Aldeyra Therapeutics Announces Improvement from Baseline in Retinal Function in Phase 2 Clinical Trial of ADX-2191 in Patients with Retinitis Pigmentosa

June 29, 2023

- Best Corrected and Low-Light Visual Acuity Statistically Significantly Improved
- As Assessed by Electroretinography, Time to Retinal Response Statistically Significantly Improved
- As Assessed by Macular and Dark-Adapted Perimetry, Retinal Sensitivity Statistically Significantly Improved
- ADX-2191 Was Well Tolerated and No Safety Concerns Were Identified
- Planned Phase 2/3 Clinical Trial to be Discussed with Regulatory Authorities
- Company to Discuss Results in Conference Call and Webcast at 8:00 a.m. ET Today

LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 29, 2023-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra), a biotechnology company devoted to discovering and developing innovative therapies designed to treat immune-mediated diseases, today announced positive top-line results from the Phase 2 clinical trial of intravitreal ADX-2191 (methotrexate injection, USP), an investigational drug candidate, in patients with retinitis pigmentosa. Relative to baseline, the clinical trial demonstrated statistically significant improvement in retinal function across a number of different physiological and psychophysical assessments.

“The improvement in retinal function relative to baseline observed in this retinitis pigmentosa clinical trial of ADX-2191 may offer hope to patients that today have no therapeutic options,” stated Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. “Based on compelling proof-of-concept clinical activity that is consistent with a well-defined mechanism of action supported by preclinical evidence, we are excited to meet with regulatory authorities to discuss initiation of a potentially pivotal Phase 2/3 clinical trial, as we enthusiastically advance ADX-2191 to the next stage of development.”

Based on preclinical evidence suggesting that methotrexate may facilitate the clearance of mutated rhodopsin[1], a protein critical for visual cycle function, an open-label, single-center Phase 2 clinical trial of ADX-2191 was performed in eight retinitis pigmentosa patients with rhodopsin misfolding mutations. Over three months of treatment with ADX-2191, four patients received monthly injections and four patients received twice-monthly injections. The primary endpoint of the clinical trial was safety. Secondary endpoints included change from baseline in visual acuity; retinal function, as assessed by macular and dark-adapted chromatic perimetry and electroretinography; and retinal morphology, as assessed by optical coherence tomography. Visual acuity, perimetry, and morphology assessments were performed monthly for four months from initiation of therapy. Electroretinography was performed at baseline and at 90 days from initiation of therapy.

All enrolled patients completed the trial per protocol. Relative to baseline, across all patients, statistical significance was achieved for improvement in best corrected visual acuity ($P<0.0001$), low-light visual acuity ($P=0.0001$), time to electroretinographic response to light ($P=0.02$), macular sensitivity to light ($P<0.0001$), and dark-adapted peripheral sensitivity to light ($P<0.0001$). ADX-2191 was well tolerated, and no safety concerns were identified. No treatment-related adverse events associated with retinal morphology were observed. No serious adverse events were reported, and no patients discontinued due to adverse events.

“Retinitis pigmentosa is a relentlessly progressive disease that inevitably leads to loss of vision,” stated Ramiro S. Maldonado, MD., the Principal Investigator of the clinical trial and Assistant Professor of Ophthalmology at Duke University Medical Center. “The promising results presented today are supportive of a potential novel approach for the treatment of retinitis pigmentosa patients with rhodopsin mutations.”

ADX-2191 is a novel intravitreal formulation of methotrexate in clinical development for proliferative vitreoretinopathy and retinitis pigmentosa, both of which are rare, sight-threatening retinal diseases with no approved therapies. The prevalence of retinitis pigmentosa is more than one million people worldwide, and genetic mutations leading to rhodopsin misfolding account for approximately one-third of cases. ADX-2191 has been granted orphan drug designation by the U.S. Food and Drug Administration for the treatment of proliferative vitreoretinopathy and retinitis pigmentosa.

Conference Call & Webcast Information

Aldeyra will host a conference call at 8:00 a.m. ET today to discuss top-line results of the Phase 2 clinical trial of ADX-2191 in retinitis pigmentosa. The dial-in numbers are (888) 415-4305 for domestic callers and (646) 960-0336 for international callers. The access code is 5858366. A live webcast of the conference call will be available on the Investor Relations page of the company’s website at https://ir.aldeyra.com. After the live webcast, the event will remain archived on the Aldeyra Therapeutics website for 90 days.

About Aldeyra

Aldeyra Therapeutics is a biotechnology company devoted to discovering innovative therapies designed to treat immune-mediated diseases. Our approach is to develop 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 product candidates include RASP (reactive aldehyde species) modulators ADX-629, ADX-246, ADX-248, and chemically related molecules for the potential treatment of systemic and retinal immune-mediated diseases. Our pre-commercial product candidates are reproxalap, a RASP modulator for the potential treatment of dry eye disease (under U.S. Food and Drug Administration New Drug Application Review) and allergic conjunctivitis, and ADX-2191, a novel formulation of intravitreal methotrexate for the potential treatment of proliferative vitreoretinopathy and retinitis pigmentosa.

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 goals, opportunity, including market size, and potential for ADX-2191 and anticipated clinical developments or regulatory milestones for 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," "on schedule," "target," "design," "estimate," "predict," "contemplates," likely," "potential," "continue," "ongoing," "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, funding, and other factors that could delay the initiation, enrollment, or completion of clinical trials. Important factors that may 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; delay in or failure to obtain regulatory approval of Aldeyra's product candidates, including as a result of the FDA not accepting Aldeyra's regulatory filings, requiring additional clinical trials or data prior to review or approval of such filings; 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 different indications; the scope, progress, expansion, and costs of developing and commercializing Aldeyra's product candidates; the current and potential future impact of the COVID-19 pandemic on Aldeyra's business, results of operations, and financial position; 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 future revenue, the timing of future revenue, the sufficiency or use of Aldeyra's cash resources and needs for additional financing; 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 commercialization, marketing and manufacturing capabilities and strategy; 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; 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; 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, 2022, and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, expected to be filed with the SEC in the third 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.

[1]FASEB J. 34(8): 10146-10167, 2020.

View source version on businesswire.com: https://www.businesswire.com/news/home/20230629018435/en/

Investor & Media:
David Burke
Tel: (917) 618-2651
investorrelations@aldeyra.com

Source: Aldeyra Therapeutics, Inc.