



New Drug Application Submitted to PMDA in Japan for Somatrogen, to Treat Pediatric Patients with Growth Hormone Deficiency

MIAMI (January 28, 2021) – OPKO Health, Inc. (NASDAQ: OPK) announced today that its partner, Pfizer Japan Inc., submitted a New Drug Application (NDA) to the Ministry of Health, Labour, and Welfare in Japan for somatrogen, 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).

This submission is based on the results of the Japan Phase 3 and global Phase 3 clinical studies, conducted in subjects with pediatric GHD, in which the efficacy and safety of somatrogen administered once weekly were compared with GENOTROPIN® (somatropin), a recombinant human growth hormone for injection, administered once daily. In both studies, somatrogen showed comparable efficacy to GENOTROPIN for the primary endpoint of annual height velocity at 12 months of treatment. Somatrogen was generally well tolerated in both studies, with comparable safety to that of GENOTROPIN administered once-daily with respect to the types, numbers and severity of the adverse events observed between the treatment arms.

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

About the Japan Phase 3 Study

The Phase 3 study of somatrogen in 44 treatment-naïve Japanese pre-pubertal children with pediatric GHD was a 12-month, open-label, randomized, active-controlled, parallel-group study of the efficacy and safety of weekly somatrogen compared to recombinant human growth hormone (r-hGH), GENOTROPIN® (somatropin) for injection treatment administered once-daily. Eligible patients were randomized in a 1:1 ratio to receive either once-weekly somatrogen or GENOTROPIN administered once-daily (reference therapy, 0.025 mg/kg/day which is equivalent to 0.175 mg/kg/week). To obtain pharmacokinetic information of three different weekly doses in Japanese pediatric GHD patients, somatrogen treated patients received 0.25 mg/kg/week for 2 weeks, followed by 0.48 mg/kg/week for 2 weeks followed by 0.66 mg/kg/week for the remaining 46 weeks. Somatrogen was administered subcutaneously using a single patient use, multi-dose, disposable, pre-filled pen, the same pen used in the global study, while GENOTROPIN was administered using approved commercial products in Japan.

About Somatrogen

Somatrogen 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. Somatrogen has received Orphan Drug designation in the U.S. and the EU for the treatment of children and adults with 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

GENOTROPIN (somatropin) 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>.

Cautionary Statement Regarding Forward-Looking Statements

This press release contains "forward-looking statements," as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as "expects," "plans," "projects," "will," "may," "anticipates," "believes," "should," "intends," "estimates," and other words of similar meaning, including statements regarding whether the Ministry of Health, Labour, and Welfare ("Ministry") may approve the NDA for somatrogon for the treatment of pediatric patients with growth hormone deficiency, whether the Ministry will be satisfied with the results from our clinical study, whether the Ministry will interpret the clinical trial data in a similar manner to us, whether, if approved, somatrogon will be commercially successful in Japan for its approved indication, that the market for somatrogon exists, that earlier clinical results of effectiveness and safety may not be reproducible or indicative of future results, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, strategies or prospects. Many factors could cause our actual activities or results to differ materially from the activities and results anticipated in forward-looking statements. These factors include those described in the OPKO Health, Inc. Annual Reports on Form 10-K filed and to be filed with the Securities and Exchange Commission and in its other filings with the Securities and Exchange Commission. In addition, forward-looking statements may also be adversely affected by general market factors, competitive product development, product availability, federal and state regulations and legislation, the regulatory process for new products and indications, manufacturing issues that may arise, and positions and litigation, among other factors. The forward-looking statements contained in this press release speak only as of the date the statements were made, and we do

not undertake any obligation to update forward-looking statements. We intend that all forward-looking statements be subject to the safe-harbor provisions of the PSLRA.

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