Aldeyra Therapeutics Announces Positive Results from Phase 2 Clinical Trial in Alcohol-Associated Hepatitis, Focuses RASP Product Candidate Pipeline on Next-Generation Molecules

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- Relative to Baseline, Signal-Finding Molecule ADX-629 Demonstrated Statistically Significant Improvement in Liver Function in Patients with Alcohol-Associated Hepatitis
- RASP Modulator Product Candidate Pipeline Focused on ADX-248 and ADX-246 for Dermatologic, Metabolic, and Retinal Immune-Mediated Diseases
- Cash Runway Extended into the Second Half of 2027

LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 28, 2025-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra), a biotechnology company devoted to discovering and developing innovative therapies designed to treat immune-mediated diseases, today announced achievement of statistically significant improvement in liver function in patients treated with ADX-629, an investigational new drug candidate, and focused the RASP modulator product candidate pipeline on next-generation molecules ADX-248 and ADX-246.

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ADX-629, a signal-finding RASP modulator for proof-of-concept clinical testing, was administered orally for one month in a single-arm, multicenter Phase 2 clinical trial in four patients with mild to moderate alcohol-associated hepatitis. Relative to baseline, statistically significant improvement was observed in clinically relevant objective markers of hepatic function and inflammation, including the Model for End-Stage Liver Disease (MELD) score ($P=0.001$), triglyceride levels ($P<0.0001$), and levels of C-Reactive Protein ($P<0.0001$). No serious adverse events were reported, and no adverse events were deemed related to ADX-629.

“Consistent with clinical trials in patients with atopic dermatitis, psoriasis, chronic cough, and asthma, the orally administered RASP modulator ADX-629, relative to baseline, demonstrated activity in improving liver function and reducing inflammation in patients with mild to moderate alcohol-associated hepatitis, a chronic and challenging disease that proves difficult to manage in many cases,” stated Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. “The positive results announced today, which we look forward to sharing in more detail in the future, mark the culmination of our clinical proof of concept with ADX-629, as we focus our pipeline on next-generation RASP modulators ADX-248 and ADX-246 for the treatment of immune-mediated diseases.”

The RASP modulator product candidate pipeline was updated:

- Based on Phase 1 clinical trial results generated to date in healthy volunteers, which suggest high levels of exposure following once-daily oral dosing, RASP modulator ADX-248 replaced ADX-743 for the treatment of metabolic inflammation, including obesity and hypertriglyceridemia; anticipated timing for the filing of an Investigational New Drug (IND) application was updated to 2026.
- Based on favorable results in an animal model of a dry form of age-related macular degeneration (dry AMD), RASP modulator ADX-246 replaced ADX-631 for the treatment of dry AMD; anticipated timing for the filing of an IND application was updated to 2026.
- Clinical development of ADX-629 was discontinued, pending further investigator-sponsored clinical testing in Sjögren-Larsson Syndrome, a RASP-mediated inborn error of metabolism.

As a result of the pipeline updates, projected operational runway based on cash, cash equivalents, and marketable securities has been extended into the second half of 2027.

“In addition to our late-stage, pre-commercial programs in dry eye disease, allergic conjunctivitis, primary vitreoretinal lymphoma, and retinitis pigmentosa, Aldeyra is committed to developing a robust pipeline of novel therapeutics in a fiscally prudent manner to maintain growth,” continued Dr. Brady.

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 protein 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-248, ADX-246, and chemically related molecules for the potential treatment of systemic and retinal immune-mediated diseases. Our late-stage product candidates are reproxalap, a RASP modulator for the potential treatment of dry eye disease and allergic conjunctivitis, and ADX-2191, a novel formulation of intravitreal methotrexate for the potential treatment of primary vitreoretinal lymphoma and retinitis pigmentosa.

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 goals, opportunity, and potential for Aldeyra's RASP modulator product candidates and pipeline; the outcome and timing of any clinical trials of Aldeyra's RASP modulator product candidates; anticipated timing of regulatory filings; 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, issuing a complete response letter, or requiring additional clinical trials or data prior to review or approval of such filings or in connection with resubmissions 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; 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, 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, 2024, and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC 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 September 30, 2025, expected to be filed with the SEC in the fourth quarter of 2025, and Aldeyra's other filings with the SEC.

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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