



**Healthy London
Partnership**



North West London
Whole Systems Integrated Care

North West London CYP asthma meeting

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02

Outcome Measures for preschool children with recurrent wheeze

Heidi Makrinioti, MD, PhD

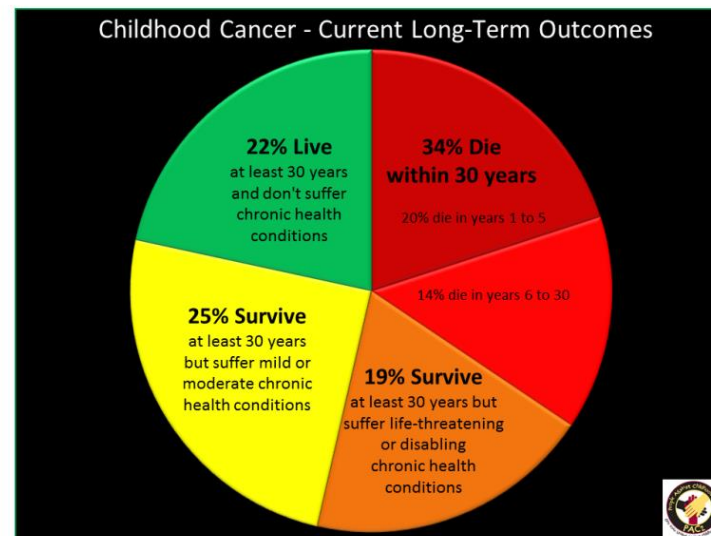
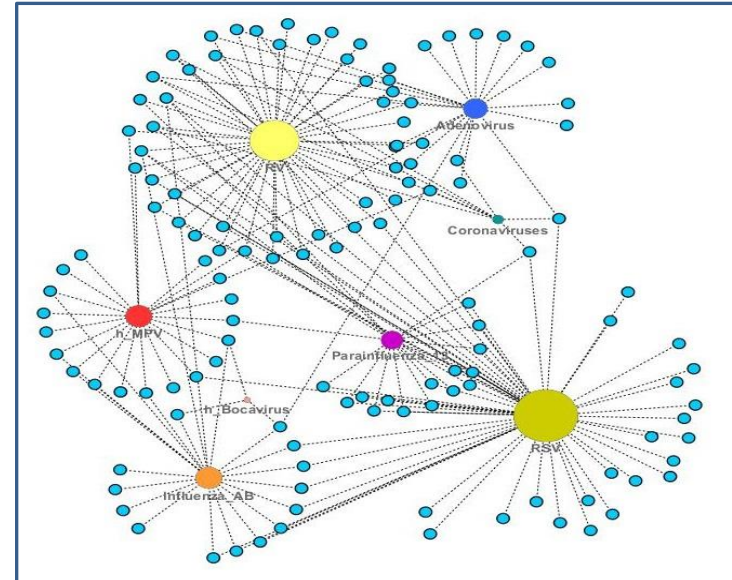
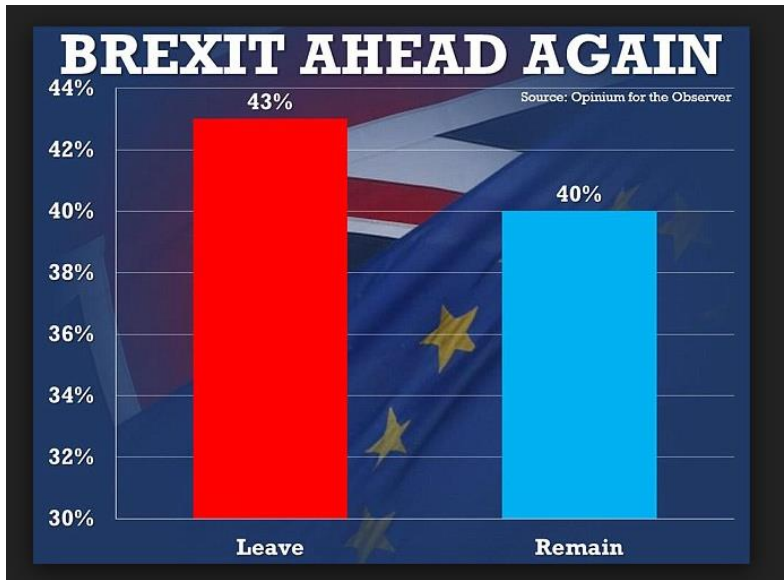
ST3 in Paediatrics

