

CytRx Announces Presentation of Interim Phase 2 Data for Aldoxorubicin for HIV-Related Kaposi's Sarcoma

Results Demonstrate Uptake of Doxorubicin in KS Lesions and High Response Rates, and that Aldoxorubicin is Well Tolerated and Shows Therapeutic Activity

Data to be Presented at the 18th International Workshop on Kaposi's Sarcoma Herpesvirus

LOS ANGELES – June 29, 2015 – CytRx Corporation (NASDAQ: CYTR), a biopharmaceutical research and development company specializing in oncology, today announced the presentation of interim results from its ongoing open-label Phase 2 pilot study evaluating the efficacy and safety of aldoxorubicin for the treatment of Kaposi's Sarcoma (KS) in HIV-infected patients. The data will be presented on Wednesday, July 1, 2015 during a poster session at the 18th International Workshop on Kaposi's Sarcoma Herpesvirus (KSHV) and Related Agents in Hollywood, Florida.

For the study, patients with biopsy-confirmed KS were administered 100 or 150 mg/m² aldoxorubicin (75 or 112 mg/m² doxorubicin equivalents) IV every three weeks. At the time of presentation, preliminary analyses were available for nine patients who received at least six cycles of drug (mean = 6.3 cycles). Four patients had received prior Doxil chemotherapy. Of these 9 patients, 6 (67%) demonstrated a partial response to aldoxorubicin at the end of study visit (EOS), and 7 (78%) demonstrated PR within 4 months of EOS. Doxorubicin could be detected in all tumor biopsies and higher doxorubicin concentrations were demonstrated within KS lesions relative to skin next to the lesions for 3/4 (75%) patients for whom adequate tissue was available for analysis. Five of 6 (83%) patients receiving aldoxorubicin and for whom data are available exhibited reduced intratumoral viral loads during therapy. A subset of patients also exhibited improvements in quality of life during treatment, and all patients exhibited either improvement or stability in immunologic and virologic HIV treatment parameters. Aldoxorubicin was well-tolerated, with only 2 patients (22%) experiencing a grade 4 adverse event (transient neutropenia and anemia), and overall AEs (44%) were mild and compared favorably with AE rates from other trials enrolling KS patients representing urban, minority-predominant populations.

“KS remains an important cause of morbidity and mortality for HIV-infected patients worldwide, yet significant toxicities limit drug exposure and outcomes for many patients when antiretroviral therapy is combined with standard treatments like liposomal doxorubicin (Doxil),” said Chris Parsons, MD, Associate Professor in the Departments of Medicine and Microbiology, Immunology, & Parasitology at the Louisiana State University Health Sciences Center, and principal investigator of the study. “These data demonstrate aldoxorubicin’s ability to leverage cancer biology to preferentially release chemotherapeutic drugs in tumors, thereby limiting toxicity, increasing drug exposure and improving

outcomes. We remain highly encouraged by the activity and tolerability of aldoxorubicin in this study, and look forward to its continued enrollment and final results.”

This open-label Phase 2 clinical trial is expected to enroll up to 30 patients, randomly assigned to three equally sized treatment arms which will receive aldoxorubicin at 100 or 150 mg/m² by 30-minute intravenous infusion. Because the KS patients in the study have compromised immune systems, the aldoxorubicin dosages administered in the trial are lower than those administered in the Company's clinical testing of aldoxorubicin in patients with soft tissue sarcomas. Patients with advanced KS receive aldoxorubicin on day 1, then every 3 weeks until evidence of tumor progression, unacceptable toxicity or withdrawal of consent. The primary objectives of preliminary efficacy include evaluation of the size, number and nodularity of skin lesions, change in size and number of lung lesions and changes in the number of tumor cells that express the KS virus DNA (Human Herpes Virus 8). The Company is also evaluating the level of aldoxorubicin uptake into lesions. Safety is being assessed through monitoring of adverse events and the ability to remain on assigned treatment. The trial is being conducted at the Louisiana State University Health Sciences Center in New Orleans, LA.

KS is an orphan indication in the U.S.

