Title Page

(i) Health-related out of pocket expenses for children living with Tuberous Sclerosis or Mitochondrial Disorders: a prospective pilot study in Australian families

(ii) Original Article

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(vi) Conflict of Interest

The authors declare no conflict of interest

Main Text

Abstract

Aim: We aimed to describe health-related out-of-pocket (OOP) expenses incurred by Australian families living with children with chronic and complex diseases.

Method: A prospective pilot study of OOP expenses in families with children with tuberous sclerosis (TS) or mitochondrial disorders (MD) in 2016-17. An initial survey assessed the family's financial situation, child's health functioning, and estimated previous 6 months' and lifetime OOP expenses. Thereafter, families completed a survey each month for six months, prospectively tracking OOP expenses.

Results: Initial surveys were completed by 13 families with 15 children; median age 7 years (range: 1-12); five with MD, 10 with TS. All families reported OOP expenses: 38% paid $2000 per annum, more than double the annual per-capita OOP costs reported for Australia by the OECD. Eight families estimated $5,000-$25,000 in OOP expenses over their child's lifetime and 62% of mothers reduced or stopped work due to caring responsibilities. Eleven families paid annual private health insurance premiums of $2000-$5122, but 72% said this was poor value-for-money. Prospective tracking by eight families (9 children) identified the median OOP expenditure was $863 (range $55-$1398) per family for 6 months. OOP spending was associated with visits to allied health professionals, non-prescription medicines, special foods, supplements, and disposable items. Eight families paid for 91 prescription medications over 6 months.

Conclusion: All families caring for children with TS or MD reported OOP expenses. A larger study is needed to explore the affordability of health care for children living with a broader range of chronic diseases.

Key Words

Rare Diseases, Health Expenditures, Tuberous Sclerosis, Mitochondrial Diseases

Introduction

Australians have the highest out of pocket (OOP) health costs (18.7%) as a proportion of total per capita health expenditure, compared with countries with similar universal health coverage systems, such as New Zealand (10.2%), the United Kingdom (9.5%) and Canada (13.6%).[1] According to the Australian Household Expenditure Survey, Australians spend, on average, 6% of their annual income on health care,[2] or $771 per capita per annum in OOP expenses.[1]

Australia provides universal health cover through the Medical Benefits Scheme (MBS) and the Pharmaceutical Benefits Scheme (PBS). [3] Australians may choose to subscribe to additional Private Health Insurance (PHI) which is subsidised by the Australian Government. [3] Despite this, health-related OOP expenses have steadily increased, challenging the sustainability of the health system for people living with chronic diseases.[3]

People living with rare chronic diseases are particularly vulnerable to OOP health costs, because they need to access multiple specialist, primary care, and allied health services and medicines to optimise their health-related function.[4] In particular, children living with rare, chronic and complex conditions require frequent access to health services and early intervention programmes to optimise their health and development. High OOP health costs may make families vulnerable to financial hardship.[4,5]

Delayed diagnosis and limited recognition of the impacts of rare disease in children mean that these children often “fall between the cracks” of our health system.[6,7] In most cases, the correct diagnosis will open doors to publicly funded health services and programs.[8] The introduction of Chronic Disease Management (CDM) Plans in 2011 addressed this problem to some extent, but even when a CDM plan is in place, the number of free consultations is limited, leading to OOP costs for additional occasions of service.[9]

Tertiary paediatric hospitals provide the bulk of specialist care, which is covered by Medicare and does not incur additional OOP costs. However, families living in regional and remote Australia have limited access to publicly funded specialist doctors and

allied health practitioners.[10] They often pay private consultation fees well above the scheduled fees reimbursed by Medicare.[11]

Children may require specialist equipment and consumables, such as dressings, tubing, syringes, and incontinence aids. Rebates for medical consumables are seldom reimbursable under PHI nor Medicare, and state health funding provides limited supplies each year. Some children also need special diets and supplements and other non-prescription items which are not covered under the PBS.[12] Early intervention programs, to maximise developmental gains, manage difficult behaviour, or control pain, often require attendance at tertiary hospitals, which are located in capital cities and, for some, attract OOP expenses for travel and accommodation.[13, 14]

