25 April 2025
EMADOC-1700519818-1911843
Human Medicines Division

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

Cibinqo

Abrocitinib

Procedure no: EMEA/H/C/005452/P46/01153/1

Note

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

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Status of this report and steps taken for the assessment			
Current step[1]	**Description**	**Planned date**	**Actual Date**
☐	Start of Procedure	25 February 2025	25 February 2025
☐	CHMP Rapporteur AR	31 March 2025	17 March 2025
☐	CHMP comments	14 April 2025	n/a
☐	Updated CHMP Rapporteur AR	16 April 2025	n/a
☒	CHMP outcome	25 April 2025	25 April 2025

Table of contents

1. Introduction

On 11 February 2025, the MAH submitted a completed paediatric study for Cibinqo, 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 B7451102 is a study on 'Treatment satisfaction and quality of life (QoL) in Korean Moderate-to-Severe atopic dermatitis (AD) patients based on current targeted therapy use.'

2.2. Information on the pharmaceutical formulation used in the study

This study is non-interventional, hybrid study (Medical chart review + patient survey).

2.3. Clinical aspects

2.3.1. Introduction

The MAH submitted a final report for:

- Study B7451102; Treatment satisfaction and quality of life (QoL) in Korean Moderate to Severe atopic dermatitis (AD) patients based on current targeted therapy use

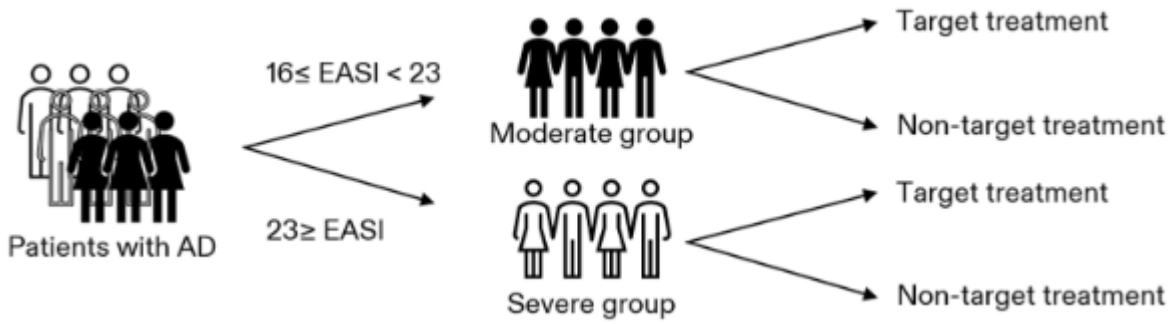
2.3.2. Clinical study

Study B7451102; Treatment satisfaction and quality of life (QoL) in Korean Moderate to Severe atopic dermatitis patients based on current targeted therapy use

Description

This study is a non-interventional, hybrid study with combined medical chart review with patient survey. Considering the time to onset of drug effect, patients surveys (EQ-5D-5L; EuroQoL five-dimensions questionnaire 5 Level version (EQ-5D-Y for youth), DLQI/CDLQI; Dermatology Life Quality Index/ Children's Dermatology Life Quality Index, TSQM; Treatment Satisfaction Questionnaire for Medication, WPAI; Work productivity loss and Activity impairment Questionnaire) were conducted after at least 12 weeks of administration of the current treatment (but no longer than 24 weeks). The patient recruitment period was expected to be 7 months.

The patients with moderate or severe AD will be recruited and divided into moderate and severe groups according to their Eczema Area and Severity Index (EASI) scores. The two groups were divided into the group that received targeted treatment and the group who did not. The primary endpoint was description of DLQI/CDLQI score and second endpoint was identification of survey results, including TSQM, WPAI, and EQ-5D score in each group.



Methods

Study participants

Patients must meet all of the following inclusion criteria to be eligible for inclusion in the study:

1. Patients who have been diagnosed with moderate or severe AD
2. Patients ≥ 12 years old
3. Patients who have been treated with the current treatment for 12 weeks or longer, but less than 24 weeks (continuously treated with current treatment at least 12 weeks not more than 24 weeks)

[A] Moderate group: defined by EASI score (16 ≤ EASI <23)

[B] Severe group: defined by EASI score (EASI ≥ 23)

Patients meeting any of the following criteria will not be included in the study:

1. Patients participating in other clinical studies in Dermatology
2. Patients on their current treatment as off-label

Evidence of a personally signed and dated informed consent document indicating that the patient (or a legally acceptable representative) has been informed of all pertinent aspects of the study**.**

Treatments

The targeted treatment therapy is defined as use of abrocitinib, baricitinib, dupilumab and upadacitinib. The non-targeted treatment therapy includes antihistamines, topical corticosteroid, calcineurin inhibitors, cyclosporin, methotrexate, azathioprine, mycophenolate mofetil, intravenous immunoglobulin, corticosteroids, alitretinoin, and others.

Objectives

This study aims to:

1. Describe the QoL and treatment satisfaction of moderate and severe AD patients
2. Investigate the QoL and treatment satisfaction of AD patients who received target treatment vs. non-targeted therapy patients in comparison to in each severity group

Outcomes/endpoints

The primary endpoint was the description of DLQI/CDLQI score and the secondary endpoint was identification of survey results, including TSQM, WPAI, and EQ-5D score in each group.

Sample size

The calculated total sample size is 480. Assuming that the t-test is performed for two severity groups' QoL, the primary endpoint, sample size was calculated based on the CDLQI/DLQI results of the previous study and the corresponding explanation. In addition, the adjusted mean treatment group difference was used for CDLQI/DLQI corresponding to QoL at the time point after 24 weeks. If the two groups are equally assigned the number of samples, the formula for calculating the number of samples required for each group is as follows;

(Assumption: treatment effect for each groups are same.)

