CIGE025ECN01 Study Results Abstract for Public Disclosure

Title

A real-world, prospective, multicenter study of safety and effectiveness of Xolair® (omalizumab) in the treatment of Chronic Spontaneous Urticaria (CSU) in Chinese adolescents inadequately controlled with H1 antihistamines

Date

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NIS Type

NIS with Primary Data Collection; Novartis Drug NIS

Keywords

Xolair® (omalizumab), Chronic Spontaneous Urticaria, prospective, real-world study, Chinese adolescents

Rationale and background

Xolair® (omalizumab) is a humanized anti-immunoglobulin E (IgE) recombinant monoclonal antibody. It is currently approved for the treatment of Chronic Spontaneous Urticaria (CSU) in adults and adolescents in more than 90 countries/region for powder and solvent for solution for injection [LYO] and more than 60 countries/region for solution for injection in pre-filled syringe [PFS]. Globally, the CSU approval was based on the efficacy and safety observed in the global Phase III program [Q4881g, Q4882g] in adults and adolescents; in addition, study CIGE025E2306 provided supporting evidence in Japanese and Korean patients. Xolair® 300 mg and 150 mg, given subcutaneous (s.c.) every 4 weeks, are recommended doses for the treatment of CSU in China.

In China, Xolair® has 2 registered dosage forms on market. Omalizumab LYO was approved by National Medical Products Administration (NMPA) on 08 April 2022 for the treatment of adults and adolescents 12 years of age and older with CSU who remain symptomatic despite H1 antihistamine (H1-AH) treatment supported by China stand-alone Phase III study [CIGE025E2305]. Omalizumab PFS was approved by NMPA on 21 June 2023 for the same indication. In the approval letters, the Center of Drug Evaluation (CDE) has a post-marketing requirement to further evaluate the safety and effectiveness of Xolair® in Chinese adolescent patients (≥ 12 to < 18 years of age). This non-interventional study (NIS) was aimed to assess the safety and effectiveness of Xolair® in a real-world setting in Chinese adolescent participants (≥ 12 to < 18 years of age) with CSU who remained symptomatic despite H1-AH treatment to fulfill this post-approval requirement.

Research question and objectives

Primary objective

1. To evaluate the safety of Xolair® in a real-world setting in Chinese adolescent participants (≥ 12 to < 18 years of age) with CSU who remained symptomatic despite H1-AH treatment.

Secondary objectives

1. To evaluate the effectiveness (measured by weekly itch severity score [ISS7], and weekly urticaria activity score [UAS7]) of Xolair® in a real-world setting in Chinese adolescent participants with CSU who remained symptomatic despite H1-AH treatment over a 12-week treatment period.

2. To evaluate the effectiveness (measured by urticaria control test [UCT]) of Xolair® in a real-world setting in Chinese adolescent participants with CSU who remained symptomatic despite H1-AH treatment over a 12-week treatment period.
3. To evaluate the quality of life (QoL, measured by Children's Dermatology Life Quality Index [CDLQI]) of participants receiving Xolair® in a real-world setting in Chinese adolescent participants with CSU who remained symptomatic despite H1-AH treatment over a 12-week treatment period.

Study design

This non-interventional, multicenter, prospective post-approval cohort study aimed to provide safety and effectiveness data of Xolair® in Chinese adolescents with CSU who remained symptomatic despite H1-AH treatment.

The study period was 16 weeks which contained a 12-week treatment period and 4-week safety follow up.

Participants had a 1-week screening period (up to 2 weeks) to establish eligibility and compliance for the study (urticaria patient diary compliance review and UAS7/ISS7 baseline data collection). Date of eligible participants initially treated according to the approved Xolair® dose regimen in China were collected as the baseline visit date.

Data was collected in conjunction with routine care visits at the site (recommended scheduled visits at Weeks 4, 8, 12). Routine clinical assessments were conducted, and safety information was collected. No extra study visits, examinations, laboratory tests or procedures were mandated.

All participants were followed up to 16 weeks regardless of their adherence to Xolair®.

Setting

The study population were Chinese adolescent participants aged between 12 years to less than 18 years and diagnosed with CSU who remained symptomatic despite H1-AH treatment. Participants were recruited from 10 tertiary hospitals. All relevant medical data and records for the participants were collected and recorded prospectively for a duration of 16 weeks.