What is an outcome

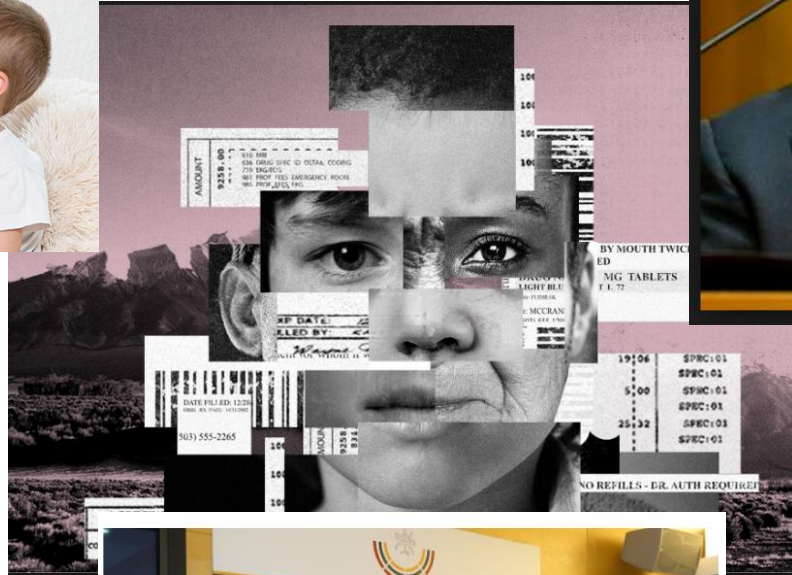
- **result**
- end **result**
- **consequence**
- net **result**
- upshot
- effect
- aftermath
- **conclusion**

synonym

Outcomes



Outcomes in child health



Outcomes in child health

“Today I’ve really been struggling with my asthma and extremely breathless feeling awful as though I’ve had a herd of elephants taking up my chest residence – I feel hopeless Hope you are all having a good night sleep”

“ I did not go kickboxing tonight because where I do it is chilly – I am more miserable than I should because I cannot exercise anything like I used to”

“There’s a family get together this weekend and my lungs have decided that this week they would become very troublesome, hence me missing out on the celebration, it’s so unfair, sometimes I hate being asthmatic. Sorry for the rant”

what doctors don't know about their patients

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Outcomes in child health



A Whole Population Approach: Patient Segments in Child Health

Integrated care is often built around patient pathways. In stratifying children and young people we strongly advocate a 'whole population' approach, where 6 broad patient 'segments' can be identified:

| | |
|---|---|
| Healthy child | <ul style="list-style-type: none">• <i>Advice & prevention</i> eg: Immunisation / Mental well-being / Healthy eating / Exercise / Dental health |
| Child with social needs | <ul style="list-style-type: none">• eg: Safeguarding issues / Self-harm / Substance misuse / Complex family & schooling issues / Looked after children |
| Child with complex health needs | <ul style="list-style-type: none">• eg: Severe neurodisability / Down's syndrome / Multiple food allergies / Child on long-term ventilation / Type 1 diabetes |
| Child with single long-term condition | <ul style="list-style-type: none">• eg: Depression / Constipation / Type 2 diabetes / Coeliac Disease / Asthma / Eczema / Nephrotic syndrome |
| Acutely mild-to-moderately unwell child | <ul style="list-style-type: none">• eg: Upper respiratory tract infection / Viral croup / Otitis media / Tonsillitis / Uncomplicated pneumonia |
| Acutely severely unwell child | <ul style="list-style-type: none">• eg: Trauma / Head injury / Surgical emergency / Meningitis / Sepsis / Drug overdose |

Preschool Wheeze

- wheeze - musical, high-pitched, expiratory sound associated with increased work of breathing, that can also sometimes be heard in inspiration ¹
- very common - about 30% of children during the first 3 years of life
- clinical phenotypes in young children - episodic viral wheeze (most common) and multiple trigger wheeze (less common) ²
- as physicians, we fail discussing environmental prevention with families
- is it a long-term condition – majority of wheezers grow out of the condition but families go through difficult years ³
- annual rate of emergency department visits is 23–42 per 1000 for preschool children with wheeze, compared with less than 15 per 1000 for those aged above six years old ⁴

¹ *Infantile Wheeze: rethinking dogma* Fernando Maria de Benedictis, Andrew Bush, Arch Dis Child 2017





² *Classification and pharmacological treatment of preschool wheezing: changes since 2008* Brand PL., ERJ 2014

³ *Around the world : preschool wheeze* Makrinioti H. et al., Lancet Resp Med 2017

⁴ *Ducharme FM, Tse SM, Chauhan B. Diagnosis, management, and prognosis of preschool wheeze. Lancet. 2014*

