Deciphera Pharmaceuticals Announces Positive, Preliminary, Top-Line Clinical Data for the Ongoing Phase 1 Clinical Study with DCC-3014 and an Update on Future Development Plans

January 2, 2019

- DCC-3014, A Highly-Selective and Potent Small Molecule Inhibitor of CSF1R, Demonstrates Tolerability, Pharmacokinetics and Biomarker Mechanistic Proof-of-Concept (mPoC) in Patients with Advanced Malignancies -

- Deciphera Pharmaceuticals Plans Expansion of Ongoing Phase 1 Study to Include Patients with Tenosynovial Giant Cell Tumors -

WALTHAM, Mass.--(BUSINESS WIRE)--Jan. 2, 2019-- Deciphera Pharmaceuticals, Inc. (NASDAQ:DCPH), a clinical-stage biopharmaceutical company focused on addressing key mechanisms of tumor drug resistance, today announced positive, preliminary, top-line data from the ongoing dose escalation part of the Phase 1 clinical study with DCC-3014, the Company's investigational small molecule switch control inhibitor of CSF1R, in patients with advanced malignancies. In addition, the Company announced a plan to expand the Phase 1 study to evaluate DCC-3014 in patients diagnosed with Tenosynovial Giant Cell Tumors (TGCT). A review of further data from this Phase 1 study will be presented at a medical meeting in 2019.

The Phase 1 study was designed to evaluate the safety, pharmacokinetics and pharmacodynamics of multiple doses of DCC-3014 in up to 55 patients.

- As of the data cut-off date of November 9, 2018, increasing doses of DCC-3014 were assessed in five dose cohorts across 24 patients with advanced solid tumor malignancies. This included one dose cohort that received 10 mg once daily (QD) and four dose cohorts that followed a schedule of once or twice-weekly maintenance dosing preceded by a five-day loading dose regimen at doses of up to 30 mg per dose. In addition, four patients are currently enrolled in a dose cohort that will receive 40 mg twice weekly preceded by a five-day loading regimen.
- Preliminary pharmacokinetic (PK) analysis showed dose-proportional exposure for DCC-3014 that we believe supports twice-weekly maintenance dosing preceded by a five-day loading dose regimen.
- Biomarker data demonstrated strong target engagement of CSF1R, including material reductions in CSF1R positive macrophages in the blood that we believe constitutes mechanistic proof-of-concept (mPoC) for DCC-3014.
- DCC-3014 was generally well tolerated in patients enrolled in the dose cohorts that received twice-weekly maintenance doses of DCC-3014 preceded by a five-day loading regimen at doses of up to 30 mg, which is summarized below:
    - Treatment emergent adverse events (TEAEs) were mostly Grade 1 or 2;
    - No Grade 3 or 4 DCC-3014 related TEAEs in ≥10% of patients;
    - No dose-limiting toxicities; and
    - A maximum tolerated dose has yet to be established.

- In the dose-cohort that received 10 mg QD, clinically asymptomatic laboratory values were recorded as dose-limiting toxicities in two of seven patients.

"We are very pleased with the progress made to date in the Phase 1 clinical study with DCC-3014, our selective, and potent small molecule inhibitor of CSF1R," said Michael D. Taylor, Ph.D., President and Chief Executive Officer of Deciphera. "Based on the preliminary tolerability, PK, and mechanistic proof-of-concept data observed to date, we believe DCC-3014 is well suited for further development. While we will continue to enroll further patients in this Phase 1 study, we are planning to expand this study to enroll patients with tenosynovial giant cell tumors and to explore combinations with other therapies in the first half of 2019."

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 the closely related kinases FLT3, KIT, PDGFRα, PDGFRß and VEGFR2 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 and other T-cell checkpoint 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 Tenosynovial Giant Cell Tumors (TGCTs)

Tenosynovial giant cell tumors (TGCTs) are a group of benign tumors that involve the synovium, bursae and/or tendon sheath. Although benign, these tumors can grow and cause damage to surrounding tissues and structures inducing pain, swelling, and limitation of movement of the joint. Surgery is the main treatment option; however, these tumors tend to recur, particularly in pigmented villonodular synovitis, a diffuse-type of TGCT. If untreated or if the tumor continually recurs damage and degeneration may occur in the affected joint and surrounding tissues, which may cause significant disability. A genetic mutation in certain cells within the tumor causes overproduction of CSF-1, the ligand for the CSF1R receptor, which attracts macrophages and certain other cells that become the bulk of these tumors and cause the associated inflammatory changes.

About Deciphera Pharmaceuticals

Deciphera Pharmaceuticals is a clinical-stage biopharmaceutical company focused on improving the lives of cancer patients by tackling 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, such as macrophages. We have used our platform to develop a diverse pipeline of tumor-targeted and immuno-targeted drug candidates designed to improve outcomes for patients with cancer by improving the quality, rate and/or durability of their responses to treatment.

Availability of Other Information About Deciphera Pharmaceuticals

Investors and others should note that Deciphera Pharmaceuticals communicates with its investors and the public using its company website (www.deciphera.com), including but not limited to investor presentations and scientific presentations, Securities and Exchange Commission filings, press releases, public conference calls and webcasts. The information that Deciphera Pharmaceuticals posts on these channels and websites could be deemed to be material information. As a result, Deciphera Pharmaceuticals encourages investors, the media and others interested in Deciphera Pharmaceuticals to review the information that it posts on these channels, including Deciphera Pharmaceuticals' investor relations website, on a regular basis. This list of channels may be updated from time to time on Deciphera Pharmaceuticals' investor relations website and may include other social media channels than the ones described above. The contents of Deciphera Pharmaceuticals' website or these channels, or any other website that may be accessed from its website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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 clinical trials with our investigational agent DCC-3014, including, without limitation, the potential for DCC-3014 as an effective and well tolerated therapy, the plan to present data at a medical meeting in 2019, the timing of and plan for expansion of the Phase 1 clinical study with DCC-3014 to include patients with TGCT and the timing of and plan to explore combinations with other therapies. 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 DCC-2618, rebastinib, and DCC-3014, our advancement of multiple early-stage and later-stage efforts, 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 efforts to scale up drug product manufacturing, our ability to implement commercial readiness, actions of regulatory agencies, any or all of which may affect the initiation, timing and progress of clinical studies and other risks identified in our SEC filings, including our Quarterly Report on Form 10-Q for the quarter ended September 30, 2018, 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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Source: Deciphera Pharmaceuticals, Inc.

Media:
Gina Nugent, The Yates Network
gina@theyatesnetwork.com
617-460-3579

Investor Relations:
Laura Perry or Sam Martin, Argot Partners
Laura@argotpartners.com or Sam@argotpartners.com
212-600-1902

Company:
Christopher J. Morl, Chief Business Officer
Deciphera Pharmaceuticals, LLC
cmorl@deciphera.com

781-209-6418