Aldeyra Announces Positive Top-Line Phase 1 Clinical Trial Results and Clinical Development Plans for ADX-629

April 14, 2020

LEXINGTON, Mass.--(BUSINESS WIRE)--Apr. 14, 2020-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra), a clinical-stage biotechnology company focused on the development of novel therapies with the potential to improve the lives of patients with immune-mediated diseases, today announced positive top-line Phase 1 clinical trial results for ADX-629, a first-in-class orally available reactive aldehyde species (RASP) inhibitor in development for the treatment of systemic immune-mediated diseases. Aldeyra also announced Phase 2 clinical development plans for ADX-629.

The Phase 1 clinical trial was a single-ascending and multiple-ascending dose trial to assess the pharmacokinetic, pharmacodynamic, safety, and tolerability profile of ADX-629 administered orally to healthy volunteers. Of the 85 subjects enrolled in the trial, 41 received ADX-629 orally as a single dose; 23 received ADX-629 orally twice per day for 10 days; and 21 received placebo. ADX-629 was well-tolerated, and no treatment-related adverse events were observed in the trial. No clinically meaningful changes were observed in vital signs, quantitative electrocardiography, or blood chemistry results. Clinically relevant plasma concentrations exceeding known levels of RASP were observed. Relative to subjects treated with placebo, reduction in the commonly described pro-inflammatory RASP malondialdehyde was observed in treated subjects.

“To our knowledge, ADX-629 is the first orally available and irreversible covalent inhibitor of pro-inflammatory RASP, and potentially represents a new paradigm in the understanding and treatment of immune-mediated disease,” stated Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. “The results from the Phase 1 clinical trial announced today support the potential to test ADX-629 broadly across serious diseases characterized by severe inflammation not well-addressed by current therapy.”

Unlike most currently available drugs, the RASP targets of ADX-629 are small molecules rather than proteins. As such, ADX-629 could represent a new pharmacotherapeutic approach with potential applications across a large number of immune-mediated diseases. RASP are upstream mediators of inflammation, regulating known mediators such as NF-κB, inflammasomes, and scavenger receptor A. ADX-629 covalently binds RASP, which are then degraded intracellularly. In animal models of cytokine storm, ADX-629 and structural analog reproxalap, now in Phase 3 clinical testing for certain inflammatory ocular diseases, have demonstrated reduction in the levels of a variety of pro-inflammatory cytokines, including TNF-α, IFN-γ, IL-1, and IL-17, while upregulating the principal anti-inflammatory cytokine, IL-10.

Aldeyra plans to facilitate the clinical testing of ADX-629 in patients with COVID-19-associated respiratory compromise by requesting a pre-investigational new drug meeting with the Infectious Disease Division of the U.S. Food and Drug Administration (FDA). In addition, Aldeyra has filed ADX-629 under the FDA’s Coronavirus Treatment Acceleration Program (CTAP). By potentially mitigating aberrant cytokine responses, ADX-629 may delay or prevent progression of acute respiratory distress syndrome and other forms of respiratory compromise that generally require mechanical ventilation.

In addition, contingent on clinical research facility availability, which is currently limited due to COVID-19 precautions, Aldeyra plans to test ADX-629 in Phase 2 clinical trials of respiratory and dermal conditions associated with elevated levels of RASP, potentially including atopic asthma, chronic cough, psoriasis, and atopic dermatitis.

“We are excited to add potential new milestones to our clinical pipeline as we advance ADX-629 to Phase 2 clinical testing and expand our clinical development focus to include systemic disease,” Dr. Brady stated.

About Aldeyra Therapeutics

Aldeyra Therapeutics is a clinical-stage biotechnology company focused on the development of novel therapies with the potential to improve the lives of patients with immune-mediated diseases. Two of the company’s lead compounds, reproxalap and ADX-629, target reactive aldehyde species (RASP), which are elevated in ocular and systemic inflammatory disease, leading to activation of a broad array of inflammatory factors, including NF-κB, inflammasomes, and Scavenger Receptor A. Reproxalap is being evaluated in Phase 3 clinical trials in patients with dry eye disease and allergic conjunctivitis. The company’s clinical pipeline also includes ADX-2191, a dihydrofolate reductase inhibitor in Phase 3 testing for proliferative vitreoretinopathy.

Safe Harbor Statement

This release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding Aldeyra's strategy, future operations, prospects, plans, and objectives and Aldeyra's plans and expectations for its product candidates, including expectations regarding the potential of its RASP inhibitors, including ADX-629 and reproxalap, for potential anti-inflammatory and antiviral activity in the treatment of COVID-19. 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; 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 recent COVID-19 outbreak and subsequent public health measures, 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, 2019, which is on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at www.sec.gov. Additional factors may be described in those sections of Aldeyra's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, expected to be filed with the SEC in the second quarter of 2020.

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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