October 18, 2021

Moleculin Announces Interim Data in Phase 1b/2 Clinical Trial of Annamycin for the Treatment of Soft Tissue Sarcoma Lung Metastases

- Preliminary data from two cohorts evaluating Annamycin for the treatment of soft tissue sarcoma lung metastases currently demonstrate 80% clinical activity, defined as stable disease and/or better

- Patient enrollment and dosing ongoing; no dose-limiting toxicity (DLT) experienced to-date

- Annamycin has Fast Track Status and Orphan Drug Designation from FDA for the treatment of soft tissue sarcoma lung metastases

HOUSTON, Oct. 18, 2021 /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 and viruses, today reported interim results from its U.S. Phase 1b/2 clinical trial evaluating Annamycin for the treatment of soft tissue sarcoma (STS) lung metastases, which documented preliminary clinical activity for Annamycin.

The Phase 1b/2 study is a U.S. multi-center, open-label, single-arm study that in Phase 1b will determine the maximum tolerated dose (MTD) or the recommended Phase 2 dose (RP2D) and safety of Annamycin. The Phase 2 portion of the study will explore the efficacy of Annamycin as a single agent for the treatment of subjects with STS with lung metastases, who have failed prior chemotherapy, and for whom new chemotherapy is considered appropriate. A minimum of 3 subjects will be enrolled in each of up to 6 cohorts of the Phase 1b portion of the study, or until an MTD is identified, whichever comes first. A maximum of 25 subjects will be enrolled at the RP2D to further evaluate efficacy.

"To witness the activity of Annamycin in the treatment of STS lung metastases, even this early in a Phase 1 trial, we believe is encouraging. Four of the five patients that have

completed scans to date demonstrated a response to treatment, including three with extended and, in one case, continuing stable disease and one patient with a substantial (>30%) reduction in tumor size. These interim data bolster our optimism about the potential for Annamycin to address the limitations with the current standard-of-care treatment options for STS lung metastases. Ultimately, we believe Annamycin has the potential to bring a new and effective treatment option to patients with a significant unmet need. With these data now in hand, we are cautiously optimistic as we begin patient enrollment and dosing in the next cohort," commented Walter Klemp, Chairman and CEO of Moleculin.

Mr. Klemp added, "We are also encouraged by the pace of recruitment so far in this trial. To have completed 2 full cohorts in just the first 4 months of the study with only one site open is faster than we would have expected, especially for a rare disease like STS lung metastases. And, with more sites joining the study before the end of the year, we believe there is the potential for this pace of recruitment to continue or even accelerate."

"As with all of the patients treated so far in our acute myeloid leukemia trials, no patients in this STS trial have exhibited any signs of cardiotoxicity," Mr. Klemp concluded. "This is an important point because, even though anthracyclines are considered a cornerstone chemotherapy for many types of cancer, including STS lung metastases, all currently approved anthracyclines are significantly cardiotoxic. Annamycin was designed to overcome this problem and we believe it has the potential to become the first non-cardiotoxic anthracycline approved for use. This could not only reduce the risk of many current anthracycline treatment regimens, but it could also enable longer treatment periods without cardiac risk."

The summary of interim data from the first two cohorts of the study are as follows:

First Cohort:

- One **s**ubject is ongoing and is currently in cycle 5 of treatment and expects to enter cycle 6 of treatment at the end of this month. The end of cycle 4 scan revealed stable disease.
- One subject discontinued after 6 cycles (4.5 months) due to progressive disease. However, stable disease was maintained up to that point.
- One subject discontinued after cycle 1. The end of study scan was performed, and stable disease was observed. The subject was discontinued from the study because cycle 2 treatment was delayed greater than 6 weeks from the prior dosing.

Second Cohort:

- One subject is ongoing and is currently in cycle 3 of treatment. The end of cycle 2 scan revealed partial response (PR, >30% reduction in tumor size).
- One subject was discontinued from the study after 2 cycles after the end of cycle scan revealed progressive disease.
- One subject received one cycle of treatment to-date and discontinued treatment for reasons unrelated to Annamycin. The end of study scans are scheduled in the coming weeks.

These are interim data and should be considered preliminary and subject to change.

