# Q1/FY2024 FINANCIAL RESULTS
ENDED JUNE 30, 2024

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**August 1, 2024**

# 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.

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# AGENDA



I Q1/FY2024 Consolidated Financial Results

II Initiatives for Sustainable Growth

# Q1/FY2024 FINANCIAL RESULTS: OVERVIEW



***Solid start* toward achieving the FY2024 initial forecast**

*Revenue*

- **Increased YoY (+26%)**
- XTANDI: Contributed to overall growth, driven especially by the US
- Strategic Brands: Expanded to **75.0 bil. yen** in total
  Robust growth of approx. **+50.0 bil. yen** YoY (**3 times** increase)

*Cost items*

- SG&A and R&D expenses: Invested as planned for future growth
- Timely cost management with a focus on ROI

*Core Operating profit*

- **Increased YoY**, with significant contributions from the expansion of XTANDI and Strategic Brands

Strategic Brands: PADCEV, IZERVAY, VEOZAH, VYLOY, XOSPATA
ROI: Return On Investment

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# Q1/FY2024 FINANCIAL RESULTS



| (billion yen) | Q1/FY23 | Q1/FY24 | Change | Change (%) | FY24 FCST* | FX impact (YoY) |
|---|---|---|---|---|---|---|
| **Revenue** | **375.0** | **473.1** | **+98.1** | **+26.2%** | **1,650,0** | +45.4 |
| Cost of sales | 68.9 | 91.1 | +22.2 | +32.2% | 326.0 | +6.1 |
| SG&A expenses | 168.2 | 206.9 | +38.7 | +23.0% | 757.0 | +20.8 |
| US XTANDI co-pro fee | 44.6 | 61.6 | +17.0 | +38.2% | 189.0 | +7.3 |
| SG&A excl. the above | 123.6 | 145.3 | +21.7 | +17.5% | 568.0 | +13.5 |
| R&D expenses | 64.6 | 86.8 | +22.2 | +34.4% | 317.0 | +6.9 |
| **Core operating profit**** | **73.3** | **88.3** | **+15.0** | **+20.5%** | **250.0** | +11.6 |
| **＜Full basis＞** | | | | | | |
| Amortisation of intangible assets | 9.1 | 35.0 | +25.9 | +285.9% | | |
| Other income | 3.9 | 4.9 | +1.0 | +25.2% | | |
| Other expenses | 23.1 | 10.4 | -12.7 | -55.0% | | |
| **Operating profit** | **45.8** | **50.7** | **+4.9** | **+10.6%** | **48.0** | |
| Profit before tax | 46.8 | 50.5 | +3.6 | +7.8% | 43.0 | |
| **Profit** | **33.1** | **37.6** | **+4.5** | **+13.5%** | **30.0** | |

Note) Amortisation of IZERVAY's intangible assets started from Q2/FY2023

Other expenses

- Fair value increase of contingent consideration (zolbetuximab) due to FX impact: 5.5

* Announced in Apr 2024, exchange rates of initial FY2024 FCST: 145 yen/USD, 155 yen/EUR. Actual exchange rates of Q1/FY2024: 156 yen/USD, 168 yen/EUR

** The definition of core-basis is 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' are newly excluded as new adjustment items. All figures above reflect this change.

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# Q1/FY2024 FINANCIAL RESULTS: XTANDI AND STRATEGIC BRANDS



*XTANDI contributed to overall growth, driven especially by the US*

| (billion yen) | Q1/FY2024 Act | YoY | FY2024 FCST* | |
|---|---|---|---|---|
| Xtandi® | 224.2 | +50.2 (+29%) | 757.0 | ✓ Global sales off to a strong start, driven by higher-than-expected US performance<br>✓ US: Demand exceeded expectations, driven by the impact of EMBARK (M0 CSPC) and market growth |

*Strategic Brands expanded to **75.0 bil. yen** in total. Robust growth of ~ **+50.0 bil. yen** YoY (**3 times** increase)*

| (billion yen) | Q1/FY2024 Act | YoY | FY2024 FCST* | |
|---|---|---|---|---|
| PADCEV™ | 38.4 | +23.2 (+152%) | 151.2 | ✓ Significant growth YoY, driven by the penetration of 1L mUC in the US and strong demand growth of 2L+ mUC in EST<br>✓ Continued quarterly growth expected from Q2 onwards |
| izervay™ | 12.7 | +12.7 | 46.4 | ✓ Demand growth stronger-than-expected following the effective J-Code in April<br>Safety profile remains consistent with clinical trial results<br>✓ Q1 results exceeded expectations; raising prospects for outperforming the initial forecast |
| VEOZAH™ | 6.6 | +6.0 (+972%) | 28.3 | ✓ Steady growth in line with initial forecast<br>✓ Overall initiatives progressing as planned, such as payer coverage and DTC |
| VYLOY™ | 0.3 | +0.3 | 3.7 | ✓ Successful Japan launch (June), accessed vast majority of target physicians<br>✓ Solid progress in available accounts for VYLOY and CLDN18.2 testing penetration |
| XOSPATA® | 17.3 | +4.3 (+33%) | 60.0 | ✓ Sales expanded in all regions<br>✓ Continued steady growth expected from Q2 onwards |

*Announced in Apr 2024, exchange rates of initial FY2024 FCST: 145 yen/USD, 155 yen/EUR. Actual exchange rates of Q1/FY2024: 156 yen/USD, 168 yen/EUR
M0: Non-metastatic, CSPC: Castration-sensitive prostate cancer, 1L: First line, mUC: Metastatic urothelial cancer, 2L+: Second or later line, DTC: Direct-to-consumer, CLDN18.2: Claudin 18.2, VEOZAH: Approved as “VEOZA” in ex-US, EST (Established Markets): Europe, Canada, etc.