About Kaposi's Sarcoma

Kaposi sarcoma is a cancer that causes lesions (abnormal tissue) to grow in the skin; the mucous membranes lining the mouth, nose, and throat; lymph nodes; or other organs. The lesions are usually purple and are made of cancer cells, new blood vessels, red blood cells, and white blood cells. Kaposi sarcoma is different from other cancers in that lesions may begin in more than one place in the body at the same time. KS remains the most common HIV-associated tumor worldwide. The condition is also endemic in certain parts of Central Africa and Central and Eastern Europe.

About Aldoxorubicin

The widely used chemotherapeutic agent doxorubicin is delivered systemically and is highly toxic, which limits its dose to a level below its maximum therapeutic benefit. Doxorubicin also is associated with many side effects, especially the potential for damage to heart muscle at cumulative doses greater than 450 mg/m². Aldoxorubicin combines doxorubicin with a novel single-molecule linker that binds directly and specifically to circulating albumin, the most plentiful protein in the bloodstream. Protein-hungry tumors concentrate albumin, thus increasing the delivery of the linker molecule with the attached doxorubicin to tumor sites. In the acidic environment of the tumor, but not the neutral environment of healthy tissues, doxorubicin is released. This allows for greater doses (3 ½ to 4 times) of doxorubicin to be administered while reducing its toxic side effects. In studies thus far there has been no evidence of clinically significant effects of aldoxorubicin on heart muscle, even at cumulative doses of drug well in excess of 2,000 mg/m².

About CytRx Corporation

CytRx Corporation is a biopharmaceutical research and development company specializing in oncology. CytRx currently is focused on the clinical development of aldoxorubicin (formerly known as INNO-206), its improved version of the widely used chemotherapeutic agent doxorubicin. CytRx has initiated under a special protocol assessment a pivotal Phase 3 global trial with aldoxorubicin as a therapy for patients with soft tissue sarcomas whose tumors have progressed following treatment with chemotherapy, and has announced that it has received approval from the FDA to continue dosing patients with aldoxorubicin until disease progression in that clinical trial. CytRx is currently evaluating aldoxorubicin in a global Phase 2b clinical trial in small cell lung cancer, a Phase 2 clinical trial in HIV-related Kaposi's sarcoma, a Phase 2 clinical trial in patients with late-stage glioblastoma (brain cancer), a Phase 1b trial in combination with ifosfamide in patients with soft tissue sarcoma, and a Phase 1b trial in combination with gemcitabine in subjects with metastatic solid tumors. CytRx has completed a global Phase 2b clinical trial with aldoxorubicin as a first-line therapy for soft tissue sarcomas, a Phase 1b/2 clinical trial primarily in the same indication, a Phase 1b clinical trial of aldoxorubicin in combination with doxorubicin in patients with advanced solid tumors and a Phase 1b pharmacokinetics clinical trial in patients with metastatic solid tumors. CytRx plans to expand its pipeline of oncology candidates at its laboratory facilities in Freiburg, Germany, based on novel linker technologies that can be utilized with multiple chemotherapeutic agents and may allow for greater concentration of drug at tumor sites. For more information about CytRx Corporation, visit www.cytrx.com.

ALDOXORUBICIN: AN ALBUMIN-BINDING PRODRUG FOR KS TREATMENT

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Abstract

Background: KS remains an important cause of morbidity and mortality for HIV-related patients worldwide. In addition to enhanced delivery, several treatments include systemic doxorubicin or paclitaxel, but significant toxicities limit drug exposure and outcomes for many patients. Improving drug delivery, systemic treatment, and patient quality of life are important unmet needs. We designed a novel prodrug, aldoxorubicin, as a novel prodrug of doxorubicin that binds to the first group of cysteine-34 of albumin and is released in an acidic environment associated with solid tumors. Patients with KS-associated KS are more eligible to receive aldoxorubicin in an early Phase 3 clinical trial performed at our institution, and we report preliminary results from this study.

