OPKO Biologics is developing bio-better long acting versions of existing therapeutic proteins utilizing a technology called CTP. The technology involves fusion of the C terminus peptide of hCG to one or both ends of the target protein. The MOD-4023 (hGH-CTP) is a long acting hGH, clinically validated and proven as a safe and efficient way for increasing the half-life of several therapeutic proteins while maintaining their biological activity.

**Introduction**

A one-year, randomized, comparator-controlled Phase 2 study that included 53 pre-pubertal GHD children with GHD was conducted. The patients received once-weekly SC injections of MOD4023 (0.25, 0.48, or 0.66 mg/kg/week), or daily hGH (34 µg/kg/day) as control.

**Study Outline**

Serum samples for immunogenicity analysis were collected at pre-dose, and after 6 and 12 months of MOD-4023/ hGH treatment. Each sample was analyzed in screening format. Samples reactive for anti-MOD-4023 Ab’s were confirmed for MOD-4023 in specificity format. Then, samples confirmed positive for anti-MOD-4023 binding Ab’s, were titered and further characterized (anti CTP, anti GH), as well as for anti-MOD-4023 and anti-hGH neutralizing Ab’s using cell-based assays.

**Results**

- **Cohort**
  - ADA
    - MOD-4023 0.25 mg/kg/wk: 0/13
    - MOD-4023 0.48 mg/kg/wk: 3/15
    - MOD-4023 0.66 mg/kg/wk: 2/14
    - MOD-4023 treatment (Total): 11.4% (5/42)
  - Genotropin treatment (0.034 µg/kg/day): 9% (1/11)

- **NAb**
  - Total: 0/53

- A comparable rate of ADAs between MOD-4023 and Genotropin treatment arms.
- Low Ab titer found for the Positive subjects to ADA.
- No neutralized anti-MOD-4023 or hGH Abs.
- No AE’s which are Abs related were reported.
- No effect on PK/PD (IGF-1) nor on HV were observed.

**Conclusions**

- Precise, sensitive and reproducible qualitative methods were validated for the detection of binding as well as neutralizing Ab’s, against MOD-4023 and it’s moieties; hGH and CTP.
- During the first 12 months of the Phase 2 study in GHD pediatric population, MOD-4023 has demonstrated a comparable immunogenicity profile to the observed (as well as reported; Peter et al 2013) with daily administrated hGH.
- The data affirms that a single weekly injection of MOD-4023 has the potential to safely replace the daily hGH injections in children with GHD.

The Immunogenicity data support the initiation of a Phase 3 study in GHD pediatric population using a single weekly injection of MOD-4023.

**Disclosure statement:** nothing to disclose.