WPD Pharmaceuticals' Annamycin Received Positive Interim Results From Phase 1/2 Clinical Studies in Acute Myeloid Leukemia

No evidence of cardiotoxicity to date; 40% of 10 patients dosed at ≥120 mg/m² demonstrate efficacy; trial progresses in Europe to 210 mg/m²

VANCOUVER, British Columbia, Feb. 27, 2020 -- WPD Pharmaceuticals Inc. (CSE: WBIO)(FSE: 8SV1) (the "**Company**" or "**WPD**"), a clinical stage pharmaceutical company is pleased to provide an update on the clinical drug studies of Annamycin in Acute Myeloid Leukemia ("**AML**"). WPD's license partner Moleculin Biotech, Inc, ("**Moleculin**") (Nasdaq: MBRX) disclosed additional positive interim safety and efficacy data from one of the two ongoing open label, single arm Phase 1/2 studies of Annamycin for the treatment of relapsed or refractory AML.

Annamycin belongs to the group of anthracyclines, considered one of the most effective groups of oncological drugs. Annamycin was originally developed for the treatment of relapsed or refractory acute AML. Unlike clinically used anthracyclines, Annamycin effectively penetrates cancer cells, regardless of the presence of MDR proteins, while maintaining high safety and lack of cardiotoxicity.

The Phase 1 portion of these clinical trials, which are described in more detail later in this press release, is designed to establish the safety of Annamycin and to determine the Recommended Phase 2 Dose to be used in the Phase 2 portion of the trials. While the Primary Endpoint of the Phase 1 portion is safety, a Secondary Endpoint is the assessment of efficacy generally defined as an improvement in bone marrow biopsy results sufficient to qualify patients for a potentially curative bone marrow transplant.

The third cohort in Poland receiving a single dose of 180 mg/m^2 in the Phase 1 dose escalation portion of the trial was completed with no adverse events and the trial will continue to the next cohort of 210 mg/m^2. In the US trial, one patient has completed treatment in the second cohort at 120 mg/m^2. This brings the total number of patients treated and evaluated at or above 120 mg/m^2 to 10. An additional patient in the US has begun treatment at 120 mg/m^2 but has yet to complete post-treatment evaluation. The interim results for these 10 patients are 1 CRi (defined as a complete response with incomplete recovery of white blood cells and/or platelets) and 2 partial responses ("PRs" or where bone marrow blasts are reduced 50% and to below 25%). One additional patient was bridged to bone marrow transplant ("BT") based on a sufficient reduction in bone marrow blasts, bringing the total to 4 out of 10 patients at or above 120 mg/m^2 who have demonstrated efficacy.

In the latest cohort in Poland, 1 of the 3 patients treated at 180 mg/m^2 had a PR sufficient to qualify for a potentially curative bone marrow transplant. The results for all 3 patients were reviewed by the Safety Review Committee, which determined that no drug-related adverse events were observed that would prevent advancing the trial to the next higher dose level of 210 mg/m^2. To date in the European trial, only one adverse event related to Annamycin has been reported; a patient experienced grade 2 mucositis (which resolved to grade 1 within 2 days). In the parallel US clinical trial, one new patient (the first of cohort #2) achieved a "morphologically leukemia free state" or MLFS, which also constitutes a CRi, after receiving a single dose of 120 mg/m^2.

WPD refers to Annamycin as a "next generation anthracycline," because it is designed to provide enhanced therapeutic benefits when compared with traditional anthracyclines (like doxorubicin) while reducing the potential for unwanted cardiotoxicity, or damage to the heart. This design intent has previously been validated with preclinical toxicology studies in animal models (as required by FDA) demonstrating Annamycin has little to no cardiotoxicity when compared with doxorubicin. Of the 14 patients treated thus far in both trials, none has shown any evidence of cardiotoxicity. This includes 7 patients in Poland who were treated at levels above the US maximum allowable cumulative anthracycline dose level (550 mg/m^2), a limitation not imposed on our trial in Europe. If upheld in further studies, this lack of toxicity could be an important differentiator between Annamycin and the currently approved anthracyclines, for which cardiotoxicity is a well-known treatment limitation.

