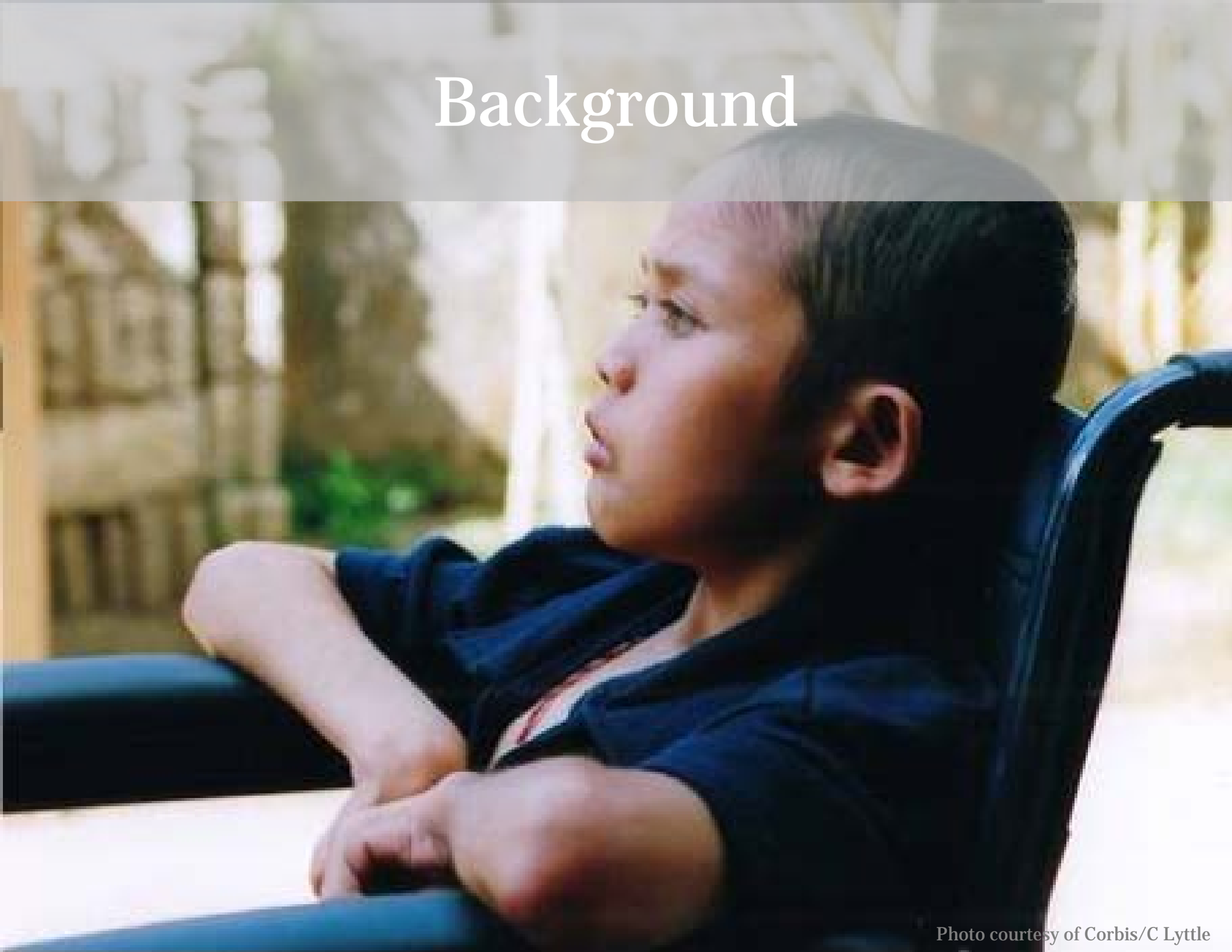


Charting the Territory: Protocol Design & Preliminary Results

Dr. Rose Steele, RN, PhD

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Background



Background: Why Children With Progressive Diseases and Their Families



- Anticipate an unknown lifespan; endure unstable and often painful symptoms; cope with erratic emotional and spiritual crises
- These children and their families are major users of health care services for acute, chronic, and end-of-life care.
- Almost half of non-traumatic deaths in childhood in Canada are from such conditions (~1250 p.a.).
- These children account for about 50% of children receiving palliative care.
- Financial costs associated with caring for these children are estimated at \$1.17 billion annually.
- Yet, there is a paucity of research in this group

Collaborative Effort



Nationwide Research Team



Principal Investigators:

