Deciphera Pharmaceuticals Announces Positive Top-line Results from MOTION Pivotal Phase 3 Study of Vimseltinib in Patients with Tenosynovial Giant Cell Tumor (TGCT) and Updated Results from Phase 1/2 Study of Vimseltinib in TGCT

October 30, 2023

– MOTION Study Met Primary Endpoint with Objective Response Rate (ORR) at Week 25 of 40% Compared to 0% for Placebo (p<0.0001) –

– MOTION Study Met All Key Secondary Endpoints with Statistically Significant and Clinically Meaningful Improvements at Week 25 Compared to Placebo, including ORR by Tumor Volume Score (TVS) of 67% vs. 0% (p< 0.0001) –

– Updated Results from Phase 1/2 Study of Vimseltinib Demonstrated Best ORR of 72% (Phase 1) and 64% (Phase 2 Cohort A) with Median Treatment Duration of 25.1 Months (Phase 1) and 21.0 Months (Phase 2 Cohort A) –

– Treatment with Vimseltinib was Well-tolerated with No Evidence of Cholestatic Hepatotoxicity in MOTION and Phase 1/2 Studies –

– Company Expects to Submit New Drug Application (NDA) in Second Quarter 2024 and Marketing Authorisation Application (MAA) in Third Quarter 2024 –

– Conference Call to be Held Today at 8:00 AM ET –

WALTHAM, Mass.--(BUSINESS WIRE)--Oct. 30, 2023-- 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 positive top-line results from the MOTION pivotal Phase 3 study of vimseltinib in patients with TGCT not amenable to surgery. Vimseltinib is the Company's investigational, orally administered, potent, and highly selective switch-control kinase inhibitor of CSF1R.

"Patients suffering from TGCT are in need of a new treatment option that offers both strong clinical benefit and a well-tolerated safety profile," said Hans Gelderblom, M.D., Ph.D., Chair of the Department of Medical Oncology at Leiden University Medical Center. "TGCT has a significant negative impact on the daily life of patients who face substantial pain, stiffness, and impaired mobility. Success across both the primary and all key secondary endpoints in MOTION underscores vimseltinib's ability to help TGCT patients feel and function better. The top-line results from MOTION, together with the impressive data announced today from the Phase 1/2 study showing that the response rates with vimseltinib continue to increase over time, and that patients continue to receive long-term clinical benefit as evidenced by the median duration of treatment, demonstrates vimseltinib's potential to become a best-in-class agent."

"We are excited about the potential for vimseltinib to become our next approved medicine, supporting our continued evolution to a company with multiple marketed products," said Steve Hoerter, President and Chief Executive Officer of Deciphera Pharmaceuticals. "The totality of data shown today demonstrate the potential for vimseltinib to offer a new and differentiated treatment option for patients with TGCT. On behalf of the entire Deciphera team, I would like to thank the patients, their caregivers, and the healthcare professionals who participated in the MOTION and Phase 1/2 studies. We look forward to working with regulatory agencies worldwide as we focus on delivering this important new treatment option to patients with TGCT."

Top-line Results from the MOTION Pivotal Phase 3 Study of Vimseltinib in TGCT

The MOTION pivotal Phase 3 study is a two-part, randomized, double-blind, placebo-controlled study to assess the efficacy and safety of vimseltinib in patients with TGCT not amenable to surgery with no prior anti-CSF1/CSF1R therapy (prior therapy with imatinib or nilotinib allowed). In Part 1, patients (n=123) were randomized two-to-one to receive either 30 mg twice weekly of vimseltinib (n=83) or placebo (n=40) for 24 weeks. The primary endpoint of the study is ORR at Week 25 as measured by Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1 by blinded independent radiologic review (IRR). The open-label Part 2 portion of MOTION, in which patients from both the vimseltinib and placebo arms receive treatment with vimseltinib, remains ongoing. The results for Part 1 of the study are based on a data cutoff date of August 22, 2023.

