Press release
11 June 2025

U.S. FDA Orphan Drug Designation Granted to Recombinant Human HGF, KP-100IT, for the Treatment of Acute Spinal Cord Injury

Kringle Pharma, Inc. (Head office located in Osaka, Japan; "KRINGLE"), a late clinical-stage biopharmaceutical company, today announced that the United States (U.S.) Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to KP-100IT, the intrathecal formulation of recombinant human hepatocyte growth factor (HGF), for the treatment of the acute phase of spinal cord injury.

The U.S. ODD is a program that targets rare diseases with fewer than 200,000 patients in the U.S. and aims to support and promote the development of drugs and biological products that are particularly necessary to address unmet medical needs. Upon receiving the ODD, a sponsor company secures seven years of market exclusivity in the U.S. following the FDA approval. The company also receives various incentives to support the development, including tax deductions for clinical costs, exemption from FDA application fees, and expedited FDA review.

Kiichi Adachi, President & CEO of KRINGLE, said, "KP-100IT for acute spinal cord injury was designated as an orphan drug in Japan in September 2019, and we are currently preparing an application for manufacturing and marketing approval in Japan. Receiving the ODD from the FDA emphasizes the importance of new treatment options for acute spinal cord injury. By leveraging various development and marketing advantages associated with ODD, we will be able to accelerate the drug development in the U.S. Needless to say, this will also be a significant advantage in the partnership discussions currently underway with the global pharmaceutical companies. We will further promote global expansion and prepare to start a clinical trial in the U.S. for treating acute spinal cord injury."

About Hepatocyte Growth Factor (HGF)

HGF was originally discovered as an endogenous mitogen for mature hepatocytes. Subsequent studies demonstrated that HGF exerts multiple biological functions based on its mitogenic, motogenic, anti-apoptotic, morphogenic, anti-fibrotic, and angiogenic activities, and facilitates regeneration and protection of a wide variety of organs. HGF exerts neurotrophic effects and enhances neurite outgrowth, and the therapeutic effect of HGF on spinal cord injury has been demonstrated in animal models by Professors Hideyuki Okano and Masaya Nakamura at Keio University School of Medicine. The expectations for HGF as a novel therapeutic agent are increasing for spinal cord injury.

A group led by Professor Shigeru Hirano of the Department of Otolaryngology and Head and Neck Surgery, Kyoto Prefectural University of Medicine, focused on the anti-fibrotic effects of HGF and demonstrated its pharmacological effects on vocal cord scar. HGF is also expected to have the potential to be an effective therapeutic agent for various fibrotic diseases including vocal fold scar.

Olemepermin alfa is a generic pharmaceutical name for recombinant human hepatocyte growth factor (HGF) developed by Kringle Pharma, Inc. Olemepermin alfa is a glycoprotein (molecular weight: approximately 84,000) consisting of 692 amino acid residues and is produced by CHO cells.

About Kringle Pharma, Inc. https://www.kringle-pharma.com/en/

Kringle Pharma is a late clinical-stage biopharmaceutical company established in December 2001 to develop novel biologics based on HGF. Currently, in the late-stage pipeline of oremepermine alfa, the development for acute spinal cord injury has completed Phase 3 clinical trial, and the development for vocal cord scarring is in Phase 3 clinical trial. Kringle's mission is to contribute to societal and global healthcare through the continued research, development, and commercialization of HGF drug for patients suffering from incurable diseases.

For more information, please contact:
Kiichi Adachi
President & CEO, Member of the Board
Kringle Pharma, Inc.
☎ +81-6-7653-6728
✉ kpinfo@kringle-pharma.com