Aldeyra Therapeutics Reports First-Quarter 2023 Financial Results and Recent Corporate Highlights

May 4, 2023

- *Top-Line Results from the Phase 2 Clinical Trial of ADX-2191 in Retinitis Pigmentosa Expected in the Second Quarter of 2023*
- *Top-Line Results from the Phase 2 Clinical Trial of ADX-629 in Chronic Cough Expected in the Second Quarter of 2023*
- *Top-Line Results from the Phase 3 INVIGORATE-2 Trial of Reproxalap in Allergic Conjunctivitis Expected in the Second Quarter of 2023*
- *NDA Priority Review PDUFA Date for ADX-2191 for the Treatment of Primary Vitreoretinal Lymphoma is June 21, 2023*
- *NDA PDUFA Date for Reproxalap for the Treatment of Dry Eye Disease is November 23, 2023*
- *Cash and Cash Equivalents of $165.0 Million as of March 31, 2023*

LEXINGTON, Mass.--(BUSINESS WIRE)--May 4, 2023-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra), a biotechnology company devoted to discovering and developing innovative therapies designed to treat immune-mediated diseases, today reported recent corporate highlights and financial results for the quarter ended March 31, 2023.

“Aldeyra continues to build a robust pipeline of novel drug candidates for the treatment of immune-mediated diseases,” stated Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. “Multiple regulatory and clinical catalysts are planned for the coming quarters, including PDUFA dates for ADX-2191 and reproxalap, top-line results from our Phase 2 clinical trials in retinitis pigmentosa and chronic cough, and top-line results from our Phase 3 INVIGORATE-2 trial in allergic conjunctivitis.”

Recent Corporate Highlights

- **Enrollment Completed in the Phase 2 Clinical Trial of ADX-2191 in Retinitis Pigmentosa:** The open-label, single-center Phase 2 clinical trial enrolled a total of eight retinitis pigmentosa patients with rhodopsin gene mutations, including the P23H gene mutation. Patients receive either monthly or twice-monthly intravitreal doses of ADX-2191 for three months. The primary endpoint of the trial is safety. Secondary endpoints include change from baseline in visual acuity; retinal function, as assessed by foveal microperimetry, electroretinography, and dark adaptation; and retinal morphology, as assessed by optical coherence tomography. ADX-2191, an investigational drug candidate, is a novel intravitreal formulation of methotrexate, which in preclinical models of retinitis pigmentosa facilitates the clearance of misfolded rhodopsin, a critical visual cycle protein susceptible to genetic mutation.[1] Top-line results from the Phase 2 clinical trial are expected in the second quarter of 2023.
- **Enrollment Completed in the Phase 2 Clinical Trial of ADX-629 in Chronic Cough:** The multicenter, randomized, double-blind, placebo-controlled, two-period Phase 2 crossover trial enrolled 51 patients with refractory or unexplained chronic cough, which is often defined as a cough that persists for more than eight weeks. Patients were randomized to receive ADX-629 or placebo twice daily for two weeks, followed by a two-week washout period prior to crossing over to two weeks of treatment with ADX-629 or placebo, whichever was not received in the first period. The primary endpoint of the trial is safety. Secondary endpoints include awake cough frequency, 24-hour cough frequency, patient-reported cough severity, quality of life, and patient and clinician global impression of change. ADX-629, an investigational new drug candidate, is a novel, orally administered RASP (reactive aldehyde species) modulator for the potential treatment of systemic immune-mediated diseases. RASP were observed in a preliminary observational study to be elevated in the bronchioalveolar lavage fluid of patients with chronic cough,[2] and may contribute to neurosensory dysfunction as well as inflammation. Top-line results from the Phase 2 clinical trial are expected in the second quarter of 2023.
- **First Patient Enrolled in the Phase 2 Clinical Trial of ADX-629 in Atopic Dermatitis:** The multicenter, adaptive, two-part Phase 2 clinical trial will evaluate the safety and efficacy of ADX-629 alone and in combination with standard of care in adults with mild, moderate, or severe atopic dermatitis. In Part 1, approximately 10 patients will receive open-label ADX-629 twice daily for three months. Outcomes will include improvement in Investigator Global Assessment and Eczema Area and Severity Index scores. In patients with atopic dermatitis, the pro-inflammatory RASP malondialdehyde is elevated compared to levels observed in healthy controls.[3] Top-line results from Part 1 are expected in the second half of 2023. Pending the results of Part 1, Part 2 will randomize patients to receive either ADX-629 or placebo treatment twice daily for three months.
- **Enrollment Completed in the Phase 3 INVIGORATE-2 Clinical Trial of Reproxalap in Allergic Conjunctivitis:** The randomized, double-masked, crossover, vehicle-controlled Phase 3 clinical trial enrolled 131 seasonal allergic conjunctivitis patients who were evaluated for 3.5 hours in an allergen chamber designed to simulate real-world pollen exposure.