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# PADCEV & VEOZAH: BUSINESS UPDATE



## PADCEV

*Robust sales growth driven by the US and EST. Expect further sales contribution going forward*

| | Q1/FY2024 Act | YoY |
|---|---|---|
| Global sales | **38.4 bil. yen** | **+23.2 (+152%)** |
| US ($ basis) | $174M | +98 (+128%) |
| EST (€ basis) | €45M | +29 (+178%) |
| Japan・China・INT | 3.7 bil.yen | +1.4 (+60%) |

**QoQ (vs. Q4/FY2023): +8.6 bil. yen (+29%)**

**<US>**

- ✓ Significant contribution from 1L mUC share expansion
  New patient share of over 50%

**<ex-US>**

- ✓ Strong demand growth in 2L+ mUC, especially in EST
- ✓ Launched countries increased to 38, with reimbursement initiated in 17 countries
- ✓ Anticipate potential approval for 1L mUC sequentially from Q2 onwards, expect sales contribution after approval

## VEOZAH™

*Steady sales growth as expected. Expect continued linear growth from Q2 onwards*

| | Q1/FY2024 Act | YoY |
|---|---|---|
| Global Sales | **6.6 bil. yen** | **+6.0 (+972%)** |
| US ($ basis) | $39M | +35 (+778%) |
| EST (€ basis) | €3M | +3 |

**QoQ (vs. Q4/FY2023): +2.9 bil. yen (+77%)**

**<US>**

- ✓ Commercial lives covered (payer coverage) expanded to over 60% as planned
- ✓ Implementing DTC with a focus on ROI, steady increase in patient activation

**<ex-US>**

- ✓ Launched countries increased to 13 (+5 countries from previous quarter)

1L: First line, mUC: Metastatic urothelial cancer, 2L+: Second or later line, DTC: Direct-to-consumer, ROI: Return On Investment
VEOZAH: Approved as “VEOZA” in ex-US, EST (Established Markets): Europe, Canada, etc., China: China, Hong Kong
INT (International Markets): Latin America, Middle East, Africa, Southeast Asia, South Asia, Russia, Korea, Taiwan, Australia, Export sales, etc.

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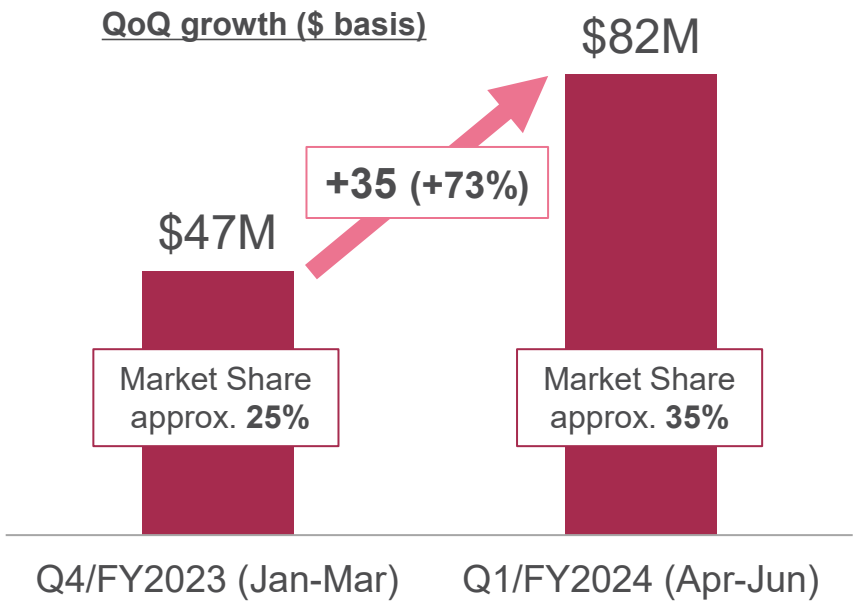
# IZERVAY: BUSINESS UPDATE (US)



*Performance exceeded expectations, particularly driven by stronger-than-expected demand following J-Code*
*Prescriber perception of safety profile is favorable*

| | Q1/FY2024 Act | YoY |
|---|---|---|
| izervay™ | **12.7** bil. yen | **+12.7** |
| US ($ basis) | $82M | +82 |




**QoQ (vs. Q4/FY2023): +5.9 bil. yen (+86%)**

**<Q1 progress>**

- ✓ Progress following effective J-Code in Apr exceeded expectations, igniting multiple new accounts
- ✓ Available in over 1,200 Retina accounts
- ✓ Market share in Q1 period (Apr-Jun) estimated at **~35%** (based on market research)
- ✓ Over 85,000 vials shipped since launch as of Q1 (excl. clinical trials)
  - **Surpassed the milestone of 100,000 vials in July**
- ✓ Post-marketing safety profile remains consistent with clinical trial results
- ✓ No new safety signals offers confidence to prescribers to select IZERVAY

**<Future expectations>**

- ✓ Q1 results exceeded expectations; raising prospects for outperforming the initial forecast
- ✓ Expect label update by Q3 based on the 2-year (GATHER 2 Year 2) clinical data, which included 24-month efficacy, safety and every other month dosing (PDUFA date: Nov 19)

PDUFA: Prescription Drug User Fee Act

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# Q1/FY2024 FINANCIAL RESULTS: COST ITEMS



- *SG&A and R&D expenses: Invested as planned for future growth*
- *Timely cost management with a focus on ROI (mainly VEOZAH)*

