

"What are patient reported outcomes and why they are important: Improving studies of preschool wheeze"

Makrinioti H.¹, Bush A.^{2,3}, Griffiths C.⁴

1: Honorary Clinical Research Fellow, Imperial College NHS Trust, London

2: Division of Medicine, Imperial College, London

3: National Heart and Lung Institute, Imperial College, London

4: Asthma UK Centre for Applied Research

5: Centre for Primary Care and Public Health, Blizard Institute, Barts and the London, School of Medicine and Dentistry

Corresponding author: Heidi Makrinioti, MD, PhD, e mail address:

heidimakrinioti@gmail.com

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Abstract

There are no outcomes-based commissioning structures in child health. This needs to change. Preschool wheeze is a very common condition. Many families present at emergency departments with their wheezy children and stay briefly. This highlights that this group of children may be lacking a more personalised approach. There are no user-friendly patient reported outcome measures in preschool wheeze. This needs to change. Research in this area is therefore highly anticipated.

Introduction

The involvement of patients in both the design and the conduct of research has been increasingly promoted by research bodies and policy makers. All members of the public can offer their expertise as users of the healthcare system but especially patients who are living with a long-term medical condition. Patients are now involved in both the design of healthcare interventions and relevant research but also in giving feedback on healthcare services provision and in reviewing research proposals and interviewing healthcare research staff (1). It is also important to note that many journals will now routinely reject submitted studies where patients have not been involved from the very beginning in the design of the study.

The success of research design and implementation can only be assessed by measurable outcomes. Traditionally, outcomes of research interventions are defined by the research team. In paediatric respiratory conditions outcomes related to number of hospital attendances, use of acute medication and lung function measurement have been used to monitor response to interventions including pharmacological treatment, but this is changing. Understanding what matters to patients has started being considered as equally important conventional outcome(s). By understanding which the important outcomes are for our patients, we will be in a better position to design meaningful interventions including behaviour modifications that are responsible for increasing numbers of avoidable emergency department admissions and poor treatment compliance in the UK.

What is a patient reported outcome measure (PROM)?

These are tools measuring outcomes that matter to patients, more specifically reflecting patients' or caregivers' perspective on the impact of the condition on their lives and how illness is experienced (for example, 'can I now climb my stairs?', rather than 'has my spirometry improved?'). *These tools aim to capture the patient's perspective, so patient involvement is required in the development of PROMs. When it comes to the subjective experience of their condition, patients and/or their carers can be considered as "experts" and they can bring value in conversations/consultations and in defining the importance of commonly used outcomes, such as length of hospital stay.* As opposed to Quality of Life Questionnaires, PROMs are brief tools designed to assess level of health and/or disability as perceived by patients/caregivers apart from health-related quality of life only. In 2009, it was suggested that value in healthcare should be assessed by reintroducing point of care use of PROMs (4). These are now being used to track outcomes of surgical operations and in the USA, PROMs and clinical data are driving improvements in results for children with

inflammatory bowel disease (5, 6). *In the NHS, PROMs have been successfully used to inform quality of care for patients undergoing hip replacement surgery, by highlighting the variation in cost between different hospitals and by suggesting new measures of assessment of efficacy which are of real importance to the patient* (7). Physicians are often reluctant to use PROMs routinely because of the time they take to use, and a lack of confidence in their value. By contrast, many patients welcome PROMs and believe that they need to be used routinely in their management (8). There are examples of validated PROMs in Box 1.

How is a PROM developed?

The first step is the definition of the conceptual and measurement model. The conceptual model provides a structured representation of the items (constructs) that will be included in the PROM. This conceptual model is usually based on literature review and provides the framework of reference. The subsequent measurement model is the result of qualitative research with patients and families and aims to map the individual items in the PROM to the construct (9). This is an important stage when some of the constructs will not be mapped to items if patients/carers do not consider these as important to be included in the PROM tool. The steps of the design of a PROM tool are depicted in Figure 1.

Why do we need PROMs for preschool wheeze?

Recurrent wheeze in preschool children is common. In the UK the highest number of hospital admissions with wheeze was in children less than 5 years old (10, 11). The majority of preschool children with wheeze suffer from recurrent commonly viral induced attacks, and although most remit over time (12), the associated morbidity and hospitalizations throw a heavy burden on both the healthcare systems and the families. However, there is often a mismatch between wheeze severity and parental response, in particular whether hospital management is sought. Several studies show that nearly a third of children who present to emergency departments with a wheeze attack are discharged within four hours (13-15). This short avoidable stay indicates that a significant number of preschool wheezers need not have sought clinical review at a secondary care setting. These differences will only be understood by studying the family perspective. *A PROM could be used in the routine management of preschool children with recurrent wheeze, to direct discussions around important aspects of care. It could also be used as a marker of quality of care to assess performance in primary care and in different hospital trusts.*

Are there suitable PROMs in children with recurrent wheeze?

For preschool children, disease control is assessed by the Test for Respiratory and Asthma control (16). The Paediatric Asthma Quality of life questionnaire is another tool used in younger children (more than 2 years old). There is also a version that is designed to be answered by parents (17, 18). There are two other instruments attempting to assess the severity of preschool wheeze attacks and the main parental feelings during the episode (19, 20). Although these instruments demonstrate how families feel during acute attacks, they do

not capture the changes in quality of their life over time and are not co-designed with families, unlike PROMs. Indeed, we recently published a spotlight which showed that there are no PROMs currently used for preschool children with wheeze in the UK, US, Argentina, Greece, Italy, Australia, Singapore and India (21). In summary, we have no PROMs for preschool wheeze, and, until such exist, we are unlikely to impact hospital attendances or assess new interventions in an appropriate manner.

Conclusion

Placing the individual at the centre of healthcare is the whole basis for modern medicine, and assessing whether this has been achieved throughout the whole cycle of care is very important. PROMs can be used to measure the success of treatment and quality of care in terms of which are important to patients. Further benefits include informing commissioning and use as endpoints in clinical trials. Clinicians should be assisted to use PROMs meaningfully as part of a management toolkit. In practice, this means they must be succinct. We do not have user-friendly PROMs for preschool wheeze. This omission needs to be rectified, and furthermore, suitable PROMs need to be developed for other paediatric diseases which currently lack them. Otherwise, the voices of parents and children will not be heard.

Box 1. Examples of Patient Reported Outcome Measures (PROMs) and their use

PROM as a clinical monitoring tool

PREOM-BMP (2), a 11-item questionnaire measuring the impact of bowel management in children and families' quality of life

PROM used as an outcome measurement tool in research

Pedi-IKDC (3), a 15-item instrument assessing health-related quality of life in children with knee ligament injury

PROM used as a quality of care indicator

EQ-5D (5), a 5-item questionnaire that assesses mobility, self-care, pain, usual activities, and psychological status and has been used in the NHS as an outcome measure for patients undergoing hip replacement operation

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Figure 1. figure describing the development, validation and adaptation of PROMs