September 13, 2018

# Moleculin's Brain Cancer Drug Candidate Begins Patient Dosing at Clinical Trial Being Conducted at MD Anderson

***Small molecule lead drug candidate blocks a critical target for tumors and crosses the blood brain barrier; begins first brain cancer patient dosing in clinical trial at MD Anderson Cancer Center***

HOUSTON, Sept. 13, 2018 (GLOBE NEWSWIRE) -- In the ongoing challenge to combat the almost always deadly brain cancers, namely Glioblastoma and melanoma metastasized to the brain, the pharmaceutical company Moleculin Biotech, Inc., (Nasdaq: MBRX) has initiated a Phase 1 clinical trial of a new first-in-class cancer drug candidate, a small molecule compound discovered by Prof. Waldemar Priebe at The University of Texas MD Anderson Cancer Center and known as WP1066. The compound has been shown in animal models to both inhibit an important cell signaling protein STAT3 that is involved in cell growth and proliferation and considered critical to tumor development, while also stimulating an immune response. The first glioblastoma patient has received the initial doses of WP1066, which were apparently well tolerated, in the physician-sponsored IND (investigational new drug) study at MD Anderson Cancer Center.

Built from the chemical backbone of the active ingredient in propolis, a natural product of honey bees, WP1066 is the first anticancer agent with drug-like properties that consistently inhibits the activated form of STAT3 within cancer cells, a target that has been long-sought because of its broad range of tumor promoting effects.

Importantly, activated STAT3 supports the survival and proliferation of tumor cells, evasion of the immune response and metastasis to distant organs, as well as angiogenesis (growth of blood vessels) essential for tumor growth. Activated STAT3 is not only connected with directly supporting tumor activity, but also suppressing the immune system, making this target even more important to cancer therapy.

With the support of extensive preclinical studies demonstrating high antitumor activity and the critically important ability to cross the blood-brain barrier, WP1066 in this Phase 1 clinical trial will focus on treating aggressive brain tumors which all share a grim prognosis. The intent is to eventually treat up to 15 relapsed brain cancer patients over the next six to eight months. Phase 1 clinical trials typically focus on exploring safe and well tolerated doses, as well as evaluating initial signals of effectiveness. Each treatment is completed over three weeks.

"Treating the first brain tumor patient with WP1066 is the start of a very exciting and encouraging program for doctors treating the worst types of brain cancers. There has been very little progress in recent years toward improved therapies for glioblastoma and other

aggressive primary or metastatic brain tumors. WP1066 has shown extremely promising results based on animal studies where we have seen inhibition of tumor growth and improvements in survival," said Dr. Sandra Silberman, a world-renowned oncologist and Moleculin's Chief Medical Officer. "This is based on the fact that although STAT3 has long been identified as an important target for treating tumors, for years most efforts have focused on attempts to indirectly inhibit STAT3 from upstream signaling, not from within the cancer cell itself. WP1066 appears to be unique in its ability in vitro and in animal models to consistently and directly inhibit the activated form of STAT3 and produce significant anticancer effects, including tumor growth inhibition and increased life span of treated animals."

"This represents a major milestone for Moleculin," commented Walter Klemp, Chairman and CEO. "There has been tremendous enthusiasm within the oncology community for targeting STAT3, a key molecular hub of multiple pathways promoting tumor growth. Although the industry has been struggling to find a way to target STAT3, we at Moleculin believe that most of these efforts have been mechanistically misguided and ended in failure because their approach would ultimately be ineffective at adequately blocking the activation of STAT3 and lack the necessary drug-like properties to succeed. The opportunity to test a unique STAT3 therapy in these patients is significant in supporting Moleculin's mission to provide benefit for those who need new and better treatments."

**How WP1066 Works in Tumor Cells**

WP1066 is a small molecule compound that can not only directly kill tumor cells, but also has the ability to overcome the tumor's ability to evade the natural immune response, which would otherwise be working to eliminate the cancerous activity. This compound is a first in class drug candidate capable of down-regulating the activated form of STAT3, a target that has been long-sought because of its role in supporting the survival and growth of tumor cells.

The compound has been shown to prevent tumor progression and increase survival in a wide range of animal models by directly attacking tumors and blocking the cell signaling by STAT3 that supports tumor development and simultaneously suppressing regulatory T cells (Tregs), which then allows stimulation of an enhanced natural anti-tumor immune response. The compound's dual functions have been shown to increase survival in a wide range of animal models, which have been documented in more than 50 peer-reviewed articles.

**About Moleculin Biotech, Inc.**

Moleculin Biotech, Inc. is a clinical stage pharmaceutical company focused on the development of oncology drug candidates, all of which are based on discoveries made at M.D. Anderson Cancer Center. Our clinical stage drugs are Annamycin, an 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, and WP1066, an immuno-stimulating STAT3 inhibitor targeting primary brain tumors and brain metastases, pancreatic cancer and hematological malignancies. We are also engaged in preclinical development of additional drug candidates, including additional STAT3 inhibitors and compounds targeting the metabolism of tumors.

For more information about the Company, please visit http://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 WP1066 to show activity in brain tumor patients and the ability to enroll and treat patients within the time period discussed. These statements relate to future events, future expectations, plans and prospects. Although Moleculin Biotech 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.

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Source: Moleculin Biotech, Inc.