Financial burden is further amplified due to lost income as one or both parents become primary caregivers. Our recent study of 462 families of children with a variety of rare diseases, including some affected by mitochondrial disease (MD) and tuberous sclerosis (TS), showed that 34% incurred OOP expenses for essential health services while seeking a diagnosis for their child.[7] These costs were associated with diagnostic tests, treatments, procedures and consultations and, based on retrospective estimates provided by families, averaged $5,700 per annum per family. Of families who incurred OOP expenses, 70% said this caused financial stress.[7] OOP expenditure among Australian families with a child with a rare chronic and complex condition has not been studied prospectively.

We aimed to retrospectively estimate OOP expenses in the last 6 months and over the child's lifetime and then to prospectively assess OOP expenses incurred over 6 months by families living with a child with either MD or TS, and to describe the health-related costs necessary to care for their child but not covered by Medicare or PHI. In this pilot study we also aimed to determine the feasibility of collecting data prospectively from families over a 6-month period.

Methods

Survey design

This study involved a partnership with the Australian Mitochondrial Disease Foundation (AMDF) and Tuberous Sclerosis Australia (TSA), we developed an initial retrospective survey and a survey for prospective data collection.

The initial survey collected data on the children's diagnosis, age, and health functioning according to the Measure of Function (MOF);[15] the family's living situation; and financial details including home ownership, annual income bracket, sources of income, employment status, and access to Private Health Insurance (PHI). Parents/caregivers were also asked to estimate the health-related OOP expenses for their child both during the last 6 months and over their entire life.

Following the initial survey, families were asked to complete a monthly survey for six months to track their health-related OOP expenditure. An email reminder was sent each month, and families who did not respond were followed up with a phone call. The monthly survey recorded changes in employment, income and family situation since the previous survey, and any OOP expenses associated with their child's health care. The costs used in the analysis are based on 2016 costs. An exit survey was sent to all families to determine the acceptability and relevance of the survey, for eight surveys in total (Figure 1).

All surveys were available on-line via the Research Electronic Data Capture (REDCap) platform [16] or in paper format if preferred by the family.

Participants

Our research partners invited families from their networks to participate in the study in October/November 2015. Only families who could read and write English were included in the survey. Eligible families were enrolled in February 2016. A second recruitment wave was conducted in October 2016 due to a low initial response rate.

Twenty-five families expressed interest to participate and received information about the study purpose and commitment required. Families who did not respond were followed up via email and phone call. Sixteen families completed and returned the consent form.

This study was reviewed and approved by the University of Sydney Human Research Ethics Committee (project number 2015/883)

A descriptive analysis was performed using the Statistical Package for Social Science (SPSS) version 20. All percentages were rounded up to whole numbers. All p-values calculated were two-tailed; the alpha level of significance was set at 0.05. We used the Wilcoxon Rank Sum test to assess statistical significance between groups.

Results

Of the 16 families who returned a consent form, 13 completed the initial survey and five of these completed all six of the prospective monthly surveys. Three families each completed one, three or four monthly surveys.

Demographics and living situation

The 13 participating families had 15 children, five with MD and 10 with TS. Two families each had two children with MD. Demographic characteristics of the families and their children are shown in Table 1. According to the MOF, most children had impaired health functioning with variable problems in some but not all areas (Figure 2).

Family financial situation

All of the 13 families had income from employment and 11 received Australian Government funding under the Carers Allowance scheme and had a Health Care Card for the child. Just over a half (54%; n=7) of the families had a weekly household income from all sources of $1500 or more, five families had a weekly income of $1000-$1499 which is at or below the Australian median weekly household income of AU$1438.[2] One family did not provide their income bracket. Eight mothers (62%) had reduced or stopped employment to care for their child. Almost half (6, 46%) said that they are unable to save and just break even, two spent more than they earned, and 5(38%) saved some money each week.

Eleven families paid annual PHI premiums of $2000-$5122 (median $3264) of whom five (46%) said that health insurance covered a small proportion of their child's health

care costs, 4(36%) said it covered some, and 2(18%) said it covered most health costs. Eight families (72%) who paid PHI premiums felt this was poor value for money.

