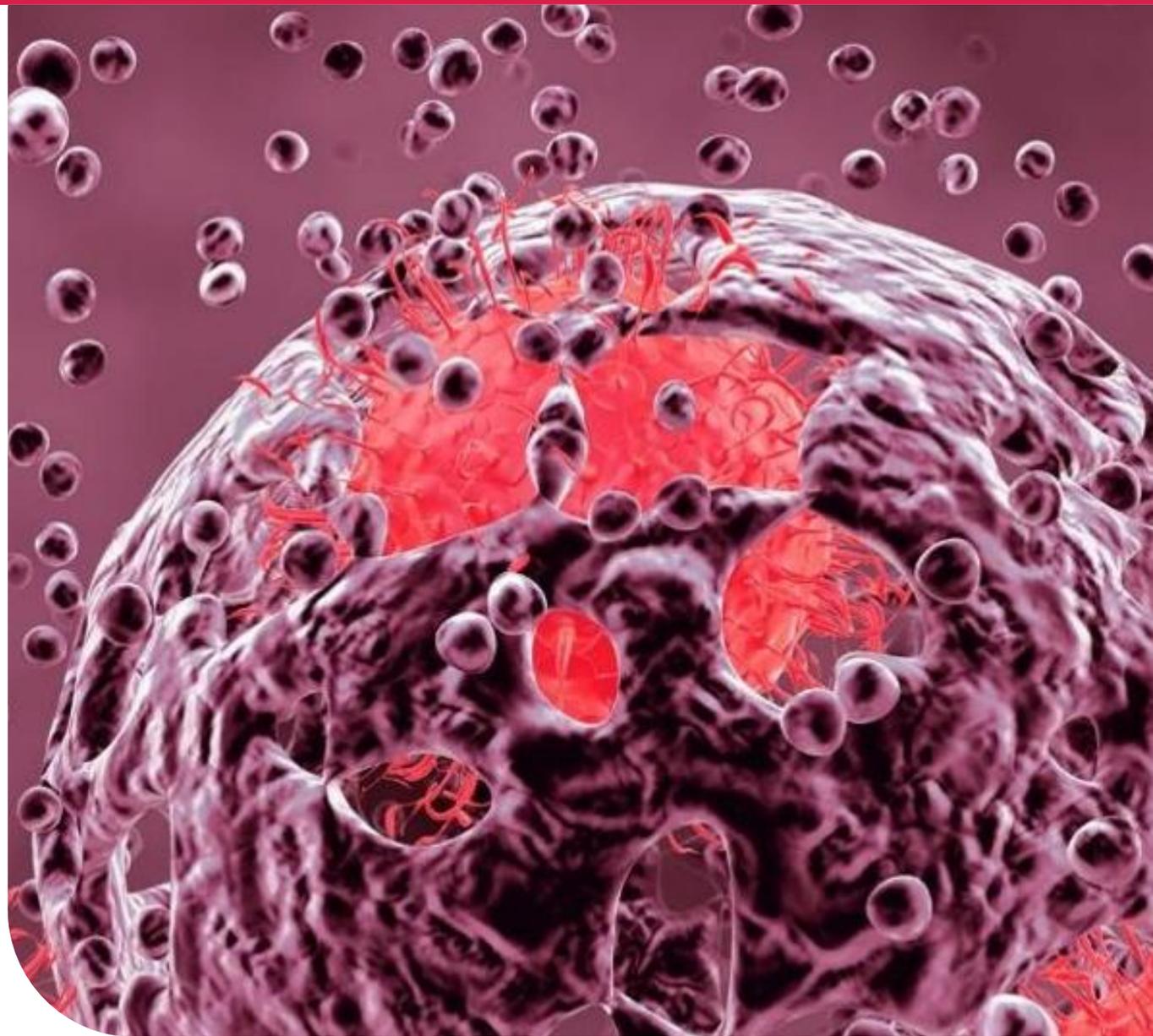




# Astellas R&D Day

Pioneering science to change tomorrow

March 31, 2026



# Cautionary statement regarding forward-looking information

In this material, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas Pharma. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties.

Information about pharmaceutical products (including products currently in development) which is included in this material is not intended to constitute an advertisement or medical advice. Information about investigational compounds in development does not imply established safety or efficacy of the compounds; there is no guarantee investigational compounds will receive regulatory approval or become commercially available for the uses being investigated.

The data presented here are based on data presented at scientific congress.

# Contents



**From CSP2021 to Today –  
Delivering VALUE to Patients**



**Transforming Our R&D Organization and  
Capabilities to Deliver for Patients Faster**



**Advancing Our Pipeline to  
Pioneer Tomorrow's Science**



**Looking Ahead:  
Sustaining Long-Term VALUE Creation**



**Q&A**

## Presenters



**Naoki Okamura**  
President and CEO



**Tadaaki Taniguchi, MD, PhD**  
Chief Research and Development Officer

On the forefront of  
healthcare change to  
turn innovative science  
into **VALUE** for patients

**VALUE =**

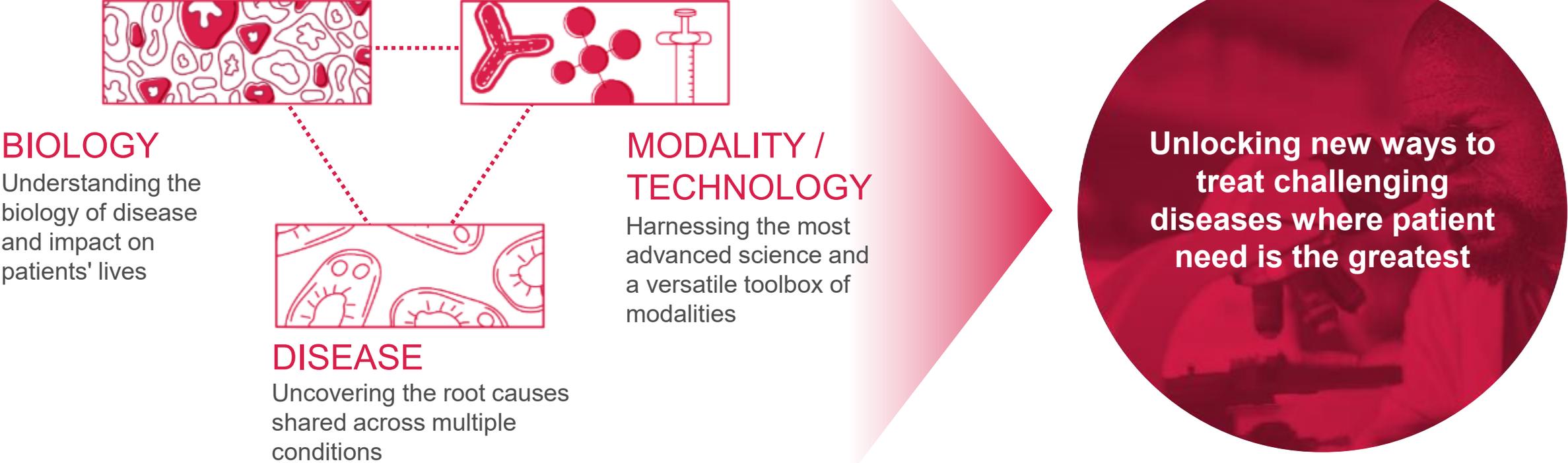
Outcomes  
that matter to patients

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Cost  
to the healthcare system of  
delivering those outcomes



# Our unique Focus Area approach drives innovation at Astellas



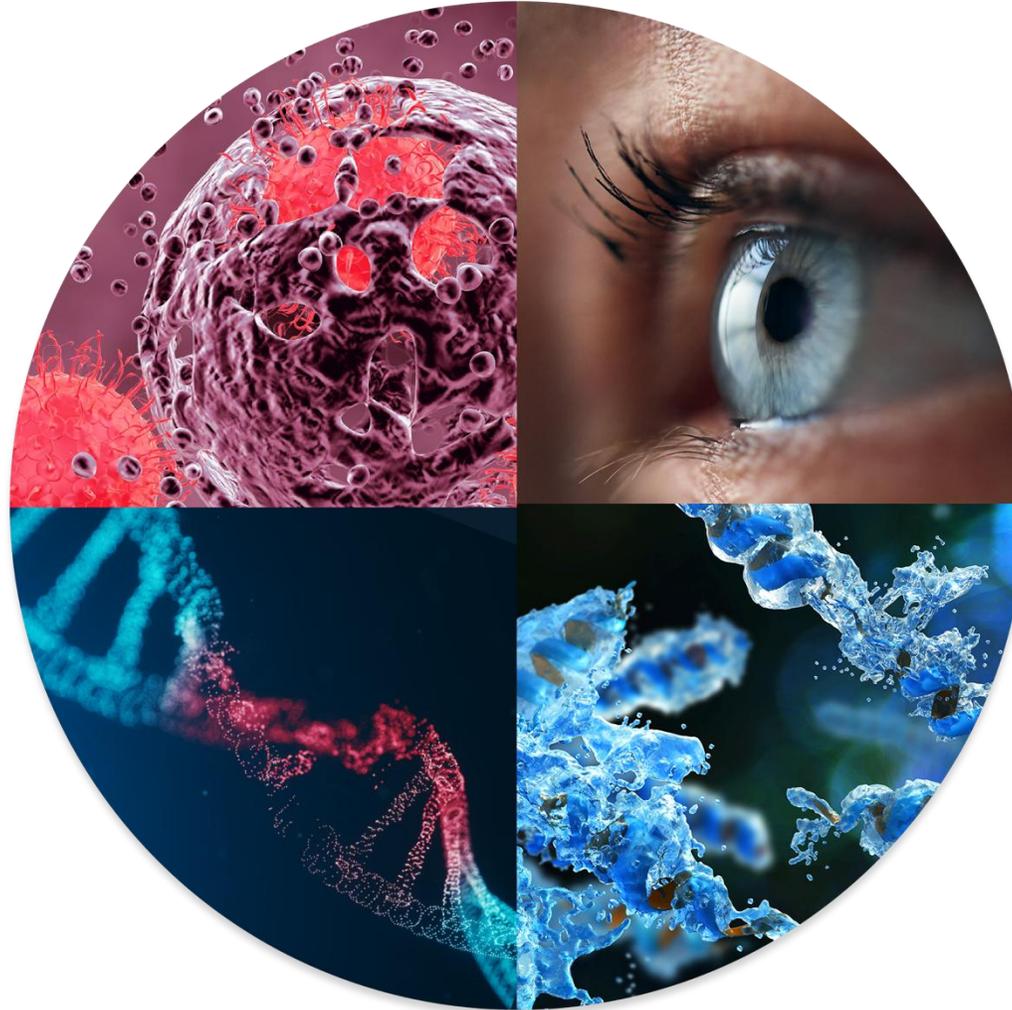
# We are advancing four flagship assets with breakthrough potential from our Primary Focuses

