Curis Announces Positive Preliminary Data from Ongoing Phase 1 Study of CA-4948 Monotherapy in Patients with Relapsed or Refractory Acute Myeloid Leukemia and Myelodysplastic Syndromes

- Reduction of marrow blasts observed in all evaluable patients -
- Marrow complete response observed in 2 patients -
- Management to host virtual KOL event today, Tuesday, December 8 at 8:00 a.m. ET -

LEXINGTON, Mass., Dec. 8, 2020 /PRNewswire/ -- Curis, Inc. (NASDAQ: CRIS), a biotechnology company focused on the development of innovative therapeutics for the treatment of cancer, today announced positive preliminary data from its ongoing open-label, single arm Phase 1 dose escalation study of CA-4948, a novel, small molecule IRAK4 kinase inhibitor, in patients with acute myeloid leukemia (AML) or high-risk myelodysplastic syndromes (MDS). IRAK4 plays an essential role in the toll-like receptor (TLR) and interleukin-1 receptor (IL-1R) signaling pathways, and these pathways are frequently dysregulated in patients with AML and MDS. Third parties have recently discovered that the long form of IRAK4 (IRAK4-L) is oncogenic and preferentially expressed in over half of patients with AML and MDS. A variety of drivers are believed to cause this, including specific spliceosome mutations.

"We are highly encouraged by the breadth of clinical activity with CA-4948 seen with this early data, especially as this study is both monotherapy and in a late line, relapsed/refractory population. Historically, monotherapy studies in AML and MDS have proven underwhelming; monotherapy studies in a relapsed/refractory setting have been especially challenging," said James Dentzer, President and Chief Executive Officer of Curis. "We also have been pleased by the pace at which our trial partners have been able to enroll patients. We look forward to continuing to advance CA-4948 and reporting additional Phase 1 data in the second half of 2021."

"As a clinician intimately familiar with the treatment challenges faced by patients with AML or high-risk MDS, I am very encouraged by the early data coming out of this study," said Dr. Guillermo Garcia-Manero, Chief of the Section of Myelodysplastic Syndromes within the Department of Leukemia at The University of Texas MD Anderson Cancer Center. "While there have been important advancements in the development of new therapeutics for patients with previously untreated AML or MDS in recent years, relapsed and refractory patients are still in need of better treatment options. These preliminary data suggest, for the first time in a clinical setting, that successfully targeting the long isoform of IRAK4, which we know to be preferentially expressed in over half of AML and MDS patients, could be the first major breakthrough in over a decade for patients with these diseases."

The reported preliminary data are from Curis's ongoing open-label, single arm Phase 1 dose escalation 3+3 study of orally administered CA-4948 monotherapy in adult patients with AML or high-risk MDS. A minimum of 3 patients will be enrolled in each cohort of the two-part study, starting with 200 mg BID, which was demonstrated to be well-tolerated, capable of achieving relevant levels of drug exposure and has demonstrated signs of biologic activity in Curis's ongoing Phase 1 study of CA-4948 for the treatment of patients with relapsed or refractory non-Hodgkin's lymphoma. The primary objective of the study is to determine the maximum tolerated dose (MTD) and recommended Phase 2 dose (RP2D) for CA-4948 based on safety and tolerability, dose-limiting toxicities (DLT), and any biologic activity, pharmacokinetic and pharmacodynamic findings from the study trial population. Additional objectives include characterization of CA-4948's pharmacokinetic parameters, and biomarker correlations. As of November 23, 2020, 4 AML patients and 3 high-risk MDS patients had been enrolled in the first 2 study cohorts and no DLTs had been observed. The data being reported from this ongoing trial are preliminary and subject to change.

Key findings include:

- Marrow blast reductions observed in all evaluable patients (6 patients).
- 6 of 7 patients enrolled remain on study.
- Patients enrolled experienced a median of 3 prior lines of treatment (range 1-4).
- Two patients experienced a marrow complete response, one with blast count going from 23% pretreatment to 1% on treatment, and the other going from 11% pretreatment to 2% on treatment.
- No DLTs observed in 7 DLT-evaluable patients in the 200 mg BID and 300 mg BID cohorts.
- Enrollment has begun in the 400 mg BID cohort.

Webcast Event Information

Curis management will host a virtual KOL event today, December 8, 2020 at 8:00 am ET to discuss these results with Dr. Amit Verma, Professor of Medicine-Oncology at Albert Einstein College of Medicine, and Director of the MDS Program at Montefiore Medical Center located in Bronx, NY. To access the webcast, please visit the Events and Presentations section of the Curis website at https://www.curis.com/.

