Deciphera Pharmaceuticals Announces Submission of New Drug Application to U.S. FDA for Ripretinib in Patients with Advanced Gastrointestinal Stromal Tumors

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- Application is Being Reviewed Under FDA's Real-Time Oncology Review (RTOR) Pilot Program -

- Positive Results from Company's INVICTUS Pivotal Phase 3 Clinical Study of Ripretinib in Patients with Advanced Gastrointestinal Stromal Tumors (GIST) Form Basis of Submission -

Deciphera Pharmaceuticals, Inc. (NASDAQ:DCPH) today announced the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for ripretinib, the Company's investigational broad-spectrum KIT and PDGFRα inhibitor, for the treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib and regorafenib.

"The NDA submission for ripretinib marks an exciting milestone as we work towards delivering a potential new treatment option for people with advanced GIST," said Steve Hoerter, President and Chief Executive Officer of Deciphera. "We look forward to working with the FDA through their review of our application, and we remain focused on preparing for the potential launch of ripretinib in the United States, if approved."

The NDA is being reviewed by the FDA under the Oncology Center of Excellence Real-Time Oncology Review, or RTOR, pilot program. The pilot program aims to explore a more efficient review process to ensure that safe and effective treatments are available to patients as early as possible, while maintaining and improving review quality. Additional information about RTOR can be found at: https://www.fda.gov/about-fda/oncology-center-excellence/real-time-oncology-review-pilot-program.

In October 2019, FDA granted Breakthrough Therapy Designation (BTD) for ripretinib for the treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib and regorafenib. BTD is designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy.

The NDA submission is supported by positive data from the Company's INVICTUS pivotal Phase 3 study of ripretinib in advanced GIST. INVICTUS is a randomized (2:1), double-blind, placebo-controlled, international, multicenter study designed to evaluate the efficacy and safety of ripretinib compared to placebo in 129 patients with advanced GIST whose previous therapies have included at least imatinib, sunitinib, and regorafenib. As previously reported, the study achieved the primary endpoint of improved progression free survival compared to placebo in patients with fourth-line and fourth-line plus GIST, as determined by blinded independent central radiologic review using modified Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1.

About Gastrointestinal Stromal Tumor

Gastrointestinal stromal tumor (GIST) is a cancer affecting the digestive tract or nearby structures within the abdomen, most often presenting in the stomach or small intestine. GIST is the most common sarcoma of the gastrointestinal tract, with approximately 4,000 to 6,000 new GIST cases each year in the United States and a similar incidence rate in European and other countries. Most cases of GIST are driven by a spectrum of mutations. The most common primary mutations are in KIT kinase, representing approximately 75% to 80% of cases, and PDGFRα kinase, representing approximately 5% to 10% of cases. Current therapies are unable to inhibit the full spectrum of primary and secondary mutations, which drives resistance and disease progression. Estimates for 5-year survival range from 48% to 90%, depending on the stage of the disease at diagnosis.

About Ripretinib

Ripretinib is an investigational tyrosine kinase switch control inhibitor that was engineered to broadly inhibit KIT and PDGFRα mutated kinases by using a unique dual mechanism of action that regulates the kinase switch pocket and activation loop. Ripretinib is currently in clinical development for the treatment of KIT and/or PDGFRα-driven cancers, including gastrointestinal stromal tumors, or GIST, systemic mastocytosis, or SM, and other cancers. Ripretinib inhibits initiating and secondary KIT mutations in exons 9, 11, 13, 14, 17, and 18, involved in GIST, as well as the primary exon 17 D816V mutation involved in SM. Ripretinib also inhibits primary PDGFRα mutations in exons 12, 14 and 18, including the exon 18 D842V mutation, involved in a subset of GIST. Ripretinib has been granted Fast Track Designation and Breakthrough Therapy Designation by the U.S. FDA for the treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib and regorafenib. Ripretinib has also been granted Orphan Drug Designation for the treatment of GIST by the U.S. FDA and European Medicines Agency (EMA). For more information about the Company's clinical trials with ripretinib, please visit www.clinicaltrials.gov.