Pfizer Logo

EMA Accepts Marketing Application for Somatrogon to Treat Pediatric Patients with Growth Hormone Deficiency

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– If approved, somatrogon will serve as a once-weekly treatment option –

NEW YORK & MIAMI--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) and OPKO Health Inc. (NASDAQ: OPK) announced today that the European Medicines Agency (EMA) has validated for review the Marketing Authorization Application (MAA) for somatrogon, a long-acting recombinant human growth hormone that is intended to be administered once-weekly for the treatment of pediatric patients with growth hormone deficiency (GHD). Pfizer expects a decision from the European Commission in 2022.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20210226005102/en/

“Today’s announcement is an example of our decades-long commitment to actively support the pediatric growth hormone deficiency community through therapeutic options that help children reach their full potential,” said Brenda Cooperstone, MD, Chief Development Officer, Rare Disease, Pfizer Global Product Development. “If approved in the EU, somatrogon will represent an important advancement, as this long-acting, weekly treatment may reduce the burden of daily injections on children, their loved ones, and caregivers. We look forward to continuing to work with the EMA to evolve the treatment paradigm for pediatric patients with growth hormone deficiency.”

“We are very appreciative of the children and their families that participated in the clinical trials, bringing us one step closer to providing a long-acting treatment option for pediatric growth hormone deficiency. If somatrogon is approved, we look forward to positively impacting the quality of life for children living with GHD,” said Phillip Frost, Chairman and CEO of OPKO.

The submission is supported by the results of a global, Phase 3 trial evaluating the safety and efficacy of somatrogon administered once-weekly to pediatric patients with GHD. This study met its primary endpoint of non-inferiority compared to GENOTROPIN® (somatropin) for injection administered once daily, as measured by annual height velocity at 12 months. In addition, change in height standard deviation scores at 6 and 12 months, key secondary endpoints, were higher in the somatrogon dosed once-weekly cohort in comparison to the somatropin dosed once-daily cohort. Moreover, at 6 months, change in height velocity, another key secondary endpoint, was higher in the somatrogon dosed once-weekly cohort in comparison to the somatropin dosed once-daily cohort. These common measures of growth are employed in the clinical setting to measure the potential level of catch-up growth that subjects may experience relative to the heights of their age and gender matched peers.

Somatrogon was generally well tolerated in the study and comparable to that of somatropin administered once-daily with respect to the types, numbers and severity of the adverse events observed between the treatment arms.

Separately, in October 2020, Pfizer and OPKO announced findings from the C0311002 trial, a Phase 3, randomized, multicenter, open-label, crossover study evaluating somatrogon dosed once-weekly in children 3 to <18 years of age with GHD, which met its primary endpoint of improved treatment burden compared to GENOTROPIN® (somatropin) for injection administered once-daily. Top-line results from the study demonstrated that treatment with somatrogon once-weekly improved the mean overall Life Interference total score after 12 weeks of treatment compared to treatment with somatropin administered once-daily. In addition, key secondary endpoints showed an overall benefit in treatment experience with the somatrogon once-weekly dosing regimen compared to the somatropin once-daily dosing regimen. These data have also been submitted to the EMA.

In January 2021, Pfizer and OPKO announced that the US Food and Drug Administration (FDA) accepted for filing the initial Biologics License Application (BLA) for somatrogon with a target PDUFA action date of October 2021. In January 2021, a New Drug Application (NDA) was submitted to PMDA in Japan for somatrogon.

In 2014, Pfizer and OPKO entered into a worldwide agreement for the development and commercialization of somatrogon for the treatment of GHD. Under the agreement, OPKO is responsible for conducting the clinical program and Pfizer is responsible for registering and commercializing the product.

About the Studies

The somatrogon Phase 3 trial is a randomized, open-label, active-controlled study conducted in over 20 countries. This study enrolled and treated 224 pediatric patients, treatment-naïve children with growth hormone deficiency who were randomized 1:1 into two arms: somatrogon administered at a dose of 0.66 mg/kg body weight once-weekly vs GENOTROPIN® (somatropin) administered at a dose of 0.034 mg/kg body weight once daily. The primary endpoint of the trial was height velocity at 12 months. Secondary endpoints included change in height standard deviation at 6 and 12 months, safety and pharmacodynamic measures. Children completing this study had the opportunity to enroll in a global, open-label, multicenter, long-term extension study, in which they were able to either continue receiving or switch to somatrogon. Approximately 95% of the patients switched into the open-label extension study and received somatrogon treatment.