Subjects and study size, including dropouts

Participants were enrolled according to the following inclusion and exclusion criteria:

Inclusion criteria:

1. Chinese male and female adolescent participants ≥ 12 to < 18 years of age at the time of screening.
2. Written assent and informed consent must be obtained per local regulations prior to any study procedures.
3. Diagnosed with CSU refractory to H1-AH at approved doses as defined by all of the following:
   - The presence of itch and hives for ≥ 6 consecutive weeks at any time prior to enrollment despite current use of second-generation H1-AH (at locally approved doses)
   - UAS7 score (range 0-42) ≥ 16 and ISS7 (range 0-21) ≥ 8 as captured in the urticaria patient diary during the 7 days prior to treatment initiation with Xolair®
4. Willing and able to complete a daily symptom Diary (urticaria patient diary) for the duration of the study, and having no more than 3 missing diary entries in the screening period.
5. Planned to receive Xolair® treatment according to the approved label in China at the time of screening.

Exclusion criteria:

1. Use of other investigational drugs for CSU treatment within 5 half-lives, or within 30 days (for small molecules) prior to screening or until the expected pharmacodynamic effect had returned to baseline (for biologics), whichever was longer.
2. History of hypersensitivity to any of the anti-IgE drugs or their excipients or to drugs of similar classes (i.e., to murine, chimeric, or human antibodies).
3. Any other skin disease associated with chronic itching that might influence, in the investigators opinion, the study evaluations and results. (e.g., atopic dermatitis, bullous pemphigoid, dermatitis herpetiformis, senile pruritus, etc.)
4. Medical examination or laboratory findings that according to the investigator rendered the participants unsuitable for the study.

This study planned to enroll approximately 59 participants aged 12 years to less than 18 years with CSU who remained symptomatic despite H1-AH treatment from 11 sites. There were no dropouts in the final enrolled study population.

Variables and data sources

Baseline participant demographics, disease characteristics, and CSU treatment history were collected.

The type and frequency of adverse events (AEs), adverse events of special interest (AESIs) and serious adverse events (SAEs) were used as variables to evaluate the primary objective.

The ISS7/ UAS7, UCT and CDLQI collected at each visit over a 12-week treatment period were used as variables to evaluate the secondary objectives.

Data available during the routine clinical practice were collected on an ongoing basis. No additional diagnostic or monitoring procedures were applied in this NIS. The investigator was asked to complete the appropriate electronic case report forms for events of interest (e.g., AE, SAE), laboratory testing, vital signs and the patient-reported outcomes (PROs) (ISS7/UAS7, UCT and CDLQI) if applicable at every visit. Participants were asked to complete the urticaria patient diary every day.

Statistical methods

Descriptive summary statistics were provided. No statistical hypotheses and inferences were planned. Frequencies and percentages were provided for categorical variables. Mean, standard deviation, median, minimum and maximum were provided for continuous variables.

With regard to analysis sets, the Enrolled Set consists of all participants who were enrolled in this study. The Safety Set comprises enrolled participants excluding those who did not receive Xolair® during this study. The Effectiveness Set comprises all enrolled participants excluding those who did not receive Xolair® during this study and participants who were missing all measurements for all PROs (ISS7, weekly hives severity score [HSS7], UAS7, UCT and CDLQI) at all post-baseline visits.

Participant disposition was summarized using the Enrolled Set, and the number of participants who completed and early discontinued were tabulated. The primary endpoint is the incidence of AEs (including SAEs, AESI) over a 16-week study period. Based on the Safety Set, treatment-emergent adverse events (TEAEs), TEAE by maximum severity, TEAE possibly related to study treatment, SAE, and AESI were summarized by primary system organ class (SOC) and preferred term with the number of participants as well as percentage with the 95% confidence interval as applicable.

The secondary endpoints were analyzed based on the Effectiveness Set. This included summary statistics for ISS7, HSS7 and UAS7 score as continuous variables by visit. Change from baseline in ISS7, HSS7 and UAS7 scores were also summarized as continuous variables by visit. The proportion of participants who achieved well-controlled CSU (defined as a UCT score ≥ 12) by visit was summarized. For CDLQI, the absolute change as well as the percentage change from baseline were summarized as continuous variables. The proportion of participants achieving CDLQI 0 or 1 over time up to Week 12 was summarized.

