



Corporate Presentation

March 2025



Note regarding forward-looking statements

This presentation of Abeona Therapeutics Inc. (the “Company”) contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. Such forward looking statements are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. We have attempted to identify forward-looking statements by such terminology as “may,” “will,” “believe,” “anticipate,” “expect,” “intend,” “hope,” and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors and numerous risks and uncertainties, including, but not limited to, the timing and outcome of the FDA’s review of our resubmitted Biologics License Application for pz-cel, including the FDA’s assessment of our responses to the items identified in the Complete Response Letter relating to pz-cel received from the FDA; potential market opportunities and commercial launch strategies for pz-cel, if approved; the FDA’s grant of a Priority Review Voucher upon a potential approval of pz-cel; continued interest in our rare disease portfolio; the timing of studies or study manuscript submissions; our ability to enroll patients in clinical trials; the outcome of future meetings with the FDA or other regulatory agencies, including those relating to our preclinical programs; our ability to achieve or obtain necessary regulatory approvals; the impact of any changes in government, financial markets, and global economic conditions; risks relating to the decline in market price of our common stock after receipt of the Complete Response Letter; risks associated with data analysis and reporting; and other risks disclosed in the Company’s most recent Annual Report on Form 10-K and subsequent periodic reports filed with the Securities and Exchange Commission. The Company undertakes no obligation to revise forward-looking statements or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments, or otherwise, except as required by the federal securities laws.

All trademarks, service marks, and trade names of the Company or its affiliates used herein are trademarks, service marks, or registered trademarks of the Company. Any other product, company names, or logos mentioned herein are the trademarks and/or intellectual property of their respective owners.

This presentation discusses pz-cel and other investigational products that have not been approved by the FDA. Nothing in this presentation should be construed as pre-approval promotion of pz-cel or any other product.

Abeona is poised for growth

Anticipated value inflection milestones in 2025 and drivers for long-term growth

Prademagene zamikeracel (pz-cel):

Differentiated clinical profile, transformative treatment potential in Recessive Dystrophic Epidermolysis Bullosa (RDEB)

- April 29, 2025 PDUFA date
- Eligible for Priority Review Voucher
- US commercial launch

AAV-based gene therapy platform:

Preclinical POC in multiple eye diseases

Proprietary AAV capsids designed to improve tropism profiles for variety of diseases

Out-licensed gene therapies:

BLA for UX111 (ABO-102) in MPS IIIA accepted in February 2025 (Ultragenyx)

→ August 18, 2025 PDUFA date

Compelling early clinical data in Rett Syndrome (Taysha)

Recessive dystrophic epidermolysis bullosa (RDEB) overview

- Rare and debilitating monogenic skin disease
- Caused by mutations in both copies of *COL7A1* gene, resulting in lack of collagen VII, a protein necessary to keep skin intact
- Leads to fragile skin that blisters easily
- RDEB wounds can lead to serious life-threatening complications
 - Patients suffer from years of large painful wounds, itch and infection
 - High risk of squamous cell carcinoma (SCC)², systemic infection & sepsis
 - 76% likelihood of death by age 40³
- Lifelong burden on patients and caregivers

RDEB treatment goals

- Provide multi-year durable wound healing
- Provide long-term pain and itch relief
- Reduce risk of SCC



>30%
of patient's
body wounded¹

Designed to deliver durable benefit for RDEB patients

Pz-cel is the only investigational therapy designed to deliver long-lasting healing and pain reduction via a single application, even in tough large, chronic RDEB wounds.



Pz-cel offers uniquely differentiated value proposition as seen in clinical trials

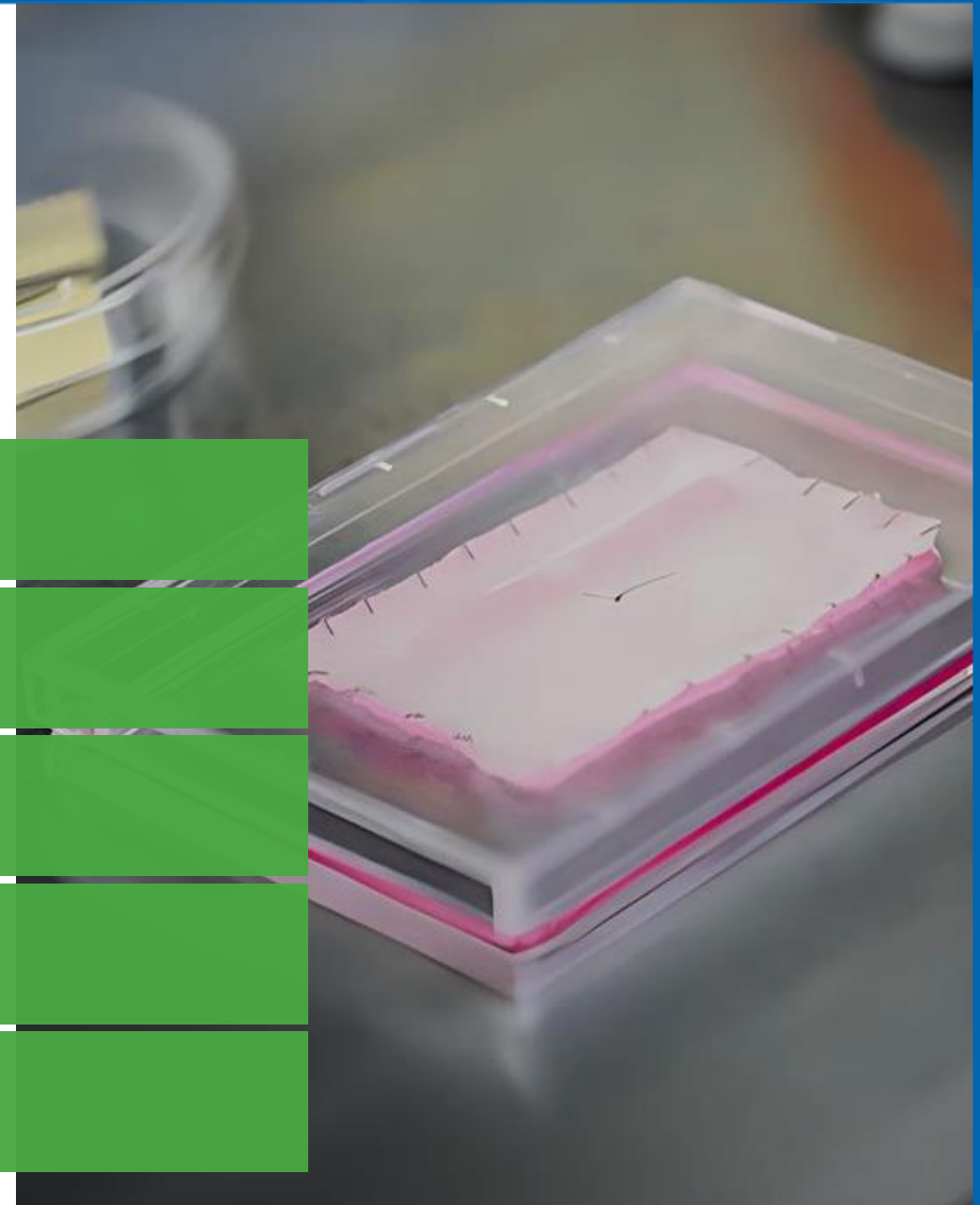
Healing in even tough large, chronic RDEB wounds^{1,2}

