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EMA Adopts a Positive CHMP Opinion for Pfizer's and OPKO's Somatrogon, a Long-Acting Treatment for Pediatric Growth Hormone Deficiency

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—If approved by the European Commission, somatrogon will offer children and adolescents living with GHD a once-weekly treatment option resulting in fewer doses per year compared to daily treatment options—

NEW YORK, NY [December 17, 2021] - Pfizer Inc. (NYSE: PFE) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending somatrogon, a once-weekly long-acting recombinant human growth hormone, for marketing authorization to treat children and adolescents from 3 years of age with growth disturbance due to insufficient secretion of growth hormone. A decision from the European Commission (EC) is expected in early 2022.

"The CHMP's positive opinion of somatrogon is an important milestone in our efforts to deliver therapeutic options that can help children reach their full potential," said Brenda Cooperstone, M.D., Chief Development Officer, Rare Disease, Pfizer Global Product Development. "For decades, Pfizer has been committed to improving the lives of those impacted by growth hormone deficiency and we look forward to working closely with the European Commission to hopefully soon provide a once-weekly treatment option for the pediatric growth hormone deficiency (GHD) community in the European Union."

GHD is a rare disease characterized by the inadequate secretion of growth hormone, and affects one in approximately 4,000 to 10,000 children worldwide.[1,2] Without treatment, affected children will have persistent growth attenuation and a very short height in adulthood.[3,4] Children may also experience other problems with physical health and mental well-being.[3,4]

The recommendation for somatrogon is based on the results from a global, Phase 3 randomized, open-label, active controlled study which evaluated the safety and efficacy of once-weekly somatrogon compared to GENOTROPIN® (somatropin) for injection administered once-daily.[5] This study met its primary endpoint of somatrogon non-inferiority compared to GENOTROPIN® (somatropin) for injection administered once daily, as measured by annual height velocity at 12 months.[5]

"Daily injections are often challenging for those impacted by pediatric growth hormone deficiency. Children may object to receiving a shot every day and caregivers may feel strains on their relationships," said Jamie Harvey, CEO of the International Coalition of Organizations Supporting Endocrine Patients (ICOSEP). "At ICOSEP, we understand these challenges and are hopeful that if somatrogon receives marketing authorization in the European Union, this once-weekly treatment option may help children living with growth hormone deficiency reach their full potential."

Separately, a Phase 3, randomized, multicenter, open-label, crossover study assessed the perception of treatment burden of once-weekly somatrogon compared to GENOTROPIN® (somatropin) for injection administered once-daily.[6] 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.[6]

In 2014, Pfizer and OPKO Health 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 somatrogon for GHD.

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, 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. This study met its primary endpoint of somatrogon non-inferiority compared to GENOTROPIN® (somatropin) for injection administered once daily, as measured by annual height velocity at 12 months. Secondary endpoints included height velocity at 6 months, 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.[5]

Separately, 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) who were stable on GH therapy. 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. 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.[6]

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 children.[1,2] In children, this disease can be caused by genetic mutations or acquired after birth.[1,7] Because the patient's pituitary gland secretes inadequate levels of somatropin, the hormone that causes growth, a child’s height may be affected and puberty may be delayed.[1,3,4] Without treatment, affected children will have persistent growth attenuation and a very short height in adulthood.[3,4] Children may also experience other problems with physical health and mental well-being.[3,4]

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 of the CTP domains account for the half-life of the molecule.

About GENOTROPIN® (somatropin)
GENOTROPIN® is a man-made, prescription treatment option. The indications GENOTROPIN® is approved for vary by country and include adult GHD, growth failure in children due to GHD, Prader-Willi Syndrome, Idiopathic Short Stature, Turner Syndrome, Small for Gestational Age (with no catch-up growth) and Chronic Renal Insufficiency. 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.

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.

About Pfizer: 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 150 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 December 17, 2021. Pfizer assumes 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 drug 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 regulatory authorities in any jurisdictions may approve any such other applications that may be pending or filed (including the applications filed in the EU, 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 business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report 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" and "Forward-Looking Information and Factors That May Affect Future Results", 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 www.pfizer.com.

Pfizer
Media Relations:
eMail: EUPress@pfizer.com
Phone: +44 (0) 1737 332 335

Investor Contact:
IR@Pfizer.com
+1 (212) 733-4848

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