CADTH

## CADTH Reimbursement Review

# Patient Input

### MECASERMIN (INCRELEX)

(Ipsen Biopharmaceuticals Canada, Inc.)

**Indication:** Severe primary insulin-like growth factor-1 deficiency.

**CADTH received patient input from:**
ICOSEP

**July 12, 2021**

CADTH

# CADTH Reimbursement Review
# Patient Input Template

| | |
|---|---|
| **Name of the Drug and Indication** | **Increlex** |
| **Name of the Patient Group** | **ICOSEP** |
| **Author of the Submission** | [redacted] |
| **Name of the Primary Contact for This Submission** | [redacted] |
| Email | [redacted] |
| Telephone Number | [redacted] |

## 1. About Your Patient Group

Describe the purpose of your organization. Include a link to your website.

**ICOSEP stands for International Coalition of Organizations Supporting Endocrine Patients. The purpose is to unite medical societies and patient organizations. Our focus is endocrine health care issues. We unite our voices globally to facilitate education to the general public for an understanding as to the critical health importance of endocrine care.**

**Currently, endocrinology is primarily associated with only diabetes. However, there are thousands of medical conditions which are endocrine related. The endocrine system impacts every living cell in a human body. Yet it is often overlooked in importance. Neglect of any imbalance in the endocrine system can lead to a lifetime of pain and suffering for patients. This agony is needless and treatable in many conditions. In particular if children are diagnosed and treated early, we can reduce the lifetime of agony they would face without medical treatments.**

## 2. Information Gathering

CADTH is interested in hearing from a wide range of patients and caregivers in this patient input submission. Describe how you gathered the perspectives: for example, by interviews, focus groups, or survey; personal experience; or a combination of these. Where possible, include **when** the data were gathered; if data were gathered **in Canada** or elsewhere; demographics of the respondents; and **how many** patients, caregivers, and individuals with experience with the drug in review contributed insights. We will use this background to better understand the context of the perspectives shared.

**ICOSEP routinely communicates with patient organization leaders and medical professionals from throughout the world. We work with patient organization leaders as well as medical professionals from throughout Europe, North America, South America, India and Latin America. Visit our website at http://icosep.org and click on the Medical or Patient tab to see all the areas and groups networked.**

## 3. Disease Experience

CADTH involves clinical experts in every review to explain disease progression and treatment goals. Here we are interested in understanding the illness from a patient's perspective. Describe how the disease impacts patients' and caregivers' day-to-day life and quality of life. Are there any aspects of the illness that are more important to control than others?

**With regards to Increlex specifically, children who are deficient may appear normal "short" to the general public. However, they untreated patients struggle each and every day and throughout the entire day. Simple tasks such as getting out of bed and playing like normal children demand huge efforts. These children often cannot concentrate like their peers and struggle to play. The energy used for play may not be available for a little body trying to grow and develop healthy.**

**Unknowingly, people who are unaware of these conditions are often are harsh to these struggling children as they "look" normal. To try and convey how they feel each day….Imagine your life with a sever case of pneumonia. You can breathe but you are exhausted. But because your "look" ok and are a child, people demand that despite your illness, you get up and function as a healthy child. These children are not lazy, incapable of concentrating to learn, or wanting to be grumpy from exhaustion. But this is their normal. Most IGF deficient children have been living with this condition since birth. They do not have an understanding that their "normal" is ill. So they struggle to comply. As they age, the signs of their problems become more dramatic and the children stress greatly. It is not about the obvious "height" of the symptoms. That is simply a visual indicator which helps people recognize a problems. Rather it is the invisible traumatic issues ( heart strength. lung capacity, and others) which cause the most life long damage to untreated children.**

## 4. Experiences With Currently Available Treatments

CADTH examines the clinical benefit and cost-effectiveness of new drugs compared with currently available treatments. We can use this information to evaluate how well the drug under review might address gaps if current therapies fall short for patients and caregivers.

Describe how well patients and caregivers are managing their illnesses with currently available treatments (please specify treatments). Consider benefits seen, and side effects experienced and their management. Also consider any difficulties accessing treatment (cost, travel to clinic, time off work) and receiving treatment (swallowing pills, infusion lines).

**Currently, there are no substitutes for Increlex. If a child is deficient in this vital hormone, they will continue to be unhealthy and suffer if they are not allowed access to this medication.**

**To put this very detailed problem into a scenario that is more easily understood:**
**If your child were on fire, would you try to put it out to avoid pain and long term scaring? Of course your would. But what if there were people around stopping you from accessing anything to put out that fire? This example is simplistic way of showing how treating a IGF-1 deficient child works. The fire extinguisher is available and we need access to the tools to put out the fire. All of the member organizations of ICOSEP hope that people would want to help innocent children by allowing medical treatments which can impact their lifetimes.**

## 5. Improved Outcomes

CADTH is interested in patients' views on what outcomes we should consider when evaluating new therapies. What improvements would patients and caregivers like to see in a new treatment that is not

achieved in currently available treatments? How might daily life and quality of life for patients, caregivers, and families be different if the new treatment provided those desired improvements? What trade-offs do patients, families, and caregivers consider when choosing therapy?