## IMMUNO-ONCOLOGY

**ASP2138** – a T-cell engager with the potential to be a first-in-class therapy in notoriously hard-to-treat **gastric, gastroesophageal junction and pancreatic cancers**

## GENETIC REGULATION

**AT845** – an AAV gene replacement therapy designed to address the underlying cause of Pompe disease, a **devastating rare neuromuscular disease**



## BLINDNESS AND REGENERATION

**ASP7317** – one of the **first ever ophthalmic cell therapy** derived from pluripotent stem cells to enter the clinic for a leading cause of blindness

## TARGETED PROTEIN DEGRADATION

**setidegrasib (ASP3082)** – a potential first-in-class targeted protein degrader for treating **solid tumor with KRAS G12D mutations, including pancreatic and lung cancer**

AAV: Adeno-associated virus

# This disciplined, focused approach is delivering VALUE for patients

CSP2021 enabled us to focus on accelerating higher-quality science, with stronger execution discipline and sustainable productivity

## Accelerated the pipeline

- **12 Phase 1 FSD NMEs**
- **1 Phase 3 study initiated on an NME**
- **4 PoCs\***, validating assets and platforms
- **1 new Primary Focus established**, Targeted Protein Degradation

## Acted with agility to drive stronger portfolio discipline

- Shift towards high value
- **21 clinical-stage programs terminated**

## Built the foundation for sustainable productivity

- **Transformed our R&D organization**, and end-to-end VALUE Creation
- **Invested in capabilities** to enhance scale and accelerate speed
- **Adopted new ways of working** to drive higher productivity and more consistent outcomes

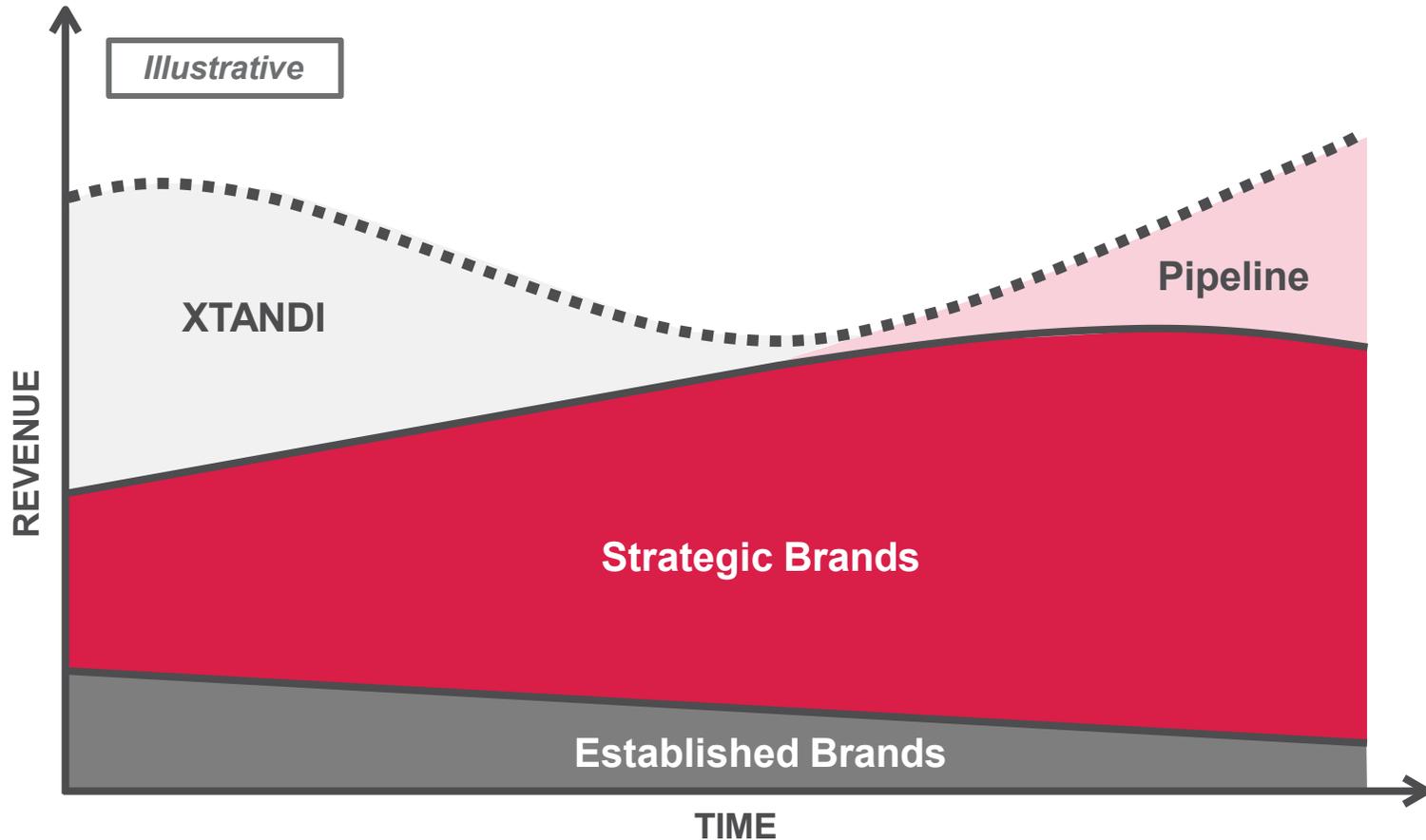


\*4 PoCs from 3 assets (setidegrasib, ASP2138, ASP7317)

CSP: Corporate Strategic Plan, FSD: First subject dosed, NME: New molecular entity, PoC: Proof of concept

