Objective

To support CIMDRN’s expert working group in developing self-reported measures of patient and family experiences with IEM by systematically reviewing the published literature to identify existing patient-oriented outcome measures and constructs

To review studies of pediatric patients with complex chronic diseases where care pathways and outcomes are relevant to IEM

Patient/family-reported data collected by CIMDRN will be linked to data collected on clinical interventions and outcomes

Methods

1. Protocol: structured study protocol developed by CIMDRN’s expert working group using existing knowledge of the literature and an iterative consensus process to specify aims, methodological approach and delineate key concepts.

2. Search: comprehensive search strategy iteratively and collaboratively developed between subject matter and information science experts, emphasizing MeSH titles and keywords derived from the protocol.

3. Study selection: pre-specified criteria from the research question and protocol were applied to a 10% random sample of the search yield to inform iterative development of an operationalized screening tool using the PICOS typology:

<table>
<thead>
<tr>
<th>Patients</th>
<th>Pediatric (i.e. ≤18yrs) w/ specific diagnosis of chronic complex diseases relevant to IEM</th>
</tr>
</thead>
<tbody>
<tr>
<td>Interventions</td>
<td>Not applicable</td>
</tr>
</tbody>
</table>

| Comparators | Not applicable |

| Outcomes | Patient/family-oriented, self-reported, and measured by self-administered instrument(s) |

Studies

- Primary research (i.e., no reviews, editorials), 95 study subjects, published between 2002-2012, any research design, peer-reviewed journal articles (i.e., no abstracts).

Two independent reviewers screened all records at the title/abstract and full-text level with discrepancies addressed by discussion and consensus, guided by conceptual definitions derived from the study protocol.

4. Data collection: structured data abstraction form developed and piloted using a 20% random sample of articles included in the study; data were abstracted for this pilot sample by one reviewer and validated by a second researcher with discrepancies resolved by discussion and consensus.

5. Descriptive analyses: a framework specific to the classification of constructs was not identified; several sources e.g., PROMIS Pediatric (Irwin, 2012) & McDowell (2009) informed and corroborated our otherwise qualitative approach to establishing and assigning constructs to domains.

Studies reporting on each domain

- General health & quality of life
- Physical & functional health status
- Social health & relationships
- Mental health
- Disease perceptions & management

Key Messages & Next Steps

Key Messages

- Studies of complex, chronic pediatric disease: many measures of patient/family-reported outcomes representing a broad range of constructs;
- Systematic review is a challenging methodological approach to studies investigating patient-oriented outcomes and measures (e.g., large study yield, reporting details not always adequate to determine self-administration)

Next Steps – completed review will:

- Inform development of tools to collect patient-centered data for CIMDRN;
- Serve as an important resource for researchers in clinical pediatrics, for identifying patient/family-centred outcomes and measurement tools