



## Common Drug Review *Patient Group Input Submissions*

### **Teduglutide (Revestive) for short bowel syndrome**

**Patient group input submissions were received from the following patient groups. Those with permission to post are included in this document.**

GI (Gastrointestinal) Society — permission granted to post.

#### **CADTH received patient group input for this review on or before December 18, 2015**

The views expressed in each submission are those of the submitting organization or individual; not necessarily the views of CADTH or of other organizations.

While CADTH formats the patient input submissions for posting, it does not edit the content of the submissions.

CADTH does use reasonable care to prevent disclosure of personal information in posted material; however, it is ultimately the submitter's responsibility to ensure no personal information is included in the submission. The name of the submitting patient group and all conflict of interest information are included in the posted patient group submission; however, the name of the author, including the name of an individual patient or caregiver submitting the patient input, are not posted.

## GI (Gastrointestinal) Society

### Section 1 – General Information

<b>Name of the drug CADTH is reviewing and indication(s) of interest</b>	Revestive® (teduglutide) for short bowel syndrome
<b>Name of the patient group</b>	GI (Gastrointestinal) Society
Patient group's contact information:	231-3665 Kingsway, Vancouver BC V5R 5W2 Tel:604-873-4876 Email: <a href="mailto:info@badgut.org">info@badgut.org</a>
<b>Permission is granted to post this submission</b>	Yes

#### 1.1 Submitting Organization

Our mission: As the Canadian leader in providing trusted, evidence-based information on all areas of the gastrointestinal tract, the GI (Gastrointestinal) Society is committed to improving the lives of people with GI and liver conditions, supporting research, advocating for appropriate patient access to health care, and promoting gastrointestinal and liver health.

Canadian health care professionals request more than 550,000 of our BadGut® Basics patient information pamphlets each year, and tens of thousands of Canadians benefit from our important quarterly publication, the Inside Tract® | Du coeur au ventreMC newsletter.

Our free BadGut® Lectures from coast to coast cover various digestive conditions for patients, caregivers, and other interested individuals. We also have dynamic websites in English ([www.badgut.org](http://www.badgut.org)) and French ([www.mauxdeventre.org](http://www.mauxdeventre.org)). Organized on a number of topics, GI Society support group meetings offer a wealth of information for those newly diagnosed with a gastrointestinal disorder, as well as those who have lived with a condition for years.

Our highly trained staff and volunteers offer additional patient resources, including responding to information requests and participating in community initiatives. Staff and advisors work closely with health care professionals, other patient groups, and governments at all levels on behalf of GI patients. The GI Society, along with its sister charity, the Canadian Society of Intestinal Research (founded in 1976), has supported a number of significant clinical, basic, and epidemiological research projects in the field of gastroenterology.

#### 1.2 Conflict of Interest Declarations

*a) We have the following declaration(s) of conflict of interest in respect of corporate members and joint working, sponsorship, or funding arrangements:*

The GI Society receives financial contributions from pharmaceutical companies in support of our independent charitable work for Canadians affected by GI/liver conditions. Supporters have no input into the editorial content of our resource material, which is approved by the GI Society's Medical

Advisory Council (made up of GI/liver health experts only). Pharmaceutical companies from whom we have received support of any kind, such as charitable donations or grants, sponsorships, subscriptions to The Inside Tract® newsletter, etc. in the last two years include: AbbVie Corporation, Allergan, AstraZeneca Canada Inc., Canada's Research-Based Pharmaceutical Companies (Rx&D), Ferring Inc., Gilead Sciences Canada Inc., GlaxoSmithKline Inc., Hoffmann-La Roche Ltd., Janssen Canada, NPS Pharma, Merck Canada Inc., Pfizer Canada Inc., and Takeda Canada Inc..

*b) We have the following declaration(s) of conflict of interest in respect of those playing a significant role in compiling this submission:*

None. The GI Society has solely prepared this submission entirely independently of any outside groups or individuals.

## **Section 2 — Condition and Current Therapy Information**

### **2.1 Information Gathering**

Information was obtained through contact (interviews, etc.) with short bowel syndrome patients and included several who participated in the clinical trials for Revestive®. Additional information came from the Short Bowel Syndrome Oley Conference Roundtable in the US, which I participate in this year and had an opportunity to discuss issues with several other short bowel syndrome patients and caregivers. I also discussed care issues with three health care providers, a physician, a nurse, and a hospital pharmacist who work directly with short bowel syndrome. We have already published information on short bowel syndrome, which we gathered from medical studies and experts.

### **2.2 Impact of Condition on Patients**

Short bowel syndrome is a potentially fatal gastrointestinal disorder in which patients are unable to absorb sufficient nutrients and fluids through the intestines. It occurs when the small intestine doesn't function properly due to trauma, disease, or when too much is removed. Conditions that could lead to a short bowel include Crohn's disease, gastrointestinal cancer, perforated bowel, blocked or restricted blood flow to the bowel, or congenital abnormalities.

