

## Progression of Feeding Abilities in Children with Infantile Pompe Disease Receiving Enzyme Replacement Therapy

Swift G<sup>1</sup>, Broomfield A<sup>2</sup> and Lozano S<sup>1</sup>

<sup>1</sup>Great Ormond Street Hospital, London, UK

<sup>2</sup>Royal Manchester Children's Hospital, Manchester, UK

Originally Presented in UKSRG-2016 Conference held at Institute of Child Health, London, UK during February 4-5, 2016.

### Abstract

**Purpose:** Infantile Pompe disease (IPD) is a genetic metabolic disorder causing progressive neuromuscular weakness and dysphagia. Enzyme replacement therapy (ERT) has extended life expectancy. However, due to low prevalence and the infancy of ERT there are no long-term studies of dysphagia outcomes.

**Methods:** Feeding outcomes of 15 infants treated with ERT at a UK tertiary centre were reviewed. These included method of feeding (oral, oral+tube, non-oral) and survival time post-treatment. Comparison was made between feeding methods at three key time points; ERT initiation, ERT+6 months, and most recent assessment age >2 (MRA). Kaplan-Meier survival analysis was used to examine the relationship between feeding at ERT initiation and survival at 2 years.

**Results:** Six children died before age 2. Children who were fully or partially orally fed at ERT initiation had higher survival rates at 2 years compared to those requiring full tube feeding (100%, 67%, 43% respectively) although this result was not significant. Of the remaining 9, more children required tube feeding at MRA than at ERT+6months. All patients who required some non-oral feeding before age 6 months went on to require long-term tube support.

**Conclusions:** Feeding in children on ERT changes over time and initial improvements may not be maintained. This demonstrates the need for careful and ongoing monitoring of dysphagia in children with IPD. The results are informative for counselling families regarding feeding prognosis and consideration of early gastrostomy insertion. Further research is required to evaluate whether feeding ability at ERT initiation is associated with overall survival.

**Citation:** Swift G, Broomfield A, Lozano S. Progression of Feeding Abilities in Children with Infantile Pompe Disease Receiving Enzyme Replacement Therapy. Proceedings of UKSRG-2016; 2016 Feb 4-5; London, UK. J Oral Health Dent. 2017;1(S1):A0013.