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Human Medicines Evaluation Division

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/P46/069

Note

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

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1. Introduction

On 14 May 2019, 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.

***CHMP comment**: It is noted that the CV submitted is for a different expert than the writer of the critical expert overview. However, considering that no regulatory actions are to be taken this issue is not pursued.*

2. Scientific discussion

2.1. Information on the development program

The MAH stated that CIGE025D1401 (a post-marketing surveillance study which overall purpose was to evaluate the safety and efficacy of Xolair in Japanese patients ≥15 years old with bronchial asthma under routine clinical practice using the revised dosing table implemented after the initial indication approval) is a stand-alone study.

2.2. Information on the pharmaceutical formulation used in the study

Xolair for subcutaneous (s.c.) injection 75mg/100 mg, as authorised in Japan.

2.3. Clinical aspects

2.3.1. Introduction

The MAH submitted a clinical study report for:

- Study CIGE025D1401, entitled "Xolair for s.c. injection 75 mg/150 mg, specified drug-use survey (dosing regimen covered by the added parts of dose conversion table).

2.3.2. Clinical study

Description

Study CIGE025D1401 was a post-marketing surveillance study. The overall purpose was to evaluate the safety and efficacy of Xolair in Japanese patients ≥15 years old with bronchial asthma under routine clinical practice using the revised dosing table implemented after the initial indication approval. Study CIGE025D1401 was performed by use of a survey and was designed to be conducted by a central registration method with an observation period of 52 weeks.

Methods

Objective

The objective of the study was to utilize a survey to evaluate the safety and efficacy of Xolair in adult patients with bronchial asthma under routine clinical practice according to the changed dosing table. The study was conducted because no Japanese adult patients with bronchial asthma had received

Xolair with the revised dosing regimen and there was limited information available from other clinical studies conducted outside of Japan.

For efficacy, physician's global evaluation of treatment effectiveness (GETE) was the primary endpoint with pulmonary function test by spirometry, frequency of asthma symptoms, and events related to asthma exacerbation compared to pre-treatment as secondary endpoints.

Study design

The survey used in the study was designed to be conducted by a central registration method, with an observation period of 52 weeks. Data were to be collected via electronic data capture system at the end of each observation period (i.e., 16 and 52 weeks after the start of Xolair treatment).

Data collected on the survey included patient characteristics, Xolair usage, asthma treatments, concomitant medications, concomitant diseases, asthma exacerbation and related episodes, asthma symptoms, pulmonary function testing, adverse events, laboratory evaluations and physician's GETE. The information was entered on case report form pages and sent to the sponsor where the post-marketing surveillance department checked the data.

Omalizumab was administered in accordance with the approved local label and from available commercial sources using the standard local clinical practice and with clinical judgement to make the decision to subscribe.

CHMP comment: *The revised dosing table is similar to the table in the SmPC for Xolair authorised in EU.*

Study population /Sample size

The study population consisted of male and female patients who met the following three conditions:

- First time users of Xolair in adult patients with bronchial asthma (those who had poorly controlled refractory asthma symptoms despite conventional therapies).
- Patients who started Xolair treatment with the dosing regimen covered by the new dosing table.
- Patients aged ≥15 years at the initiation of treatment.

Results

Study results for patients less than 18 years of age

A total of 6 patients aged <18 years were registered with the survey. Four patients were 15 years old and 2 patients were 17 years old.

Of the 6 patients 4 were rated as "effective" (66.67%) and 2 as "not effective" (33.33%) in terms of treatment effectiveness at final assessment. Of the 4 patients rated as "effective" 2 patients each were considered "excellent" and "good". Both of the patients rated as "not effective" were considered "moderate".

One patient (15 years old) experienced asthma as an adverse event (AE) was hospitalized but recovered. No other AE or adverse events of special interest were reported in the patients <18 years.

CHMP comment:

Due to the small number of patients and open design no firm conclusions can be drawn from this study. There were however no unexpected findings. The results on efficacy and safety in the paediatric population appear similar to the overall population.

2.3.3. Discussion on clinical aspects

The submitted study was a post marketing surveillance study with an overall purpose to evaluate the safety and efficacy of Xolair in Japanese patients ≥15 years old with bronchial asthma under routine clinical practice using a revised dosing table implemented after the initial indication approval.
The results on efficacy and safety in the paediatric population (in this study 15-18 years old) were similar to the overall population and no unexpected safety concerns were identified.

The investigation of paediatric use was not a primary objective of this study and the paediatric data in the clinical overview is descriptive only.

Due to the small number of patients and open design no firm conclusions can be drawn from this study. There were however no unexpected findings. The results on efficacy and safety in the paediatric population appear similar to the overall population.

The results of this study do not change the overall benefit/risk profile of Xolair (omalizumab) in the paediatric population.

3. CHMP overall conclusion and recommendation

The presented data do not change the B/R profile for Xolair which remains positive. No regulatory action is warranted.

☒ **Fulfilled:**

No regulatory action required.