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FDA Grants Orphan Drug Designation to MAIA Biotechnology for THIO as a Treatment for Glioblastoma

- Third orphan drug designation (ODD) granted to THIO by the FDA; drug also holds ODDs for hepatocellular carcinoma and small cell lung cancer
- Benefits include 7 years of U.S. market exclusivity after drug approval and tax credits for qualified clinical testing
- Expected glioblastoma market growth from $2.2 billion to $3.2 billion globally in the next three years

CHICAGO--(BUSINESS WIRE)-- MAIA Biotechnology, Inc., (NYSE American: MAIA) ("MAIA" or the "Company"), a clinical-stage biopharmaceutical company developing telomere-targeting immunotherapies for cancer, announced today that the U.S. Food and Drug Administration ("FDA") has granted orphan drug designation to its lead asset THIO, a cancer telomere-targeting agent, for the treatment of glioblastoma. This is the third orphan drug designation granted to THIO, following the receipt of orphan drug designations for hepatocellular carcinoma (HCC) and small cell lung cancer (SCLC) in 2022.

"We are pleased to receive a third orphan drug designation for THIO, further highlighting FDA's recognition of THIO's potential in the treatment of multiple cancer indications, including rare ones such as glioblastoma," said Vlad Vitoc, M.D., MAIA's Chairman and Chief Executive Officer. "Each year, globally, more than 300,000 people are diagnosed with brain tumors, of which, 25,000 are in the United States. Glioblastoma represents the majority of these cases in the U.S., with 15,000 new patients diagnosed and more than 10,000 deaths yearly, making it an orphan indication. Given this prevalence there is significant room for growth in the $2.2 billion glioblastoma market, which is expected to reach $3.2 billion globally in the next three years.[1] We consider this ODD an important milestone for our development strategy and for glioblastoma patients who could benefit from a potentially revolutionary therapy."

"In the data presented to the FDA, THIO successfully penetrated the blood brain barrier (BBB) in syngeneic and humanized mouse models of telomerase-expressing brain cancers. Treatment with THIO resulted in potent anticancer activity and significant expansion of the animal lifespan for several difficult to treat cell lines and xenograft mouse models," added Sergei Gryaznov, Ph.D., MAIA's Chief Scientific Officer. "These results stem from THIO's remarkable mechanism of action and its BBB penetrating property that allows for direct targeting of brain tumors in vivo and potentially in glioblastoma patients."

"Glioblastoma is the most aggressive and most common type of cancer that originates in the brain. With very limited treatment options available, glioblastoma patients have exceptionally

short survival durations, and only 7% remain alive five years after being diagnosed with the condition,"[2] said Mihail Obrocea, MD, MAIA's Chief Medical Officer. "We are optimistic about our telomere-targeting agent's ability to provide clinical benefit in patients with glioblastoma, and we look forward to studying THIO for the treatment of this highly unmet medical indication in a future trial."

Enrollment is ongoing in a Phase 2 trial of THIO, THIO-101, evaluating the drug candidate in patients with advanced non-small cell lung cancer (NSCLC). THIO is the only direct telomere targeting agent currently in clinical development.

About Orphan Drug Designation

The FDA's Orphan Drug Act of 1983 was designed to incentivize the development of therapies that demonstrate promise for the treatment of rare (orphan) diseases or conditions. A disease is classified as "rare" if it affects fewer than 200,000 people total in the U.S., or if the cost of developing a drug and making it available in the U.S. for such diseases will exceed any potential profits from its sale due to the small target population size. The FDA's ODD program provides multiple incentives to make orphan drug development more financially possible for companies to pursue, such as up to seven years of market exclusivity for the approved orphan drug, up to 20 years of 25% federal tax credit for expenses incurred in conducting clinical research within the U.S. and waiver of Prescription Drug User Fee Act (PDUFA) fees for orphan drugs, a value of approximately $2.9 million in 2021.

