

Q3 YTD/FY2024 Financial Results



Atsushi Kitamura
Chief Financial Officer (CFO)
Astellas Pharma Inc.
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Agenda

I

**Q3 YTD/FY2024 Consolidated Financial Results
FY2024 Revised Forecast**

II

Initiatives for Sustainable Growth

Q3 YTD/FY2024 Financial Results: Key Message

Revenue

- **Increased significantly YoY (+22%)**
- Strategic Brands: Expanded to over 240.0 bil. yen **(+140.0 bil. yen YoY)**

*SG&A expenses**

- SG&A ratio **improved by 4.0ppt** YoY, driven by robust progress of SMT (Sustainable Margin Transformation)

Core operating profit

- **Increased significantly YoY (+44%)**, driven by growth of XTANDI, Strategic Brands and SMT cost optimization

Revised full-year forecast

- Upward revision of revenue **(+100.0 bil. yen)**, core OP **(+70.0 bil. yen)** based on robust core business progress


Q3 YTD/FY2024 Financial Results

(billion yen)	Q3 YTD FY2023	Q3 YTD FY2024	Change	Change (%)	FY2024 Latest FCST	FX impact (YoY)
Revenue	1,189.1	1,453.0	+264.0	+22.2%	1,900.0	+66.3
Cost of sales	219.3	272.3	+53.1	+24.2%	345.0	+7.6
SG&A expenses	547.0	631.7	+84.8	+15.5%	845.0	+31.7
US XTANDI co-pro fee	146.2	200.1	+53.9	+36.8%	255.0	+12.1
SG&A excl. the above	400.7	431.6	+30.9	+7.7%	590.0	+19.6
(SG&A ratio*)	33.7%	29.7%	-4.0ppt		31.1%	
R&D expenses	216.3	251.4	+35.1	+16.2%	340.0	+9.9
(R&D ratio)	18.2%	17.3%	-0.9ppt		17.9%	
Core operating profit**	206.5	297.5	+91.0	+44.1%	370.0	+17.1
(Core OP margin)	17.4%	20.5%	+3.1ppt		19.5%	
< Full basis >						
Amortisation of intangible assets	66.2	104.2	+38.0	+57.5%		Note) Amortisation of IZERVAY's intangible assets started from Q2/FY2023
Other income	8.5	4.4	-4.1	-47.9%		Other expenses (booked in Q3)
Other expenses	84.0	220.6	+136.6	+162.7%		• Impairment losses on intangible assets: 180.5 Major impairment losses include: IZERVAY (Ex-US): 115.1, AT466: 51.8, iota: 8.0
Operating profit	74.1	-22.5	-96.6	-	11.0	
Profit before tax	73.6	-29.3	-102.9	-	1.0	
Profit	50.3	-24.1	-74.5	-	14.0	






Latest FCST for revenue and profit at each stage were announced on Jan 24, 2025. FX rates for Latest FCST: 153 yen/USD, 164 yen/EUR. Actual FX rates for Q3 YTD/FY2024: 152 yen/USD, 165 yen/EUR
 *Excl. US XTANDI co-pro fee, **The definition of core-basis was changed from Q1/FY2024. In addition to the old definition's adjustments, 'Amortisation of intangible assets', 'Gain on divestiture of intangible assets' and 'Share of profit (loss) of investments accounted for using equity method' were newly excluded as new adjustment items.

Q3 YTD/FY2024 Financial Results: XTANDI and Strategic Brands

XTANDI: US performance exceeded expectations, while other regions expanded as expected

(billion yen)	Q3 YTD/FY2024	YoY	FY2024 FCST*	
	703.1	+143.1 (+26%)	909.9	<ul style="list-style-type: none"> ✓ Strong US growth driven by EMBARK impact (M0 CSPC) ✓ Upward revision of FCST based on Q3 overperformance, despite the anticipated negative impact from US IRA Medicare Part D redesign in Q4

Strategic Brands: On track to achieve total FCST of over 340.0 bil. yen, building confidence towards FY2025 target 500.0 bil. yen

(billion yen)	Q3 YTD/FY2024	YoY	FY2024 FCST*	
Strategic Brands Total	243.8	+138.1 (+131%)	344.9	<ul style="list-style-type: none"> ✓ Significant contribution to profit growth ✓ Expect further growth in FY2025 and beyond
	117.0	+61.4 (+110%)	165.2	<ul style="list-style-type: none"> ✓ Continues to demonstrate strong global growth ✓ Expect steady growth moving forward, primarily driven by ex-US 1L mUC
	44.4	+39.2 (+743%)	71.5	<ul style="list-style-type: none"> ✓ Label update resubmission accepted by FDA (PDUFA date: Feb 26) ✓ Expect growth to accelerate after approval
	24.4	+20.9 (+586%)	32.5	<ul style="list-style-type: none"> ✓ Steady global sales growth, in line with expectations ✓ Expect steady linear growth moving forward
	4.9	+4.9	9.5	<ul style="list-style-type: none"> ✓ Uptake in Japan, US and Europe exceeded expectations Aided by higher-than-expected rates of CLDN18.2 testing ✓ Upward revision of FCST reflecting strong performance
	53.1	+11.8 (+29%)	66.2	<ul style="list-style-type: none"> ✓ Sales expanded in all regions, led by the US performance ✓ Expect continued moderate growth moving forward

*FY2024 Latest FCST announced in Feb 2025, FX rates for Latest FCST: 153 yen/USD, 164 yen/EUR (Q4 forecast: 155 yen/USD, 163 yen/EUR)

M0: Non-metastatic, CSPC: Castration-sensitive prostate cancer, IRA: Inflation Reduction Act, 1L: First line, mUC: Metastatic urothelial cancer, FDA: Food and Drug Administration, PDUFA: Prescription Drug User Fee Act, CLDN18.2: Claudin 18.2, VEOZAH: Approved as "VEOZA" in ex-US

Business Update: PADCEV, IZERVAY, VYLOY



Global sales driven by 1L mUC

- **Strong quarterly global growth driven by ex-US**, while maintaining steady growth in the US (QoQ growth: Global +12%, outside US +29%)
- **Ex-US 1L mUC** demonstrating strong uptake
- 1L mUC approval countries increased to 16 (+5 countries from Q2)
Expect increase in approval and reimbursement
- US 1L mUC share continues to be at a high level, with both new patient start and market share approaching 55%
- Overall sales growth expected to be driven by ex-US performance, while moderate growth trend expected to continue in the US
- Expect continued solid global growth in FY2025
- Next potential growth opportunity is **MIBC** with TLR expected in FY2025



US business entering a robust growth phase

- Q3 sales affected by temporary impact from CRL and changes in inventory levels
- High level share maintained even before label update. Continues to be the #1 chosen treatment option for new patient start (Oct-Nov)
 - ✓ **New patient start share: ~60%**
 - ✓ **Market share: ~40%**
- Over 210K vials* shipped since launch as of Q3
- Available in ~1,800 Retina accounts
- Post-marketing safety profile remains consistent with clinical trial results
- DTC campaign progressing on track, expect market growth to accelerate moving forward
- Label update resubmission accepted by the FDA (PDUFA date: Feb 26)
- **Expect growth to accelerate after approval**



Encouraging uptake, expect further growth

- Approved in 38 countries, launched in 9 countries (as of Q3)
 - ✓ Launched in the US in Oct, Germany in Nov
 - ✓ Approved in China in Dec
- Uptake exceeded expectations, primarily driven by Japan and US performance
- Higher-than-expected rates of CLDN18.2 testing
- Listed as preferred recommendation in major treatment guidelines
 - ✓ US: NCCN Guidelines (Category 1)
 - ✓ Japan: Gastric cancer treatment guideline (Preferred)
- For FY2025, expect further growth in Japan, US and Europe, as well as contribution from China
- Expect substantial sales contribution as one of the key growth drivers

*Excl. clinical trials

1L: First line, mUC: Metastatic urothelial cancer, MIBC: Muscle-invasive bladder cancer, TLR: Topline results, CRL: Complete response letter, DTC: Direct-to-consumer, FDA: Food and Drug Administration, PDUFA: Prescription Drug User Fee Act, CLDN18.2: Claudin 18.2, NCCN: National Comprehensive Cancer Network



Q3 YTD/FY2024 Financial Results: SG&A and R&D Expenses

- Robust progress of SMT initiatives toward the FY2024 target of 40.0 bil. yen in cost optimization
- **SG&A ratio improved to 29.7% (-4.0ppt YoY)**

Core basis: YoY comparison and ratio to revenue

Cost Items	YoY change	Ratio to Revenue	
			(billion yen)
SG&A expenses excl. US XTANDI co-pro fee	+7.7% (+2.8% excl. FX impact)	SG&A ratio: 29.7%	<ul style="list-style-type: none"> ✓ Global organizational restructuring (approx. -12.0 YoY) ✓ Reduction of mature products-related expenses (approx. -8.0 YoY) ✓ Enhance company-wide efficiency with AI and digital (approx. -4.0 YoY) Allocate generated resources to Strategic Brands investment
R&D expenses	+16.2% (+11.6% excl. FX impact)	R&D ratio: 17.3%	<ul style="list-style-type: none"> ✓ PF, Strategic Brands LCM and enhanced R&D functions (approx. +16.0 YoY) ✓ One-time co-development cost payments in Q1 ✓ Steady progress in outsourcing reduction through strengthening in-house capabilities

FY2024 Revised Forecast

- **Core basis:** Upward revision based on robust progress of revenue and SMT
- **Full basis:** Downward revision of profit mainly due to impairment losses on IZERVAY (Ex-US) and AT466
- No change in dividend forecast of 74 yen

Exchange rates for Latest forecast: 153 yen/USD, 164 yen/EUR
(Forecast rates Q4: 155 yen/USD, 163 yen/EUR)

(billion yen)	FY2023 Actual	FY2024			Main items of revision
		Previous FCST	Latest FCST	Change	
Revenue	1,603.7	1,800.0	1,900.0	+100.0	<ul style="list-style-type: none"> • XTANDI: approx. +30.0 • FX impact: approx. +45.0
SG&A expenses	740.1	823.0	845.0	+22.0	
US XTANDI co-pro fee	194.9	229.0	255.0	+26.0	<ul style="list-style-type: none"> • Incorporate robust progress of SMT
SG&A excl. the above	545.2	594.0	590.0	-4.0	
(SG&A ratio*)	34.0%	33.0%	31.1%	-1.9ppt	
R&D expenses	294.2	341.0	340.0	-1.0	<ul style="list-style-type: none"> • No significant change
(R&D ratio)	18.3%	18.9%	17.9%	-1.0ppt	
Core operating profit**	276.9	300.0	370.0	+70.0	<ul style="list-style-type: none"> • FX impact: approx. +10.0
(Core OP margin)	17.3%	16.7%	19.5%	+2.8ppt	

< Full basis >

Operating profit	25.5	80.0	11.0	-69.0	<ul style="list-style-type: none"> • Impairment loss: approx. -180.0 (Ex-US IZERVAY: -120.0, AT466: -50.0, iota: -10.0) • Release of impairment loss risk and other expenses incorporated in initial FCST
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FY2024 Previous FCST announced in Oct 2024. FX rates for Previous FCST: 149 yen/USD, 160 yen/EUR

SMT (Sustainable Margin Transformation): See slide 28 for overview

*Excl. US XTANDI co-pro fee, **The definition of core-basis was changed from Q1/FY2024. In addition to the old definition's adjustments, 'Amortisation of intangible assets', 'Gain on divestiture of intangible assets' and 'Share of profit (loss) of investments accounted for using equity method' were newly excluded as new adjustment items

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Initiatives for Sustainable Growth

XTANDI and Strategic Brands: FY2024 Key Expected Events

(Blue: Updates since the last financial results announcement)

	Q1 (Apr-Jun)	Q2 (Jul-Sep)	Q3 (Oct-Dec)	Q4 (Jan-Mar)
enzalutamide/ XTANDI	★ Jun	Approval (M1 CSPC; China)		
enfortumab vedotin/ PADCEV		★ Aug	Approval (2L+ mUC; China, 1L mUC; Europe)	★ Jan
		★ Sep	Approval (1L mUC; Japan)	
zolbetuximab/ VYLOY	★ May	Resubmission acknowledgment (US)	★ Oct	★ Dec
		★ Sep	Approval (Europe)	★ Dec
			Approval (US)	Approval (China)
				Interim analysis (Pancreatic)
				IDMC recommended study continuation to final analysis (expected in 2H/FY2025*)
avacincaptad pegol/ IZERVAY			Complete response (Label update; US) ★ Nov	★ Jan
			Resubmission acknowledgment	🎯 PDUFA date Feb
		Withdrawal of MAA (Europe) ★ Oct		Filing (Japan)

As of Feb 2025. *The timeline is subject to shift due to its event-driven nature.