The study met its primary endpoint in the intent-to-treat (ITT) population demonstrating statistically significant and clinically meaningful improvement versus placebo in ORR at Week 25 based on IRR per RECIST v1.1. In the ITT population, the ORR at Week 25 was 40% (95% CI: 29%, 51%) for the vimseltinib arm and 0% (95% CI: 0%, 9%) for the placebo arm resulting in a response difference (vimseltinib versus placebo) of 40% (95% CI: 29%, 51%) (p<0.0001).

In addition to meeting the primary endpoint, the study also achieved statistically significant and clinically meaningful improvements versus placebo in all key secondary endpoints assessed at Week 25 including ORR per tumor volume score (TVS), active range of motion (ROM), physical function, stiffness, quality of life, and pain.

In the ITT population, the ORR at Week 25 based on IRR per TVS was 67% (95% CI: 56%, 77%) for the vimseltinib arm and 0% (95% CI: 0%, 9%) for the placebo arm (p<0.0001). Treatment with vimseltinib also demonstrated an improvement in mean change from baseline in active ROM at Week 25 of 18.4% versus a 3.8% improvement for placebo (p=0.0077).

Vimseltinib was well-tolerated and the safety profile in the MOTION study was consistent with previously disclosed data. There was no evidence of cholestatic hepatotoxicity in patients treated with vimseltinib. Patients with treatment-emergent adverse events (TEAEs) leading to treatment discontinuation was 6% in the vimseltinib arm. The table below lists TEAEs ≥15% in either arm during Part 1 of the study.

	Vimseltinib (n=83)		**Placebo (n=39)[1]**	
Preferred Term n (%)	**All Grades**	**Grade 3/4**	**All Grades**	**Grade 3/4**
Periorbital edema^	37 (45%)	3 (4%)	5 (13%)	0
Fatigue ^	27 (33%)	0	6 (15%)	0
Face edema^	26 (31%)	1 (1%)	3 (8%)	0
Pruritus^	24 (29%)	2 (2%)	3 (8%)	0
Headache^	23 (28%)	1 (1%)	10 (26%)	0
Asthenia^	22 (27%)	1 (1%)	9 (23%)	1 (3%)
Nausea ^	21 (25%)	0	8 (21%)	1 (3%)
CPK increased	20 (24%)	8 (10%)	0	0
AST increased	19 (23%)	0	1 (3%)	0
Arthralgia ^	16 (19%)	0	6 (15%)	1 (3%)
Rash ^	16 (19%)	0	2 (5%)	0
Rash maculo-papular^	16 (19%)	1 (1%)	0	0
Edema peripheral ^	15 (18%)	0	3 (8%)	0
Hypertension	14 (17%)	4 (5%)	4 (10%)	1 (3%)
Diarrhea	10 (12%)	0	8 (21%)	1 (3%)

(1) Does not include one patient randomized to placebo that did not receive study drug.

TEAE incidence is based on maximum grades per CTCAE v5.0. The only Grade 4 adverse events were CPK Increased observed in two patients. TEAEs leading to dose interruption were 44 (53%) and dose reduction 35 (42%). ^ Denotes adverse events without Grade 4 criteria per CTCAE v5.0.

The Company expects to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for vimseltinib for the treatment of patients with TGCT in the second quarter of 2024 and a Marketing Authorisation Application (MAA) to the European Medicines Agency in the third quarter of 2024.

Additional efficacy and safety results from Part 1 of the MOTION study are expected to be presented at an upcoming medical meeting.

Updated Results from the Phase 1/2 Study of Vimseltinib in TGCT

The Company also announced today updated results from the Phase 1/2 open-label, multicenter study evaluating the safety and efficacy of vimseltinib in patients with TGCT, which will be presented at the Connective Tissue Oncology Society 2023 Annual Meeting in Dublin, Ireland on November 1-4, 2023.