Core basis: YoY comparison and ratio to revenue, for major cost items

(billion yen)

| Cost Items | YoY change | Ratio to Revenue | |
|---|---|---|---|
| **Cost of sales** | +32.2% | 19.3%<br>(+0.9 ppt YoY) | YoY increase due to one-off factors including provision for US mirabegron inventory disposal and royalty payment adjustment |
| **SG&A expenses**<br>**excl. US XTANDI co-pro fee** | +17.5%<br>(+6.6%<br>excl. FX impact) | 30.7%<br>(-2.3 ppt YoY) | YoY increase excl. FX impact: approx. +8.0<br>✓ Strategic Brands-related expenses (mainly IZERVAY and VEOZAH) (approx. +12.0 YoY)<br>✓ Reduction of mature products-related expenses (approx. -4.0 YoY)<br>✓ Global organizational restructuring in FY2023 (approx. -2.0 YoY) |
| **R&D expenses** | +34.4%<br>(+23.6%<br>excl. FX impact) | 18.4%<br>(+1.1 ppt YoY) | YoY increase excl. FX impact: approx. +15.0<br>✓ Primary Focus and enhanced R&D functions (approx. +7.0 YoY)<br>✓ One-time co-development cost payments |

VEOZAH: Approved as “VEOZA” in ex-US
ROI: Return On Investment

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# AGENDA



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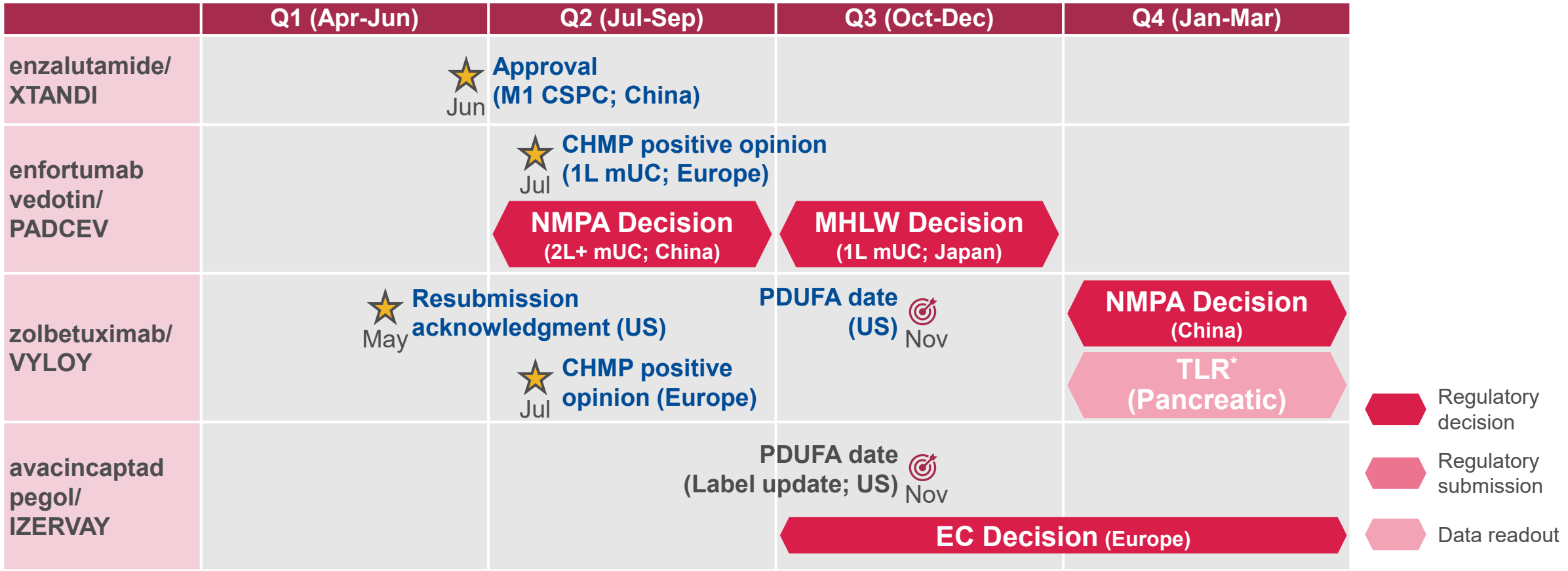
# XTANDI AND STRATEGIC BRANDS: FY2024 KEY EXPECTED EVENTS

**(Blue: Updates since the last financial results announcement)**






**<Other updates>**

- zolbetuximab / VYLOY: Phase 3 study in combination with checkpoint inhibitor and chemotherapy to start in 1H/CY2025

As of Jul 2024. *The timeline of TLR is subject to shift due to its event-driven nature. M1: Metastatic, CSPC: Castration-sensitive prostate cancer, CHMP: Committee for Medicinal Products for Human Use, 1L: First line, mUC: Metastatic urothelial cancer, NMPA: National Medical Products Administration, 2L+: Second or later line, MHLW: Ministry of Health, Labour and Welfare, PDUFA: Prescription Drug User Fee Act, TLR: Topline results, EC: European Commission

# ZOLBETUXIMAB / VYLOY: LATEST STATUS

*Pursuing product value maximization as the first-in-class anti-CLDN18.2 treatment*

## Geography

**Progressing toward global launch (G/GEJ cancer)**

- Launched on Jun 12
- Resubmission acknowledged on May 30 (PDUFA date: Nov 9)
- Positive CHMP opinion received on Jul 26
- Regulatory decision anticipated for Q4/FY2024

VYLOY™ zolbetuximab

## Pancreatic cancer

**Phase 2 registrational study ongoing**

- ✓ Enrollment completed in Mar 2024 (earlier than expected)
- ✓ TLR anticipated for Q4/FY2024*

## Combination

**New Phase 3 study in combination with CPI and chemotherapy to start in 1H/CY2025**

- 1L G/GEJ cancer
- HER2 negative
- CLDN18.2 positive
- CPS ≥1

→ **zolbetuximab + CPI + Chemo**

→ **placebo + CPI + Chemo**

Chemo: CAPOX or mFOLFOX6

*The timeline of TLR is subject to shift due to its event-driven nature. CLDN18.2: Claudin 18.2, G/GEJ: Gastric/gastroesophageal junction, PDUFA: Prescription Drug User Fee Act, CHMP: Committee for Medicinal Products for Human Use, TLR: Topline results, 1L: First line, CPS: Combined positive score, CPI: Checkpoint inhibitor, Chemo: Chemotherapy, CAPOX: Capecitabine and oxaliplatin, mFOLFOX6: 5-FU, leucovorin and oxaliplatin