Multiple years of wound healing after single application²

Demonstrated significant pain reduction in wounds¹

Patient-reported improvements in itch and blistering²

Favorable long-term safety profile^{1,2}



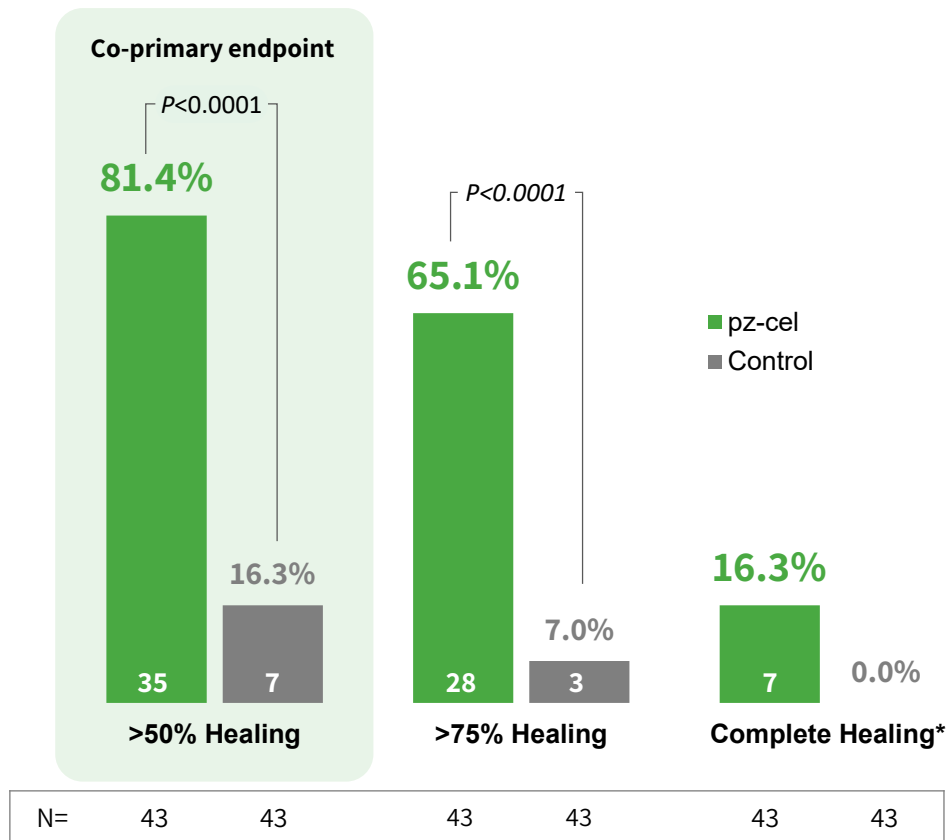
1. Phase 3 VIITAL study : 43 treated wounds vs matched control wounds in 11 patients with RDEB in a multicenter, randomized, inpatient-controlled study.

2. Phase 1/2a study : 38 treated wounds in 7 patients with RDEB in an open-label, single-arm study.

Significant wound healing and reduction in pain¹ reported with pz-cel

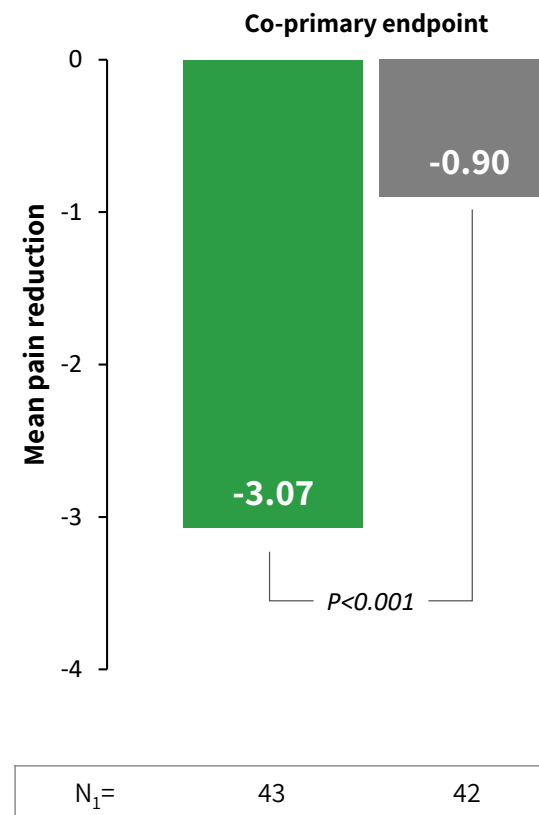
Wound healing

from baseline at Week 24



Pain reduction

from baseline at Week 24



Phase 3 VIITAL study topline results:

- Pz-cel evaluated in 43 randomized large chronic wound pairs and 14 non-randomized wounds in 11 patients with RDEB
- Baseline wounds >20 cm², open for 5 years (median)
- Met both co-primary endpoints for wound healing and pain reduction at six months
- Pz-cel was well-tolerated with no serious treatment-related adverse events observed, consistent with past clinical experience

Healed wounds at Week 24 were confirmed ≥ 2 weeks later to be included. Complete wound healing defined as re-epithelialization with no drainage or erosion and presence of only minor crusting.

1. Pain assessed using Wong-Baker FACES rating scale (0-10). Pain reduction calculated as difference between baseline and postbaseline pain scores.