Out-of-pocket expenses

All of the 13 families reported paying health-related OOP expenses during their child's lifetime, including for travel and accommodation to access care, consultation fees for specialist doctors and allied health professionals, non-medicines, procedures and equipment (Table 2). One family reported the need to travel interstate to access care and another family had to install air-conditioning ($5000 OOP expense) because their child is unable to auto-regulate body temperature. Seven families (54%) retrospectively estimated their OOP expenses in the 6 months prior to participating in the study at $1,000-$5,000, the other six at < $1000. OOP expenses over the child's lifetime were estimated at $5,000-$25,000 for eight families and over $75,000 by two families (Table 3a and 3b). When OOP expenses were calculated per year of the child's life, expenses varied between children with MD (N=5) and TS (N=10) and also between children with the same disease (Table 3a and 3b).

Prospective tracking of expenses for 6 months

Of the 8 families (with 9 children) who completed the prospective monthly surveys, three families reported changed financial circumstances since the initial survey - one family reduced their PHI cover to reduce expenses, income increased for one family by ~$200 per week, and one family said an increase in their PHI premium caused financial worries.

The monthly OOP costs varied across the eight families from $9 to $233. The total amounts spent over 6 months ranged from $55 to $1398 (median $863). Five families lived in major cities and three in regional centres. Eight of the nine children had variable problems in some, but not all areas (MOF score 5) and one child had no more than slight problems (MOF score 3). There were no associations between the amount of OOP costs and the child's health functioning or the family's geographical location ($p = 0.41$).

The items associated with OOP costs collected in the prospective surveys are summarised in Table 4. Some children needed frequent visits to allied health

professionals, and for 63% of these visits, OOP expenses were incurred. There were 21 visits to specialist doctors among eight children, and 14% of these visits incurred OOP expenses (Table 4).

For the prospective OOP costs (Table 5), eight families had prescription medicines dispensed 91 times over six months, paying a nominal fee of $5 or $7, for a total cost of $1042 for all eight families or $130 per family on average. Non-prescription items, such as fluid replacement formulations, stool softeners and probiotics, incurred a total cost of $662 for all five families, but two families with children with mitochondrial disorders, accounted for most of this - $140 for one family and $333 for the other. Four families had OOP expenses associated with consumable items for a total of $1189. Consumables included continence aids for children aged 4 to 7 years, one family spending $842 over 6 months. Other expenses included costs of parking while visiting health professionals, education/therapy activities and respite care.

Two families completed the exit survey, indicating that the survey questions were relevant and important, and the monthly surveys took between 10 and 15 minutes, while the initial survey took 20-30 minutes to complete.

Discussion

Our study supports previous anecdotal evidence, that families living with a child with a rare chronic and complex condition face financial impacts because of health-related OOP expenses. In this study, almost two-thirds of mothers had reduced working hours, or left employment to care for their child, thereby reducing household income. All families reported OOP spending on their children's healthcare that was not covered by PHI or Medicare.

Families in our study estimated spending more than double that amount on their children's health care, and 38% estimated spending more than $2,000 per annum. Health expenditure data on children with chronic disease are not readily available to enable international comparisons, either from the Organisation for Economic Co-operation and Development (OECD) or the Commonwealth Fund. The number of children living with chronic disease is increasing,[17] and population-based estimates of health costs, including OOP expenses, is important for this group to inform

development of sustainable models of care, in both the paediatric and the adult health sectors, as these children are increasingly surviving into adulthood.[18]

In addition to health-related OOP expenses, PHI premiums were a substantial expense for families. In line with current consumer sentiment[19], most families in our study felt that PHI premiums were poor value for money, because many health care items and services were not covered.

