

October 2019

**CANTOR GLOBAL HEALTHCARE CONFERENCE** 

Innovating
Transformative Therapies



#### Disclaimers and Forward-Looking Statements

This presentation and various remarks which may be made during this presentation contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 and Section 21E of the Securities Exchange Act of 1934, as amended, including statements regarding Aldeyra's possible or assumed future results of operations, expenses and financing needs, business strategies and plans, research and development plans or expectations, trends, the structure, timing and success of Aldeyra's planned or pending clinical trials, expected milestones, market sizing, pricing and reimbursement, competitive position, regulatory matters, industry environment and potential growth opportunities, among other things. Forward-looking statements include all statements that are not historical facts and, in some cases, can be identified by terms such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "target," "design," "estimate," "predict," "potential," "plan" or similar expressions and the negatives of those terms.

Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause Aldeyra's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These statements reflect Aldeyra's current views with respect to future events and are based on assumptions and subject to risks and uncertainties, including the development, clinical and regulatory plans or expectations for Aldeyra's product candidates and Aldeyra's continuing review and quality control analysis of clinical data. Important factors that could cause actual results to differ materially from those reflected in Aldeyra's forward-looking statements are described in Aldeyra's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q, as well as Aldeyra's subsequent filings with the Securities and Exchange Commission. 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, completion, or reporting 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 presentation is provided only <u>as of October 04, 2019</u>, and Aldeyra undertakes no obligation to update any forward-looking statements contained in this presentation on account of new information, future events, or otherwise, except as required by law.



#### Our Mission and Value Proposition

**Developing Next-Generation Medicines to Improve the Lives of Patients** with Immune-Mediated Diseases





**Near-Term** Development **Catalysts** support path to commercialization



Solid Track **Record** of development success



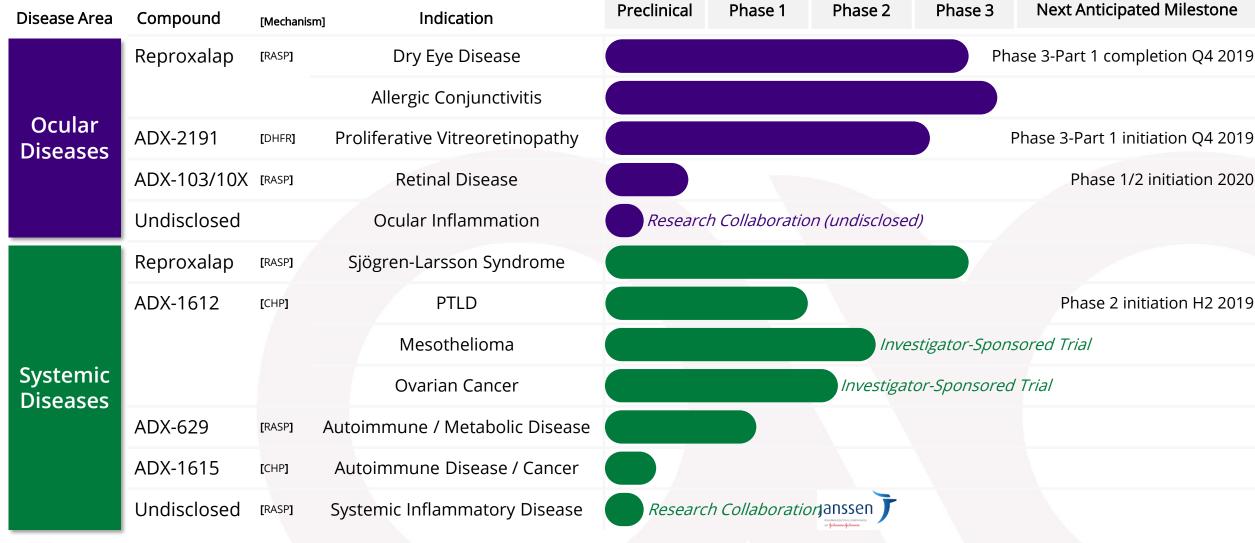
Large Market Potential of latestage pipeline

#### Solid Cash **Position**

Cash, cash equivalents and marketable securities were \$69.5 million as of June 30, 2019



