Ayala Pharmaceuticals Announces AL102 Receives Orphan Drug Designation for Desmoid Tumors

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REHOVOT, Israel and MONMOUTH JUNCTION, N.J., Nov. 06, 2023 (GLOBE NEWSWIRE) -- Ayala Pharmaceuticals, Inc. (OTCQX: ADXS), a clinical-stage oncology company, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to AL102, a Gamma Secretase Inhibitor (GSI), for the treatment of desmoid tumors (DT). AL102 is an investigational small molecule GSI, currently being evaluated in the Phase 3 RINGSIDE study in DT. Orphan drug designation is granted by the FDA to drugs and biologics intended for treatment, prevention or diagnosis of a rare disease or condition that affects fewer than 200,000 people in the U.S. at the time of designation.

Desmoid tumors 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, or other parts of the body. They do not metastasize, but often aggressively infiltrate neurovascular structures and vital organs. 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. 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. There are currently no FDA-approved systemic therapies for the treatment of unresectable, recurrent or progressive desmoid tumors.

Under the FDA's Orphan Drug Act, orphan drug status provides incentives, including tax credits, grants and waiver of certain administrative fees for clinical trials, and seven years of market exclusivity following drug approval.

"Receiving FDA orphan drug status for AL102 underscores the significant unmet need for novel treatment options for people living with desmoid tumors. We look forward to continuing to work closely with regulators, clinical investigators, patients and their families to advance this potentially important medicine and make it available to those who may benefit from it," said Kenneth Berlin, President and CEO of Ayala.

About AL102

AL102 is an investigational small molecule Gamma Secretase Inhibitor (GSI) that is designed to potently and selectively inhibit Notch 1, 2, 3 and 4, and is currently being evaluated in the Phase 2/3 RINGSIDE clinical studies in patients with progressing desmoid tumors. AL102 is designed to inhibit the expression of Notch gene targets by blocking the final cleavage step by the gamma secretase required for Notch activation. Ayala obtained an exclusive, worldwide license to develop and commercialize AL102 from Bristol-Myers Squibb Company in November 2017. AL102 was granted U.S. FDA Fast Track Designation for the treatment of DT.

About Ayala Pharmaceuticals, Inc.

Ayala Pharmaceuticals, Inc. is a clinical-stage oncology company primarily focused on developing and commercializing small molecule therapeutics for people living with rare tumors and aggressive cancers. The Company's lead candidates under development are the oral gamma secretase inhibitor, AL102, for desmoid tumors, and aspacytarabine (BST-236), a novel proprietary anti-metabolite for first line treatment in unfit acute myeloid leukemia (AML). AL102 has received Fast Track Designation from the U.S. FDA and is currently in the Phase 3 segment of a pivotal study for patients with desmoid tumors (RINGSIDE). For more information, visit www.ayalapharma.com.

Contacts:

Ayala Pharmaceuticals:
+1-857-444-0553
info@ayalapharma.com

Media:

Tim McCarthy
LifeSci Advisors, LLC
tim@lifesciadvisors.com
917-679-9282

Cautionary Statement Regarding Forward-Looking Statements

Certain statements contained in this filing may be considered forward-looking statements that involve a number of risks and uncertainties, including statements regarding the future conduct of our studies and the potential efficacy and success of product candidates. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "would," "expect," "anticipate," "plan," "likely," "believe," "estimate," "project," "intend," and other similar expressions among others. Statements that are not historical facts are forward-looking statements. Forward-looking statements are based on current beliefs and assumptions that are subject to risks and uncertainties and are not guarantees of future performance. Actual results could differ materially from those contained in any forward-looking statement as a result of various factors, including, without limitation: our ability to integrate the business of Biosight, Ltd., with which we recently consummated a merger ("Biosight"), successfully with ours and to achieve anticipated synergies; the possibility that other anticipated benefits of the merger with Biosight will not be realized, including without limitation, anticipated revenues, expenses, earnings and other financial results, and growth and expansion of the combined company's operations, and the anticipated tax treatment of the combination; potential litigation relating to the transaction that could be instituted against us, Biosight or our respective directors; possible disruptions from the merger with Biosight

that could harm our and/or Biosight's respective businesses; the ability of us and Biosight to retain, attract and hire key personnel; potential adverse reactions or changes to relationships with customers, employees, suppliers or other parties resulting from the announcement or completion of the merger; the success and timing of clinical trials, including subject accrual, the ability to avoid and quickly resolve any clinical holds and the ability to obtain and maintain regulatory approval and/or reimbursement of product candidates for marketing; the ability to obtain the appropriate labeling of products under any regulatory approval; plans to develop and commercialize our products; our ability to continue as a going concern; our levels of available cash and our need to raise additional capital, including to support current and future planned clinical activities; the successful development and implementation of our sales and marketing campaigns; the size and growth of the potential markets for our product candidates and our ability to serve those markets; our ability to successfully compete in the potential markets for our product candidates, if commercialized; regulatory developments in the United States and other countries; the rate and degree of market acceptance of any of our product candidates; new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements; market conditions in the pharmaceutical and biotechnology sectors; our available cash, including to support current and planned clinical activities; uncertainties as to our ability to obtain a listing of our common stock on Nasdaq; our ability to obtain and maintain intellectual property protection for our product candidates; the success and timing of our preclinical studies including IND-enabling studies; the timing of our IND submissions; our ability to get FDA approval for study amendments; the timing of data read-outs; the ability of our product candidates to successfully perform in clinical trials; our ability to initiate, enroll, and execute pilots and clinical trials; our ability to maintain our existing collaborations; our ability to manufacture and the performance of third-party manufacturers; the performance of our clinical research organizations, clinical trial sponsors and clinical trial investigators; our ability to successfully implement our strategy; legislative, regulatory and economic developments; unpredictability and severity of catastrophic events, including, but not limited to, acts of terrorism or outbreak of war or hostilities, as well as management's response to any of the aforementioned factors; and such other factors as are set forth in our periodic public filings with the SEC, including but not limited to those described under the heading "Risk Factors" in the Form 10-K for the fiscal year ended December 31, 2022 of Old Ayala, Inc. (f/k/a Ayala Pharmaceuticals, Inc.) and the Form 10-K for the fiscal year ended October 31, 2022 of Ayala Pharmaceuticals, Inc. (f/k/a Advaxis, Inc.) ("Ayala" or "we," "us" or "our"), and such entities' periodic public filings with the SEC, including but not limited to those described under the heading "Risk Factors" in Ayala's Form 10-K for the fiscal year ended October 31, 2022. Except as required by applicable law, we undertake 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.