Aldeyra Therapeutics Advances Investigational Oral RASP Modulator ADX-629 Into New Phase 2 Systemic Disease Trials

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Previously Announced Phase 2 Clinical Trials Initiated in Minimal Change Disease and Sjögren-Larsson Syndrome

Phase 2 Clinical Trial in Minimal Change Disease Expanded to Encompass Idiopathic Nephrotic Syndrome, a Broad Group of Rare Kidney Disorders

New Phase 2 Clinical Trial Initiated in Atopic Dermatitis

Previously Announced Credit Facility Amendment Extends Cash Runway into the Second Half of 2024

LEXINGTON, Mass.--(BUSINESS WIRE)--Feb. 16, 2023-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced the initiation of Phase 2 clinical trials evaluating the safety and efficacy of ADX-629, a novel, internally developed, investigational oral RASP modulator, for the treatment of minimal change disease and Sjögren-Larsson Syndrome. Aldeyra also announced the expansion of the minimal change disease clinical trial to encompass idiopathic nephrotic syndrome, a broad group of rare immune-mediated kidney disorders that includes minimal change disease. Additionally, Aldeyra announced the initiation of a Phase 2 clinical trial of ADX-629 in atopic dermatitis.

“Following the completion of successful proof-of-concept trials in psoriasis, asthma, COVID-19, and alcohol toxicity, the ADX -629 trials announced today further advance the promising novel pharmacology of our proprietary RASP modulator platform for the treatment of systemic diseases,” said Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. “ADX-629 has the potential to become a first-in-class therapy that may allow for convenient, non-injected, orally administered, broad-based treatment of immune-mediated diseases.

“With the recently amended credit facility, we believe that our cash runway is extended into the second half of 2024 and that we are well positioned to advance our novel investigational product candidates for systemic diseases while executing on initial commercialization activities for reproxalap and ADX-2191, if approved,” Dr. Brady stated.

Disease Targets

- ***Idiopathic Nephrotic Syndrome:*** Idiopathic nephrotic syndrome is comprised of a broad group of renal inflammatory diseases, including minimal change disease, and is characterized by edema, proteinuria, and hypoalbuminemia. The adaptive, multicenter, two-part Phase 2 clinical trial will evaluate the safety and efficacy of ADX-629 and placebo over 12 weeks of treatment in children and adults. Part 1 is expected to enroll five patients who will be treated with ADX-629. Pending the results of Part 1, Part 2 will compare ADX-629 to placebo. Outcomes will include frequency of relapse, as defined by requirement for corticosteroid therapy. Top-line results from Part 1 of the clinical trial are expected in 2023.
- ***Sjögren-Larsson Syndrome:*** Sjögren-Larsson Syndrome is an autosomal recessive neurocutaneous inborn error of metabolism that prevents degradation of specific RASP, and most commonly affects children and adolescents. The investigator-sponsored, open-label, Phase 1/2 clinical trial will evaluate the safety, pharmacodynamics, and exploratory clinical activity in up to 10 patients over 12 weeks of treatment. Outcomes will include plasma and imaging markers of metabolism and brain activity, quality of life, neurological function, and skin assessments. Top-line results are expected in 2023.
- ***Atopic Dermatitis:*** Atopic dermatitis is a chronic hypersensitivity condition that is characterized by dry, itchy, and inflamed skin, and commonly affects children and adults. The adaptive, multicenter, two-part Phase 2 clinical trial will evaluate the safety and efficacy of ADX-629 over 12 weeks of treatment. Part 1 of the trial is expected to enroll approximately 10 patients. Pending the results of Part 1, Part 2 will compare ADX-629 to placebo. Outcomes will include improvement in Investigator Global Assessment and Eczema Area and Severity Index scores. Top-line results from Part 1 of the clinical trial are expected in 2023.

In addition to the trials in idiopathic nephrotic syndrome, Sjögren-Larsson Syndrome, and atopic dermatitis, the ADX-629 program includes the following clinical development initiatives:

- ***Chronic Cough*****:** ADX-629 is currently being evaluated in a multicenter, randomized, double-blind, placebo-controlled, two-period Phase 2 crossover trial in approximately 50 patients with refractory or unexplained chronic cough. Top-line results are expected in the first half of 2023.
- ***Moderate Alcohol-Associated Hepatitis*****:** Aldeyra plans to support an investigator-sponsored Phase 2 clinical trial of ADX-629 in moderate alcohol-associated hepatitis. The trial is expected to be initiated in 2023. In a Phase 2 clinical trial announced last year, ADX-629 reduced dermal flushing and improved balance time following alcohol intoxication.

About ADX-629

ADX-629 is a novel, orally administered investigational RASP modulator for the potential treatment of systemic and retinal immune-mediated diseases. RASP modulators potentially represent upstream immunological switches that shift immune systems from pro-inflammatory states to anti-inflammatory states. ADX-629 is a member of the same chemical class as reproxalap, an investigational new drug under New Drug Application review for the treatment of dry eye disease, a common ocular inflammatory disease.