M1: Metastatic, CSPC: Castration-sensitive prostate cancer, 2L+: Second or later line, mUC: Metastatic urothelial cancer, 1L: First line, IDMC: Independent Data Monitoring Committee, MAA: Marketing Authorization Application, PDUFA: Prescription Drug User Fee Act

avacincaptad pegol / IZERVAY: Latest Status

Regulatory activities are in progress globally to maximize product potential



US

Revised sNDA for label update accepted in Jan
✓ PDUFA date: Feb 26



EU

Individual discussions with regulatory authorities in major countries ongoing



Japan

NDA submission for Conditional Approval based on overseas clinical study results planned in Feb

Others

Regulatory applications completed in 9 countries (UK, Canada, Australia, etc.) and additional submissions being planned

Progress in Focus Area Approach: Current Status of Programs in Clinical Trial

(Blue: Updates since the last financial results announcement)

Primary Focus	Biology/Modality/Technology	Program	Mechanism of Action	Current status
Immu- Oncology	Checkpoint	ASP1570 ●	DGKζ inhibitor	Phase 1 study ongoing
	Bispecific immune cell engager	★ ASP2138 ●	Anti-CLDN18.2 and anti-CD3	Phase 1 study ongoing
		ASP1002 ●	Anti-CLDN4 and anti-CD137	Phase 1 study ongoing
	Oncolytic virus (systemic)	ASP1012 ●	Leptin-IL-2	Phase 1 study ongoing
Cancer cell therapy	ASP2802 ●	CD20 <i>convertible</i> CAR-T (autologous)	Terminated	
Targeted Protein Degradation	Protein degradation	★ ASP3082 ●	KRAS G12D degrader	Phase 1 study ongoing
		ASP4396 ●	KRAS G12D degrader	Phase 1 study ongoing
Genetic Regulation	Gene replacement (AAV)	AT132 ●	MTM1 gene	ASPIRO study put on clinical hold by FDA in Sep 2021
		★ AT845 ●	GAA gene	Phase 1 study ongoing
		ASP2016 ●	FXN gene	Terminated
Blindness & Regeneration	Cell replacement	★ ASP7317 ●	RPE cells	Phase 1b study ongoing
Others (Non-PF)	Long-acting abiraterone prodrug	ASP5541 (PRL-02) ●	CYP17 lyase inhibitor	Phase 1 study ongoing
	Immune modulation*	ASP5502 ●	STING inhibitor	Phase 1 study ongoing

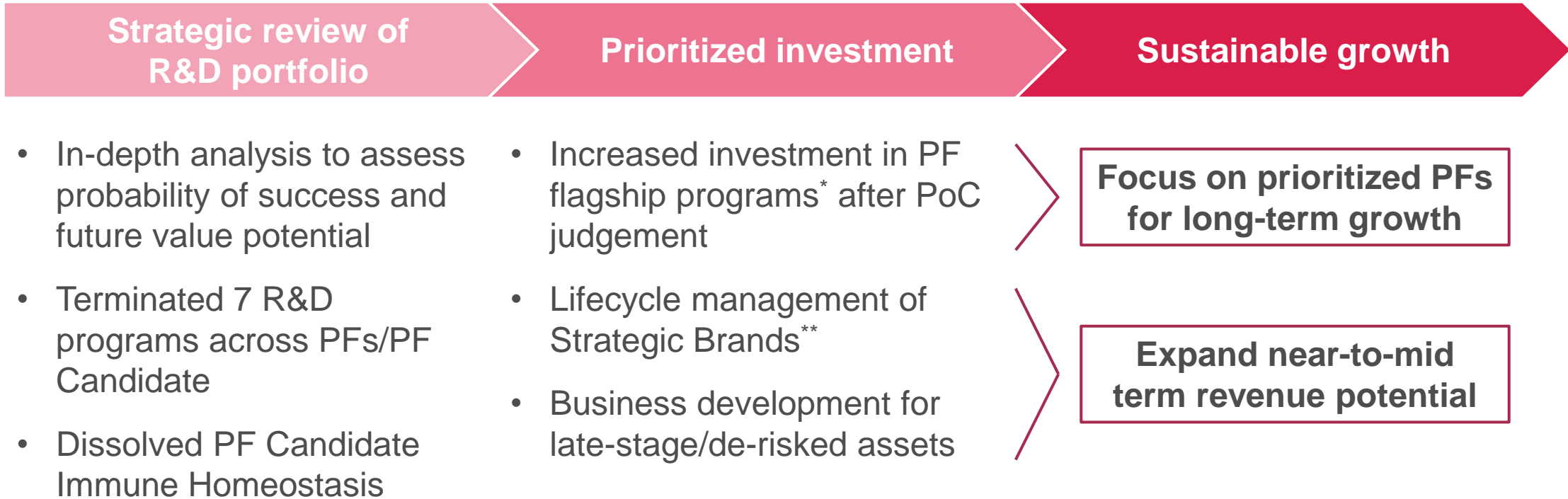
Modality	
●	Small molecule
●	Antibody
●	Gene
●	Cell

*PF Candidate Immune Homeostasis dissolved

★: Flagship program (See [slides 30-31](#) for overview)

Improvement of R&D Productivity

Strategically reviewed R&D portfolio with discipline to allocate resource to prioritized assets



*Targeted Protein Degradation: ASP3082, Immuno-Oncology: ASP2138, Genetic Regulation: AT845, Blindness & Regeneration: ASP7317. See [slides 30-31](#) for overview.

**See [slide 29](#) for details of LCM activities.

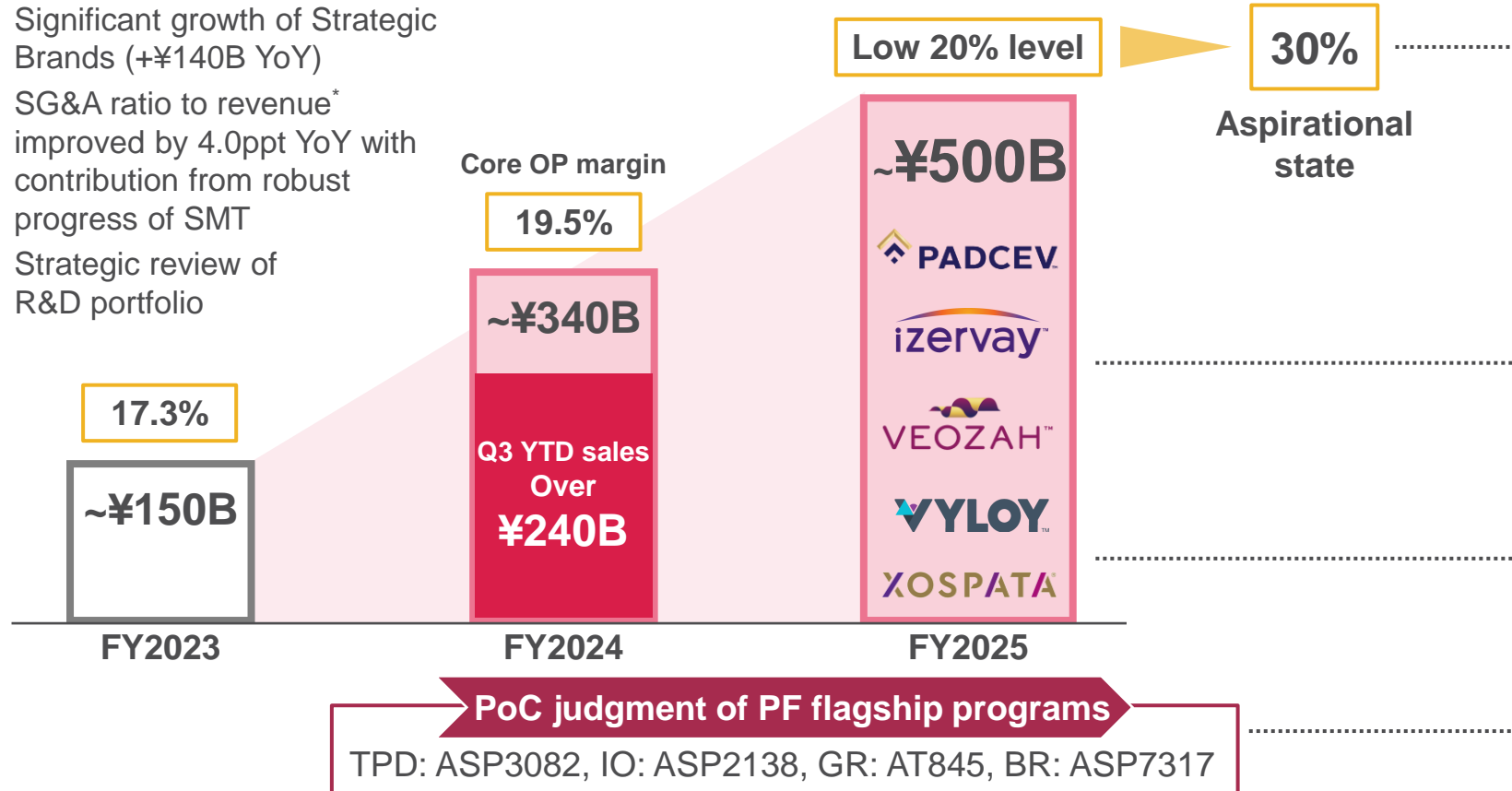
PF: Primary Focus, PoC: Proof of concept

Progress in Q3 YTD/FY2024 and Latest Outlook

Entering a fundamental growth phase to overcome XTANDI LOE and pursue further growth

Q3 Progress

- Significant growth of Strategic Brands (+¥140B YoY)
- SG&A ratio to revenue* improved by 4.0ppt YoY with contribution from robust progress of SMT
- Strategic review of R&D portfolio