The symptoms and severity vary according to the part of the intestine that has been removed, as the small intestine is not identical composition through its entire distance. Different sections of the small intestine are responsible for different nutrient absorption. As such, patient experiences can vary. Common symptoms include vitamin and mineral deficiencies, frequent diarrhea, extreme fatigue, cramping, dehydration, and weight loss. Complications of these can include peptic ulcer disease, kidney stones, gallstones, small bowel bacterial overgrowth, and metabolic bone disease.

This condition is most often managed by a specialized diet. However, many individuals must rely on Total Parenteral Nutrition (TPN) therapy. This is an intravenous (IV) administration of nutrients into the bloodstream. For patients with short bowel syndrome, being on TPN has a negative physical, social, and emotional impact. These include:

#### **Severely Limited Activities**

Individuals must schedule their days around their infusion or nourishment ingestion schedule. It is usually impractical to infuse in public as an individual might not have access to a portable pump or

sanitary conditions. TPN and Enteral Nutrition (EN) equipment and formulas are often heavy and cumbersome. Since many who have short bowel syndrome will have to carry medical supplies with them, their mobility is limited. The fatigue brought on by malnourishment restricts patients from participating in sport, recreational, self-care, and most social activities.

### **Inability to Eat**

Since short bowel syndrome patients are not able to digest food and fluids properly, their ability to eat normal foods and beverages can be profoundly impaired. Not only do they miss out on the taste and texture of foods, they also cannot fully participate in social interactions because many gatherings and holidays are centered around food or occur in restaurants.

### **Mental Health Problems**

Living with short bowel syndrome can cause significant stress, anxiety, and depression. Social interactions can be restricted due to medical limitations and fatigue. This can result in isolation and reduce access to support networks. Fear of incontinence for those who do not have ostomies can also cause the patient anxiety. A patient says: “It’s not just these physical conditions I have to deal with and fight everyday, there is also a mental battle raging inside me all the time”.

### **Economic Dependence**

Fatigue and abdominal pain may reduce the ability to work or go to school. This can have negative financial consequences. An individual might have to reach out to family members for financial support or be dependent on government financial aid. As such, they are more likely to be low-income and unable to afford the necessities to help manage their health and wellbeing.

### **Impaired Family Relationships**

Due to the complex medical needs of the patient, a spouse, if there is one, will have to take on extra duties as a caregiver. Intimate relationships and sexual functioning can be negatively impacted due to night time infusions and wearing medical equipment. Parenting duties might be impaired, primarily due to intense fatigue. A child might have to take on the role of a caregiver with their parent early in life.

## **2.3 Patients’ Experiences With Current Therapy**

Treatment is determined upon the individual needs of the patient. Many will need to use a combination of therapies. These include:

### **Dietary Adjustments**

A dietitian will devise and monitor customized menus and eating plans for each patient’s individual medical needs. In some cases, individuals need to ingest exceptionally large meals 5-7 times a day because they cannot digest the amount of nutrients from regular meals. Other dietary adjustments may need to be made for the consumption of protein sources, carbohydrates, and fluids. However, cooking specialized meals can also be difficult and time-consuming. Nutritional supplements and formulas can also be expensive. Even with dietary adjustments, the individual will still suffer from insufficient nutrient and mineral absorption.

A woman spoke to us of the difficulties of having to survive on chicken broth, Ensure® nutritional drinks, and only the tiniest bites of food all day long and, in spite of that, is still dealing with malnutrition, dehydration, weight loss, abdominal pain, nausea, and vomiting. Another woman said it was hard to

participate in meals because she would often have to leave the table multiple times to empty her ostomy bag, as stool emptying in to the bag was always liquefied and foul-smelling.

### **Enteral Nutrition (EN)**

This process involves the delivery of a special liquid food mixture to the stomach or small intestine through a feeding tube. This could help maintain the absorptive properties of the remaining intestine, but patients must have a partially functioning GI tract in order for this to be effective. This therapy can be difficult to manage as blockages in the feeding tubes occur often. Bacterial contamination in the tube can cause serious infections.

Gastroesophageal reflux disease occurs frequently with enteral feeding. Symptoms such as abdominal bloating, cramps, nausea, diarrhea and constipation are also common. Re-feeding syndrome can also occur. This causes a large increase in insulin levels which leads to a dramatic increase in oxygen consumption, and increased respiratory and cardiac demand.

### **Total Parenteral Nutrition (TPN)**

This process involves the delivery of fluids, electrolytes, and liquid nutrients into the bloodstream through a tube placed in the vein (intravenous or IV). This is a complex and sometimes dangerous therapy.

Complications include bacterial infections, IV catheter complications, blood clots, low bone calcium uptake, gallbladder disease, kidney disease, and liver problems. Liver and kidney problems can ultimately result in liver or kidney failure.