# We are building on this momentum as we inflect to growth

Astellas is managing its transition with focus and control



## Maximize Revenue

Elevate the peak and flatten the dip

## Accelerate Pipeline

Expected sales contribution in 2030s

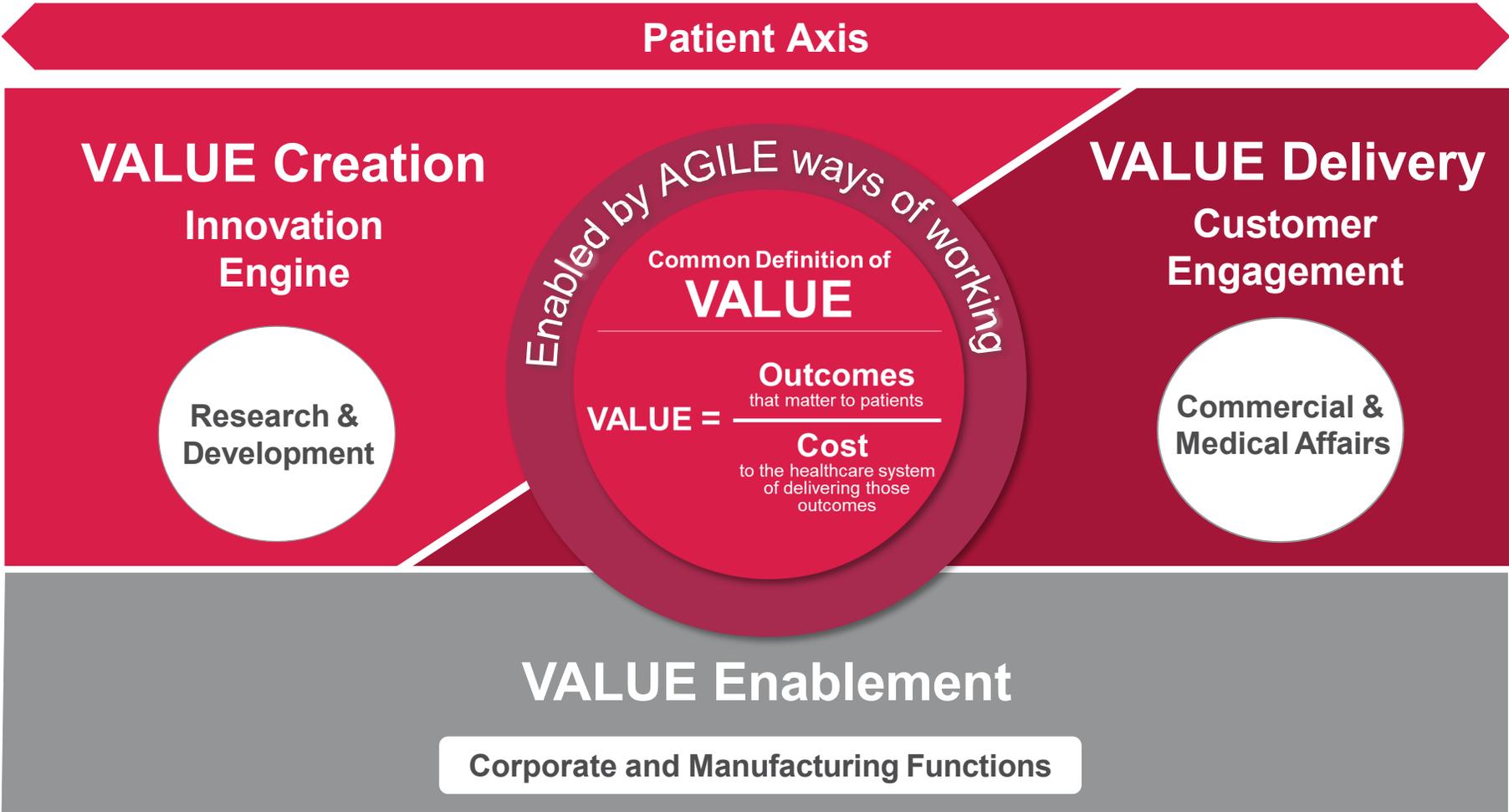
## Operational Efficiency

Elevate profitability and invest in Strategic Brands and pipeline

Strategic Brands: PADCEV, IZERVAY, VYLOY, VEOZAH, XOSPATA

# Enabled by our end-to-end model with patient VALUE at the center

From discovery through development to delivery, aligned around patient VALUE

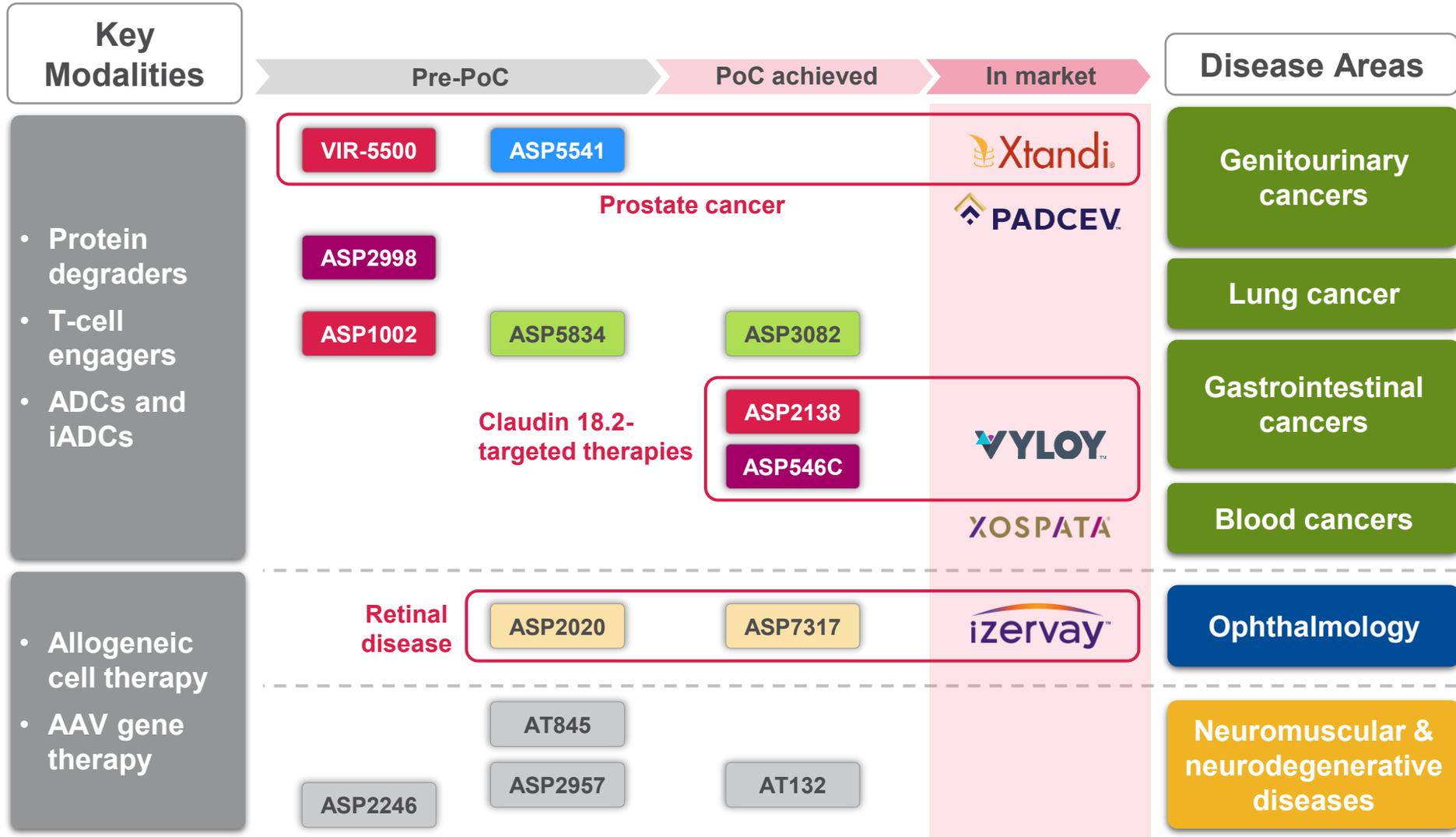


# Our Focus Area approach is translating into early clinical progress

## Primary Focuses



## Biotech and Academic Partners



AAV: Adeno-associated virus, (i)ADC: (immunostimulatory) Antibody-drug conjugate, mRNA: messenger RNA, PoC: Proof of concept

# For those living with serious diseases, science can change everything

From treating the toughest cancers, freeing people from the limits of chronic illness, to reimagining life after organ transplant, scientific breakthroughs are changing lives every day

**But too many patients are still waiting**



# We are delivering **VALUE** at global scale

Our transformative therapies in oncology, ophthalmology, urology, women's health and immunology have **improved millions of lives**

We're **broadening access and unlocking new possibilities** across earlier and more extensive stages of disease to ensure every eligible patient can benefit from our therapies

Astellas' products  
have reached  
more than

**174 million**  
patients

in  
**100+**  
countries  
around the world



# Translating scientific innovation into patient benefit across multiple diseases with high unmet needs



Helping patients with bladder cancer live twice as long compared to chemotherapy<sup>1</sup>



Delaying prostate cancer progression by over 60% when used with hormone therapy<sup>2</sup>



Adding nearly 3 extra months of life in gastric cancers when combined with chemo compared to chemotherapy alone<sup>3</sup>



Slowing geographic atrophy growth versus sham<sup>4</sup>



First-in-class non-hormonal option for VMS

1. EV-302 study (previously untreated locally advanced or metastatic urothelial cancer, combination with pembrolizumab); N Engl J Med 2024;390:875-888, 2. EMBARK study (non-metastatic castration-sensitive prostate cancer with high-risk biochemical recurrence); N Engl J Med 2026;394:563-575, 3. SPOTLIGHT study (Caudin 18.2-positive, HER2-negative, untreated, locally advanced unresectable or metastatic gastric or gastroesophageal junction adenocarcinoma); Lancet 2023;401:1655-1668, 4. GATHER2 study; Ophthalmology 2026;133:451-465  
VMS: Vasomotor symptoms

# PADCEV is redefining outcomes for patients with muscle-invasive bladder cancer

EV-304: perioperative enfortumab vedotin + pembrolizumab significantly improved EFS and OS and increased pCR vs. neoadjuvant chemotherapy

## Primary Endpoint: EFS by BICR ITT Population



NR, not reached. \* denotes statistical significance (one-sided boundary 0.0082).

Data cutoff date: 27 October 2025

Data presented at ASCO GU 2026

ASCO GU: American Society of Clinical Oncology Genitourinary Cancers Symposium, BICR: Blinded Independent Central review, cis: Cisplatin, EV: enfortumab vedotin, gem: Gemcitabine, HR: Hazard ratio, ITT: Intent to treat, pembro: Pembrolizumab



# We are advancing a focused pipeline in synergy with our Strategic Brands

As of March 2026

Prostate Cancer	Phase
<b>XTANDI (enzalutamide)</b>	In market
<b>ASP5541/PRL-02</b>	● 2 ●
<b>VIR-5500</b>	● 1 ● ●

Bladder and Urothelial Cancer	Phase
<b>PADCEV (enfortumab vedotin)</b> mUC, Cisplatin-ineligible MIBC	In market
<b>enfortumab vedotin</b> Cisplatin-eligible MIBC	● ● ● 3

Ophthalmology	Phase
<b>IZERVAY</b> GA secondary to AMD	In market
<b>ASP7317</b> GA secondary to AMD	● 1 ● ●

Upper GI and Pancreatic Cancer	Phase
<b>VYLOY (zolbetuximab)</b> Gastric and GEJ cancer	In market
<b>zolbetuximab</b> Gastric and GEJ cancer	● ● ● 3
<b>ASP2138</b> Gastric, GEJ, pancreatic cancer	● 1 ● ●
<b>ASP546C</b>	● 1 ● ●
<b>setidegrasib (ASP3082)</b> PDAC	● 1 ● ●

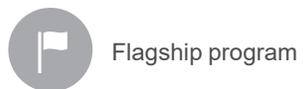
Neuromuscular & Neurodegenerative	Phase
<b>AT132</b> X-linked myotubular myopathy	● 2 ● ●
<b>ASP2957</b> X-linked myotubular myopathy	IND cleared

Acute Myeloid Leukemia	Phase
<b>XOSPATA (gilteritinib)</b> AML	In market
<b>gilteritinib</b> Earlier-stage AML, pediatric use	● ● ● 3
<b>gilteritinib</b> Newly diagnosed AML, HIC-ineligible	● 2 ●

Neuromuscular & Neurodegenerative	Phase
<b>AT845</b> Pompe disease	● 2 ● ●
<b>ASP2246</b> Motor dysfunction associated with ischemic stroke	PMDA cleared

Solid Tumors	Phase
<b>setidegrasib (ASP3082)</b> NSCLC	● 1 ● ●
<b>ASP5834</b>	● 1 ● ●
<b>gilteritinib</b> ALK-positive NSCLC	● 1 ● ●
<b>ASP1002</b>	● 1 ● ●
<b>ASP2998</b>	● 1 ● ●

Vasomotor Symptoms (VMS)	Phase
<b>VEOZAH (fezolinetant)</b> VMS due to menopause	In market
<b>fezolinetant</b> VMS due to menopause: China, Japan and VMS in breast cancer women	● ● ● 3



\*Not exhaustively listed.

