Ayala Pharmaceuticals Announces Publication Highlighting Clinical Activity of its Gamma Secretase Inhibitor AL101 in Desmoid Tumors

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REHOVOT, Israel and WILMINGTON, Del., Sept. 23, 2021 (GLOBE NEWSWIRE) -- Ayala Pharmaceuticals, Inc. (NASDAQ: AYLA), a clinical-stage oncology company focused on developing and commercializing small molecule therapeutics for patients suffering from rare and aggressive cancers, today announced the publication of two case studies of adult patients with desmoid tumors treated with AL101 in *Current Oncology*. This publication highlights the potential of a gamma secretase inhibitor for the treatment of desmoid tumors.

The data included in the case study are based on earlier Phase 1 results and compassionate use of AL101 in desmoid tumors. Both patients showcased in these case studies, Case One and Case Two, presented with significant tumor burden and symptomatic and life-threatening disease due to disease bulk and location. Both patients achieved long-lasting partial responses (PR) with AL101 treatment with a maximal decrease in tumor size from baseline of 41% after approximately 1 year (55 weeks) of treatment in Case One, and a maximal decrease in tumor size from baseline of 60% after about 1.6 years (82 weeks) of treatment in Case Two. With continued monitoring, one patient was able to discontinue AL101 after 4.6 years of treatment, while maintaining a PR, and the other patient has maintained a PR at a reduced AL101 dose.

“Both of these patients’ case studies represent additional evidence to support the development of our gamma secretase inhibitor, AL102 for the treatment of desmoid tumors. The body of data conducted by BMS in patients with desmoid tumors implicating the role of gamma secretase inhibition furthers our hypothesis for treating desmoid tumors through AL102’s mechanism of action,” said Roni Mamluk, Ph.D., Chief Executive Officer of Ayala. “Desmoid tumors continue to be an area of high unmet medical with a significant impact to patients’ quality of life, and we are pleased to have initiated our pivotal Phase 2/3 RINGISDE trial of AL102 to potentially address this gap in the existing treatment paradigm.”

The pivotal Phase 2/3 RINGSIDE trial is designed to evaluate the efficacy, safety and tolerability of AL102 in adult and adolescent patients with desmoid tumors. Part 1 of the study is open label and will enroll up to 36 patients with progressive desmoid tumors in three study arms across three doses of AL102: 1.2 mg daily (QD), 2 mg twice weekly (QIW) and 4mg twice weekly (QIW) with initial follow up of safety, tolerability and tumor volume by MRI after 16 weeks in order to determine the optimal dose. At the end of part 1, all patients will be eligible to enroll into an open label extension study at the selected dose where long-term efficacy and safety will be monitored.

Part 2 of the study will start immediately after dose selection from part 1 and will be a double-blind placebo-controlled study enrolling up to 156 patients with progressive disease, randomized 2:1 between AL102 or placebo. The study’s primary endpoint will be progression free survival (PFS) with secondary endpoints including objective response rate (ORR), duration of response (DOR) and patient reported Quality of Life (QOL) measures.

About Desmoid Tumors

Desmoid tumors, also called aggressive fibromatosis or desmoid-type fibromatosis, are rare connective tissue tumors that typically arise in the upper and lower extremities, abdominal wall, head and neck area, mesenteric root and chest wall with the potential to arise in additional parts of the body. Desmoid tumors do not metastasize, but often aggressively infiltrate neurovascular structures and vital organs. People living with desmoid tumors are often limited in their daily life due to chronic pain, functional deficits, general decrease in their quality of life and organ dysfunction. Desmoid tumors have an annual incidence of approximately 1,700 patients in the United States and typically occur in patients between the ages of 15 and 60 years. They are most commonly diagnosed in young adults between 30-40 years of age and are more prevalent in females. Today, surgery is no longer regarded as the cornerstone treatment of desmoid tumors due to high rate of recurrence post-surgery and there are currently no FDA-approved systemic therapies for the treatment of unresectable, recurrent or progressive desmoid tumors.

About Ayala Pharmaceuticals

Ayala Pharmaceuticals, Inc. is a clinical-stage oncology company focused on developing and commercializing small molecule therapeutics for patients suffering from rare and aggressive cancers, primarily in genetically defined patient populations. Ayala’s approach is focused on predicating, identifying and addressing tumorigenic drivers of cancer through a combination of its bioinformatics platform and next-generation sequencing to deliver targeted therapies to underserved patient populations. The company has two product candidates under development, AL101 and AL102, targeting the aberrant activation of the Notch pathway with gamma secretase inhibitors to treat a variety of tumors including Adenoid Cystic Carcinoma, Triple Negative Breast Cancer (TNBC), T-cell Acute Lymphoblastic Leukemia (T-ALL), Desmoid Tumors and Multiple Myeloma (MM) (in collaboration with Novartis). AL101, has received Fast Track Designation and Orphan Drug Designation from the U.S. FDA and is currently in a Phase 2 clinical trial for patients with ACC (ACCURACY) bearing Notch activating mutations and in a Phase 2 clinical trial for patients with TNBC (TENACITY) bearing Notch activating mutations and other gene rearrangements. AL102 is currently in a Pivotal Phase 2/3 clinical trials for patients with Desmoid Tumors (RINGSIDE) and is being evaluated in a Phase 1 clinical trial in combination with Novartis’ BMCA targeting agent, WVT078, in patients with relapsed/refractory Multiple Myeloma. For more information, visit www.ayalapharma.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including statements relating to our development of AL101, including its treatment potential, the promise and potential impact of our preclinical or clinical trial data, and the timing of additional data from clinical trials of AL101. These forward-looking statements are based on management’s current expectations. The words

“may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the following: the impact of the COVID-19 pandemic on our operations, including our preclinical studies and clinical trials, and the continuity of our business; we have incurred significant losses, are not currently profitable and may never become profitable; our need for additional funding; our cash runway; our limited operating history and the prospects for our future viability; the lengthy, expensive, and uncertain process of clinical drug development, including potential delays in regulatory approval; our requirement to pay significant payments under product candidate licenses; the approach we are taking to discover and develop product candidates and whether it will lead to marketable products; the expense, time-consuming nature and uncertainty of clinical trials; enrollment and retention of patients; potential side effects of our product candidates; our ability to develop or to collaborate with others to develop appropriate diagnostic tests; protection of our proprietary technology and the confidentiality of our trade secrets; potential lawsuits for, or claims of, infringement of third-party intellectual property or challenges to the ownership of our intellectual property; risks associated with international operations; our ability to retain key personnel and to manage our growth; the potential volatility of our common stock; costs and resources of operating as a public company; unfavorable or no analyst research or reports; and securities class action litigation against us. These and other important factors discussed under the caption “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the U.S. Securities and Exchange Commission (SEC) on March 24, 2021 and our other filings with the SEC could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. New risk factors and uncertainties may emerge from time to time, and it is not possible to predict all risk factors and uncertainties. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this press release.

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