As of the June 27, 2023 cutoff date, 97 patients were enrolled in the study as follows:

- Phase 1: 32 patients enrolled in three cohorts across multiple doses.
- Phase 2: 65 patients enrolled in two cohorts at the recommended Phase 2 dose of 30 mg twice weekly: Cohort A (n=46) in TGCT patients with no prior anti-CSF1/CSF1R therapy (prior therapy with imatinib or nilotinib allowed); and Cohort B (n=19) in TGCT patients with prior anti-CSF1/CSF1R therapy (prior therapy with imatinib or nilotinib alone not eligible).

Antitumor Activity and Treatment Duration:

93 patients were evaluable for efficacy by RECIST v1.1 at the data cutoff date; response data is based on IRR summarized as follows:

	Phase 1 (n=32)	Phase 2 Cohort A (n=45)	Phase 2 Cohort B (n=16)
Best ORR per RECIST v1.1 by IRR (%)	72%	64% (38% at Week 25)	44%
Median Duration of Response (months) (Range)	NR (3.8+, 45.2+)	NR (0.03+, 25.4+)	NR (4.0+, 21.0+)
Median Treatment Duration (months) (Range)	25.1 (0.7, 46.9)	21.0 (0.2, 30.3)	7.3 (0.7, 27.4)
Patients Active on Treatment at Cutoff Date (%)	47%	48%	74%

NR: Not Reached by Kaplan-Meier analysis.

In addition, updated data from Cohorts A and B of the Phase 2 study demonstrate that patients experienced clinically meaningful symptomatic benefit at Week 25 across multiple secondary efficacy measures including best ORR per TVS (Cohort A), active range of motion, physical function, stiffness, and pain.

Safety and Tolerability

- Treatment with vimseltinib was well-tolerated in patients with TGCT and consistent with previously disclosed data.
- There was no evidence of cholestatic hepatotoxicity.
- Treatment discontinuation due to TEAEs occurred in 9% of patients.

Results from the Phase 1 portion of the study are being presented in an oral presentation, titled “Safety and Efficacy Updates from a Phase 1 Study of Vimseltinib in Patients with Tenosynovial Giant Cell Tumor.” Results from Cohorts A and B of the Phase 2 portion of the study are being presented in two poster presentations, titled “Safety, Efficacy, and Patient-Reported Outcomes with Vimseltinib in Patients with Tenosynovial Giant Cell Tumor Who Received No Prior Anti-Colony-Stimulating Factor 1 Therapy: Ongoing Phase 2 Update” and “Safety, Efficacy, and Patient-Reported Outcomes with Vimseltinib in Patients with Tenosynovial Giant Cell Tumor Who Received Prior Anti-Colony-Stimulating Factor 1 Therapy: Ongoing Phase 2 Update.” The presentations are available on the Company’s website at www.deciphera.com/presentations-publications.

Conference Call and Webcast

Deciphera will host a conference call and webcast to discuss this announcement today, October 30, 2023, at 8:00 AM ET. The conference call may be accessed via this link: https://register.vevent.com/register/BIc2885b197da74145bfc30deb5fb11858. A live webcast of the conference call will be available in the “Events and Presentations” page in the “Investors & News” section of the Company’s website at https://investors.deciphera.com/events-presentations. A replay will be available on the Company’s website approximately two hours after the conference call and will be available for 30 days following the call.

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, Israel, Macau, New Zealand, Singapore, 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 potential for our preclinical and/or clinical stage pipeline assets to be first-in-class and/or best-in-class treatments; the potential for vimseltinib to become our second approved medicine, the potential for vimseltinib to become a new treatment option for patients with TGCT, plans to submit an NDA for vimseltinib in the second quarter of 2024 and an MAA in the third quarter of 2024, and plans to present additional data at upcoming medical congresses. 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, 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, including the FDA and the EMA, 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 and total addressable markets, 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 September 30, 2023, 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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