Results

All 55 enrolled Chinese adolescent participants with CSU completed the study through Week 16. The proportion of male participants was 54.5%. The mean age of participants at informed consent was 14.3 years. Participants had a mean duration of urticaria of 2.04 years, with majority (70.9%) of them having < 2 years of urticaria history.

Participants received Xolair® at a dose of either 150 mg or 300 mg during the treatment period. Majority (over 70%) of the participants received Xolair® via PFS, and the rest received reconstitute from a vial. As a concomitant medication, 85.5% of the participants had at least one H1-AH medication after the study enrollment.

There were 22 (40.0%) participants who reported at least one TEAE during the study. The same number (n=13 [23.6%] each) of participants experienced mild or moderate (by maximum severity) TEAEs, and no participant reported severe TEAE. No SAEs or AESIs were reported during the study period. Neither deaths nor TEAEs leading to study drug discontinuation occurred during the study period. The most frequently reported TEAEs by primary SOC were infections and infestations (25.45%), followed by skin and subcutaneous tissue disorders (12.73%) and gastrointestinal disorders (9.09%). Five (9.09%) participants had at least one TEAE considered as related to Xolair® based on investigators' assessment.

After 12 weeks of treatment, the changes from the baseline score of ISS7 and UAS7 were -11.73 and -25.07, respectively. The proportion of participants who achieved well-controlled CSU (defined as a UCT score ≥ 12) at Week 12 was 70.9%. The absolute change and the percentage change in CDLQI score from baseline were -9.7 and -81.5%, respectively, at Week 12.

Discussion

The safety profile of Xolair® observed in this study was consistent with the known safety profile observed in previous global adults and adolescent Phase III trials and the China CSU adults Phase III study, with no new or unexpected safety signals identified. All TEAEs were mild or moderate in severity. No SAEs or AESIs were reported during the study period.

Effectiveness outcomes demonstrated favorable responses for all endpoints. The ISS7 and UAS7 scores were improved at Week 12, and more than two-thirds of the participants achieved well-controlled UCT after 12 weeks' treatment with Xolair®. Decrease in the CDLQI score was observed by Week 12, indicating improvement in participants' QoL.

Global clinical studies have demonstrated a favorable safety profile and positive effectiveness outcomes of Xolair® in adult and adolescent patients with CSU who remained symptomatic despite H1-AH treatment. The observations in this study of Chinese adolescents with CSU were consistent with the previously known safety and effectiveness profile of Xolair® among global general populations as well as Chinese adult patients.

Conclusion

This non-interventional, multicenter, prospective post-approval cohort study provided safety and effectiveness data of Xolair® in Chinese adolescents with CSU who remained symptomatic despite H1-AH treatment in a real-world setting.

Treatment with Xolair® was generally well tolerated and no new or unexpected safety concerns were identified during the 16-week study period. Clinically meaningful effectiveness (ISS7/ UAS7, UCT, and CDLQI) of Xolair® was observed at Week 12 among the study population. These findings further support the favorable benefit-risk profile of Xolair® in Chinese adolescents with CSU who remained symptomatic despite H1-AH treatment and fulfill post-marketing requirements from the regulatory authority.

List of abbreviations

AE	Adverse Event
AESI	Adverse Events of Special Interest
CDE	Center of Drug Evaluation
CDLQI	Children's Dermatology Life Quality Index
CSU	Chronic Spontaneous Urticaria
CU	Chronic Urticaria
EU	European Union
H1-AH	H1 Antihistamine
HSS	Hives Severity Scores
HSS7	Weekly Hives Severity Score
IgE	Immunoglobulin E
ISS	Itch Severity Score
ISS7	Weekly Itch Severity Score
LYO	Powder and Solvent for Solution for Injection
NIS	Non-Interventional Study
NMPA	National Medical Products Administration
PAS	Post-Authorization Study
PFS	Pre-filled syringe
PRO	Patient-Reported Outcome
PT	Preferred Term
QoL	Quality of Life
SAE	Serious Adverse Event
s.c.	Subcutaneous
SOC	System Organ Class
TEAE	Treatment-Emergent Adverse Event
UAS	Urticaria Activity Score
UAS7	Weekly Urticaria Activity Score
UCT	Urticaria Control Test