

Summary Report

# Incidence, Efficacy, and Safety of Long-Term Use of Omalizumab for the Treatment of Chronic Idiopathic Urticaria

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# Executive Summary

Omalizumab is recommended for the treatment of patients with moderate to severe chronic idiopathic urticaria (CIU) when their symptoms do not respond to non-sedating H<sub>1</sub> antihistamines.

Omalizumab is being used for long-term treatment of CIU, but there is limited evidence regarding its chronic use beyond 24 weeks. A Drug Utilization Study and Systematic Review aimed to determine real-world omalizumab use patterns in patients with CIU in 4 Canadian provinces, the efficacy and effectiveness of long-term use, and whether extended use is safe for patients. Researchers found that the use of omalizumab for CIU frequently exceeds 24 weeks, with most treatments exceeding 48 weeks. No demographic differences were observed in age, sex, or location (urban versus rural) between short-term and long-term use. The evidence on efficacy and safety suggests that omalizumab is effective after 24 weeks, and there is no indication of increased harm. However, the studies had some limitations, and findings should be interpreted with caution.



# Background

Urticaria, or hives, presents as itchy, red welts that appear on the skin. CIU occurs when hives appear for at least 6 weeks without any apparent cause, sometimes paired with angioedema (swelling under the skin, often affecting the lips, face, and neck). Nonsedating antihistamines are the first line of treatment for patients with CIU. However, some patients' symptoms do not respond to them, even at higher doses. In these cases, omalizumab can be used.

## Policy Issue

Omalizumab is being used for the long-term treatment of patients with CIU in the real-world setting. However, there is limited evidence regarding the chronic use of omalizumab (beyond 24 weeks). There is a need for information on the incidence of longer-term use across Canada, as well as the long-term efficacy and safety of this drug.

## Policy Questions

1. What is the incidence of omalizumab long-term use (i.e., > 24 weeks) for individuals being treated for CIU?
2. What are the characteristics of patients with CIU who use omalizumab for longer than 24 weeks?
3. Is long-term ( $\geq$  24 weeks) treatment with omalizumab effective, and if so, for which patients?

## Objectives

The objectives of this Drug Utilization Study and Systematic Review were to determine real-world omalizumab use patterns in patients with CIU, to determine the efficacy and effectiveness of long-term use, and to establish whether extended use is safe for patients.

# Findings

## Drug Utilization

A **treatment episode** is defined as the continuous period that a patient fills a prescription (dispensations). In this study, **new** treatment episodes were classified as patients with no prior dispensing of omalizumab within the previous 6 months.

Overall, researchers identified 1,159 treatment episodes of omalizumab for CIU using the National Prescription Drug Utilization Information System (NPDUIS) database for 4 Canadian provinces (747 in British Columbia, 203 in Saskatchewan, 171 in Manitoba, and 38 in Newfoundland and Labrador).

Researchers found that:

- omalizumab use has increased since it was first recommended for listing on drug plans for CIU in 2015
- approximately two-thirds of treatment episodes lasted more than 24 weeks in Manitoba, Saskatchewan, and British Columbia; in these 3 provinces, more than one-third of treatment episodes lasted more than 48 weeks
- in Newfoundland and Labrador (where data are only available for those eligible for public drug benefits), only 26.3% of episodes were longer than 48 weeks, and the most common treatment episode duration was 16 weeks to 24 weeks (31.6%).

The majority of those prescribed omalizumab for CIU were between the ages of 25 and 64 years, were female, and were dispensed omalizumab at a pharmacy located in an urban area. Age, sex, and location did not vary for long-term (> 24 weeks) versus short-term ( $\leq$  24 weeks) use of omalizumab across provinces.

## Efficacy and Safety

Researchers used a systematic review approach and included 37 reports in the final analysis: 2 randomized controlled trials (RCTs) on the use of omalizumab or placebo, 4 cohort studies comparing up to 2 years of treatment with omalizumab to other treatments or no treatment, and 4 single-group prospective studies.

The findings from the 2 RCTs suggest that long-term use of omalizumab:

- continues to provide symptom relief when compared to placebo
- provides meaningful improvements in quality of life and symptom reduction
- does not increase the risk of severe adverse events or withdrawals due to adverse events.

The findings from the 4 comparative cohort observational studies suggest that long-term use of omalizumab:

- continues to provide symptom relief when compared to antihistamines
- does not increase the risk of severe adverse events or withdrawals due to adverse events.

## Limitations

There were a few key limitations to both the Drug Utilization Study and the Systematic Review.

Data from Newfoundland and Labrador in the Drug Utilization Study only included claims made to public drug programs, which were not representative of the whole population. Additionally, information on the specific diagnoses or conditions for which omalizumab prescriptions were written was not available across all provinces. Efforts were made to remove episodes in which omalizumab was used for asthma, but some treatment episodes for comorbid asthma (the presence of both CIU and asthma) may have also been excluded.

Many analyses in the Systematic Review for efficacy and safety were not feasible due to a shortage of data on outcomes of interest, variations in the studied treatments, and limitations in the study data. The cohort studies did not consider confounding variables or adjust for imbalances across treatment groups, which may have led to a biased estimation of the treatment effects. Readers should use caution when reviewing and interpreting these results.

## Implications for Policy-Making

The Drug Utilization Study found that the use of omalizumab has increased since it was first recommended for listing on drug plans for CIU in 2015. The majority of new treatment episodes exceeded 24 weeks, with many episodes exceeding 48 weeks. Across all provinces, no clear demographic differences were observed in age, sex, or location (urban versus rural) between short-term and long-term recipients of omalizumab.

Based on studies included in the Systematic Review, there is some evidence that omalizumab remains effective after 24 weeks, and there was no indication of increased harm, but the studies had several limitations, as previously noted.

## Considerations

Post-Market Drug Evaluation (PMDE) projects aim to produce health policy issue evidence and are not linked to a recommendation.

This work was intended to inform health policy. Clinical questions regarding omalizumab should be directed to a health care professional.

For more information on CoLab and its work, visit the [CoLab website](#).

Full scientific reports:

[Long-Term Use of Omalizumab for CIU](#)

[Efficacy and Safety of Long-Term Use of Omalizumab for the Treatment of CIU](#)



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This Drug Utilization Study and Systematic Review was conducted by the Ontario Drug Policy Research Network (ODPRN) and the POst-Market Drug Evaluation Team (PODET), respectively, through the Post-Market Drug Evaluation CoLab Network. This work was supported by CADTH and its Post-Market Drug Evaluation Program, through funding provided by Health Canada.

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About CoLab: CoLab is a pan-Canadian network of experts in applied research, scientific methods, and data analysis. CoLab members work with CADTH's Post-Market Drug Evaluation Program to produce credible and timely evidence on post-market drug safety and effectiveness.

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