

Patient Reported Outcome Measurement in children and adolescents with Tracheomalacia and Esophageal Atresia

Abstract

Background: Tracheomalacia (TM) and esophageal atresia (EA) are rare birth defects that often occur together. Although rare, EA is one of the most common congenital malformations. It is well known that respiratory symptoms occur among these children. In recent years, a quality-of-life questionnaire for EA was developed focusing on the general aspects of the EA. However, there is barely any focus on the respiratory comorbidity in EA with TM.

Aim: This study aimed to assess respiratory symptoms in patients with TM and EA and develop a lung specific Patient Reported Outcome Measure (PROM) for children and adolescents with EA and TM.

Methods: A concept of symptoms was developed based on literature and interviews with patients, parents, and clinicians. Exclusion criteria were patients with competing lung diseases other than tracheomalacia. The data from the interviews were analyzed and put together as a questionnaire for patients and clinicians to review and evaluate.

Preliminary results: 12 patients were included, and with a total of 19 interviews conducted, involving 3 clinicians, 14 parents, 4 children, and 2 adults with EA/TM. The average age of the patients was 10.75 years (SD 10.47; IQR 0-36 years). The outcomes identified 8 domains with reported percentages: breathing (75%, n=9), coughing (100%, n=12), illness (92%, n=11), sleep (67%, n=8), physical activity (75%, n=9), Esophageal atresia (67%, n=8), mucus (75%, n=9) and parents and welfare (92%, n=11). Respiratory related symptoms or outcomes were nightly coughing (33%, n=4), sound of coughing (75%, n=9), coughing more than other (75%, n=9), coughing attack (50%, n=6), phlegm (67%, n=8), illness takes longer to recover (58%, n=7), a cold is merely a cold (58%, n=7), pneumonia (58%, n=7), preventative antibiotics during winter seasons (58%, n=7), have or had asthma medication (17%, n=2), hospitalization (42%, n=5), intensive care unit (25%, n=3), apnea periods (25%, n=3), affected sleep due to EA/TM (50%, n=6), poor sleep quality affecting daily life activity (50%, n=6), unable to inflate a balloon (42%, n=5), distinctive breathing noise (67%, n=8) and visible thoracic retractions (25%, n=3).

A total of 21 key points related to airways emerged from the interviews were used for generating items for the questionnaire.

Secondary findings revealed that parents received compensation for loss of earning (7 patients). Additionally, for 5 patients the parents expressed concerns or unable to entrust the care of their child to others during the first few years of their childhood. Furthermore, parents of 3 patients indicated limitation in traveling outside of Denmark's borders.

Conclusion: Although the reported outcomes for the interviews showed saturation, we would need to validate and test the questionnaire both at our center, nationally and cross culture to ensure applicability.