Consistent with pivotal trials of approved allergic conjunctivitis products, the primary endpoint of INVIGORATE-2 is patient-reported ocular itching. The protocol of INVIGORATE-2 is substantially identical to that of the Phase 3 INVIGORATE clinical trial and a Phase 2 clinical trial,[4] both of which achieved the ocular itching endpoint (P<0.001). Reproxalap, an investigational new drug candidate, is a first-in-class small-molecule modulator of RASP, which are elevated in ocular and systemic inflammatory disease. Top-line results from the Phase 3 INVIGORATE-2 clinical trial are expected in second quarter of 2023.

Additional Planned Clinical and Regulatory Milestones

- **Results from Phase 2 Clinical Trials of ADX-629:** Top-line results from Phase 2 clinical trials of ADX-629 in idiopathic nephrotic syndrome (Part 1) and Sjögren-Larsson Syndrome are expected in the second half of 2023. Idiopathic nephrotic syndrome is a rare inflammatory kidney disease characterized by inflammation. Sjögren-Larsson Syndrome is an inborn error of metabolism characterized by mutations in an enzyme that metabolizes RASP.
- **Type C Meeting with the FDA for ADX-2191 in Proliferative Vitreoretinopathy:** Aldeyra plans to conduct a Type C meeting with the U.S. Food and Drug Administration in the second half of 2023 to discuss the completion of clinical development of ADX-2191 for the prevention of proliferative vitreoretinopathy, a rare sight-threatening disease that occurs following retinal detachment.
- **Initiation of Clinical Trials of Next-Generation RASP-Modulator Drug Candidates for Systemic Immune-Mediated Diseases and Geographic Atrophy:** Pending completion of Investigational New Drug requirements, a Phase 1 clinical trial of orally administered ADX-246 for the treatment of systemic immune-mediated diseases, and a Phase 1/2 clinical trial of intravitreally injected ADX-248 for the treatment of geographic atrophy, a sight-threatening retinal disease, are expected to initiate in the second half of 2023 or early 2024.

First-Quarter 2023 Financial Results

Cash and cash equivalents as of March 31, 2023 were $165.0 million. Based on its current operating plan, Aldeyra believes that existing cash and cash equivalents will be sufficient to fund currently projected operating expenses into the second half of 2024, including the initial commercialization and launch plans for reproxalap and ADX-2191, if approved, and continued early and late-stage development of Aldeyra's product candidates in ocular and systemic immune-mediated diseases.

Net loss for the three months ended March 31, 2023 was $15.6 million, or $0.27 per share, compared with a net loss of $16.8 million, or $0.29 per share, for the comparable period of 2022.

Research and development expenses for the three months ended March 31, 2023 were $11.2 million, compared with $12.2 million for the same period in 2022. The decrease of $1.0 million was primarily related to a decrease in external clinical development costs, offset by an increase in personnel costs, drug product manufacturing expenditures, external preclinical development costs, and consulting expenditures.

