

**Patient-reported outcomes in children and adolescents
born with esophageal atresia
–condition-specific aspects of health-related quality of life and coping**

Akademisk avhandling
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Avhandlingen baseras på följande delarbeten:

- I. **Dellenmark-Blom M., Chaplin J. E., Gatzinsky V., Jönsson L. & Abrahamsson, K. (2015)**
Health-related quality of life among children, young people and adults with esophageal atresia: a review of the literature and recommendations for future research.
Quality of Life Research, 24, 2433–45
- II. **Dellenmark-Blom M., Chaplin J. E., Gatzinsky V., Jönsson L., Wigert H., Apell J., Sillén U. & Abrahamsson K. (2016)**
Health-related quality of life experiences among children and adolescents born with esophageal atresia: development of a condition-specific questionnaire for pediatric patients.
Journal of Pediatric Surgery, 51(4):563-9
- III. **Dellenmark-Blom M., Chaplin J.E. Jönsson L., Gatzinsky V., Quitmann J.H. & Abrahamsson K. (2016)**
Coping strategies used by children and adolescents born with esophageal atresia – a focus group study obtaining the child and parent perspective.
Child: Care, Health and Development, 42(5):759-67
- IV. **Dellenmark-Blom M., Abrahamsson K., Quitmann J.H., Sommer R., Witt S., Dingemann J., Flieder S., Jönsson L., Gatzinsky V., Bullinger M., Ure B.M., Dingemann C., & Chaplin J.E. (2017)**
Development and pilot-testing of a condition-specific instrument to assess the quality-of-life in children and adolescents born with esophageal atresia.
Diseases of the Esophagus, accepted for publication.



Abstract

Background: Survival rates in children with esophageal atresia (EA) have reached 90-95%, but they are at risk of chronic morbidity, mainly related to esophageal and respiratory dysfunction. Knowledge of condition-specific health-related quality of life (HRQOL) and coping is needed in order to properly understand the impact of the disease and treatment in the child's daily life.

Aim: The aim was to advance knowledge of condition-specific aspects of HRQOL and coping among children and adolescents with EA, including to develop and establish the field test version of a condition-specific HRQOL questionnaire for children with EA.

Methods: The study design followed international guidelines for the development of a patient-reported outcome measure. A systematic literature review of HRQOL in patients with EA was conducted in Pubmed, Cinahl, and PsycINFO, from inception to January 2015. Ten standardized focus groups with 30 Swedish families of EA children 2–17 years old were held to capture the child and parent perspectives of HRQOL and coping. The reported experiences were content analyzed. The HRQOL experiences were used for item generation of pilot questionnaires which, after translation from Swedish to German, were offered to a cross-cultural sample of 89 families of EA children 2–17 years old. Predefined psychometric criteria were used in the pilot test in order to eliminate or revise poor items for the field test questionnaire. The shortened questionnaires were analyzed for internal reliability and convergent and known-groups validity.

Results: Twelve studies (published 1995-2014) were included in the literature review. Five articles (published 2003–2014) described HRQOL among EA children. The studies had employed four different HRQOL questionnaires, and HRQOL results were heterogeneously reported. A standardized condition-specific HRQOL questionnaire for children with EA was needed. Thirty families of children with EA (18 children 8–17 years old, 32 parents of children 2–17 years old) participated in the focus groups (response rate 100%) and produced 1371 HRQOL statements, which formed the basis of two age-specific versions of pilot questionnaires. The 30-item pilot questionnaire for children aged 2–7 years was completed by 34 families (parent report), and the 50-item pilot questionnaire for children aged 8–17 years was completed by 52 families (51 child-report, 52 parent-report) from Sweden and Germany, with a response rate of 96% in the total sample. After omitting poorly performing items, the field test questionnaire for children aged 2–7 years (parent report) consisted of 18 items and three domains (*Eating, Physical health & treatment, and Social isolation & stress*), and the field test questionnaire for children 8–17 years old consisted of 26 items with four domains (*Eating, Social relationships, Body perception, and Health & well-being*). The initial reliability and validity of the shortened questionnaires were adequate. The focus groups also generated 590 coping statements, which revealed nine different coping strategies (*Problem solving, Avoidance, Confronting, Recognizing responsibility, Seeking social support, Positive reappraisal, Emotional expression, Acceptance, Distancing*) that were used in nine situational contexts. The majority of coping experiences (68.6%) were described by children with severe EA and by their parents.

Conclusions: Following the need for advancement in the field, the perspectives of children with EA and their parents have been incorporated into the field test version of the first condition-specific HRQOL questionnaire for EA children. The foremost HRQOL issues are related to eating, physical health and social dimensions; in children 8–17 years old, body perception issues are also prominent. Condition-specific coping strategies seem to be adopted at an early age and may affect HRQOL. The findings shed light on issues of relevance for follow-up routine care, and can improve the evaluation of pediatric surgical care and treatment. Future research is warranted.

Keywords: Esophageal atresia, Rare condition, Patient-reported outcome, Quality of life, Coping, DISABKIDS