The Company has now opened enrollment in the third cohort of the Phase 1b portion of the study. Three subjects minimum (6 maximum) for each dosing cohort will be enrolled until a maximum tolerated dose is identified. Therefore, up to 36 subjects may be enrolled in the Phase 1b portion of the study.

Annamycin currently has Fast Track Status and Orphan Drug Designation from the U.S. Food and Drug Administration for the treatment of STS lung metastases, in addition to Orphan Drug Designation for the treatment of relapsed or refractory acute myeloid leukemia. For more information about the Phase 1b/2 study evaluating Annamycin for the treatment of STS lung metastases, please visit clinicaltrials.gov and reference identifier NCT04887298.

Study Design

In Phase 1b, Annamycin is administered as an intravenous (IV) infusion over 2 hours on Day 1, followed by 20 days off (1 cycle = 21 days). Subjects visit the study site every 21 days (±3 days) at which time safety monitoring, including adverse events (AEs), a physical examination, laboratory evaluations (clinical chemistry, complete blood count), vital signs, weight measurements, Eastern Cooperative Oncology Group (ECOG) performance status, and electrocardiograms (ECGs) will be performed, followed by an IV infusion of study drug. Additional laboratory evaluations (clinical chemistry, complete blood count) will be performed at 7 days (± 3 days) and 14 days (± 3 days) from the Annamycin infusion for additional safety monitoring during each cycle. Cardiac function will be followed by echocardiogram (ECHO) scans at screening, at the end of the first two cycles and then every other cycle thereafter, at the end of treatment visit, and if feasible, during follow up at 6 months (±1 month) and 1 year (±1 month) after study drug discontinuation. As long as the Investigator considers that the benefits of treatment with Annamycin continue to outweigh the risks, treatment will continue every 21 days until tumor progression is observed or unacceptable toxicity occurs.

Tumor response is monitored every 6 weeks (±1 week) from Cycle 1 Day 1 during treatment, at the End of Treatment visit, and then every 3 months (±1 month) until disease progression using RECIST criteria. Those subjects who leave the study after a maximum response is achieved and who do not start another therapy will be followed every 3 months (±1 month) for progression-free survival (PFS). If a subject receives further therapy after discontinuing from the study, they will only be followed for overall survival (OS) and if feasible, follow-up ECHO scans at 6 months (±1 month) and 1 year (±1 month) after study drug discontinuation.

About Annamycin

Annamycin is the Company's next-generation anthracycline that has been shown in animal models to accumulate in the lungs at up to 30-fold the level of doxorubicin. Importantly, Annamycin has also demonstrated a lack of cardiotoxicity in recently conducted human clinical trials for the treatment of acute myeloid leukemia (AML), and the Company believes that the use of Annamycin may not face the same usage limitations imposed on doxorubicin. Annamycin is currently in development for the treatment of AML and STS lung metastases.

About Moleculin Biotech, Inc.

Moleculin Biotech, Inc. is a clinical stage pharmaceutical company focused on the development of a broad portfolio of drug candidates for the treatment of highly resistant

tumors and viruses. The Company's lead program, Annamycin is a next-generation anthracycline designed to avoid multidrug resistance mechanisms with little to no cardiotoxicity. Annamycin is currently in development for the treatment of relapsed or refractory acute myeloid leukemia (AML) and soft tissue sarcoma (STS) lung metastases.

Additionally, the Company is developing 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 and other cancers, and WP1220, an analog to WP1066, for the topical treatment of cutaneous T-cell lymphoma. Moleculin is also engaged in the development of a portfolio of antimetabolites, including WP1122 for the potential treatment of COVID-19 and other viruses, as well as cancer indications including brain tumors, pancreatic and other cancers.

For more information about the Company, please visitwww.moleculin.com and connect on [Twitter](), [LinkedIn]() and [Facebook]().

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 Annamycin to demonstrate safety and efficacy in patients, and the ability of the clinical trial to accelerate or continue the recruitment of 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 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.

Investor Contact:
JTC Team, LLC
Jenene Thomas
(833) 475-8247
MBRX@jtcir.com

View original content to download multimedia:https://www.prnewswire.com/news-releases/moleculin-announces-interim-data-in-phase-1b2-clinical-trial-of-annamycin-for-the-treatment-of-soft-tissue-sarcoma-lung-metastases-301401833.html

SOURCE Moleculin Biotech, Inc.