Parent Reported Outcomes in Young Children With Disorders/ Differences of Sex Development


1. Developmental Endocrinology Research Group, University of Glasgow, Glasgow, UK
2. Susan B Meister Child Health Evaluation & Research Center, Department of Pediatrics, University of Michigan Medical School, USA
3. Health Economics & Health Technology Assessment, Institute of Health & Wellbeing, University of Glasgow, UK

Introduction

• There is paucity of information of health-related quality of life outcomes in parents and young children with Disorders/ Differences of Sex Development (DSD).
• There are a lack of parent reported outcome measures (PRO) that can be routinely assessed in a busy outpatient setting.

Aims

• Develop PRO questionnaires for children <7 years.
• Explore feasibility of integrating questionnaires into routine clinic setting.
• Determine whether the psychosocial impact on parents of a child with DSD is different to that of other endocrine conditions.

Methods

Questionnaires

Parent Report Outcomes Measures (PROs)

- Parent Self-Report (PSR) 0 - <7y
- Parent Proxy-Report (PPR) 2 - 6y

Assessment of parental experiences

- Parental report of child’s experiences

Questionnaire domains and scoring

<table>
<thead>
<tr>
<th>Questionnaire Domains</th>
<th>Items</th>
<th>Derived from</th>
<th>High subscale scores indicate</th>
<th>Sample mean (SD) from reference data</th>
</tr>
</thead>
<tbody>
<tr>
<td>Parent Self-Report</td>
<td>2</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>74.86 (15.93) 69.97 (23.15)</td>
</tr>
<tr>
<td>Communication</td>
<td>5</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>64.03 (24.52) 85.55 (16.80)</td>
</tr>
<tr>
<td>Talking to Others</td>
<td>7</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>55.37 (25.86) 79.14 (14.71)</td>
</tr>
<tr>
<td>Future concerns</td>
<td>4</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>70.39 (28.20) 49.10 (28.31)</td>
</tr>
<tr>
<td>Medication</td>
<td>4</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>72.08 (27.81) 33.38 (25.86)</td>
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<tr>
<td>Surgery</td>
<td>4</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>38.03 (25.12) 81.10 (24.51)</td>
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<tr>
<td>Stigma</td>
<td>10</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>1.76 (0.63) 1.56 (0.44)</td>
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<tr>
<td>Mood</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>2.5 (2.8)</td>
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<tr>
<td>Parent Proxy-Report</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>- - - - - -</td>
</tr>
<tr>
<td>Anxiety</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>- - 50 (10) -</td>
</tr>
<tr>
<td>Depression</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>- - 50 (10) -</td>
</tr>
<tr>
<td>Peer Relations</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>- - 50 (10) -</td>
</tr>
<tr>
<td>Stigma</td>
<td>4</td>
<td>PROMIS</td>
<td>Poorer outcome</td>
<td>2.28 (0.91) 2.05 (0.81)</td>
</tr>
<tr>
<td>Clinic visits</td>
<td>7</td>
<td>QOL-DSD</td>
<td>Better outcome</td>
<td>64.98 (24.49) 78.10 (22.56)</td>
</tr>
</tbody>
</table>

Case recruitment

Children <7y listed to attend DSD and Endocrine Clinics, n=166

- Children attending clinic with caregiver, n=123 (74%)
- Did not attend, n=43
- Interpreter required, n=5
- Unable to approach, n=7
- Unlikely to approach, n=16
- Children of parents approached to complete questionnaires, n=111 (90%)
- Unable to complete, n=16
- Children of parents completing questionnaires, n=95 (86%)
- DSD cases, n=54

Results

• 100% parent acceptability reported.
• Less than 10 minutes to complete.

PSR questionnaire

• Fathers of children with DSD had less stress associated with Clinic Visits (p=0.02) and managing their child’s Medication (p=0.04).
• Parents of children with DSD reported greater Future Concerns in relation to their child’s condition than parents of children with other Endocrine conditions (p<0.05).

PPR questionnaire

• Parents of children with DSD and other Endocrine conditions reported less Depressive symptoms compared to reference data (p<0.05).

Conclusions

• The use of PRO tools in parents and young children with DSD is an acceptable practice.
• PRO tools can be routinely used in the outpatient setting to assess and monitor parent and patient needs.
• DSD was associated with greater parental concerns over a child’s future than other Endocrine conditions - Opportunities for targeted intervention