About Curis, Inc.

Curis is a biotechnology company focused on the development of innovative therapeutics for the treatment of cancer. In 2015, Curis entered into a collaboration with Aurigene in the areas of immuno-oncology and precision oncology. As part of this collaboration, Curis has exclusive licenses to oral small molecule antagonists of immune checkpoints including the VISTA/PDL1 antagonist CA-170, and the TIM3/PDL1 antagonist CA-327, as well as the IRAK4 kinase inhibitor, CA-4948. CA-4948 is currently undergoing testing in a Phase 1 trial in patients with non-Hodgkin's lymphoma and in a Phase 1 trial in patients with acute myeloid leukemia and myelodysplastic syndromes. In addition, Curis is engaged in a collaboration with ImmuNext for development of CI-8993, a monoclonal anti-VISTA antibody, which is currently undergoing testing in a Phase 1a/1b trial in patients with solid tumors. Curis is also party to a collaboration with Genentech, a member of the Roche Group, under which Genentech and Roche are commercializing Erivedge® for the treatment of advanced basal cell carcinoma. For more information, visit Curis' website at www.curis.com.

Forward-Looking Statements:

This press release contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, including, without limitation, statements regarding any expectations of the potential for the Company's proprietary drug candidate CA-4948, including with respect to the activity and tolerability of CA-4948, future studies with respect to CA-4948, the potential advantages and benefits of CA-4948 and small molecule checkpoint antagonists, statements with respect to the timing of the Company's studies, including enrollment and reporting of data, and the Company's plans to advance its development programs. Forward-looking statements may contain the words "believes," "expects," "anticipates," "plans," "intends," "seeks," "estimates," "assumes," "will," "may," "could" or similar expressions. These forward-looking statements are not guarantees of future performance and involve risks, uncertainties, assumptions and other important factors that may cause actual results to be materially different from those indicated by such forward-looking statements. For example, Curis may experience adverse results, delays and/or failures in its drug development programs and may not be able to successfully advance the development of its drug candidates in the time frames it projects, if at all. Curis's drug candidates may fail to demonstrate sufficient safety and efficacy in clinical studies and/or may never receive regulatory approval. Favorable results seen in preclinical studies and early clinical trials of Curis's drug candidates may not be replicated in later trials. There can be no guarantee that Curis's collaborations with Aurigene and ImmuNext will continue for their full terms and receive sufficient financing and other resources, or that the parties will successfully discover, develop or commercialize drug candidates under the collaborations. Regulatory authorities may delay or restrict Genentech's and/or Roche's ability to continue to develop or commercialize Erivedge in BCC. Erivedge may not merit further development in disease indications other than BCC. Competing drugs may be developed that are superior to Erivedge. Curis faces risks relating to the transfer and encumbrance of certain royalty and royalty-related payments on commercial sales of Erivedge, which could have a material adverse effect on its business, financial condition and stock price. Based on its available cash resources, Curis does not have sufficient cash on hand to support current operations for the next 12 months. If it is not able to obtain sufficient funding, it will be forced to delay, reduce in scope or eliminate some of its research and development programs, including related clinical trials and operating expenses, potentially delaying the time to market for, or preventing the marketing of, any of its product candidates, which could adversely affect its ability to continue operations and pursue its business strategies. Curis faces substantial competition. Curis also faces the risk of potential adverse decisions made by the FDA and other regulatory authorities, investigational review boards, and publication review bodies. Curis may not obtain or maintain necessary patent protection and could become involved in expensive and time-consuming patent proceedings. Unstable market and economic conditions, natural disasters, public health crises, political crises and other events outside of Curis's control could significantly disrupt its operations or the operations of third parties on which Curis depends. For example, the COVID-19 pandemic may result in closures of third-party facilities, impact clinical trial enrollment or impact sales of Erivedge. The extent to which the COVID-19 pandemic may impact Curis's business is uncertain. Other important factors that may cause or contribute to actual results being materially different from those indicated by forward-looking statements include the factors set forth under the caption "Risk Factors" in Curis's most recent Form 10-K and Form 10-Q and the factors that are discussed in other filings that Curis periodically makes with the Securities and Exchange Commission ("SEC"). In addition, any forward-looking statements represent the views of Curis only as of today and should not be relied upon as representing Curis's views as of any subsequent date. Curis disclaims any intention or obligation to update any of the forward-looking statements after the date of this press release whether as a result of new information, future events or otherwise, except as may be required by law.

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