Deciphera Presents Updated Preliminary Data from DCC-3014, its CSF1R Inhibitor Program, in Tenosynovial Giant Cell Tumor Patients at the Connective Tissue Oncology Society (CTOS) 2020 Virtual Annual Meeting

November 11, 2020

– Preliminary Results Showed 41% (9 of 22 Patients) Objective Response Rate, Including One Confirmed Complete Response –

– Treatment was Generally Well-Tolerated with Treatment-Emergent Adverse Events Mostly Grade 1 or 2 –

– Based on Preliminary Results, the Expansion Cohorts for DCC-3014 in TGCT Patients Opened at the Recommended Phase 2 Dose of 30 mg Twice Weekly –

WALTHAM, Mass.--(BUSINESS WIRE)--Nov. 11, 2020-- Deciphera Pharmaceuticals, Inc. (NASDAQ:DCPH), today announced the presentation of encouraging preliminary results from the ongoing Phase 1/2 study of DCC-3014, a highly selective, oral, investigational switch-control kinase inhibitor of CSF1R, in patients with tenosynovial giant cell tumor (TGCT). The presentation, titled “Phase 1 Dose-Escalation Study of the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of DCC-3014 in Advanced Solid Tumors and Tenosynovial Giant Cell Tumor (TGCT)”, will be presented at the CTOS 2020 Virtual Annual Meeting, being held November 18-21, 2020. Posters and presentations are available to meeting participants as of November 11, 2020.

“We are very encouraged by the updated data in additional TGCT patients from the ongoing Phase 1/2 study of DCC-3014,” said Matthew L. Sherman, MD, Executive Vice President and Chief Medical Officer of Deciphera. “The preliminary results presented in TGCT patients at the CTOS 2020 Virtual Annual Meeting demonstrated highly encouraging evidence of anti-tumor activity, and DCC-3014 was shown to be generally well-tolerated. These results further support the evaluation of DCC-3014 in patients with TGCT and bolster our confidence in its potential to make a meaningful impact in this disease, which is typically associated with progressively reduced mobility and function. We have selected the recommended Phase 2 dose and initiated the TGCT expansion cohorts to further evaluate the safety and efficacy of DCC-3014.”

“TGCT is a rare and debilitating disease that is most often treated with surgery,” said William D. Tap, MD, Chief of the Sarcoma Medical Oncology Service at Memorial Sloan Kettering Cancer Center in New York. “Despite the best surgical intervention, the disease may advance to the point where surgery is no longer an option, and so there remains a need for effective, well-tolerated systemic therapies to help these patients. These preliminary results demonstrate that DCC-3014 has the potential to be a safe and effective treatment option for TGCT patients.”

The Company’s international, multicenter, open-label Phase 1/2 study of DCC-3014 was designed to evaluate the safety, efficacy, pharmacokinetics, and pharmacodynamics of DCC-3014 in patients with malignant solid tumors and TGCT. Data presented at the CTOS 2020 Virtual Annual Meeting are from 25 TGCT patients enrolled in the dose-escalation portion of the Phase 1/2 study. The cutoff date for the safety data was September 23, 2020, and the cutoff date for the efficacy data was October 5, 2020.

Preliminary results from DCC-3014 in TGCT patients:

Dose Cohorts and Demographics

- 25 TGCT patients enrolled in the study in three dose cohorts:
  - Cohort 5 (n=7): 30 mg loading dose daily for five days followed by a maintenance dose of 30 mg twice a week;
  - Cohort 8 (n=12): 30 mg loading dose daily for three days followed by a maintenance dose of 10 mg daily; and
  - Cohort 9 (n=6): 20 mg loading dose daily for three days followed by a maintenance dose of 6 mg daily.
- Seven patients (28%) had at least one prior surgery and four patients (16%) had received at least one prior systemic therapy.

Preliminary Efficacy and Duration of Treatment

- 22 patients were evaluable for efficacy by RECIST v1.1 at the data cutoff with central assessment available for 21 of the 22 patients; three patients had not yet reached first efficacy assessment.
- Objective response rate of 41%.
  - Nine of 22 patients achieved an objective response, including one complete response.
  - Of the nine responses, three were confirmed and six are awaiting confirmation.
- 78% of responders (7 of 9 patients) had a partial response at their first restaging scan at Cycle 3 Day 1 (week 9).
- 22 of 25 patients were receiving treatment with DCC-3014 at the data cutoff. Two patients withdrew from the study and one patient discontinued due to an adverse event.
- Two TGCT patients were on treatment for 12 or more months with responses that deepened over time.