#### Deep and Innovative Pipeline Focused on Immune-Mediated Diseases





## Our Lead Programs Represent Compelling Commercial Opportunities

**Late Stage Programs Current Standard Drug Candidate Estimated Potential** U.S. Population<sup>†</sup> Competitive Advantages † of Care and Dev. Stage Ocular Diseases Reproxalap: Rapid onset, broad activity, 20 million DED **Dry Eye Disease** Xiidra®, Restasis® Phase 3 reduction in itch Up to 10 million with DED & AC Allergic Reproxalap: Non-drying, durable activity; **Antihistamines** 30 million AC Conjunctivitis Phase 3 Responder superiority vs. vehicle **Proliferative** ADX-2191: Clinically demonstrated activity; None 4,000 Currently no FDA- or EMA-approved therapy Vitreoretinopathy Phase 3 (repeat surgeries) Systemic Diseases Sjögren-Larsson Reproxalap: Clinically demonstrated activity: None 1,000 **Syndrome** Phase 3 Currently no FDA- or EMA-approved therapy (manage symptoms)



<sup>&</sup>lt;sup>†</sup>Pending clinical data, regulatory discussions, payor negotiations, competition, potential legislative changes, and other factors, which may not be in Aldeyra's control. Preliminary assumptions are subject to change.

### Upcoming and Recently Achieved Development Milestones:\* Novel Approaches to Address Immune-Mediated Disease

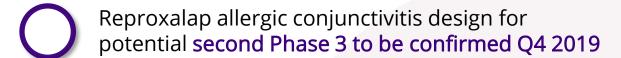
O = Ocular Diseases

O = Systemic Diseases





ADX-629 systemic Phase 1 clinical trial initiation H2 2019



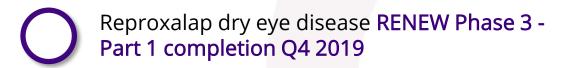


Reproxalap Sjögren-Larsson Syndrome RESET Phase 3 - Part 1 completion Q2 2019





Positive reproxalap allergic conjunctivitis environmental chamber trial results June 2019





Reproxalap dry eye disease RENEW Phase 3 - Part 1 clinical trial initiation April 2019



Positive reproxalap allergic conjunctivitis ALLEVIATE Phase 3 trial results March 2019



# aldeyra

October 2019

#### **CANTOR GLOBAL HEALTHCARE CONFERENCE**

### Ocular Disease Area

- DRY EYE DISEASE
- ALLERGIC CONJUNCTIVITIS
- PROLIFERATIVE VITREORETINOPATHY

#### Dry Eye Disease: A Persistently Disturbing Condition Inadequately Treated with Currently Available Therapy

#### **Dry Eye Disease**

#### Reproxalap



20 million or more adults in the 🗼 🕦 U.S. suffer from DED



Current Rx options may require up to six weeks or longer to achieve even modest efficacy



DED increases with age, with those over age 50 three times more likely to suffer from DED



Up to 75% of patients with DED are not satisfied with current prescription options



Women are twice as likely to suffer from DED than men



Up to 50% of patients treated for DED with current therapies fail and discontinue