ALK: Anaplastic lymphoma kinase, AMD: Age-related macular degeneration, AML: Acute myeloid leukemia, GA: Geographic atrophy, GEJ: Gastroesophageal junction, GI: Gastrointestinal, HIC: High-intensity chemotherapy, IND: Investigational New Drug application, MIBC: Muscle-invasive bladder cancer, mUC: Metastatic urothelial carcinoma, NSCLC: Non-small cell lung cancer, PDAC: Pancreatic ductal adenocarcinoma, PMDA: Pharmaceuticals and Medical Devices Agency



# We have established a clear path to deliver a dynamic, competitive portfolio to fuel long-term growth

2030-2034



## Excel

- R&D productivity trending toward top-level industry performance
- Deliver consistent high-value, de-risked pipeline progression

2027-2029



## Enrich the Pipeline

- Increase quality and coherence of the pipeline
- Enhance prioritization

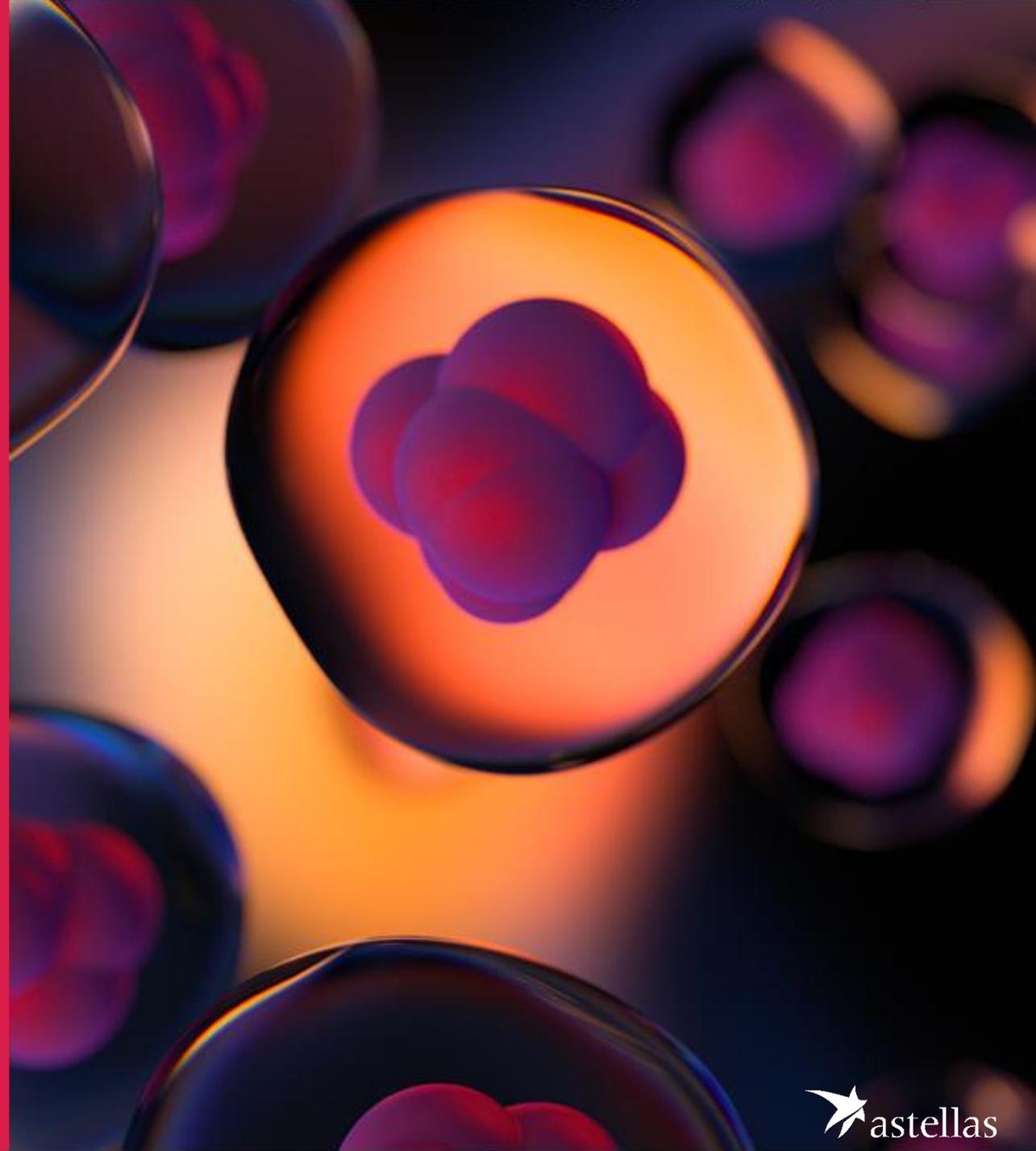
2024-2026



## Enhance the Foundation

- Transform the R&D organization
- Strengthen productivity and governance

# Transforming Our R&D Organization and Capabilities to Deliver for Patients Faster



# We have taken BOLD measures to increase productivity and efficiency, delivering tangible gains



**Driving Internal & External Collaboration**



**Accelerating & Maximize our Pipeline**



**Investing in Talent**

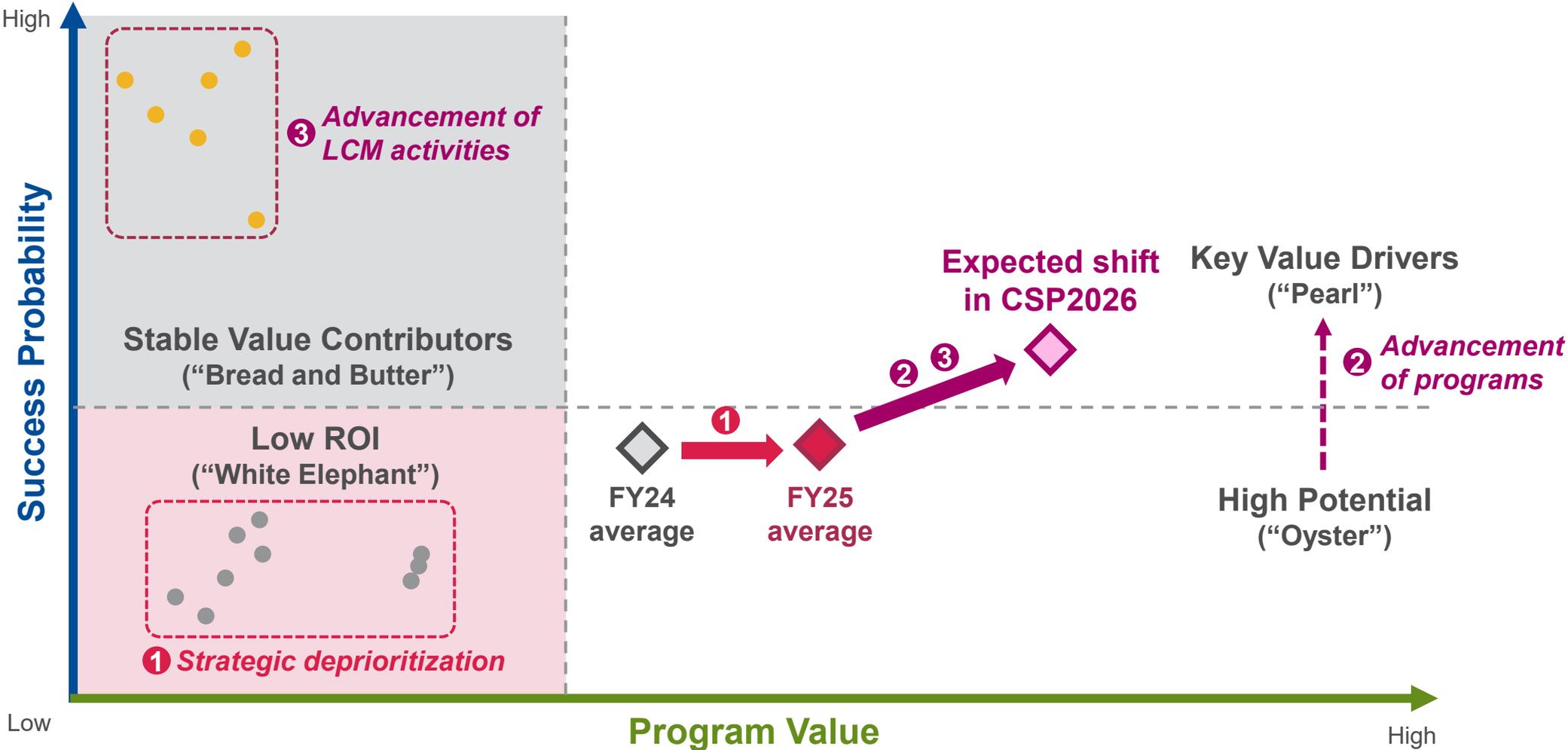


**Accelerating Clinical Trial Execution**



**Fostering a Data-Driven Culture**

# We are actively prioritizing our portfolio with discipline for maximum pipeline value



Including LCM and preclinical-stage programs  
 Ref: PDMA Visions Magazine 4, 2013;37;4  
 CSP: Corporate Strategic Plan, LCM: Lifecycle management, ROI: Return on investment

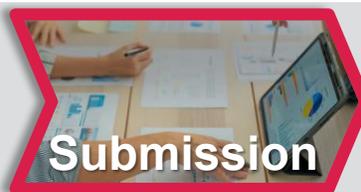
● LCM of Strategic Brands    ● Program deprioritized in FY24



# By bringing clinical operations in-house, we have significantly enhanced our clinical trial execution to deliver innovative medicines to patients, faster



- **69% reduction** in time from IND to First Site Activation\*
- **67% reduction in time** from IND to FSD\*



- Median of **115 days** from topline results to submission date in FY25, compared to 147 days industry benchmark\*\*
- **14% reduction** in submission timelines in FY25 compared to FY23



- **8 approvals** in FY24/25 in major markets (US, EU and Japan)



Enhancing global KOL relationships



Bringing us closer to the patient



Strengthening in-house R&D capability

\*FY24/FY25 compared to FY23

\*\*KMR benchmark <https://kmrgroup.com/>

FSD: First subject dose, IND: Investigational New Drug application, KOL: Key opinion leader