Preschool Wheeze

Preschool wheeze hospital admissions

Oral prednisolone in preschool children with virus-associated    
wheeze: a prospective, randomised, double-blind,
placebo-controlled trial

S J Foster, M N Cooper, S Oosterhof, M L Borland

| | Placebo (n=300) | Prednisolone (n=305) | Unadjusted model coefficient (95% CI)* | p value | Adjusted model coefficient (95% CI)† | p value |
|-----------------------------|--------------------|-------------------------|---|---------|---|---------|
| Primary outcome | | | | | | |
| Length of stay, min | 540 (124-971) | 370 (121-709) | 0.79 (0.64-0.97) | 0.0227 | 0.80 (0.65-0.99) | 0.0393 |
| Sensitivity analysis | | | | | | |
| Length of stay >4 h | 196 (65%) | 195 (64%) | 0.98 (0.87-1.10) | 0.72 | 1.01 (0.89-1.14) | 0.89 |
| Length of stay >7 h | 170 (57%) | 139 (46%) | 0.80 (0.69-0.94) | 0.0067 | 0.82 (0.69-0.96) | 0.0166 |
| Length of stay >12 h | 115 (38%) | 75 (25%) | 0.64 (0.50-0.82) | 0.0003 | 0.67 (0.51-0.86) | 0.0018 |

Data are median (IQR) or n (%), unless indicated otherwise. *Model coefficient is either the ratio, for prednisolone relative to placebo, of geometric means (logged continuous variable) from a linear regression model or the relative risk (dichotomous variable) from a log-binomial regression. †Model adjustments include age, personal and family history of atopy, baseline pulmonary score, and presence of virus.

Table 2: Unadjusted and adjusted model outputs for length of stay until ready for discharge (primary outcome) and sensitivity analysis (post-hoc superiority analysis)

Preschool Wheeze

Around the world: preschool wheeze, Lancet Respiratory Medicine, August 2017
Makrinioti H., Klaber R., Watson M.

Of all UK admissions for children aged 1–16 years with acute wheeze or asthma attacks between 1998–2005, approximately 75% each year were for children younger than 5 years old (median age 3 years), and the rate in preschool age children has remained unchanged. The cost to families caring for an unwell preschool age child at home or in hospital is significant, including costs of taking unforeseen time off work.

Because the main burden from preschool wheezing is attributable to emergency healthcare visits and hospitalisation admissions, the primary outcome measure included in interventional studies is consistently emergency health care use. Outcomes that are important for patients and families are not considered even as secondary outcomes. The huge effects on caregivers and families of an unwell preschool age child, especially if illnesses are recurrent and result in frequent hospitalisation cannot be underestimated. Disappointingly few effective therapies exist for preschool wheezing, but the poor management for this condition might partly be explained by our failure to address appropriate outcomes. Few validated patient-reported outcomes are available for school-aged children with asthma, and none are currently available or used even in a research setting, let alone in clinical practice, for preschool age children with wheeze in the UK.

The Test for Respiratory and Asthma Control in Kids (TRACK) is a validated questionnaire that allows monitoring of symptom control in preschool age children. Although TRACK is completed by caregivers, the five items included (frequency of respiratory symptoms, activity limitation, night time awakenings, rescue medication use, and oral corticosteroid use) only incorporate aspects of control considered important by the clinician. There is no assessment of the effects of symptoms on the caregiver, or aspects that the caregiver might think are most debilitating for the child, such as reduced sleep because of difficulty in breathing.

To make progress in the management of preschool wheezing disorders, the outcomes that are important for caregivers, families, and children need to be understood, to enable effective tailoring of treatment regimens, wheeze management plans, and avoid the huge burden of this disease on health-care services.

Sejal Saglani, Faculty of Medicine, National Heart and Lung Institute, London, UK

When considering wheezing disorders the question becomes one of defining the problem. Wheezing is a musical-like sound from the airways, most prominent on expiration, which can stem from multiple causes. Wheezing can be monophonic when air passes through a single narrowed airway; this usually involves a foreign body or anatomical defect that obstructs the airway involved. But more commonly a wheezing disorder refers to one or more phenotypes of asthma in which the wheezing is polyphonic from air passing through multiple airways, often described as sounding like the instruments of an orchestra tuning up. These asthma phenotypes are characterised by their clinical pattern. The most common clinical pattern (phenotype) of asthma in preschool age children involves acute episodes of wheezing, cough, and respiratory distress, associated with common cold viruses. These are episodic illnesses with the child completely well between these episodes. Less common (about 20% of preschool age

In Latin America, a high prevalence and increasing severity of wheezing disorders has been reported in pre-school age children.

