



## New Option For Patients With Rare Intestinal Disorder

(NAPSA)—It has been well documented that conditions affecting the masses, such as diabetes and obesity, continue to be on the rise in the U.S. Yet while significant funding continues to be dedicated to researching new medicines for these types of widespread conditions, the needs of those with rare diseases are often overlooked.

Thirty years ago, the Orphan Drug Act was passed as a means to facilitate the process of developing drugs to treat rare diseases, termed orphan drugs. Even though this legislation has been in place for three decades, many patients with rare diseases still lack options for treatment. In fact, there are only about 200 government-approved treatments for about 7,000 rare diseases recognized in the U.S.

However, there is newfound hope for a small group of patients living with a rare intestinal disorder. Scientists have been exploring a new treatment option for adults with short bowel syndrome called Gattex® (teduglutide [rDNA origin]) for injection. The U.S. Food and Drug Administration recently approved Gattex as a prescription medication for the treatment of adults with short bowel syndrome who are dependent on parenteral support. Considering this is the first major advance for the long-term treatment of short bowel syndrome in almost four decades, this is welcome news, and an example of how the Orphan Drug Act was designed to work.

Short bowel syndrome is a rare and debilitating disorder that leaves patients unable to absorb adequate amounts of nutrients and fluids from the foods they eat. Those with the disorder either have their intestines shortened considerably through surgery or in rare cases, it is congenital. Since the intestine is where we absorb the nutrients and fluids from food, patients living with short bowel syndrome are unable to live on a normal diet. Often plagued by debilitating diarrhea, some patients need to use the restroom as frequently as 25 times a day. If left untreated, short bowel syndrome can lead to dehydration, malnutrition, weight loss and even death.



**A new treatment may help people with a rare but difficult disease.**

Many years of research and clinical studies have culminated in the introduction of Gattex, which has experts hailing it as a breakthrough therapy that may significantly change the management of this life-threatening disorder. One such researcher, Dr. Lauren Schwartz of Mount Sinai School of Medicine, explains.

“Gattex may be a significant medical advance, because it is the first treatment to improve the intestine’s ability to absorb. As a result, patients treated with Gattex may reduce their dependence on lengthy and frequent treatment with parenteral support that has them tethered to an IV for as many as seven nights each week for as long as 12 hours at a time.”

Previously, many patients have relied on supplementing their nutritional needs through parenteral support, which ensures they receive the nutrients and fluids they do not absorb from the food they eat. While parenteral support has been a lifeline for these patients for decades, it is complex and demanding for many, because it is typically infused through a catheter into the bloodstream. It does not treat the intestine’s inability to absorb and even though parenteral support keeps patients alive, it exposes them to serious risks. The long-term use of parenteral support can sometimes lead to dangerous and life-threatening complications. The demands of parenteral support can create a socially-restrictive lifestyle for some.

As one of the researchers involved in the development of Gattex, Dr. Schwartz explains that the drug is similar to a peptide called GLP-2, which the body

produces naturally and works to enhance the intestine’s ability to absorb fluids and nutrients. In a key study, after 24 weeks of treatment, 54 percent of Gattex-treated patients were able to reduce the number of infusion days per week by one or more days, compared to 23 percent of those treated with placebo. And, in one study, some patients achieved complete independence from parenteral support.

What does this mean for patients? Reducing the number of days patients are tethered to an IV may give them at least one day of a fairly normal life. One day free of parenteral support a week would allow patients to experience the simple activities that many of us take for granted—joining family functions, traveling from home for a weekend, or just getting a full night of uninterrupted sleep.

To help facilitate access to treatment, the manufacturer of Gattex, NPS Pharmaceuticals, has developed a support program designed to provide assistance to patients and healthcare professionals, all at no charge. This patient support program, which is called “NPS Advantage,” aims to help in a number of ways, from insurance authorizations and assisting with appeals, to answering questions about how to use the drug. The company has created a dedicated website for NPS Advantage, [www.NPSAdvantage.com](http://www.NPSAdvantage.com), where patients and those who treat them can access more information about the program.