# Strategic investments in AI will help us improve speed, quality and decision-making across R&D

## Research

**AI-enabled Protein Station** to increase the speed and productivity of biologics discovery

**AI-driven gene therapy** for precise organ targeting, reduced toxicity and enhanced treatment accuracy

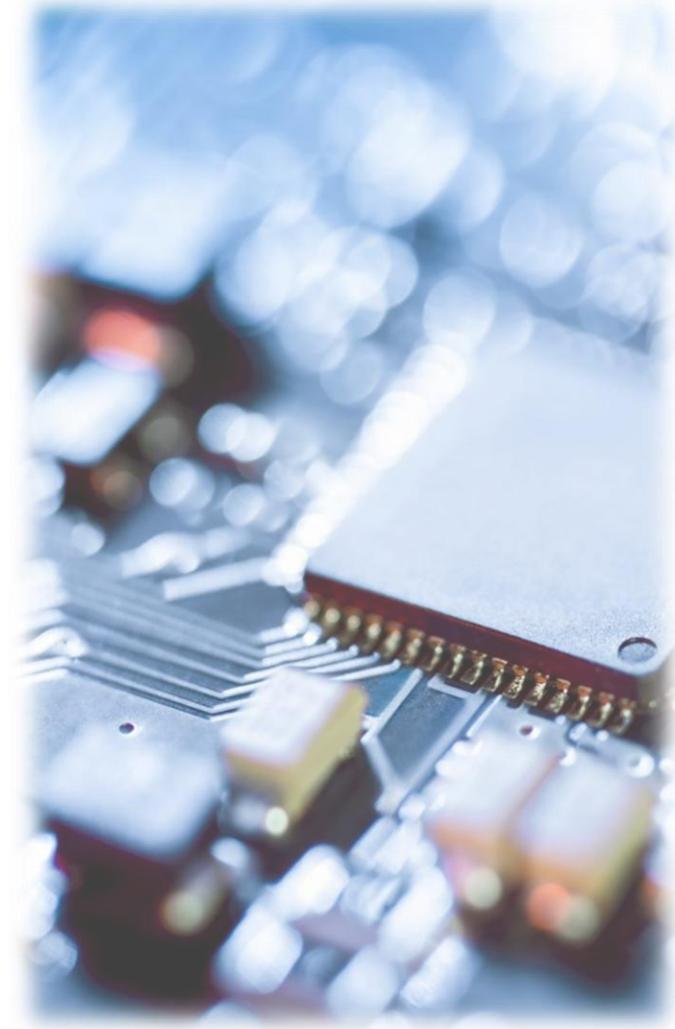
**Human-in-the-Loop platform**, an AI and robotics integrated system to optimize drug candidates

**NVIDIA-powered supercomputing** to dramatically accelerate early-stage drug discovery

## Development and Manufacturing

**Evinova AI-native Study Designer platform** to design smarter, more patient centered studies

**Mahol-A-Ba robotics platform**, automating complex cell therapy manufacturing processes



# Our Research Centers of Excellence drive focused and connected innovation to enrich our early pipeline



## ONCOLOGY RESEARCH

Advancing **early-stage cancer therapies** through cutting edge-modalities including **targeted protein degradation** and **immuno-stimulatory approaches**



## CELL AND GENE THERAPY RESEARCH

Developing the cell and gene therapies of the future with **AAV-delivered gene therapies** and **pluripotent stem cell-based allogeneic therapies** for diverse and complex diseases



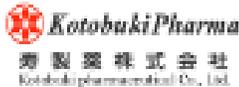
## INNOVATION LABS

Harnessing the best external innovation underpinned by strong biology through **open innovation** and **external R&D**, to expand our pipeline beyond oncology, and cell and gene therapy research

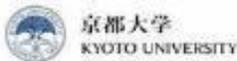
AAV: Adeno-associated virus

# Strong partnerships enhance and accelerate our innovation potential

## Biotech Collaborations



## Academic Collaborations



## Open Innovation



# We can translate innovation into reliable, development-ready supply at scale and with confidence

## Resilient Global Network



- **Diversified manufacturing sites** and technology centers across Japan, US, Ireland and China

## Advanced Technologies Across Modalities

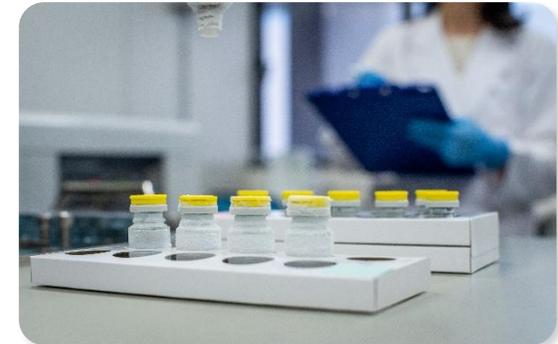


- Small molecules and biologics
- Cell-based therapies
- Nucleic-acid base therapies
- Antibody-drug conjugates

## Manufacturing at Scale

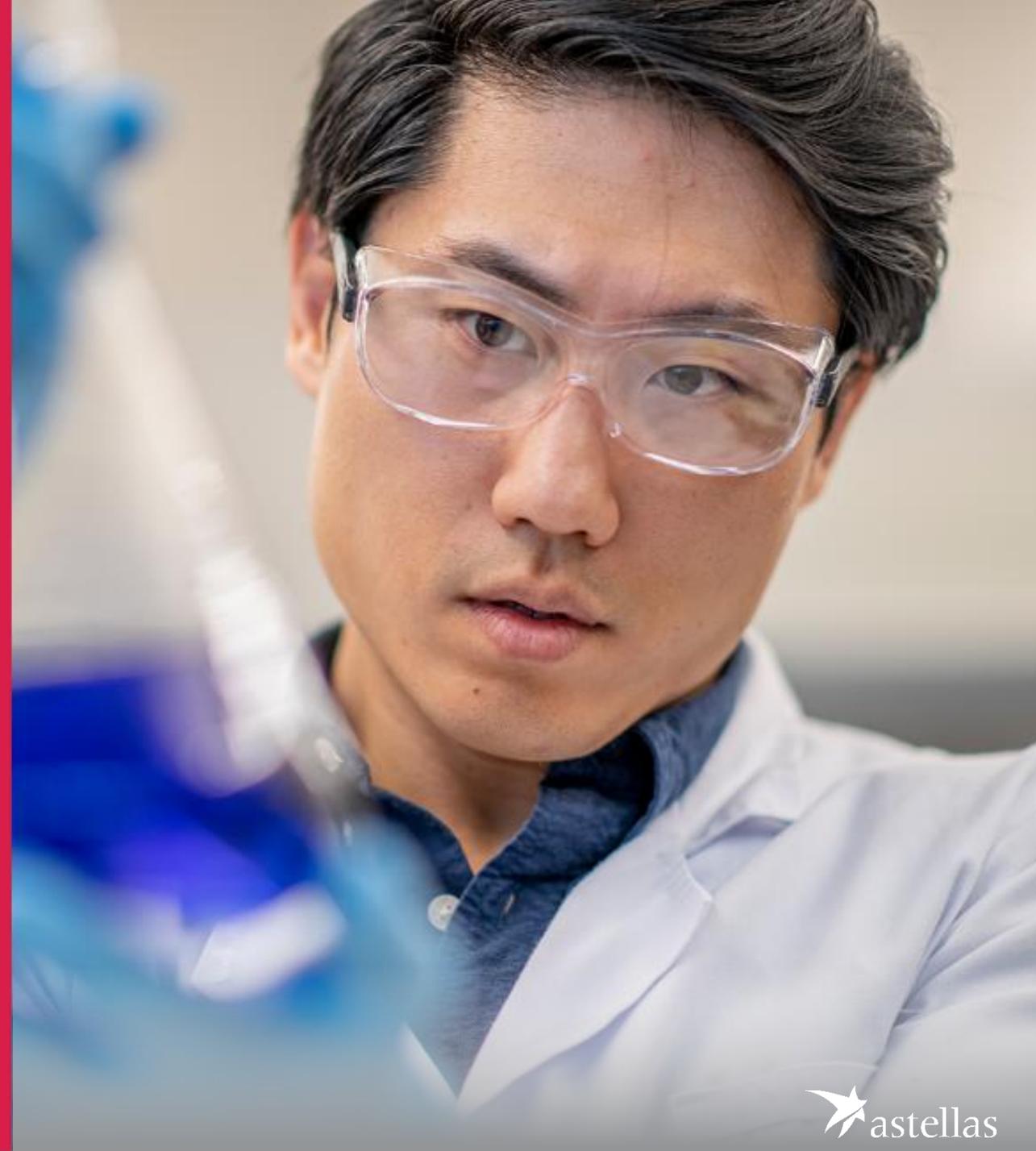


- Combining cGMP infrastructure with regulatory expertise ensuring **reliable, high-quality supply at every development stage**



cGMP: Current Good Manufacturing Practice

# Advancing Our Pipeline to Pioneer Tomorrow's Science



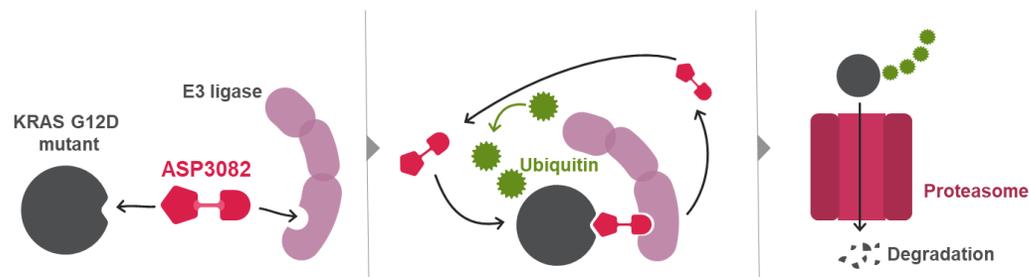
# Primary Focus Targeted Protein Degradation: Leading a new era of medicine by reshaping treatment expectations

We are working to target “undruggable” proteins and transform treatment for patients  
Starting with cancer, we plan to expand to other diseases that need better treatment options

## Flagship program

**setidegrasib (ASP3082):** a potential first-in-class targeted protein degrader for treating **solid tumors with KRAS G12D mutations**, which had been considered undruggable

- ✓ Rate of patients with KRAS G12D mutation:  
~40% in PDAC, ~5% in NSCLC<sup>1</sup>



## Follow-on program

**ASP5834:** A pan-KRAS targeted protein degrader targeting multiple KRAS alterations (KRAS G12V/D/C/R/A, G13D mutations and KRAS WT amplification)

- ✓ 11.6% of all cancer patients have a KRAS mutation<sup>2,3</sup>

## Status

- ✓ Phase 1 FSD in Aug 2025 (27 days after IND clearance)

1. npj Precis Oncol. 2022;6:91, 2. American Cancer Society. Cancer Facts & Figures (2020), 3. Hofmann, M.H. et al. Cancer Discovery 12(4):924-937 (2022)  
FSD: First subject dosed, IND: Investigational New Drug application, NSCLC: Non-small cell lung cancer, PDAC: Pancreatic ductal adenocarcinoma

# setidegrasib (ASP3082): Proof of Concept achieved with clear path to registrational studies

## Pancreatic ductal adenocarcinoma (PDAC)

- **Proof of concept achieved**
- Data presented at ASCO GI 2026
- **Enrollment initiated for Phase 3 study** in 1L PDAC (NCT07409272)
- Primary analysis anticipated: FY2029