#### Reproxalap in DED



Early and consistent symptom and sign improvements in Phase 2b clinical trial



Broad symptom and sign improvements in Phase 2b clinical trial

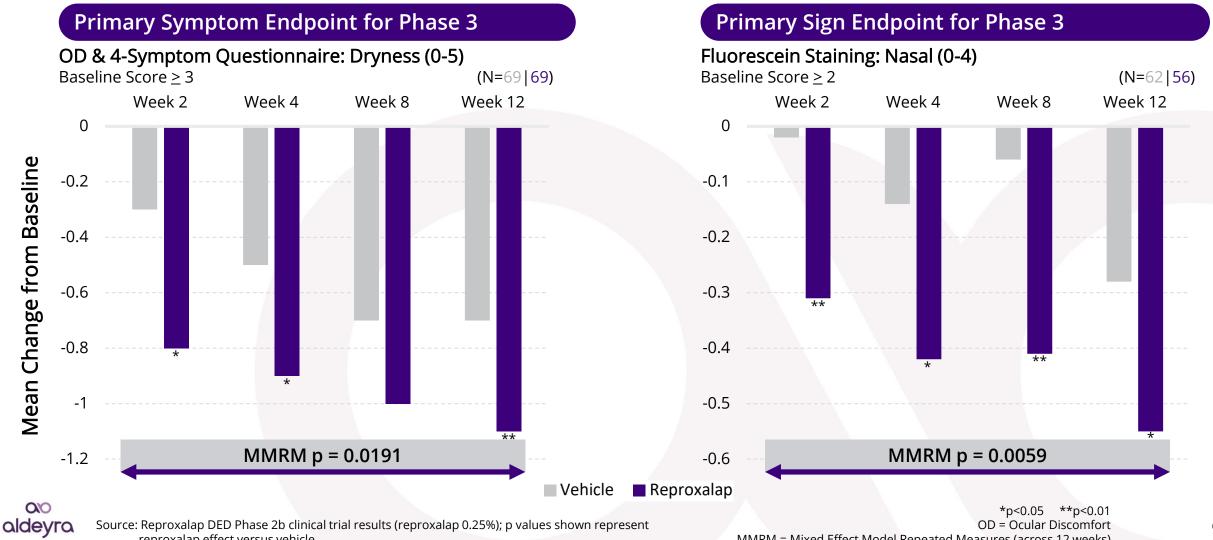


Significant **negative** quality of life impact

**Underserved Patient Population** 

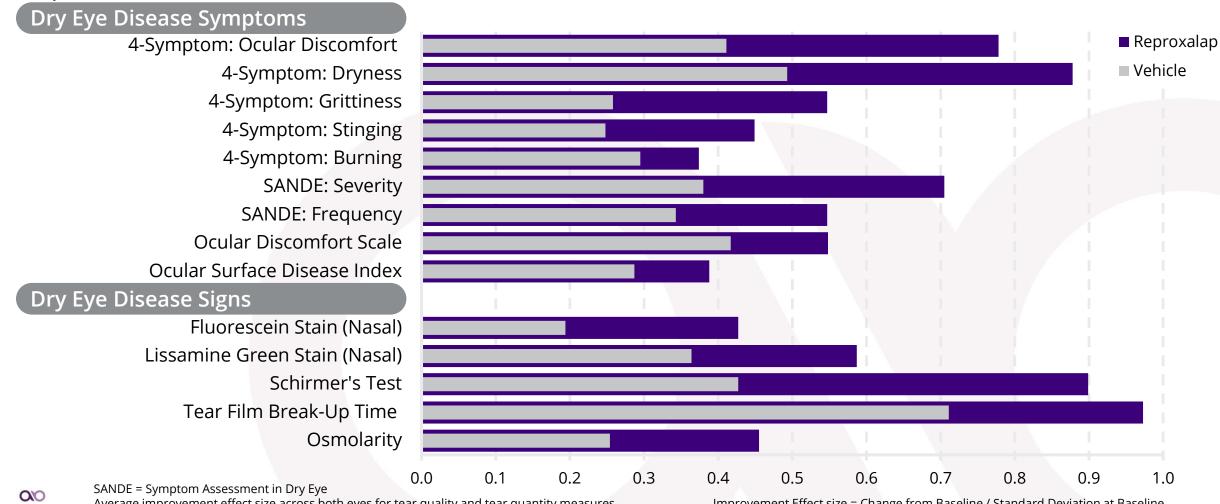


#### Phase 3 Dry Eye Disease Symptom and Sign Endpoints Achieved in Phase 2b Clinical Trial



### Broad Drug Activity Across All Measured Dry Eye Disease Symptoms and Signs in Phase 2b Clinical Trial Supports Differentiated Product Profile

#### Improvement Effect Size at Week 12





#### Adaptive Phase 3 Dry Eye Disease Clinical Program

#### **Adaptive Phase 3 Program**



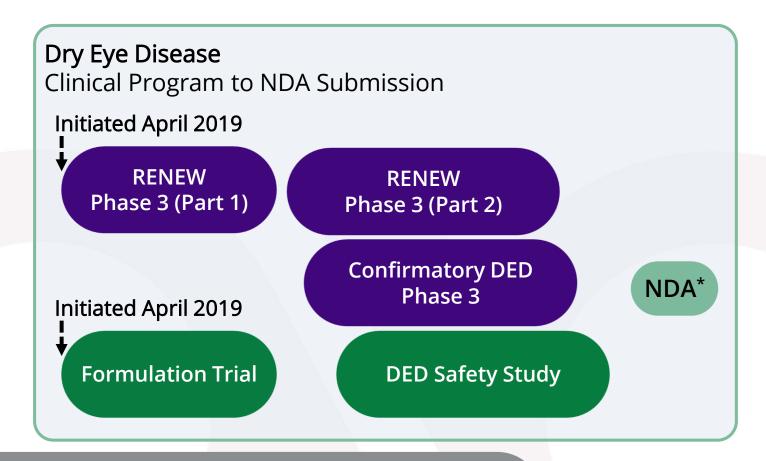
Confirm symptom and sign endpoints from Phase 2b trial