Designing an outcome measurement tool

During the last month how often did your child cough during their night sleep ?

During the last month how often did you use inhalers for your wheezy child?

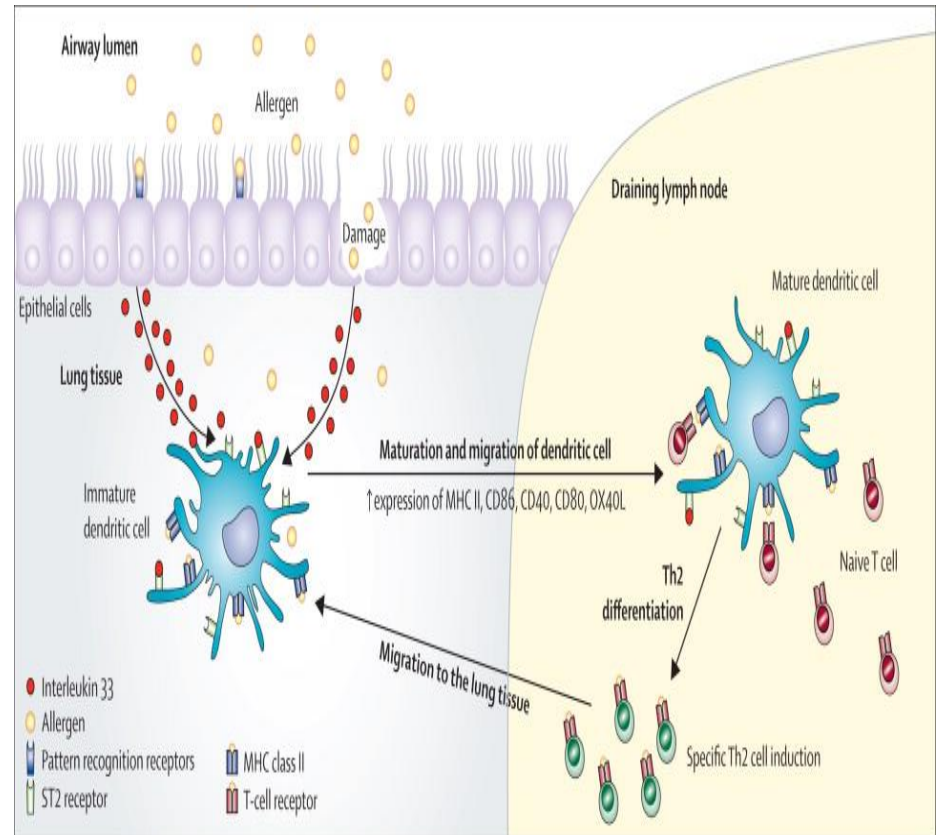
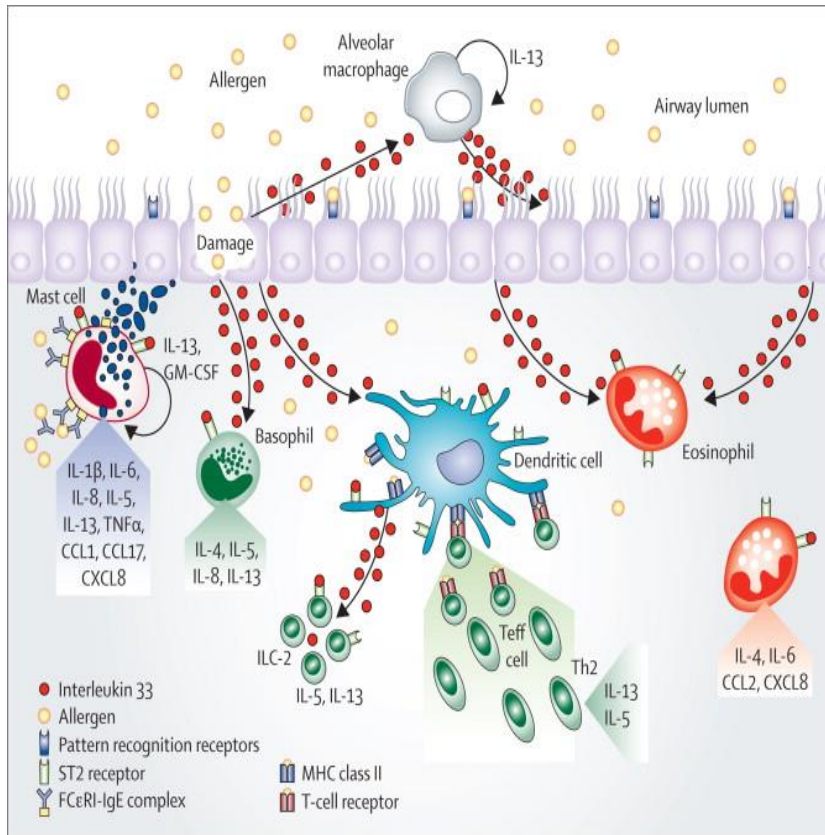
What matters to you ?