Methods: Patients with HIV-associated KS and histologic confirmation of an advanced KS (≥150 ng/ml) administered 175 or 112 mg/m² aldoxorubicin (ALDO) to every three weeks. Primary endpoints are overall survival (OS) and disease-related mortality at study onset, and four of these six patients displayed histologic response to therapy. One patient (P001) displayed disease progression after an initial therapeutic response to treatment. Doxorubicin could be detected in all tumor lesions (≥10 ng/ml) before and two weeks post-treatment. Significant reductions in HIV-related opportunistic infection lesions during treatment. A reduction in cancer-related symptoms, quality of life, and overall survival were observed. ALDO may improve the quality of life of patients with HIV-associated KS, the majority of whom exhibit advanced disease at study enrollment. ALDO may also be used to extend therapeutic activity in the majority of patients, with reduction in both histologic and pathologic disease burden. ALDO may offer a safe and effective therapeutic alternative for HIV-associated KS.

Background

KS trials involving active, minority preselected cohorts with moderate to severe disease show CD4⁺ T-cell counts and raised KS-related morbidity and lack of efficacy for advanced disease.

- A first-in-class, phase 1b/2, open-label, randomized, controlled trial (P12) was conducted in patients with advanced KS who had not received prior systemic cytotoxic chemotherapy.
- CR + PR response rates (5% (P1X), 4% (P1D), 12% (P1E), 12% (P1F), 12% (P1G), 12% (P1H), 12% (P1I), 12% (P1J), 12% (P1K), 12% (P1L), 12% (P1M), 12% (P1N), 12% (P1O), 12% (P1P), 12% (P1Q), 12% (P1R), 12% (P1S), 12% (P1T), 12% (P1U), 12% (P1V), 12% (P1W), 12% (P1X), 12% (P1Y), 12% (P1Z), 12% (P1AA), 12% (P1AB), 12% (P1AC), 12% (P1AD), 12% (P1AE), 12% (P1AF), 12% (P1AG), 12% (P1AH), 12% (P1AI), 12% (P1AJ), 12% (P1AK), 12% (P1AL), 12% (P1AM), 12% (P1AN), 12% (P1AO), 12% (P1AP), 12% (P1AQ), 12% (P1AR), 12% (P1AS), 12% (P1AT), 12% (P1AU), 12% (P1AV), 12% (P1AW), 12% (P1AX), 12% (P1AY), 12% (P1AZ), 12% (P1BA), 12% (P1BB), 12% (P1BC), 12% (P1BD), 12% (P1BE), 12% (P1BF), 12% (P1BG), 12% (P1BH), 12% (P1BI), 12% (P1BJ), 12% (P1BK), 12% (P1BL), 12% (P1BM), 12% (P1BN), 12% (P1BO), 12% (P1BP), 12% (P1BQ), 12% (P1BR), 12% (P1BS), 12% (P1BT), 12% (P1BU), 12% (P1BV), 12% (P1BW), 12% (P1BX), 12% (P1BY), 12% (P1BZ), 12% (P1CA), 12% (P1CB), 12% (P1CC), 12% (P1CD), 12% (P1CE), 12% (P1CF), 12% (P1CG), 12% (P1CH), 12% (P1CI), 12% (P1CJ), 12% (P1CK), 12% (P1CL), 12% (P1CM), 12% (P1CN), 12% (P1CO), 12% (P1CP), 12% (P1CQ), 12% (P1CR), 12% (P1CS), 12% (P1CT), 12% (P1CU), 12% (P1CV), 12% (P1CW), 12% (P1CX), 12% (P1CY), 12% (P1CZ), 12% (P1DA), 12% (P1DB), 12% (P1DC), 12% (P1DD), 12% (P1DE), 12% (P1DF), 12% (P1DG), 12% (P1DH), 12% (P1DI), 12% (P1DJ), 12% (P1DK), 12% (P1DL), 12% (P1DM), 12% (P1DN), 12% (P1DO), 12% (P1DP), 12% (P1DQ), 12% (P1DR), 12% (P1DS), 12% (P1DT), 12% (P1DU), 12% (P1DV), 12% (P1DW), 12% (P1DX), 12% (P1DY), 12% (P1DZ), 12% (P1EA), 12% (P1EB), 12% (P1EC), 12% (P1ED), 12% (P1EE), 12% (P1EF), 12% (P1EG), 12% (P1EH), 12% (P1EI), 12% (P1EJ), 12% (P1EK), 12% (P1EL), 12% (P1EM), 12% (P1EN), 12% (P1EO), 12% (P1EP), 12% (P1EQ), 12% (P1ER), 12% (P1ES), 12% (P1ET), 12% (P1EU), 12% (P1EV), 12% (P1EW), 12% (P1EX), 12% (P1EY), 12% (P1EZ), 12% (P1FA), 12% (P1FB), 12% (P1FC), 12% (P1FD), 12% (P1FE), 12% (P1FF), 12% (P1FG), 12% (P1FH), 12% (P1FI), 12% (P1FJ), 12% (P1FK), 12% (P1FL), 12% (P1FM), 12% (P1FN), 12% (P1FO), 12% (P1FP), 12% (P1FQ), 12% (P1FR), 12% (P1FS), 12% (P1FT), 12% (P1FU), 12% (P1FV), 12% (P1FW), 12% (P1FX), 12% (P1FY), 12% (P1FZ), 12% (P1GA), 12% (P1GB), 12% (P1GC), 12% (P1GD), 12% (P1GE), 12% (P1GF), 12% (P1GG), 12% (P1GH), 12% (P1GI), 12% (P1GJ), 12% (P1GK), 12% (P1GL), 12% (P1GM), 12% (P1GN), 12% (P1GO), 12% (P1GP), 12% (P1GQ), 12% (P1GR), 12% (P1GS), 12% (P1GT), 12% (P1GU), 12% (P1GV), 12% (P1GW), 12% (P1GX), 12% (P1GY), 12% (P1GZ), 12% (P1HA), 12% (P1HB), 12% (P1HC), 12% (P1HD), 12% (P1HE), 12% (P1HF), 12% (P1HG), 12% (P1HH), 12% (P1HI), 12% (P1HJ), 12% (P1HK), 12% (P1HL), 12% (P1HM), 12% (P1HN), 12% (P1HO), 12% (P1HP), 12% (P1HQ), 12% (P1HR), 12% (P1HS), 12% (P1HT), 12% (P1HU), 12% (P1HV), 12% (P1HW), 12% (P1HX), 12% (P1HY), 12% (P1HZ), 12% (P1IA), 12% (P1IB), 12% (P1IC), 12% (P1ID), 12% (P1IE), 12% (P1IF), 12% (P1IG), 12% (P1IH), 12% (P1II), 12% (P1IJ), 12% (P1IK), 12% (P1IL), 12% (P1IM), 12% (P1IN), 12% (P1IO), 12% (P1IP), 12% (P1IQ), 12% 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Structure of AldoXorubicin

