FDA Approves Natpara® (parathyroid hormone) for Injection as an Adjunct to Calcium and Vitamin D to Control Hypocalcemia in Patients with Hypoparathyroidism

First FDA-approved parathyroid hormone for hypoparathyroidism

Supported by largest clinical program ever conducted in patients with hypoparathyroidism

BEDMINSTER, N.J.--(BUSINESS WIRE)-- NPS Pharmaceuticals, Inc. (NASDAQ:NPSP), a global biopharmaceutical company pioneering and delivering therapies that transform the lives of patients with rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has approved Natpara® (parathyroid hormone) as an adjunct to calcium and vitamin D to control hypocalcemia in patients with hypoparathyroidism. Hypoparathyroidism is a rare endocrine disorder characterized by insufficient levels of parathyroid hormone, or PTH. Natpara, a bioengineered replica of human PTH, is expected to be available in the second quarter of 2015.

Because of the potential risk of osteosarcoma, Natpara is recommended only for patients who cannot be well-controlled on calcium supplements and active forms of vitamin D alone. Natpara was not studied in patients with hypoparathyroidism caused by calcium-sensing receptor mutations or in patients with acute post-surgical hypoparathyroidism.

"Natpara is a significant advance in the care of hypoparathyroidism and we are very pleased to offer the first and only parathyroid hormone approved for people living with this rare disorder. In clinical studies, Natpara has been shown to increase serum calcium levels and reduce the need for large doses of calcium and active vitamin D," said Francois Nader, MD, president and chief executive officer of NPS Pharma. "We extend our thanks to the patients and physicians who participated in our clinical development program, as we could not have achieved this important milestone without their contributions."

Hypoparathyroidism is a rare endocrine disorder in which the parathyroid glands fail to produce sufficient amounts of parathyroid hormone (PTH) or where the hormone lacks biologic activity. PTH plays a central role in a variety of critical physiological functions in the body. Insufficient levels of PTH lead to low levels of calcium and high levels of phosphate in the blood, and an inability to convert native vitamin D into its active state, which helps the body properly absorb oral calcium. Parathyroid hormone increases serum calcium by increasing renal tubular calcium reabsorption, increasing intestinal calcium absorption (i.e., by converting native vitamin D (25 OH) into its active form (1,25 OH2 vitamin D)) and by increasing bone turnover which releases calcium into the circulation.

"As someone who has endured the challenges of living with this rare disorder for more than 50 years and seen all five of my children diagnosed with it, I know first-hand how devastating hypoparathyroidism can be and how important it is to have new treatment options," said James Sanders, president of the Hypoparathyroidism Association. "It's critical that companies like NPS Pharma continue to research and develop medicines for people with rare diseases, as so many of us are often overlooked."

The FDA approval of Natpara was supported by 12 pharmacology studies and four company-sponsored efficacy and safety studies. The pivotal Phase 3 study, known as REPLACE, was a randomized, double-blind, placebo-controlled study and the largest clinical trial conducted to date in patients with hypoparathyroidism.

"Patients with hypoparathyroidism may benefit from having a replica of the actual human parathyroid hormone molecule that they are lacking," said Tamara Vokes, MD, professor of medicine at the University of Chicago, and program director of the University of Chicago Fellowship Training Program in Diabetes, Endocrinology and Metabolism. "In clinical studies, Natpara has been shown to control hypocalcemia in patients with hypoparathyroidism and reduce their need for oral calcium and active vitamin D."

Natpara will be made available through a Risk Evaluation and Mitigation Strategy (REMS) Program to mitigate the potential risk of osteosarcoma.

Natpara contains a Boxed Warning citing the potential risk of osteosarcoma. See below for Important Safety Information about Natpara, including the Boxed Warning, and Warnings and Precautions.
In Europe, the European Medicines Agency (EMA) has validated and initiated its review of NPS Pharma's marketing authorization application for Natpar™.

**NPS Advantage™**

NPS Pharma is committed to ensuring that patients have access to our medicines. To assist patients and healthcare professionals in facilitating care with Natpara, NPS Pharma has a free support program called NPS Advantage that includes a dedicated team of care coordinators and specialized nurses for patients treated with Natpara. This program is designed to help patients navigate all aspects of care and includes help with insurance authorizations and appeals, answering questions about Natpara and its use, and locating resources for patients to connect them to care. For more information, please visit [www.npsadvantage.com](http://www.npsadvantage.com).

NPS Pharma also provides support to independent non-profit organizations that provide assistance to patients who need help covering out-of-pocket medication costs.

**About Natpara® (parathyroid hormone) for Injection**

Natpara® (parathyroid hormone) for injection is indicated as an adjunct to calcium and vitamin D to control hypocalcemia in patients with hypoparathyroidism. Natpara is a bioengineered replica of human parathyroid hormone.

Because of the potential risk of osteosarcoma, Natpara is recommended only for patients who cannot be well-controlled on calcium supplements and active forms of vitamin D alone. Natpara was not studied in patients with hypoparathyroidism caused by calcium-sensing receptor mutations or in patients with acute post-surgical hypoparathyroidism.

In clinical studies, Natpara has been shown to increase serum calcium levels while reducing the need for oral calcium and active vitamin D and, in some cases, eliminate the need for active vitamin D altogether. The most common adverse reactions associated with Natpara and occurring in greater than 10% of individuals were: paresthesia, hypocalcemia, headache, hypercalcemia, nausea, and hypoesthesia, diarrhea, vomiting, arthralgia, hypercalciuria and pain in extremity.

