April 19, 2016

Cerulean Announces Presentation at 2016 American Association for Cancer Research Annual Meeting of Stage 1 Data from Phase 2 Trial in Platinum-Resistant Ovarian Cancer

Late-Breaker Presents Results from Investigator-Sponsored Trial of CRLX101 in Combination with Avastin® (bevacizumab) in Patients with Platinum-Resistant Ovarian Cancer

WALTHAM, Mass.--(BUSINESS WIRE)-- Cerulean Pharma Inc. (NASDAQ:CERU), a clinical-stage company developing nanoparticle-drug conjugates (NDCs), today announced the presentation of clinical data in a late-breaker at the 2016 American Association for Cancer Research (AACR) Annual Meeting. Adrian Senderowicz, M.D., Senior Vice President and Chief Medical Officer of Cerulean will present results from the first 18 patients enrolled in an investigator-sponsored trial.

The trial studies CRLX101 in combination with Avastin in patients with platinum-resistant ovarian cancer. The trial employs a Simon Two-Stage Design and has met the stage gate criteria for advancement into Stage 2. In order to advance to Stage 2, more than two events of progression-free survival at six months (PFS6) were needed. Eighteen patients were treated in Stage 1, and 25 patients will be enrolled in Stage 2. The study is sponsored by Massachusetts General Hospital. Cerulean and Genentech provide the trial with CRLX101 and Avastin, respectively.

"This arm of the study is designed to assess whether combining Avastin with CRLX101 improves the antitumor activity observed with CRLX101 in the monotherapy arm of the study," said Carolyn N. Krasner, M.D., principal investigator of the trial. "VEGF inhibitors such as Avastin starve tumors of oxygen, resulting in tumor shrinkage, but they may exacerbate the hypoxic conditions present in solid tumors, which would increase HIF-1α. CRLX101 inhibits HIF-1α and mitigates HIF-associated resistance to VEGF inhibitors. The Stage 1 results show that CRLX101 plus Avastin appears to provide a greater benefit than CRLX101 alone. I am pleased that the study has advanced into Stage 2."

"The increased tumor activity observed by combining CRLX101 with Avastin is encouraging," said Dr. Senderowicz. "We also are evaluating the combination of CRLX101 and Avastin in our randomized Phase 2 study in 3rd and 4th line renal cell carcinoma, and there are many other opportunities to combine our HIF-inhibitor with Avastin or other therapies, including TKIs, chemotherapy or radiation therapy."

Results from Stage 1 Arm of Phase 2 Trial of CRLX101 in Combination with Avastin in Platinum-Resistant Ovarian Cancer

In this open-label study, patients were dosed in two arms: a CRLX101 monotherapy arm (Group A) and a combination arm of CRLX101 with Avastin (Group B). Group A patients received CRLX101 at 15 mg/m^2 every other week (presented at ASCO 2014) and Group B patients received CRLX101 at 15 mg/m^2 with Avastin 10 mg/kg every other week. The primary endpoint for both groups was the rate of PFS6 using RECIST 1.1 criteria. Secondary endpoints were objective response rate (ORR), PFS, ≥50% reduction of CA125 over baseline, and safety. Adverse events (AEs) were assessed by CTCAE v4.0. Pre- and post-treatment tumor biopsies were collected from a cohort of patients on CRLX101 as monotherapy to evaluate relevant progressive disease endpoints.

In Stage 1 of Group B, a total of 18 platinum-resistant ovarian cancer patients were evaluated. PFS6 (defined as patients that are free of progression at six months) was demonstrated in 56% of patients (10 out of 18). The overall response rate was 17% (3 out of 18 patients). The overall stable disease rate was 78% (14 out of 18 patients). The clinical benefit rate (defined as patients that either achieved a partial response or stable disease) was 94% (17 out of 18). Median PFS (defined as time from first dose to discontinuation of treatment) is 6.2 months so far, and 2 of 18 patients have been on treatment after 11 months. A ≥ 50% decline in CA125 was demonstrated in 44% patients (8 out of 18). Thus far, Group B showed increased antitumor activity relative to Group A on all of the aforementioned dimensions.

AEs most commonly observed with the combination were anemia (10 patients, 56%); nausea (10 patients, 56%); fatigue (6 patients, 33%); and proteinuria (5 patients, 28%). The majority of AEs were Grade 1. There were four drug-related AEs that were Grade 3 or 4: Grade 3 anemia, elevated alanine transaminase levels, and non-infective cystitis, and Grade 4 febrile neutropenia.

Details of the AACR late-breaking poster presentation are as follows:

Title:	Phase 2 trial of the NDC CRLX101 in combination with Avastin in patients with Platinum-Resistant OvarianCancer (PROC)
Date and Time:	Tuesday, April 19 - 8:00 am to 12:00 pm
Abstract number:	CT090
Location:	Section 13
Poster board number:	18

A copy of the poster will be available upon request following AACR by emailing ir@ceruleanrx.com.

About CRLX101

CRLX101 is a nanoparticle-drug conjugate (NDC) designed to concentrate in tumors and slowly release its anti-cancer payload, camptothecin, inside tumor cells. CRLX101 inhibits topoisomerase 1 (topo 1), which is involved in cellular replication, and also inhibits hypoxia-inducible factor-1α (HIF-1α), which research suggests is a master regulator of cancer cell survival mechanisms. CRLX101 has shown activity in four different tumor types, both as monotherapy and in combination with other cancer treatments. CRLX101 is in Phase 2 clinical development and has been dosed in more than 350 patients. The U.S. FDA has granted CRLX101 Orphan Drug designation for the treatment of ovarian cancer and Fast Track designation in combination with Avastin in metastatic renal cell carcinoma.

About Cerulean Pharma

The Cerulean team is committed to improving treatment for people living with cancer. We apply our Dynamic Tumor Targeting™ Platform to create a portfolio of NDCs designed to selectively attack tumor cells, reduce toxicity by sparing the body's normal cells, and enable therapeutic combinations. Our first platform-generated NDC clinical candidate, CRLX101, is in multiple clinical trials in combination with other cancer treatments, all of which aim to unlock the power of combination therapy. Our second platform-generated NDC clinical candidate, CRLX301, is in a Phase 1/2a clinical trial. For more information, please visit www.ceruleanrx.com.

About Cerulean's Dynamic Tumor Targeting™ Platform

Cerulean's Dynamic Tumor Targeting Platform creates NDCs that are designed to provide safer and more effective cancer treatments. We believe our NDCs concentrate their anti-cancer payloads inside tumors while sparing normal tissue because they are small enough to pass through the "leaky" vasculature present in tumors but are too large to pass through the wall of healthy blood vessels. Once inside tumors, our NDCs enter tumor cells where they slowly release anti-cancer payloads from within the tumor cells.

Cautionary Note on Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including statements about the clinical development of our product candidates, statements about our estimated research and development expenses and sufficiency of cash to fund specified use of cash and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "hypothesize," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation of clinical trials, availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 10, 2016, and in other filings that we make with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent our views only as of the date of this release and should not be relied upon as representing our views as of any subsequent date. We specifically disclaim any obligation to update any forward-looking statements included in this press release.

Avastin is a registered trademark of Genentech, Inc.

Cerulean Pharma
Nicole P. Jones, 781-209-6385
Director, Investor Relations and
Corporate Communications
njones@ceruleanrx.com

Source: Cerulean Pharma Inc.