Visits to allied health professionals and specialist doctors commonly incurred OOP costs. Community-based allied health professionals and specialist doctors work in a fee-for-service business model and may set their own fees, often above the Medicare scheduled fee rebate.[20, 21] Furthermore, Medicare rebates for visits to allied health professionals are limited to five funded sessions per calendar year for a child with a CDM plan.[9] Children with chronic conditions often need ongoing therapy beyond five sessions, leaving families to pay for additional sessions.[7] For children with disability, the National Disability Insurance Scheme (NDIS) [22] may address this gap and provide funding for ongoing allied health therapy, however, detailed data on this are not available.

Strengths and Limitations

We captured unique, prospective data about OOP expenses that is not captured by any other routine data collection. Our study is not representative or generalizable to all families with children with MD or TS, nor to families with children with other chronic and complex conditions. Our survey was available only in English, limiting potential respondents. Future studies would benefit in translating our surveys, to improve representativeness.

Data on adults living with a chronic disease and in a regional areas is associated with limited access to health care services and higher OOP costs, compared with the population average.[23] Our sample was too small to show such associations, and a larger prospective study of families living with children with a variety of chronic, complex conditions is needed.

Methodological challenges included recruitment and retention of families, most likely due to the time commitment required to complete the surveys by families who are already time-poor due to caring responsibilities and often stressed.[7] We chose the longitudinal prospective design based on a previous study in adults[5] in order to reduce bias of potentially inaccurate retrospective estimates of health-related OOP costs. However, our data were incomplete. A smart-phone app or a monthly phone survey might have improved completion and should be considered for future studies.

Conclusion

Australians have higher OOP health expenses that in similar OECD countries [1] and we demonstrated that families of children with MD and TS have OOP health costs that are more than double those estimated in the general Australian population [1, 2]. OOP costs were incurred in addition to PHI premiums and in the context of declining household incomes because of caregiver responsibilities limiting employment. Families were systematically disadvantaged through OOP costs associated with visits to health professionals, non-prescription medicines, and consumables not covered by our health system. These costs are not sustainable for families of children with chronic complex diseases. A larger, prospective study is needed to explore the affordability of health care for children living with a broad range of chronic conditions in Australia and to develop models of care that are affordable for these families.

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Table 1. Demographic characteristics of participating families (n=13) and their children (n=15)

Characteristic	n
Families	***(n=13)***
Number with one child	13
Number with two children	2
Ethnicity:	
White European	12
European/Middle Eastern	1
Main language spoken:	
English	13
Australian jurisdiction of residence:	
New South Wales	4
Victoria	4
Queensland	2
South Australia	2
Tasmania	1
Number of adult partners:	
One	1
Two	12
Living status:	
In own home	11
Renting	1
With relatives	1
Children	***(n=15)***
Median age in years (range)	7 (1-19)
Male	7
Condition:	
Tuberous Sclerosis (TS)	10

Mitochondrial disorders (MD)	55
Country of birth:	
Australia	15
Siblings:	
Yes	7
No	6

Table 2. Items associated with past health-related OOP expenses over the child's whole life

Items requiring OOP	Number of families reporting this expense N (%)
Number of families	13
Travel and accommodation when visiting health services	11 (85)
Visits to specialist doctors	10 (77)
Non-prescription medicines	10 (77)
Prescription medicines	9 (68)
Visits to allied health professionals	7 (54)
Visits to general practitioners	5 (38)
Operations/procedures	3 (23)
Hospital admissions	2 (15)
Other (medical equipment, educational resources about the child's condition)	2 (15)

Table 3a. Amount of health-related OOP expenses estimated by parents for the child's lifetime (mitochondrial disorders) (N=5 children)

Estimated OOP expenses for the child's lifetime	Number of families N	Median Age of child	Costs per year of child's life**(AUD $)
<$5000	1*	5.5	<909
$5001-$10,000	1	9	556 -1111
10,001 – 15,000	- -	-	-
15,001 – 20,000	-	-	-
20,001 – 25,000	1*	6	3334-4167
25,001 - 50,000	-	-	-
50,001 – 75,000	-	-	-
75,001 – 100,000	-	-	-

*family had two children

** children with mitochondrial disorders can expect to have a normal life expectancy

Table 3b. Amount of health-related OOP expenses estimated by parents for the child's lifetime (tuberous sclerosis) (N=10 children)