Abbreviations: N, number of total wounds with non-missing healing improvement category; N₁, total number of wounds with non-missing pain reduction score.

Example RDEB wounds before & after pz-cel treatment

Baseline:



Upper left thigh

Week 24:



Upper left thigh

**Wounds
scored as at
least 75%
healing at
week 24**

Example RDEB wounds before & after pz-cel treatment

Baseline:



Upper trunk

Week 24:

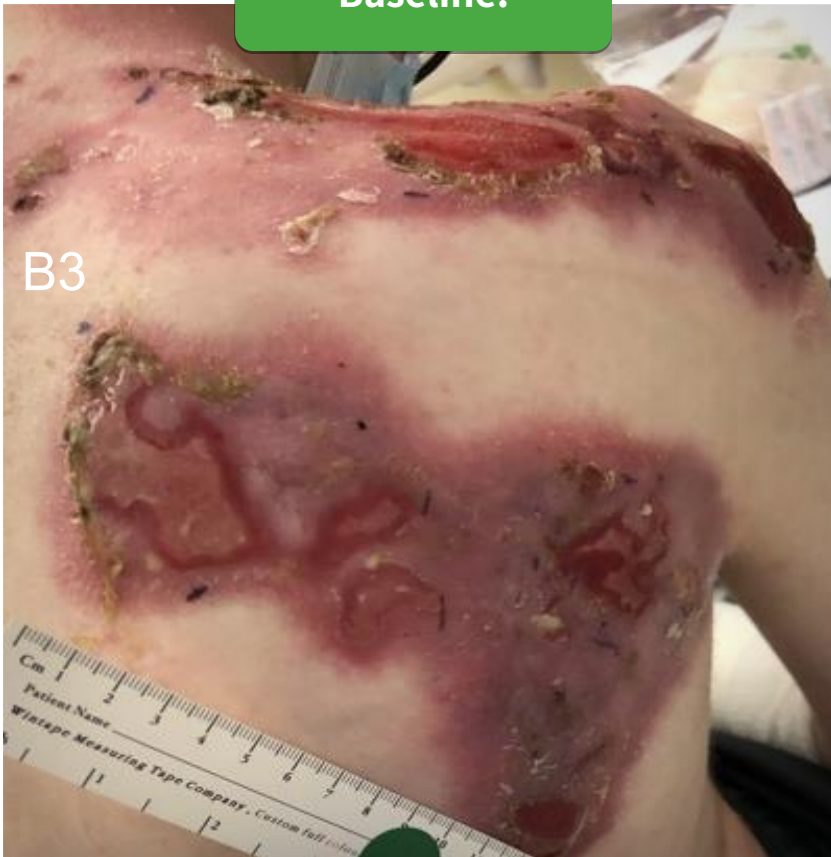


Upper trunk

B4 scored as at least 75% healing, E9 scored as complete wound healing at week 24

Example RDEB wounds before & after pz-cel treatment

Baseline:



Upper back

Week 24:



Upper back

**B3 scored as
at least 75%
healing at
week 24**

Example RDEB wounds before & after pz-cel treatment

Baseline:



Left flank

Week 24:

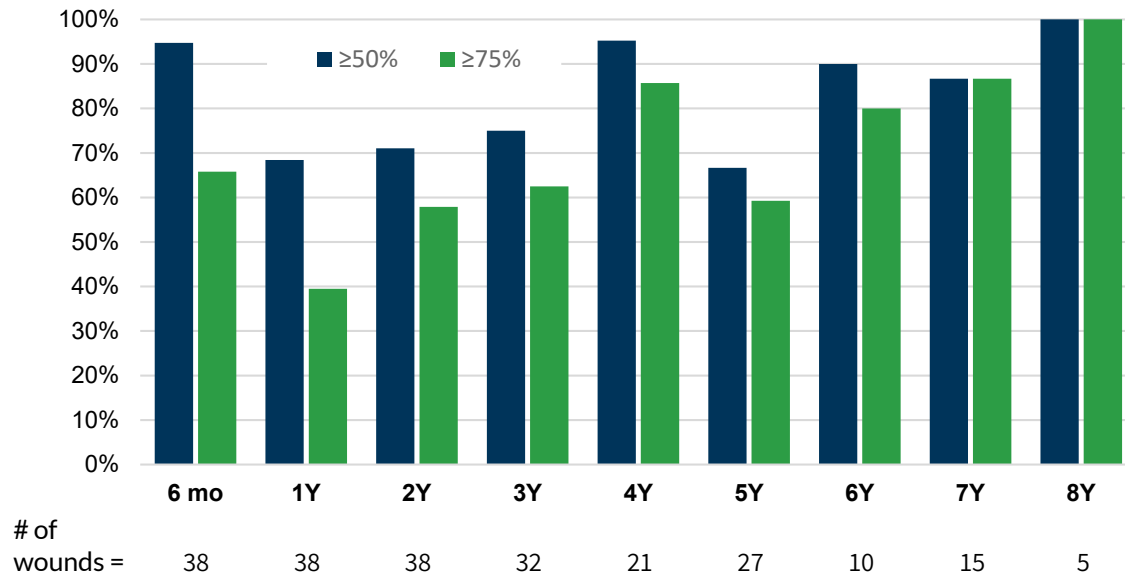


Left flank

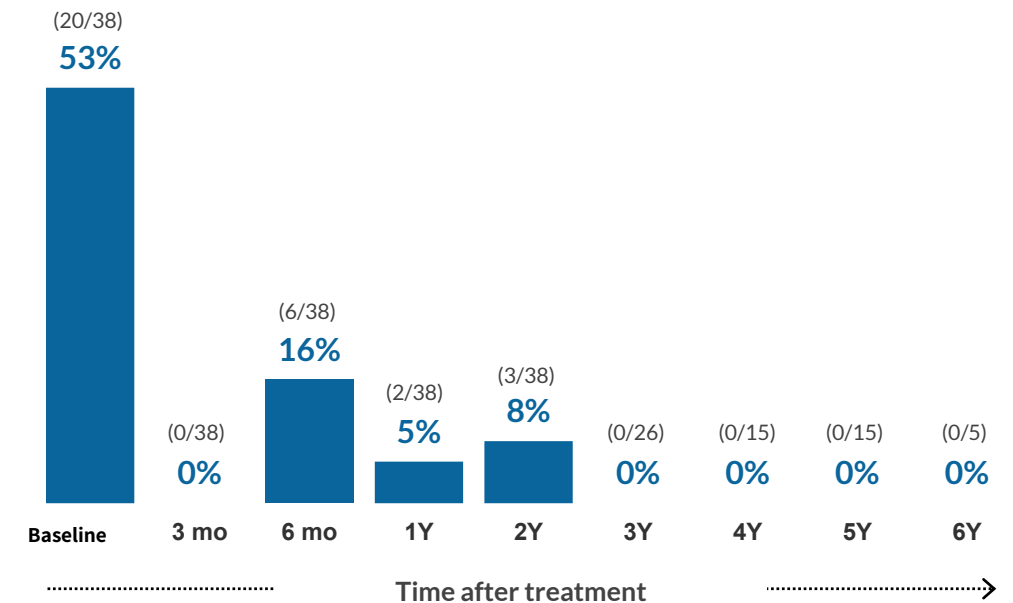
Wounds scored as at least 75% healing and complete wound healing at week 24