For example, a recent review published in Cardiovascular Drugs and Therapy (https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5346598/) reported that 65% of patients who received the equivalent of 550 mg/m^{2}of doxorubicin (a current standard of care anthracycline) exhibited sub-clinical cardiotoxicity, defined as a reduction in left ventricular ejection fraction >10% points to a value <50%. Of the 5 patients mentioned above who were treated in our European trial above 550 mg/m^2, no evidence of cardiotoxicity was detected. The same published review also suggested that a better long-term indicator of cardiotoxicity may be the measurement of an increase in a biomarker called Troponin. When measured as an early biomarker of cancer therapy-related cardiotoxicity, Troponin rise occurs consistently in 21% - 40% of patients after treatment with current standard of care anthracycline chemotherapy and, per the published review, such an increase in Troponin is associated with an increased risk of heart disease later in life. Of the 14 patients treated thus far in both of our Annamycin clinical trials, none has shown an increase in Troponin levels.

Study Design

The study of Annamycin in both the US and Europe in open label, single arm clinical trials is to assess the safety and efficacy of Annamycin for the treatment of adults with relapsed or refractory acute myeloid leukemia. The US and European trials have the same study design, consisting of a Phase 1 intended to establish a "Recommended Phase 2 Dose" ("RP2D"), to which the studies will then proceed. The Phase 1 studies provide for escalating doses in cohorts of 3 patients each, with each successive cohort receiving the next higher dose level until "dose limiting toxicities" prevent further increases. Cohorts 1, 2 and 3 in Poland received a dose of 120, 150 and 180 mg/m^2, respectively, and the results now permit moving to 210 mg/m^2. Cohort 1 in the US started at 100 mg/m^2, and the results supported moving to 120 mg/m^2, at which 1 patient has now been treated and evaluated as having achieved a "morphologically leukemia free state" or MLFS, which also constitutes a CRi. Because one patient in US cohort 1 did not complete the evaluation protocol, a fourth patient was added to complete that cohort. Once an RP2D is established, the intent is for each trial to advance to a Phase 2 arm planned to assess the safety and efficacy of Annamycin in 21 additional patients.

The data reported here is preliminary as collected by independent CRO site monitors per standard practice and is subject to subsequent quality assurance review.

In collaboration with Moleculin, WPD intends to continue reporting top-line results by cohort in each trial, with each announcement also including an update on the other trial. Top-line results will include reporting of any drug-related adverse events ("AEs") and assessment of cardiotoxicity, including ECHO or MUGA scans measuring change in ejection fraction and measuring blood Troponin level, which is considered a biomarker for potential long-term cardiovascular impairment. To date, one patient experienced grade 2 mucositis (which resolved to grade 1 within 2 days) and no other drug-related AEs have been reported. Also, no loss of ejection fraction or rise in Troponin levels has been reported. Top-line results will also include the number of partial responses ("**PRs**"), complete responses ("**CRs**") and patients deemed capable of progressing to a potentially curative bone marrow transplant, which we term "bridge to transplant" ("**BTs**"), each of which is essentially a function of the magnitude of reduction in a patient's bone marrow blasts. For purposes of these clinical trials, a CR means that the patient's bone marrow blasts reduced to 5% or less (with CRi meaning a CR where there was incomplete recovery of white blood cell and/or platelet counts), a PR means the patient's bone marrow blasts reduced by 50% and resulted in a blast count of 25% or less, and a BT means patients are deemed capable of progressing to a potentially curative bone marrow transplant. To date, there has been 1 CRi in the US (@ 120 mg/m^2), 2 PRs in Europe (1 @ 120 mg/m^2 and 1 @ 180 mg/m^2) and 4 BTs (1 in the US and 3 in Europe).