Confirm dosing regimen (QID vs. QID to BID taper)



Confirm sample size for subsequent trial



Adaptive design, co-primary endpoints, and innovative analysis strategy confirmed with FDA at EOP2 Meeting



### The RENEW Phase 3 Clinical Trial in Dry Eye Disease Part 1 Initiated April 2019

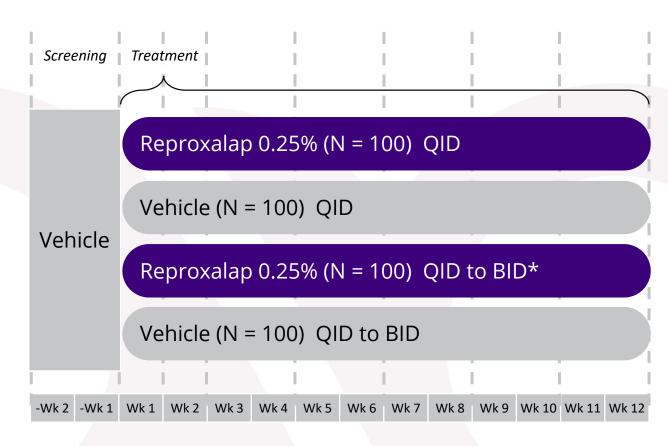
#### Primary objective:

 Evaluate efficacy of reproxalap ophthalmic solution (0.25%) vs. vehicle to confirm dosing regimen and sample size for Part 2

#### Inclusion/exclusion criteria:

- Same as used for Phase 2b
- Moderate to severe dry eye disease
- Co-primary endpoints:
  - Ocular dryness score (0-100mm VAS) and fluorescein nasal region staining
- Analysis strategy:
  - Both co-primary endpoints will be assessed using Mixed Model Repeated Measures (MMRM) from week 2 to 12
  - Both co-primary endpoints will be assessed in separate pre-specified patient populations
    - Ocular dryness score (OD4SS): baseline score of > 3
    - Fluorescein nasal staining: baseline score ≥ 2

#### Phase 3 Dry Eye Disease Clinical Trial: Part 1





# aldeyra

October 2019

#### **CANTOR GLOBAL HEALTHCARE CONFERENCE**

### Ocular Disease Area

- DRY EYE DISEASE
- ALLERGIC CONJUNCTIVITIS
- PROLIFERATIVE VITREORETINOPATHY

### Allergic Conjunctivitis: A Common Disease with Unmet Medical Need

#### **Allergic Conjunctivitis**



Up to 30 million of AC sufferers in the U.S. do not respond adequately to or are dissatisfied with antihistamines



Many AC patients make significant sacrifices due to lack of drug activity





Antihistamines are not effective in an estimated 24% of treated AC patients





~2% of AC patients have severe symptoms and **may be corticosteroid-dependent** 



Significant negative quality of life impact

**Underserved Patient Population** 

#### Reproxalap

#### Reproxalap in AC



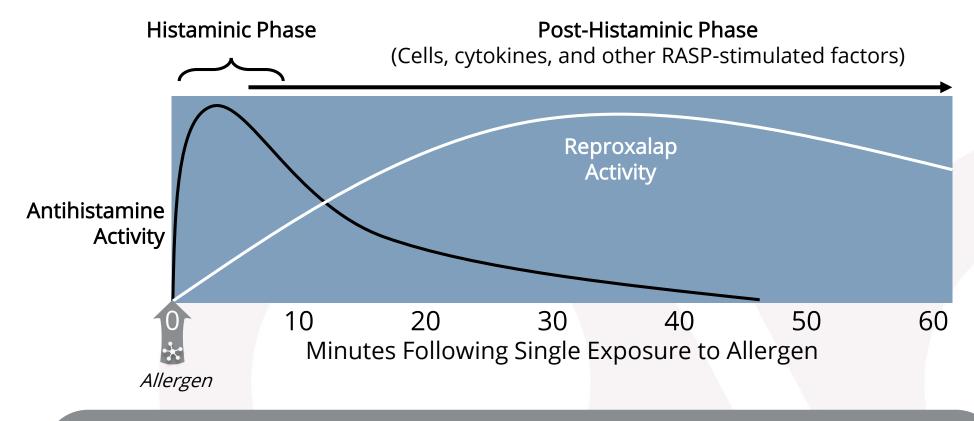
Clinically significant and durable symptom response in Phase 3 clinical trial



