Abstract Number: 5PSQ-065

EVALUATION OF RESPIRATORY FUNCTION AND SAFETY OF ELEXACAFTOR/TEZACAFTOR/IVACAFTOR IN CHILDREN AND ADOLESCENTS WITH CYSTIC FIBROSIS FOLLOWED AT A REGIONAL REFERENCE CENTER

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Background and importance

Elexacaftor/Tezacaftor/Ivacaftor (ETI) represents a highly potent cystic fibrosis (CF) transmembrane conductance regulator (CFTR) modulating treatment for individuals with CF who have at least one F508del mutation. However, there is a paucity of data on the safety and efficacy of ETI in in children and adolescents.

Aim and objectives

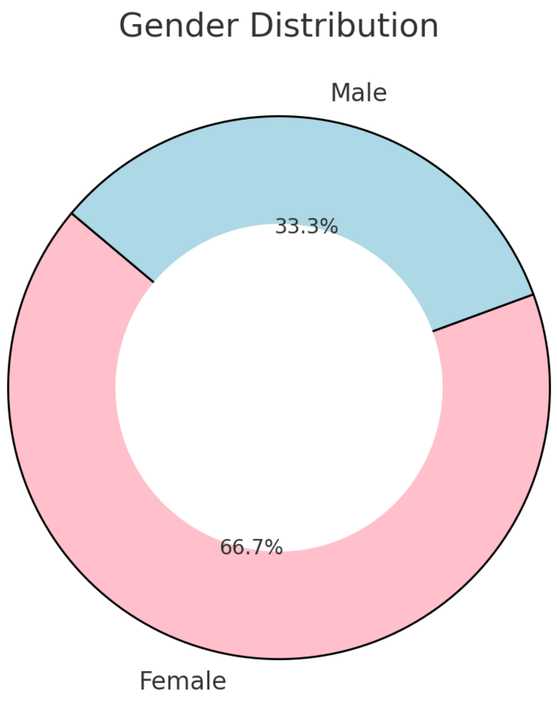
This study aimed to evaluate the improvement in lung function and the safety of ETI in pediatric setting after 12 months of treatment.