Three Enterprise Priorities

- Sustainable Margin Transformation**
 - Company-wide cost optimization before XTANDI LOE
 - Fund growth investment and profit improvement
- Growth Strategy**
 - New operating model by brand level to maximize brand performance
 - Agile ways of working to promote faster decision-making
- Bold Ambition**
 - Agile R&D model to accelerate from discovery to PoC, acquiring new assets, etc.
 - Strengthening in-house capabilities for clinical trial execution and data-driven decision making

*Excl. US XTANDI co-pro fee

LOE: Loss of exclusivity, SMT: Sustainable Margin Transformation, PoC: Proof of concept, PF: Primary Focus, TPD: Targeted Protein Degradation, IO: Immuno-Oncology, GR: Genetic Regulation, BR: Blindness & Regeneration



Sustainability Meeting 2024

➤ Feb 21st 2025, 10:00-11:30 (JST)

Appendix



XTANDI and Strategic Brands: Potential Peak Sales (as of Feb 2025)

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Brand	Potential Peak Sales (Global, billions of yen)
XTANDI (enzalutamide)	over 700.0
PADCEV (enfortumab vedotin) *	400.0 – 500.0
IZERVAY (avacincaptad pegol)	200.0 – 400.0
VEOZAH (fezolinetant)	150.0 – 250.0
VYLOY (zolbetuximab)	100.0 – 200.0
XOSPATA (gilteritinib)	100.0 – 200.0

Only indications undergoing pivotal studies are included for projection (as of Feb 2025), VEOZAH: Approved as "VEOZA" in ex-US

*Disclosed as "in-market sales," not Astellas revenue. Sales for Americas are calculated based on the sales booked by Pfizer

Q3 YTD/FY2024 Actual: FX Rate

Average rate for the period

Currency	Q3 YTD/FY2023	Q3 YTD/FY2024	Change
USD	143 yen	152 yen	+9 yen
EUR	155 yen	165 yen	+9 yen

<Impact of exchange rate on financial results>

- Revenue: +66.3 billion yen
- Core OP: +17.1 billion yen

FY2024 Forecast: FX Rate & FX Sensitivity

Exchange rate Average for the period	FY2024 Previous FCST	FY2024 Latest FCST	Change
USD	149 yen	153 yen	+4 yen
EUR	160 yen	164 yen	+4 yen

Forecast rates for Q4: 155 yen/USD, 163 yen/EUR

Estimated FX sensitivity for Q4 (Jan-Mar 2025) of FY2024 latest forecasts by 1 yen depreciation

Currency	Average rate 1 yen depreciation from assumption	
	Revenue	Core OP
USD	Approx. +1.8 bil. yen	Approx. +0.3 bil. yen
EUR	Approx. +0.8 bil. yen	Approx. +0.4 bil. yen

Balance Sheet & Cash Flow Highlights

(billion yen)	FY2023 end	Dec 31, 2024
Total assets	3,569.6	3,451.6
Cash and cash equivalents	335.7	179.9
Total equity attributable to owners of the parent	1,596.0	1,493.8
Equity ratio (%)	44.7%	43.3%
(billion yen)	Q3 YTD/FY2023	Q3 YTD/FY2024
Cash flows from operating activities	100.5	93.4
Cash flows from investing activities	-823.6	-86.5
Free cash flows	-723.1	7.0
Cash flows from financing activities	583.1	-170.6
Increase/decrease in short-term borrowings and commercial papers	263.2	-175.6
Proceeds from issuance of bonds and long-term borrowings	471.6	200.0
Redemption of bonds and repayments of long-term borrowings	-6.7	-32.7
Dividends paid	-116.7	-129.0

Balance of Bonds and Borrowings Highlights

(billion yen)	Sep 30, 2024	Dec 31, 2024
Balance of bonds and borrowings	927.5	915.4
Non-current liabilities	620.2	585.5
Bonds	350.0	320.0
Long-term borrowings	270.2	265.5
Current liabilities	307.3	329.9
Commercial papers	164.8	179.8
Short-term borrowings	91.8	67.2
Current portion of long-term borrowings	50.6	52.9
Current portion of bonds	-	30.0

Main Intangible Assets (as of Dec 31, 2024)

	Bil. yen	Foreign currency*
AT132	17.1	\$109M
AT845	11.4	\$73M
Gene therapy related technology**	69.0	\$439M
VEOZAH	91.0	€524M
VYLOY	61.0	€470M
IZERVAY (US)	687.0	\$4,371M
IZERVAY (Ex-US)	54.2	\$345M

VEOZAH: Approved as "VEOZA" in ex-US

*VEOZAH, VYLOY: foreign currency is a reference value based on the currency at the time of acquisition of the intangible asset

**Acquired during the acquisition of Audentes (now Astellas Gene Therapies)

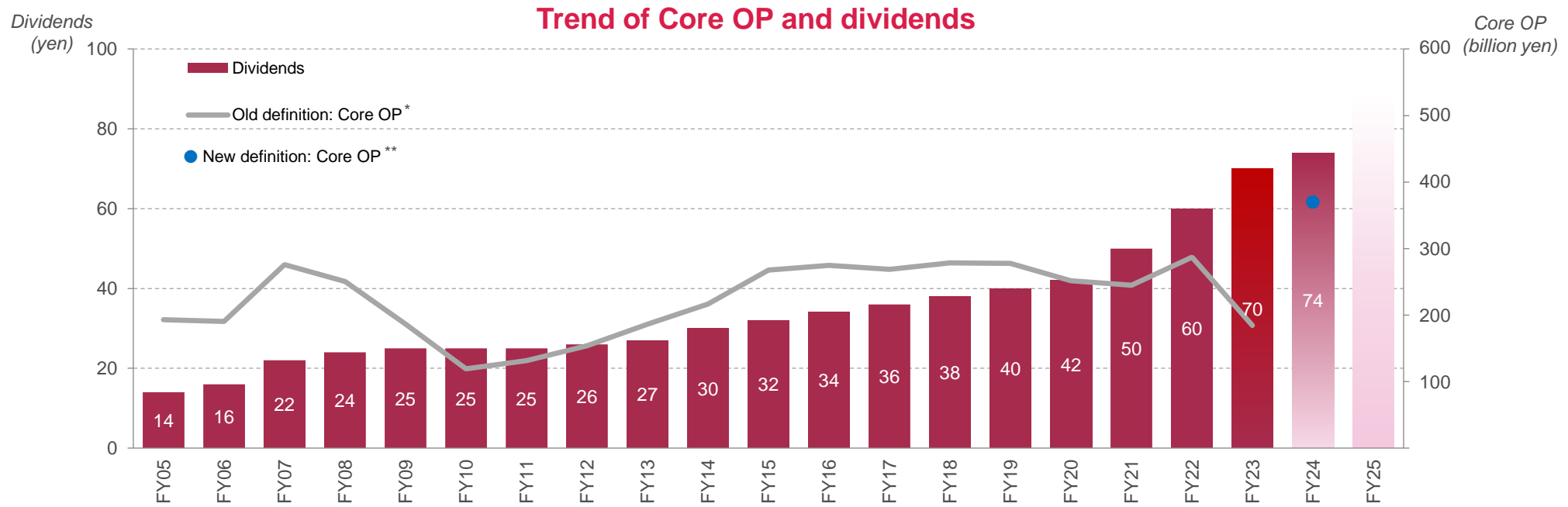
Capital Allocation

1 Top priority is investment for business growth

2 Raise dividend level aligned with profit / cashflow plan and actual performance throughout CSP2021 period

3 Flexibly execute share buyback by excess cash

Aiming for higher level of dividends increase during CSP2021 aligned with the robust profit growth forecast



For illustrative purposes only

CSP: Corporate Strategic Plan

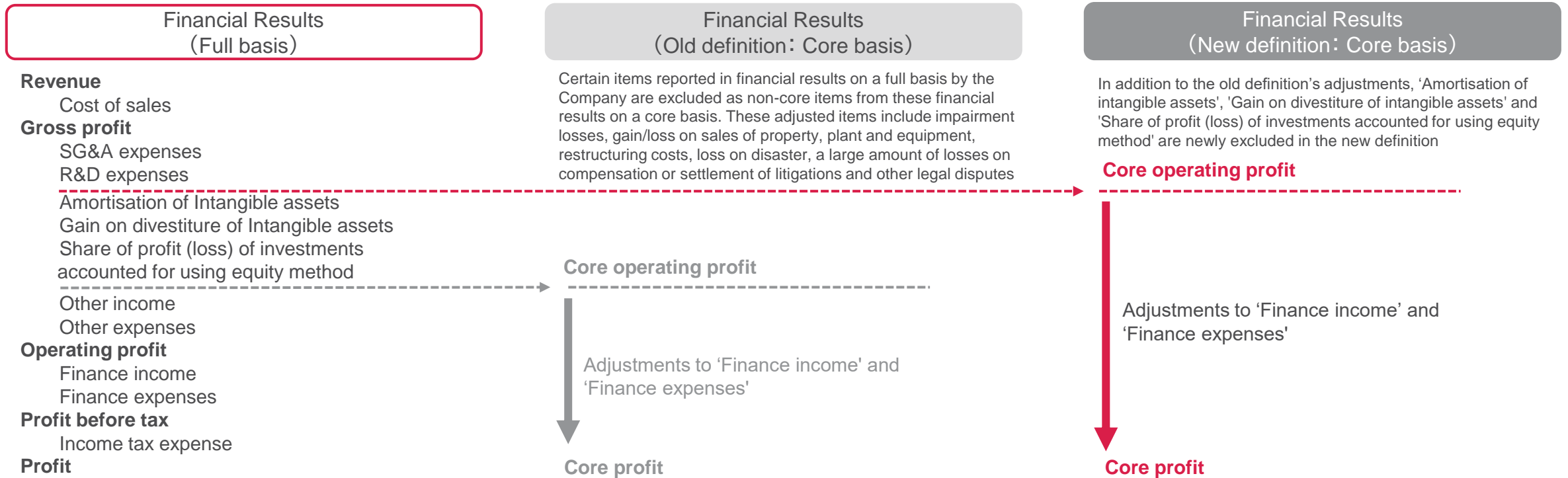
*Prior to FY2012, operating profit is in accordance with J-GAAP

