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Moleculin to Seek Accelerated FDA Approval and Plans for Pivotal Phase 2 AML Trial

Following Successful Completion of US Phase 1 Trial

HOUSTON, Feb. 5, 2020 /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 it intends to discuss with the FDA and EMA (European Medicines Agency) plans to conduct a single arm Phase 2 trial that would serve as the basis for accelerated approval of Liposomal Annamycin ("Annamycin") to treat relapsed or refractory acute myeloid leukemia ("AML"). This will follow the establishment of a recommended Phase 2 dose ("RP2D") in the Company's ongoing Phase 1 dose escalation trial in Europe. The FDA has already granted Annamycin Fast Track status and Orphan Drug Designation for AML. FDA grants Fast Track designation to drugs intended to treat serious conditions that demonstrate the potential to address unmet medical needs, which can include providing efficacy comparable to available therapy while avoiding toxicity associated with the existing treatment. The benefits of Fast Track include FDA actions to expedite development and review, including "rolling review," where the agency reviews portions of a marketing application before the complete application is submitted.

The announcement follows continuing positive results from Moleculin's open label, single arm Phase 1 trials in AML patients in Europe and the US. Most recently, the US Phase 1 study met its primary objective of demonstrating the safety of Annamycin at a dose that was cumulatively at or below the lifetime maximum anthracycline dose. Those results are consistent with results achieved with the parallel Phase 1 study being conducted in Europe, which has demonstrated the safety of escalating doses of Annamycin in AML patients, including doses that significantly exceed the maximum lifetime dose of anthracyclines imposed in the US. In both trials, the primary endpoints are aimed at demonstrating the product's safety, primarily the lack of cardiovascular risk. This is a key characteristic that, if borne out, could significantly differentiate Annamycin from other anthracyclines, which generally are well-known to have treatment-limiting cardiotoxicity.

Based on these results, Moleculin will continue to focus the Company's efforts on the

European trial to establish an RP2D. Once that is complete, the Company intends to enter discussions with the FDA and EMA about conducting a single arm Phase 2 study that would be the pivotal trial supporting US and European approval of Annamycin for relapsed or refractory AML.

Walter Klemp, Chairman and CEO of Moleculin commented, "We believe relying upon the European trial to establish an RP2D is the fastest and most efficient way to reach a pivotal Phase 2 trial. Recruitment in Europe has been faster than in the US and the trial is progressing well. The US Phase 1 trial was designed to demonstrate that Annamycin is indeed non-cardiotoxic when delivered to patients at or below the lifetime maximum anthracycline dose, and it has served that purpose. Beyond that, we have now treated 9 patients in the European trial above the lifetime maximum, also without any evidence of cardiotoxicity."

The U.S. trial met its primary endpoint, demonstrating the safety of Annamycin in AML patients, most importantly the absence of cardiotoxicity (potential damage to the heart), as determined by echocardiograms, as well as cardiac health biomarkers, principally blood troponin levels. Based on testing to date, no patients in either the US or European trial have exhibited evidence of cardiotoxicity. Additionally, there were no unexpected serious adverse events (SAE) and no dose limiting toxicities (DLT) at any dose tested. Although a primary objective of the Phase 1 trial was to evaluate safety, the study also gathered data to support a preliminary assessment of the product's efficacy. Among other things, the study recorded complete response (CR), partial response (PR), event-free survival (EFS), overall survival (OS; Kaplan-Meier), and time to and duration of remission/response. The Company reported efficacy in 33% of the US patients, even though the drug was dosed at what was expected to be sub-therapeutic levels. The evidence of efficacy consisted of 1 patient who achieved a "morphologically leukemia-free state," which the protocol defined as a CR with incomplete recovery of platelets or neutrophils, and another patient who had a substantial remission of leukemia cutis (a somewhat rare leukemia symptom), from diffuse to 3 lesions.

Dr. Rob Shepard, Chief Medical Officer – Annamycin, added, "Because of this early success in the US trial and our continued progress in the parallel European trial, and given our new Fast Track status, we have made a strategic decision on how best to move forward. Once we have established an RP2D with the European trial, we intend to discuss with the FDA conducting a Phase 2 single arm registration trial that would form the basis for accelerated approval. This should allow us to combine efforts between the U.S. and Europe, creating one global Phase 2 trial. The European trial continues with its dose escalation, and although dosing had now taken patients well beyond the lifetime maximum anthracycline limit with no evidence of cardiotoxicity, we are still below what we believe to be a therapeutic dose. We look forward to reaching a therapeutic dose and a recommended Phase 2 dose in the coming quarters."

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, under investigation for brain tumors, pancreatic cancer and hematologic malignancies; and WP1220, an analog to WP1066, being developed 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, the ability of Annamycin to show safety and efficacy in patients, and the ability for Annamycin to be an alternative to currently approved anthracyclines for treating cancers other than AML. 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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