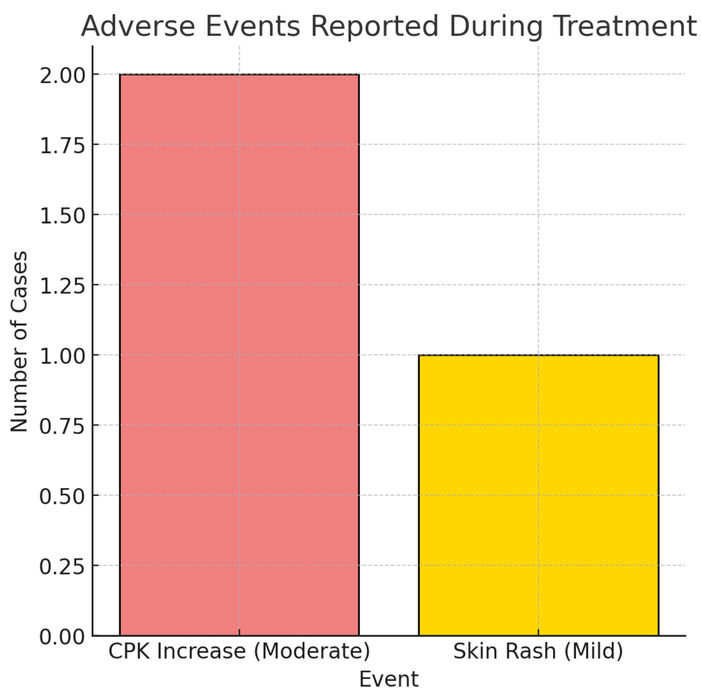
Material and methods

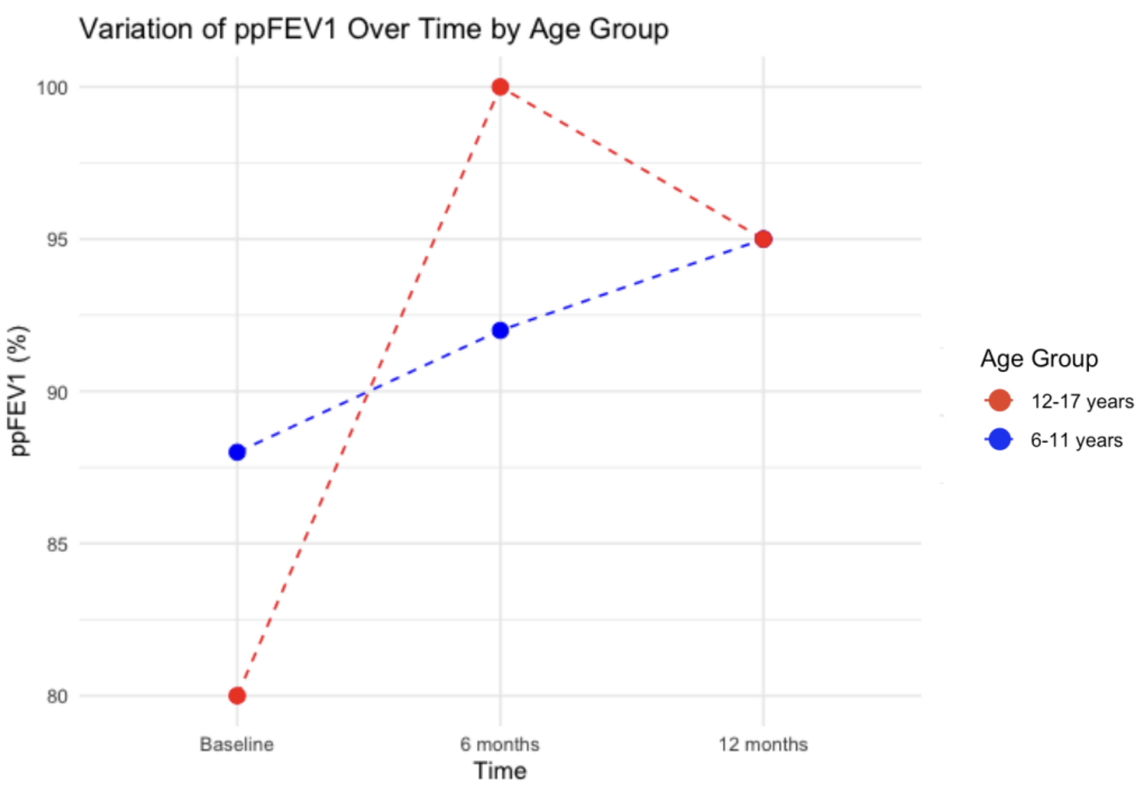
This observational, single-center, retrospective study analyzed data from 27 pediatric patients treated with ETI at a Regional Reference Center for Cystic Fibrosis between July 2021 and November 2023. Patients were divided into two age groups: 6-11 years (N=10) and 12-17 years (N=17). The primary outcome was the change in the percent predicted forced expiratory volume in one second (ppFEV1) at 6 and 12 months. Secondary outcomes included the evaluation of adverse drug reactions (ADRs), mortality rates, and transplantation rates. Statistical analyses were performed using Mann-Whitney, Kruskal-Wallis, and one-way ANOVA tests using R software.

Results

The average age of the 27 patients analyzed was 12 (7,17) years, with 66.7% being female. After six months of ETI therapy, an increase in ppFEV1 of 5% and 22.7% from baseline was observed for the 6-11 years and 12-17 years groups, respectively, with stabilization of values at the second follow-up after 12 months of treatment [ppFEV1=96 vs ppFEV1=94.5; ($p<0.001$)].
The statistically significant difference in ppFEV1 between the two groups is a consequence of the more critical baseline clinical conditions observed in the 12-17 age group. During the study, no transplants or deaths occurred among patients treated with ETI. Three ADRs were recorded: two cases of moderate increase in creatine phosphokinase (CPK) and one mild episode of skin rash. All reactions were resolved by dosage reduction.







Conclusion and relevance

The results showed a significant improvement in respiratory function in patients after 12 months of therapy, suggesting that early treatment with ETI could prevent severe long-term complications. However, further long-term observations and real-world data are needed to confirm the safety and efficacy of the treatment in children and adolescents with CF.

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