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Knopp Biosciences receives FDA Orphan Drug Designation for Treatment of Hypereosinophilic Syndrome

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Knopp Biosciences LLC, a privately held drug discovery and development company focused on delivering breakthrough treatments for inflammatory and neurological diseases with high unmet needs has announced that the U.S. Food and Drug Administration has granted Orphan Drug Designation to dexpramipexole, its oral drug candidate for the treatment of hypereosinophilic syndrome (HES). HES is a rare and often incapacitating disorder with limited treatment options.

The FDA Orphan Drug Designation program provides a special status to drugs and biologics intended to treat, diagnose, or prevent diseases and disorders that affect fewer than 200,000 people in the U.S. This designation provides for a seven-year marketing exclusivity period against competition, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees.

“We are very pleased to receive orphan drug designation for dexpramipexole for HES,” said Michael Bozik, M.D., CEO of Knopp Biosciences. “There is an urgent need for effective treatments for patients suffering from this under-recognized, debilitating disease, especially for oral medications that selectively deplete eosinophils, and this designation represents an important regulatory milestone as we advance our dexpramipexole program toward late-stage clinical development.”

Knopp is developing dexpramipexole as a targeted, oral therapeutic for eosinophilic inflammatory disorders. Eosinophils are white-blood cells that play a central role in severe diseases such as asthma and HES, and dexpramipexole has been shown to selectively, profoundly, and persistently reduce blood and tissue eosinophil levels in multiple clinical trials. Results of a Phase 2 study of dexpramipexole in HES have been presented to the American Society of Hematology and published in the journal Blood.

Knopp Biosciences, based in Pittsburgh, PA, USA, is a privately held drug discovery and development company focused on delivering breakthrough treatments for inflammatory and neurological diseases with a high unmet need. Knopp’s clinical-stage small molecule, dexpramipexole, is entering Phase 2 clinical trials in eosinophilic asthma and Phase 3 development in hypereosinophilic syndrome. Knopp’s preclinical Kv7 platform is directed to small molecule treatments for neonatal epileptic encephalopathy, other rare epilepsies, tinnitus, and neuropathic pain.