



August 9, 2016

## **Aldeyra Therapeutics Announces Positive Results from a Randomized, Double-Blind, Vehicle-Controlled Clinical Trial of Topical Dermatologic NS2 in Patients With Sjögren-Larsson Syndrome**

### **Data Demonstrate Superiority of NS2 over Vehicle in Achieving Clinically Meaningful Improvement All NS2-Treated Patients Demonstrated Improvement During Therapy Dermal Biomarker Changes Consistent with Biochemical Modification of Disease**

LEXINGTON, MA -- (Marketwired) -- 08/09/16 -- Aldeyra Therapeutics, Inc. (NASDAQ: ALDX) (Aldeyra), a biotechnology company focused on the development of products to treat diseases related to aldehydes, today reported positive data from a randomized, parallel-group, double-blind, vehicle-controlled, multi-center clinical trial of topical dermatologic NS2, a novel aldehyde trap, for the treatment of the dermal manifestations of Sjögren-Larsson Syndrome (SLS).

"The results of this randomized and controlled clinical trial represent a significant advance in the effort to develop a treatment for SLS," said Dr. William Rizzo, the principal investigator of the trial and a leading expert in SLS. "The data from this trial suggest that NS2, when applied topically to the skin, has the potential to help patients suffering from SLS by lowering toxic aldehyde levels and preventing the dermal dysfunction that causes ichthyosis. I am excited about these results, and I am particularly excited for SLS patients and their caregivers."

Patients with SLS, a rare inborn error of aldehyde metabolism, suffer from ichthyosis, a debilitating dermatologic disease characterized by dry, thickened, scaly skin. The symptoms of SLS are thought to be related to high levels of toxic fatty aldehydes due to genetic mutations in fatty aldehyde dehydrogenase, an enzyme critical for the normal function of skin and other organs. NS2 is specifically designed to reduce levels of toxic aldehydes, and is the first therapy to be directed at the putative cause of SLS.

Twelve subjects with SLS and moderate to severe ichthyosis were randomized equally to receive NS2 1.0% dermatologic formulation or vehicle formulation administered once-daily (QD) on a 4 x 10 inch area of skin for two months. Investigators and subjects were blinded to treatment group. Ichthyosis was graded by a blinded central review of digital photographs, as well as by clinical exam, using the Ichthyosis Severity Score, which is comprised of assessments of global impression, scaling, erythema (redness), lichenification (thickness) and excoriation (abrasion). In addition, dermal tissue from subjects was analyzed for biomarkers relevant to fatty aldehyde dehydrogenase deficiency.

NS2 consistently produced clinically meaningful effects in reducing the severity of ichthyosis. As assessed by central review, five of six subjects (83%) treated with NS2 achieved a rating of "almost clear" or "mild" on global assessment. Six of six (100%) subjects treated with NS2 improved over the course of therapy as assessed by central review, and the improvement was greater than that observed with vehicle-treated patients ( $p < 0.05$ ). For NS2-treated subjects, mean reductions in ichthyosis severity were greater after 8 weeks of therapy than after 4 weeks of therapy, suggesting a disease modifying effect of NS2. Consistent with the clinical activity of NS2, significant dermal biomarker changes were noted in NS2-treated patients but not vehicle-treated patients ( $p < 0.004$ ). Cholesterol, which is significantly elevated in the skin of SLS patients, was reduced in NS2-treated patients more than in vehicle-treated patients ( $p < 0.001$ ). NS2 was observed to be generally well tolerated and there were no significant adverse events, serious adverse events or discontinuations in the trial.

"We are thrilled to report positive data from this rigorous and controlled clinical trial studying the use of topical dermatologic NS2 to treat the severe skin disease associated with SLS," said Todd C. Brady, M.D., Ph.D., President and CEO of Aldeyra. "There is currently no FDA-approved therapy for SLS, and we believe these results represent a major milestone for patients afflicted with this disease."

#### **Conference Call and Webcast Information**

The Company will hold a conference call on Tuesday, August 9, 2016 at 8:00 a.m. EDT to discuss the results of the clinical trial. The dial-in numbers are 1-888-296-4215 for domestic callers and 1-719-325-2480 for international callers. The conference ID number for both is 4144869. A live webcast of the conference call and accompanying slides will also be available on the investor relations page of the Aldeyra Therapeutics corporate website at [www.aldeyra.com](http://www.aldeyra.com).

After the live webcast, the call will remain archived on the Aldeyra Therapeutics website for one year. In addition, a telephonic replay of the call will be available until August 8, 2017. The replay dial-in numbers are 1-888-203-1112 for domestic callers and 1-719-457-0820 for international callers. Please use event passcode 4144869.

### ***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, NS2, is an aldehyde trap in development for ocular inflammation, as well as for Sjögren-Larsson Syndrome and Succinic Semi-Aldehyde Dehydrogenase Deficiency, two inborn errors of aldehyde metabolism. NS2 has not been approved for sale in the U.S. or elsewhere.

### ***About Sjögren-Larsson Syndrome***

Sjögren-Larsson Syndrome is a rare inborn error of aldehyde metabolism caused by mutations in fatty acid aldehyde dehydrogenase, leading to elevated toxic fatty aldehyde levels that are thought to contribute to ichthyosis (scaly, thickened, dry skin), neurological disorders, and retinal disease. There is no therapy for SLS that has been approved by the U.S. Food and Drug Administration.

### ***About NS2***

NS2 is an aldehyde-binding small molecule based on an innovative platform technology focused on trapping aldehydes, which are toxic and pro-inflammatory mediators of numerous diseases. By decreasing aldehyde load, NS2 may mitigate excessive inflammation and address diseases where aldehyde metabolism is impaired, including certain inborn errors of metabolism.

### ***Safe Harbor Statement***

This release contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, including statements regarding Aldeyra's plans for its product candidates. 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 commencement, enrollment and completion of Aldeyra's clinical trials, the timing and success of preclinical studies and clinical trials conducted by Aldeyra and its development partners; 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 use or 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, 2015 and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, 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 set forth in Aldeyra's Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, to be filed with the SEC in the third quarter of 2016.

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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