Aadi Bioscience Announces Publication of its Phase 2 Registrational (AMPECT) Trial of nab-Sirolimus in Patients with Malignant Perivascular Epithelioid Cell Tumors in the Journal of Clinical Oncology

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Results Published from First Clinical Trial in this Rare Disease

LOS ANGELES, Oct. 22, 2021 (GLOBE NEWSWIRE) -- Aadi Bioscience, Inc. ("Aadi") (Nasdaq: AADI), a clinical-stage biopharmaceutical company focusing on precision therapies for genetically-defined cancers with alterations in mTOR pathway genes, today announced the publication of "*nab*-Sirolimus for Patients with Malignant Perivascular Epithelioid Cell Tumors", detailing its AMPECT study of investigational ABI-009 in the American Society of Clinical Oncology's *Journal of Clinical Oncology*. The authors concluded that investigational *nab*-sirolimus (ABI-009, formerly known as *nab*-rapamycin), if approved, may represent an important new treatment option in malignant PEComa, a rare cancer and aggressive form of sarcoma, with no currently approved treatment.

The content of the publication included the primary analysis, which occurred 6 months after the last patient on the AMPECT study initiated treatment, as well as a 1.5-year follow-up after the primary analysis with a data cutoff of November 23, 2020. In the trial, 34 patients were treated with ABI-009. In the 31 evaluable patients, the overall independently assessed response rate was 39% (12 of 31; with a 95% confidence interval, 22% to 58%) with 1 complete and 11 partial responses. In addition, 52% (16 of 31) of patients had stable disease. The responses were durable and median duration of response was not reached after a median follow-up for response of 2.5 years, and 7 of 12 responders with treatment ongoing (range 5.6-47.2+ months). Responses were of rapid onset (67% by week 6). The safety profile was found to be acceptable with most treatment-related adverse events characterized as low grade and manageable for long-term treatment.

Andrew Wagner, M.D., Ph.D., a senior oncologist at the Dana-Farber Cancer Institute and Principal Investigator of the study, said, "We are excited to publish the data obtained from this trial. *nab*-Sirolimus delivered highly durable responses in patients with advanced PEComa. Most of the responding patients rapidly achieved a response by their first assessment at 6 weeks following initiation of therapy and these patients have stayed on therapy for extended periods with an acceptable safety profile. We are encouraged by the outcomes in this first-ever prospective clinical trial in patients with this extremely rare disease".

Neil Desai, Ph.D., co-author of the article and Founder, Chief Executive Officer and President of Aadi Bioscience, said, "The Aadi Bioscience team is extremely grateful to the patients, families, and clinical trial teams who help expand the boundaries of available care through their participation in clinical trials. The publication of these results is an important milestone not only for malignant PEComa patients but also for the ongoing development of *nab*-sirolimus across the planned investigation in *TSC1* and *TSC2* indications and other combination strategies."

A New Drug Application for investigational ABI-009 is under review by the U.S. Food and Drug Administration for treatment of patients with PEComa and has a Prescription Drug User Fee Action target date of November 26, 2021. If approved, ABI-009 will be known as FYARRO.

About Aadi Bioscience and FYARRO™

Aadi is a clinical-stage biopharmaceutical company developing precision therapies for genetically-defined cancers. Aadi's primary goal is to bring transformational therapies to cancer patients with mTOR pathway driver alterations such as alterations in *TSC1* or *TSC2* genes, where other mTOR inhibitors have not or cannot be effectively exploited due to problems of pharmacology, effective drug delivery, safety, or effective targeting to the disease site. Aadi's lead product candidate is FYARRO ™ (sirolimus albumin-bound nanoparticles for injectable suspension; *nab*-sirolimus; ABI-009), an mTOR inhibitor bound to human albumin that has demonstrated significantly higher tumor accumulation, greater mTOR target suppression, and increased tumor growth inhibition over other mTOR inhibitors in preclinical models[1].

Aadi's registration trial of FYARRO in advanced malignant PEComa (the "AMPECT trial") demonstrated meaningful clinical efficacy in malignant PEComa[2], a type of cancer with the highest known alteration rate of *TSC1* or *TSC2* genes. FYARRO has received Breakthrough Therapy, Fast-Track and Orphan Designations from the U.S. Food and Drug Administration (FDA). A rolling New Drug Application (NDA) submission was completed in May 2021 for this indication and the FDA accepted the NDA in July 2021 and granted Aadi Priority Review status with a Prescription Drug User Fee Act ("PDUFA") target action date of November 26, 2021.

Based on the AMPECT trial and emerging data for FYARRO in other solid tumors with *TSC1* or *TSC2* inactivating alterations[3], and following discussions with the FDA, Aadi plans to initiate a tumor-agnostic registrational trial in mTOR inhibitor-naïve solid tumors harboring *TSC1* or *TSC2* inactivating alterations by the end of 2021. Aadi also has ongoing studies to evaluate dosing of FYARRO in combination regimens. FYARRO is an investigational drug that has not been approved by the FDA for commercial distribution in the United States. More information is available on the Aadi website at www.aadibio.com.

Forward-Looking Statements

Aadi Bioscience, Inc. (“Aadi”, “The Company”) cautions you that certain statements included in this press release that are not a description of historical facts are forward-looking statements. These statements are based on Aadi’s current beliefs and expectations. Forward-looking statements include statements regarding: FYARRO, including expectations regarding the clinical responses and safety profile, regulatory approval and commercialization, and the timing of the initiation of additional clinical trials. Actual results could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation: risks related to Aadi’s ability to obtain, or the timeline to obtain, regulatory approval from the FDA and other regulatory authorities for FYARRO in advanced malignant PEComa; risks related to Aadi’s ability to successfully commercialize, including the timing of a commercial launch of FYARRO in advanced malignant PEComa; uncertainties associated with the clinical development and regulatory approval of FYARRO, including potential delays in the commencement, enrollment and completion of clinical trials; the risk that interim results of clinical trials may not be reproduced and do not necessarily predict final results; the risk that one or more of the clinical outcomes may materially change as patient enrollment continues, following more comprehensive reviews of the data, and as more patient data become available; the risk that unforeseen adverse reactions or side effects may occur in the course of developing and testing FYARRO; risks associated with the failure to realize any value from FYARRO in light of inherent risks and difficulties involved in successfully bringing product candidates to market; risks related to Aadi’s estimates regarding future expenses, capital requirements and need for additional financing; and risks related to the impact of the COVID-19 outbreak on Aadi’s operations, the biotechnology industry and the economy generally.

Additional risks and uncertainties that could cause actual outcomes and results to differ materially from those contemplated by the forward-looking statements are included under the caption "Risk Factors" and elsewhere in Aadi’s reports and other documents that Aadi has filed, or will file, with the SEC from time to time and available at www.sec.gov.

All forward-looking statements in this press release are current only as of the date hereof and, except as required by applicable law, Aadi undertakes no obligation to revise or update any forward-looking statement, or to make any other forward-looking statements, whether as a result of new information, future events or otherwise. All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

FYARRO™ is a trademark of Aadi Bioscience, Inc.

References:

[1] AACR-NCI-EORTC Molecular Targets and Cancer Therapeutics 2021 Abstract: https://aadibio.com/wp-content/uploads/2021/10/ABI-009-Poster-091321-Final-Oral.pdf

[2] ASCO 2020 Abstract: https://ascopubs.org/doi/abs/10.1200/JCO.2020.38.15_suppl.11516?af=R

[3] ASCO 2021 Abstract: https://meetings.asco.org/abstracts-presentations/197602

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