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# PROGRESS IN FOCUS AREA APPROACH: CURRENT STATUS OF PROJECTS IN CLINICAL TRIAL

**(Blue: Updates since the last financial results announcement)**

| Primary Focus | Biology/Modality/Technology | Project | Mechanism of Action | Current status |
|---|---|---|---|---|
| Immuno-Oncology | Checkpoint | ASP1570 | DGKζ inhibitor | Phase 1 study ongoing. **Initial data to be presented at ESMO in Sep 2024** |
| | Bispecific immune cell engager | ASP2138 | Anti-Claudin 18.2 and anti-CD3 | Phase 1 study ongoing. **Orphan drug designation granted by FDA in Jun 2024 (pancreatic cancer)** |
| | | ASP1002 | **Anti-Claudin 4 and anti-CD137** | Phase 1 study ongoing |
| | Oncolytic virus (systemic) | ASP1012 | Leptin-IL-2 | **FSFT in Phase 1 study in May 2024** |
| | Cancer cell therapy | ASP2802 | CD20 *convertible* CAR-T (autologous) | Phase 1 study under preparation to start in Q2/FY2024 |
| Targeted Protein Degradation | Protein degradation | ASP3082 | KRAS G12D degrader | Phase 1 study ongoing, **dose expansion initiated. Initial data to be presented at ESMO in Sep 2024** |
| | | 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 | Phase 1 study under preparation to start in Q3/FY2024 |
| Blindness & Regeneration | Cell replacement | ASP7317 | RPE cells | Phase 1b study ongoing |
| Immune Homeostasis (PF Candidate) | Immune modulation | **ASP5502** | **STING inhibitor** | **Phase 1 study under preparation to start in Q2/FY2024** |
| Others (Non-PF) | Long-acting abiraterone prodrug | PRL-02 | CYP17 lyase inhibitor | Phase 1 study ongoing |

Modality
- Small molecule
- Antibody
- Gene
- Cell

DGK: Diacylglycerol kinase, ESMO: European Society for Medical Oncology, FDA: Food and Drug Administration, IL-2: Interleukin-2, FSFT: First subject first treatment, CAR: Chimeric antigen receptor, KRAS: Kirsten rat sarcoma viral oncogene homologue, AAV: Adeno-associated virus, MTM1: Myotubularin 1, GAA: Acid alpha-glucosidase, FXN: Frataxin, RPE: Retinal pigment epithelium, PF: Primary Focus, STING: Stimulator of interferon genes

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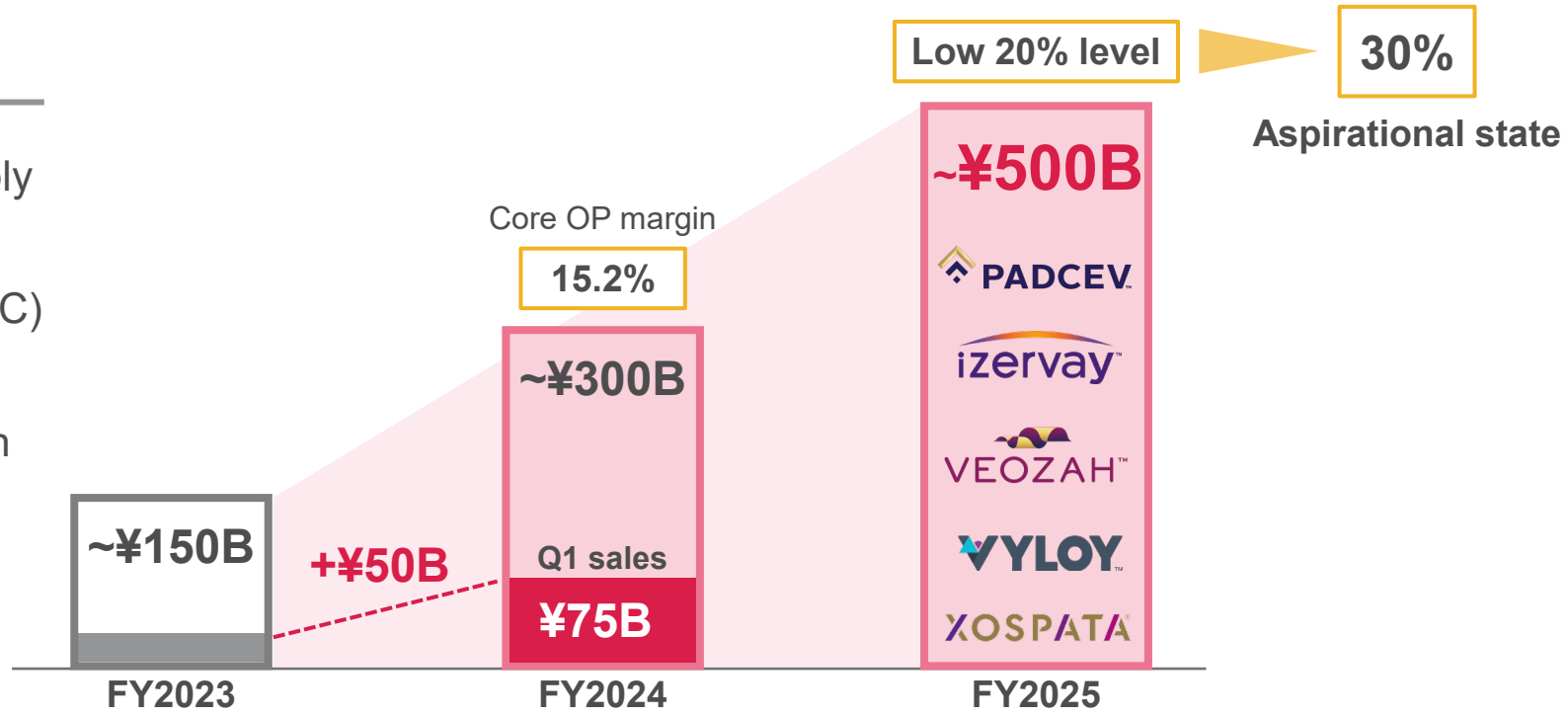
# Q1/FY2024 PROGRESS



