Aldeyra Therapeutics Receives Orphan Medicinal Product Designation from the European Commission for ADX-2191 Retinal Disease Program

June 15, 2020

LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 15, 2020-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced that ADX-2191 has been designated an orphan medicinal product by the European Commission for the treatment of retinal detachment. ADX-2191 is an investigational therapy in clinical development in the U.S. for the prevention of proliferative vitreoretinopathy (PVR), a rare but serious sight-threatening retinal disease with no approved treatment. PVR is the leading cause of failure of rhegmatogenous retinal detachment surgery.

“The Commission’s broad orphan medicinal product designation, covering the treatment of retinal detachment, is an important step in advancing the clinical development of ADX-2191 in the European Union,” stated Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. “ADX-2191 represents a novel therapeutic approach that we believe has the potential to decrease the development of scar tissue that characterizes PVR, breaking the cycle of complex re-attachment surgeries and severe vision loss that often are a consequence of this devastating disease. ADX-2191 has the potential to be the first pharmacological prophylaxis to prevent retinal detachment associated with PVR.”

The Commission’s decision follows a positive opinion on Aldeyra’s application for orphan medicinal product designation from the European Medicines Agency’s Committee for Orphan Medicinal Products. To qualify for the designation, an investigational medicine must be intended to treat a life-threatening or chronically debilitating condition that affects no more than five in 10,000 people in the European Union (EU). Sponsors with medicines that receive this designation are entitled to a range of incentives, including protocol assistance, potential research funding, access to a centralized market authorization procedure, and, if approved, 10 years of EU market exclusivity.

ADX-2191 previously was granted orphan drug status and fast track designation by the U.S. Food and Drug Administration for the prevention of PVR. In December 2019, Aldeyra initiated patient enrollment in its Phase 3 GUARD Trial, a two-part, multicenter, randomized, controlled, adaptive clinical trial evaluating the efficacy of intravitreal injections of ADX-2191 versus standard-of-care for the prevention of PVR. Aldeyra is exploring additional indications for ADX-2191, including primary intraocular lymphoma, a rare but serious ocular cancer that can affect the retina, uvea, optic nerve, and other ocular structures.

About ADX-2191

ADX-2191, the intravitreal formulation of methotrexate, is designed to inhibit dihydrofolate reductase, an enzyme involved in cellular replication and activation. The observed clinical activity of ADX-2191 in patients with PVR is believed to be the result of down-regulation of aberrant retinal cell proliferation and activity, thereby leading to reduced retinal scarring that is characteristic of PVR. Aldeyra retains an exclusive license to certain patents related to the use of ADX-2191 for the prevention of PVR.

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 elevated levels of cytokine release via 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, and ADX-1612, a chaperome inhibitor in Phase 2 testing for COVID-19 and ovarian cancer. 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 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. As a result of the COVID-19 pandemic, clinical site availability, staffing, and patient recruitment have been negatively affected and the timelines to complete our clinical trials may be delayed. 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 trials involving Aldeyra's product

candidates; 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 our business, results of operations and financial position; 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 and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at www.sec.gov.

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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