Multi-year wound healing and pain reduction after single application of pz-cel

% treated wounds with
≥50% or ≥75% healing



% treated wounds with pain
(n/N)



Phase 1/2a study

key patient characteristics:

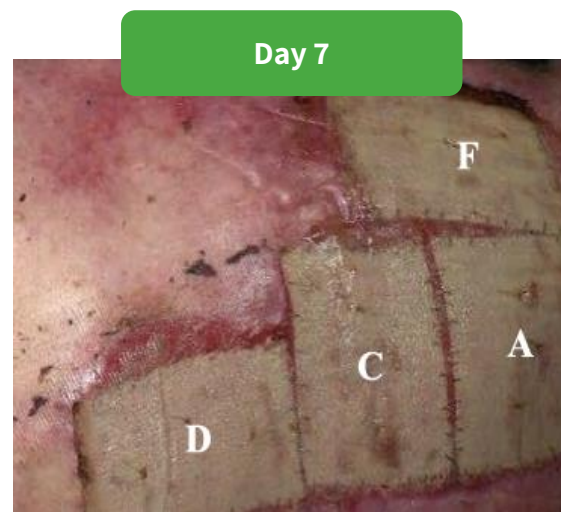
- Baseline wounds remained open for average of 11 years
- Average treated body surface area >100 cm²

Multi-year wound healing after one-time pz-cel application



Baseline

Upper back



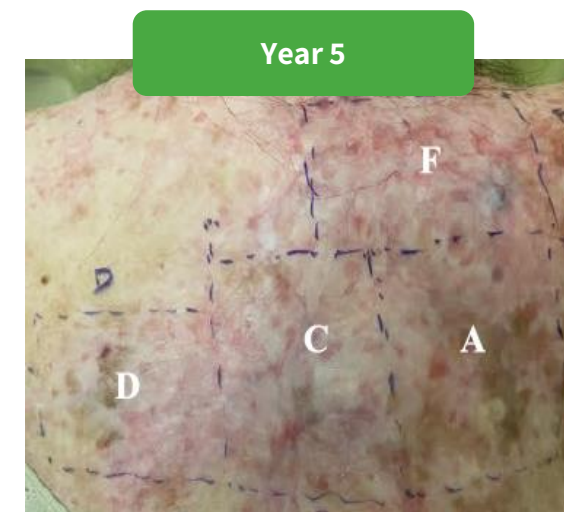
Day 7

Upper back



Year 2

Upper back

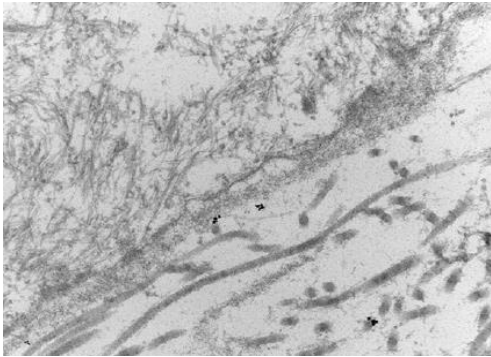


Year 5

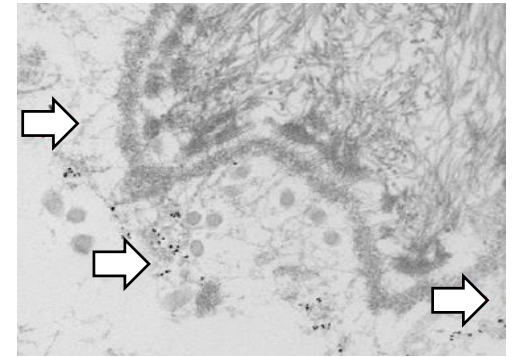
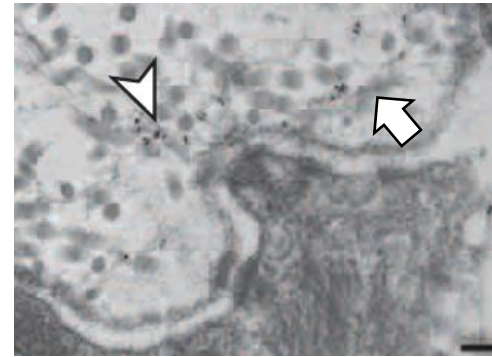
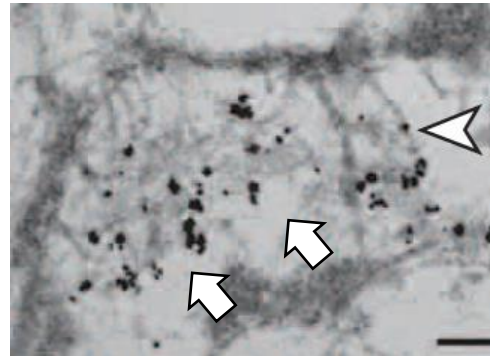
Upper back

Collagen VII protein expression observed for 24 months at a pz-cel treated site

No anchoring fibrils
Baseline



Anchoring fibril expression 2 years after pz-cel treatment
3 months Year 1 Year 2



