London, 20 September 2018
EMA/CHMP/583550/2018
Committee for Medicinal Products for Human Use (CHMP)

Assessment report for paediatric studies submitted according to Article 46 of the Regulation (EC) No 1901/2006

Xolair

omalizumab

Procedure no: EMEA/H/C/000606/P46/064

Note

Assessment report as adopted by the CHMP with all information of a commercially confidential nature deleted.

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1. Introduction

In July 2018, the MAH submitted a completed paediatric study for Xolair, in accordance with Article 46 of Regulation (EC) No1901/2006, as amended.

A short critical expert overview has also been provided.

2. Scientific discussion

2.1. Information on the development program

The MAH stated that Study CIGE025APT02 (A 12-months multi-center, non-interventional prospective study (with a retrospective cohort) to investigate the steroid-sparing effect of Xolair® (omalizumab) in patients with severe asthma treated under Portuguese medical practice) is a stand alone study.

2.2. Information on the pharmaceutical formulation used in the study

Xolair, marketed in Portugal.

2.3. Clinical aspects

2.3.1. Introduction

The MAH submitted a final report for:

- Study CIGE025APT02: A 12-months multi-center, non-interventional prospective study (with a retrospective cohort) to investigate the steroid-sparing effect of Xolair® (omalizumab) in patients with severe asthma treated under Portuguese medical practice

2.3.2. Clinical study

Study CIGE025APT02: A 12-months multi-center, non-interventional prospective study (with a retrospective cohort) to investigate the steroid-sparing effect of Xolair® (omalizumab) in patients with severe asthma treated under Portuguese medical practice

Description

Methods

Objective

The primary objective of the study was to evaluate the prescribed OCS sparing effect of Xolair (omalizumab) treatment in patients with uncontrolled persistent allergic asthma in routine Portuguese real-life clinical settings following 12 months of treatment. Some secondary and exploratory objectives were also included.

Study design

Study CIGE025APT02 was a single armed non-interventional, prospective study aimed primarily to provide additional data regarding the effect of add-on Xolair® therapy in Portuguese patients with uncontrolled severe persistent allergic asthma receiving oral corticosteriods (OCS), either as chronic maintenance treatment or in the form of frequent courses to manage exacerbations, following local standard clinical practice, for 12 months. Additionally, this study evaluated the changes in effectiveness parameters, such as asthma-related medical resource utilization, asthma control and patient health-related quality of life, associated to Xolair® as well as its potential inhaled corticosteroid (ICS) sparing effect.

Study population /Sample size

The study population consisted of male and female patients (≥ 12 years of age) with uncontrolled severe persistent allergic asthma and followed in the study sites (20 sites) as outpatients. A total of sixty (60) patients were enrolled in the study and inference statistics were performed in a cohort of 53 patients (maximum) which included 1 paediatric patient (12 years of age).

Treatments

Xolair, used according to the approved SmPC.

Outcomes/endpoints

- Regarding maintenance OCS treatment:
    - Percentage of patients that stopped maintenance OCS.
    - Percentage of patients that reduced maintenance OCS.
    - Mean daily OCS dose.
- Regarding acute OCS treatment in cycles (for exacerbation management):
    - Number of OCS cycles/year.
    - Mean daily OCS dose.
- Overall (acute and maintenance) mean daily OCS dose.

Results

Efficacy results

Inference analyses of the data demonstrated that Xolair® treatment for 12 months significantly decreased the number of OCS cycles ($p<0.001$) and maintenance OCS treatments (MOCs; $p<0.001$) prescribed at Visit 3 versus Visit 0 together with a reduction in the mean daily dose of acute OCS (AOCs; $p<0.001$), thereby showing an overall OCS sparing effect.

Based on the data reported for this single patient, inference analyses showed an overall OCS sparing effect as demonstrated by a reduction in the number of oral corticosteroid (OCS) cycles and maintenance OCS treatments (MOCs) prescribed at the end of the Xolair® treatment period (Visit 3 versus Visit 0) together with a reduction in acute OCS (AOCs) consumption.

The data also showed a decrease in the number of ICS treatments as well as the number of clinically significant asthma exacerbations. No medical resource utilization (i.e. number of hospitalizations, emergency room visits that did not lead to hospitalization and unscheduled doctor visits) were reported for this patient.

Asthma scores in this patient, as demonstrated by analysis of Asthma Control Test (ACT) data, was also shown to improve after 12 months of Xolair® treatment. The Control of Allergic Rhinitis and Asthma Test (CARAT) was not performed in this patient. Quality of life (Mini- AQLQ) was not applicable for this patient given the patient was a minor.

Safety results

At the end of the Xolair® treatment period, AEs were reported by 43 (40.2%) patients and SAEs reported by 2 (1.9%) patients; none of which were suspected to be related to Xolair®. Overall, Xolair® showed a favourable safety profile, with no safety concerns.

No adverse events were reported for the single paediatric patient.

2.3.3. Discussion on clinical aspects

As only one patient was included this is to be regarded as a case report of Xolair use with positive outcome.

3. Rapporteur's overall conclusion and recommendation

The inclusion of the single paediatric patient is study CIGE025APT02 is noted. No regulatory action is warranted.

☒ Fulfilled:

No regulatory action required.

4. Additional clarification requested

N/A