Active in post-histaminic allergy, for which no drug is approved



### Reproxalap's Novel Mechanism of Action has the Potential to Provide Differentiated Activity Versus Antihistamines



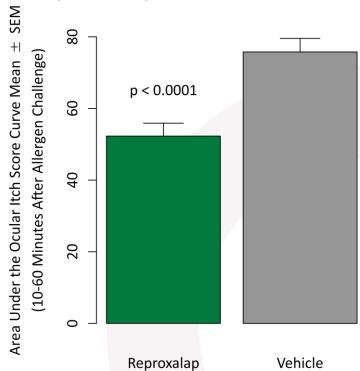
Reproxalap has the potential to be uniquely effective in posthistaminic allergy, which affects all allergic conjunctivitis patients



### Reproxalap Achieved Primary and Key Secondary Endpoints in ALLEVIATE Phase 3 Clinical Trial

#### **Primary Endpoint**

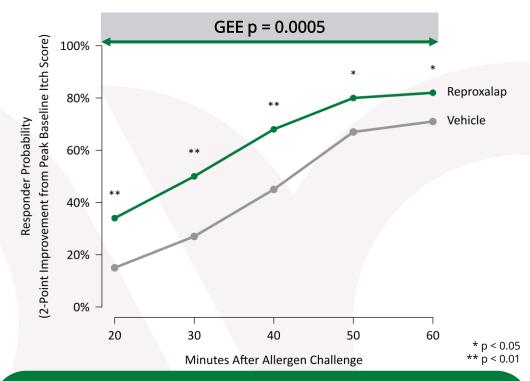
Area Under the Curve: Ocular Itch Score (0-4) 10 to 60 Minutes After Allergen Challenge



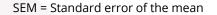
Improvement in itch score over one hour after allergen exposure statistically greater for reproxalap vs. vehicle

#### Key Secondary Endpoint

Probability of Two-Point Response: Ocular Itch Score (0-4)



Clinically significant response rate of reproxalap statistically higher than that of vehicle

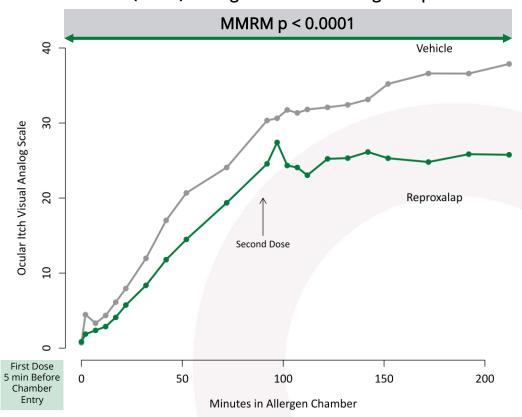


GEE = Generalized estimating equation analysis



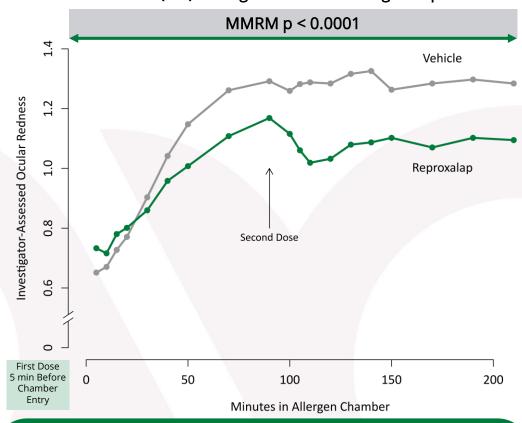
### Reproxalap Treatment Led to Durable Reduction in Ocular Itch and Redness in Allergen Chamber Clinical Trial