The US trial also differs from the European trial in that the FDA would like to review safety data relating to cardiotoxicity from patients treated prior to advancing beyond 120 mg/m^2, as exceeding this dose level would require the patient to exceed the established lifetime maximum exposure to anthracyclines (presuming all anthracyclines are cardiotoxic). To date, 100% of all 14 patients treated in both the US and EU trials have shown no incidence of cardiotoxicity, including 7 patients out of 9 treated in Poland who exceeded the lifetime maximum anthracycline exposure level. The Company believes that the additional patient safety data gained from the European trial may also assist in the FDA's review of Annamycin's cardiac safety.

About Moleculin Biotech, Inc.

Moleculin Biotech, Inc. is a clinical stage pharmaceutical company focused on the development of a broad portfolio of oncology drug candidates for the treatment of highly resistant tumors. The Company's clinical stage drugs are: Annamycin, a Next Generation Anthracycline, designed to avoid multidrug resistance mechanisms with little to no cardiotoxicity being studied for the treatment of relapsed or refractory acute myeloid leukemia, more commonly referred to as AML, WP1066, an Immune/Transcription Modulator capable of inhibiting p-STAT3 and other oncogenic transcription factors while also stimulating a natural immune response, targeting brain tumors, pancreatic cancer and hematologic malignancies, and WP1220, an analog to WP1066, for the topical treatment of cutaneous T-cell lymphoma. Moleculin is also engaged in preclinical development of additional drug candidates, including other Immune/Transcription Modulators, as well as compounds capable of Metabolism/Glycosylation Inhibition.

For more information about the Company, please visit http://www.moleculin.com.

About WPD Pharmaceuticals

WPD is a biotechnology research and development company with a focus on oncology, namely research and development of medicinal products involving biological compounds and small molecules. WPD has 10 novel drug candidates with 4 that are in clinical development stage. These drug candidates were researched at institutions including MD Anderson Cancer Center, Mayo Clinic and Emory University, and WPD currently has ongoing collaborations with Wake Forest University and leading hospitals and academic centers in Poland.

WPD has entered into license agreements with Wake Forest University Health Sciences and sublicense agreements with Moleculin Biotech, Inc. and CNS Pharmaceuticals, Inc., respectively, each of which grant WPD an exclusive, royalty-bearing sublicense to certain technologies of the licensor. Such agreements provide WPD with certain research, development, manufacturing and sales rights, among other things.

On Behalf of the Board

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Cautionary Statements:

Neither the Canadian Securities Exchange nor the Investment Industry Regulatory Organization of Canada accepts responsibility for the adequacy or accuracy of this release.

This press release contains forward-looking statements. Forward-looking statements are statements that contemplate activities, events or developments that the Company anticipates will or may occur in the future. Forward-looking statements in this press release include that Annamycin can provide enhanced therapeutic benefits when compared with traditional anthracyclines while reducing the potential for damage to the heart; that this lack of toxicity could be an important differentiator between Annamycin and the currently approved anthracyclines; and that WPD's drugs could be developed into novel treatments for cancer. These forward-looking statements reflect the Company's current expectations based on information currently available to management and are subject to a number of risks and uncertainties that may cause outcomes to differ materially from those projected. Factors which may prevent the forward looking statement from being realized is that competitors or others may successfully challenge a granted patent and the patent could be rendered void; that we are unable to raise sufficient funding for our research; that our drugs don't provide positive treatment, or if they do, the side effects are damaging; competitors may develop better or cheaper drugs; and we may be unable to obtain regulatory approval for any drugs we develop. Readers should refer to the risk disclosure included from time-to-time in the documents the Company files on SEDAR, available at www.sedar.com. Although the Company believes that the assumptions inherent in these forward-looking statements are reasonable, they are not guarantees of future performance and, accordingly, they should not be relied upon and there can be no assurance that any of them will prove to be accurate. Finally, these forward-looking statements are made as of the date of this press release and the Company assumes no obligation to update them except as required by applicable law.