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Moleculin Announces Positive Interim Results in First Cohort of Phase 1/2 Clinical Studies of Annamycin in Acute Myeloid Leukemia in the US and in Poland

One patient to proceed to potentially curative bone marrow transplant; no cardiotoxicity observed

HOUSTON, March 26, 2019 /PRNewswire/ -- Moleculin Biotech, Inc., (Nasdaq: MBRX) ("Moleculin" or the "Company"), a clinical stage pharmaceutical company with a broad portfolio of drug candidates targeting highly resistant tumors, today announced positive interim safety and efficacy data from two ongoing open label, single arm Phase 1/2 studies of Annamycin. In the first study, being conducted in the US, four patients have completed treatment at 100 mg/m^2 with no significant adverse events related to Annamycin, and the study will now proceed to the next higher dose of 120 mg/m^2. The second trial, taking place in Poland, started at a 120 mg/m^2 dose of Annamycin and has treated three patients. The first patient treated in that trial received a single course of Annamycin and his bone marrow blasts have reduced from 60% to 11%. Our principal investigator considers this response sufficient for the patient to proceed to consolidation therapy, with the goal of receiving a potentially curative bone marrow transplant. To date in Poland, one patient experienced grade 2 mucositis (which resolved to grade 1 within 2 days) and no other adverse events related to Annamycin have been reported. Trial results for the other two patients treated in Poland will not be known until the second quarter of this year.

"We are very pleased to have completed the treatment of patients in cohort 1 of the US trial and move to the next higher dose," commented Walter Klemp, Moleculin's Chairman and CEO. "In Poland, we are pleased to have such a positive response at the starting dose level in this trial. Of course the response of a single patient doesn't necessarily predict the outcome of the trial, but this is a great way to begin and it's consistent with our expectations for Annamycin."

Mr. Klemp continued: "One of the advantages we believe Annamycin will offer is a lack of cardiotoxicity. We have seen no evidence of cardiotoxicity in any of the patients treated thus far. We intend to advance the clinical study of Annamycin with the goal of ultimately

demonstrating the drug's safety and effectiveness to support regulatory approval in the US and European Union."

Dr. Robert Shepard, Moleculin's Chief Medical Officer for Annamycin added: "A prior clinical trial for Annamycin in acute leukemia demonstrated activity at its Maximum Tolerable Dose of 150 mg/m^2, so we are pleased for the US trial to move to the next higher dose in cohort 2 of 120 mg/m^2. In Poland, we consider reducing bone marrow blasts for Patient 1 down to 11% with a single course of Annamycin to be very encouraging. Of course, significant additional study is necessary to definitively demonstrate causality."

"We have begun a second course of Annamycin as a consolidation phase for Patient 1," commented Dr. Lidia Gil, Principal Investigator in the Polish Annamycin clinical trial. "Considering that this patient was refractory to standard of care induction therapy, I am very pleased to see that Annamycin appears to be showing activity. While this is considered a 'Partial Response,' I believe it's enough of a reduction to serve as a 'bridge to transplant' for this patient. Importantly, with the first course of Annamycin, no toxicities have been observed that would limit continued dosing with Annamycin."

Study Design

The Company is studying Annamycin in both the US and Poland in open label, single arm clinical trials to assess the safety and efficacy of Annamycin for the treatment of adults with relapsed or refractory acute myeloid leukemia. Both the US and Polish trials have the same study design, providing for a Phase 1 intended to establish a "Recommended Phase 2 Dose," ("RP2D") with cohorts of 3 patients each where the first cohort starts at a low beginning dose and each successive cohort receives the next higher dose level until "dose limiting toxicities" prevent further increases. In the case of cohort 1 in the US, one patient did not complete the evaluation protocol, so a fourth patient was added to complete that cohort.

A key difference in the US is that the starting dose was 100 mg/m^2, whereas, in Poland, the starting dose was 120 mg/m^2. Having completed the first cohort in the US, the Company is seeking patients for the second cohort at a dose level of 120 mg/m^2. Once 3 patients have completed the safety evaluation period of the first cohort in Poland, the second cohort will begin there at a dose level of 150 mg/m^2. Once the Company establishes an RP2D, 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 US trial also differs from the Polish 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. The Company believes that the additional patient safety data gained from the Polish trial may also assist in the FDA's review of 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 additional Immune/Transcription Modulators, as well as compounds capable of Metabolism/Glycosylation Inhibition.

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

Forward-Looking Statements

Some of the statements in this release are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995, which involve risks and uncertainties. Forward-looking statements in this press release include, without limitation, the ability of the Company to successfully recruit patients to complete its clinical trials and the ability of Annamycin to show safety and efficacy in patients. Although Moleculin believes that the expectations reflected in such forward-looking statements are reasonable as of the date made, expectations may prove to have been materially different from the results expressed or implied by such forward-looking statements. Moleculin Biotech has attempted to identify forward-looking statements by terminology including "believes," "estimates," "anticipates," "expects," "plans," "projects," "intends," "potential," "may," "could," "might," "will," "should," "approximately" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. These statements are only predictions and involve known and unknown risks, uncertainties, and other factors, including those discussed under Item 1A. "Risk Factors" in our most recently filed Form 10-K filed with the Securities and Exchange Commission ("SEC") and updated from time to time in our Form 10-Q filings and in our other public filings with the SEC. Any forward-looking statements contained in this release speak only as of its date. We undertake no obligation to update any forward-looking statements contained in this release to reflect events or circumstances occurring after its date or to reflect the occurrence of unanticipated events. The Company cautions investors not to place undue reliance on the interim results announced today.

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