Safety and Tolerability

- Treatment with DCC-3014 was generally well-tolerated in patients with TGCT not amenable to surgery. One patient (4%) discontinued treatment due to an adverse event (asymptomatic Grade 3 AST elevation, from Grade 1 at baseline).
- Treatment-emergent adverse events (TEAEs) (mostly grade 1/2) occurring in ≥25% of patients with TGCT regardless of relatedness were blood creatine phosphokinase (CPK) increased (52%), aspartate amino transferase (AST) increased (44%), periorbital edema (44%), fatigue (40%), lipase increased (32%), and alanine aminotransferase (ALT) increased (28%). No serious adverse events related to DCC-3014 were reported.
- All bilirubin levels were within the normal limit.
- Observed transaminase and pancreatic enzyme elevations were asymptomatic and not clinically significant.

Pharmacokinetics, Pharmacodynamics and Recommended Phase 2 Dose

- Similar steady state pharmacokinetic profiles were observed between the 30 mg twice weekly (cohort 5) and 10 mg daily (cohort 8) dosing regimens; lower exposure was observed in the 6 mg daily (cohort 9) dosing regimen.
- Across all cohorts, DCC-3014 treatment resulted in an increase in plasma CSF1/IL-34 and a decrease in non-classical sub-type of monocytes, indicating pharmacodynamic inhibition of CSF1R.
- The recommended Phase 2 dose for DCC-3014 in TGCT patients was determined to be 30 mg twice weekly (no loading dose).

Based on these encouraging results, the Phase 1/2 study of DCC-3014 is ongoing and enrolling up to 60 patients into two expansion cohorts, one for TGCT patients with no prior exposure to anti-CSF1/CSF1R agents (n=40) and a second for patients with prior exposure to anti-CSF1/CSF1R agents (n=20). In addition, enrollment of an additional six patients in cohort 9 of the dose escalation portion of the study is ongoing to complete enrollment in this cohort.

About Tenosynovial Giant Cell Tumor (TGCT)
TGCT is a rare disease caused by a translocation in colony-stimulating factor 1 (CSF1) gene resulting in overexpression of CSF1 and recruitment of colony-stimulating factor 1 receptor (CSF1R)-positive inflammatory cells into the lesion. TGCT is also known as giant cell tumor of the tendon sheath (GCT-TS) or pigmented villonodular synovitis (PVNS), a diffuse-type of TGCT. 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 diffuse-type 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.

About DCC-3014
DCC-3014 is an investigational, orally administered, potent and highly selective switch-control kinase inhibitor of CSF1R. DCC-3014 was designed using the Company's proprietary discovery platform to selectively bind to the CSF1R switch pocket. Through inhibition of CSF1R, DCC-3014 has demonstrated encouraging preliminary efficacy and safety data in tenosynovial giant cell tumor (TGCT) and is currently being evaluated in a Phase 1/2 clinical study. For more information about the clinical trial design, please visit www.clinicaltrials.gov (NCT03069469).

About Deciphera Pharmaceuticals
Deciphera is a biopharmaceutical company focused on discovering, developing and commercializing important new medicines to improve the lives of people with cancer. We are leveraging our proprietary switch-control kinase inhibitor platform and deep expertise in kinase biology to develop a broad portfolio of innovative medicines. In addition to advancing multiple product candidates from our platform in clinical studies, QINLOCK® is Deciphera's FDA-approved switch-control kinase inhibitor for the treatment of fourth-line gastrointestinal stromal tumor (GIST). QINLOCK is also approved for fourth-line GIST in Canada and Australia. For more information, visit www.deciphera.com and follow us on LinkedIn and Twitter (@Deciphera).

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, our expectations regarding our DCC-3014 drug candidate, the expansion cohorts in our phase 1/2 study of DCC-3014 in TGCT patients and the potential of DCC-3014 to be a safe and effective treatment option for TGCT patients. 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 severity and duration of the impact of COVID-19 on our business and operations, our ability to successfully demonstrate the efficacy and safety of our drug candidates and in additional indications for our existing drug, the preclinical or clinical results for our product candidates, which may not support further development of such product candidates, our ability to manage 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 commercialize QINLOCK and execute on our marketing plans for any drugs or indications that may be approved in the future, the inherent uncertainty in estimates of patient populations, 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 additional regulatory approvals, and other risks identified in our Securities and Exchange Commission (SEC) filings, including our Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, 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 our views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

View source version on businesswire.com: https://www.businesswire.com/news/home/20201111005254/en/

Investor Relations:
Jen Robinson
Deciphera Pharmaceuticals, Inc.
jrobinson@deciphera.com
781-906-1112

Media:
David Rosen
Argot Partners
David.Rosen@argotpartners.com
212-600-1902

Source: Deciphera Pharmaceuticals, Inc.