interviews with children and young people with neurodisability, and separately with parents
3. an online Delphi survey with health professionals working with children and young people affected by neurodisability.

The systematic review was designed in two stages. First, we sought to identify all eligible PROMs used to measure the health of children and young people <18 years of age. We considered three categories of PROMs: (i) generic, for use across all people; (ii) chronic-generic, for use across people with chronic conditions; and (iii) preference-based measures (PBMs), which incorporate a weighting of scores based on a reference valuation of health states into a single index score. Then, we identified peer-reviewed publications of studies in which the psychometric performance of identified candidate PROMs had been evaluated with children and young people. Studies were categorised by whether they evaluated PROMs in (i) general population or (ii) children and young people with neurodisability, either specifically or in mixed samples.

The domain scales and items of candidate PROMs were coded with reference to the WHO's *International Classification of Functioning, Disability and Health Children and Youth Version* (ICF-CY), to provide an indication of what each instrument measures. The methodological quality of studies was assessed using the COnsensus-based Standards for the selection of health Measurement INstruments (COSMIN) checklist. Evidence of the psychometric properties or performance of instruments was extracted and examined, including content validity (theoretical framework and/or qualitative research), construct validity (structural validity and hypothesis testing), internal consistency, test-retest reliability, proxy reliability, precision, responsiveness and acceptability. Evidence of psychometric performance was rated using data extracted from included studies, with reference to standard criteria.

Qualitative research involved focus groups and interviews with children and young people affected by neurodisability, and parents, to identify important outcomes of NHS care and their feedback about example PROM questionnaires. Participants were recruited through networks maintained by the Council

for Disabled Children, and were purposively sampled to capture diagnostic, demographic and geographic variation. Appropriate topic guides were developed for children and parents in consultation with parents working with the researchers. Modifications were made to include children and young people with a range of abilities, including the 'Talking Mat approach' with children with profound communication impairment. The framework approach was used for the analysis, with reference to the WHO ICF-CY to enable the comparison of the results of the different streams.

An online Delphi survey was conducted with a multidisciplinary sample of health professionals working with children and young people with neurodisability in England. Health professionals were recruited initially through child development teams, supplemented by purposive sampling through professional societies to recruit under-represented professions. Data were collected using several iterative rounds of an online Delphi survey, an established method for seeking consensus. Questions in each round addressed (i) aspects of health clinicians target (rounds 1 and 2); (ii) aspects of health that the NHS should routinely assess (rounds 2 and 3); (iii) appropriateness of constructs of health covered by candidate PROMs (round 4); and (iv) proposed definitions of neurodisability (rounds 1, 2 and 3).

A consensus meeting with a small group of young people, parents and professionals was convened to seek agreement on a core set of more important aspects of health that could represent key health outcomes for neurodisability.

Results

The systematic review identified 41 eligible PROMs, and 126 papers that reported evidence of psychometric performance of 25 PROMs using an English-language questionnaire: 19 generic PROMs, two chronic-generic PROMs and four PBMs. Stronger evidence was found for a small number of PROMs: KIDSCREEN (generic), DISABKIDS (chronic-generic), and Child Health Utility 9D (preference-based measure). The Healthy Pathways may also be a promising instrument, with emerging evidence. Pediatric Quality of Life Inventory and KINDL provide a broader age spectrum and include self- and proxy-report versions, but evidence of psychometric properties was weaker. Robust evidence was lacking in one or more respects for all candidate PROMs, both in general populations and in those with neurodisability. Proxy reporting using PROMs was found generally to be poorly correlated with self-reports by children.

In the qualitative research, 54 children and young people participated: 50 participated in focus groups and four in interviews. There were 53 parents who participated in the research: 47 in focus groups and six in interviews. Children, young people and parents viewed health outcomes as inter-related and with reference to a hierarchy. Participants identified clearly the contribution foundation and intermediary outcomes made to a smaller set of higher-level outcomes that they felt were most important to have a good quality of life.

Health outcomes that were highlighted more frequently by young people and parents were communication, mobility, pain, self-care, temperament, interpersonal relationships and interactions, community and social life, emotional well-being, and gaining independence/future aspirations. Some parents were also particularly concerned with sleep, behaviour and/or safety if those issues were pertinent to their children. In terms of hierarchy, children and young people identified as most important interpersonal relationships and interactions, community and social life and emotional well-being; and parents identified community and social life, gaining independence/future aspiration and emotional well-being. Key factors that might be considered when using PROMs with disabled children and their families included contextual issues associated with questionnaires and entitlements for families of disabled children, problems with face validity, the cognitive task, and enhancing presentation and administration procedures to encourage participation.