**The outcome of a child who is deficient and receives medical treatment is astronomical. Many people incorrectly view IGF-1 care as a cosmetic issue for a child's growth. In reality, growth is only a visual indicator of the serious and lifelong aspects of this deficiency. Treating children will help their bodies internal systems, (their heart and lungs etc.) reach a normal size and capacity. Their bones will be normal strength. The outcome for them is simply "normal". They are not asking to be professional athletes or super humans. They simply want to have energy to be a child, laugh, play and grow up to be a healthy adult.**

## 6. Experience With Drug Under Review

CADTH will carefully review the relevant scientific literature and clinical studies. We would like to hear from patients about their individual experiences with the new drug. This can help reviewers better understand how the drug under review meets the needs and preferences of patients, caregivers, and families.

How did patients have access to the drug under review (for example, clinical trials, private insurance)? Compared to any previous therapies patients have used, what were the benefits experienced? What were the disadvantages? How did the benefits and disadvantages impact the lives of patients, caregivers, and families? Consider side effects and if they were tolerated or how they were managed. Was the drug easier to use than previous therapies? If so, how? Are there subgroups of patients within this disease state for whom this drug is particularly helpful? In what ways? If applicable, please provide the sequencing of therapies that patients would have used prior to and after in relation to the new drug under review. Please also include a summary statement of the key values that are important to patients and caregivers with respect to the drug under review.

**Canada is a progressive country. Review and consideration of this medical treatment is vital for the life and health of deficient children. Currently, Increlex is available in the USA and a few European countries. Governments throughout the world are beginning to review and learn more about this critical care with positive responses. The disadvantage of no access to this medication; an affected child will suffer the insufficiency and complications of the lack of normal heart, lung and bone strength for their lifetime. The short term costs would not compare to the lifetime of specialised care required by those untreated.**

## 7. Companion Diagnostic Test

**If** the drug in review has a companion diagnostic, please comment. Companion diagnostics are laboratory tests that provide information essential for the safe and effective use of particular therapeutic drugs. They work by detecting specific biomarkers that predict more favourable responses to certain drugs. In practice, companion diagnostics can identify patients who are likely to benefit or experience harms from particular therapies, or monitor clinical responses to optimally guide treatment adjustments.

What are patient and caregiver experiences with the biomarker testing (companion diagnostic) associated with regarding the drug under review?

Consider:

- Access to testing: for example, proximity to testing facility, availability of appointment.
- Testing: for example, how was the test done? Did testing delay the treatment from beginning? Were there any adverse effects associated with testing?

- Cost of testing: Who paid for testing? If the cost was out of pocket, what was the impact of having to pay? Were there travel costs involved?
- How patients and caregivers feel about testing: for example, understanding why the test happened, coping with anxiety while waiting for the test result, uncertainty about making a decision given the test result.

There are few if any parents anywhere in the world who live with a child suffering, who would question a test for this issue or any medical problem. They would only panic if a solution treatment was available but not allowed.

## 8. Anything Else?

Is there anything else specifically related to this drug review that CADTH reviewers or the expert committee should know?

## Appendix: Patient Group Conflict of Interest Declaration

To maintain the objectivity and credibility of the CADTH reimbursement review process, all participants in the drug review processes must disclose any real, potential, or perceived conflicts of interest. This Patient Group Conflict of Interest Declaration is required for participation. Declarations made do not negate or preclude the use of the patient group input. CADTH may contact your group with further questions, as needed.

1. Did you receive help from outside your patient group to complete this submission? If yes, please detail the help and who provided it.

**No. We were made aware of this potential opportunity for patient care by Ipsen. However, it was our choice as the only global leader of this patient population of both patient groups and medical societies to eagerly participate in your survey.**

2. Did you receive help from outside your patient group to collect or analyze data used in this submission? If yes, please detail the help and who provided it.

**No**

3. List any companies or organizations that have provided your group with financial payment over the past two years AND who may have direct or indirect interest in the drug under review.

   **ICOSEP is the international division of The MAGIC Foundation, USA. However, ICOSEP operates independently and is governed independently. As such, ICOSEP does receive educational grants to assist with our worldwide Children's Growth Awareness Campaign. It is a campaign which is not specific to any one medical condition.**

   The only company who supports ICOSEP and operates with a direct interest in this drug is as follows:

| Company | Check Appropriate Dollar Range | | | |
|---|---|---|---|---|
| | $0 to 5,000 | $5,001 to 10,000 | $10,001 to 50,000 | In Excess of $50,000 |
| Ipsen | | | 40,000 | |
| | | | | |
| | | | | |

I hereby certify that I have the authority to disclose all relevant information with respect to any matter involving this patient group with a company, organization, or entity that may place this patient group in a real, potential, or perceived conflict of interest situation.

Name: Jamie Harvey
Position: CEO of ICOSEP and MAGIC Foundation Co-founder
Patient Group: ICOSEO
Date: May 17, 2021