Estimated OOP expenses for the child's lifetime	Number of families N	Median Age of child	Costs per year of child's life (AUD $)**
<$5000	1	1	<5000
$5001-$10,000	3	4	1250-2500
10,001 – 15,000	2	5.5	1818-2727
15,001 – 20,000	-	-	-
20,001 – 25,000	1	7	2857-3571
25,001 - 50,000	1	21	1190-2381

50,001 – 75,000	-	-	-
75,001 – 100,000	2	8	9375-12,500

** children with tuberous sclerosis can expect to have a normal life expectancy

Table 4. Visits to health professionals recorded by N=8 families over 6 months of prospective tracking and the proportion requiring OOP costs

Service	Number of families	Number of visits	Number (%) of visits where OOP costs were incurred	Total OOP costs spent by all families (AUD $)
Visits to specialists	8	21	3 (14)	394
Visits to GPs	4	5	nil	nil
Visits to allied health professionals*	4	19	12 (63)	993

* Allied health professionals = dentists, physiotherapists, speech therapists, audiologists, and psychologists.

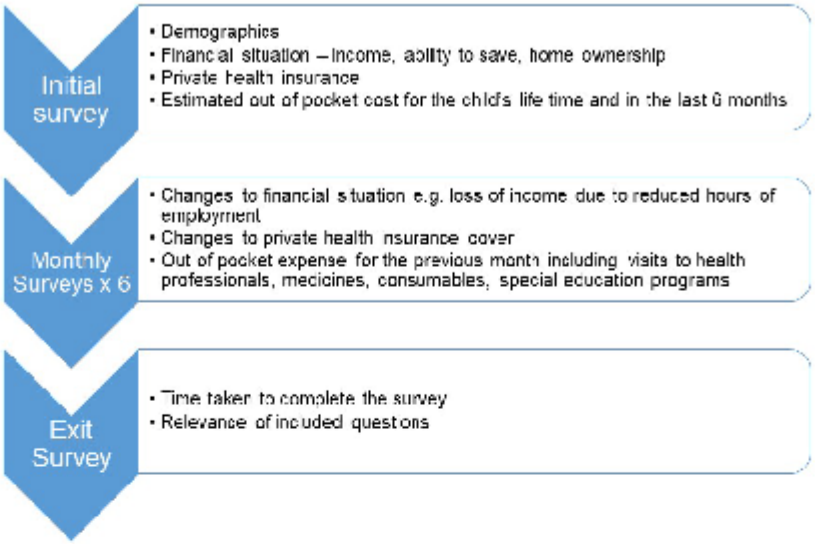
Table 5. Prospective health-related out of pocket expenses tracked over 6 months by N= 8 families)

Type of expense	Total costs (A$)	Projected annual costs (AUD $)
Health Service Visits	1387	2774
Hospital admissions	0	0
Prescription medications	1042	2084
Non-prescription medications	662	1324
Health related consumable items	1189	2378
Equipment*	0	0
Travel related costs	196	392
Special programs	1143	2286
Total costs	5289	10,578