Infusions are usually done during sleep. Due to nausea caused by this feeding process, often patients become sleep-deprived. Mobility is compromised as the equipment can be heavy or cumbersome. This can lead to limitations in employment, education, parenting, and social interactions. TPN therapy is very expensive; it can cost more than \$100,000 a year and still the patient has ongoing debilitating symptoms.

### **Surgery**

There are a few surgical procedures that have been devised to increase the absorptive properties of the intestine. These involve artificially lengthening the intestine. Small bowel transplantation is sometimes attempted. However, complications from these surgeries can be severe and life threatening. Patients may require frequent hospitalizations due to infections and transplantation may cause serious damage to the liver or gallbladder.

## **2.4 Impact on Caregivers**

Caregivers need to devote physical, emotional, and financial resources to a family member with short bowel syndrome. They might need to take time off work and other necessary obligations to assist with preparing and administering feeding. Their time will also be limited by assisting the patient with other tasks. These can include cooking, cleaning, errands, physical hygiene care, and transporting the patient to medical appointments. These demands can result in financial hardship, stress, and anxiety. Relationships may become strained, which could lead to struggles within the family dynamic.

## Section 3 — Information about the Drug Being Reviewed

### 3.1 Information Gathering

Information was obtained through contact (interviews, etc.) with short bowel syndrome patients and included several who participated in the clinical trials for Revestive®. Additional information came from the Short Bowel Syndrome Oley Conference Roundtable in the US, which I participate in this year and had an opportunity to discuss issues with several other short bowel syndrome patients and caregivers. I also discussed care issues with three health care providers, a physician, a nurse, and a hospital pharmacist who work directly with short bowel syndrome.

### 3.2 What Are the Expectations for the New Drug or What Experiences Have Patients Had With the New Drug?

Patients have seen remarkable results from Revestive® when other treatments have failed. Not everyone has responded to the current available treatments, so more options are essential. Short bowel syndrome patients have been suffering for too long from an inadequate variety of not very effective treatment options.

Revestive® has the potential to improve the health and quality of life of individuals currently suffering from ineffective treatments that put an unnecessary burden on them. When other forms of management do not work well or specific patients cannot tolerate them, Revestive® could be an extremely valuable next step in getting symptoms under control.

Patients reported that they had more energy and less fatigue while taking Revestive®. One patient stated that “my husband said I was 10 years younger”. Another mentioned that he felt stronger while taking it and because of that he was able to become more active. A patient reported that her health improved to the extent that she was able to go back to work.

Another significant positive experience of this medication was that the patients were able to reduce their dependence on TPN. One patient was able to reduce her TPN feeding from six nights a week for 12 hours with two liters of formula to four times a week with 950 ml. Another patient was able to reduce her TPN use from 12 hours a day to three hours a day. This is life-altering, according to these patients with Revestive® experience.

Patients also reported that they were able to eat and actually enjoy eating. Typically, for a person with short bowel syndrome, food is the ‘enemy’. One patient stated that before treatment she had to “force-feed” herself because she had to constantly consume large amounts of food to be able to absorb nutrients. Another patient was able to eat food with her family at holiday dinners once she started taking Revestive®. Patients also reported that they were also able to gain and maintain a healthy body weight. This was a significant improvement for these patients.

Patients taking Revestive® told us that they noticed a dramatic reduction in diarrhea and an increase in regular bowel movements. One patient stated that when on the medication she no longer suffered from dehydration caused by frequent diarrhea. Prior to treatment one patient had to take large amounts of anti-diarrheal medications daily, however, she didn’t need them while taking Revestive®.

The patients found Revestive® easy and convenient to administer. The medication was also very well-tolerated by the users who we spoke with. Individuals who took it reported no negative side-effects, other than a patient who experienced abdominal cramping during the 2<sup>nd</sup> and 3<sup>rd</sup> week of treatment.

The medication also worked quickly to treat the condition. One patient said she gained four pounds after taking it for one day.

Other patients stated:

- “It’s miraculous”
- “I got my life back”
- “It completely changed my life”
- “Improvement in quality of life was huge”

Patients have seen remarkable results from Revestive® when other treatments have failed. Not everyone has responded to the current available treatments, so more options are essential. Short bowel syndrome patients have been suffering for too long from an inadequate variety of treatment options.

Revestive® has the potential to improve the health and quality of life of individuals currently suffering from ineffective treatments that put an unnecessary burden on them and the health care system. When other forms of management do not work well or specific patients cannot tolerate them, Revestive® could be an extremely valuable next step in getting symptoms under control.

We know this medication is expensive, but in the long-term, unhealthy people are more of a burden on the health care system than are healthy people. We urge you to ensure that this Health Canada approved medication for short bowel syndrome patients who are on TPN is included with your list of positive recommendations for coverage.

## Section 4 — Additional Information

One further note: With sadness, I want to report that one of the women with short bowel syndrome whom I got to know during the past year or so passed away recently. Her husband is distraught. Just knowing this couple, learning about her struggles with short bowel syndrome, and then hearing about her death has affected me substantially. Giving sick patients access to this medication is so very necessary and compassionate.