*Solid start toward achieving the FY2024 initial forecast*

## Major Progress

- Robust growth of Strategic Brands notably driven by PADCEV and IZERVAY
- PADCEV positive CHMP opinion (1L mUC)
- VYLOY successful launch in Japan, US resubmission, positive CHMP opinion
- Acceptance of initial clinical data presentation for ASP3082 and ASP1570 at ESMO




**PoC judgment of PF lead programs**

Immuno-Oncology: ASP2138, Targeted Protein Degradation: ASP3082, Genetic Regulation: AT845, Blindness & Regeneration: ASP7317

CHMP: Committee for Medicinal Products for Human Use, 1L: First line, mUC: Metastatic urothelial cancer, ESMO: European Society for Medical Oncology, PoC: Proof of concept, PF: Primary Focus

# APPENDIX

# XTANDI AND STRATEGIC BRANDS: POTENTIAL PEAK SALES (AS OF JUL 2024)



| 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 Jul 2024), 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

# Q1/FY2024 ACTUAL: FX RATE



**Average rate for the period**

| Currency | Q1/FY2023 | Q1/FY2024 | Change |
|---|---|---|---|
| USD | 137 yen | 156 yen | +19 yen |
| EUR | 150 yen | 168 yen | +18 yen |

**<Impact of exchange rate on financial results>**

- 45.4 billion yen increase in revenue, 11.6 billion yen increase in core OP

# FY2024 FORECAST: FX RATE & FX SENSITIVITY



| Exchange rate<br>Average for the period | FY2023 | FY2024 FCST | Change |
|---|---|---|---|
| USD | 145 yen | 145 yen | - |
| EUR | 157 yen | 155 yen | -2 yen |

**Estimated FX sensitivity of FY2024 forecasts by 1 yen depreciation**

| Currency | Average rate<br>1 yen depreciation from assumption | |
|---|---|---|
| | Revenue | Core OP |
| USD | Approx. +6.1 bil. yen | Approx. +0.3 bil. yen |
| EUR | Approx. +3.0 bil. yen | Approx. +1.2 bil. yen |

# BALANCE SHEET & CASH FLOW HIGHLIGHTS



| (billion yen) | FY2023 end | Jun 30, 2024 |
|---|---|---|
| Total assets | 3,569.6 | 3,735.5 |
| Cash and cash equivalents | 335.7 | 302.9 |
| Total equity attributable to owners of the parent | 1,596.0 | 1,676.4 |
| Equity ratio (%) | 44.7% | 44.9% |

| (billion yen) | Q1/FY2023 | Q1/FY2024 |
|---|---|---|
| Cash flows from operating activities | 12.2 | 12.6 |
| Cash flows from investing activities | -12.3 | -39.3 |
| Free cash flows | -0.1 | -26.7 |
| Cash flows from financing activities | 165.0 | -11.9 |
| Increase/decrease in short-term borrowings and commercial papers | 234.0 | 71.8 |
| Redemption of bonds and repayments of long-term borrowings | - | -6.7 |
| Acquisition of treasury shares | -10.7 | -7.0 |
| Dividends paid | -53.9 | -62.8 |

# BALANCE OF BONDS AND BORROWINGS HIGHLIGHTS



| (billion yen) | FY2023 end | Jun 30, 2024 |
|---|---|---|
| Balance of bonds and borrowings | 920.0 | 992.7 |
| Non-current liabilities | 447.7 | 443.1 |
| Bonds | 250.0 | 250.0 |
| Long-term borrowings | 197.7 | 193.1 |
| Current liabilities | 472.3 | 549.6 |
| Commercial papers | 285.0 | 325.7 |
| Short-term borrowings | 135.4 | 170.4 |
| Current portion of long-term borrowings | 51.9 | 53.5 |

# MAIN INTANGIBLE ASSETS (AS OF JUN 30, 2024)



| | Bil. yen | Foreign currency* |
|---|---|---|
| AT132 | 17.5 | $109M |
| AT845 | 11.7 | $73M |
| Other gene therapy related program** | 61.1 | $380M |
| Gene therapy related technology** | 74.1 | $461M |
| VEOZAH | 97.9 | €545M |
| EVRENZO | 4.1 | - |
| VYLOY | 63.6 | €489M |
| IZERVAY（US） | 752.0 | $4,676M |
| IZERVAY (Ex-US） | 176.9 | $1,100M |

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)

