April 2, 2020

Moleculin Announces Additional Positive Safety Data in EU AML Trial

A Total of 19 Patients Now Have Shown No Signs of Cardiotoxicity

HOUSTON, April 2, 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 has completed the latest (210 mg/m^2) cohort in its European open label, single arm Phase 1/2 clinical trial of Annamycin for the treatment of relapsed or refractory acute myeloid leukemia ("AML"). A total of 19 patients have been treated in the US and Europe, and all results continue to show Annamycin to be safe, and, especially, all have shown Annamycin to be free of cardiotoxicity. Of those, 10 have been treated at or above the FDA lifetime maximum anthracycline exposure.

The Phase 1 portion of this clinical trial, which is 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 trial. 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 Company cautions not to place undue reliance on interim results.

The fourth cohort in Poland receiving a single dose of 210 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 240 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 Company's recently completed Phase 1 portion of a parallel US Phase 1/2 clinical trial, there were no unexpected serious adverse events (SAE) and no dose limiting toxicities (DLT) at any dose tested.

We refer to Annamycin as a "next generation anthracycline," because it is designed to provide enhanced therapeutic benefits when compared with traditional anthracyclines 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 19 patients treated thus far in both trials, none has shown any evidence of cardiotoxicity. This includes 10 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, we believe this lack of toxicity would be an important differentiator between Annamycin and the currently approved anthracyclines, for which cardiotoxicity is a well-known treatment limitation.

Walter Klemp, Chairman and CEO of Moleculin commented, "Now that we are relying upon the European trial to establish an RP2D, and now that it is becoming apparent that the safety profile of Annamycin is even better than we expected, we will explore opportunities to increase the dosing increment between cohorts to speed up the establishment of the Recommended Phase 2 Dose or RP2D. We continue to target establishing the RP2D before the end of the year, but of course this will depend upon the continued rate of recruitment and the actual point at which we begin to see dose limiting toxicities."

The U.S. trial met its primary endpoint, demonstrating the safety of Annamycin in AML patients. Most importantly, all patients reflected 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.

In all but one of the 7 relapsed patients treated in our Poland trial, a reduction in blasts in the bone marrow aspirate occurred. Of those 7, 2 qualified as PRs and 1 qualified as a bridge-to-transplant. In the last cohort, only 1 patient was relapsed, and that patient had a 33% reduction in bone marrow blasts. In all three patients in the 210 mg/m2 cohort (1 relapsed and 2 refractory), a reduction of circulating blasts to <2% was achieved during treatment.

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.

Contacts

James Salierno / Carol Ruth
The Ruth Group
646-536-7028 / 7000
jsalierno@theruthgroup.com
cruth@theruthgroup.com

View original content to download multimedia:http://www.prnewswire.com/news-releases/moleculin-announces-additional-positive-safety-data-in-eu-aml-trial-301033941.html

SOURCE Moleculin Biotech, Inc.