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New Publication Finds Combination of WP1066 and Radiation Resulted in Long-Term Survival in Human Brain Tumor Mouse Model

Findings published in Clinical Cancer Research

HOUSTON, July 1, 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 and viruses, today announced that a peer-reviewed article published in Clinical Cancer Research (Clin Cancer Res June 30 2020 DOI:10.1158/1078-0432.CCR-19-4092) reported findings that Moleculin's STAT3 inhibitor, WP1066, used in combination with traditional whole brain radiation therapy (WBRT) resulted in long-term survivors and enhanced median survival time relative to monotherapy in mice with implanted human brain tumors.

The paper can be accessed at: https://clincancerres.aacrjournals.org/content/early/2020/06/30/1078-0432.CCR-19-4092.full-text.pdf

The study was performed by lead author Martina Ott, Ph.D., Instructor of Neurosurgery, senior author Amy Heimberger, M.D., professor of Neurosurgery, and a team of researchers at The University of Texas MD Anderson Cancer Center. Heimberger also is the Principle Investigator of the current investigator-initiated clinical trial of WP1066 for brain tumors.

In the current study, C57BL/6 mice underwent intracerebral implantation of GL261 glioma cells, WBRT, and treatment with WP1066, a blood-brain barrier penetrant inhibitor of the STAT3 pathway, or the two in combination. The role of the immune system was evaluated using tumor rechallenge strategies, immune incompetent backgrounds, immunofluorescence, immune phenotyping of tumor-infiltrating immune cells (via flow cytometry), and nanostring gene expression analysis of 770 immune-related genes from immune cells, including those directly isolated from the tumor microenvironment.

The combination of WP1066 and WBRT resulted in long-term survivors and enhanced median survival time relative to monotherapy in the GL261 glioma model (combination vs.

control p<0.0001). Immunological memory appeared to be induced, because mice were protected during subsequent tumor rechallenge. The therapeutic effect of the combination was completely lost in immune incompetent animals. Nanostring analysis and immunofluorescence revealed immunological reprogramming in the brain tumor microenvironment specifically affecting dendritic-cell antigen presentation and T cell effector functions. The study indicates that the combination of STAT3 inhibition and WBRT enhances the therapeutic effect against gliomas in the CNS by inducing dendritic-cell and T cell interactions in the brain tumor, which seems to be a requirement for a fully functional immune response

"This impressive study confirms preliminary data we announced last year," commented Walter Klemp, Chairman and CEO of Moleculin. "Importantly, the study indicated that the combination of STAT3 inhibition with whole brain radiotherapy had the capacity to enhance the therapeutic effect against established tumors as well as developing immune memory that appears to prevent recurrence."

The research was supported by the Cancer Prevention & Research Institute of Texas (IIRA-RP160482), the National Institutes of Health (CA1208113, P50 CA093459, P50 CA127001 and P30 CA016672), the Ben and Catherine Ivy Foundation, MD Anderson's Glioblastoma Moon Shot, and the Brockman Foundation.

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 and viruses. 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, being studied for brain tumors, pancreatic cancer and hematologic malignancies; and WP1220, an analog to WP1066, being studied 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, such as WP1122. Moleculin has the exclusive worldwide rights (subject to certain territories for which it has issued sublicenses) to all of the above technologies.

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 WP1066, alone or in combination with WBRT, to show safety and efficacy in patients with brain tumors. 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.

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