**Change in definition of core basis from FY2024



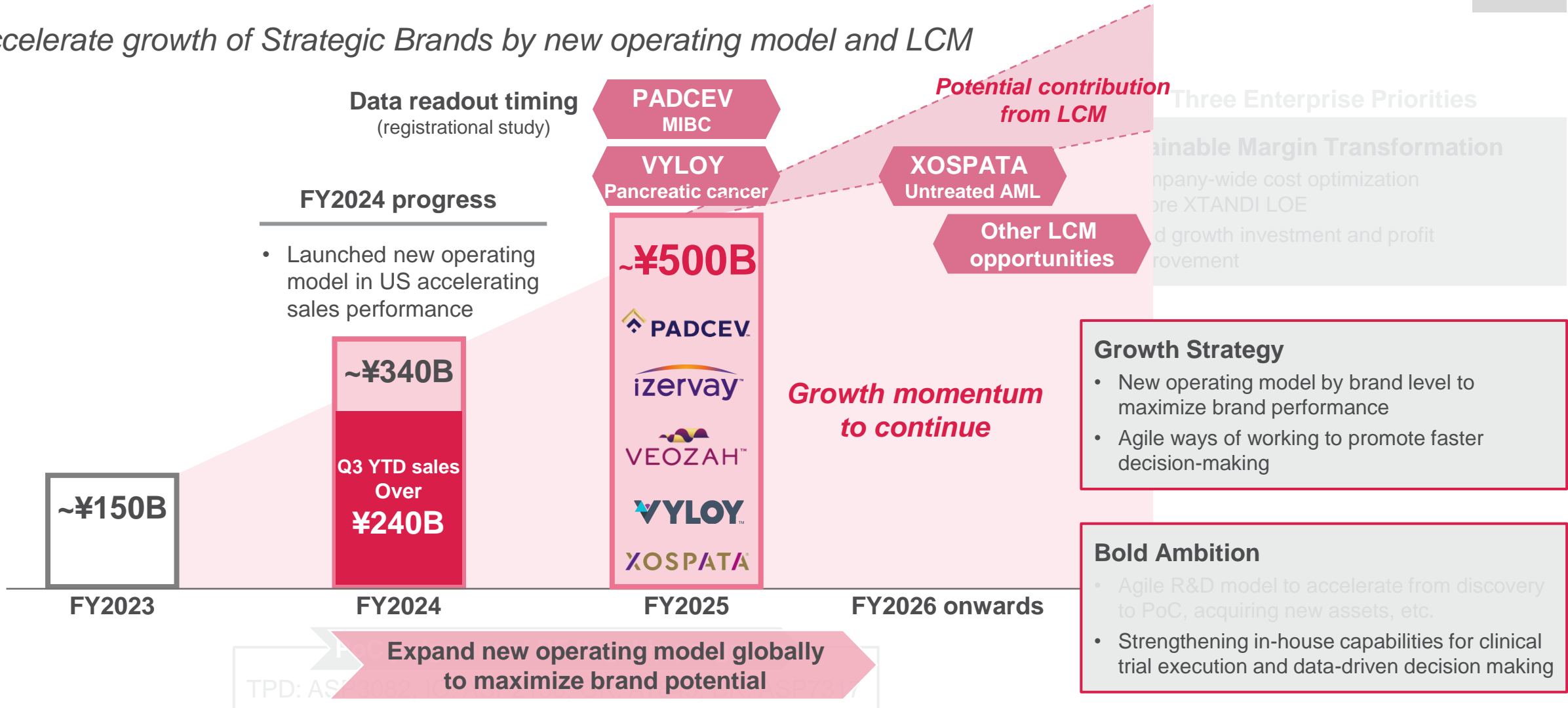
Core Basis Performance: Changes in Definitions and Context

Introduce New definition of core-based performance from FY2024



Maximize Potential of Strategic Brands

Accelerate growth of Strategic Brands by new operating model and LCM



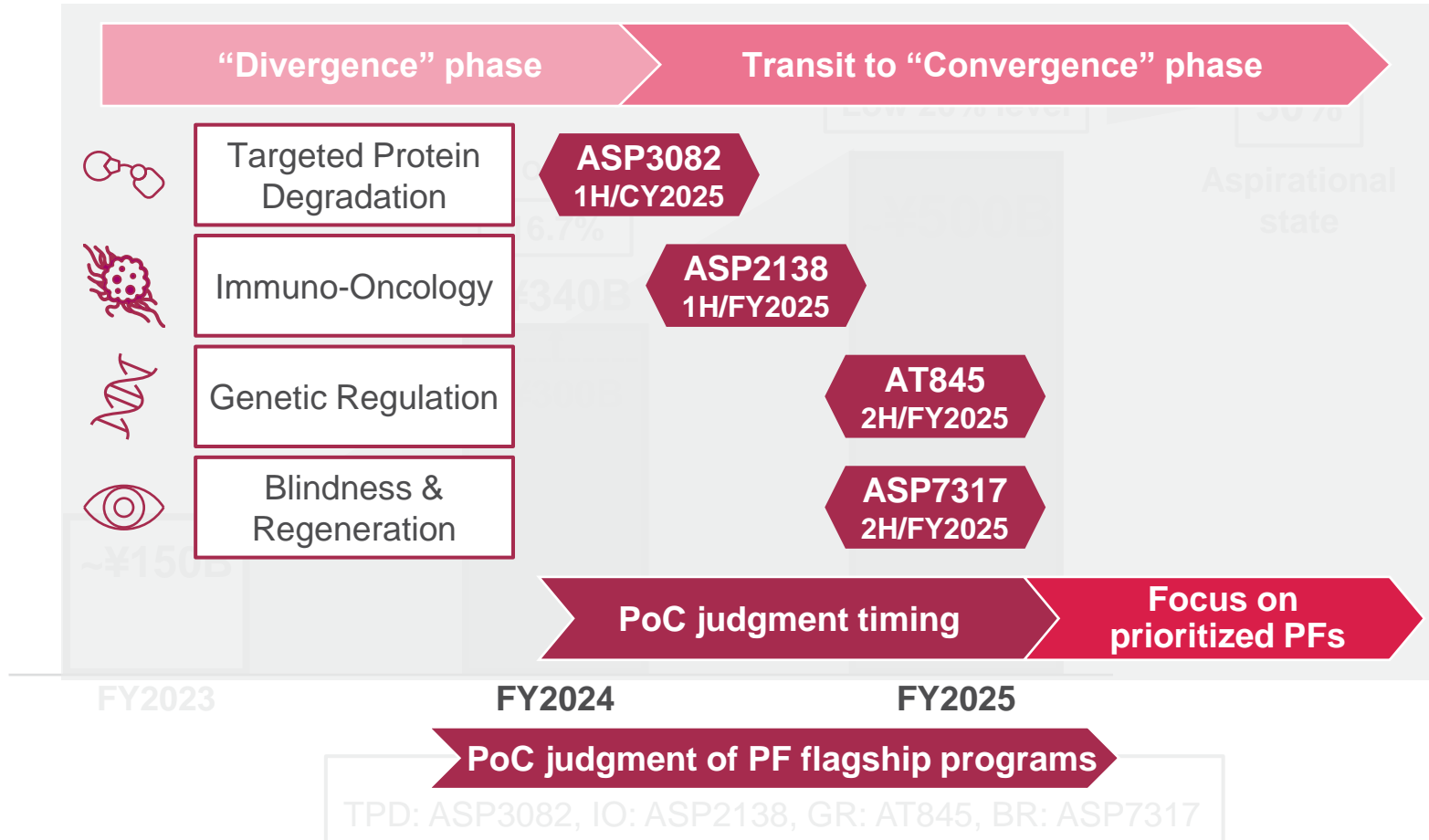
See slide 29 for details of LCM activities.

LCM: Lifecycle management, MIBC: Muscle-invasive bladder cancer, AML: Acute myeloid leukemia



Advance Focus Area Approach

- Focus on prioritized Primary Focuses and increase pipeline value based on PoC judgment of flagship programs
- Continue exploratory research at the frontier with discipline, to generate new programs for future growth



Sustainable Margin Transformation

- Company-wide cost optimization before XTANDI LOE
- Fund growth investment and profit improvement

Expect sales contribution in 2030's

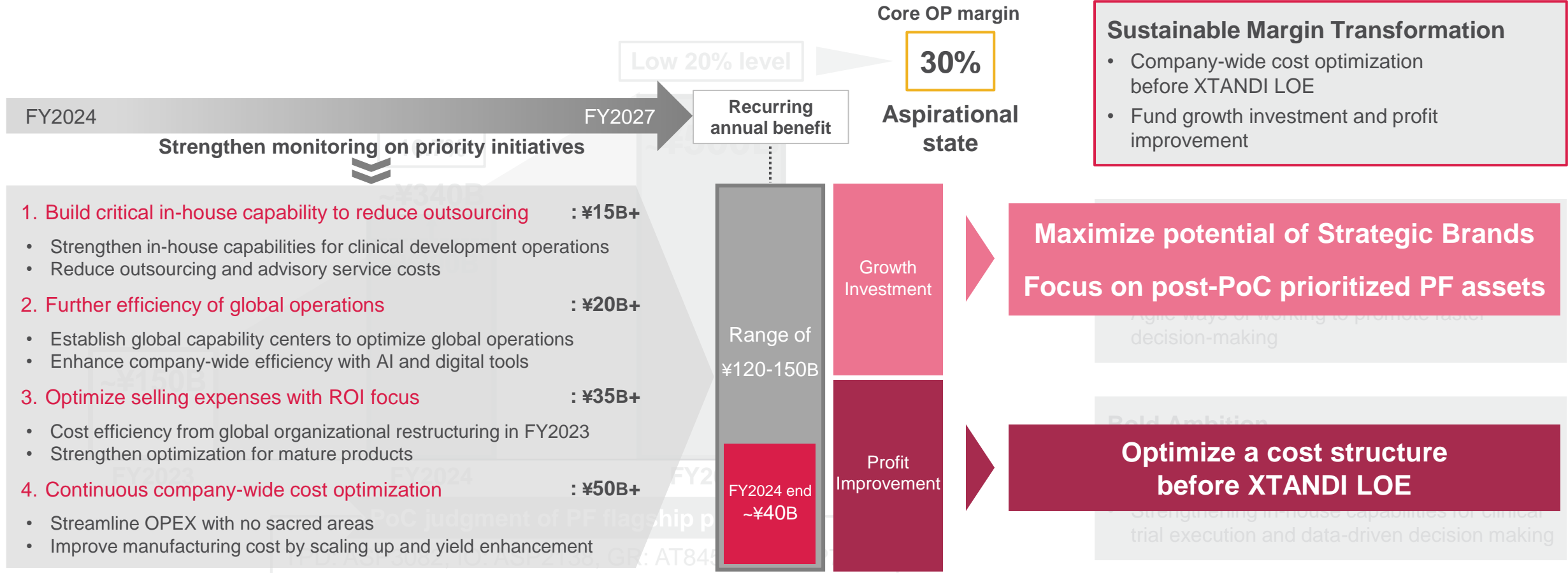
- New operating model by brand level to maximize brand performance
- Agile ways of working to promote faster decision-making

Bold Ambition

- Agile R&D model to accelerate from discovery to PoC, acquiring new assets, etc.
- Strengthening in-house capabilities for clinical trial execution and data-driven decision making

Sustainable Margin Transformation

- Company-wide cost optimization of 120-150 billion yen before XTANDI LOE
- Fund growth investment and profit improvement








LOE: Loss of exclusivity, ROI: Return On Investment, PoC: Proof of concept, PF: Primary Focus

Lifecycle Management of Strategic Brands

(Blue: Updates since the last financial results announcement)

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Product	Indication	Current status	Next milestone
 <p>PADCEV enfortumab vedotin Injection for IV infusion 20 mg & 30 mg vials</p>	MIBC	Phase 3 EV-303 & EV-304 studies ongoing	TLR anticipated for FY2025
	NMIBC	Phase 1 EV-104 study ongoing	TLR anticipated for FY2025
	Head and neck cancer	2L+: Next step under discussion	(Under discussion)
		1L: Phase 2 EV-202 study ongoing	TLR anticipated for FY2025
 <p>izervay (avacincaptad pegol intravitreal solution) 2 mg</p>	GA secondary to AMD	Japan: NDA submission under preparation	NDA submission in Feb 2025
	Stargardt disease	LCM opportunities under consideration (e.g. prefilled syringe, sustained release)	(Under discussion)
 <p>VEOZAH (fezolinetant) tablets 45 mg</p>	VMS associated with menopause	Japan: Phase 3 STARLIGHT 2 & 3 studies ongoing	TLR anticipated for FY2026
	VMS in breast cancer women	Phase 3 HIGHLIGHT 1 study ongoing	TLR anticipated for FY2027
 <p>VYLOY zolbetuximab for injection 100mg vial</p>	Gastric and GEJ cancer	Phase 3 study in combo with CPI and chemotherapy under preparation	Study start in Q1/FY2025
	Pancreatic cancer	Registrational Phase 2 GLEAM study ongoing	TLR anticipated for 2H/FY2025
 <p>XOSPATA gilteritinib 40mg tablets</p>	Newly diagnosed AML (HIC-eligible)	Phase 3 PASHA study ongoing	TLR anticipated for FY2026

