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

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LEXINGTON, Mass.--(BUSINESS WIRE)--Jul. 20, 2021-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to the company for ADX-2191 (methotrexate for intravitreal injection) for the treatment of primary vitreoretinal lymphoma (PVRL). There are no approved treatments for PVRL, a rare, aggressive, high-grade cancer that affects approximately 2,800 people in the United States, with approximately 600 new cases diagnosed annually.

“PVRL is an often fatal cancer that creates unique challenges for diagnosis and treatment,” stated Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. “The FDA’s orphan drug designation fosters an important clinical development and commercialization pathway for ADX-2191, which represents the first methotrexate preparation specifically formulated for intraocular injection. The designation is another important step forward for our retinal disease program, complementing our ongoing clinical development of ADX-2191 for the prevention of proliferative vitreoretinopathy, 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 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 ADX-2191

ADX-2191 (methotrexate for intravitreal injection) inhibits dihydrofolate reductase, an enzyme involved in cellular replication and activation. Methotrexate is the most commonly used intravitreal medication for the treatment of PVRL. ADX-2191 was previously granted orphan drug status and fast track designation by the FDA for the prevention of proliferative vitreoretinopathy, a rare but serious sight-threatening retinal disease with no approved treatment.

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 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.

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