

September 6, 2016



OPKO to Present Long-acting Human Growth Hormone (hGH-CTP) Phase 2 Pediatric Growth Hormone Deficiency Data at the 55th Annual Meeting of the European Society for Paediatric Endocrinology

MIAMI--(BUSINESS WIRE)-- OPKO Health, Inc. (NASDAQ:OPK) will present abstracts at the 55th Annual Meeting of the European Society for Paediatric Endocrinology (ESPE) to be held in Paris, France on September 10-12, 2016.

Twenty four month phase 2 efficacy, safety, pharmacokinetic, and pharmacodynamic data from OPKO's long acting human growth hormone, hGH-CTP, in growth hormone deficient children will be presented.

OPKO will also hold a workshop for participating endocrinologists to review final phase 3 study plans.

OPKO expects to shortly initiate a global pivotal phase 3 study in pre-pubertal growth hormone deficient children to evaluate weekly single doses of hGH-CTP versus daily injections of a presently used growth hormone product. OPKO has a world-wide collaboration agreement with Pfizer Inc. for the development and commercialization of hGH-CTP.

OPKO presentations on hGH-CTP include the following:

Oral Presentations

Date

**Session
name:
Growth:
Clinical
Date:
September
12, 2016**

**Batch-to-Batch Consistency of a Highly O-Glycosylated Long-Acting
Human Growth Hormone (MOD-4023)**

**Session
time: 09:15
- 10:45 am
CEST**

[ESPE
Abstracts
\(2016\) 86
RFC8.3](#)

**Session
name:
Growth:
Clinical
Date:
September
12, 2016**

**Efficacy of Once-Weekly Administration of CTP-Modified Human Growth
Hormone (MOD-4023): 24-Month Complete Database Results of a Phase
2 Study in Children with Growth Hormone Deficiency**

**Session
time: 09:15
- 10:45 am
CEST**

[ESPE
Abstracts
\(2016\) 86
RFC8.8](#)

**Session
name:
Growth:
Clinical
Date:
September
12, 2016**

**Safety and Tolerability of Once-Weekly Administration of CTP-Modified
Human Growth Hormone (MOD-4023): 24-month Complete Dataset
Results of a Phase 2 Study in Children with Growth Hormone Deficiency**

**Session
time: 9:15-
10:45 am
CEST**

[ESPE
Abstracts
\(2016\) 86
RFC8.7](#)

**Session
name:
Growth:
Clinical
Date:
September
12, 2016**

**Optimal Sampling of IGF-1 During Weekly Administration of a Long
Acting Human Growth Hormone (MOD 4023)**

**Session
time: 09:15
- 10:45 am
CEST**

[ESPE
Abstracts
\(2016\) 86
RFC8.5](#)

Additional information will be presented as e-posters:

2nd Year Pharmacokinetic and Pharmacodynamic Modeling of Long Acting Human

Growth Hormone (MOD-4023) in Growth Hormone Deficient Children: [ESPE Abstracts \(2016\) 86 P-P1-625](#)

Immunogenicity Results of Once-Weekly Administration of CTP-Modified Human Growth Hormone (MOD-4023): A Phase 2 Study in Children with Growth Hormone Deficiency: [ESPE Abstracts \(2016\) 86 P-P1-615](#)

About hGH-CTP

hGH-CTP is a novel, long acting recombinant human growth hormone analog being developed by OPKO for the treatment of children with growth failure due to inadequate growth hormone secretion, and adults with growth hormone deficiency.

OPKO's proprietary technology prolongs a therapeutic protein's half life without the use of polymers, encapsulation techniques, or nanoparticles. This technology uses a natural peptide, the C-terminal peptide (CTP) of the beta chain of human chorionic gonadotropin (hCG). hGH-CTP has been granted orphan drug designation in the U.S. and Europe for both adults and children with growth hormone deficiency.

About OPKO Health

OPKO Health, Inc. is a diversified healthcare company that seeks to establish industry-leading positions in large, rapidly growing markets. Our diagnostics business includes Bio-Reference Laboratories, the third-largest clinical laboratory in the U.S. with a core genetic testing business and a 420 person sales force to drive growth and sell new products, such as the 4Kscore prostate cancer test and the Claros 1 in office immunoassay platform. Our pharmaceutical business features RAYALDEE, an FDA approved treatment for stage 3-4 CKD patients with secondary hyperparathyroidism and vitamin D insufficiency, and VARUBI™ for chemotherapy-induced nausea and vomiting (oral formulation launched by partner Tesaro and IV formulation PDUFA is January 2017). Our biologics products under development are hGH-CTP, a once weekly human growth hormone for injection (partnered with Pfizer), long acting Factor VIIa for hemophilia (in Phase 2a) and a long acting oxyntomodulin for diabetes and obesity (in Phase 1). OPKO has production and distribution assets in several countries abroad, strategic investments and an active business development strategy. More information is available at www.opko.com.

SAFE HARBOR STATEMENT

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 expected timing of study completion, OPKO's ability to commence its phase 3 pediatric study for hGH-CTP later this year, whether OPKO's clinical trials for hGH-CTP in adult and pediatric growth hormone deficiency will be successful or generate data to support marketing approval, whether study results will demonstrate hGH-CTP is non-inferior compared to daily hGH, whether it will prove to be safe and effective, whether hGH-CTP will be successfully developed or commercialized, expectations regarding the product and its market potential, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, 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 our filings with the Securities and Exchange Commission, as well as the risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments. 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, patent positions, litigation, and the success of our collaboration on hGH-CTP with Pfizer, Inc. 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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