Parent-Reported Experiences with Health Care for Children with Inherited Metabolic Diseases in Canada: Results from a Multi-Centre Survey

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Study Rationale & Goal

Rationale

- Patient experience with care is a key pillar of high-quality health care systems and is also associated with clinical and safety outcomes[1,2]
- Patient experience is founded on principles of patient-centred care:[3-5]
  - Accessible services, respect and support, clear communication, coordination and continuity of care, involvement of family/friends
- In pediatrics, family-centred care further emphasizes developmental needs and the central role of family[6,7]
  - Strategies to promote family-centred care for children with chronic conditions: co-developed care plans, a 'Medical Home', a designated key provider, provider collaboration, strong family involvement[6-10]
- Our previous qualitative research identified that care for children with inherited metabolic diseases (IMDs) does not always adhere to these principles of family-centered care[11]

Study goal

- To describe experiences with health care for children with IMD in Canada, from parents' perspectives, based on principles of family-centred care

Methods & Respondents to Date

Sample

- Parents/guardians of children (<12 years old) with an IMD enrolled in the Canadian Inherited Metabolic Diseases Research Network (CIMDRN) cohort across 13 participating treatment centres (www.cimdrn.ca) are eligible to participate in a cross-sectional survey (Fig 1)
- Survey currently underway; implemented at participating centres as they receive research ethics board approval

Study Design and Data Collection

- Eligible families invited to participate by mail (contacted up to 4 times[12])
- Questionnaire completed by the person in the family most knowledgeable about the child's care and returned by mail

Questionnaire

- Informed by literature review and qualitative study[11]; pilot tested with 6 parents of children with IMD using cognitive interviews
- *Instruments used:*
  - Family-centred care in the metabolic clinic: Measures of Process of Care (MPOC-20)[13]
  - Care coordination, community service needs, and out-of-pocket expenses: Family Experiences with Care Coordination (FECC)[14] (questions adapted)
- Study-specific questions developed about satisfaction with care in common care settings for IMD; factors considered important to satisfaction with care

Analysis and presentation: Descriptive analysis conducted with data received **and verified** to date (**n=154**); preliminary findings presented

Enrolled in CIMDRN cohort
n = 731
13 centres

↓

Families invited to date
n = 393,
from 13 centres

↓

Surveys completed to-date
n = 210
11 centres

Figure 1. Participant flow chart to date (survey ongoing)

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Results: 1. Participant Characteristics

- A majority of **survey respondents** were the biological mothers of children with IMD, born in Canada, aged 35 -44, lived in a large city, worked full or part-time, and had completed some post-secondary education (Table 1)
- In families with more than 1 child diagnosed with an IMD (20%), one child was the "**index child**"
- The respondent was asked to consider this child when reporting experiences with care
- Index children were born 2006-2015; 51% were male (Table 2)
- MCAD deficiency and PAH deficiency were the most common diagnoses

Table 1. Respondent characteristics

Respondent (n=154)	n (%)
Female (n=153)	135 (88%)
Biological parent (n=153)	150 (98%)
Age	
25-34 years	43 (28%)
35-44 years	91 (59%)
45 years or older	20 (13%)
Working part/full-time	106 (69%)
Education (n=152)	
High school or less	19 (13%)
Vocational/technical	14 (9%)
College/university	93 (61%)
Graduate school	26 (17%)
Born in Canada (n=153)	116 (76%)
Community size (n=151)	
Large city	85 (56%)
Medium city	25 (17%)
Small community	36 (24%)
Other	5 (3%)
> 1 child with IMD	31 (20%)

Table 2. Child characteristics

Index Child (n=154)	n (%)
Male	78 (51%)
Birth Year	
2006-2007	28 (18%)
2008-2009	30 (19%)
2010-2011	25 (16%)
2012-2013	32 (21%)
2014-2015	39 (25%)
IMD (specific disease)	
MCAD deficiency	32 (21%)
Other FAO disorder	14 (9%)
PAH deficiency	38 (25%)
Other AA disorder	9 (6%)
OA disorder	12 (8%)
Other	49 (32%)

AA = amino acid, FAO = fatty acid oxidation, MCAD=medium-chain Acyl-CoA dehydrogenase; OA = organic acid; PAH=phenylalanine hydroxylase

2. Metabolic Clinic Care and Primary Care

97% of index children **received care at a metabolic clinic** in the past year

86% of respondents reported satisfaction with metabolic clinic care for their child

57% of respondents identified a metabolic clinic physician or dietitian as their **child's main health care provider** (Table 3)