How do you cope with your child being in and out of hospital so often?

How do you feel living with a child having this condition?

My design is better than yours

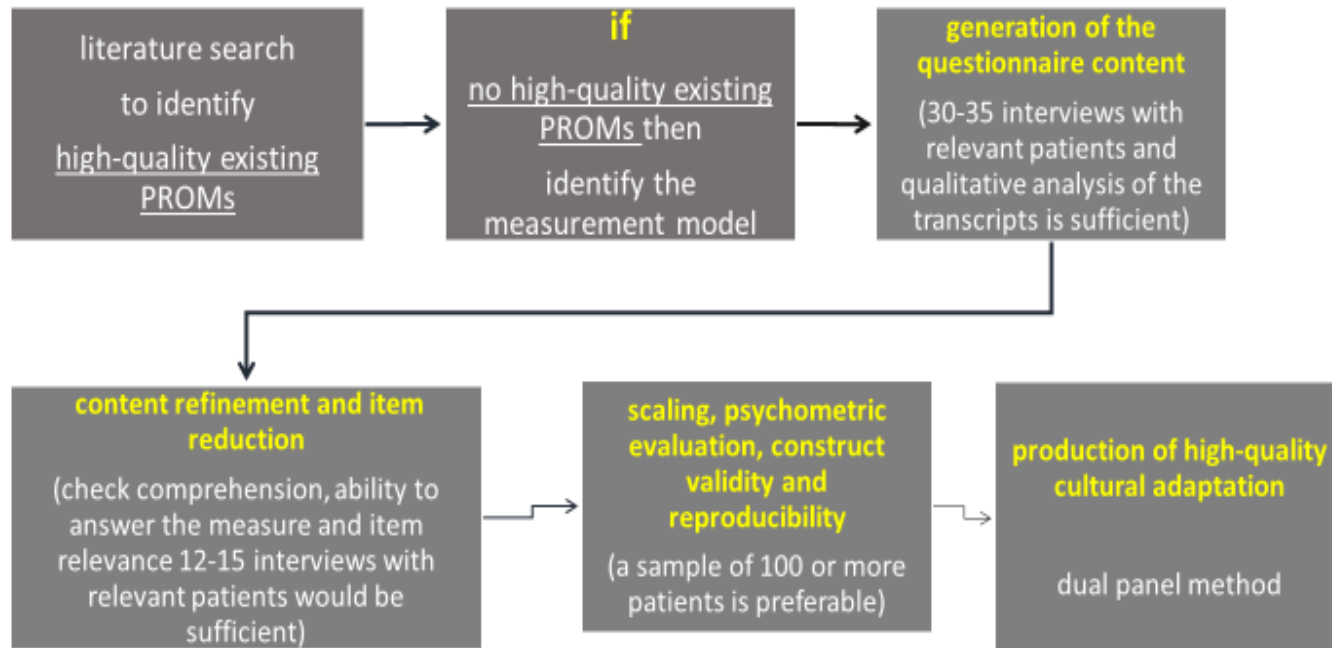
Role of interleukin 33 in respiratory allergy and asthma , Lancet Respiratory Medicine Journal



Dr Heidi Makrinioti, Marie Toussaint, David J Jackson, Ross P Walton, Prof Sebastian L Johnston