## Non-small cell lung cancer (NSCLC)

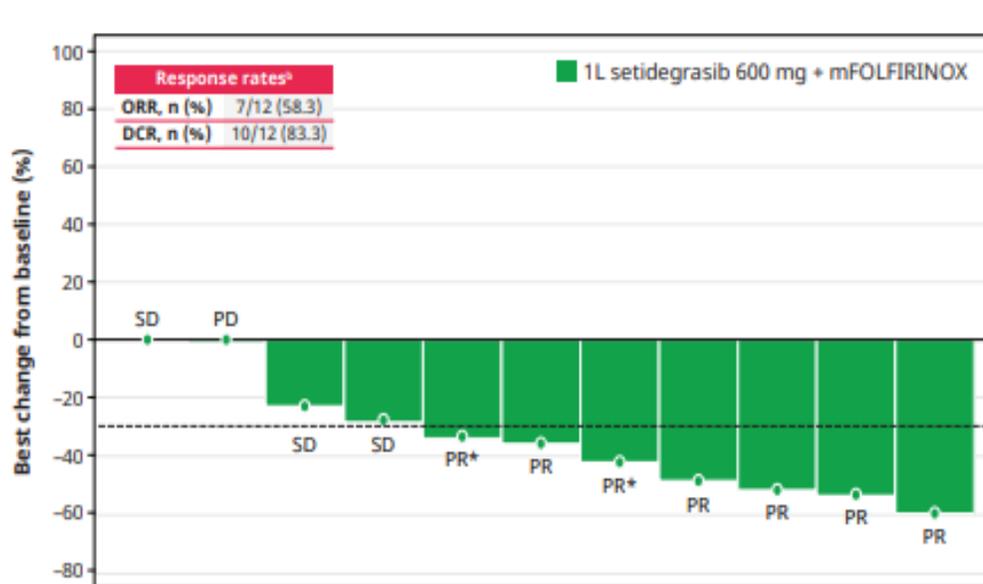
- **Proof of concept achieved**
- Data presented at AACR-NCI-EORTC meeting Oct 2025 and ELCC Mar 2026 with simultaneous NEJM publication<sup>1</sup>
- **Preparing to initiate Phase 3 study** in 2L+ NSCLC
- Primary analysis anticipated: FY2028
- Data generation in 1L in progress

- 2L+ monotherapy & combo with cetuximab for colorectal cancer: decision not to pursue development
- Data generation in progress to support development in other KRASG12D mutant cancers

1. N Engl J Med 2026 Mar; doi: 10.1056/NEJMoa2600752

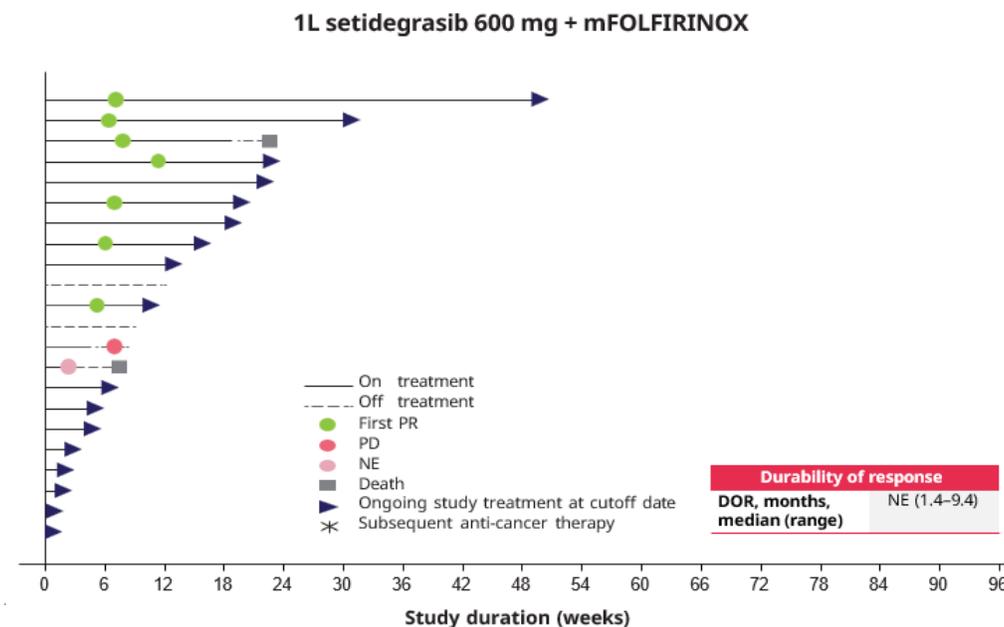
1L: First line, 2L+: Second or later line, ASCO: American Society of Clinical Oncology, GI: Gastrointestinal, AACR: American Association for Cancer Research, NCI: National Cancer Institute, EORTC: European Organisation for Research and Treatment of Cancer, ELCC: European Lung Cancer Conference, NEJM; The New England Journal of Medicine

# setidegrasib (ASP3082) shows antitumor activity in combination therapy in PDAC



Dashed lines at -30% represent PR<sub>1</sub>, per RECIST version 1.1. Asterisks represent unconfirmed PR per RECIST version 1.1 in patients with ongoing treatment.

ORR/DCR analysis included all patients who received at least 1 dose of setidegrasib. Three patients were excluded from the waterfall plot of best change from baseline (withdrawal by patient, n = 1; withdrawal due to AE, n = 1; PD, n = 1); Ten patients who received at least 1 dose of setidegrasib were excluded from the ORR/DCR analysis (lack of postbaseline tumor assessment before withdrawal, n = 2; lack of postbaseline tumor assessment at data cutoff due to insufficient follow up time, n = 8). One additional patient was excluded from the waterfall plot of best change from baseline (lack of evaluable postbaseline tumor assessment).

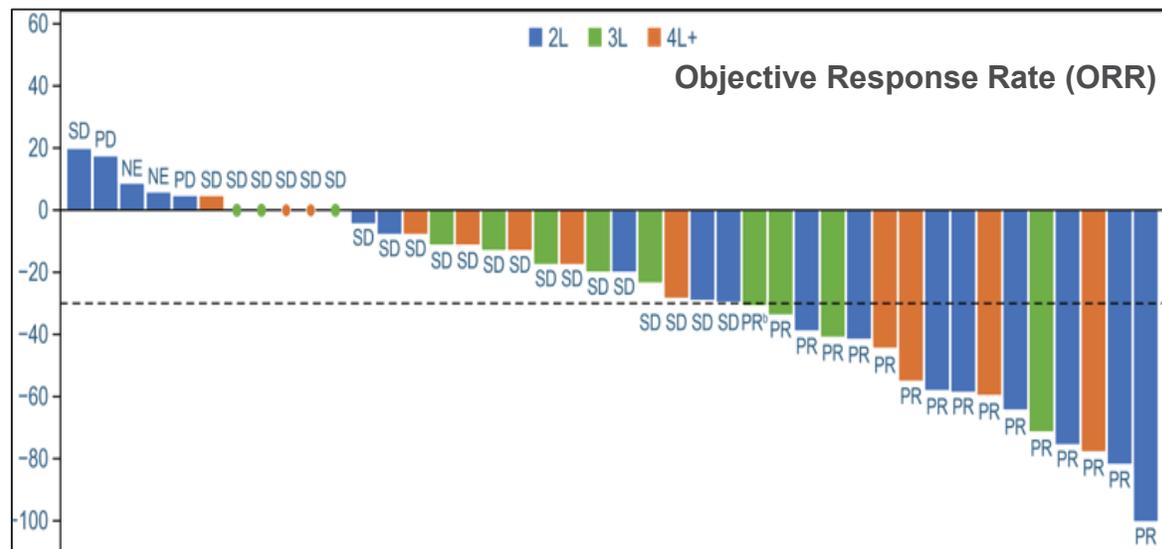


- Infusion-related reactions (IRRs) were reported in 16/22 (72.7%) patients
- IRRs were low grade with rash/urticaria, occurred mostly during the first infusion and were well managed with antihistamine prophylaxis, short pause in administration and slowed infusion rate. No patients discontinued treatment due to IRRs

Enrollment has been initiated for a Phase 3 registrational study to evaluate setidegrasib + mFOLFIRINOX/NALIRIFOX in 1L PDAC patients with KRAS G12D mutation (NCT07409272)

**Presented at ASCO GI 2026.** 1L: First line, DCR: Disease control rate, DOR: Duration of response, ORR: Objective response rate, PDAC: Pancreatic ductal adenocarcinoma, AE: Adverse event, SD: Stable disease, PD: Progressive disease, PR: Partial response, NE: Not evaluable, mFOLFIRINOX: Leucovorin, fluorouracil, irinotecan and oxaliplatin, NALIRIFOX: Leucovorin, fluorouracil, liposomal irinotecan and oxaliplatin

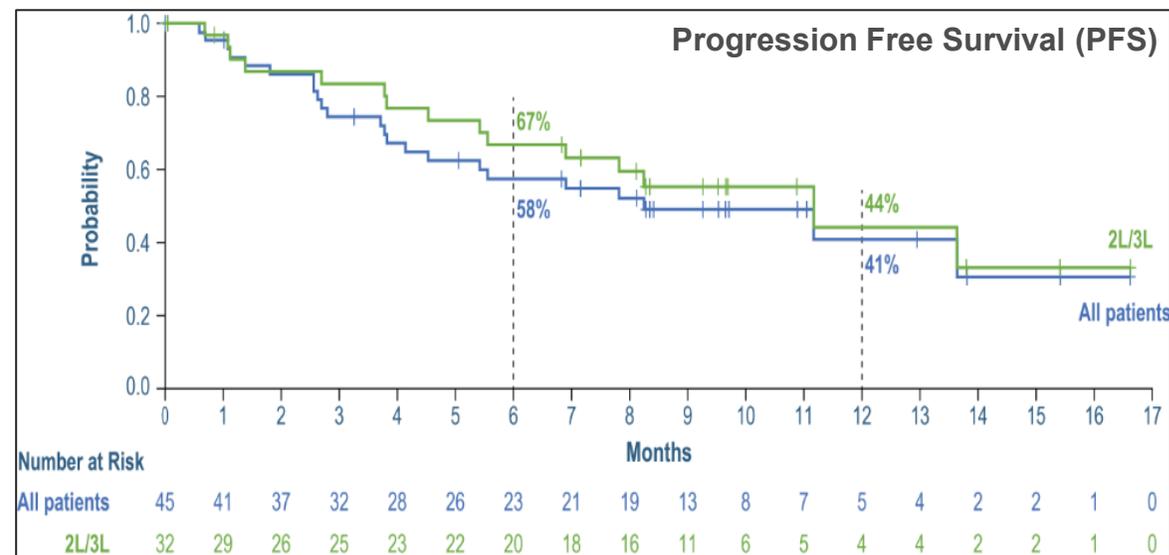
# setidegrasib (ASP3082) monotherapy demonstrates deep and durable clinical activity, with no new safety signals, in patients with advanced NSCLC with *KRAS G12D* mutation



