Deciphera Pharmaceuticals, Inc. Presents Updated Phase 1/2 Data for Vimseltinib in TGCT at the European Society for Medical Oncology (ESMO) Congress 2022

September 11, 2022

– Updated Results for Vimseltinib Showed Objective Response Rate of 69% in Phase 1, 53% in Phase 2 Cohort A, and 46% in Phase 2 Cohort B; Demonstrated a Clinical Benefit Rate of 100% Across All Phase 1/2 Patients –

– Preliminary Patient-Reported Outcome Data in Phase 2 Demonstrate Clinically Meaningful Improvements in Pain and Stiffness –

– Updated Safety and Efficacy Data Support Ongoing Phase 3 MOTION Study –

– Company to Host Virtual Investor Event Sunday, September 11 at 7:30 AM ET/ 1:30 PM CEST –

WALTHAM, Mass.--(BUSINESS WIRE)--Sep. 11, 2022-- Deciphera Pharmaceuticals, Inc. (NASDAQ: DCPH), a biopharmaceutical company focused on discovering, developing, and commercializing important new medicines to improve the lives of people with cancer, today announced updated results from the ongoing Phase 1/2 study of vimseltinib, an orally administered, potent, and highly selective switch-control kinase inhibitor of CSF1R, for the treatment of patients with tenosynovial giant cell tumor (TGCT) not amenable to surgery. Results from the Phase 1 and Phase 2 portions of the study are being presented as separate posters, titled “Efficacy and safety of vimseltinib in tenosynovial giant cell tumour (TGCT): Phase 2 expansion” and “Safety and efficacy of vimseltinib in tenosynovial giant cell tumour (TGCT): Long-term phase 1 update” at the ESMO Congress 2022 on September 11 and September 12, respectively.

“The updated data presented at ESMO underscore the best-in-class potential of vimseltinib for patients with TGCT. Additionally, preliminary patient-reported outcome results found a clinically meaningful symptomatic benefit at week 25 compared with baseline for both pain and stiffness, highlighting the important impact that vimseltinib can have on a patient’s quality of life,” said Matthew L. Sherman, M.D., Chief Medical Officer of Deciphera. “These results support vimseltinib’s evaluation in the Phase 3 MOTION study, a two-part, randomized, double-blind, placebo-controlled study, which is currently enrolling patients. We believe vimseltinib has the potential to become a best-in-class therapy for TGCT patients who are not amenable to surgery.”

Jean-Yves Blay, M.D., Ph.D., General Director of the Centre Léon Bérard Lyon said, “There remains a substantial unmet medical need for a highly effective and well-tolerated drug for patients with TGCT whose disease is not amenable to surgery. These updated Phase 1/2 data demonstrate not only the strong clinical activity of vimseltinib, but also the favorable safety and tolerability profile that is essential for TGCT patients. Vimseltinib has the potential to address this unmet need and offer a new option for patients around the world.”

Summary of Data and Findings from Phase 1/2 Studies

Results from the Phase 2 portion of the study are being presented today in a poster presentation, summarized below. Updated results from the Phase 1 study are being presented in a poster presentation tomorrow, Monday, September 12. The Phase 1 data summarized below are based on the previously released abstract with a data cutoff date of February 18, 2022. The Phase 1 poster presentation remains under embargo until tomorrow and will include updated data based on a May 6, 2022 data cutoff date.

Safety and Efficacy of Vimseltinib in Tenosynovial Giant Cell Tumour (TGCT): Long-term Phase 1 Update

The Phase 1/2 study of vimseltinib is an open-label, multicenter study evaluating the safety, efficacy, pharmacokinetics, and pharmacodynamics of vimseltinib in patients with solid tumors and TGCT. The data presented from the Phase 1 update include long-term safety and efficacy for patients with TGCT from the dose escalation portion of the study.

Dose Cohorts and Demographics

- As of the February 18, 2022 cutoff date, 32 patients were enrolled in three dose cohorts:
  - Phase 1 Cohort 5 (n=8): 30 mg loading dose daily for five days followed by a maintenance dose of 30 mg twice a week.
  - Phase 1 Cohort 8 (n=12): 30 mg loading dose daily for three days followed by a maintenance dose of 10 mg daily.
  - Phase 1 Cohort 9 (n=12): 20 mg loading dose daily for three days followed by a maintenance dose of 6 mg daily.
- 32 patients were evaluable for efficacy by Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1 at the data cutoff; response data is based on independent radiological review (IRR) except for one patient that did not have an IRR.

Antitumor Activity and Treatment Duration

- Clinical Benefit Rate: 100% of patients demonstrated clinical benefit, defined as patients with complete response, partial response, or stable disease, without disease progression.
- Objective Response Rate: 69% ORR (CR=1, PR=21).
  - Most responses were achieved within six months.
- Treatment Duration: The median duration of treatment was 16.4 months with 59% of patients remaining on treatment as of the data cutoff date in February 2022.

Safety and Tolerability

- Treatment with vimseltinib was generally well-tolerated in patients with TGCT and consistent with previously disclosed data.
- Grade 3 or 4 treatment-emergent adverse events (TEAEs) (>5%) included increases in creatine phosphokinase, aspartate aminotransferase, lipase, amylase, and hypertension.
- Observed transaminase, lipase, amylase, and creatine phosphokinase enzyme elevations were mostly low grade, asymptomatic, and consistent with mechanism of action of CSF1R inhibitors.
- No postbaseline bilirubin elevations observed.
- There were no new treatment-related serious adverse events since the June 7, 2021 data cutoff date.