Drs. Harold Siden & Rose Steele

Co-Investigators:

Drs. Rollin Brant, Susan Cadell, Betty Davies, Lynn Straatman

Site Lead Investigators:

Montreal, Toronto, Ottawa, Calgary, Edmonton, Vancouver

Specialist Clinicians:

Neurology, Metabolic, Genetics, Palliative and Complex Care

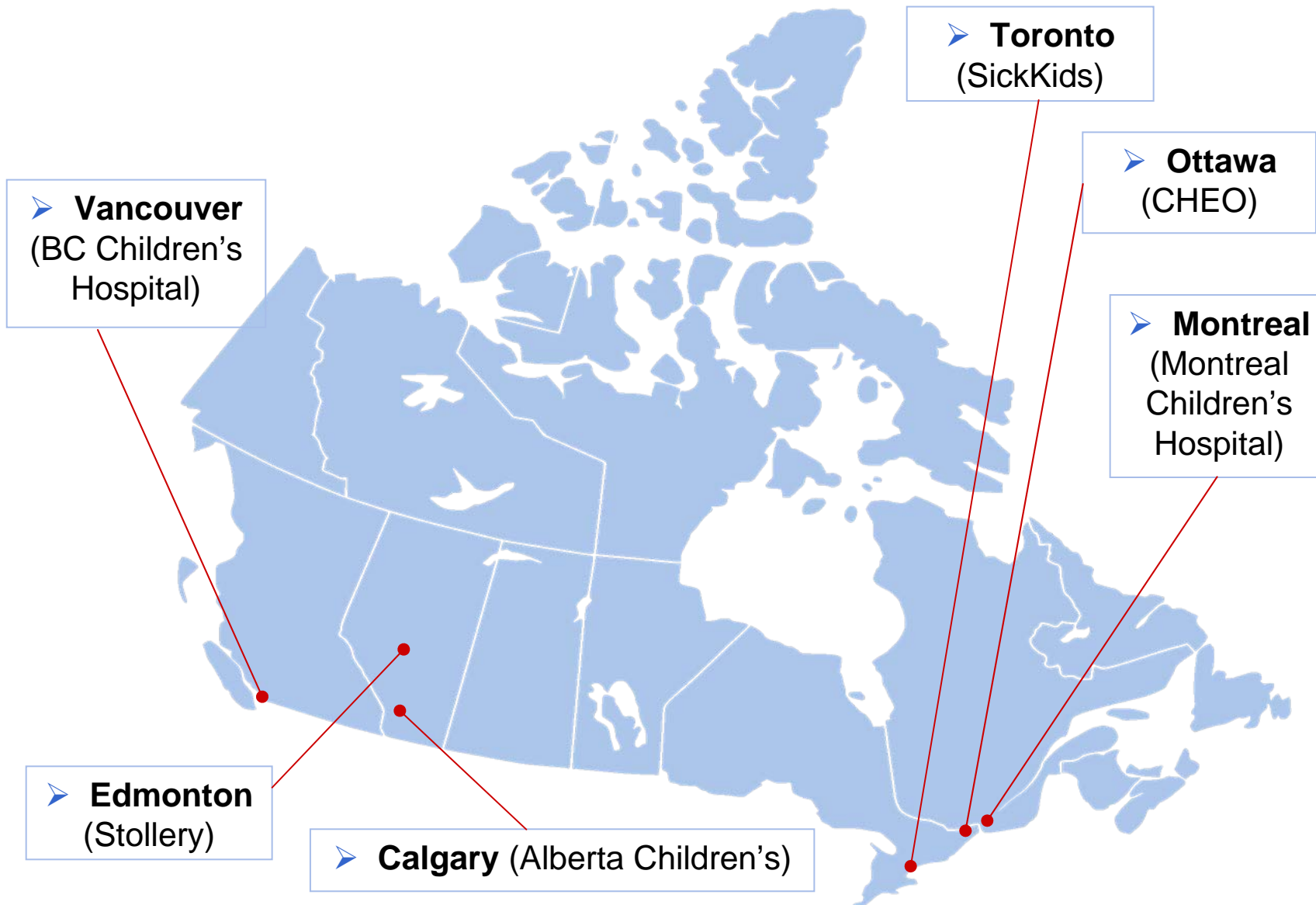
Organizations:

CORD, Canadian MPS Society

Research Coordinator:

Gail Andrews, MEd

Nationwide Participating Sites



Participant Eligibility



- Newly diagnosed children or those with established conditions are eligible (n= 300).
- Age: affected children 0-19; their siblings 7-18.
- Both parents, where possible, will be enrolled.
- Recruitment on an ongoing basis through collaboration with genetic, metabolic, and neurology clinics, as well as palliative care teams and complex care & community physicians in 6 Canadian cities.