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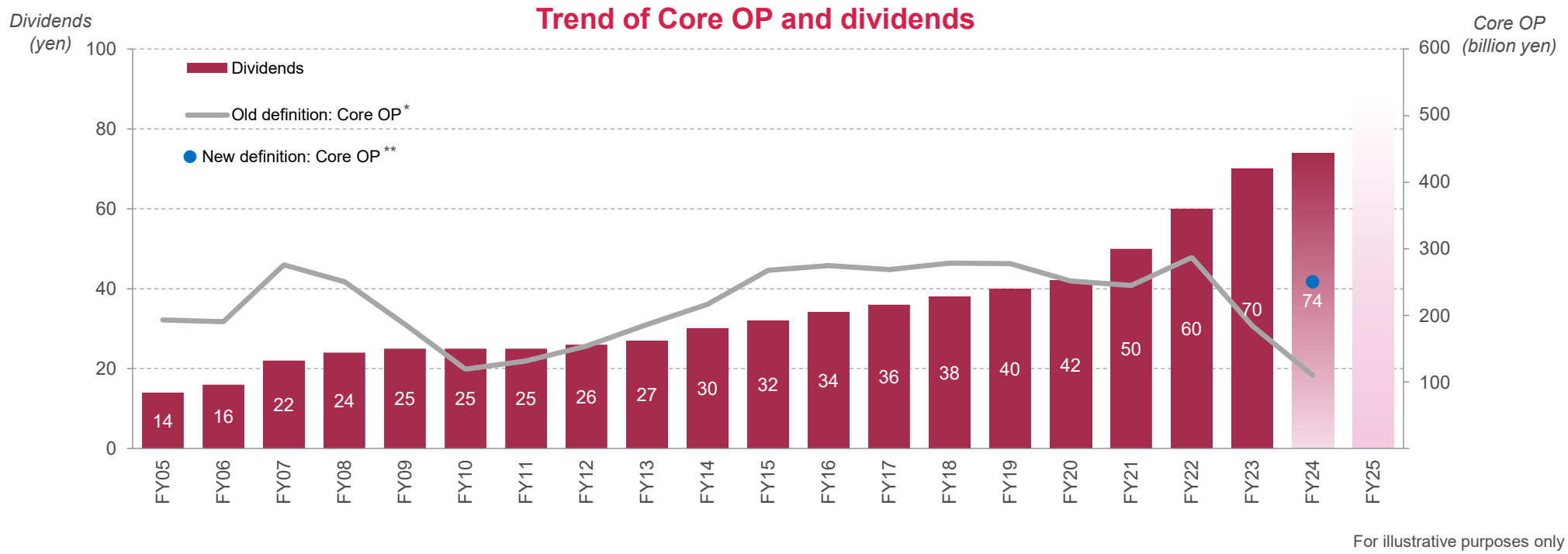
# CAPITAL ALLOCATION



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



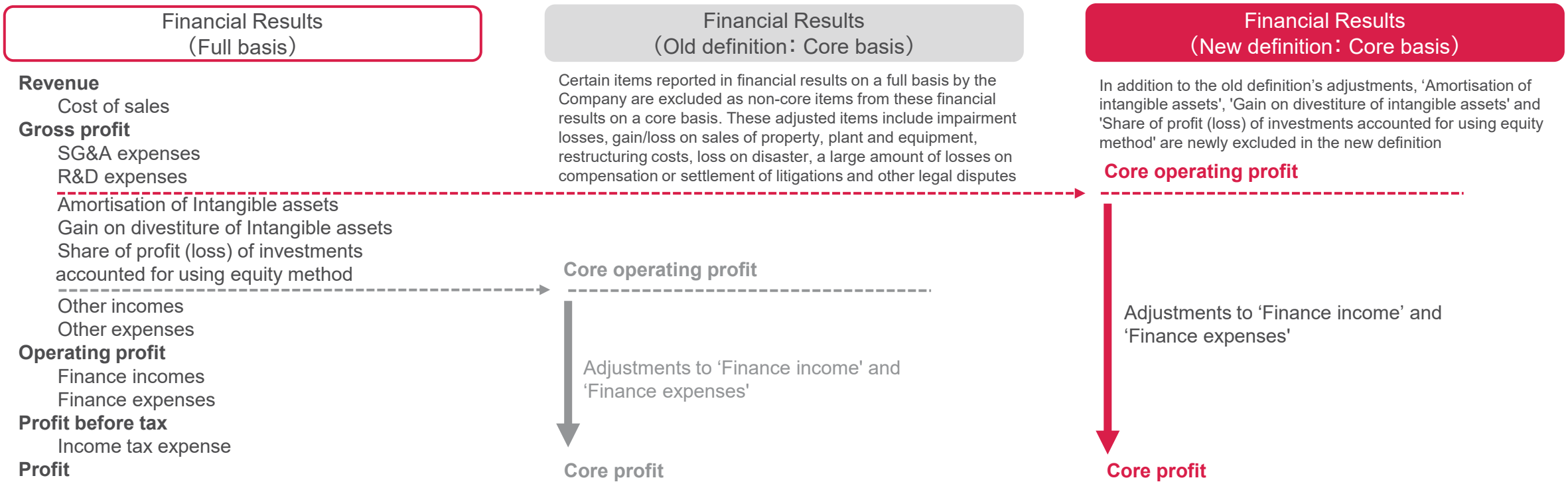

For illustrative purposes only

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*



# ENFORTUMAB VEDOTIN (EV): 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

UC: Urothelial cancer, ORR: Objective response rate, HR+: Hormone receptor positive, HER2-: HER2 negative, GEJ: Gastroesophageal junction, 1L: First line

# ROBUST PIPELINE OF ASTELLAS



## Phase 1

- enfortumab vedotin (NMIBC)
- gilteritinib (Newly diagnosed AML, HIC-ineligible)
- ASP1570
- ASP2138
- ASP1002
- ASP1012
- ASP2802
- ASP3082
- ASP4396
- zocaglusagene nuzaparvovec/ AT845
- ASP2016
- ASP7317
- ASP5502
- abiraterone decanoate/ PRL-02/ASP5541

## Phase 2

- enfortumab vedotin (Other solid tumors)
- 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; Induced 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)

## Submitted/Filed

- enfortumab vedotin (mUC previously untreated: Europe, Japan, China; mUC pretreated: China)
- zolbetuximab (Gastric and GEJ adenocarcinoma, combo with chemotherapy: US, Europe, China)
- avacincaptad pegol (GA secondary to AMD: Europe)
- mirabegron (NDO, pediatric use (aged 3 to less than 18 years): Europe)
- peficitinib (Rheumatoid arthritis: China)

XTANDI and Strategic Brands

Projects 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, CKD: Chronic kidney disease,
NDO: Neurogenic detrusor overactivity, mUC: Metastatic urothelial cancer, GA: Geographic atrophy, AMD: Age-related macular degeneration

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# PROGRESS IN OVERALL PIPELINE

## Phase 1 Entry to Approval since the Last Financial Results Announcement



| Phase 1 Entry | Phase 2 Entry | Phase 3 Entry | Filing | Approval |
|---|---|---|---|---|
| **ASP5502**<br>Primary Sjogren's syndrome | | **zolbetuximab**<br>Gastric and gastroesophageal junction adenocarcinoma (combo with checkpoint inhibitor and chemotherapy) | **zolbetuximab**<br>Gastric and gastroesophageal junction adenocarcinoma (combo with chemotherapy): US | **enzalutamide**<br>Metastatic hormone-sensitive prostate cancer: China |

Note: Phase 1 entry is defined as confirmation of IND open.
Phase transition is defined by approval of company decision body for entering to next clinical phase.
Filing is defined as submission of application to health authorities.
Discontinuation is defined by the decision of company decision body.