No collagen VII expression
Baseline

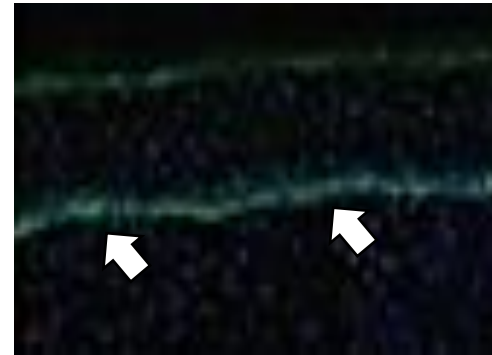


Collagen VII expression 2 years after pz-cel treatment

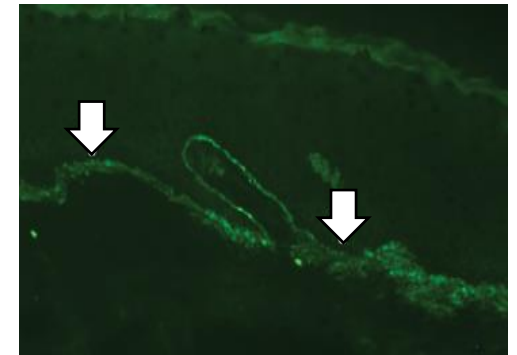
3 months



Year 1



Year 2



Pz-cel cumulative clinical trial experience

Durable wound healing and reduction in pain & itch

- **Wound healing and reduction of pain & itch** seen with pz-cel treatment, even in tough large, chronic RDEB wounds
- Sustained wound healing with a mean of **5.9 years** of follow-up from Phase 1/2a study¹

Favorable long-term safety profile

- **No serious adverse events (SAEs)** related to pz-cel (most common AE was pain related to surgery)^{1,2}
- **No squamous cell carcinoma (SCC)** at treated sites^{1,2} with longest follow-up time of 11 years (occurrences in non-treated sites)²
- **No positive replication competent retrovirus (RCR)**²

Study	# pz-cel patients	# wounds treated
Phase 1/2a	7	42
Phase 3	11	57
Phase 3b	3	21
Total	21	120

- Treated **multiple, different anatomical locations**, including anterior or lateral, posterior, extremities and trunk²
- Treated **large wound areas** (up to 240 cm²)^{1,2}

1. Phase 3 VIITAL study : 43 treated wounds vs matched control wounds in 11 patients with RDEB in a multicenter, randomized, inpatient-controlled study.

2. Phase 1/2a study : 38 treated wounds in 7 patients with RDEB in an open-label, single-arm study.

Robust US commercial opportunity

~1,500 pz-cel treatment opportunities based on current prevalent pool

~1,300 US DEB patients¹;

~750 RDEB pz-cel eligible patients

Anticipate **2 treatment cycles** per patient, on average,
to cover impacted body areas

Pricing expected per each treatment cycle, and in line with value
of a one-time gene therapy offering multi-year clinical benefit

Payer willingness to cover RDEB lives^{2,3}

>\$500M

est. peak annual
US revenue

~85-90%

est. gross margin
after initial ramp

Partnering with high-volume EB Centers of Excellence (COEs) to drive successful pz-cel launch

Ongoing commercial readiness activities:

Early engagement

- Driving **medical awareness** through scientific exchange and peer-to-peer education
- Engaging **commercial payers*** representing >80% of RDEB lives and Medicaid programs

Operations

- Initiated **onboarding process** with multiple EB COEs to become pz-cel Qualified Treatment Centers (QTCs)
- Developing comprehensive **patient assistance and provider support programs**
- Standing up **specialty distributor and pharmacy distribution** channels
- Increasing commercial **manufacturing capacity**

Scaled launch

- Commercialization team with robust **cell and gene therapy launch experience**
- Launch at **geographically dispersed QTCs** to maximize patient treatment access
- **Site readiness** to start treating patients ~2-3 months post-approval
- Drive demand through high pz-cel awareness across COEs to **enable referrals**

Launch year goals:

Onboard

5 EB COEs

that see ~30% of pz-cel eligible patients

Treat

1 to 2 patients

per center per month

Ensure broad and timely market access

Physicians, patients, and payers confirm unmet need in RDEB and enthusiasm for pz-cel

“I think it's much more important to focus on after a wound closes, does it remain closed for the next 2 years with incidental trauma or activities of daily life? I think that'll be the most important measurement.”

US KOL

“I wouldn't have any concerns referring...they would have to be admitted but being in the hospital isn't new to them.”

COE Physician

“I am hopeful and excited... I have questions on the before and after process, but when can I get it?”

31-year-old Patient

“These before & after photos [of pz-cel patients] are extremely compelling.”

Regional Payer

“These are clinically meaningful endpoints, especially the >50% wound healing.”

Commercial Payer

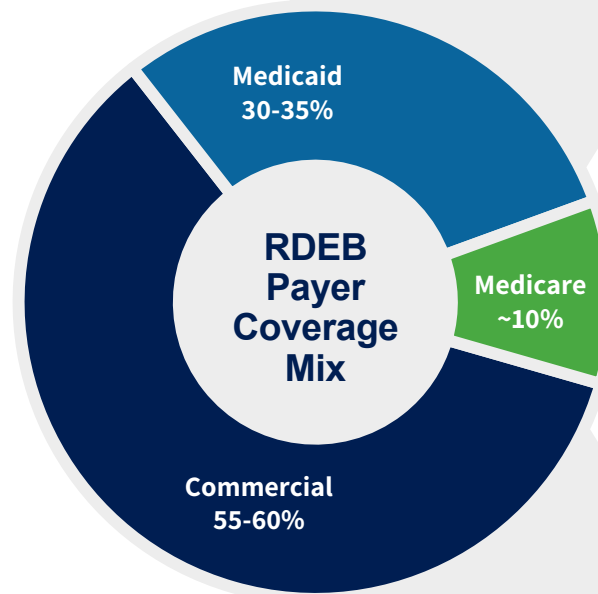
Building strong payer support across reimbursement pathways for pz-cel

Commercial & Medicaid

- Pre-approval engagement ongoing
- Targeting key payers representing >80% of RDEB covered lives
- Positive early engagement builds confidence to secure broad coverage and reimbursement

