Aldeyra Therapeutics Receives Orphan Drug Designation from the U.S. Food and Drug Administration for ADX-2191 to Treat Retinitis Pigmentosa

August 4, 2021

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 4, 2021-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for ADX-2191 (methotrexate for intravitreal injection) for the treatment of retinitis pigmentosa (RP). There are no approved drug treatments for patients with RP, a clinical group of rare genetic eye diseases characterized by retinal cell death and loss of vision. The disease affects an estimated 82,000-110,000 individuals in the United States, and approximately 1 in 4,000 people worldwide.

“Retinitis pigmentosa is a serious and incurable sight-threatening disease that represents a major unmet need in the field of ophthalmology,” stated Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. "ADX-2191 has now received orphan designations for three distinct clinical indications, highlighting the broad platform potential of ADX-2191 to treat an array of rare retinal disorders.”

Methotrexate inhibits dihydrofolic reductase, an enzyme involved in cellular replication and activation. In addition to RP, the FDA has granted orphan drug designation to ADX-2191 for the treatment of primary vitreoretinal lymphoma, a rare, aggressive, high-grade cancer; and both orphan drug and fast track designation to ADX-2191 for the prevention of proliferative vitreoretinopathy, a rare inflammatory disorder of the retina that leads to severe retinal scarring and blindness and is the leading cause of failure of retinal reattachment surgery.

The FDA’s orphan drug designation program is designed to provide financial incentives to sponsors for developing drugs and biologics for rare diseases and conditions, in part defined as affecting fewer than 200,000 people in the United States. Sponsors of designated orphan drugs are eligible for partial tax credits for clinical trial costs, waiver of the user fee for marketing applications and, upon approval, consideration for seven years of marketing exclusivity.

About Aldeyra Therapeutics

Aldeyra Therapeutics is a biotechnology company developing novel immune-modulating therapies to treat ocular and systemic diseases. Two of the company’s lead product candidates, reproxalap and ADX-629, target RASP (reactive aldehyde species), which are pre-cytokine, systems-based mediators of inflammation. 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 (methotrexate for intravitreal injection), a drug candidate in Phase 3 testing for the prevention of proliferative vitreoretinopathy. 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 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 smaller clinical trials or portions of clinical trials may not accurately predict results of larger scale 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 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, 2020 and Form 10-Q for the quarter ended March 31, 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 set forth in those sections of Aldeyra's Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, expected to be filed with the SEC in the third quarter of 2021.

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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210804005122/en/

Corporate:
Joshua Reed
Aldeyra Therapeutics, Inc.
Tel: 781-761-4904 ext. 218
jreed@aldeyra.com

Investor & Media:
Scott Solomon
Sharon Merrill Associates, Inc.
Tel: 617-542-5300
ALDX@investorrelations.com

Source: Aldeyra Therapeutics, Inc.