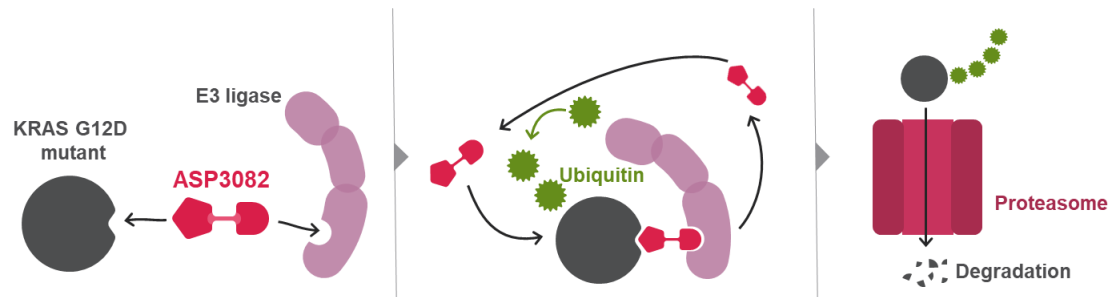
As of Feb 2025. Not exhaustively listed. VEOZAH: Approved as "VEOZA" in ex-US. MIBC: Muscle-invasive bladder cancer, TLR: Topline results, NMIBC: Non-muscle-invasive bladder cancer, GA: Geographic atrophy, AMD: Age-related macular degeneration, NDA: New Drug Application, LCM: Lifecycle management, VMS: Vasomotor symptoms, GEJ: Gastroesophageal junction, CPI: Checkpoint inhibitor, AML: Acute myeloid leukemia, HIC: High-intensity chemotherapy

Overview of Primary Focus Flagship Programs (1/2)

ASP3082 (Targeted Protein Degradation)

Protein degrader targeting KRAS G12D mutant

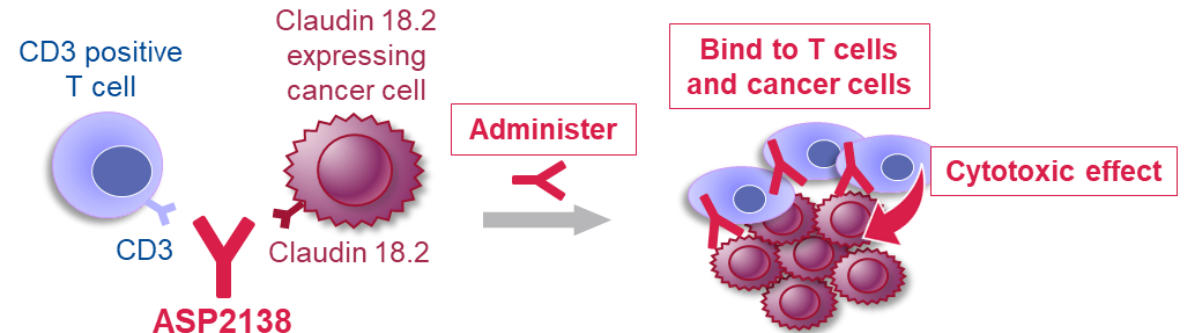
- Target disease: Cancers harboring KRAS G12D mutation
 - ✓ Rate of patients with KRAS G12D mutation: ~40% in PDAC, ~15% in CRC, ~5% in non-squamous NSCLC¹
- Standard of care (metastatic PDAC): Chemotherapy
- Status: Phase 1 study ongoing ([NCT05382559](#))
 - ✓ PDAC: 2L+ (monotherapy), 1L (combo with chemotherapy)
 - ✓ CRC: 2L+ (monotherapy, combo with cetuximab)
 - ✓ NSCLC: 2L+ (monotherapy)
- Anticipated PoC judgment timing: 1H/CY2025



ASP2138 (Immuno-Oncology)

Bispecific antibody targeting Claudin 18.2 and CD3

- Target disease: Gastric and GEJ (G/GEJ) adenocarcinoma, pancreatic adenocarcinoma
 - ✓ Rate of Claudin 18.2-positive patients*: ~70% in G/GEJ adenocarcinoma² and ~60% in pancreatic adenocarcinoma³
- Standard of care (HER2-, advanced G/GEJ adenocarcinoma)
 - ✓ 1L: chemotherapy +/- checkpoint inhibitor
 - ✓ 2L+: paclitaxel + ramucirumab
- Status: Phase 1 study ongoing ([NCT05365581](#))
 - ✓ G/GEJ adenocarcinoma, 1L & 2L, monotherapy & combo
- Anticipated PoC judgment timing: 1H/FY2025



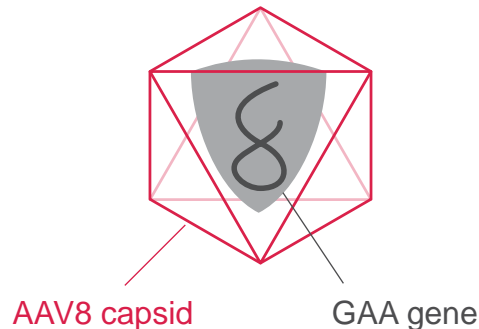
*Represents % of patients with any level of Claudin 18.2+ staining ($\geq 1\%$). 1. npj Precis Oncol. 2022;6:91, 2. Gastric Cancer. 2024;27:1058, 3. Int J Cancer. 2013;134:731
KRAS: Kirsten rat sarcoma viral oncogene homologue, PDAC: Pancreatic ductal adenocarcinoma, CRC: Colorectal cancer, NSCLC: Non-small cell lung cancer, 2L+: Second or later line, 1L: First line, PoC: Proof of concept, GEJ: Gastroesophageal junction, HER2-: HER2 negative

Overview of Primary Focus Flagship Programs (2/2)

AT845 (Genetic Regulation)

Recombinant AAV8 expressing hGAA gene specially in muscle

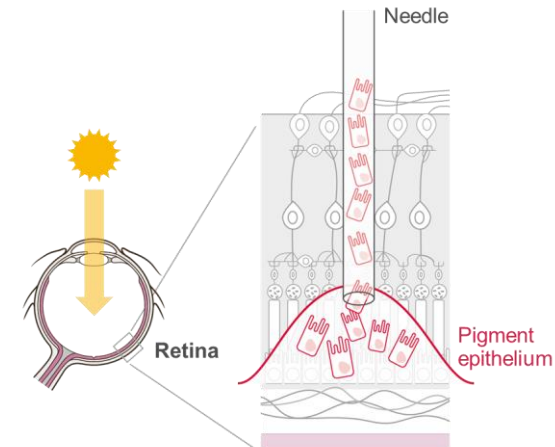
- Target disease: Pompe disease
 - ✓ Estimated incidence: 1 in ~40,000¹
- Standard of care: Enzyme replacement therapy (ERT)
 - ✓ Chronic, repeated infusions every 2 weeks
 - ✓ Secondary disease progression after 2-3 years on ERT^{2,3,4}
- Status: Phase 1/2 FORTIS study ongoing ([NCT04174105](https://clinicaltrials.gov/ct2/show/study/NCT04174105))
 - ✓ Disease stability observed for up to 2 years while off ERT⁵
- Anticipated PoC judgment timing: 2H/FY2025



ASP7317 (Blindness & Regeneration)

Replacement therapy with retinal pigment epithelial cells aiming to maintain and restore visual functions

- Target disease: Geographic atrophy secondary to AMD
 - ✓ Estimated Number of patients: ~5 million worldwide⁶
- Standard of care: Complement inhibitors
 - ✓ Slows progression, does not improve vision
- Status: Phase 1b study ongoing ([NCT03178149](https://clinicaltrials.gov/ct2/show/study/NCT03178149))
- Anticipated PoC judgment timing: 2H/FY2025



1. NORD (National Organization for Rare Disorders) at <https://rarediseases.org/rare-diseases/pompe-disease/>, 2. Neuromuscul Disord. 2021;31:91-100, 3. J Neurol. 2021;268:2482-2492, 4. Mol Genet Metab. 2012;106:301-309, 5. MDA Clinical & Scientific Conference 2024, 6. Retina. 2017;37:819-835
AAV: Adeno-associated virus, hGAA: Human acid alpha-glucosidase, PoC: Proof of concept, AMD: Age-related macular degeneration

Robust Pipeline of Astellas

Phase 1

enfortumab vedotin (NMIBC)
ASP1570
ASP2138
ASP1002
ASP1012
ASP3082
ASP4396
zocaglusagene nuzaparvovec/ AT845
ASP7317
abiraterone decanoate/ ASP5541 (PRL-02)
ASP5502

Phase 2

enfortumab vedotin (Other solid tumors)
gilteritinib (Newly diagnosed AML, HIC-ineligible)
zolbetuximab (Pancreatic adenocarcinoma)
avacincaptad pegol (Stargardt disease)
resamirigene bilparvovec/ AT132 (XLMTM)

Phase 3

enfortumab vedotin (MIBC)
gilteritinib (Earlier-stage AML, pediatric use)
fezolinetant (VMS due to menopause: China, Japan; VMS in breast cancer patients on adjuvant endocrine therapy)
zolbetuximab (Gastric and GEJ adenocarcinoma, combo with CPI and chemotherapy)
mirabegron (NDO, pediatric use (aged 6 months to less than 3 years): Europe)
roxadustat (Anemia associated with CKD, pediatric use: Europe)

- Strategic Brands
- Programs with Focus Area approach
- Others

Please refer to R&D pipeline list for details including target disease.