Anticipated Access

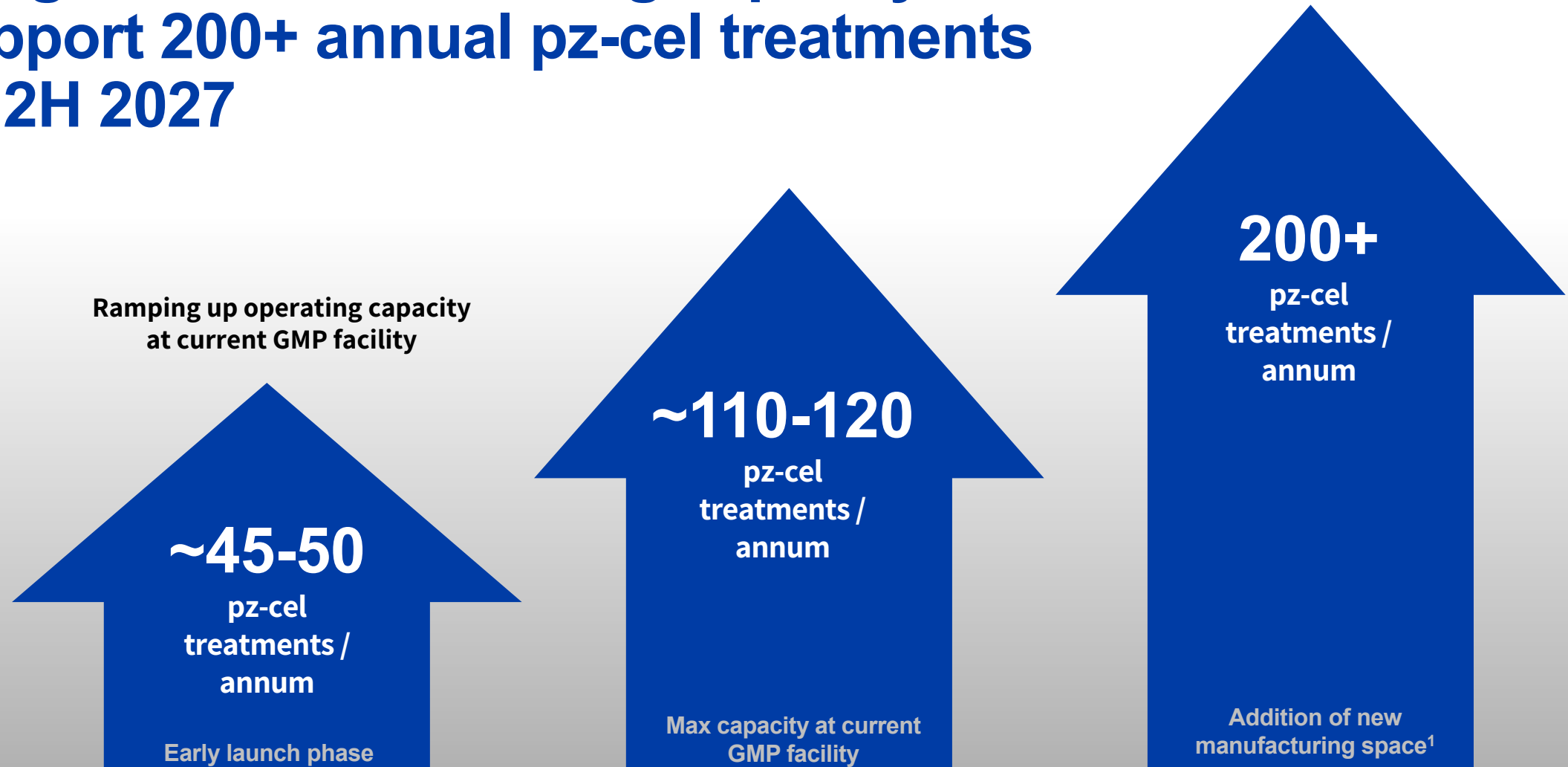
- Inpatient hospital payment methodologies are established
- Payers appreciate high unmet need and pz-cel clinical value
- Payers expect similar coverage to existing gene therapies



Medicare

- **Product-Specific Coding:** ICD-10-PCS pz-cel procedure code granted for genetically engineered autologous cell therapy
- **Favorable DRG Payment:** MS-DRG¹ reimbursement for pz-cel (Pre-MDC DRG 018) mapped to highest inpatient hospital reimbursement levels for cell and gene therapies
- **Additional Potential Payments:** New Technology Add-on Payment (NTAP) and Outlier payment methodologies to be pursued for additional Medicare reimbursement

Long-term manufacturing capacity to support 200+ annual pz-cel treatments by 2H 2027