IND: Investigational New Drug

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# XTANDI AND STRATEGIC BRANDS: STATUS UPDATE

**(Blue: Updates since the last financial results announcement)**



| Project / Product | Indication | Current status |
|---|---|---|
| **enzalutamide/ XTANDI** | M1 CSPC | • **Approved in China in Jun 2024** |
| **enfortumab vedotin/ PADCEV** | Metastatic urothelial cancer | • Previously untreated (first line): sNDA accepted in Japan in Jan 2024. sBLA accepted in China in Mar 2024. **Received positive CHMP opinion in Europe in Jul 2024**<br>• Pretreated: BLA accepted in China in Mar 2023 |
| | Muscle-invasive bladder cancer | • Phase 3 studies ongoing **(enrollment completed)** |
| | Non-muscle-invasive bladder cancer | • Phase 1 study ongoing |
| | Other solid tumors | • Phase 2 study ongoing. **Additional data from Phase 2 EV-202 study presented at ASCO in Jun 2024** |
| **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) | • Phase 1 study ongoing |
| | AML, post-chemotherapy | • Obtained topline results from Phase 2 GOSSAMER study |
| **zolbetuximab/ VYLOY** | Gastric and GEJ adenocarcinoma | • BLA accepted in China in Jul 2023. **Resubmission acknowledged in US in May 2024**. **Received positive CHMP opinion in Europe in Jul 2024**<br>• **Phase 3 study in combo with CPI and chemotherapy under preparation to start in 1H/CY2025** |
| | Pancreatic adenocarcinoma | • Phase 2 study ongoing (enrollment completed) |
| **fezolinetant/ VEOZAH** | VMS due to menopause | • China: Obtained topline results from Phase 3 MOONLIGHT 1 and MOONLIGHT 3 studies<br>• Japan: Phase 3 studies ongoing |
| | Induced VMS in breast cancer patients | • Phase 3 study under preparation to start in Q2/FY2024 |
| **avacincaptad pegol/ IZERVAY** | GA secondary to AMD | • MAA accepted in Europe in Aug 2023. sNDA for label update accepted in US in Mar 2024 |
| | Stargardt disease | • Phase 2b study ongoing |

VEOZAH: Approved as “VEOZA” in ex-US. M1: Metastatic, CSPC: Castration-sensitive prostate cancer, (s)NDA: (Supplemental) New Drug Application, BLA: Biologics License Application, CHMP: Committee for Medicinal Products for Human Use, ASCO: American Society of Clinical Oncology, 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, MAA: Marketing Authorization Application

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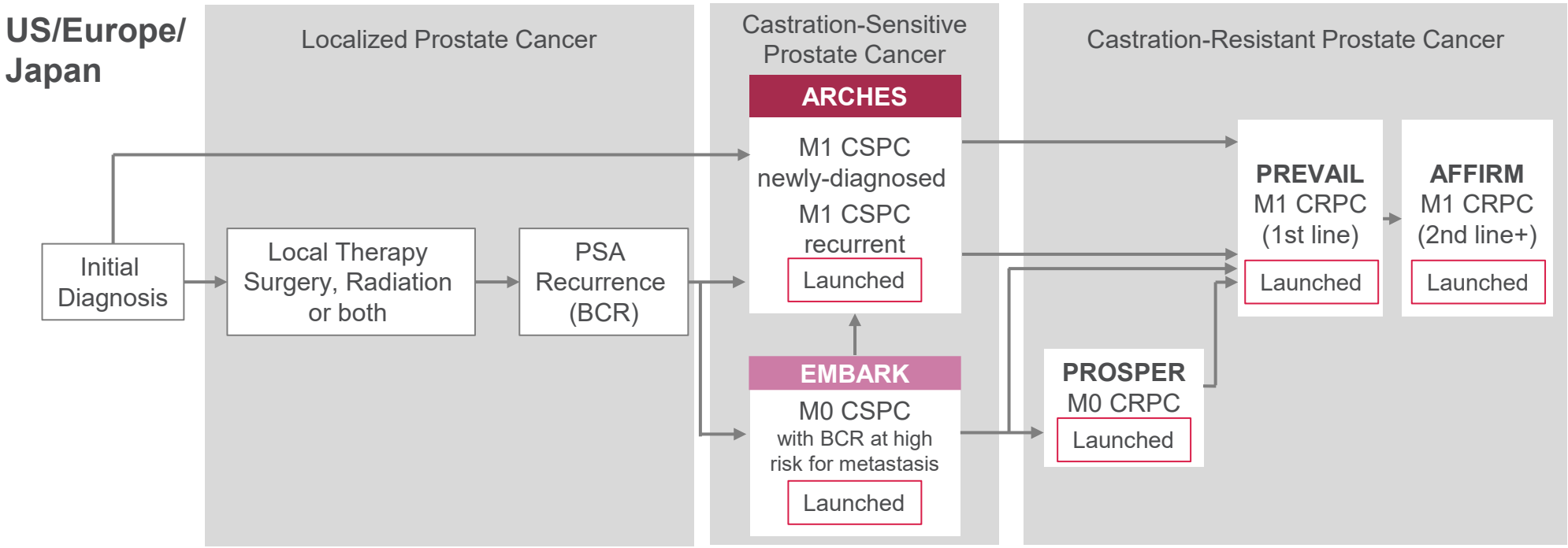
# ENZALUTAMIDE (1/2): ANDROGEN RECEPTOR INHIBITOR