My design is better than yours

Development, validation and adaptation of PRO measures

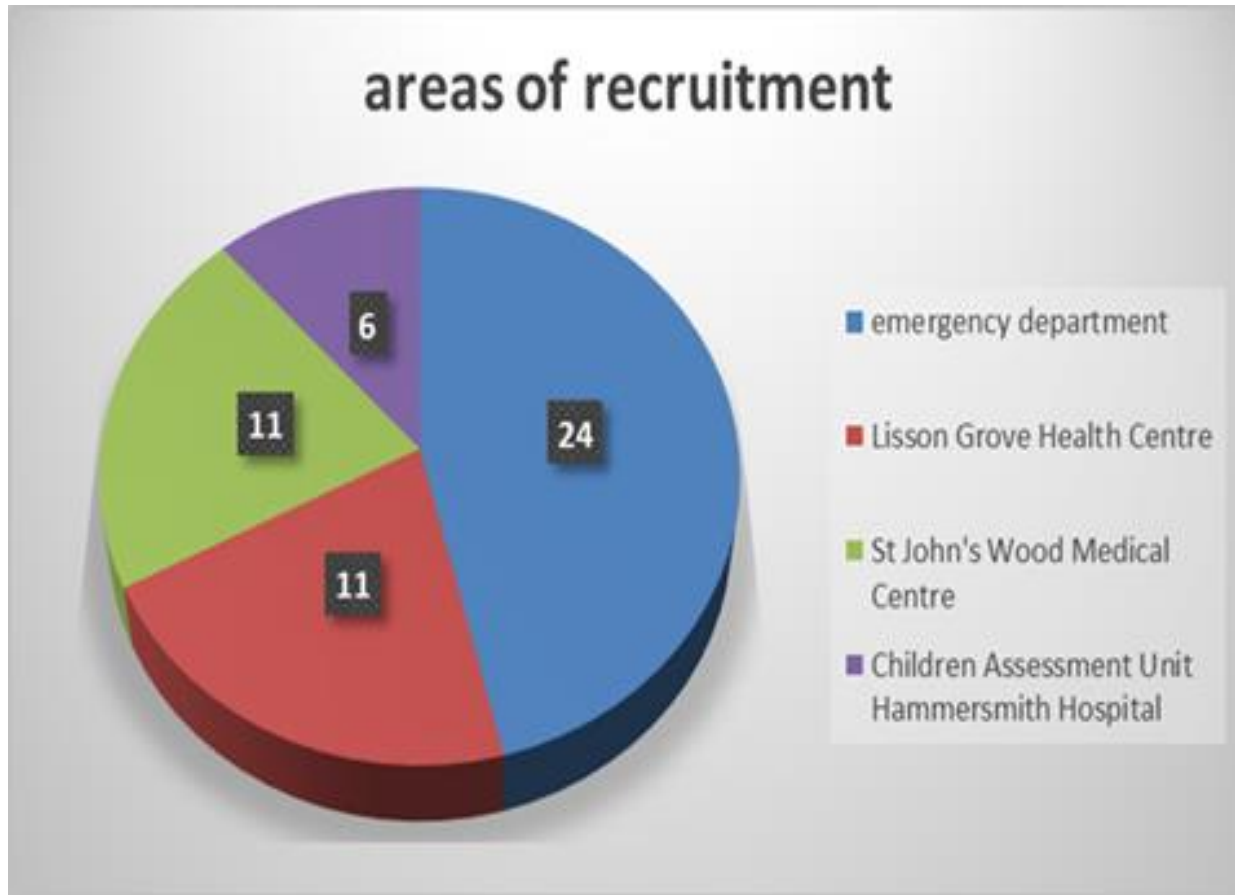


My design is better than yours

 connecting care for children