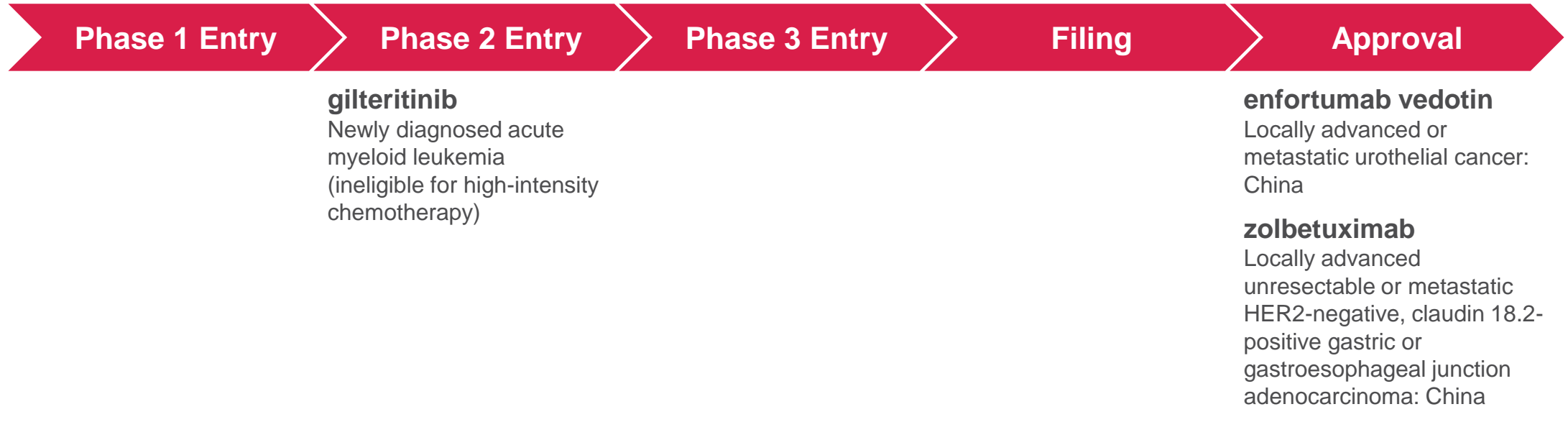
NMIBC: Non-muscle-invasive bladder cancer, AML: Acute myeloid leukemia, HIC: High-intensity chemotherapy, XLMTM: X-linked myotubular myopathy, MIBC: Muscle-invasive bladder cancer, VMS: Vasomotor symptoms, GEJ: Gastroesophageal junction, CPI: Checkpoint inhibitor, NDO: Neurogenic detrusor overactivity, CKD: Chronic kidney disease



Progress in Overall Pipeline

Phase 1 Entry to Approval Since the Last Financial Results Announcement

33



Discontinuation

ASP2802: B-cell lymphoma (Phase 1)

ASP2016: Cardiomyopathy associated with Friedreich ataxia (Phase 1)

Note: Phase 1 entry and Phase transition are defined by first subject first treatment.
Filing is defined as submission of application to health authorities.
Discontinuation is defined by the decision of company decision body.

Strategic Brands: Status Update

(Blue: Updates since the last financial results announcement)

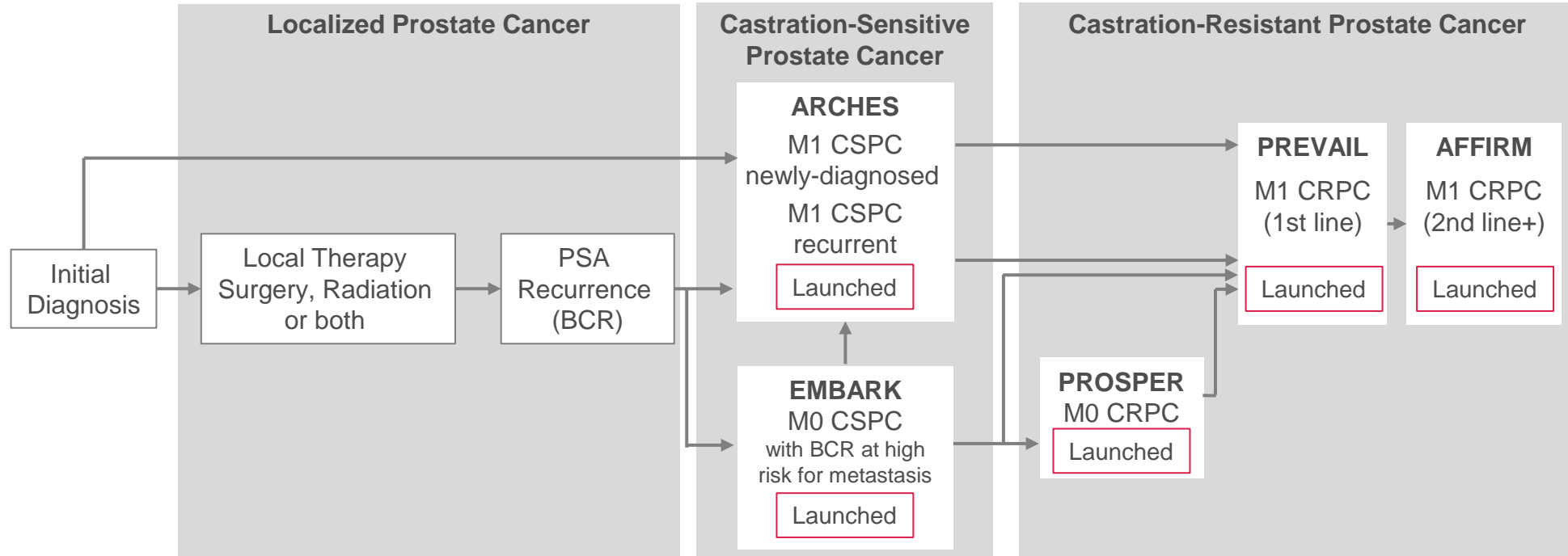
Generic / Brand name	Indication	Current status
enfortumab vedotin / PADCEV	Metastatic urothelial cancer	• Previously untreated (first line): Approved in China in Jan 2025
	Muscle-invasive bladder cancer	• Phase 3 studies ongoing (enrollment completed)
	Non-muscle-invasive bladder cancer	• Phase 1 study ongoing (enrollment completed)
	Other solid tumors	• Phase 2 study ongoing (enrollment completed)
gilteritinib/ XOSPATA	Relapsed and refractory AML	• China: Phase 3 study stopped due to efficacy
	AML, post-HSCT maintenance	• Development based on Phase 3 MORPHO study discontinued
	AML, newly diagnosed (HIC-eligible)	• Phase 3 study ongoing (enrollment completed)
	AML, newly diagnosed (HIC-ineligible)	• Progressed to Phase 2
zolbetuximab/ VYLOY	AML, post-chemotherapy	• Obtained topline results from Phase 2 GOSSAMER study
	Gastric and GEJ adenocarcinoma	• Approved in China in Dec 2024 • Phase 3 study in combo with CPI and chemotherapy under preparation to start in Q1/FY2025
fezolinetant/ VEOZAH	Pancreatic adenocarcinoma	• Phase 2 study ongoing (enrollment completed)
	VMS due to menopause	• China: Obtained topline results from Phase 3 MOONLIGHT 1 and MOONLIGHT 3 studies • Japan: Phase 3 studies ongoing
avacincaptad pegol/ IZERVAY	VMS in breast cancer patients on adjuvant endocrine therapy	• Phase 3 study ongoing
	GA secondary to AMD	• Revised sNDA for label update accepted in US in Jan 2025
	Stargardt disease	• Phase 2b study ongoing

VEOZAH: Approved as "VEOZA" in ex-US.

AML: Acute myeloid leukemia, HSCT: Hematopoietic stem cell transplant, HIC: High-intensity chemotherapy, GEJ: Gastroesophageal junction, CPI: Checkpoint inhibitor,

VMS: Vasomotor symptoms, GA: Geographic atrophy, AMD: Age-related macular degeneration, sNDA: Supplemental New Drug Application

enzalutamide (1/2): Androgen Receptor Inhibitor



enzalutamide (2/2): Phase 3 Study Data by Disease Stage

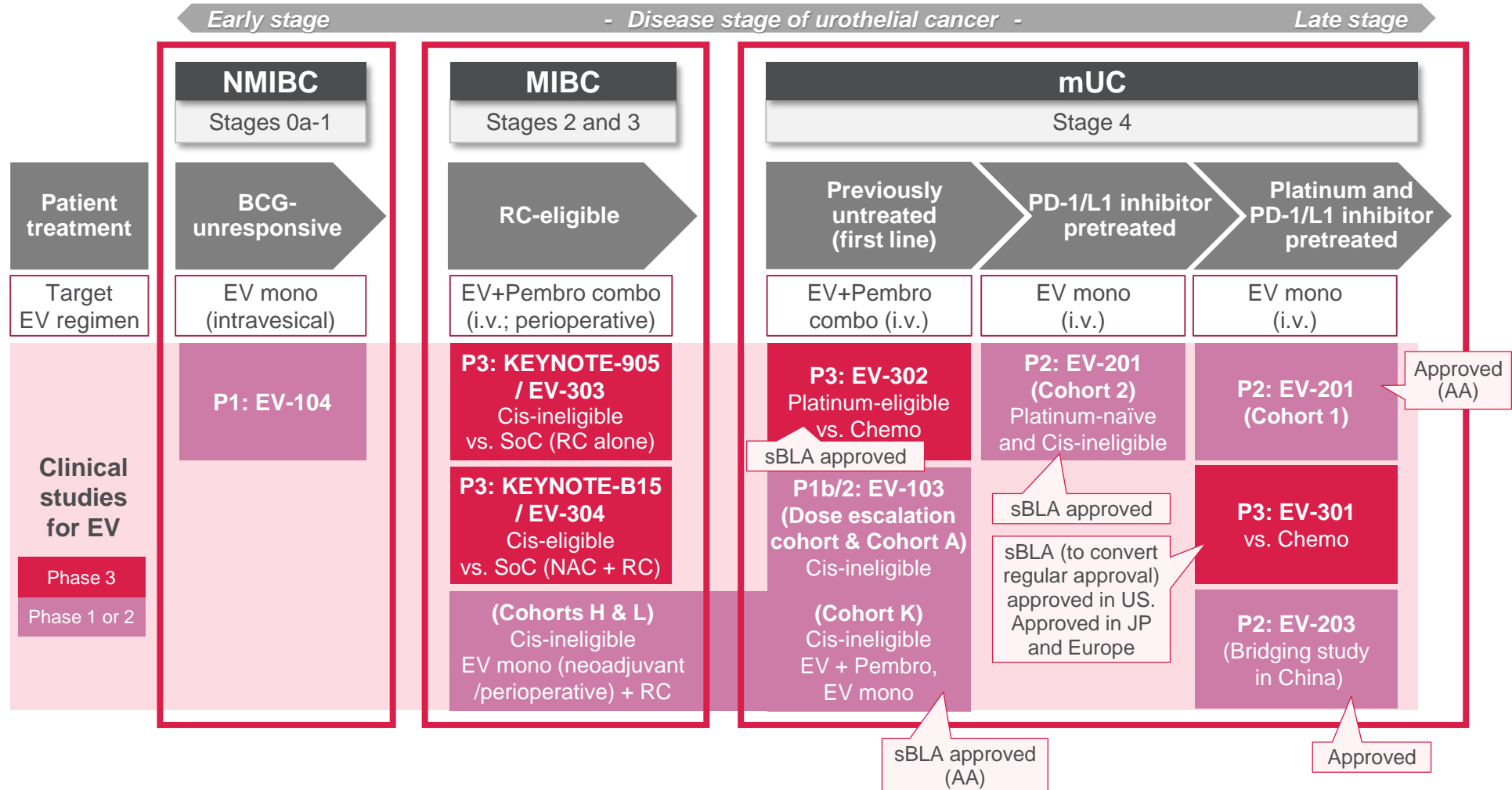
Continued potential in earlier lines with consistent survival benefit and longer duration of treatment