Table 3. Child's main care provider[14]

Care provider (n=153)	n (%)
Physician at the metabolic clinic	61 (40%)
Dietitian at the metabolic clinic	26 (17%)
Family physician	41 (27%)
Community pediatrician	15 (10%)
Other	10 (7%)

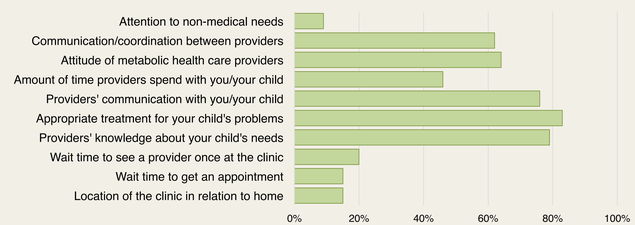

Figure 2. Proportion considering factors "essential" to level of satisfaction with metabolic clinic care (n=149)

Respondents generally reported that their child's care needs were met at the metabolic clinic (MPOC[13] results, Table 4):

- For most MPOC* subscales, needs were perceived as met to a fairly great or great (median score of 5 or 6) extent
- For "Providing General Information", needs were perceived as met to a small or moderate (median score of 3 or 4) extent
- Respondents who stated they were not satisfied with care in the metabolic clinic reported needs being met to a lesser extent

Table 4. Extent to which needs were met for domains of family-centred care (MPOC)[13]

	Enabling & Partnership		Providing General Information		Providing Specific Information		Coordinated & Comprehensive Care		Respectful & Supportive Care	
	N	Med (IQR)	N	Med (IQR)	N	Med (IQR)	N	Med (IQR)	N	Med (IQR)
Overall	133	6.00 (2.33)	124	4.20 (2.90)	130	5.17 (2.67)	142	6.00 (2.00)	142	6.23 (1.40)
Non-Satisfied	18	4.17 (2.00)	17	3.00 (2.00)	19	4.00 (1.67)	19	4.25 (1.50)	18	4.60 (1.55)
Satisfied	115	6.00 (2.00)	107	4.60 (2.95)	111	7.00 (2.33)	123	6.25 (1.75)	124	6.40 (1.40)
p-value**		<.0001		.0043		.0002		<.0001		<.0001

*MPOC subscale scores are calculated as a participant's average rating across the items within a subscale, from 1-7 (higher scores reflect needs being met to a greater extent); **Normally approximated p-values for Mann-Whitney U non-parametric test of the difference in scores for those who were non-satisfied vs satisfied with care; IQR = interquartile range, Med = median.

3. Emergency Department & Hospital Care

33% of children **visited the emergency department** (ED) in the past year, while 20% had experienced an **overnight hospital stay** (Table 5)

- **71% of parents of children who received ED care reported being satisfied with the ED care their child received**
- **Appropriate treatment** was most commonly rated as essential to satisfaction with ED care (>80%, Figure 3)
- **Communication** and **health care provider attitude** were reported as essential to satisfaction by nearly 80% of respondents

Table 5. Child's use of health services, past year

Receipt/frequency of use	n (%)
Emergency Department (n=153)	
Not at all	101 (66%)
1-2 times	38 (25%)
At least 3 times	14 (8%)
Overnight hospital stay (n=154)	
Not at all	122 (79%)
1 time	22 (14%)
At least 2 times	10 (6%)

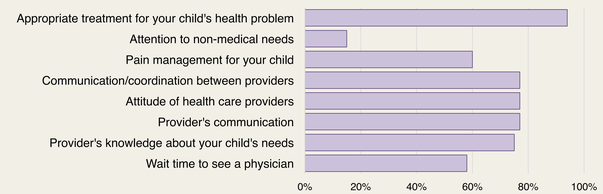

Figure 3. Proportion considering factors to be "essential" to level of satisfaction with ED care (n=52)

4. Blood Laboratory & Pharmacy Care

89% of index children had **visited the blood lab** in the past year

- **74% of respondents were satisfied with care provided by the blood laboratory**
- Only **attitude of care providers** and **communication** were rated as essential to care satisfaction at the blood laboratory by a majority of parents (Figure 4)

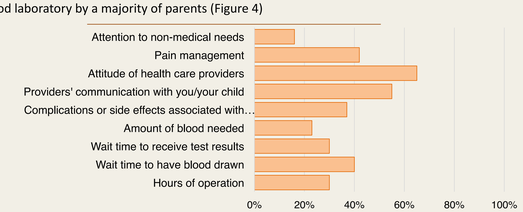

Figure 4. Proportion considering factors to be "essential" to level of satisfaction with care provided by the blood laboratory (n=132)