**ORR was 37.5% in the 2L/3L setting**

Data cutoff date: November 10, 2025

Dashed line represents 30% reduction in tumor size. ORR defined as patients with best overall response of CR/PR with confirmation, or without confirmation but with possibility of confirmation at subsequent assessment; One patient had an unconfirmed PR per RECIST v1.1



**Median PFS was 11.2 months in the 2L/3L setting**

Data cutoff date: November 10, 2025

Median follow-up time, months (95% CI): All patients, 9.7 (9.1–12.4); 2L/3L, 9.7 (8.7–13.3)  
 For all patients, PFS rate (95% CI) was 58% (41–71) at 6 months and 41% (22–59) at 12 months  
 For 2L/3L patients, PFS rate (95% CI) was 67% (47–81) at 6 months and 44% (20–66) at 12 months

A Phase 3 registrational study to evaluate setidegrasib monotherapy in advanced NSCLC patients with *KRAS G12D* mutation, is being prepared for initiation in FY2026

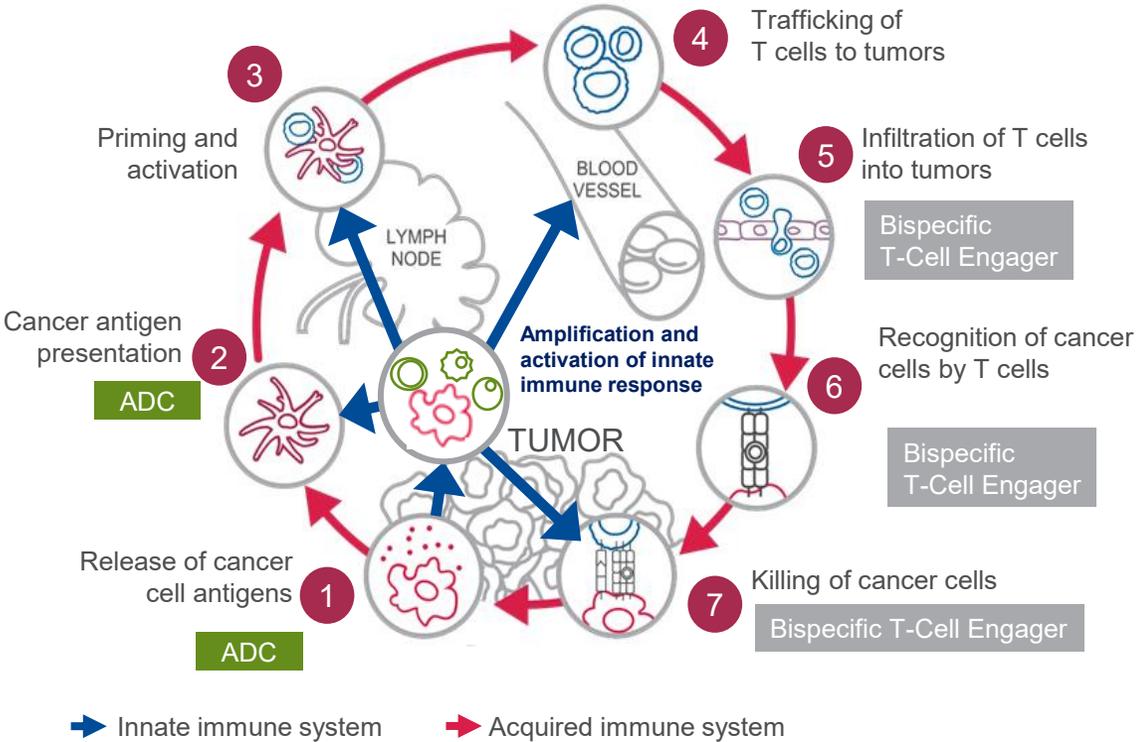
Data presented at the European Lung Cancer Conference 2026; N Engl J Med 2026 Mar; doi: 10.1056/NEJMoa2600752

NSCLC: Non-small cell lung cancer, 2L+: Second or later line, 4L+: Fourth or later line, SD: Stable disease, PD: Progressive disease, PR: Partial response, NE: Not evaluable

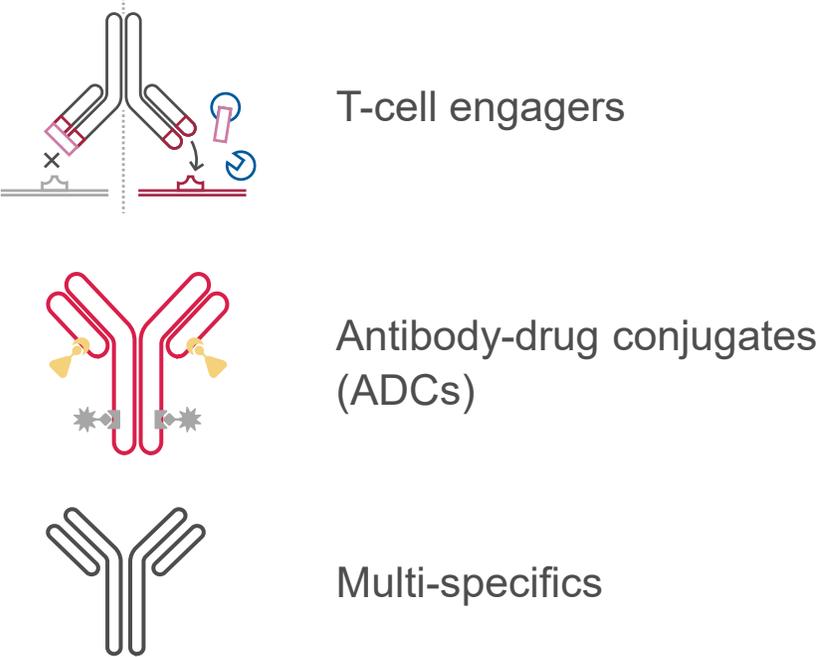
# Primary Focus Immuno-Oncology: Harnessing the immune system to generate deep and durable responses to prolong survival in patients with cancer

We are working to identify, develop and deliver treatments by targeting multiple steps of the cancer immunity cycle

Our early-stage platforms are built to trigger anti-tumor immune response by stimulating multiple immune functions at the same time



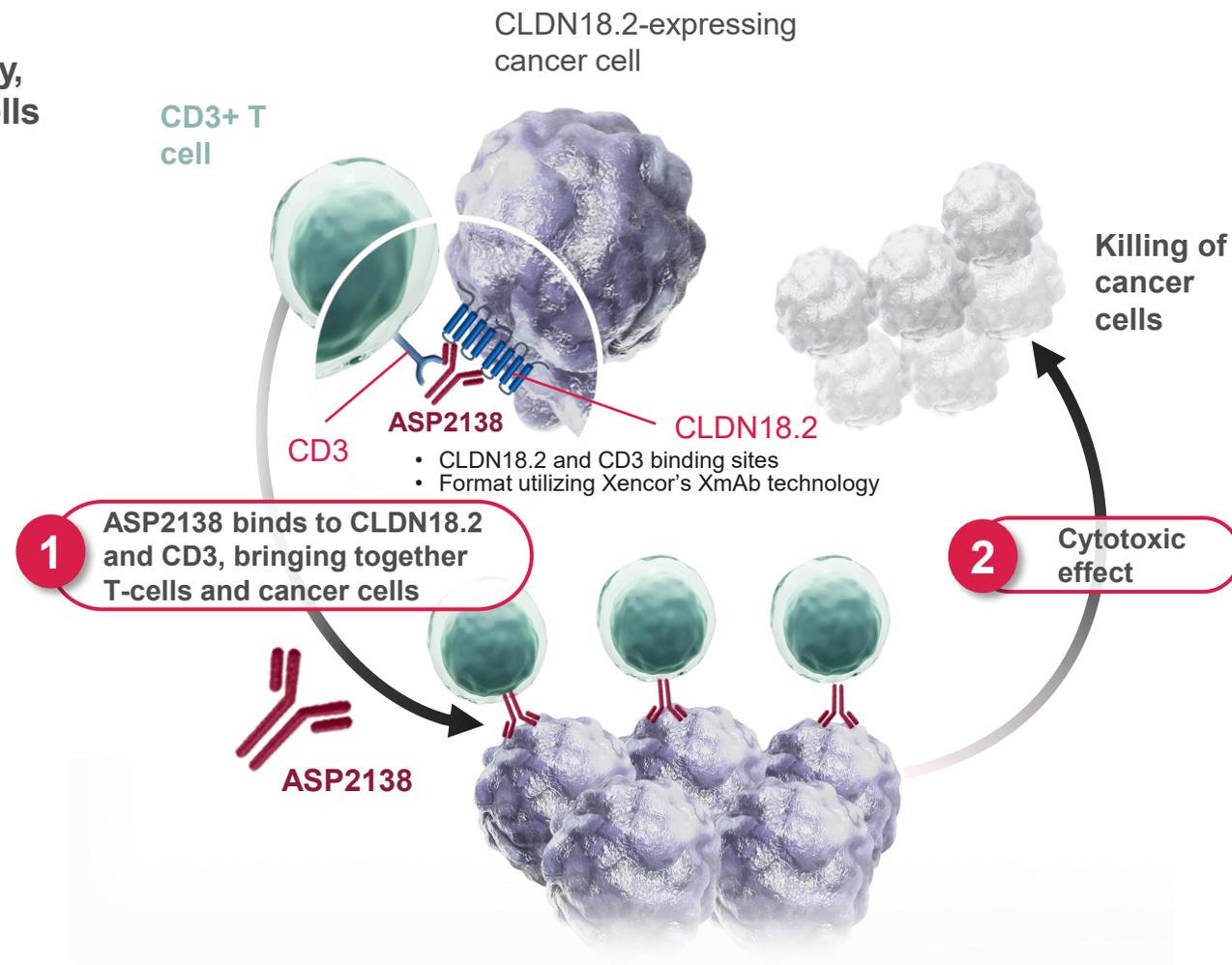
We are leveraging next-generation modality platforms with the potential to create highly differentiated therapies