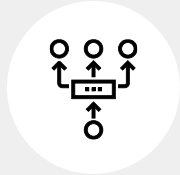
Pz-cel patient journey



**Patient
intake**



**Biopsy sample
procurement**



**Pz-cel
manufacturing**



**Pz-cel surgical
procedure**



**Monitoring/
discharge**

1

2

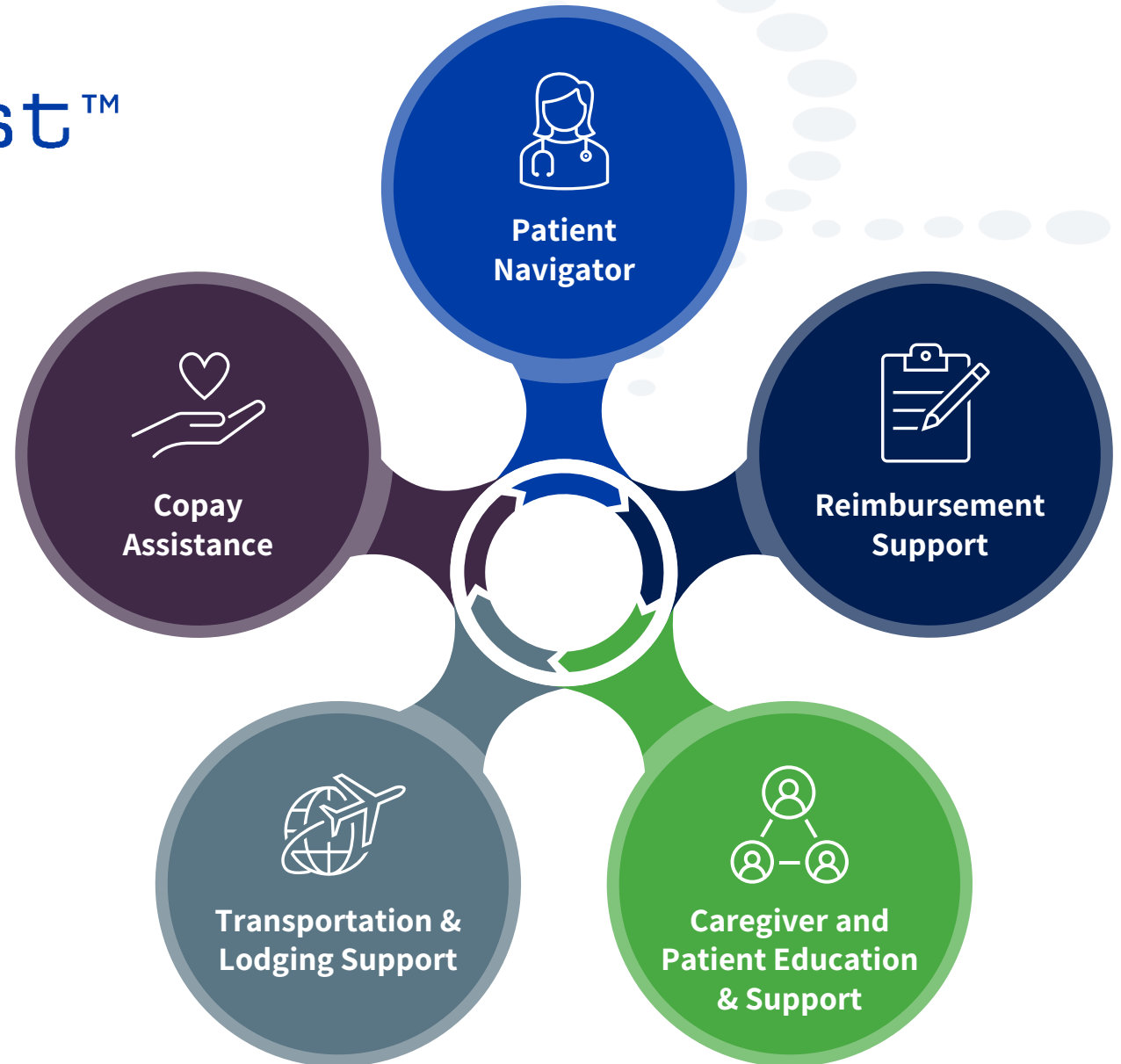
3

4

5



Patient hub providing support throughout the pz-cel treatment journey



Pipeline of differentiated cell and gene therapies

Pz-cel
RDEB

ABO-503
X-linked retinoschisis (XLRS)

ABO-504
Stargardt disease

ABO-505
Autosomal dominant optic atrophy (ADOA)

UX111*
Sanfilippo syndrome type A (MPS IIIA)

TSHA-102**
Rett syndrome

TSHA-118**
Infantile batten disease (CLN1 disease)

DISCOVERY

PRECLINICAL

PHASE 1/2

PHASE 3

BLA accepted, Apr 29, 2025 PDUFA

BLA accepted, Aug 18, 2025 PDUFA*

PARTNERED

Novel and differentiated approaches for genetic ophthalmic conditions with high unmet medical need

Proprietary AAV capsids

Designed for **improved tissue/cell tropism** and **reduced immune responses** for distinct routes of administration including **ocular, systemic and direct CNS**

AAV204 identified for exceptional ability to penetrate retinal cell layers after injection directly into vitreous

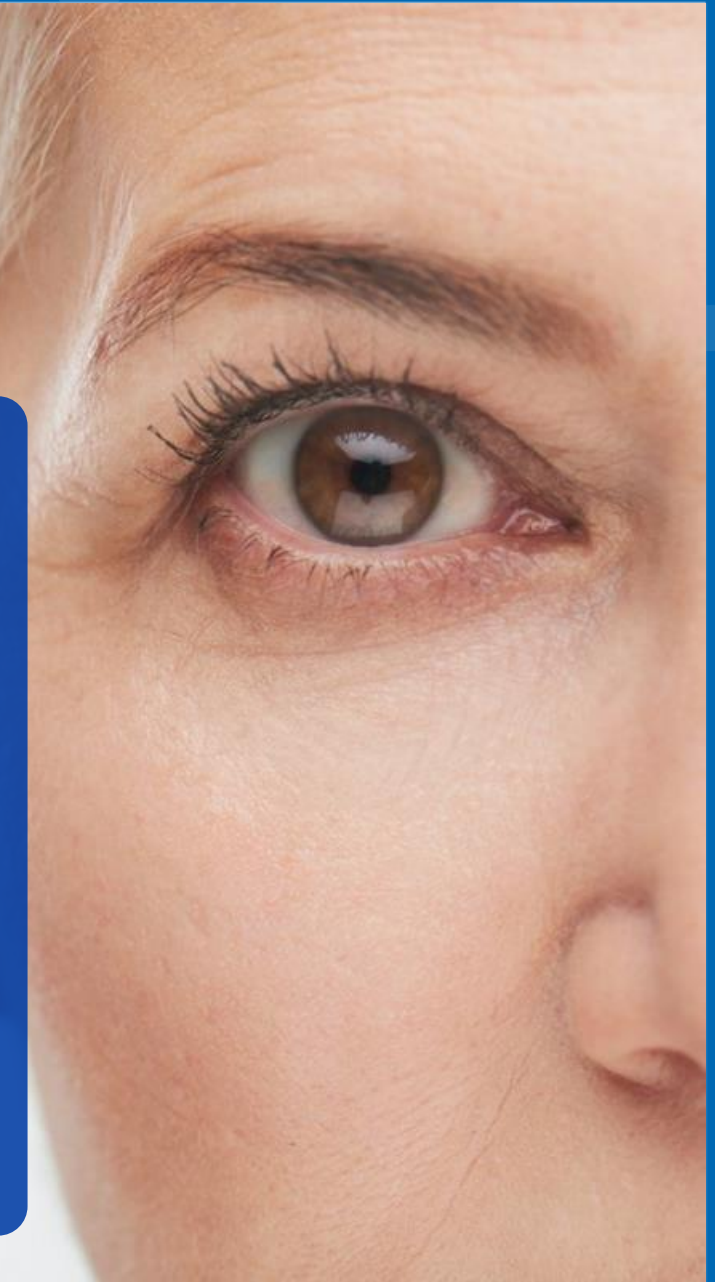
Licensing opportunities for Abeona proprietary capsids in various **ocular conditions**

Novel therapeutic approaches

Developing therapies for higher prevalence ophthalmic disorders including **Stargardt disease, X-linked retinoschisis (XLRS), and autosomal dominant optic atrophy (ADOA)**

XLRS and ADOA programs leverage **AAV204 capsid** combined with **para-retinal injection**, a potentially safer route compared to subretinal administration

Stargardt disease asset produces **full length ABCA4 protein** by using **proprietary AAV-based Cre-Lox system** to overcome packaging capacity limits of traditional AAV genome