General and administrative expenses for the three months ended March 31, 2023 were $5.6 million, compared with $4.2 million for the same period in 2022. The increase of $1.4 million was primarily related to higher personnel costs and legal expenditures, offset by a decrease in consulting expenditures.

Total operating expenses for the three months ended March 31, 2023 were $16.8 million, compared with total operating expenses of $16.5 million for the same period in 2022.

Beginning with this announcement of first quarter 2023 financial results, Aldeyra will no longer conduct quarterly conference calls to discuss financial results. Aldeyra plans to continue hosting conference calls, as appropriate, to report the results of certain clinical trials and other material information concerning regulatory and clinical developments.

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 primary vitreoretinal lymphoma (under U.S. Food and Drug Administration New Drug Application Priority Review), proliferative vitreoretinopathy, and other rare sight-threatening retinal 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 the expected timing of top-line results from the Phase 2 clinical trials of ADX-2191 in retinitis pigmentosa and ADX-629 in chronic cough; the timing of top-line results from the Phase 3 INVIGORATE-2 Trial of reproxalap for the treatment of allergic conjunctivitis; the likelihood and timing of the FDA's potential approval of the NDAs for ADX-2191 and reproxalap by the respective PDUFA dates, or at any other time, and the adequacy of the data included in the NDA submissions; the timing of a Type C meeting with the FDA to discuss the completion of clinical development of ADX 2191 for the prevention of proliferative vitreoretinopathy; and Aldeyra's projected cash runway. 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,” “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 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; 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, which is 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 March 31, 2023, expected to be filed with the SEC in the second 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.

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[1] Neural Regen Res. 17(1): 110-112, 2022. FASEB J. 34(8): 10146-10167, 2020.

[2] Date on file.

[3] Heliyon 28;7(3): e06621, 2021. J Clin Diagn Res. 7(12): 2683-5, 2013.

[4] Clin Ophthalmol. 16: 15-23, 2022.

ALDEYRA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS

	March 31, 2023	December 31, 2022
ASSETS	**(Unaudited)**	
Current assets:		
Cash and cash equivalents	$ 165,028,105	$ 144,419,364
Marketable securities	—	29,881,520
Prepaid expenses and other current assets	2,989,115	6,722,229
Total current assets	168,017,220	181,023,113
Right-of-use assets	189,033	249,265
Fixed assets, net	12,539	19,279
Total assets	$ 168,218,792	$ 181,291,657
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 429,685	$ 133,625
Accrued expenses	14,433,679	14,065,885
Current portion of long-term debt	954,325	911,763
Operating lease liabilities	190,202	249,265
Total current liabilities	16,007,891	15,360,538
Long-term debt, net of current portion	14,967,688	14,923,090

Total liabilities	30,975,579	30,283,628
Stockholders' equity:		
Preferred stock, $0.001 par value, 15,000,000 shares authorized, none issued and outstanding	—	—
Common stock, $0.001 par value; 150,000,000 authorized and 58,791,603 and 58,560,078 shares issued and outstanding, respectively	58,792	58,560
Additional paid-in capital	509,516,738	507,770,045
Accumulated other comprehensive loss	—	(103,938)
Accumulated deficit	(372,332,317)	(356,716,638)
Total stockholders' equity	137,243,213	151,008,029
Total liabilities and stockholders' equity	$ 168,218,792	$ 181,291,657

ALDEYRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(UNAUDITED)

	Three Months Ended March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 11,235,861	$ 12,234,320
General and administrative	5,567,416	4,249,387
Loss from operations	(16,803,277)	(16,483,707)
Other income (expense):		
Interest income	1,678,885	101,382
Interest expense	(491,287)	(405,967)
Total other income (expense), net	1,187,598	(304,585)
Net loss	$ (15,615,679)	$ (16,788,292)
Net loss per share - basic and diluted	$ (0.27)	$ (0.29)
Weighted average common shares outstanding - basic and diluted	58,791,603	58,297,861

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Source: Aldeyra Therapeutics, Inc.