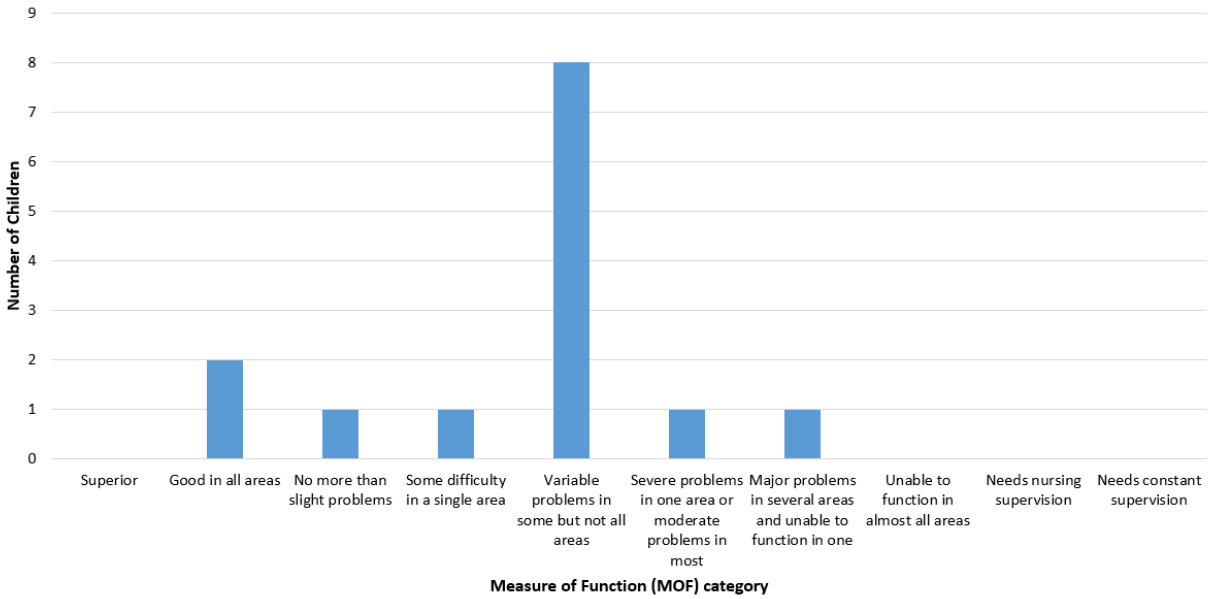
* either purchasing new equipment or paying for maintenance on existing equipment
** special programs include education and therapy activities, respite care

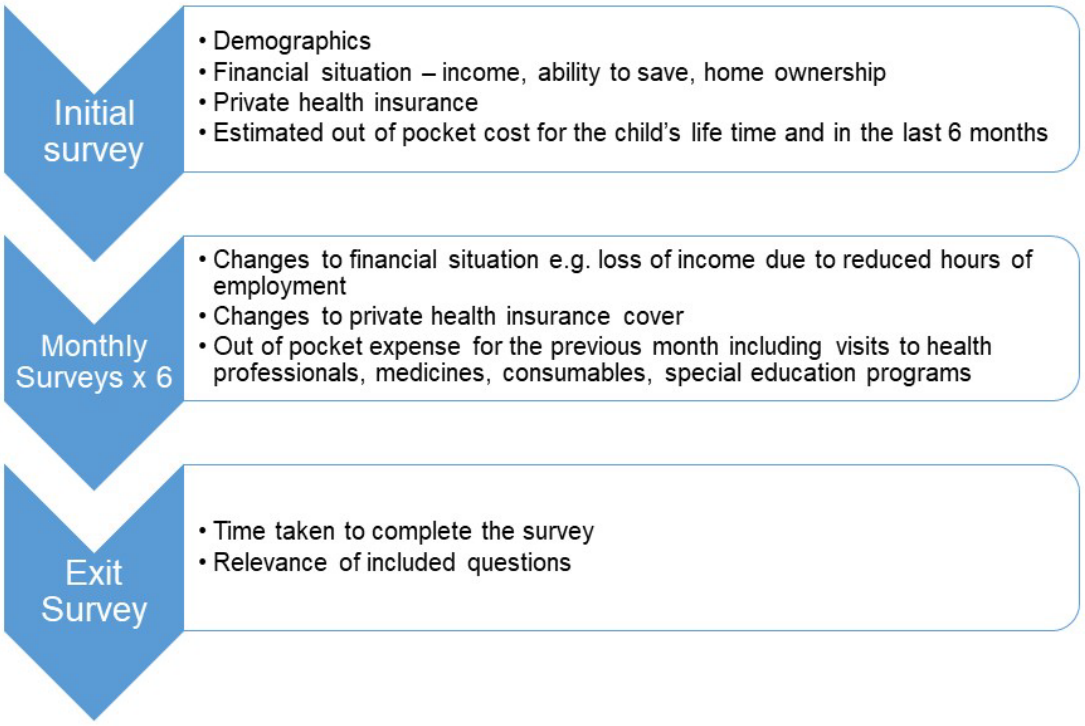
Figure 1. Process of data collection via a series of surveys completed either via an online form or on paper

Figure 2. Health functioning according to the MOF* of the 15 children with mitochondrial disease and tuberous sclerosis



† Out of Pocket Expenses include expenses related to visits to health providers; medicines, supplements and special foods; equipment; travel; education; early intervention programmes; and adjunct therapies such as music therapy.