Disease stage	Early stage			Late stage		
	Castration-sensitive (CSPC)			Castration-resistant (CRPC)		
	M0	M1		M0	M1 (pre-chemo)	M1 (post-chemo)
Phase 3 study	EMBARC	ARCHES	ENZAMET	PROSPER	PREVAIL	AFFIRM
Control	Placebo	Placebo	Conventional NSAA	Placebo	Placebo	Placebo
Primary endpoint	✓ MFS HR 0.42	✓ rPFS HR 0.39	✓ OS HR 0.67	✓ MFS HR 0.29	✓ rPFS HR 0.17 ✓ OS HR 0.71*	✓ OS HR 0.63
OS	(Ongoing)	✓ HR 0.66	✓ HR 0.67	✓ HR 0.73	✓ HR 0.77	✓ HR 0.63
DoT	✓ 32.4 months**	✓ 40.2 months	✓ 29.5 months	✓ 33.9 months	✓ 17.5 months	✓ 8.3 months

✓: Data obtained, *: Prespecified interim analysis, **: excluding treatment suspension period

enfortumab vedotin (EV) (1/6): Nectin-4 Targeted ADC

Overall UC Program



ADC: Antibody-drug conjugate, mUC: Metastatic urothelial cancer, NMIBC: Non-muscle-invasive bladder cancer, MIBC: Muscle-invasive bladder cancer, BCG: Bacillus Calmette-Guerin, RC: Radical cystectomy, mono: Monotherapy, Pembro: Pembrolizumab, i.v.: Intravenous, Cis: Cisplatin, SoC; Standard of care, NAC: Neoadjuvant chemotherapy, Chemo: Chemotherapy, sBLA: Supplemental Biologics License Application, AA: Accelerated Approval



enfortumab vedotin (EV) (2/6): Clinical Studies

(Blue: Updates since the last financial results announcement)

For urothelial cancer

P3: EV-302	NCT04223856	mUC, Previously untreated, Platinum-eligible; EV + Pembro vs. Chemo	n=886	Approved in US in Dec 2023, in Europe in Aug 2024, in Japan in Sep 2024, in China in Jan 2025
P3: EV-303 /KEYNOTE-905	NCT03924895	MIBC, Cis-ineligible; Pembro +/- EV (perioperative) + RC vs. RC alone	n=595	Enrollment completed
P3: EV-304 /KEYNOTE-B15	NCT04700124	MIBC, Cis-eligible; EV + Pembro (perioperative) + RC vs. Chemo (neoadjuvant) + RC	n=784	Enrollment completed
P1b/2: EV-103	NCT03288545	Cohorts A - G and K (mUC): A-G: Combo with Pembro and other chemo K: EV mono, EV + Pembro Cohorts H, J and L (MIBC, Cis-ineligible, + RC): H: EV mono (neoadjuvant) J (optional): EV + Pembro (neoadjuvant) L: EV mono (perioperative)	n=348	Dose Escalation/Cohort A and Cohort K: sBLA approved (under the Accelerated Approval program) in US in Apr 2023. Enrollment completed
P1: EV-104	NCT05014139	NMIBC, High-risk BCG-unresponsive; Intravesical EV mono	n=58	Enrollment completed

For other solid tumors

P2: EV-202	NCT04225117	HR+/HER2- breast cancer, Triple-negative breast cancer, Squamous NSCLC, Non-squamous NSCLC, Head and neck cancer, Gastric and esophageal adenocarcinoma including GEJ adenocarcinoma, Esophageal squamous cell carcinoma; EV mono Head and neck squamous cell carcinoma; EV + Pembro	n=329	Enrollment completed
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enfortumab vedotin (EV) (3/6): Study Data by Disease Stage of UC

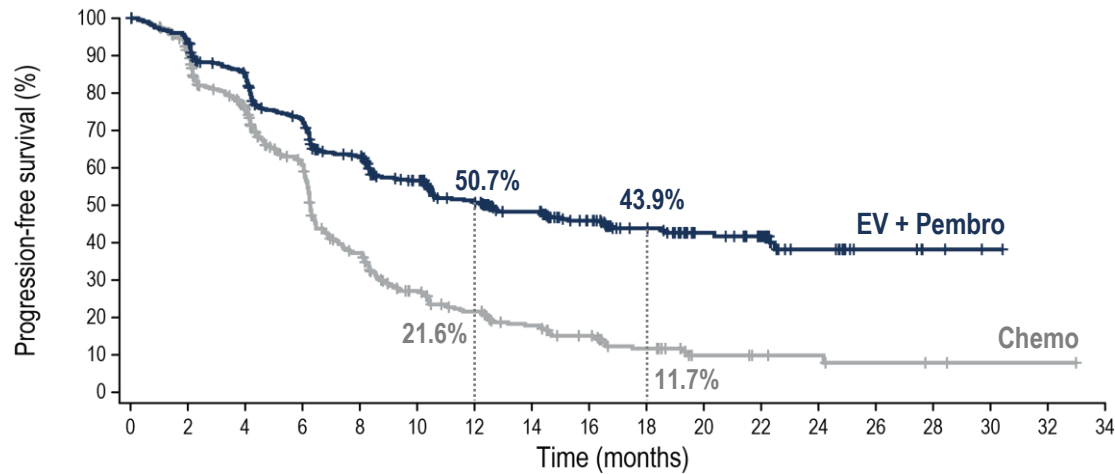
Disease stage	Early stage					Late stage			
	MIBC		mUC						
	Surgery eligible		Previously untreated (first line)			PD-1/L1 inhibitor pretreated			
	Cis-eligible	Cis-ineligible	Platinum eligible	Cis-ineligible		Platinum naïve & Cis-ineligible	Platinum pretreated		
Study phase	Phase 3	Phase 3	Phase 3	Phase 1b/2		Phase 1b/2	Phase 2	Phase 2	Phase 3
Study No.	KN-B15 / EV-304	KN-905 / EV-303	EV-302	EV-103 Cohort K		EV-103 Cohort A & Others	EV-201 Cohort 2	EV-201 Cohort 1	EV-301
No. of subjects	784 (2 arms)	595 (3 arms)	886	76	73	45	89	125	608 (2 arms)
EV regimen	Combo w/ Pembro (perioperative)	Combo w/ Pembro (perioperative)	Combo w/ Pembro	Combo w/ Pembro	Mono	Combo w/ Pembro	Mono	Mono	Mono
Control	Chemo (neoadjuvant)	SoC	Chemo	n/a	n/a	n/a	n/a	n/a	Chemo
Primary endpoint	EFS	EFS	✓ PFS: HR 0.45 ✓ OS: HR 0.47	✓ ORR 64% (CR 11%)	✓ ORR 45% (CR 4%)	✓ ORR 73% ** (CR 16% **)	✓ ORR 51% ** (CR 22% **)	✓ ORR 44% (CR 12%)	✓ OS HR 0.70 *
OS	(Ongoing)	(Ongoing)	✓ HR 0.47 (31.5 mos vs.16.1 mos)	(Ongoing)	✓ (21.7 mos)	✓ (26.1 mos **)	✓ (14.7 mos)	✓ (12.4 mos **)	✓ HR 0.70 * (12.9 mos vs.9.0 mos)
PFS	(Ongoing)	(Ongoing)	✓ HR 0.45 (12.5 mos vs.6.3 mos)	(Ongoing)	✓ (8.2 mos)	✓ (12.7 mos **)	✓ (5.8 mos)	✓ (5.8 mos)	✓ HR 0.62 * (5.6 mos vs.3.7 mos)
ORR	(Ongoing)	(Ongoing)	✓ 67.7% vs. 44.4% (CR 29.1% vs. 12.5%)	✓ 64% (CR 11%)	✓ 45% (CR 4%)	✓ 73% ** (CR 16% **)	✓ 52% (CR 20%)	✓ 44% (CR 12%)	✓ 41% vs.18% * (CR 4.9% vs.2.7%)
DoR	(Ongoing)	(Ongoing)	(Ongoing)	(Ongoing)	✓ 13.2 mos	✓ 22.1 mos **	✓ 13.8 mos **	✓ 7.6 mos	✓ 7.4 mos vs. 8.1 mos *

✓: Data obtained, *: Prespecified interim analysis, **: Updated data

enfortumab vedotin (EV) (4/6): Study Data in 1L mUC (EV-302)

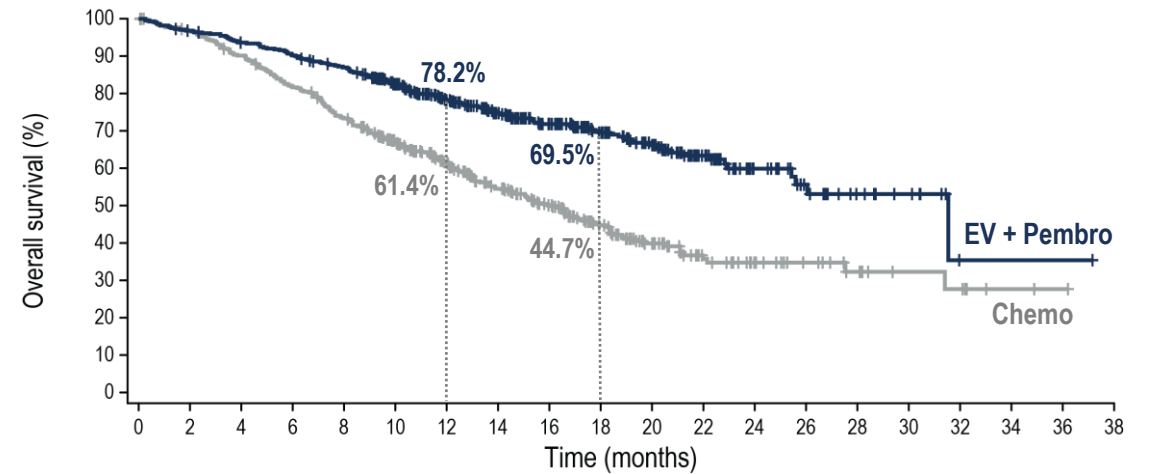
Statistically significant and clinically meaningful improvement over chemotherapy with nearly doubled mOS and mPFS

<Progression-free survival>



	N	Events (%)	HR (95% CI)	2-sided P value	mPFS (95% CI), months
EV + Pembro	442	223 (50.5)	0.45	<0.00001	12.5 (10.4-16.6)
Chemo	444	307 (69.1)	(0.38-0.54)		6.3 (6.2-6.5)

<Overall survival>



	N	Events (%)	HR (95% CI)	2-sided P value	mOS (95% CI), months
EV + Pembro	442	133 (30.1)	0.47	<0.00001	31.5 (25.4-NR)
Chemo	444	226 (50.9)	(0.38-0.58)		16.1 (13.9-18.3)

- Chemo: cisplatin or carboplatin + gemcitabine
- 30.4% of patients in Chemo arm received subsequent avelumab maintenance therapy

enfortumab vedotin (EV) (5/6): Study Data in Solid Tumors Other than UC (EV-202)

Cohort	Cancer type	n	ORR	
			Target*	Result
1	HR+/HER2- breast cancer	45	30%	15.6%
2	Triple-negative breast cancer	42	25%	19.0%
3	Squamous non-small cell lung cancer	23	17.5%	4.3%
4	Non-squamous non-small cell lung cancer	43	25%	16.3%
5	Head and neck cancer	46	17.5%	23.9%
7	Gastric and esophageal adenocarcinoma incl. GEJ adenocarcinoma	42	17.5%	9.5%
8	Esophageal squamous cell carcinoma	44	17.5%	18.2%
9	1L head and neck squamous cell carcinoma	Ongoing		

