Deciphera Pharmaceuticals Presents Updated Data from Ripretinib and DCC-3014 Programs at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics

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- Median Progression Free Survival (mPFS) of 46 Weeks for the Second-Line Gastrointestinal Stromal Tumors (GIST) Cohort from the Phase 1 Study of Ripretinib in Patients Receiving 150 mg QD as Starting Dose -

- DCC-3014 Phase 1 Data Demonstrated Tolerability, Pharmacokinetics and Biomarker Mechanistic Proof-of-Concept in Patients with Advanced Malignancies -

WALTHAM, Mass.--(BUSINESS WIRE)--Oct. 29, 2019-- Deciphera Pharmaceuticals, Inc. (Nasdaq:DCPH), a clinical-stage biopharmaceutical company addressing key mechanisms of tumor drug resistance, today announced the presentation of updated results from its ongoing Phase 1 study of ripretinib, a broad-spectrum KIT and PDGFRα inhibitor, in patients with second-line through fourth-line plus GIST, as well as its Phase 1 study of DCC-3014, an oral inhibitor of CSF1R, in patients with advanced solid tumors. The data are being presented today at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Boston.

“These updated results continue to underscore the potential of our diverse pipeline of product candidates, all generated using our proprietary kinase switch control inhibitor platform, to improve the lives of cancer patients,” said Matthew L. Sherman, M.D., Executive Vice President and Chief Medical Officer of Deciphera. “Of note, we believe ripretinib continues to demonstrate strong clinical benefit in post-imatinib GIST patients, particularly in the second-line setting. These results bolster our confidence in the ongoing INTRIGUE pivotal Phase 3 clinical study, which is designed to support potential regulatory approvals in patients with second-line GIST.”

Ripretinib

Updated results from the Company’s ongoing Phase 1 study of ripretinib in patients with second-line through fourth-line plus GIST included data from 142 GIST patients receiving 150 mg of ripretinib once daily (QD) as the starting dose, which is the dose being utilized in the Company’s INVICTUS and INTRIGUE registration-enabling studies, as of an August 10, 2019 data cutoff date. The table below includes local, investigator-assessed objective response rate (ORR) by best response as determined by Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1, median duration of response, median progression free survival (mPFS) and mean treatment duration.

Line of Therapy	2nd Line (n=31)	3rd Line (n=28)	≥4th Line (n=83)
ORR (confirmed responses only)	19%	14%	7%
Median Duration of Response	80 weeks	NE(1)	76 weeks
mPFS	46 weeks	36 weeks	24 weeks
Mean Treatment Duration(2)	56 weeks	58 weeks	45 weeks

(1) NE = not estimable; (2) Includes 64 patients who elected for intra-patient dose escalation from 150 mg QD to 150 mg twice daily (BID).
Data from GIST patients receiving ≥ 100 mg of ripretinib daily (n=178) in the ongoing Phase 1 study, as of an August 10, 2019 cutoff date, including 2nd line (n=37), 3rd line (n=31), and ≥4th line (n=110) patients were (a) ORR (confirmed responses only): 2nd line (22%), 3rd line (13%), ≥4th line (7%); (b) median duration of response: 2nd line (80 weeks), 3rd line (NE), ≥4th line (48 weeks); (c) mPFS: 2nd line (46 weeks), 3rd line (40 weeks), ≥4th line (24 weeks); (d) mean treatment duration (includes 72 patients who elected for intra-patient dose escalation to 150 mg BID): 2nd line (53 weeks), 3rd line (54 weeks), ≥4th line (48 weeks).

Ripretinib was generally well tolerated and the updated adverse events were consistent with previously presented Phase 1 data in patients with GIST. Grade 3 or 4 treatment-emergent adverse events (TEAEs) in >5% of patients were increase in lipase level (n=25; 18%), anemia (n=11; 8%), and abdominal pain (n=11; 8%).

