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Pfizer and OPKO Provide Update on the Biologics License Application for Somatrogon for Pediatric Growth Hormone Deficiency

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NEW YORK & MIAMI--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) and OPKO Health, Inc. (NASDAQ: OPK) announced today that the U.S. Food and Drug Administration (FDA) issued a Complete Response Letter (CRL) for the Biologics License Application (BLA) for somatrogon. Somatrogon is an investigational once-weekly long-acting recombinant human growth hormone for the treatment of growth hormone deficiency (GHD) in pediatric patients. Pfizer is evaluating the FDA's comments and will work with the agency to determine an appropriate path forward.

"We remain confident in the potential treatment benefits that somatrogon has to offer patients around the world," said Brenda Cooperstone, MD, Chief Development Officer, Rare Disease, Pfizer Global Product Development. "We will work closely with the FDA to determine the best path forward to bring this important once-weekly treatment option to pediatric growth hormone deficiency patients and their families."

Regulatory applications for somatrogon have been submitted to several countries around the world for review. Earlier this week, Japan's Ministry of Health, Labour and Welfare approved NGENLA® (somatrogon) Inj. 24 mg Pens and 60mg Pens, for the long-term treatment of pediatric patients who have growth failure due to an inadequate secretion of endogenous growth hormone. In 2021, Health Canada approved NGENLA® for the long-term treatment of pediatric patients who have GHD, and Australia's Therapeutic Goods Administration (TGA) approved NGENLA® for the long-term treatment of pediatric patients with growth disturbance due to insufficient secretion of growth hormone. Furthermore, in December 2021, the Committee for Medicinal Products for Human Use (CHMP) of EMA issued a positive opinion recommending somatrogon for marketing authorization in the EU, 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.

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 for GHD.

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.[1,3] 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]

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 January 21, 2022. 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 U.S. 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; uncertainties regarding the company's ability to address the comments in the complete response letter to the satisfaction of the FDA;whether and when drug applications may be filed in any other jurisdictions for any potential indication for somatrogon; whether and

when the BLA pending with the FDA for somatrogon for the treatment of pediatric patients with growth hormone deficiency may be approved and whether and when regulatory authorities in any jurisdictions may approve any such other applications that may be pending or filed (including the application filed in the EU), 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 and 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 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, www.pfizer.com, and www.opko.com.

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.

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[1] National Organization for Rare Disorders. Growth Hormone Deficiency. https://rarediseases.org/rare-diseases/growth-hormone-deficiency/. Accessed August 24, 2021.
[2] Stanley T. Diagnosis of growth hormone deficiency in childhood. *Curr Opin Endocrinol Diabetes Obes*. 2012;19(1):47-52. doi:10.1097/MED.0b13e32834ec952.
[3] Cerbone M, Dattani MT. Progression from isolated growth hormone deficiency to combined pituitary hormone deficiency. *Growth Horm IGF Res*. 2017;37:19-25. doi:10.1016/j.ghir.2017.10.005.
[4] Ergun-Longmire B, Wajnrajch M. Growth and growth disorders. Feingold KR, Anawalt B, Boyce A, et al., editors. Endotext [Internet]. South Dartmouth (MA): MDText.com, Inc.; 2000. Available from: https://www.ncbi.nlm.nih.gov/books/NBK279142/

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