74% of index children had used the pharmacy in the past year

- **80% of respondents were satisfied with the services provided by the pharmacy**
- **Receiving the correct products and dose, availability of products**, and **timely access** were considered essential to satisfaction by >70% of parents (Figure 5)

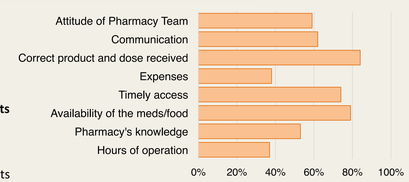

Figure 5. Proportion considering factors to be "essential" to level of satisfaction with pharmacy services (n=112)

5. Care Coordination & Out-of-pocket Expenses

Care coordination (Table 6)

- Of 152 children who used multiple health services, 55% received help in coordinating care (for more than 3/4, help was received from the main health care provider's office)
- Main providers infrequently provided written summaries or shared care plans

Community Services (Table 6)

- 20% of parents reported a need for community services (e.g., community-based intervention programs, home health care)
- Half of those who required community services reported barriers to access (e.g., financial barriers, wait times)

Out-of-pocket expenses (Table 7)

- 52% of respondents reported out-of-pocket expenses related to their child's care
- Most common of such expenses: medications and medical foods or supplements

Table 6. Care coordination & community services, past year[14]

Service queried and denominator*	n (%)
Child used multiple health services, past year (n=152)	85 (56%)
↳ If used multiple services, family received support in coordinating those services (n=84)	46 (55%)
↳ If received care coordination support, source of this support (n=46):	
Main provider's office	15 (33%)
Other	11 (24%)
Both main provider's office and other	20 (43%)
The main provider…	
…provided written summaries of clinic visits (n=152)	51 (34%)
…created a shared care plan (n=153)	17 (11%)
…created an emergency care plan (n=152)	66 (43%)
Family needed / used community services (n=153)	31 (20%)
↳ If needed community services, had trouble accessing them (n = 31)	16 (52%)

*Care coordination questions adapted from the FECC instrument[14]

Table 7. Care expenses paid out-of-pocket by family, past year

	n (%)
Household paid from personal income to care for index child (n = 154)	92 (52%)
↳ If household paid, items paid for (n=92):	
Early intervention programs	11 (12%)
Medical equipment	11 (12%)
Special transportation	0 (0%)
Medications	42 (46%)
Medical foods or supplements	39 (42%)
Other (travel, psychosocial services)	26 (28%)

Discussion

The role of the metabolic clinic as a potential 'medical home' for pediatric IMD

- 57% of parents identified a physician or dietitian at the metabolic clinic as their child's main provider (Table 3)
- Among children requiring multiple health services (56%), 55% received care coordination support, most often (76%) from the main provider's office (Table 6)
- However, while a vast majority of parents (86%) were satisfied with care at the metabolic clinic and perceived family-centred care needs as met in most areas, scores for "Providing General Information" were relatively low (Table 4), indicating unmet needs regarding connections to community services and other resources. Further, few respondents indicated that their main provider developed written visit summaries (34%) or shared care plans (11%; Table 6)
- **These findings indicate that parents saw the metabolic clinic as central to their child's care and generally perceived care there as family-centred. There is potential for the metabolic clinic to play a greater role as a medical home with respect to care coordination and developing shared care plans**

Other health care services, factors essential to satisfaction, and out-of-pocket expenses

- A majority of respondents (>70%) reported that they were satisfied with care within each service outside of the metabolic clinic, including ED care, the pharmacy, and the blood laboratory
- Across all services, receiving appropriate care and high quality communication from providers were most often rated as essential to satisfaction, while timely access and attention to non-medical needs were less often rated as essential (Figures 2-5). More research is needed to determine the most important factors contributing to experiences with care at the blood laboratory (Figure 4)
- More than half of families (52%) reported out-of-pocket expenses related to their child's care (Table 7)

Conclusion and next steps

- Data collection and analysis is ongoing
- We plan to also investigate variation in care experiences across Canadian centres and gaps in care reported by parents whose experiences were less positive
- **Parents of children with IMD in Canada reported satisfaction with their children's care. They most valued appropriate treatment and high quality communication with providers. Gaps in family-centred care relate to care coordination and absence of shared care plans. The impact of out-of-pocket expenses for families is a potentially important concern that requires further investigation.**

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