Aldeyra Therapeutics Initiates Phase 2 Clinical Trial of ADX-2191 for the Treatment of Retinitis Pigmentosa

March 8, 2022

LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 8, 2022-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra), a biotechnology company discovering and developing innovative therapies for the treatment of immune-mediated diseases, today announced that it has initiated a Phase 2 clinical trial of ADX-2191 (intravitreal methotrexate 0.8%), an investigational new drug product, for the treatment of retinitis pigmentosa (RP). RP is a clinical group of rare genetic eye diseases characterized by retinal cell death and loss of vision. There are no approved treatments for RP, which affects an estimated 82,000-110,000 individuals in the United States, and approximately 1 in 4,000 people worldwide.

“ADX-2191 represents a novel approach for the treatment of patients with retinitis pigmentosa, an incurable, sight-threatening condition,” stated Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. “We are excited about the opportunity to advance our ADX-2191 retinal program across a variety of rare and underserved diseases, and look forward to reporting the top-line results of the retinitis pigmentosa Phase 2 clinical trial in the second half of this year.”

The single-center, open-label Phase 2 clinical trial will evaluate the safety and tolerability of ADX-2191 in patients diagnosed with RP due to mutations of the rhodopsin gene, including the P23H gene mutation. The trial is expected to enroll eight patients, with four patients receiving monthly and four patients receiving twice-monthly intravitreal injections of ADX-2191 over a period of three months. The trial is being conducted at Duke University Medical Center in Durham, North Carolina. Top-line results are expected in the second half of 2022.

About ADX-2191

ADX-2191, the intravitreal formulation of methotrexate, is a compound that inhibits cellular replication and activation. In vivo research has identified the activity of methotrexate in inducing misfolded rhodopsin clearance, suggesting the potential to treat forms of retinitis pigmentosa that are characterized by misfolded rhodopsin.[1] ADX-2191 has been granted orphan drug designation by the U.S. Food and Drug Administration for the treatment of retinitis pigmentosa. Orphan drug designation qualifies sponsors for incentives including tax credits for qualified clinical trials; exemption from user fees; and market exclusivity after approval, if received.

About Aldeyra Therapeutics

Aldeyra Therapeutics discovers and develops innovative therapies designed to treat immune-mediated diseases. Our approach is to develop therapies 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. Two of our lead product candidates, reproxalap and ADX-629, target pre-cytokine, systems-based mediators of inflammation known as RASP (reactive aldehyde species). Reproxalap is in Phase 3 clinical trials in patients with dry eye disease and allergic conjunctivitis. ADX-629, an orally administered RASP modulator, is in Phase 2 proof-of-concept clinical trials in psoriasis, asthma, and COVID-19. Our pipeline also includes ADX-2191 (intravitreal methotrexate 0.8%), in development for the prevention of proliferative vitreoretinopathy and the treatment of retinitis pigmentosa and primary vitreoretinal lymphoma. 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 strategy, future operations, prospects, plans, and objectives and Aldeyra's plans and expectations for its product candidates, including ADX-2191. 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," "scheduled," "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 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 review and quality control analysis of clinical data, Aldeyra's ability to design clinical trials with protocols 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) 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 related 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, 2020 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, 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, 2021, expected to be filed with the SEC in the first quarter of 2022.

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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[1] Xi Y, Chen Y. Pharmacological strategies for treating misfolded rhodopsin-associated autosomal dominant retinitis pigmentosa. Neural Regen Res. 2022 Jan;17(1):110-112. doi: 10.4103/1673-5374.314306. PMID: 34100444; PMCID: PMC8451548.

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