Proposed Mechanism of Action

Patient Profile

Patient	Initial CD4 count/ml (%)	T1S	Prior Dose (mg/m ²)
001	16 (1.5)	T1, T1, S1	120
002	302 (31)	T1, T0, S0	180
003	400 (27)	T1, T0, S1	None
004	444 (27)	T0, T0, S1	None
005	98 (11.7)	T1, T1, S1	150
006	40 (5.3)	T1, T1, S1	None
007	180 (11.8)	T1, T1, S1	None
008	49 (2.8)	T1, T1, S1	None
009	27 (8.4)	T1, T1, S1	20

HIV Parameters on Study

Results

Patient	Cycle 4	EOS	Cycle 4	EOS
001	24%	24%	-17%	-21%
002	-30%	NA	0%	0%
003	-8%	-23%	-4%	-4%
004	NT	NT	1%	-24%
005	0%	NA	4%	NA
006	NA	NA	NA	NA
007	0%	-22%	0%	0%
008	-50%	-67%	-9%	-6%
009	NA	NA	12%	29%

Study Design

- Open-label phase 2 pilot study evaluating the preliminary efficacy and safety of aldoxorubicin administered at 150 or 110 mg/m² (2x and 1.5x doxorubicin equivalents) by full weekly 21 days until evidence of tumor progression, unacceptable toxicity, withdrawal of consent, or at the discretion of medical oncologist.
- Staging assessed using the AIDS Clinical Trials Group (ACTG) tumor, response, toxicity, systemic illness (TRIS) criteria.
- Response to therapy (baseline, prior to Cycle 4, and end of study to evaluate visceral lesions)
 - skin lesions (number, size, and nodularity)
 - chest computed tomography (CT)
 - abdominal/magnetic resonance imaging (MRI)
- Safety monitoring
 - physical examination, KPS, ECOG performance
 - laboratory evaluation (serum chemistry, complete blood count)
 - cardiac (ECHO) and ECG
- Laboratory monitoring
 - intratumoral LANA expression (biopsy)
 - tumor vs non-tumor skin AldoXorubicin concentrations

Background

KS trials involving active, minority preselected cohorts with moderate to severe disease show CD4⁺ T-cell counts and raised KS-related morbidity and lack of efficacy for advanced disease.