DCC-3014

The Company’s Phase 1 study of DCC-3014 was designed to evaluate the safety, pharmacokinetics and pharmacodynamics of multiple doses of DCC-3014 in patients with advanced solid tumors. The Company expects to present preliminary data from initial tenosynovial giant cell tumor (TGCT) patients at the 2019 Connective Tissue Oncology Society (CTOS) Annual Meeting being held November 13-16 in Tokyo, Japan.

- As of the data cut-off date of September 10, 2019, increasing doses of DCC-3014 were assessed in seven dose cohorts across 36 patients with advanced solid tumor tumors. This included one dose cohort that received 10 mg once daily and six dose cohorts that received a three to five day loading dose regimen at doses of up to 50 mg followed by a schedule of daily, once-weekly or twice-weekly maintenance dosing with DCC-3014.

- Data demonstrated dose-proportional exposure for DCC-3014 and exposure to DCC-3014 was associated with an increase in plasma CSF1 and IL-34, rapid and sustained reduction of CD16+ monocytes in peripheral blood, and substantial decreases in CD163+ macrophages in tumor.
- DCC-3014 was generally well-tolerated, with most treatment-emergent adverse events (TEAEs) Grade 1 or 2. Most common related TEAEs ≥10% were fatigue (n=6;17%), diarrhea (n=4; 11%), and nausea (n=4; 11%). Grade 3 or 4 related TEAEs occurred in 4 patients, which were grade 3 aspartate aminotransferase (AST) increase, grade 4 lipase increase, grade 3 amylase increase, and grade 3 colitis. Serious adverse events were reported in 17 patients; none of which were related to DCC-3014.
- The dose escalation evaluation is ongoing to determine a recommended phase 2 dose for advanced solid tumors and diffuse-type TGCT.

A copy of each presentation is available at www.deciphera.com/science/presentation-publications/.

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 D816V exon 17 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. In June 2019, the U.S. FDA granted Fast Track Designation to ripretinib for the treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib and regorafenib. 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 DCC-3014

DCC-3014 is an investigational, orally administered, potent and highly selective inhibitor of CSF1R. DCC-3014 was designed using the Company's proprietary switch control kinase inhibitor platform to selectively bind to the CSF1R switch pocket. DCC-3014 has greater than 100-fold selectivity for CSF1R over other closely related kinases and has an even greater selectivity for CSF1R over approximately 300 other human kinases. CSF1R controls the differentiation and function of macrophages including Tumor Associated Macrophages (TAMs) whose density within certain tumors including cancers of the breast, cervix, pancreas, bladder and brain correlates with poor prognosis. Tumors induce TAMs to suppress a natural immune response mediated by cytotoxic T-cells, a type of lymphocyte that would otherwise eradicate the tumor; a process known as macrophage checkpoints. Through inhibition of CSF1R, DCC-3014 has in preclinical studies demonstrated potent macrophage checkpoint inhibition as both a single agent and in combination with PD1 inhibitors. DCC-3014 is currently being evaluated in a Phase 1 clinical study. For more information about the clinical trial design please visit www.clinicaltrials.gov (NCT03069469).

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 drug 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 drug 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 updated Phase 1 study of ripretinib in patients with GIST to support our pivotal Phase 3 INTRIGUE study in second-line GIST patients, the potential of our pipeline drug candidates to improve the lives of patients with cancer, and the expectation to present additional data from our Phase 1 study of DCC-3014 in patients with diffuse-type tenosynovial giant cell tumor at an upcoming medical meeting. 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 drug candidates, including ripretinib, our ability to successfully demonstrate the efficacy and safety of our drug candidates including in later-stage studies, the preclinical and clinical results for our drug candidates, which may not support further development of such drug candidates, our ability to timely complete and prepare the information required for and file an NDA for ripretinib, our ability to manage and our reliance on third parties such as our third party drug substance and drug product contract manufacturers, actions of regulatory agencies, 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 available to patients, and other risks identified in our SEC filings, including our Quarterly Report on Form 10-Q for the quarter ended June 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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