



Deciphera Pharmaceuticals Announces U.S. Food and Drug Administration Acceptance of New Drug Application and Priority Review for Ripretinib in Patients with Advanced Gastrointestinal Stromal Tumors

February 14, 2020

- FDA Grants Priority Review and sets PDUFA Date of August 13, 2020 -

WALTHAM, Mass.--(BUSINESS WIRE)--Feb. 12, 2020-- [Deciphera Pharmaceuticals, Inc.](#) (NASDAQ:DCPH) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review its New Drug Application (NDA) seeking approval for ripretinib, the Company's investigational broad-spectrum KIT and PDGFR α inhibitor, for the treatment of patients with advanced gastrointestinal stromal tumors (GIST). The FDA granted Priority Review for the NDA, which provides for a six-month review, and assigned a Prescription Drug User Fee Act (PDUFA) target action date of August 13, 2020.

"The FDA's acceptance of our NDA brings us one step closer to our goal of providing patients with advanced GIST a potential new treatment option," said Steve Hoerter, President and Chief Executive Officer of Deciphera. "With commercial preparations already underway, we believe we will be well positioned for a potential U.S. launch in 2020. We look forward to working with the FDA during their review of this application."

Priority Review designation is for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. Under the PDUFA, a Priority Review targets a review time of six months compared to a standard review time of ten months. The FDA previously 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 is being reviewed by the FDA under the Oncology Center of Excellence Real-Time Oncology Review (RTOR) pilot program. This 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 addition, the Company has submitted additional marketing applications in Canada and Australia for ripretinib in advanced GIST. These submissions are part of the FDA's Project Orbis pilot program and both received Priority Review designations. The Project Orbis pilot program, an initiative of the FDA Oncology Center of Excellence, is designed to provide a framework for concurrent submission and review of oncology products among international partners. Additional information about Project Orbis can be found at: <https://www.fda.gov/about-fda/oncology-center-excellence/project-orbis>.

The NDA submission is supported by positive results 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 its 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 Ripretinib

Ripretinib is an investigational tyrosine kinase switch control inhibitor that was engineered to broadly inhibit KIT and PDGFR α 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 PDGFR α -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 PDGFR α 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 FDA for the treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib, and regorafenib. The FDA also granted Priority Review for the NDA for ripretinib, and assigned a PDUFA target action date of August 13, 2020. In addition, ripretinib has been granted Orphan Drug Designation for the treatment of GIST by the FDA and European Medicines Agency (EMA). For more information about the Company's clinical trials with ripretinib, please visit www.clinicaltrials.gov.

Deciphera Pharmaceuticals has an exclusive license agreement with Zai Lab (Shanghai) Co., Ltd. for the development and commercialization of ripretinib in Greater China (Mainland China, Hong Kong, Macau, and Taiwan). Deciphera Pharmaceuticals retains development and commercial rights for ripretinib in the rest of the world.

About Deciphera Pharmaceuticals

Deciphera Pharmaceuticals is a clinical-stage biopharmaceutical company focused on improving the lives of cancer patients by addressing key mechanisms of drug resistance that limit the rate and/or durability of response to existing cancer therapies. Our small molecule product candidates are directed against an important family of enzymes called kinases, known to be directly involved in the growth and spread of many cancers. We use our deep understanding of kinase biology together with a proprietary chemistry library to purposefully design compounds that maintain kinases in a “switched off” or inactivated conformation. These investigational therapies comprise tumor-targeted agents designed to address therapeutic resistance causing mutations and immuno-targeted agents designed to control the activation of immunokinases that suppress critical immune system regulators, and agents designed to inhibit reprogramming of cancer cell metabolism. We have used our platform to develop a diverse pipeline of tumor-targeted, immuno-targeted, and metabolism-targeted product candidates designed to improve outcomes for patients with cancer by improving the quality, rate, and/or durability of their responses to treatment.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding our expectations regarding our goal of bringing ripretinib to patients with advanced GIST, the potential for ripretinib to serve as a new treatment option for people with advanced GIST, commercial preparations and our readiness for a potential U.S. launch in 2020, working with the FDA through its review of our NDA application via the FDA's Real-Time Oncology Review pilot program, working with the FDA, Health Canada and the Therapeutic Goods Administration on our Canadian and Australian regulatory approval filings under the Project Orbis pilot program, and the possible benefits of those pilot programs and breakthrough therapy designation, and receipt of priority review and the possible benefits of that designation. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned clinical studies or the development of our product candidates, including ripretinib, our ability to successfully demonstrate the efficacy and safety of our product candidates including in later-stage studies, the preclinical and clinical results for our product candidates, which may not support further development of such product candidates, the possibility that results experienced in early, preliminary, top-line or initial data may not be indicative of the results experienced in final data, our ability to work with the FDA under its RTOR pilot program and our ability to work with the FDA, Health Canada and the TGA under the Project Orbis pilot program and timely respond to information requests or requirements in connection with our recently-filed NDAs and marketing approval applications in Canada and Australia for ripretinib in advanced GIST, that acceptance into the RTOR and Project Orbis pilot programs does not guarantee or influence approvability of our NDAs for ripretinib in advanced GIST, which are subject to the standard benefit-risk evaluation by FDA, Health Canada and the TGA, and that we may not derive any benefit from inclusion in the RTOR or Orbis pilot programs, including, but not limited to, a more efficient review process compared to investigational drugs evaluated without these pilot programs or under FDA, Health Canada or TGA's standard review procedures, the fact that these pilot programs are being tested by FDA, are not formal regulatory pathways with regulatory process, regulations or procedures, and may be suspended or halted at any time, including, without limitation, because FDA decides not to continue these pilots, or because FDA determines that our application no longer meets its criteria for inclusion in one or both of these pilot programs, the fact that receipt of a breakthrough therapy designation for a product candidate, such as ripretinib, may not result in us receiving any of the benefits of such designation such as a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures, the fact that such designation does not assure ultimate approval by FDA and is subject to the risk that FDA may later decide that the products no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened, the fact that the priority reviews we have received may not result in any more efficient review or other benefits, including that such designation does not alter the scientific/medical standard for approval or the quality of evidence necessary, our ability to manage and our reliance on sole-source third parties such as our third party drug substance and drug product contract manufacturers, actions of regulatory agencies, our ability to plan for potential commercialization of our product candidates, such as ripretinib, and if approved execute on our marketing plans, the inherent uncertainty in estimates of patient populations and incidence and prevalence estimates, competition from other products, our ability to obtain and maintain reimbursement for any approved product and the extent to which patient assistance programs are utilized, our ability to comply with healthcare regulations and laws, our ability to obtain, maintain and enforce our intellectual property rights, any or all of which may affect the initiation, timing and progress of clinical studies and the timing of and our ability to obtain regulatory approval, if at all, and make our investigational drugs, including ripretinib, available to patients, and, once commercial, to derive revenue from product sales, and other risks identified in our SEC filings, including our Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, and subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

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