Ontario Ministry of Health and Long-Term Care

Ontario Public Drug Programs
Exceptional Access Program
Ilaris® (canakinumab) – Cryopyrin-Associated Periodic Syndrome (CAPS)
Reimbursement Guidelines

Version 1 – April 2012

The Ministry will consider requests for the reimbursement of Ilaris® (canakinumab) for the treatment of cryopyrin-associated periodic syndrome (CAPS).

Please note that patients must be eligible through the Ontario Drug Benefit (ODB) Program in order to receive coverage for this medication. Coverage is not retroactive and reimbursement may only be provided for medications dispensed after Exceptional Access Program (EAP) approval has been granted. In addition, the reimbursement criteria must always be met – even in cases where EAP approval is required to provide continued treatment that was previously supplied through a clinical trial, or paid for by other means (such as a third party payor).

Physicians may use the attached EAP request form to ensure that the necessary clinical information is provided, and to facilitate the review process. Please ensure that all relevant clinical information is provided on the request or by including copies of laboratory results. Requests should be faxed to EAP at (416) 327-7526 or toll free 1-866-811-9908.

Background on Ontario's Drug for Rare Diseases (DRDs) Evaluation Process for Public Drug Reimbursement

The Ontario Ministry of Health has moved forward to develop a funding framework for Drugs for Rare Diseases (DRDs). This approach recognizes that an innovative approach is required that considers the level of available clinical evidence, patient need, and the current funding gap.

In December 2007, Ontario Public Drug Programs (OPDP) established a working group comprised of clinical experts (including genetic medicine) and health economists to develop a new evaluation framework to review and evaluate DRDs for funding by the province.

The new approach is based on the "best available evidence", to assist us in predicting the potential benefit or lack of benefit of a drug treatment in specific groups of patients. This new approach will help identify groups of individuals that may potentially benefit from treatment with a particular drug, and where we may consider funding.

EAP Reimbursement Criteria:

Initial funding for Ilaris® (canakinumab) under the ODB program may be considered where the patient meets the following criteria:

Muckle-Wells Syndrome:

Diagnosis of Muckle-Wells Syndrome (MWS) based on the following confirmatory results:

- Clinical diagnosis of MWS

AND

- NLRP3 mutation* (mutational analysis required)

AND

- SAA levels ≥ 10 mg/L

AND

- Assessment score** of patient's disease activity determined by the following 4 parameters: skin disease, arthralgia, conjunctivitis, and fatigue/malaise ≥ mild, moderate, or severe

Initial Approval Period: 1 year

NOMID Syndrome:

Diagnosis of Neonatal-Onset Multisystem Inflammatory Disease (NOMID) based on the following confirmatory results:

- Clinical diagnosis of NOMID

AND

- Presentation of symptoms of NOMID made in a patient < 6 months of age

AND

- NLRP3 mutation* (mutational analysis required)

AND

- Assessment score** of patient's disease activity determined by the following 4 parameters: skin disease, arthralgia, conjunctivitis, and fatigue/malaise ≥ mild, moderate, or severe

Initial Approval Period: 6 months

** For patients without NLRP3 mutation, please provide full details of the patient's presentation. These requests will be considered on a case-by-case basis. Initial approval period is 6 months. If the patient demonstrates a sustained SAA level < 10 mg/L, renewal of funding for a 1-year period may be considered.*

*** For assessment of disease activity, please see Lachmann et al. N Engl J Med 2009;360:2416-25.*

- Patient must NOT be bedridden where any physical activity brings on discomfort and symptoms which occur at rest AND not amenable to surgical/medical intervention

- Patient has no other life-threatening disease where prognosis is unlikely to be influenced by Ilaris® (canakinumab) therapy (e.g. neuroblastoma, leukemia etc.)

- Treatment is supervised by medical specialists with knowledge in the management of CAPS

- Dosage:
  - Bodyweight > 40 kg: 150 mg subcutaneously every 8 weeks
  - Bodyweight = 15 kg – 40 kg: 2 mg/kg subcutaneously every 8 weeks. (For children 15 kg – 40 kg with an inadequate response, the dose can be increased to 3 mg/kg.)

Exclusion Criteria:
Patients with Familial Cold Auto-Inflammatory Syndrome (FCAS) are not eligible for funding.

If a patient falls under the following categories, the Ministry will NOT consider funding of Ilaris® (canakinumab)) since it is unlikely that therapy will benefit the patient in terms of disease stabilization.
- Patient's life expectancy less than 6 months irrespective of cause

Extension of funding will be considered where the patient meets ALL of the following criteria:

MWS & NOMID:

- SAA levels < 10 mg/L. *[If SAA levels are > 10 mg/L over a 6 month interval and are sustained over two consecutive 6 month intervals, the patient may not be eligible for continued funding of Ilaris® (canakinumab).]*

- Assessment score of patient's skin disease, arthralgia, conjunctivitis, and fatigue/malaise = no or minimal disease activity. (*For assessment of disease activity, please see Lachmann et al. N Engl J Med 2009;360:2416-25.)*

- Patient must NOT be bedridden where any physical activity brings on discomfort and symptoms which occur at rest AND not amenable to surgical/medical intervention

- Patient has no other life-threatening disease where prognosis is unlikely to be influenced by Ilaris® (canakinumab) therapy (e.g. neuroblastoma, leukemia etc.)

- Patient has not developed a life-threatening complication or a severe injection reaction to Ilaris® (canakinumab) not treatable by other therapeutic measures and is unlikely to benefit from further Ilaris® (canakinumab) therapy

- Patient has adhered with prescribed injection schedule for optimal management of the disease

- Patient has adhered to all safety and effectiveness monitoring of the treatment

- Treatment is supervised by medical specialists with knowledge in the management of CAPS

- Dosage:
  - Bodyweight > 40 kg: 150 mg subcutaneously every 8 weeks
  - Bodyweight = 15 kg – 40 kg: 2 mg/kg subcutaneously every 8 weeks. (For children 15 kg – 40 kg with an inadequate response, the dose can be increased to 3 mg/kg.)

Renewal Period: 1 year

If all the above renewal criteria are not met, the patient may NOT be eligible for continued public funding.

Your feedback is welcomed. Please contact us at:
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