A key feature of NPS Advantage is the involvement of experienced guides, called Care Coordinators. NPS Advantage Care Coordinators provide a single point of contact for all support services available through the program and can help patients manage challenges that can prevent them from accessing NPS Pharmaceuticals’ therapies, such as Gattex.

You can find more information about Gattex, including full prescribing information and patient medication guide, at [www.Gattex.com](http://www.Gattex.com). You can also read about NPS Advantage at [www.NPSAdvantage.com](http://www.NPSAdvantage.com), or for more on the disorder, visit [www.shortbowelsupport.com](http://www.shortbowelsupport.com).

**Important Safety Information:** What is the most important information I should know about GATTEX? GATTEX may cause serious side effects, including: Making abnormal cells grow faster: GATTEX can make abnormal cells that are already in your body grow faster. There is an increased risk that abnormal cells could become cancer. If you get cancer of the bowel (intestines), liver, gallbladder or pancreas while using GATTEX, your healthcare provider should stop GATTEX. If you get other types of cancers, you and your healthcare provider should discuss the risks and benefits of using GATTEX. Polyps in the colon (large intestine): Polyps are growths on the inside of the colon. Your healthcare provider will have your colon checked for polyps within 6 months before starting GATTEX and have any polyps removed. To keep using GATTEX, your healthcare provider should have your colon checked for new polyps at the end of 1 year of using GATTEX. If no polyp is found, your healthcare provider should check you for polyps as needed and at least every 5 years and have any new polyps removed. If cancer is found in a polyp, your healthcare provider should stop GATTEX. Blockage of the bowel (intestines): A bowel blockage keeps food, fluids, and gas from moving through the bowels in the normal way. Tell your healthcare provider if you have any of these symptoms of a bowel blockage:

- trouble having a bowel movement or passing gas
- stomach area (abdomen) pain or swelling
- nausea
- vomiting
- swelling and blockage of your stoma opening, if you have a stoma.

If blockage is found, your healthcare provider may temporarily stop GATTEX. Swelling (inflammation) or blockage of your gallbladder or pancreas: Your healthcare provider will do tests to check your gallbladder and pancreas within 6 months before starting GATTEX and at least every 6 months while you are using GATTEX. Tell your healthcare provider right away if you get stomach area (abdomen) pain and tenderness, chills, fever, change in your stools, nausea, vomiting, dark urine, or yellowing of your skin or the whites of eyes. Fluid overload: Your healthcare provider will check you for too much fluid in your body. Too much fluid in your body may lead to heart failure, especially if you have heart problems. Tell your healthcare provider if you get swelling in your feet and ankles, you gain weight very quickly (water weight), or you have trouble breathing.

The most common side effects of GATTEX include:

- stomach area (abdomen) pain or swelling
- skin reaction where the injection was given
- nausea
- headache
- cold- or flu-like symptoms
- vomiting

Tell your healthcare provider if you have any side effect that bothers you or that does not go away.

What should I tell my healthcare provider before using GATTEX?

Tell your healthcare provider if you:

- Have cancer or a history of cancer
- Have or had polyps anywhere in your bowel (intestines) or rectum
- Have heart problems
- Have high blood pressure
- Have problems with your gallbladder, pancreas, kidneys
- Have any other medical condition

• Are pregnant or planning to become pregnant. It is not known if GATTEX will harm your unborn baby. Tell your healthcare provider right away if you become pregnant while using GATTEX.

• Are breastfeeding or plan to breastfeed. It is not known if GATTEX passes into your breast milk. You and your healthcare provider should decide if you will use GATTEX or breastfeed. You should not do both. Tell your healthcare providers about all the medicines you take, including prescription or over-the-counter medicines, vitamins, and herbal supplements. Using GATTEX with certain other medicines may affect each other, causing side effects. Your other healthcare providers may need to change the dose of any oral medicines you take while using GATTEX. Tell the healthcare provider who gives you GATTEX if you will be taking a new oral medicine. Call your doctor for medical advice about side effects. To report suspected side effects, contact NPS Pharmaceuticals at 1-855-5GATTEX (1-855-542-8839) or the FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).