Helixmith shows long-term safety and efficacy of VM202 for painful DPN patients in DPN phase 3-1b extension study

Results to be presented at the 2019 Fall International Convention of The Pharmaceutical Society of Korea

October 7, 2019 -- Helixmith announces results showing VM202 met the primary and secondary endpoints – safety and efficacy at 12 months, respectively – in a phase 3 extension study (DPN 3-1b) conducted in the US for painful diabetic peripheral neuropathy (DPN) subjects. This study was an extension of the original 500-subject DPN 3-1 study conducted under a separate protocol that was submitted to the US FDA.

The objective of the DPN 3-1b study was to assess the safety and efficacy of VM202 at 12 months after the first injection among patients who participated in the DPN 3-1 study. The DPN 3-1b study was initiated to follow regulatory guidance to collect long-term safety data. Efficacy was also collected as a secondary endpoint.

The extension study, which began in January 2019, enrolled 101 subjects (n=65 in the VM202 group and n=36 in the placebo group) of the original 500 subjects who had been in the DPN 3-1 study; of these, 99 completed the full 3-month extension. The study included 12 of the 25 clinical trial sites that actively participated in the original DPN 3-1 study.

The key results from the DPN 3-1b study are as follows:

(1) Safety: The study showed that VM202 appeared to be safe and well-tolerated, as was seen in the DPN 3-1 study. The occurrence of adverse events (AEs) was no different between the VM202 and placebo groups; the Treatment-Emergent AE occurrence rate was lower in the VM202 group (21.5%) than in the placebo group (25.0%). No serious adverse events (SAEs) related to the medication were observed.

(2) Efficacy (Pain reduction): In the intent-to-treat (ITT) population (N=101), VM202 showed clinically meaningful and statistically significant pain reductions vs. placebo at months 6, 9, and 12. The p-values were 0.010, 0.044, and 0.046, respectively. The pain reduction differences (delta) between the two arms at months 6, 9, and 12 were –1.1, –0.9, and –0.9, respectively. The data showed a trend toward efficacy at 3 months after the initial injection but the difference not statistically significant. (Note: Data for the 3-, 6-, and 9-month timepoints were obtained during the DPN 3-1 trial.)

(3) Greater efficacy in subjects not taking DPN medication gabapentin and pregabalin: The difference in pain reduction between the VM202 and placebo groups was greater in the population not on gabapentinoid medication (N=53 total, of which 34 subjects received VM202 and 19 subjects received placebo). At 6, 9, and 12 months, the pain reduction differences vs. placebo were –1.34, –1.24, and –1.48. The p-values were 0.031, 0.050 and 0.016, respectively.

(4) Regeneration potential: The last follow-up visit of this study occurred at 12 months (Day 365) from the first injection of VM202 and 8.7 months, or 261 days, from the last injection of VM202, which occurred at Day 104. Bioanalytical data shows that 99.9999% of VM202 DNA disappears from the systemic blood circulation at 3 days after injecting VM202 into the calf muscle, and the human hepatocyte growth factor (HGF) gene is expressed for merely 2 weeks after injection. Therefore, the fact that the VM202 group sustains pain reductions for more than 8 months in the absence of DNA and the protein expressed from the gene strongly suggests that VM202 may have nerve regeneration properties.

The results will be presented by Dr. Sunyoung Kim, CEO of Helixmith at the 2019 Fall International Convention of The Pharmaceutical Society of Korea (10/15, 3 PM Yeosu Expo Convention Center).

"It is a pleasant surprise that DPN 3-1b shows statistically significant efficacy with a relatively small sample size. This indicates that we can potentially design our future phase 3 trials with only 100-150 subjects and still be powered to show significance," said Dr. William Schmidt, Head of Clinical Development of Helixmith.

Analysis of the DPN 3-1 trial continues as Helixmith is reviewing patient-level data anomalies and clinical trial operations reported on September 23. The company is advancing clinical development plans for VM202 and furthering planning for the development of other plasmid-based DNA therapies.

"We are very glad to see the data that continues to confirm the promise of VM202. The 12 months of safety and efficacy data will be particularly important to our regulatory package. We will work on designing our next phase 3 and include a robust management plan to confirm the good data we see today," said Dr. Sunyoung Kim, CEO of Helixmith.

About VM202
VM202 (donaperminogene seltoplasmid) is a first-in-class, proprietary, non-viral, potentially regenerative plasmid DNA gene therapy. VM202 is a novel genomic cDNA hybrid human hepatocyte growth factor (HGF) gene with a novel and proprietary coding sequence (HGF-X7) expressing two isoforms - a configuration that closely mimics HGF productions in humans that is needed for optimal therapeutic benefits. Because there is no change in the coding region of the HGF gene, HGF proteins generated from VM202 are identical to wild-type human HGF proteins.
When introduced into the body, HGF protein is expressed from VM202 and induces the formation of new blood vessels, suppresses levels of select pain mediators, supports regeneration/repair of damaged peripheral nerves, and ameliorates atrophic condition of skeletal muscle.

About Helixmith Co., Ltd.
Helixmith is an R&D focused biopharmaceutical company based in Seoul, Korea, developing new and innovative biopharmaceuticals based upon its proprietary scientific platform technology to meet unmet medical needs. Currently, the company is actively focusing on developing the proprietary plasmid DNA-based drug VM202 at various clinical stages in the U.S., Korea, and China, for cardiovascular and neurological diseases, including but not limited to, PDPN, diabetic foot ulcer (DFU), amyotrophic lateral sclerosis (ALS) and ischemic heart disease (IHD).
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