Condition Eligibility



Eligible patients include those children with severe metabolic, central nervous system, or chromosomal/genetic conditions who meet all of the following criteria:

- 1) The condition is progressive and not curable
- 2) There is no effective treatment available or treatment is failing in child.
- 3) The condition is known to have/likely to have a genetic or metabolic cause.
- 4) The condition manifests in neurological (CNS) impairment.
- 5) There is a good probability that a child with this condition will die before their 20s.

N.B. Ideally patients will be referred to the study as early in their diagnosis as possible. The child does not have to be at risk of dying in the near-term. Conditions such as “CP”, which are due to a single, identifiable event (e.g., trauma, infection) are not the subject of this study.

Design & Timeline



Design

- Longitudinal cohort (5-year study).
- Descriptive (clinical and psychosocial).
- Correlational (child and family, etc.).

Timeline

- Minimum of 18 months (4 data points).
- Maximum of 48 months.
- Through bereavement (if applicable).

Parallel Data Streams

- Clinical / Biomedical
- Psychosocial and Family Experiences

Clinical Measures: Child



- **Clinical Symptoms Checklist**

Respiratory status, feeding issues, pain, alertness and interaction, sleep issues, seizures, and constipation.

Completed by parent (same parent each time).

Baseline and once a month: telephone or internet.

- **PEDI®**

Completed by RA through observation or parent report.

Baseline and annually: face-to-face or by telephone.



Psychosocial Measures: Parents

- Demographics
- Family functioning (FACES-II)
- Marital Status (Norton)
- Health Outcomes (SF-12)
- Anxiety (STAI)
- Depression (CES-D) [$*\alpha = 0.92$]
- Perceived Stress (PSS)
- Burden Scale [$*\alpha = 0.78$]
- Post Traumatic Growth Inventory (PTGI) [$*\alpha = 0.92$]
- Spirituality (SIBS) [$*\alpha = 0.93$]
- Grief scale
- Impact of participation (at 1 year; again at study end)

Completed separately by each parent who agrees to participate in study.

At baseline and every 6 months: mailed or face-to-face.

* = used in Parent Caregiver study n=273

Psychosocial Measures: Siblings



- **Kidcope**

Completed by siblings between 7 and 18 years of age.

- **Youth Self Report (YSR)**

Completed by siblings between 11 and 18 years of age.

- **Child Behaviour Checklist (CBCL)**

Completed by parent about each participating sibling.

At baseline and every 6 months: mailed or face-to-face.

Preliminary Recruitment



- Study recruitment opened in April 2009, with 55 families across 6 sites in Canada recruited until August 31, 2010.
- The most often diagnosed conditions are amino acid metabolic disorders, mucopolysaccharidoses, severe mitochondrial disease and gene deletions.
- The largest group of children are classified as having a Severe Neurological Impairment (NYD).

Preliminary Recruitment



- Families include 55 mothers; 35 fathers; 34 siblings and 58 affected children (total study participants = 182)
- Of affected children, 30 boys and 28 girls
- Majority of families speak English (73%)

Age of Parents	Mothers	Fathers	Age of Children	Siblings	Affected children
25 to 34	21	6	0 to 6	0	28
35 to 44	23	17	7 to 11	17	17
45 to 60	9	11	12 to 18	17	13
Missing	2	1	Missing	0	0
Total	55	35	Total	34	58
Mean	37.4 yrs	41.4 yrs	Mean	11.3 yrs	7.2 yrs

Preliminary Recruitment



Timeline: 42 completed baseline only
7 contacted for six-months follow-up
5 completed six-months follow-up
1 family contacted for one-year follow-up

Bereaved: 3 families are now in bereavement phase

Baseline data collection: 36 face-to-face, 14 partially done in person and 5 completed baseline over the phone

Online Symptoms Survey: 90.1 % by email/Internet

Thank you!



Thank you to the participating sites:

Montreal Children's Hospital

**CHEO (Children's Hospital
of Eastern Ontario)**

**SickKids (The Hospital for
Sick Children)**

Stollery Children's Hospital

Alberta Children's Hospital

BC Children's Hospital

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For more information on this study, please go to:

www.chartingterritory.com

