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Helixmith Selects Worldwide Clinical Trials as ALS Phase 2 CRO

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Helixmith Co., Ltd. has recently selected Worldwide Clinical Trials (Worldwide) as the contract research organization (CRO) for its amyotrophic lateral sclerosis (ALS) phase 2 study to be conducted in the US using its flagship gene therapy, Engensis (VM202).

On August 7th, Helixmith signed a Master Service Agreement (MSA) with Worldwide, a top-performing, full-service, mid-sized CRO renowned for its scientific and medical insight, global operations, and collaborative approach. Following the agreement, Worldwide will manage the clinical operations activities of the ALS phase 2 study.

Worldwide is a global company headquartered in the United States with networks in more than 60 countries. The company specializes in therapeutic areas where there remain unmet medical needs, with a focus on central nervous system, cardiovascular, and metabolic disorders; general medicine; oncology; and rare diseases. With 30+ years of experience supporting central nervous system research, Worldwide is an internationally acclaimed expert in clinical trial management for studies in ALS and other diseases affecting the central nervous system.

Also called Lou Gehrig's disease, ALS is a fatal neuromuscular disease characterized by the progressive deterioration of motor neurons. Death usually occurs within 2 to 5 years of symptom onset, most commonly due to respiratory failure following the loss of the ability to carry out all voluntary movements. The incidence of the disease is 1 per 20,000 people, and about 30,000 Americans are estimated to have ALS. The specific causes of the disease are yet unknown, however, and no curative treatments have yet been approved.

Engensis is a gene therapy that uses plasmid DNA to express hepatocyte growth factor (HGF). Data from 15 years of research and clinical trials have shown that simple intramuscular injections of Engensis can produce HGF proteins in the body, which could potentially be effective in nerve regeneration, angiogenesis, and muscle atrophy prevention. Engensis was granted Orphan Drug and Fast Track designation by the FDA in 2016 for ALS, recognized for its outcomes in scientific and clinical research in the disease.

"Helixmith made the decision to conduct the upcoming trial with Worldwide because of their expertise in ALS clinical studies," co mmented Dr. Sunyoung Kim, CEO of Helixmith. "In our Phase 1 trial, patients' disease progression showed trends toward improv ement for 2 to 3 months after Engensis injection. We hope to replicate these results in our upcoming Phase 2. Slowing disease pr ogression could be of great help to ALS patients."

Dr. Michael F. Murphy, Chief Medical and Scientific Officer for Worldwide echoed the sentiments. "The collaborative engagement demonstrated between the subject matter experts and operational staff of Helixmith and Worldwide has been exceptional. Both t he trial design and operational concepts which have been developed will assure an efficient and informative clinical evaluation o f this unique therapeutic approach in a neuromuscular disorder with exceptional unmet clinical need."

[About Helixmith]
Helixmith is a gene therapy company headquartered in Seoul, Korea, that is developing new and innovative biopharmaceuticals to tackle previously untreated diseases and is listed on KOSDAQ. The company has an extensive gene therapy pipeline, including a CAR-T program targeting several different types of solid tumors and an AAV vector program targeting neuromuscular diseases. Engensis (VM202), the most advanced pipeline candidate, is a plasmid DNA therapy being studied for diabetic peripheral neuropa thy, diabetic foot ulcers, claudication, amyotrophic lateral sclerosis (Phase 2 beginning in late 2020), coronary artery disease and Charcot-Marie-Tooth disease.

Engensis received the first-ever regenerative medicine advanced therapy (RMAT)designation from the US FDA for a plasmid-DNA-based therapeutic for diabetic peripheral neuropathy.

Helixmith clinical development and manufacturing activities are based in San Diego, California, where the company co-owns a c GMP-ready DNA production facility, Genopis, Inc., an affiliated CDMO also in San Diego. Genopis serves both Helixmith and exter nal customers in need of plasmid DNA for medical purposes.

[About Worldwide Clinical Trials]
Worldwide Clinical Trials employs more than 1,700 professionals around the world, with offices in North and South America, East ern and Western Europe, Russia, and Asia. Founded by physicians committed to advancing medical science, Worldwide is out to c hange how the world experiences CROs—in the best possible way. From early phase and bioanalytical sciences through late phas e, post-approval and real-world evidence, we provide world-class, full-service drug development services.

With infrastructure and talent spanning 60 countries, we execute predictable, successful studies with operational excellence acro ss a range of therapeutic areas, including central nervous system, cardiovascular, metabolic, general medicine, oncology and rar e diseases. We never compromise on science or safety. We're never satisfied with the status quo. We're the Cure for the Common CRO.

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