- A first-in-class, phase 1b/2, open-label, randomized, controlled trial (P12) was conducted in patients with advanced KS who had not received prior systemic cytotoxic chemotherapy.
- CR + PR response rates (5% (P1X), 4% (P1D), 12% (P1E), 12% (P1F), 12% (P1G), 12% (P1H), 12% (P1I), 12% (P1J), 12% (P1K), 12% (P1L), 12% (P1M), 12% (P1N), 12% (P1O), 12% (P1P), 12% (P1Q), 12% (P1R), 12% (P1S), 12% (P1T), 12% (P1U), 12% (P1V), 12% (P1W), 12% (P1X), 12% (P1Y), 12% (P1Z), 12% (P1AA), 12% (P1AB), 12% (P1AC), 12% (P1AD), 12% (P1AE), 12% (P1AF), 12% (P1AG), 12% (P1AH), 12% (P1AI), 12% (P1AJ), 12% (P1AK), 12% (P1AL), 12% (P1AM), 12% (P1AN), 12% (P1AO), 12% (P1AP), 12% (P1AQ), 12% (P1AR), 12% (P1AS), 12% (P1AT), 12% (P1AU), 12% (P1AV), 12% (P1AW), 12% (P1AX), 12% (P1AY), 12% (P1AZ), 12% (P1BA), 12% (P1BB), 12% (P1BC), 12% (P1BD), 12% (P1BE), 12% (P1BF), 12% (P1BG), 12% (P1BH), 12% (P1BI), 12% (P1BJ), 12% (P1BK), 12% (P1BL), 12% (P1BM), 12% (P1BN), 12% (P1BO), 12% (P1BP), 12% (P1BQ), 12% (P1BR), 12% (P1BS), 12% (P1BT), 12% (P1BU), 12% (P1BV), 12% (P1BW), 12% (P1BX), 12% (P1BY), 12% (P1BZ), 12% (P1CA), 12% (P1CB), 12% (P1CC), 12% (P1CD), 12% (P1CE), 12% (P1CF), 12% (P1CG), 12% (P1CH), 12% (P1CI), 12% (P1CJ), 12% (P1CK), 12% (P1CL), 12% (P1CM), 12% (P1CN), 12% (P1CO), 12% (P1CP), 12% (P1CQ), 12% (P1CR), 12% (P1CS), 12% (P1CT), 12% (P1CU), 12% (P1CV), 12% (P1CW), 12% (P1CX), 12% (P1CY), 12% (P1CZ), 12% (P1DA), 12% (P1DB), 12% (P1DC), 12% (P1DD), 12% (P1DE), 12% (P1DF), 12% (P1DG), 12% (P1DH), 12% (P1DI), 12% (P1DJ), 12% (P1DK), 12% (P1DL), 12% (P1DM), 12% (P1DN), 12% (P1DO), 12% (P1DP), 12% (P1DQ), 12% (P1DR), 12% (P1DS), 12% (P1DT), 12% (P1DU), 12% (P1DV), 12% (P1DW), 12% (P1DX), 12% (P1DY), 12% (P1DZ), 12% (P1EA), 12% (P1EB), 12% (P1EC), 12% (P1ED), 12% (P1EE), 12% (P1EF), 12% (P1EG), 12% (P1EH), 12% (P1EI), 12% (P1EJ), 12% (P1EK), 12% (P1EL), 12% (P1EM), 12% (P1EN), 12% (P1EO), 12% (P1EP), 12% (P1EQ), 12% (P1ER), 12% (P1ES), 12% (P1ET), 12% (P1EU), 12% (P1EV), 12% (P1EW), 12% (P1EX), 12% (P1EY), 12% (P1EZ), 12% (P1FA), 12% (P1FB), 12% (P1FC), 12% (P1FD), 12% (P1FE), 12% (P1FF), 12% (P1FG), 