# Our flagship asset, ASP2138, builds on our expertise from VYLOY

ASP2138 is a Claudin 18.2 (CLDN18.2) targeting bispecific antibody, helping to activate T-cells and enhance their ability to kill tumor cells

- **Target disease:** Gastric/GEJ (G/GEJ) adenocarcinoma, PDAC, and other tumors expressing Claudin18.2
  - ✓ CLDN18.2-positive patients: ~70% in G/GEJ adenocarcinoma<sup>1</sup> and ~60% in PDAC<sup>2</sup>
- **Clinical PoC achieved in G/GEJ adenocarcinoma**
  - ✓ Efficacy observed irrespective of CLDN18.2 expression or CPS status
- **Preparing to initiate Phase 3 trial in 1L GC**
  - ✓ Intent to treat population – patients with low-intermediate Claudin 18.2 expression not eligible for VYLOY
  - ✓ Primary analysis anticipated – FY2029



1. Gastric Cancer. 2024;27:1058, 2. Int J Cancer. 2013;134:731

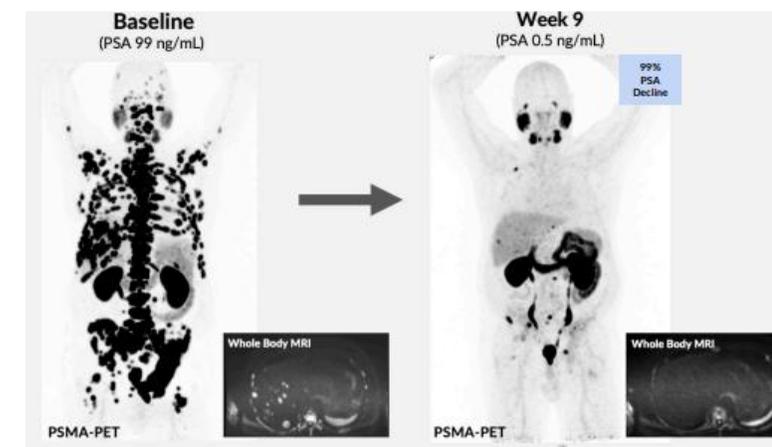
GEJ: Gastroesophageal junction, CPS: Combined Positive Score (for PD-L1), PDAC: Pancreatic ductal adenocarcinoma, PoC: Proof of Concept

# Advancing next-generation modalities in prostate cancer: Strategic collaboration with Vir Bio to advance PSMA-targeting dual-masked T-cell engager VIR-5500\*

## VIR-5500-V101 First in Human Phase 1 study: presented at ASCO GU 2026

- Dose-dependent **anti-tumor activity** (53% (9/17) PSA90, 82% (14/17) PSA 50) observed) at doses  $\geq 3,000$   $\mu\text{g}/\text{kg}$  Q3W
- **Well-tolerated** with a **favorable safety profile with no DLTs: low rate of  $\geq$  G3 TRAEs** without prophylactic corticosteroids or anti-IL-6 at doses up to  $3500$   $\mu\text{g}/\text{kg}$  Q3W

99% PSA decline; 63% decrease in tumor diameter and a complete resolution of liver lesions



Q3W Cohort 800/1500/3000  $\mu\text{g}/\text{kg}$

### Case study detail:

- 63-year-old male
- High disease burden: liver and bone lesions
- 5 Prior Lines of Treatment
- Metabolic response of PSMA-avid bone and hepatic lesions



\*Pending transaction closing

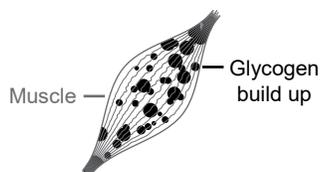
ASCO: American Society of Clinical Oncology, DLT: dose limiting toxicity, GU: Genitourinary, PSA: Prostate-specific antigen, PSMA: Prostate-specific membrane antigen, TRAE: Treatment-related adverse event

# Primary Focus Genetic Regulation: Treating neuromuscular and neurodegenerative conditions by correcting their underlying genetic causes

We are focused on making adeno-associated virus (AAV) gene therapies scalable and accessible to more patients, with the ambition to address both rare and common diseases

## Flagship program

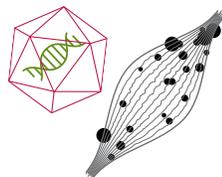
**AT845:** An AAV gene replacement therapy designed to address the **underlying cause of Pompe disease** – a devastating rare neuromuscular disease



In Pompe disease, the body cannot produce a functional enzyme, causing glycogen accumulation in muscle cells and progressive weakness

### Status

- PoC analysis ongoing



AAV gene therapy provides a healthy version of the gene which allows muscle cells to produce their own, effective enzyme

## Follow-on program

**ASP2957:** A next-generation investigational gene therapy for **XLMTM**

- Delivers a functional human MTM1 gene using a novel muscle-targeted MyoAAV capsid
- Developed via the DELIVER<sup>1</sup> platform, MyoAAV capsids are engineered for high muscle specificity and reduced liver targeting

### Status

- ASP2957 has cleared IND, with first patient expected to be dosed in Q1/FY2026
- VALOR is a Phase 1/2 clinical trial evaluating the safety, tolerability, and preliminary efficacy of ASP2957 gene therapy in young boys with XLMTM<sup>2</sup>

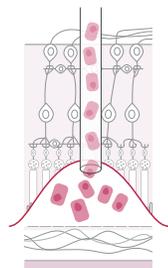
1. DELIVER: "Directed evolution of AAV capsids leveraging in vivo expression of transgene RNA". Tabebordbar M, et al. Cell. 2021;184:4919–49382, 2. ClinicalTrials.gov NCT07052929  
AAV: Adeno-associated virus, IND: Investigational New Drug, PoC: Proof of concept, XLMTM: X-linked myotubular myopathy

# Primary Focus Blindness & Regeneration: Realizing a brighter future for people with retinal diseases

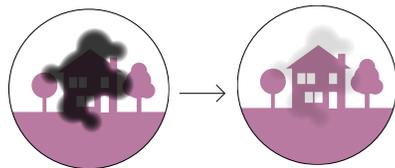
We are looking to preserve and restore vision with a multi-modality approach of potentially transformative treatments and regenerative medicines, including pluripotent stem cells and adeno-associated viruses

## Flagship program

**ASP7317:** One of the **first ever ophthalmic cell therapy** derived from pluripotent stem cells to enter the clinic



RPE



Retinal pigment epithelium (RPE) cells created from pluripotent stem cells can be transplanted into the eye to replace the damaged cells.

Potentially restoring retinal function and vision.

## Status

- **PoC achieved** in patients with severe vision impairment due to geographic atrophy
- Preliminary Phase 1b safety and efficacy data to be presented at ARVO in May 2026

ARVO: Association for Research in Vision and Ophthalmology, IND: Investigational New Drug application, PoC: Proof of Concept

## Follow-on program

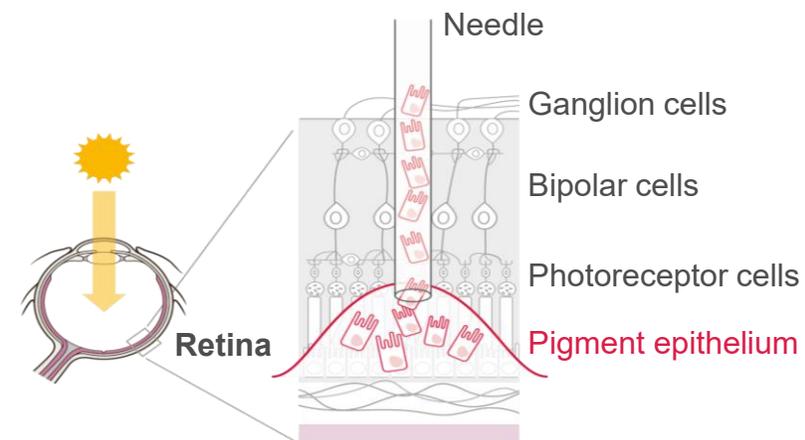
**ASP2020:** A **universal donor cell allogeneic cell therapy**, replacing retinal pigment epithelium to restore lost sight in Stargardt-type macular dystrophies



Universal Donor Cell



Retinal pigment epithelium



## Status

- IND anticipated in Q1/FY2026

Looking Ahead:  
Sustaining Long-term  
VALUE Creation



# We are building an innovation engine to deliver the next generation of breakthrough medicines at Astellas

2024-2026



## Enhance the Foundation

- Transform the R&D organization
- Strengthen productivity and governance

2027-2029



## Enrich the Pipeline

- Increase quality and coherence of the pipeline
- Enhance prioritization

2030-2034



## Excel

- R&D productivity trending toward top-level industry performance
- Deliver consistent high-value, de-risked pipeline progression



# With one goal in mind – creating VALUE for patients

1

## Accelerate Flagship Programs and LCM Initiatives

- Maximize LCM value
- Accelerate Phase 3 start
- Enroll faster and more efficiently

2

## Focus on Follow-on Programs

- Increase success rate of programs
- Enhance decision-making
- Build capabilities to increase productivity

3

## Target Next Innovation

- Enrich quantity/quality of preclinical and clinical programs
- Terminate faster and earlier
- Focus on biology, hiring top talent and partnership

by  
2034

Take **10 New Molecular Entities (NMEs)** into Phase 3 registrational programs by the end of 2034

LCM: Lifecycle management

# In Summary



We have fundamentally transformed our R&D organization and operating model...

- Successfully building **global, end-to-end capabilities** across our transformed R&D organization
- **Increasing R&D productivity** through a disciplined, quality-driven framework
- **Accelerating and enriching the pipeline** by focusing on key platforms with multiple assets

**...to deliver long-term, sustainable growth and VALUE for patients**



Q&A