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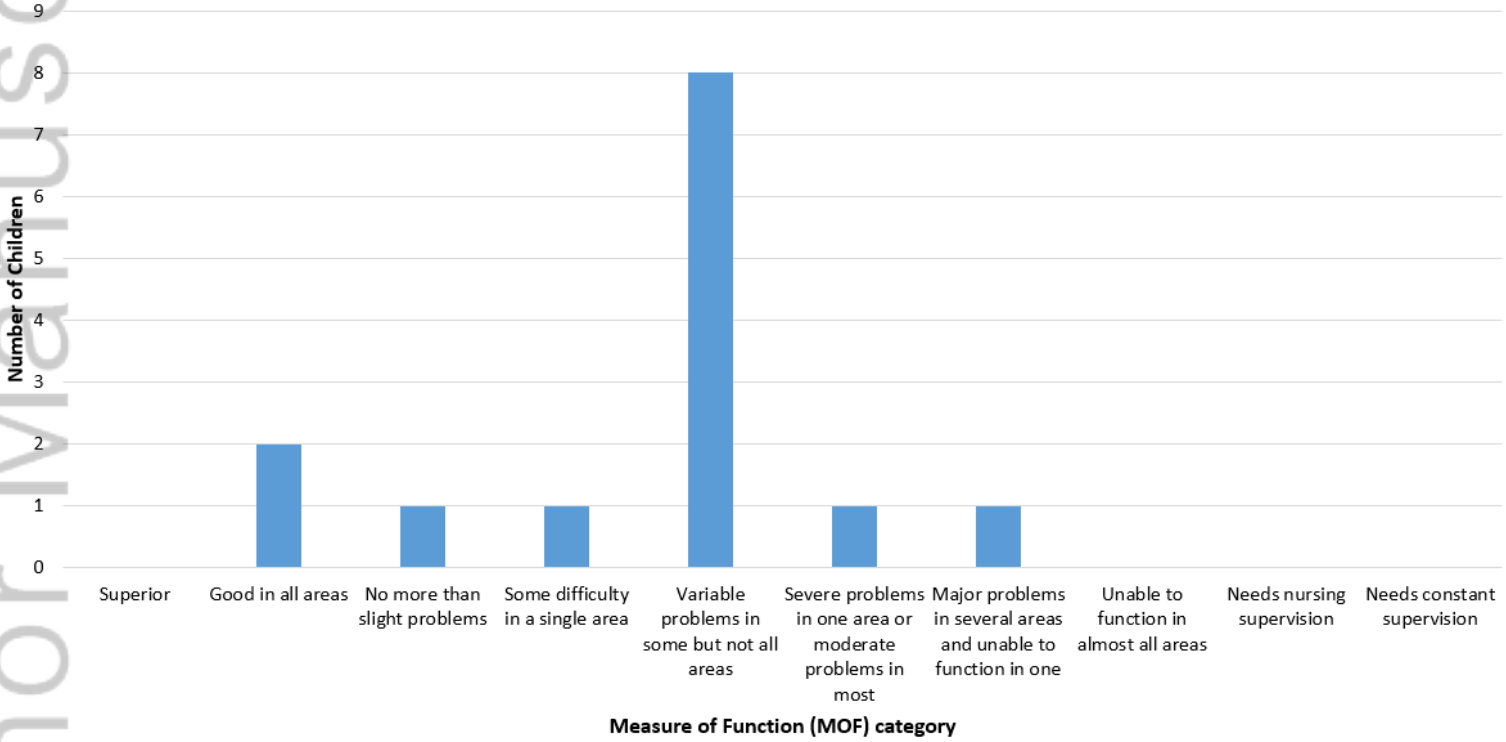

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(vi) Conflict of Interest

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