Ocular Itch Score (0-100) During 3.5 Hours of Allergen Exposure



Statistically significant reduction in ocular itch vs. vehicle for more than three hours of exposure to allergen

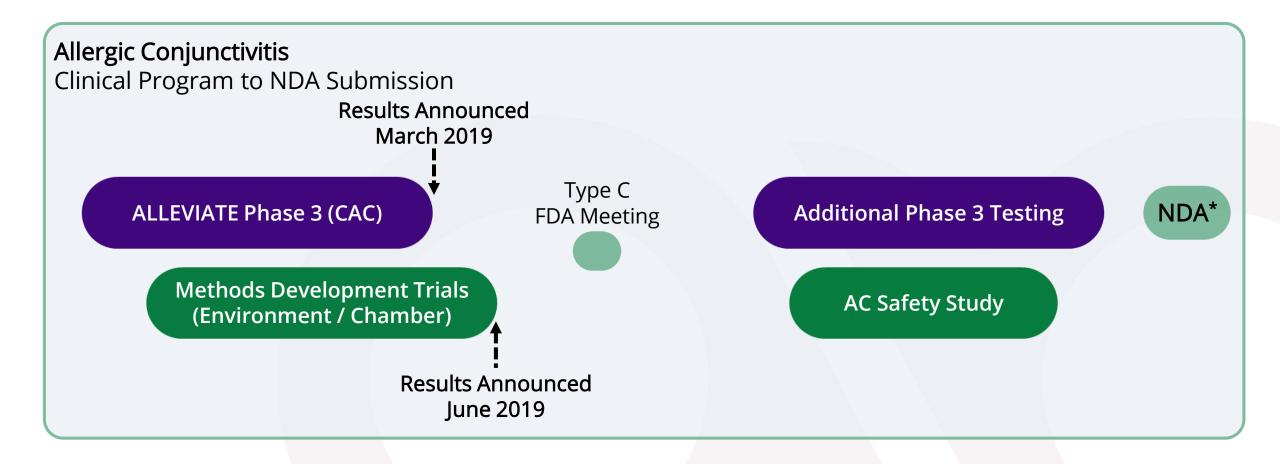
Ocular Redness Score (0-4) During 3.5 Hours of Allergen Exposure



Statistically significant reduction in ocular redness vs. vehicle for more than three hours of exposure to allergen



#### Allergic Conjunctivitis Phase 3 Clinical Program





# aldeyra

October 2019

#### **CANTOR GLOBAL HEALTHCARE CONFERENCE**

### Ocular Disease Area

- DRY EYE DISEASE
- ALLERGIC CONJUNCTIVITIS
- PROLIFERATIVE VITREORETINOPATHY

# Proliferative Vitreoretinopathy: A Rare Sight-Threatening Retinal Disease With No Approved Therapy

#### Proliferative vitreoretinopathy

ADX-2191



PVR is a **rare disease**, with ~4,000 patients per year in the U.S. and nearly twice as many in Europe and Japan.



Left untreated, retinal detachment due to PVR can progress to permanent blindness.



No FDA- or EMA-approved therapy



Repeat surgery, which can lead to vision loss, is currently the only possible course of action.