Natpara is self-administered once daily by subcutaneous injection. The starting dose of Natpara is 50 mcg once daily.

Natpara received orphan drug status for the treatment of hypoparathyroidism from the FDA in 2007 and the EMA in 2013.

**Important Safety Information**

**What is NATPARA?**

- NATPARA is a prescription parathyroid hormone (PTH) used with calcium and vitamin D to control low blood calcium (hypocalcemia) in people with low PTH blood levels (hypoparathyroidism).
- NATPARA is only for people who do not respond well to treatment with calcium and active forms of vitamin D alone, because it may increase the possible risk of bone cancer (osteosarcoma).
- NATPARA was not studied in people with hypoparathyroidism caused by calcium sensing receptor mutations.
- NATPARA was not studied in people who get sudden hypoparathyroidism after surgery.
- It is not known if NATPARA is safe and effective for children 18 years of age and younger. NATPARA should not be used in children and young adults whose bones are still growing.

**What is the most important information I should know about NATPARA?**

**NATPARA may cause serious side effects, including:**

**Possible bone cancer (osteosarcoma).**

- During animal drug testing, NATPARA caused some rats to develop a bone cancer called osteosarcoma. It is not known if people who take NATPARA will have a higher chance of getting osteosarcoma.
  - **NATPARA is only available through the NATPARA Risk Evaluation and Mitigation Strategy (REMS) Program.** The purpose of the NATPARA REMS program is to inform patients about the potential risk of osteosarcoma associated with the use of NATPARA. For more information about this REMS program, call 1-855-628-7272 or go to [www.NATPARAREMS.com](http://www.nATPARAREMS.com).

**High blood calcium (hypercalcemia)**
NATPARA can cause some people to have a higher blood calcium level than normal.
- Your doctor should check your blood calcium before you start and during your treatment with NATPARA
- Tell your doctor if you have nausea, vomiting, constipation, low energy, or muscle weakness. These may be signs that you have too much calcium in your blood.

Low blood calcium (hypocalcemia)

- People who stop using or miss a dose of NATPARA may have an increased risk of severe low blood calcium levels
- Tell your doctor if you have tingling of your lips, tongue, fingers and feet, twitching of face muscles, cramping of feet and hands, seizures, depression, or have problems thinking or remembering.

Tell your doctor right away if you have any of these signs and symptoms of high or low blood calcium levels.

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

What are the most common side effects of NATPARA?

The most common side effects of NATPARA include

- Tingling, tickling, or a burning feeling of your skin (paresthesia), headache and nausea

These are not all the possible side effects of NATPARA. For more information, ask your doctor. Call your doctor for medical advice about side effects.

You may report side effects to the NPS Adverse Event/Product Complaint Line at 1-855-215-5550 or by calling the Food and Drug Administration (FDA) at 1-800-FDA-1088 or www.fda.gov/medwatch.


About NPS Pharma

NPS Pharma is a global biopharmaceutical company pioneering and delivering therapies that transform the lives of patients with rare diseases. The company's current therapeutic areas of focus are gastrointestinal disease and endocrine disorders. These include Short Bowel Syndrome, a potentially fatal gastrointestinal disorder in which patients may have to rely on parenteral nutrition for their survival; Hypoparathyroidism, a complex endocrine disorder in which the parathyroid glands are either absent or damaged, and the body produces insufficient or no parathyroid hormone; and Autosomal Dominant Hypocalcemia, an ultra-rare, genetic disorder of calcium homeostasis caused by mutations of the calcium-sensing receptor gene. NPS Pharma continues to seek in-licensing opportunities to develop new therapies for a broad range of rare diseases, and complements its proprietary programs with a royalty-based portfolio of products and product candidates that includes agreements with Amgen, GlaxoSmithKline, Janssen Pharmaceuticals, and Kyowa Hakko Kirin. NPS Pharma has operations in the U.S., Canada, Europe, Latin America and Japan. Learn more at: www.npsp.com

"NPS Pharma" and "NPS Pharmaceuticals" are the company's trademarks.

Disclosure notice

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 that could cause actual results to differ materially from those described in the forward-looking statements. Forward looking statements include, but are not limited to, statements concerning the company's future financial performance and plans for the commercialization of its products, beliefs or expectations regarding our products in development, statements concerning the company's plans for international expansion, beliefs or expectations regarding potential revenue and earnings from product sales, including beliefs regarding our ability to grow sales, expectations regarding the market size for our products, including those in development, and beliefs or expectations regarding our operating expenses. Risks associated to the company's business include, but are not limited to, the risks associated with any failure by the company to successfully commercialize Gattex/Revestive (teduglutide [rDNA origin]) for injection and Natpara (parathyroid hormone) for injection, including the risk that physicians and patients may not see the advantages of Gattex/Revestive or Natpara and may therefore be reluctant to utilize the products, the risk that private and
public payers may be reluctant to cover or provide reimbursement for Gattex or Natpara, the risks associated with the company’s strategy, global macroeconomic conditions, the impact of changes in management or staff levels, the effect of legislation effecting healthcare reform in the United States, 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 press release and the company undertakes no duty to update this information, whether as a result of new information, future events or otherwise.

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