Patient (parent) reported outcomes

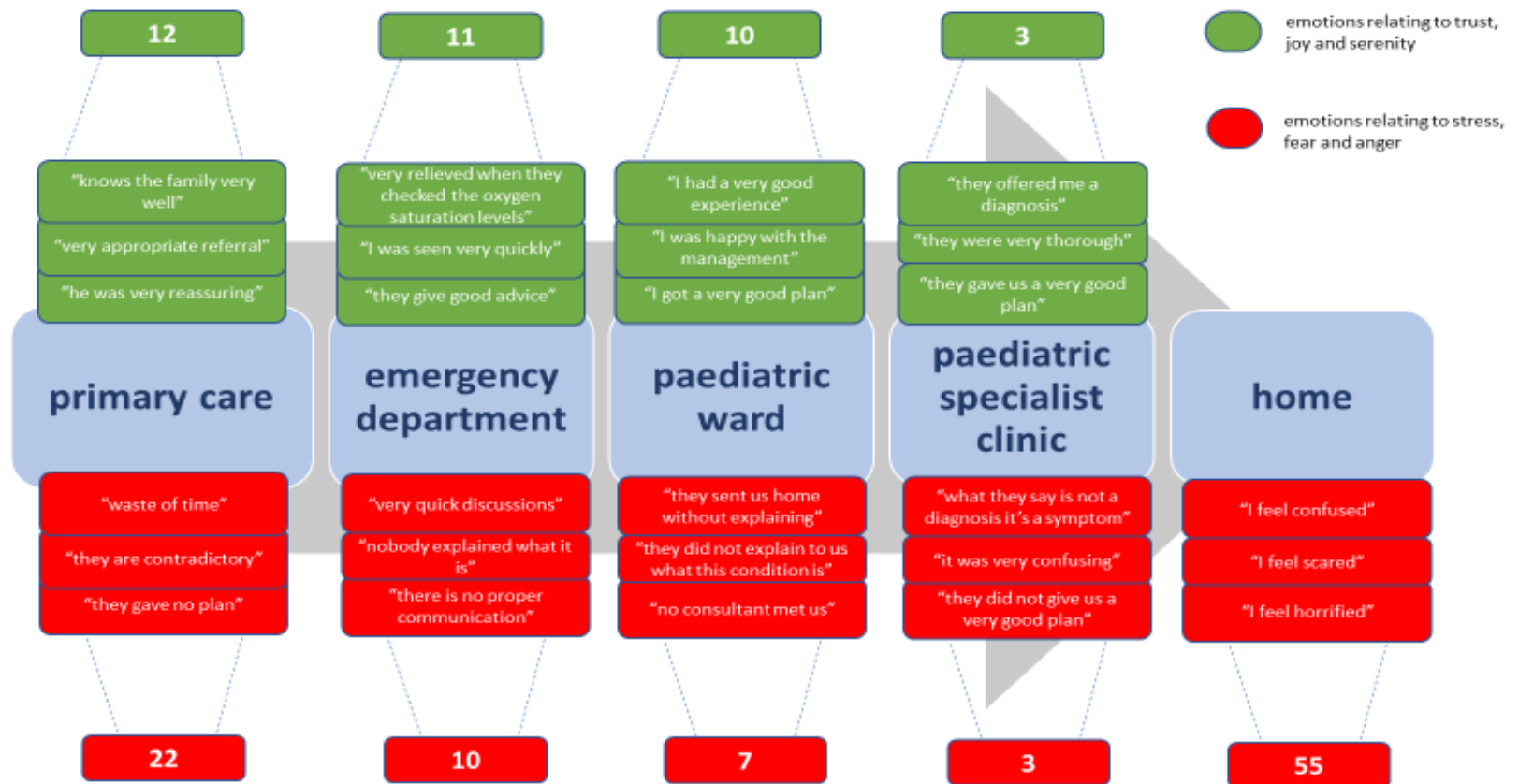


How do parents of preschool wheezers feel when they experience healthcare services