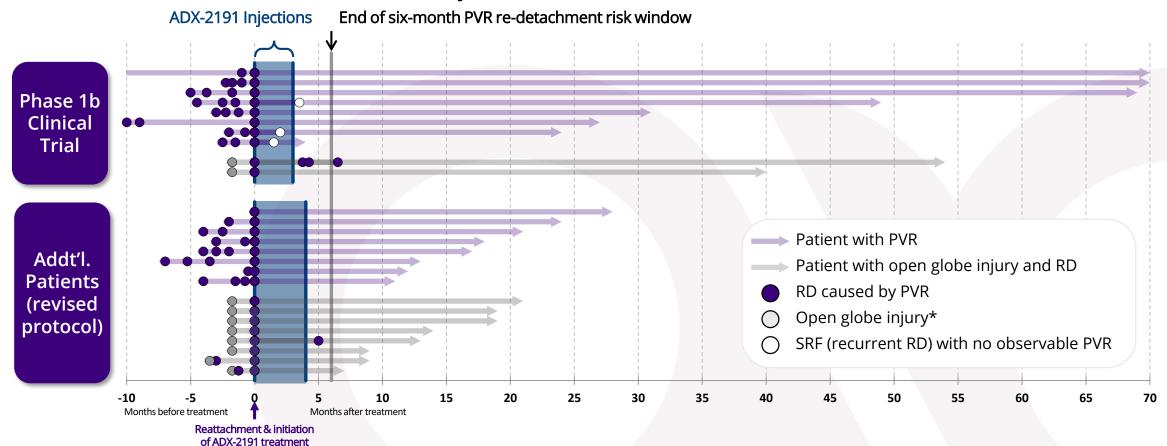
#### ADX-2191

- A novel approach and potential therapeutic breakthrough in PVR treatment
- Granted U.S. orphan designation for the prevention of PVR
- Tolerability and reattachment success during study period demonstrated in Phase 1b open-label investigator sponsored clinical trial
- GUARD adaptive Phase 3 clinical trial expected to initiate Q4 2019



## ADX-2191 Reduced Recurrent Retinal Detachment in Investigator Sponsored Phase 1b Clinical Trial and in Additional In-Practice Use

#### Retinal Detachments Over Time by Patient





RD = Retinal detachment PVR = Proliferative vitreoretinopathy SRF = Subretinal fluid

# ADX-2191: GUARD Trial Design in Proliferative Vitreoretinopathy Adaptive Phase 3 (Part 1) Clinical Trial Design\*

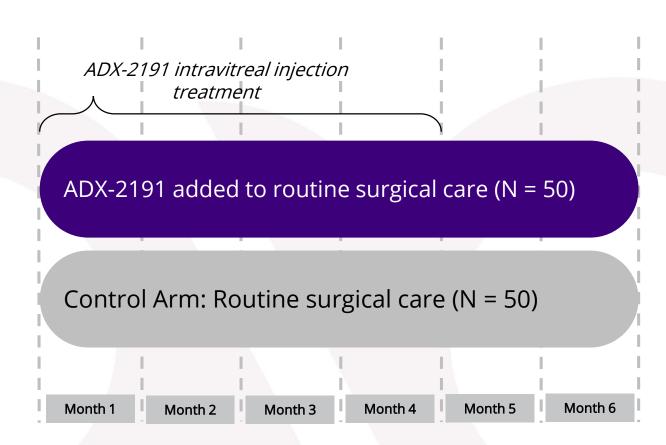
#### Primary objective:

 Evaluate efficacy of intravitreal ADX-2191 injections for prevention of recurrent retinal detachment due to proliferative vitreoretinopathy (PVR)

#### Design:

- Multi-center, randomized, controlled, two-part, adaptive Phase 3 clinical trial
- Inclusion highlights:
  - Recurrent retinal detachment due to PVR, or
  - Retinal detachment associated with open-globe injury
- Dosing regimen:
  - At surgery, weekly (x8), and then every other week (x4) intravitreal ADX-2191 injections
- Endpoint:
  - Retinal re-detachments due to PVR requiring reoperation within 6 months:
    - 1. OCT demonstrating fovea-off retinal detachment
    - 2. Photographic documentation retinal detachment

Adaptive Phase 3 PVR Clinical Trial Design: Part 1







October 2019

#### **CANTOR GLOBAL HEALTHCARE CONFERENCE**

# Upcoming and Recently Achieved Development Milestones

### Upcoming and Recently Achieved Development Milestones:\* Novel Approaches to Address Immune-Mediated Disease

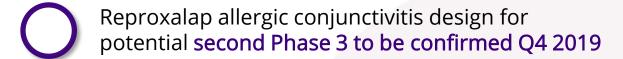
O = Ocular Diseases

O = Systemic Diseases





ADX-629 systemic Phase 1 clinical trial initiation H2 2019



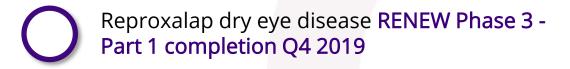


Reproxalap Sjögren-Larsson Syndrome RESET Phase 3 - Part 1 completion Q2 2019





Positive reproxalap allergic conjunctivitis environmental chamber trial results June 2019





Reproxalap dry eye disease RENEW Phase 3 - Part 1 clinical trial initiation April 2019



Positive reproxalap allergic conjunctivitis ALLEVIATE Phase 3 trial results March 2019





Innovating
Transformative Therapies