Aldeyra Therapeutics Announces Positive Primary Vitreoretinal Lymphoma Pre-NDA Meeting with the FDA

December 1, 2022

Primary Vitreoretinal Lymphoma New Drug Application (NDA) for ADX-2191 Expected to be Submitted as Soon as the End of 2022

Company Intends to Request Priority Review Designation

LEXINGTON, Mass.--(BUSINESS WIRE)--Dec. 1, 2022-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced that, following the recent receipt of official minutes from its pre-NDA (New Drug Application) meeting with the U.S. Food and Drug Administration (FDA), the Company plans to submit an NDA as soon as the end of 2022 for marketing approval of the investigational drug candidate ADX-2191 for the treatment of primary vitreoretinal lymphoma.

“Pending FDA review, ADX-2191 could be the first FDA-approved therapy for primary vitreoretinal lymphoma, a rare but potentially fatal cancer with a median survival of less than five years,” stated Todd C. Brady, M.D., Ph.D., Aldeyra’s President and Chief Executive Officer.

ADX-2191, which has received FDA Orphan Drug Designation for the treatment of primary vitreoretinal lymphoma, is a novel, vitreous-compatible formulation of methotrexate. The planned NDA submission is expected to include a combination of published literature on the safety and efficacy of methotrexate for the treatment of primary vitreoretinal lymphoma and safety data from the recently completed Phase 3 GUARD Trial of ADX-2191 in proliferative vitreoretinopathy. During the Phase 3 GUARD Trial, no safety signals were observed, and ADX-2191 was well tolerated; there were no observed treatment-emergent serious adverse events. The most common adverse event associated with ADX-2191 treatment was punctate keratitis, a frequently observed side effect of intravitreal methotrexate, that was most commonly mild in severity. In the Phase 3 GUARD Trial, the incidence of punctate keratitis with ADX-2191 administration was observed to be less than that previously reported with intravitreal injection of compounded methotrexate.[1]

Based on the pre-NDA meeting minutes, Aldeyra intends to request Priority Review designation, which reduces the review period in which the FDA aims to take action on an NDA to within 6 months (compared to 10 months under standard review). The designation is intended to direct overall attention and resources to the evaluation of applications for drugs that, if approved, would represent significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.

In addition to the planned NDA submission for ADX-2191 in primary vitreoretinal lymphoma, a Type C meeting with the FDA to discuss the completion of clinical development of ADX-2191 for the prevention of proliferative vitreoretinopathy is planned for the first half of 2023, and results from the Phase 2 clinical trial of ADX-2191 in retinitis pigmentosa are expected in the first half of 2023.

About ADX-2191

ADX-2191 (methotrexate injection, USP) is a sterile, non-compounded intravitreal formulation of methotrexate for the potential prevention or treatment of specific rare retinal diseases, including primary vitreoretinal lymphoma, proliferative vitreoretinopathy, and retinitis pigmentosa. The ADX-2191 intravitreal formulation is preservative-free, designed to be vitreous-compatible, and optimized for excipient composition, viscosity, density, tonicity, pH, concentration, and volume of administration. ADX-2191 has received FDA Orphan Drug Designation for the prevention of proliferative vitreoretinopathy, and the treatment of primary vitreoretinal lymphoma and retinitis pigmentosa.

About Primary Vitreoretinal Lymphoma

Primary vitreoretinal lymphoma is a rare, aggressive, and potentially fatal retinal cancer that is diagnosed in approximately 300 to 600 patients in the United States per year. The median survival for newly diagnosed patients is 4.83 years. The most common ocular complaints reported by patients include blurred vision, painless loss of vision, floaters, red eye, and photophobia. No approved treatments are currently available, though methotrexate represents the current standard of care.[2]

About Aldeyra

Aldeyra Therapeutics is a clinical-stage biotechnology company developing innovative therapies designed to treat immune-mediated diseases. Our approach is to discover 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 pre-commercial product candidates are reproxalap, a potential treatment for dry eye disease and allergic conjunctivitis, and ADX-2191, a potential treatment for primary vitreoretinal lymphoma, proliferative vitreoretinopathy, and other rare sight-threatening retinal diseases. In addition, we are developing other product candidates, including ADX-629 and chemically related molecules, for the potential treatment of systemic and retinal immune-mediated diseases. 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, but not limited to, statements regarding Aldeyra's future expectations, plans, and prospects, including, without limitation, statements regarding the expected timing of the NDA submission for ADX-2191 for primary vitreoretinal lymphoma; the data expected to be included in the NDA submission; the Company’s plan to request Priority Review designation; the planned timing of the Type C meeting with the FDA to discuss the completion of clinical development of

ADX-2191 for the prevention of proliferative vitreoretinopathy; and the expected timing of results from the Phase 2 clinical trial of ADX-2191 in retinitis pigmentosa. 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,” “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, enrollment 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 or post-hoc review and quality control analysis of clinical data, Aldeyra's ability to design clinical trials with protocols, data analysis methodologies, 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 earlier clinical trials, portions of clinical trials, or pooled clinical data may not accurately predict results of subsequent 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) 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 revenue, the sufficiency or use of Aldeyra's cash resources and needs for additional financing; 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; 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, 2021, and Aldeyra’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at https://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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[1] Annals of Hematology, 95(4), 593–601, 2016.

[2] Aldeyra internal estimates. Data on file; Primary Vitreoretinal Lymphoma by D. J. Wilson on AAO EyeWiki; M. Sagoo, Survey of Ophthalmology (2014); Grimm et. al., Annals of Oncology (2007).

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Investor & Media Contact:
Scott Solomon
Sharon Merrill Associates, Inc.
Tel: (857) 383-2409
ALDX@investorrelations.com

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