Safety and Efficacy of Vimseltinib in Tenosynovial Giant Cell Tumour (TGCT): Phase 2 Expansion

The data presented from the Phase 2 expansion portion of the ongoing Phase 1/2 study includes safety, efficacy, and preliminary patient-reported outcome data in patients with TGCT treated with vimseltinib at the recommended Phase 2 dose (RP2D; 30 mg twice weekly) enrolled in two cohorts. Cohort A includes TGCT patients with no prior anti-CSF1/CSF1R (previous therapy with imatinib or nilotinib is allowed) and Cohort B includes patients with prior anti-CSF1/CSF1R (previous therapy with only imatinib or nilotinib are not eligible).

Dose Cohorts and Demographics

- As of the May 6, 2022 cutoff date, 58 TGCT patients treated with vimseltinib were included in the safety population, including 46 patients enrolled in Cohort A and 12 patients enrolled in Cohort B.
- 56 patients were evaluable for efficacy by RECIST version 1.1 at the data cutoff in the Phase 2 across both cohorts; response data is based on independent radiological review.

Antitumor Activity, Treatment Duration, and Preliminary Patient-Reported Outcomes

- Clinical Benefit Rate: 100% of patients demonstrated clinical benefit, defined as patients with complete response, partial response, or stable disease, without disease progression.
- Objective Response Rate: 53% ORR (PR=24) in Cohort A and 46% ORR (CR=1, PR=4) in Cohort B.
  - In Cohort B, responses were observed in patients who had not achieved a response to prior anti-CSF1/CSF1R therapy.
  - Median duration of response was not reached in both cohorts.
  - 75% of responses and 80% of responses were achieved within six months in Cohorts A and B, respectively.
  - ORR at 25 weeks was 38% (PR=17) in Cohort A.
- Treatment Duration: The median duration of treatment was 9.8 months in Cohort A and 5.9 months in Cohort B. As of the data cutoff date, 61% of patients remained on treatment in Cohort A and 67% of patients remained on treatment in Cohort B.
- Preliminary Patient-reported Outcomes: Initial data demonstrate that patients achieved clinically meaningful symptomatic benefit as of week 25 by two measures of patient-reported outcomes.
  - Brief Pain Inventory (BPI): 48% (Cohort A) and 56% (Cohort B) of patients had a BPI worse pain response at week 25.

- Worse Stiffness Numeric Rating Scale: Patients showed progressive improvements in stiffness from baseline to week 25, with mean changes from baseline of -2.0 (Cohort A) and -2.7 (Cohort B). Improvement observed is considered clinically meaningful change with a threshold for meaningful change is estimated to be 1.

Safety and Tolerability

- Treatment with vimseltinib was generally well-tolerated in patients with TGCT at the recommended Phase 2 dose of 30 mg twice weekly.
- Most non-laboratory TEAEs were Grade 2 or lower.
- The only Grade 3/4 TEAE observed in >5% of patients was elevated creatine phosphokinase.

Phase 3 MOTION Study

The pivotal Phase 3 MOTION study of vimseltinib for the treatment of TGCT is ongoing. MOTION is a two-part, randomized, double-blind, placebo-controlled study of vimseltinib to assess the efficacy and safety in patients with TGCT who are not amenable to surgery. The primary endpoint of the study is objective response rate at week 25 as measured by RECIST version 1.1 by blinded independent radiologic review. https://www.clinicaltrials.gov/ct2/show/NCT05059262

Conference Call and Webcast

Deciphera will host a conference call and webcast to discuss data presentations from the Company's DCC-3116 and vimseltinib clinical programs at the ESMO Congress 2022 on Sunday, September 11, 2022, at 7:30 AM ET/ 1:30 PM CEST. The event may be accessed by registering at https://deciphera-pharmaceuticals.open-exchange.net/registration. A webcast of the event will be available in the "Events and Presentations" page in the "Investors" section of the Company's website at https://investors.deciphera.com/events-presentations. The archived webcast will be available on the Company's website within 24 hours after the event and will be available for 30 days following the event.

About Vimseltinib

Vimseltinib is an investigational, orally administered, potent and highly selective switch-control kinase inhibitor of CSF1R. It was discovered using Deciphera's proprietary drug discovery platform and was designed to selectively bind to the CSF1R switch pocket. Vimseltinib has demonstrated encouraging preliminary efficacy and safety data in patients with TGCT and is currently being evaluated in a Phase 1/2 clinical study. The Phase 3 MOTION study, a two-part, randomized, double-blind, placebo-controlled study of vimseltinib to assess the efficacy and safety in patients with symptomatic TGCT who are not amenable to surgery, is currently enrolling.

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 switch control inhibitor for the treatment of fourth-line GIST. QINLOCK is approved in Australia, Canada, China, the European Union, Hong Kong, Switzerland, Taiwan, the United Kingdom, and the United States. 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 and timing regarding the best-in-class potential of vimseltinib in TGCT patients not amenable to surgery, enrollment in the pivotal Phase 3 MOTION study of vimseltinib in TGCT patients, and presenting updated vimseltinib data from our Phase 1/2 study in TGCT patients at ESMO 2022. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "seek," "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 or drug candidates, the preclinical or clinical results for our product candidates, which may not support further development of such product candidates, comments, feedback and 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 and other risks identified in our Securities and Exchange Commission (SEC) filings, including our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 , 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.

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