C0311002 is a Phase 3, randomized, multicenter, open-label, crossover study assessing subject perception of treatment burden with use of somatrogon administered once-weekly versus GENOTROPIN® administered once-daily in children 3 to <18 years of age with growth hormone deficiency (GHD). The primary objective of the crossover study, which included 87 randomized and treated subjects (43 randomized to Sequence 1 [somatropin followed by somatrogon] and 44 randomized to Sequence 2 [somatrogon followed by somatropin], was to evaluate the treatment burden of a somatrogon once-weekly injection schedule and a somatropin once-daily injection schedule, as assessed by the difference in mean overall Life Interference total scores after each 12-week treatment schedule experience.

About Somatrogon

Somatrogon is an investigational biologic product that is glycosylated and comprises the amino acid sequence of human growth hormone and one copy of the C-terminal peptide (CTP) from the beta chain of human chorionic gonadotropin (hCG) at the N-terminus and two copies of CTP (in tandem) at the C-terminus. The glycosylation and CTP domains account for the half-life of the molecule. Somatrogon has received Orphan Drug designation in the U.S. and the EU for the treatment of growth hormone deficiency.

About Growth Hormone Deficiency

Growth hormone deficiency is a rare disease characterized by the inadequate secretion of growth hormone from the pituitary gland and affects one in approximately 4,000 to 10,000 people. In children, this disease can be caused by genetic mutations or acquired after birth. Because the patient's pituitary gland secretes inadequate levels of somatropin, the hormone that causes growth, his or her height may be affected and puberty may be delayed. Without treatment, he or she will have persistent growth attenuation, a very short height in adulthood, and may experience other health problems.

About GENOTROPIN® (somatropin)

GENOTROPIN is a man-made, prescription treatment option, approved in the United States for children who do not make enough growth hormone on their own, have the genetic condition called Prader-Willi syndrome (PWS), were born smaller than most other babies, have the genetic condition called Turner syndrome (TS) or have idiopathic short stature (ISS). GENOTROPIN is also approved to treat adults with growth hormone deficiency. GENOTROPIN is taken by injection just below the skin and is available in a wide range of devices to fit a range of individual dosing needs. GENOTROPIN is just like the natural growth hormone that our bodies make and has an established safety profile.

About OPKO Health, Inc.

OPKO is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development, and commercialization expertise and novel and proprietary technologies. For more information, visit http://www.OPKO.com.

Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

Pfizer Inc.: Breakthroughs that change patients' lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 170 years, we have worked to make a difference for all who rely on us. We routinely post information that may

be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE:

The information contained in this release is as of February 26, 2021. Pfizer and OPKO assume no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about an investigational growth hormone deficiency therapy, somatrogon, including a potential indication in the EU for once-weekly treatment of pediatric patients with growth hormone deficiency, including its potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when applications may be filed in any additional jurisdictions for somatrogon for the treatment of pediatric patients with growth hormone deficiency or in any jurisdictions for any other potential indications for somatrogon; whether and when the European Commission may approve the marketing authorization application for somatrogon for the treatment of pediatric patients with growth hormone deficiency and whether and when regulatory authorities in any jurisdictions may approve any such other applications that may be pending or filed (including the applications pending in the U.S. and Japan), which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether somatrogon will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of somatrogon; uncertainties regarding the impact of COVID-19 on Pfizer’s or OPKO’s business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's and OPKO’s respective Annual Reports on Form 10-K for the fiscal year ended December 31, 2020 and in their respective subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors", "Forward-Looking Information and Factors That May Affect Future Results" and “Cautionary Statement Regarding Forward-Looking Statements”, as well as in their respective subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and, as applicable, www.pfizer.com and www.OPKO.com .

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