Cohorts 1-8: Second or later line, monotherapy

Cohort 9: First line, combo with pembrolizumab

*Minimum responders needed to declare promising antitumor activity

enfortumab vedotin (EV) (6/6): Future Outlook

- The most significant growth driver is 1L mUC indication, which is expected to account for more than half of total sales in the future
- Success in NMIBC and other solid tumors will provide further growth potential

<Already approved / pivotal phase> (Included in potential peak sales)

Patient segment		Pivotal study (EV regimen)	Target filing timing	Number of eligible patients*
MIBC	Cis-ineligible	EV-303 (combo w/ Pembro)	FY2025 or later	19,000**
	Cis-eligible	EV-304 (combo w/ Pembro)	FY2025 or later	64,000**
1L mUC		EV-302 EV-103 Cohorts [Phase 1b/2 for AA in US] (combo w/ Pembro)	Approved Approved [AA in US]	87,000
2L+ mUC	PD-1/L1 inhibitor pretreated & Cis-ineligible	EV-201 Cohort 2 (monotherapy)	Approved	1,500 (US, Cis-ineligible)
	Platinum & PD-1/L1 inhibitor pretreated	EV-301 EV-201 Cohort 1 [Phase 2 for AA in US] (monotherapy)	Approved	46,000

<Early clinical phase> (Not included in potential peak sales)

Patient segment	Study (EV regimen)
NMIBC High-risk BCG-unresponsive	EV-104 [Phase 1] (monotherapy, intravesical)
Other solid tumors	EV-202 [Phase 2] (monotherapy* / combo w/ Pembro**)

*Monotherapy:

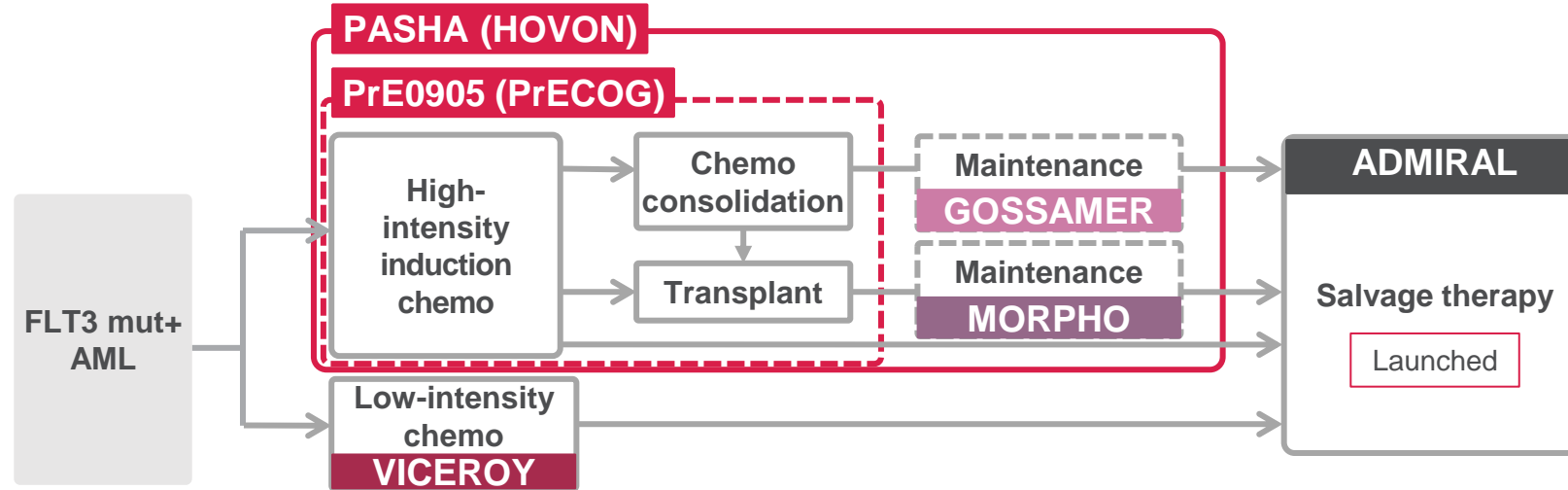
- HR+/HER2- breast cancer
- Triple-negative breast cancer
- Squamous non-small cell lung cancer
- Non-squamous non-small cell lung cancer
- Head and neck cancer
- Gastric and esophageal adenocarcinoma including GEJ adenocarcinoma
- Esophageal squamous cell carcinoma

**Combo w/ Pembro:

- Head and neck squamous cell carcinoma

gilteritinib: FLT3 Inhibitor

(Blue: Updates since the last financial results announcement)



Relapsed or refractory	P3: ADMIRAL	NCT02421939	Monotherapy vs. salvage chemo (2:1)	n=371	Launched in US, JP, and Europe
Newly diagnosed (HIC-eligible)	P3: PASHA (HOVON)	NCT04027309	Combo with high intensity chemo gilteritinib vs. midostaurin (1:1)	n=766	Enrollment completed (Sponsor: HOVON)
	P2: PrE0905 (PrECOG)	NCT03836209		n=181	Topline results presented at ASH 2024 (Sponsor: PrECOG, LLC.)
Post-HSCT maintenance	P3: MORPHO	NCT02997202	Monotherapy vs. placebo (1:1)	n=356	Development based on MORPHO study discontinued
Post-chemo maintenance	P2: GOSSAMER	NCT02927262	Monotherapy vs. placebo (2:1)	n=98	Topline results obtained in Aug 2021
Newly diagnosed (HIC-ineligible)	P1/2: VICEROY	NCT05520567	Combo with venetoclax and azacitidine	n=70	FSFT: Jan 2023 Progressed to Phase 2

- China**
- **R/R AML:** Conditional approval obtained in Jan 2021, based on ADMIRAL study data (full approval contingent on COMMODORE study data) and launched in Apr 2021. Phase 3 COMMODORE study (including China and other countries) stopped due to efficacy based on the planned interim analysis

zolbetuximab: Anti-Claudin 18.2 Monoclonal Antibody

(Blue: Updates since the last financial results announcement)

Target: Claudin 18.2 (CLDN18.2)

- Claudin is a major structural component of tight junctions and seals intercellular space in epithelial sheets
- 38% of patients had CLDN18.2-positive tumors* in SPOTLIGHT and GLOW studies for gastric and GEJ adenocarcinoma
- 27.7% of patients had CLDN18.2-positive tumors* in GLEAM study for pancreatic adenocarcinoma

Gastric and GEJ adenocarcinoma

- Five-year survival rate is ~6% for metastatic gastric cancer patients at Stage IV

Pancreatic adenocarcinoma

- Five-year survival rate is <5% for patients at the metastatic stage

Gastric and GEJ adenocarcinoma	P3: SPOTLIGHT	NCT03504397	First line, Combo with mFOLFOX6, DB, vs. placebo	n=566	Approved in Japan in Mar 2024, in Europe in Sep 2024, in US in Oct 2024, in China in Dec 2024
	P3: GLOW	NCT03653507	First line, Combo with CAPOX, DB, vs. placebo	n=507	
	P2: ILUSTRO	NCT03505320	Cohort 1: Third or later line, zolbetuximab monotherapy Cohort 2: First line, Combo with mFOLFOX6 Cohort 3: Third or later line, Combo with pembrolizumab Cohort 4: First line, Combo with mFOLFOX6 and nivolumab Cohort 5: Perioperative, Combo with FLOT	n=143	Enrollment completed
Pancreatic adenocarcinoma	P2: GLEAM	NCT03816163	First line, Combo with nab-paclitaxel and gemcitabine, open	n=393	Enrollment completed

*CLDN18.2 positivity is defined as ≥75% of tumor cells demonstrating moderate to strong membranous CLDN18 immunohistochemical staining

GEJ: Gastroesophageal junction, mFOLFOX6: 5-FU, leucovorin and oxaliplatin, DB: Double-blind, CAPOX: Capecitabine and oxaliplatin,

FLOT: Fluorouracil, leucovorin, oxaliplatin and docetaxel

fezolinetant: NK3 receptor antagonist

VMS has a significant negative impact on QoL

- Physical symptoms include hot flashes and night sweats, which can impact sleep
- Physical symptoms may lead to emotional impact including embarrassment, irritability, anxiety, and sadness
- Symptoms have a negative impact on multiple aspects of everyday life¹

Women’s Health Initiative (WHI) Study²

- Initial data analyses showed an association between chronic HRT use and increased risk of cardiovascular disease and breast cancer
- Since WHI’s findings, use of HRT has dropped
- Although subsequent analysis of the WHI data have demonstrated that HRT is safe and effective when initiated in the appropriate patient in the appropriate manner (i.e. right time, formulation, dose and duration), prescriptions have not rebounded, leaving some women with minimal options to satisfactorily manage their VMS

VMS associated with menopause

Japan	P3: STARLIGHT 2	NCT06206408	Mild to severe VMS associated with menopause; 12 weeks: DB, 2 doses vs. placebo (1:1:1)	n=390	FSFT: Mar 2024
	P3: STARLIGHT 3	NCT06206421	VMS associated with menopause; 52 weeks: DB, vs. placebo (1:1)	n=260	FSFT: Feb 2024
China	P3: MOONLIGHT 1	NCT04234204	Moderate to severe VMS associated with menopause; The first 12 weeks: DB, 30 mg vs. placebo (1:1) The last 12 weeks: Active extension treatment period, 30 mg	n=302	Primary endpoints not met (12w DB period topline results)
	P3: MOONLIGHT 3	NCT04451226	VMS associated with menopause; open label, 30 mg for 52 weeks	n=150	Topline results obtained in Sep 2022

VMS in breast cancer women receiving adjuvant endocrine therapy

P3: HIGHLIGHT 1	NCT06440967	Moderate to severe VMS associated with adjuvant endocrine therapy for breast cancer; 52 weeks (efficacy endpoints at 4 and 12 weeks): DB, vs. placebo (1:1)	n=540	FSFT: Aug 2024
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1: DelveInsight, Epidemiology Forecast, Jun 2018. 2: Data Source - IMS NPA (2000-2016), IMS NSP (2000-2016). (3 HTs and SSRI) NAMS 2015 Position Statement
VMS: Vasomotor symptoms, QoL: Quality of life, HRT: Hormone replacement therapy, DB: Double-blind, FSFT: First subject first treatment



avacincaptad pegol (ACP): Complement C5 Inhibitor / Pegylated RNA Aptamer

(Blue: Updates since the last financial results announcement)

Geographic atrophy (GA)

- Advanced form of dry age-related macular degeneration (AMD)
- Globally, approximately 5 million people are estimated to have GA at least in one eye¹
- Approximately 75% of people living with GA in the US are believed to be undiagnosed²
- Without timely treatment, an estimated 66% of people with GA may become blind or severely visually impaired³

Characteristics of ACP

- Pegylated RNA aptamer (Chemically synthesized)
- ACP inhibits complement C5, and slows inflammation and cell death associated with development and progression of GA

GA secondary to AMD	P2/3: GATHER1	NCT02686658	Part 1: 1 mg, 2 mg vs. Sham (n=77) Part 2: 2 mg, 4 mg vs. Sham (n=209)	n=286	Revised sNDA for label update accepted in US in Jan 2025
	P3: GATHER2	NCT04435366	2 mg vs. Sham	n=448	
Stargardt disease	P2b	NCT03364153	vs. Sham	n=121	FSFT: Jan 2018

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