MOD-4023 (hGH–CTP)
Long Acting Human Growth Hormone
for Growth Hormone Replacement Therapy

Company background
OPKO Biologics is a clinical-stage company, a subsidiary of OPKO Health Ltd, which develops long-acting versions of therapeutic drugs utilizing two proprietary technologies: CTP for therapeutic proteins and reversible PEGylation for peptides and small molecules.

Technology
CTP is a naturally-occurring human peptide evolved to provide the fertility hormone hCG with the durability required for maintaining pregnancy. Fusing CTP by rDNA technology to a protein significantly extends its half-life. Clinical and pre-clinical studies show that the CTP technology appears to be safe and effective in extending the duration of all proteins tested to date. CTP’s safety and efficacy have also been validated by the 2010 EU marketing approval of Merck’s long-acting fertility drug Elonva® (FSH-CTP). A single injection of Elonva® replaces a week-long regimen of 7 daily FSH injections. CTP was discovered by researchers at Washington University in St. Louis and is exclusively licensed to OPKO for all proteins and peptides, except for four endocrine proteins that are licensed to Merck & Co.

Target indication
Growth hormone replacement therapy in growth hormone-deficient (GHD) adults and children.

Product description
MOD-4023 is a novel long-acting human growth hormone (hGH) which contains the authentic hGH amino acid sequence linked to 3 copies of the CTP “cassette”.

- Final Presentation: The drug product will be a refrigerated, liquid non viscous formulation
- Injected using a disposable easy to handle pen device with a thin needle and low injection volume
- Route of administration: subcutaneous injection

Target frequency of administration
Single weekly subcutaneous injection

Pfizer and OPKO have entered into a worldwide agreement for the development and commercialization of OPKO’s long-acting growth hormone for the primary indications of adult growth hormone deficiency, pediatric growth hormone deficiency, and the treatment of growth failure in children born small for gestational age who fail to demonstrate catch-up growth by 2 years of age.
Clinical Program

The interim analysis of the pediatric phase 2 study suggests that MOD-4023 has an efficacy and safety profile comparable to daily GH therapy. Twelve-month data suggests that MOD-4023 achieved annual HV similar to the Genotropin® comparator arm.

Regulatory information

- Granted orphan drug designation in both the USA and the EU for growth hormone deficiency (pediatric and adult).
- Plans for parallel regulatory submissions to the FDA and EMA.

About our planned phase 3 pediatric study

officially begun study initiation activities that will enable a global Phase 3 Pediatric GHD study start in 2016.

Study objective

To demonstrate that weekly MOD-4023 administration is clinically comparable (non-inferior) to daily Genotropin® administration in terms of safety and efficacy features.

Study outline

A 12-month, open-label, randomized, active-controlled, parallel-group study comparing efficacy and safety of weekly MOD-4023 to daily growth hormone (Genotropin®) at a proposed dose of 34 µg/kg/day.

Anticipated study initiation

H1 2016

For the upcoming study we are looking for:

1. Pre-pubertal girls and boys between ≥ 3 and ≤ 10 (girls) or ≤ 11 (boys) years old with either isolated GHD or GH insufficiency as part of a multiple pituitary hormone deficiency.
2. GHD was confirmed by GH levels ≤10 ng/ml
3. No prior exposure to any r-hGH therapy (naïve).

If you would like to participate in our upcoming study, please contact:

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For more information please approach us at our ESPE booth: no. 22