Economic Burden of Care and Treatment Options for Patients With Rett Syndrome: Two Systematic Literature Reviews

OBJECTIVE
To identify clinical trials in Rett syndrome and assess the economic burden of care for patients with this condition

INTRODUCTION
• Rett syndrome is a rare, non-inherited, genetic, post-natal, neurological disorder that occurs almost exclusively in girls and leads to severe impairments in a child’s development. It is characterized by apparently normal development for the first 6 to 18 months of life, followed by the loss of acquired fine and gross motor skills and the ability to engage in social interaction, and the development of stereotypic hand movements.

METHODS
• Two systematic literature reviews related to Rett syndrome were performed on 25 June 2018

1) ECONOMIC BURDEN
• Outcomes of interest were costs (direct and indirect medical costs), medical resource use (hospital admissions, length of stay, physician and specialist visits, medications) and non-medical resource use (lost productivity and homecare or caregiver’s time)

RESULTS
ECONOMIC BURDEN
• Four studies were included in the qualitative synthesis of 133 articles identified from database searching

DIRECT MEDICAL RESOURCE USE – LENGTH OF STAY
• Labianca, et al. (2013)
  - A mean length of inpatient stay of 18.2 days (range: 8–42) was seen for 11 patients with Rett syndrome who underwent a surgical correction of scoliosis. (posterior instrumented fusion of the curve)
  - Complications had considerable implications on the length of hospital stay; patients without complications were discharged on average 8.7 days postoperatively, whereas those with complications remained inpatients for an average of 21.8 days.

DIRECT MEDICAL RESOURCE USE – HOSPITAL ADMISSION
• Union, et al. (2016)
  - A total of 652 articles were identified, out of which 28 publications (20 studies) were included in the qualitative synthesis

STUDY DESIGN AND POPULATION CHARACTERISTICS

TREATMENT OPTIONS
• A total of 625 articles were identified, out of which 28 publications (20 studies) were included in the qualitative synthesis

STATISTICALLY SIGNIFICANT RESULTS FROM CLINICAL STUDIES

LIMITATIONS OF THE STUDIES
• In the economic burden analysis, studies by Union, et al. (2016) and Labianca, et al. (2013) were published as conference abstracts. Findings should be interpreted cautiously as detailed information on these studies is lacking
• Almost half of the treatment options studies followed a crossover design and almost half had small sample sizes of 12 participants or fewer. Outcome data are limited, and evidence for PediQLTM, HAI, Peds-m, and Peds-Q are lacking

CONCLUSIONS
• Data on the costs of Rett syndrome management are sparse. Economic burden data were available only for direct medical resource use and direct medical costs; data on non-medical and indirect resource use and costs within the last 5 years were not identified
• There is an unmet need for safe and effective treatments for Rett syndrome
• Available treatment options address symptoms but do not improve disease progression
• Treatments that target the underlying cause of disease (e.g., gene-replacement therapy) could improve quality of life and overall prognosis for patients with Rett syndrome
• Further research is needed to better understand the impact of medical interventions for patients with Rett syndrome and their potential to reduce costs and/or increase utility

**Note:** The above text is a summary of the findings and methods discussed in the original document, focusing on the key points and the economic burden and treatment options for patients with Rett syndrome. The full text is available for further detail and context.