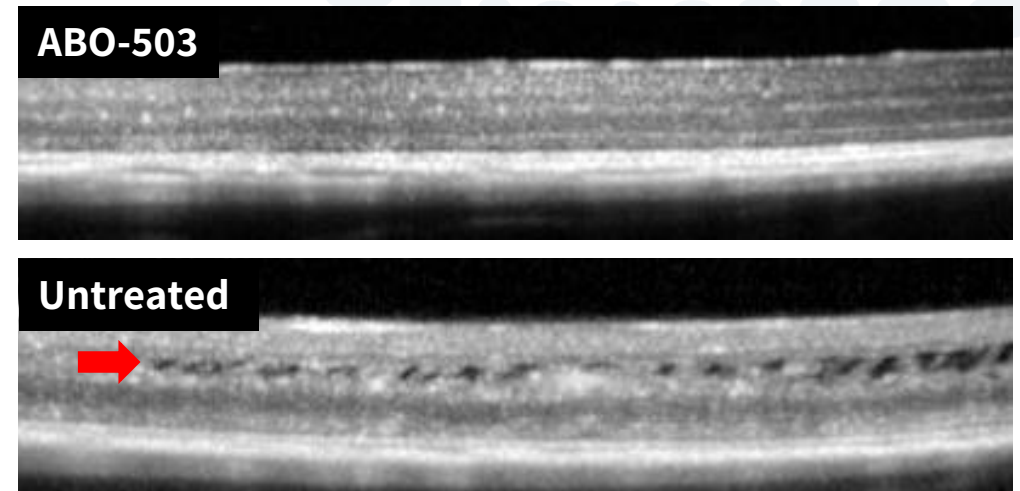


ABO-503 for X-linked retinoschisis

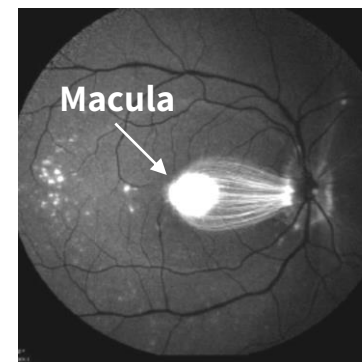
First-in-human opportunity in 1H 2026

Key findings

- ABO-503 treatment of very young mutant mice **significantly prevents formation of cavities** and **preserves photoreceptor function** compared to untreated mutant mice
- Robust RS1 expression observed in photoreceptor cells near injection site and in adjacent inner retina in mutant mice 3 months after treatment with ABO-503
- RS1 expression associated with **improved cone photoreceptor density** and **increased thickness of photoreceptor outer nuclear layer**
- Full-field flicker electroretinogram (ERG) analysis showed **significant improvement in cone photoreceptor function**
- Proprietary AAV204 capsid allows for **less invasive para-retinal injection route** to achieve high macular transduction rate

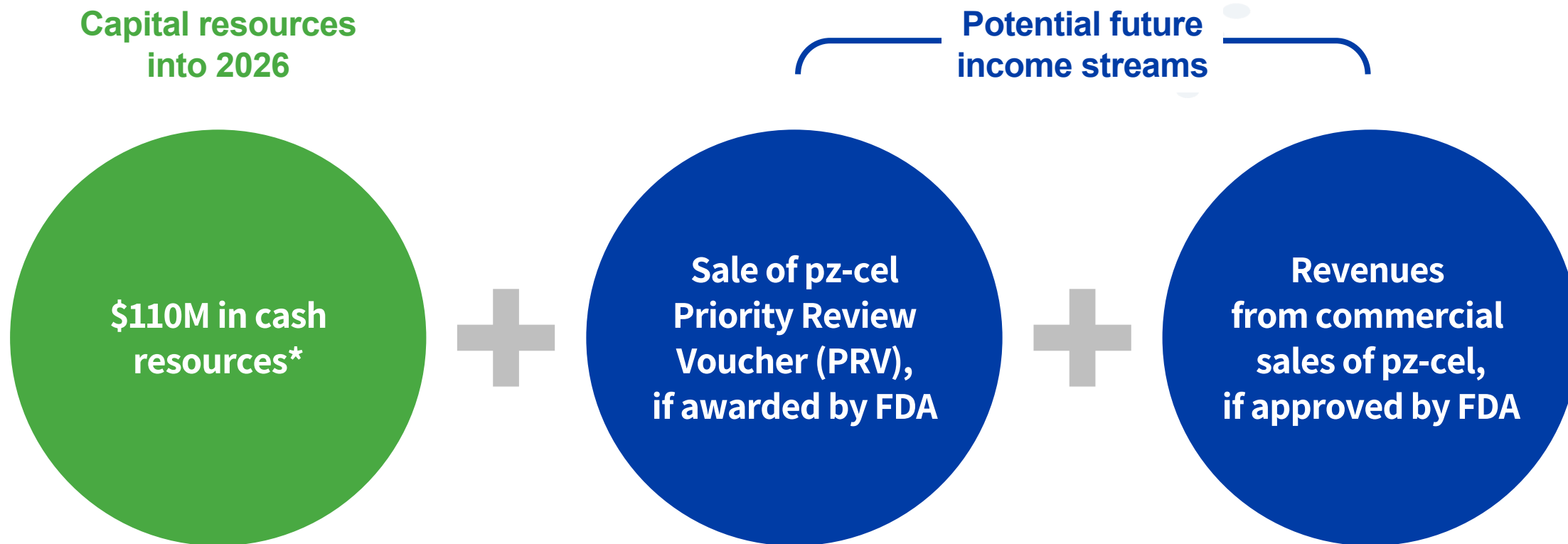


Imaging of mutant mice 2 months after treatment



Para-retinal injection of AAV204 in non-human primates

Current capital resources provide runway through potential launch into 2026, with potential future income streams to extend further



2025 milestones and catalysts

PZ-CEL

- ☐ Potential FDA approval in RDEB (April 29, 2025 PDUFA)
- ☐ Potential PRV grant
- ☐ Activate pz-cel treatment sites
- ☐ Build referral networks to pz-cel treatment centers
- ☐ Pursue pz-cel ex-US opportunity post-FDA approval

PIPELINE

- ☐ Potential FDA approval for UX111 (ABO-102) in MPS IIIA (Aug 18, 2025 PDUFA)*
- ☐ RS1 preclinical data presentation at scientific congress
- ☐ Complete RS1 IND-enabling work
- ☐ Continue to assess external assets to expand pipeline