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Transforming Gene Therapy

1Q20 Call and Webcast | May 28, 2020

Agenda & Participants

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- Corporate update
- Progress advancing lead gene therapy candidate ADVM-022:
  - Data and enrollment highlights from OPTIC Phase 1 Clinical Trial of ADVM-022 in wet AMD
  - New INFINITY Phase 2 Clinical Trial of ADVM-022 in Diabetic Macular Edema
- Q&A

Participants

- Leone Patterson, President and Chief Executive Officer
- Aaron Osborne, MBBS, Chief Medical Officer
- Thomas Leung, Chief Financial Officer
- Myesha Lacy, Vice President, Investor Relations & Corporate Communications

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Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME): Significant, Growing Opportunity

Diabetic Retinopathy (DR)

- >30M people living with diabetes in the U.S. all at risk of DR[1]
- DR affects approximately one in three adults with diabetes[2]
- DR can be diagnosed at different severity levels, with a greater risk of vision loss at higher severity levels[3]
- DR is the most common cause of blindness in working age adults in the US[4,5]

Diabetic Macular Edema (DME)

- DME is a vision-threatening complication of DR that can occur at any severity stage of DR[11,12]
- The risk of DME increases with the higher severity of DR[13,14]
- DME is characterized by retinal thickening in the area of the macula[15]
- DME affects approximately 5% of people with diabetes[16,17]
- DME is the leading cause of vision loss in patients with DR[15]

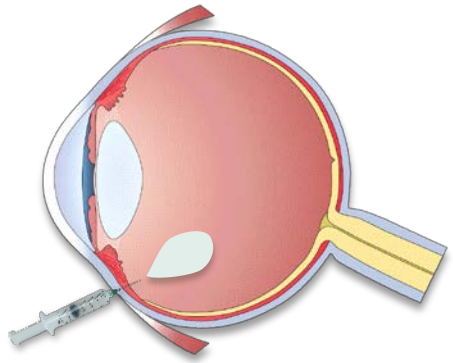
DME Market Overview

- Standard-of-care therapy is anti-VEGF intravitreal injections [6]
- Frequent and long-term injections needed to maintain vision[7]
- Real-world outcomes meaningfully worse than clinical trials[8]
- $11B global sales for approved anti-VEGF therapies[9]
- Wet AMD and DME are two largest indications[10]

ADVM-022 Gene Therapy

One-time Intravitreal Injection

- In-office delivery for durable expression of therapeutic protein
- Wide distribution of vector, thus able to transduce broader tissue area
- Produces codon-optimized aflibercept for efficient VEGF and PlGF inhibition



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VEGF= vascular endothelial growth factor, PlGF= placental growth factor

INFINITY: Phase 2 Trial of ADVM-022 in DME

Key Objectives and Eligibility Criteria

Key Objectives:

Primary

- Time to worsening of DME disease activity

Secondary

- Safety and tolerability
- Frequency of rescue aflibercept in study eye
- Change in visual acuity (BCVA) over time
- Change in central subfield thickness (CST) and macular volume over time
- Change in diabetic retinopathy severity score (DRSS) over time

Key Eligibility Criteria

- Age ≥ 18
- Controlled type 1 or 2 diabetes mellitus
- Vision impairment due to center-involving diabetic macular edema
- No prior laser treatment to the study eye
- No high-risk proliferative diabetic retinopathy (PDR) in the study eye
- No acute coronary syndrome, myocardial infarction or coronary artery revascularization, cerebrovascular accident, transient ischemic attack in last 6 months
- No current or planned pregnancy or breastfeeding

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INFINITY: Phase 2 Trial of ADVM-022 in Diabetic Macular Edema (DME)

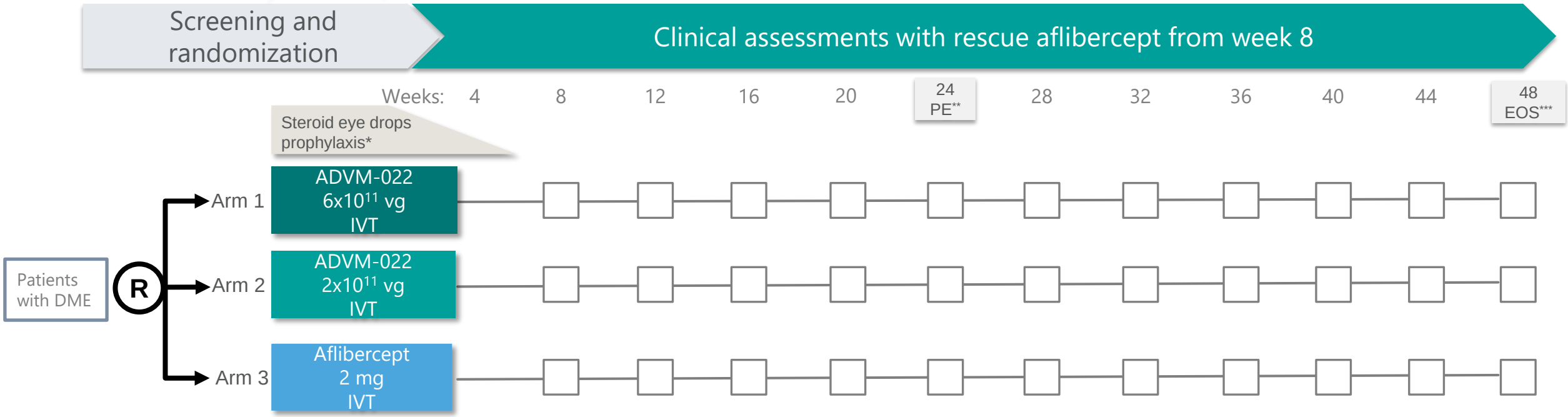
Multi-center, Randomized, Double-masked, Active Comparator-controlled

- Evaluate a single IVT injection of ADVM-022 for patients with DME
- Designed to demonstrate superior disease control compared to a single aflibercept injection, measured by time to worsening of DME disease activity
- Additional objectives include assessing treatment burden, visual acuity, retinal anatomy and safety outcomes
- Will enroll approximately 33 patients

Day 1: Aflibercept/Sham

Day 8: ADVM-022/Sham

Patients receive rescue aflibercept (2 mg IVT) if **either** of the following disease activity criteria are met:
1. Loss of >5 letters in BCVA from best prior BCVA, due to worsening DME disease activity
2. Increase in central subfield thickness (CST) >50 µm from best prior CST



R=Randomized
BCVA = Best-Corrected Visual Acuity
CST = Central Subfield Thickness

*All subjects receive a 7-week course of difluprednate eye drops, starting at QID and tapering to QD
**PE= Primary Endpoint assessment
***EOS= End of Study assessment

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