12% (P1FH), 12% (P1FI), 12% (P1FJ), 12% (P1FK), 12% (P1FL), 12% (P1FM), 12% (P1FN), 12% (P1FO), 12% (P1FP), 12% (P1FQ), 12% (P1FR), 12% (P1FS), 12% (P1FT), 12% (P1FU), 12% (P1FV), 12% (P1FW), 12% (P1FX), 12% (P1FY), 12% (P1FZ), 12% (P1GA), 12% (P1GB), 12% (P1GC), 12% (P1GD), 12% (P1GE), 12% (P1GF), 12% (P1GG), 12% (P1GH), 12% (P1GI), 12% (P1GJ), 12% (P1GK), 12% (P1GL), 12% (P1GM), 12% (P1GN), 12% (P1GO), 12% (P1GP), 12% (P1GQ), 12% (P1GR), 12% (P1GS), 12% (P1GT), 12% (P1GU), 12% (P1GV), 12% (P1GW), 12% (P1GX), 12% (P1GY), 12% (P1GZ), 12% (P1HA), 12% (P1HB), 12% (P1HC), 12% (P1HD), 12% (P1HE), 12% (P1HF), 12% (P1HG), 12% (P1HI), 12% (P1HJ), 12% (P1HK), 12% (P1HL), 12% (P1HM), 12% (P1HN), 12% (P1HO), 12% (P1HP), 12% (P1HQ), 12% (P1HR), 12% (P1HS), 12% (P1HT), 12% (P1HU), 12% (P1HV), 12% (P1HW), 12% (P1HX), 12% (P1HY), 12% (P1HZ), 12% (P1IA), 12% (P1IB), 12% (P1IC), 12% (P1ID), 12% (P1IE), 12% (P1IF), 12% (P1IG), 12% (P1IH), 12% (P1II), 12% (P1IJ), 12% (P1IK), 12% (P1IL), 12% (P1IM), 12% (P1IN), 12% (P1IO), 12% (P1IP), 12% (P1IQ), 12% (P1IR), 12% (P1IS), 12% (P1IT), 12% (P1IU), 12% (P1IV), 12% (P1IW), 12% (P1IX), 12% (P1IY), 12% (P1IZ), 12% (P1JA), 12% (P1JB), 12% (P1JC), 12% (P1JD), 12% (P1JE), 12% (P1JF), 12% (P1JG), 12% (P1JH), 12% (P1JI), 12% (P1JJ), 12% (P1JK), 12% (P1JL), 12% (P1JM), 12% (P1JN), 12% (P1JO), 12% (P1JP), 12% (P1JQ), 12% (P1JR), 12% (P1JS), 12% (P1JT), 12% (P1JU), 12% (P1JV), 12% (P1JW), 12% (P1JX), 12% (P1JY), 12% (P1JZ), 12% (P1KA), 12% (P1KB), 12% (P1KC), 12% (P1KD), 12% (P1KE), 12% (P1KF), 12% (P1KG), 12% (P1KH), 12% (P1KI), 12% (P1KJ), 12% (P1KL), 12% (P1KM), 12% (P1KN), 12% (P1KO), 12% (P1KP), 12% (P1KQ), 12% (P1KR), 12% (P1KS), 12% (P1KT), 12% (P1KU), 12% (P1KV), 12% (P1KW), 12% (P1KX), 12% (P1KY), 12% (P1KZ), 12% (P1LA), 12% (P1LB), 12% (P1LC), 12% (P1LD), 12% (P1LE), 12% (P1LF), 12% (P1LG), 12% (P1LH), 12% (P1LI), 12% (P1LJ), 12% (P1LK), 12% (P1LL), 12% (P1LM), 12% (P1LN), 12% (P1LO), 12% (P1LP), 12% (P1LQ), 12% (P1LR), 12% (P1LS), 12% (P1LT), 12% (P1LU), 12% (P1LV), 12% (P1LW), 12% (P1LX), 12% (P1LY), 12% (P1LZ), 12% (P1MA), 12% (P1MB), 12% (P