About THIO

THIO (6-thio-dG or 6-thio-2'-deoxyguanosine) is a first-in-class investigational telomere-targeting agent currently in clinical development to evaluate its activity in Non-Small Cell Lung Cancer (NSCLC). Telomeres, along with the enzyme telomerase, play a fundamental role in the survival of cancer cells and their resistance to current therapies. The modified nucleotide 6-thio-2'-deoxyguanosine (THIO) induces telomerase-dependent telomeric DNA modification, DNA damage responses, and selective cancer cell death. THIO-damaged telomeric fragments accumulate in cytosolic micronuclei and activates both innate (cGAS/STING) and adaptive (T-cell) immune responses. The sequential treatment with THIO followed by PD-(L)1 inhibitors resulted in profound and persistent tumor regression in advanced, in vivo cancer models by induction of cancer type–specific immune memory. THIO is presently developed as a second or later line of treatment for NSCLC for patients that have progressed beyond the standard-of-care regimen of existing checkpoint inhibitors.

About THIO-101, a Phase 2 Clinical Trial

THIO-101 is a multicenter, open-label, dose finding Phase 2 clinical trial. It is the first trial designed to evaluate THIO's anti-tumor activity when followed by PD-(L)1 inhibition. The trial is testing the hypothesis that low doses of THIO administered prior to an anti-PD1 agent will enhance and prolong immune response in patients with advanced NSCLC who previously did not respond or developed resistance and progressed after first-line treatment regimen containing another checkpoint inhibitor. The trial design has two primary objectives: (1) to evaluate the safety and tolerability of THIO administered as an anticancer compound and a priming immune activator (2) to assess the clinical efficacy of THIO using Overall Response Rate (ORR) as the primary clinical endpoint. For more information on this Phase II trial, please visit ClinicalTrials.gov using the identifier NCT05208944.

About MAIA Biotechnology, Inc.

MAIA is a targeted therapy, immuno-oncology company focused on the development and commercialization of potential first-in-class drugs with novel mechanisms of action that are intended to meaningfully improve and extend the lives of people with cancer. Our lead program is THIO, a potential first-in-class cancer telomere targeting agent in clinical development for the treatment of NSCLC patients with telomerase-positive cancer cells. For more information, please visit www.maiabiotech.com.

Forward Looking Statements

MAIA cautions that all statements, other than statements of historical facts contained in this press release, are forward-looking statements. Forward-looking statements are subject to known and unknown risks, uncertainties, and other factors that may cause our or our industry's actual results, levels or activity, performance or achievements to be materially different from those anticipated by such statements. The use of words such as "may," "might," "will," "should," "could," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward looking statements. However, the absence of these words does not mean that statements are not forward-looking. For example, all statements we make regarding (i) the initiation, timing, cost, progress and results of our preclinical and clinical studies and our research and development programs, (ii) our ability to advance product candidates into, and successfully complete, clinical studies, (iii) the timing or likelihood of regulatory filings and approvals, (iv) our ability to develop, manufacture and commercialize our product candidates and to improve the manufacturing process, (v) the rate and degree of market acceptance of our product candidates, (vi) the size and growth potential of the markets for our product candidates and our ability to serve those markets, and (vii) our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates, are forward looking. All forward-looking statements are based on current estimates, assumptions and expectations by our management that, although we believe to be reasonable, are inherently uncertain. Any forward-looking statement expressing an expectation or belief as to future events is expressed in good faith and believed to be reasonable at the time such forward-looking statement is made. However, these statements are not guarantees of future events and are subject to risks and uncertainties and other factors beyond our control that may cause actual results to differ materially from those expressed in any forward-looking statement. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law. In this release, unless the context requires otherwise, "MAIA," "Company," "we," "our," and "us" refers to MAIA Biotechnology, Inc. and its subsidiaries.

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[1] Market size and forecast from the Business Research Company

[2] National Brain Tumor Society, About Glioblastoma

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