$\alpha = 0.025, \beta = 0.20, \Delta/\sigma$ (effect size) = 0.4

$$n = \frac{2(z_{1-\alpha/2}+z_{1-\beta})^2}{\left(\frac{\Delta}{\sigma}\right)^2} = \frac{2\times(2.24+0.84)^2}{(0.4)^2} = 118.58$$

➔ Almost 120 subjects per group (120 x 4 = 480)

Randomisation and blinding (masking)

N/A

Statistical Methods

Primary Analysis:

The mean, standard deviation, median (interquartile range), and range (minimum, maximum) of DLQI and CDLQI are presented, and multivariable linear regression analysis is performed to evaluate the level of QOL according to epidemiological and clinical characteristics that affect DLQI and CDLQI, respectively, but data transformation or quantile regression can be utilised by checking the distribution of the data.

The study population was defined as all patients who fulfilled the inclusion and exclusion criteria and were enrolled in the study. For all participants, demographic and clinical characteristics were summarised. This study estimated all endpoints (DQLI/CDQLI, WPAI, TSQM, EQ-5D-5L/Y, EQ-VAS) according to targeted treatment and severity, with summary statistics.

There was no control for multiplicity in the statistical analyses performed for this study.

Amendments:

The amended SAP (version 2.0) was translated into English (SAP version 1.0 was documented in Korean). The 5.4 SUBGROUPS were added. Perform primary and secondary analyses for each age group (adolescents and adults). However, if the number of subjects in a particular age group is too small to perform a subgroup analysis, include the interaction of the group variable (treatment-experienced and treatment-naïve) and age group in the regression analysis, or perform the analysis only in certain age groups where the number of subjects is relatively stable.

Results

Participant flow and recruitment

A total of 171 patients with moderate-to-severe AD were enrolled from 12 hospitals between October 2023 and April 2024.

Baseline data

A total of 171 patients with moderate-to-severe AD were enrolled from 12 hospitals between October 2023 and April 2024. Of these, 84 patients (49.1%) had moderate AD. The patients with moderate AD had mean ± standard deviation (SD) EASI score of 17.1±1.5, and severe AD patients had EASI score of 26.6±3.9.

The proportion of males was 53 patients with moderate AD (63.1%), and 67 patients (77.0%) with severe AD. The mean age was 25.5±7.7 years for moderate AD, and 27.3± 9.9 years for severe AD. In the total study population of 171 patients, 24 (14.0%) were adolescents (aged between 12 and 17 years). Among patients with moderate AD, 14 (16.7%) were adolescents, and 6 (42.9% of adolescents) received targeted treatment. Of the 87 patients with severe AD, 10 (11.5%) were adolescents, and 7 (70.0% of adolescents) received targeted treatment. The patients with severe AD had higher mean body weight and were more likely to have a family history of AD.

The disease duration was found to be significantly longer in patients with severe AD compared to moderate AD (88.7± 82.9 vs 51.8±65.8 months, p-value <0.0001).

Number analysed

A total of 169 patients had data on DLQI/CDLQI. The number of subjects for DLQI was 153 patients, and 16 patients for CDLQI. A total of 170 patients with AD had data on TSQM and EQ-5D.

Efficacy results

Moderate AD:

The DLQI/CDLQI score was 8.1±7.6. for targeted treatment (28 patients), and 6.7 ± 4.8 for non-targeted treatment (55 patients). There was no statistically significant difference (p=0.8130). In terms of TSQM, the domain of side effects was significantly lower for targeted treatment compared to non-targeted treatment. There was no difference in WPAI on the use of targeted treatment. For EQ-5D score, self-care and anxiety/depression score were significantly higher in targeted treatment compared to non-targeted treatment.

Severe AD:

The DLQI/CDLQI score for targeted treatment with severe AD was 6.0 ± 4.9 (43 patients), while for non-targeted treatment was 9.7 ± 8.2 (43 patients). However, the difference was not significant (p=0.0583). For TSQM score, targeted treatment group had significantly higher TSQM in the domains effectiveness and global satisfaction. Moreover, non-targeted treatment group had a significantly higher WPAI score compared to targeted treatment in the domains presenteeism, work productivity loss, and activity impairment. There were no statistically significant differences in EQ-5D and EQ VAS scores based on the use of targeted treatment.

No subgroup analyses in adolescents were reported.

Safety results

No safety results were reported.

2.3.3. Discussion on clinical aspects

The MAH planned to include 480 subjects to the study but ended up with 171 study subjects. The reason for the smaller study size than aimed for has not been described by the MAH. Of the 171

included subjects, 24 (14.0%) were adolescents aged between 12 and 17 years. In total, 13 of the adolescents received targeted treatment. No information is available on how many of these subjects received abrocitinib. Treatment results were not analysed for each individual targeted treatment, hence there are no specific QoL and treatment satisfaction results for abrocitinib from this study. No subgroup analysis in adolescents were reported, even though it was mentioned in the statistical analysis plan. Nevertheless, no further clarifications were sought as those were not considered to have any impact on the benefit risk assessment of Cibinqo (abrocitinib) nor lead to any updates to the product information/RMP.

3. CHMP's overall conclusion and recommendation

The information from this study is very limited, since only 13 adolescents received active treatment, with no information on how many received abrocitinib. No subgroup analysis in adolescents was reported. In addition, no safety results were reported. Nevertheless, the available data from the study B7451102 do not provide any new information regarding efficacy or safety in adolescents in addition to that already described in the product information of Cibinqo (abrocitinib).

☒ **Fulfilled:**

No regulatory action required.