About Aldeyra

Aldeyra Therapeutics is a clinical-stage biotechnology company developing innovative therapies designed to treat immune-mediated diseases. Our approach is to discover pharmaceuticals that modulate immunological systems, instead of directly inhibiting or activating single protein targets, with the goal of optimizing multiple pathways at once while minimizing toxicity. Our pre-commercial product candidates are reproxalap, a potential treatment for dry eye disease and allergic conjunctivitis, and ADX-2191, a potential treatment for primary vitreoretinal lymphoma, proliferative vitreoretinopathy, and retinitis pigmentosa. In addition, we are developing other product candidates, including ADX-629 and chemically related molecules, for the potential treatment of systemic and retinal immune-mediated diseases. For more information, visit https://www.aldeyra.com/ and follow us on LinkedIn, Facebook, and Twitter.

Safe Harbor Statement

This release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Aldeyra's future expectations, plans, and prospects, including, without limitation, statements regarding the expected timing of top-line results from the clinical trials of ADX-629 in idiopathic nephrotic syndrome, Sjögren-Larsson Syndrome, atopic dermatitis, and chronic cough; the planned initiation of the investigator-sponsored Phase 2 clinical trial of ADX-629 in moderate alcohol-associated hepatitis; the Company's plans to execute on its initial commercialization and launch plans for reproxalap and ADX-2191; the Company's plans for continued development of its novel investigational product candidates for systemic diseases; and the Company's operating plans and expectations regarding the sufficiency and uses of its existing cash, cash equivalents and marketable securities. Aldeyra intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. In some cases, you can identify forward-looking statements by terms such as, but not limited to, "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "on track," "on schedule," "target," "design," "estimate," "predict," "potential," "aim," "plan," or the negative of these terms, and similar expressions intended to identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions, and uncertainties. Aldeyra is at an early stage of development and may not ever have any products that generate significant revenue. All of Aldeyra's development timelines may be subject to adjustment depending on recruitment rate, regulatory review, preclinical and clinical results, and other factors that could delay the initiation, enrollment or completion of clinical trials. Important factors that could cause actual results to differ materially from those reflected in Aldeyra's forward-looking statements include, among others, the timing of enrollment, commencement and completion of Aldeyra's clinical trials, the timing and success of preclinical studies and clinical trials conducted by Aldeyra and its development partners; updated or refined data based on Aldeyra's continuing or post-hoc review and quality control analysis of clinical data, Aldeyra's ability to design clinical trials with protocols, data analysis methodologies, and endpoints acceptable to applicable regulatory authorities; delay in or failure to obtain regulatory approval of Aldeyra's product candidates; the ability to maintain regulatory approval of Aldeyra's product candidates, and the labeling for any approved products; the risk that prior results, such as signals of safety, activity, or durability of effect, observed from preclinical or clinical trials, will not be replicated or will not continue in ongoing or future studies or clinical trials involving Aldeyra's product candidates in clinical trials focused on the same or on different indications; the risk that the results from earlier clinical trials, portions of clinical trials, or pooled clinical data may not accurately predict results of subsequent trials or the remainder of a clinical trial; the scope, progress, expansion, and costs of developing and commercializing Aldeyra's product candidates; uncertainty as to Aldeyra's ability to commercialize (alone or with others) and obtain reimbursement for Aldeyra's product candidates following regulatory approval, if any; the size and growth of the potential markets and pricing for Aldeyra's product candidates and the ability to serve those markets; Aldeyra's expectations regarding Aldeyra's expenses and revenue, the sufficiency or use of Aldeyra's cash resources and needs for additional financing; political, economic, legal, social, and health risks, including the COVID-19 pandemic and subsequent public health measures, and war or other military actions, that may affect Aldeyra's business or the global economy; the rate and degree of market acceptance of any of Aldeyra's product candidates; Aldeyra's expectations regarding competition; Aldeyra's anticipated growth strategies; Aldeyra's ability to attract or retain key personnel; Aldeyra's limited sales and marketing infrastructure; Aldeyra's ability to establish and maintain development partnerships; Aldeyra's ability to successfully integrate acquisitions into its business; Aldeyra's expectations regarding federal, state, and foreign regulatory requirements; regulatory developments in the United States and foreign countries; Aldeyra's ability to obtain and maintain intellectual property protection for its product candidates; the anticipated trends and challenges in Aldeyra's business and the market in which it operates; and other factors that are described in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of Aldeyra's Annual Report on Form 10-K for the year ended December 31, 2021, and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at https://www.sec.gov/. Additional factors may be described in those sections of Aldeyra's Annual Report on Form 10-K for the year ended December 31, 2022, expected to be filed with the SEC in the first quarter of 2023.

In addition to the risks described above and in Aldeyra's other filings with the SEC, other unknown or unpredictable factors also could affect Aldeyra's results. No forward-looking statements can be guaranteed, and actual results may differ materially from such statements. The information in this release is provided only as of the date of this release, and Aldeyra undertakes no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.

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