Early insurance coverage of elexacaftor/tezacaftor/ivacaftor for cystic fibrosis in children 6 to 11 years of age

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Abstract

Introduction: Elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) is a highly effective cystic fibrosis transmembrane conductance regulator (CFTR) modulator therapy (HEMT) originally approved in 2019 for use in patients 12 years of age and older with at least one F508del mutation or a mutation in the CFTR gene that is responsive based on in vitro data. This report describes coverage of ELX/TEZ/IVA for CF in children 6 to 11 years of age prior to the recent age expansion by the Food and Drug Administration (FDA). Methods: An email was sent to pharmacists and pharmacy technicians through the CF Foundation LISTSERV and all responses regarding ELX/TEZ/IVA use in children 6 to 11 years of age were collected. Results: A total of 65 children from 15 CF care centers were included in the study. A total of 55 children had early coverage of ELX/TEZ/IVA for an overall approval rate of 84.6%. The median time to approval was 15 days. Lung function and weight outcomes were also positive. Conclusions: Early insurance coverage of ELX/TEZ/IVA for CF in children 6 to 11 years was achieved regardless of insurance type and should be considered an option for patients in need of HEMT therapy.

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Methods: An email was sent to pharmacists and pharmacy technicians through the CF Foundation LISTSERV and all responses regarding ELX/TEZ/IVA use in children 6 to 11 years of age were collected.

Results: A total of 65 children from 15 CF care centers were included in the study. A total of 55 children had early coverage of ELX/TEZ/IVA for an overall approval rate of 84.6%. The median time to approval was 15 days. Lung function and weight outcomes were also positive.

Conclusions: Early insurance coverage of ELX/TEZ/IVA for CF in children 6 to 11 years was achieved regardless of insurance type and should be considered an option for patients in need of HEMT therapy.

To the Editor,

Introduction

The highly effective cystic fibrosis (CF) transmembrane conductance regulator (CFTR) modulator therapy (HEMT), elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) was approved in October 2019 for patients 12 years of age and older with at least one F508del mutation or a mutation that is responsive based on *in vitro* data. Recently released phase 3 data for ELX/TEZ/IVA use in children 6 to 11 years of age showed positive safety and tolerability results, as well as secondary endpoint improvements in ppFEV1, sweat chloride, Cystic Fibrosis Questionnaire-Revised (CFQ-R) scores, body mass index (BMI), as well as number of pulmonary exacerbations and hospitalizations.[1] Studies have also documented that initiating HEMT at an earlier age has the potential to alter the progression and prognosis of CF.[2-4] Based on this information, a supplemental New Drug Application to expand the use of ELX/TEZ/IVA to include children 6 to 11 years was submitted in early 2021 with a targeted decision date of June 2021. Given the positive study results and the potential time-sensitive nature of its use, several CF centers submitted prior authorizations (PA) for ELX/TEZ/IVA use in younger children. The aim of this project was to describe early insurance coverage of ELX/TEZ/IVA for CF in children ages 6 to 11 years.

Methods

An email was sent to pharmacists and pharmacy technicians through the CF Foundation LISTSERV and all responses regarding ELX/TEZ/IVA use in children 6 to 11 years of age were collected. Data included patient demographics (age, genetic mutations, weight, previous CFTR modulator use and regimen), insurance type (state or commercial), prior authorization attempts and overall approval or denial, and ELX/TEZ/IVA regimen. Descriptive statistics were used to analyze data and a prior authorization approval rate was calculated.

Results

A total of 65 patients from 15 different CF care centers were included in the analysis. The average age of the patients was 10 (1.5) years and ranged from 6-11.8 years. Most patients were male (52.8%) and homozygous F508del genotype (56.9%). (Table 1) Thirty-six patients (55.4%) were on a different CFTR modulator prior to ELX/TEZ/IVA, while 29 patients (44.6%) were modulator naive. Patient insurance was evenly split with 32 patients (49.2%) having commercial coverage and the same number having state-funded insurance plans, such as Medicaid.

A total for 55 children were able to obtain early access to ELX/TEZ/IVA. The overall approval rate was 84.6%. The median time until insurance approval was 15 days. Approval occurred on the initial prior authorization (PA) for 19 (29.2%), 22 (33.8%) on first appeal, 10 (15.4%) on the second appeal, and 2 (3%)

on the third appeal. The most common types of appeals utilized were letter of medical necessity (LMN) and external review (ER). For first appeal, there were 39 LMN, 3 peer to peer reviews (P2P), and 2 additional information requested. On second appeal, there were 6 P2P, 2 LMN, 6 ER, 1 additional information, 2 LMN/ER combination, and 1 family LMN. Those that required third appeals were 2 ER.

There was some variation in the dose of ELX/TEZ/IVA prescribed for these patients. The majority, 30 (54.6%), received full dose, 12 (21.8%) received half dose, and 8 (15%) had a dose titration. Drug interactions accounted for 3 of the dose adjustments. The average duration of ELX/TEZ/IVA at data collection was 0.5 (0.4) years. After the first quarter of ELX/TEZ/IVA, patients had an average weight gain of 2.5 kg and mean change in ppFEV1 of 13.3%. After the second quarter of therapy, average weight gain was 4.5 kg from baseline and mean change in ppFEV1 was 16.4% from baseline.

Discussion

Despite the potential benefits of initiating HEMT at an earlier age, obtaining insurance coverage for "off label" use of a medication can be difficult. Based on these results, we found a relatively high overall approval rate for early coverage of ELX/TEZ/IVA for CF in children 6 to 11 years of age. Early insurance coverage was achieved regardless of insurance type.

Most cases required at least one appeal to obtain approval. Additionally, many care centers were required to submit supporting patient data and evidence of clinical need. The increased workload required for obtaining approval may be reduced through collaboration between CF care centers on successful appeal letters.

While ELX/TEZ/IVA was approved by the FDA in June 2021 for children 6 to 11 years of age, the results of this study may still be applicable for patients looking for earlier initiation of ELX/TEZ/IVA or patients seeking off label use based on their genetic mutation.

One limitation of this study was that not all CFF-accredited care centers provided information. Although this study found a high overall approval rate regardless of payer type, certain CF care centers may find more difficulty depending on the predominant insurance providers in their geographical region.

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