In the Delphi survey, in total, 309 health professionals registered interest in participating; registrants identified themselves as being from a range of professions. Responses to all four rounds including only participants from England were, respectively, 233 out of 284 (82.0%), 232 out of 294 (78.9%), 227 out of 293 (77.5%) and 191 out of 292 (65.4%). Those aspects of health that were rated by health professionals as most commonly targeted, and also viewed as the responsibility of the NHS, were predominantly located in the WHO ICF 'body functions and structures' – pain, hearing, seeing, sleep and toileting – or were those domains of 'activities and participation' most readily influenced by provision of available assistive technologies: mobility and communication. Less frequently endorsed as the responsibility of the NHS, by consensus among participants, were play, relationships with family and friends, sport and leisure, and learning and applying knowledge. Professionals also identified treating various neurological-specific symptoms that are less amenable to assessment using generic PROMs.

Responses to the first three rounds that included iterations of proposed definitions of neurodisability, and several participants from outside England, were, respectively, 245 out of 290 (84.4%), 242 out of 300 (80.6%) and 237 out of 297 (79.7%). There was agreement (93% of respondents agreed or strongly agreed) with the final iteration of the proposed definition:

Neurodisability describes a group of congenital or acquired long-term conditions that are attributed to impairment of the brain and/or neuromuscular system and create functional limitations. A specific diagnosis may not be identified. Conditions may vary over time, occur alone or in combination, and include a broad range of severity and complexity. The impact may include difficulties with movement, cognition, hearing and vision, communication, emotion and behaviour.

There were 15 participants at the consensus meeting: three young people, five parents and seven health professionals (physiotherapist, occupational therapist, two paediatricians, nurse, paediatric surgeon, child and adolescent psychiatrist); apologies were received from a speech and language therapist and an orthopaedic surgeon. There appeared to be agreement between participating young people, parents and professionals regarding a suite of more important health outcomes: communication, emotional well-being, pain, mobility, independence/self-care, worry/mental health, social activities and sleep and, for children with intellectual impairments, also behaviour, toileting and safety.

Conclusions and recommendations

Selection of any PROM should be consistent with the purpose of measurement and satisfactory evidence of psychometric properties; the questionnaire must also have face validity to respondents. There was only partial overlap between the key outcomes identified by children, young people, parents and professionals, and the items and content assessed by more competitive candidate PROMs from the review. General feedback on the questionnaires indicated poor face validity. Even though several questions were felt to be relevant, other key health outcomes were identified as missing. In addition, young people and parents disliked questions that were perceived as negatively phrased. Careful cognitive interviewing should be undertaken with children, young people and parents to ensure that questionnaires have face validity to potential respondents with reference to the purpose of measurement. Further research is required to evaluate the psychometric properties of generic PROMs for children and young people with neurodisability, particularly testing item invariance across conditions, age groups and ability to detect meaningful change.

Parents identified discomfort in being able to respond to some questions as their child's proxy, particularly those about emotional domains and about activities that take place away from them such as school and with friends. Hence, there should be consideration as to whether or not these questions should be asked of parents, especially as ample evidence identified in this review suggests strongly that proxy reporting of such domains is unreliable. Parents' reports may be desirable to be able to assess those children who are too young to respond, or do not have the cognitive capacity to do so. There were some outcomes that parents felt were more important to assess for children with intellectual impairments,

including behaviour and safety. These may be important outcomes to include in parent-reported instruments, but less relevant to include in self-reported questionnaires for children and young people.

This research has proposed a new definition of neurodisability. The findings provide an incremental step towards a vision for what health services might seek to achieve for children and young people with neurodisability. The findings of this research can also inform health service policy regarding the NHS Outcomes Framework and the selection of PROMs. We have identified psychometric issues and contextual factors that affect the implementation of PROMs to assess NHS outcomes. Families may find it difficult to partition health outcomes that are a consequence of the NHS, and health professionals perceive a limit on what 'health services' can do for children and young people. The findings may also have salience for other health-related outcome policy initiatives. Current policy initiatives include considering measuring cross-sector integrated education, health and social care outcomes, and including young adults up to 20 and 25 years of age. Assessing health outcomes with a common metric through these age bands would offer strong advantages in terms of monitoring and evaluation of services.

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