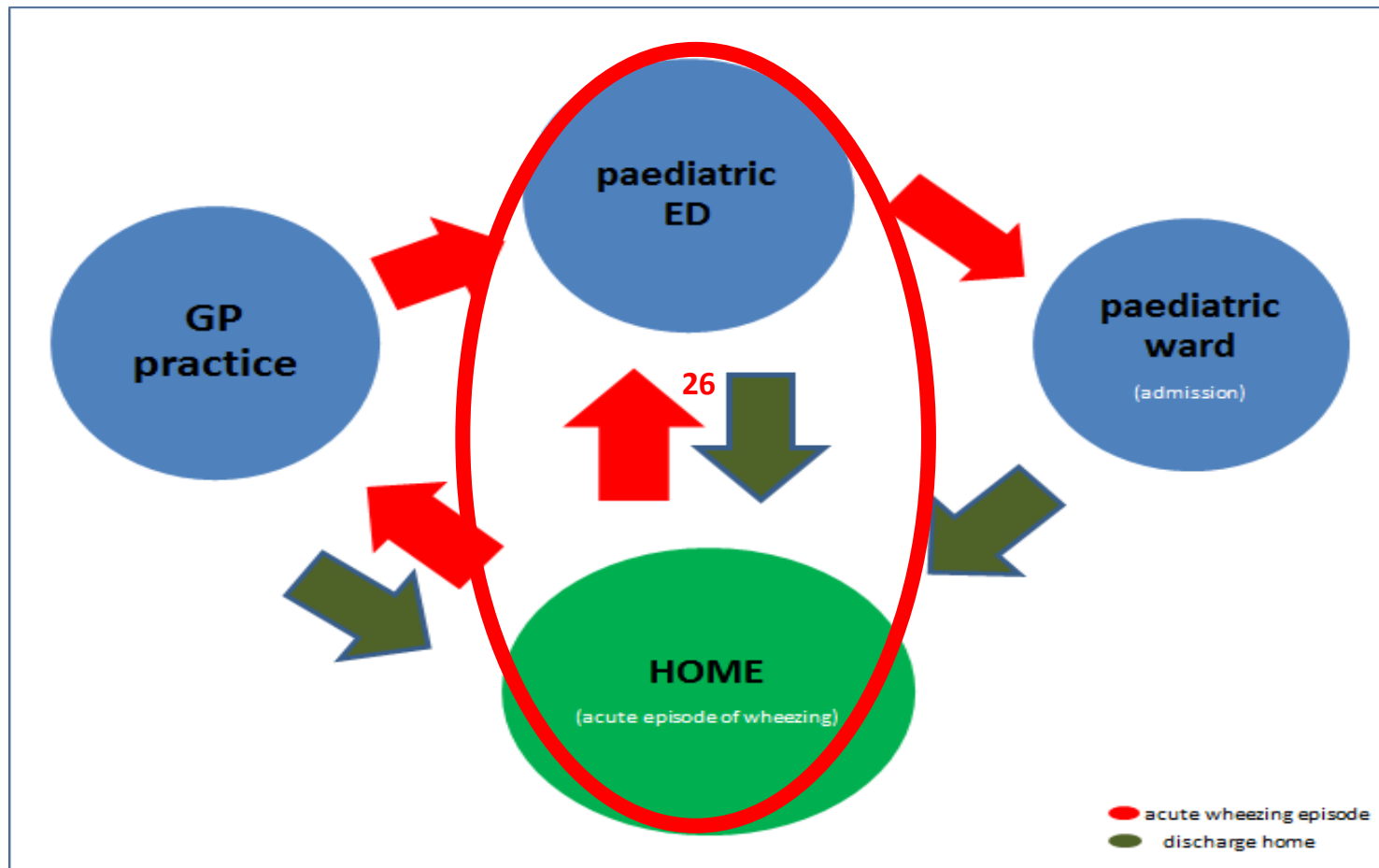
connecting care for children

emotional map describing parental feelings

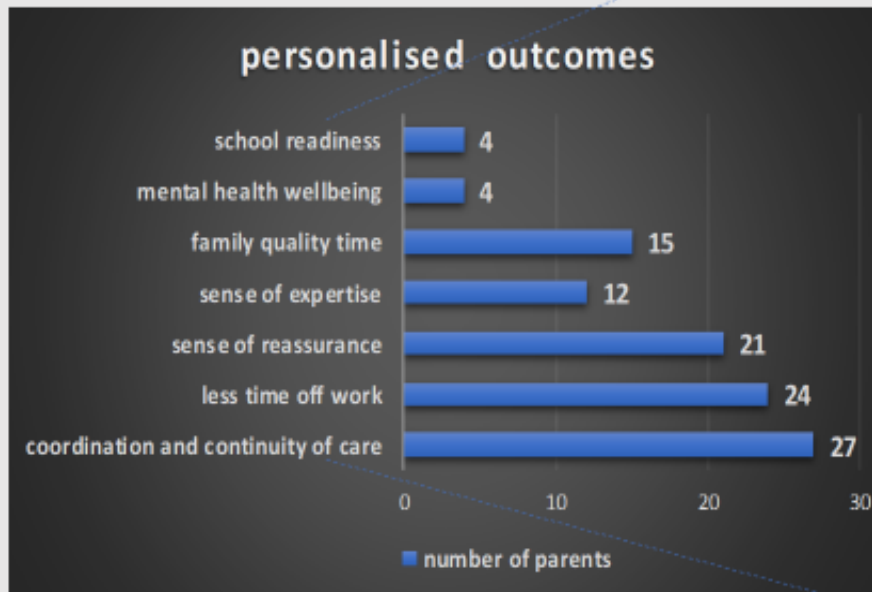


where they seek for help

process map of patient's journey



which are the main outcomes



"I need to have some time to teach him things – my niece is far more advanced than him"

"I need to maintain my mental health wellbeing – I can't stay calm and I feel always stressed"

"I need to save time and effort and focus on family"

"I need to have a better idea of what this is in order to feel better"

"knowing more about it would provide reassurance"

"I need more time for work, I have no time for education, I need something simple to have at home"

"I need a plan for his management that doesn't change all the time"

Wheeze and me questionnaire

9-item questionnaire that measures

1. incidence of wheeze attacks and use of rescue medications
2. parental/caregiver perception of recurrent wheeze control
3. parental/caregiver perception of effect of recurrent wheeze on family/child quality of life

The challenge

- a variety of instruments measuring outcomes in childhood conditions
- few of them successfully used to provide insightful information around outcomes of research interventions
- couple of them commissioned to be used in bedside

Will we be able to translate **patients' voices into healthcare management tools?**

Will these tools be used **to assess outcomes in population-wide open studies?**