NPS Pharmaceuticals Announces Completion of Treatment Phase in STEPS Registration Study of GATTEX in Short Bowel Syndrome

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BEDMINSTER, N.J., Jan 10, 2011 (BUSINESS WIRE) -- NPS Pharmaceuticals, Inc. (NASDAQ: NPSP) a specialty pharmaceutical company developing innovative therapeutics for rare gastrointestinal and endocrine disorders, today announced the completion of the 24-week treatment phase of the company's Phase 3 registration study of GATTEX® (teduglutide). The double-blind, placebo-controlled safety and efficacy study known as STEPS randomized 86 patients with parenteral nutrition (PN) dependent short bowel syndrome (SBS) to receive drug or placebo over a 24-week treatment period.

"With the last patient's final clinical visit, we remain on track with our development timelines for GATTEX in short bowel syndrome," said Francois Nader, MD, president and chief executive officer of NPS Pharmaceuticals. "We look forward to reporting top line results later this quarter and to filing for marketing authorization with the U.S. Food and Drug Administration if the results are positive."

NPS is also advancing STEPS 2, an open-label continuation study in which all participants will receive up to an additional 24 months of GATTEX therapy. Ninety-seven percent of eligible patients who completed STEPS elected to enroll in STEPS 2.

SBS is a rare disorder characterized by inadequate absorption of fluids and nutrients in people who have had a significant portion of their small intestine removed. Some SBS patients require the use of chronic PN or intravenous feeding to supplement and stabilize their nutritional needs. GATTEX is NPS' proprietary analog of human glucagon-like peptide 2 (GLP-2), a naturally-occurring peptide involved in the repair and maintenance of normal structure and function of the intestine. The goal of treatment with GATTEX is to restore the structural and functional integrity of the remaining intestine to reduce PN dependence.

About the STEPS Studies

STEPS is an international, double-blind, placebo-controlled Phase 3 registration study to confirm that GATTEX is well tolerated and reduces PN dependence in adults with SBS. The company believes positive results from STEPS will enable it to seek U.S. marketing approval for GATTEX.

Eighty-six PN-dependent SBS patients were randomized in the STEPS study at approximately 30 sites in North America and Europe. The trial included an initial three- to eight-week optimization and stabilization period, after which patients were randomized 1:1 to compare daily subcutaneous dosing of 0.05 mg/kg of GATTEX to placebo over a 24-week treatment period.

The primary efficacy endpoint is the percentage of patients who achieve a 20 percent or greater reduction in weekly PN volume at week 20 and maintain that response at week 24, when compared to baseline. The study's secondary endpoints will evaluate efficacy variables based on reductions in PN volume or the direct effects of improved intestinal absorption of fluid.

Patients who participated in STEPS had the option to enroll in STEPS 2, an open-label continuation study in which all participants will receive up to 24 months of GATTEX therapy.

NPS is advancing STEPS and STEPS 2 with the support of its partner Nycomed and the two companies are sharing the external clinical costs for the studies.

About Short Bowel Syndrome

Short bowel syndrome, or SBS, is a highly disabling condition that can impair a patient's quality-of-life and lead to serious life-threatening complications. SBS typically arises after extensive resection of the bowel due to Crohn's disease, ischemia or other conditions. SBS patients often suffer from malnutrition, severe diarrhea, dehydration, fatigue, osteopenia, and weight loss due to the reduced intestinal capacity to absorb nutrients, water, and electrolytes. The usual treatment for short bowel syndrome is nutritional support, including parenteral nutrition (PN) or intravenous feeding to supplement and stabilize nutritional needs.

Although PN can provide nutritional support for short bowel syndrome patients, it does not improve the body's own ability to absorb nutrients. PN is also associated with serious complications, such as infections, blood clots or liver damage, and the risks increase the longer patients are on PN. Patients on PN often experience a poor quality-of-life with difficulty sleeping, frequent urination and loss of independence.

There are an estimated 10,000 to 15,000 SBS patients in North America who are dependent on PN, the direct cost of which can exceed $100,000 annually per patient.

About GATTEX® (teduglutide)

GATTEX (teduglutide) is a novel, recombinant analog of human glucagon-like peptide 2, a protein involved in the rehabilitation of the intestinal lining. GATTEX is in Phase 3 development to reduce dependence on parenteral nutrition (PN) in patients with short bowel syndrome (SBS). NPS has reported findings from completed studies in which GATTEX demonstrated a favorable safety profile and reductions in mean PN volume from pretreatment baseline were observed. NPS is also advancing preclinical studies to evaluate teduglutide in additional intestinal failure related conditions.

Teduglutide has received orphan drug designation for the treatment of SBS from the U.S. Food and Drug Administration and the European Medicines Agency.

In 2007, NPS granted Nycomed the rights to develop and commercialize teduglutide outside the United States, Canada and Mexico. NPS retains all rights to teduglutide in North America.

About NPS Pharmaceuticals

NPS Pharmaceuticals is developing new treatment options for patients with rare gastrointestinal and endocrine disorders. The company is currently advancing two Phase 3 registration programs. Teduglutide, a proprietary analog of GLP-2, is being evaluated as GATTEX® in a Phase 3 registration study known as STEPS for intestinal failure associated with short bowel syndrome and is in preclinical development for chemotherapy-induced gastrointestinal mucositis and other pediatric indications. NPSP558 (parathyroid hormone 1-84 [rDNA origin] injection) is being evaluated in a Phase 3 registration study known as REPLACE as a hormone replacement therapy for hypoparathyroidism. NPS complements its proprietary programs with a royalty-based portfolio of products and product candidates that includes agreements with Amgen, Kyowa Hakko Kirin, Nycomed, and Ortho-McNeil Pharmaceutical.

Statements made in this press release, which are not historical in nature, constitute forward-looking statements for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. These statements are based on the company's current expectations and beliefs and are subject to a number of factors and uncertainties tha could cause actual results to differ materially from those described in the forward-looking statements. Risks associated to the company's business include, but are not limited to, the risks associated with any failure by the company to successfully complete its preclinical and clinical studies within the projected time frames or not at all, the risk of not gaining marketing approvals for GATTEX, the risks associated with the company's strategy, as well as other risk factors described in the company's periodic filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K and Form 10-Qs. All information in this press release is as of the date of this release and NPS undertakes no duty to update this information.

SOURCE: NPS Pharmaceuticals, Inc.

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