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## **Aldeyra Therapeutics Presents Evidence for Aldehyde Sequestration as a Potential Therapeutic Approach in Succinic Semialdehyde Dehydrogenase Deficiency at the American Society of Human Genetics 2017 Annual Meeting**

### **ADX-102 Reduces Toxic Levels of Gamma-Hydroxybutyrate and Gamma-Aminobutyrate in Brain Tissue from Knockout Mouse Model**

LEXINGTON, Mass., Oct. 24, 2017 /PRNewswire/ -- Aldeyra Therapeutics, Inc. (NASDAQ: ALDX) (Aldeyra), a clinical-stage biotechnology company devoted to treating inflammation, inborn errors of metabolism, and other diseases related to endogenous aldehyde toxicity, presented novel data at the American Society of Human Genetics 2017 Annual Meeting demonstrating that ADX-102 reduced levels of gamma-hydroxybutyrate (GHB) and gamma-aminobutyrate (GABA) in a knockout mouse model of Succinic Semialdehyde Dehydrogenase (SSADH) Deficiency.

SSADH Deficiency is an orphan neurological disease caused by mutations in SSADH, resulting in elevated levels of succinic semialdehyde. Excess succinic semialdehyde is converted into GHB, GABA, and other metabolites that lead to severe neurological dysfunction, including cognitive delay, seizures, and motor dysfunction. Excess levels of GHB (also known as sodium oxybate, a drug marketed for psychiatric disorders and used illicitly as an intoxicant) and GABA impair neuronal transmission. A small number of patients with a diagnosis of autism have been found to have SSADH Deficiency.

"SSADH Deficiency is a debilitating orphan neurological disease with no approved therapy," commented Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. "The results presented last week suggest the potential therapeutic applicability of aldehyde traps as a novel approach to treating SSADH Deficiency by sequestering succinic semialdehyde and other toxic aldehydes."

The data presented at the American Society of Human Genetics 2017 Annual Meeting showed analyses of GHB and GABA levels in the brains of SSADH knockout mice. The results demonstrated that ADX-102 diminished brain levels of GHB and GABA, and thus serve as proof-of-concept for the utility of aldehyde sequestration as a potential treatment for SSADH Deficiency.

#### ***About Succinic Semialdehyde Dehydrogenase Deficiency***

Succinic Semialdehyde Dehydrogenase (SSADH) Deficiency is a neurological disease caused by genetic mutations that result in elevated levels of succinic semialdehyde, which are toxic and ultimately result in severe neurological dysfunction, including motor dysfunction, seizures, speech impairment, intellectual disability, and autistic behavior. There is currently no FDA-approved therapy for SSADH Deficiency.

#### ***About Aldeyra Therapeutics***

Aldeyra Therapeutics, Inc. is a biotechnology company devoted to improving lives by inventing, developing and commercializing products that treat diseases thought to be related to endogenous aldehydes, a naturally occurring class of pro-inflammatory and toxic molecules. Aldeyra's lead product candidate, ADX-102, is an aldehyde trap in development as topical eye drops for the treatment of ocular inflammation. ADX-102 has now been tested in over 250 patients in Phase 2 clinical trials in dry eye disease, allergic conjunctivitis, and noninfectious anterior uveitis. A dermatologic form of ADX-102 is in late-stage clinical development for the treatment of ichthyosis due to Sjögren-Larsson Syndrome, an inborn error of aldehyde metabolism. ADX-102 has not been approved for sale in the U.S. or elsewhere.

#### ***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 the development of ADX-102 and the timing thereof; and the potential of ADX-102 as an agent for the treatment of Succinic Semialdehyde Dehydrogenase Deficiency (SSADHD). 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 "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "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. 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, the ability to obtain and maintain regulatory approval to conduct clinical trials and to commercialize Aldeyra's product candidates, and the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing Aldeyra's product candidates; the size and growth of the potential markets for Aldeyra's product candidates and the ability to serve those markets; Aldeyra's expectations regarding Aldeyra's expenses and revenue, the sufficiency 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 ability to establish and maintain development partnerships; 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, 2016 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at [www.sec.gov](http://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, 2017, to be filed with the SEC in the fourth quarter of 2017. 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.

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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