**(Blue: Updates since the last financial results announcement)**






**China** • **M1 CSPC: Approved in Jun 2024**

BCR: Biochemical recurrence, M1: Metastatic, CSPC: Castration-sensitive prostate cancer, M0: Non-metastatic, CRPC: Castration-resistant prostate cancer, ENZA: enzalutamide, ADT: Androgen deprivation therapy, mono: Monotherapy, NDA: New Drug Application

# ENZALUTAMIDE (2/2): PHASE 3 STUDY DATA BY DISEASE STAGE



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

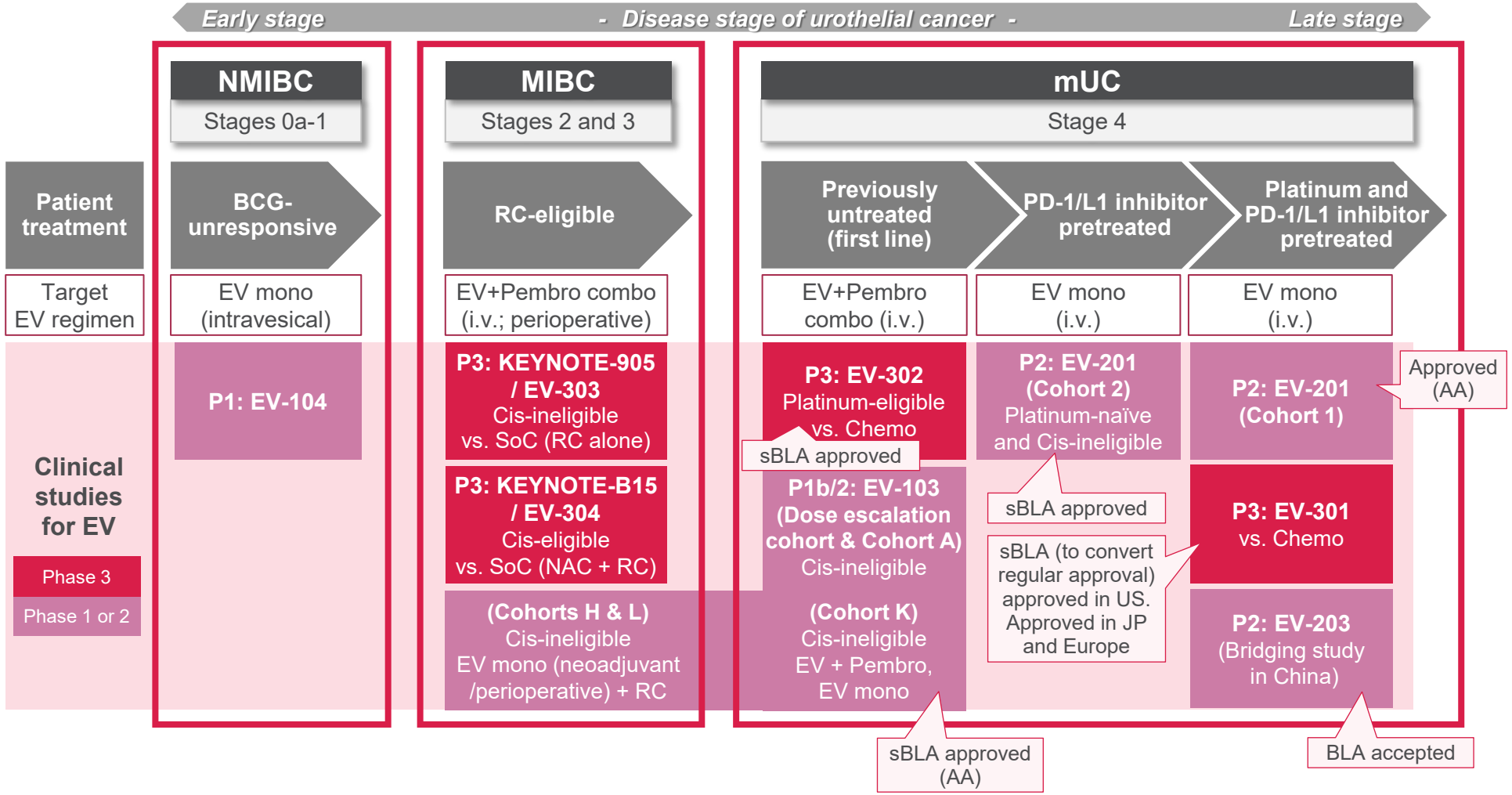
| Disease stage | Early stage → Late stage | | | | | |
|---|---|---|---|---|---|---|
| | Castration-sensitive (CSPC) | | | Castration-resistant (CRPC) | | |
| | M0 | M1 | | M0 | M1 (pre-chemo) | M1 (post-chemo) |
| Phase 3 study | **EMBARK** | **ARCHES** | **ENZAMET** | **PROSPER** | **PREVAIL** | **AFFIRM** |
| 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<br>✓ 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

CSPC: Castration-sensitive prostate cancer, CRPC: Castration-resistant prostate cancer, M0: Non-metastatic, M1: Metastatic, NSAA: Non-steroidal antiandrogen, HR: Hazard ratio, MFS: Metastasis-free survival, rPFS: Radiographic progression-free survival, OS: Overall survival, DoT: Duration of treatment

# ENFORTUMAB VEDOTIN (EV) (1/4): 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) (2/4): CLINICAL STUDIES

**(Blue: Updates since the last financial results announcement)**



## For urothelial cancer

| | | | | | |
|---|---|---|---|---|---|
| | **P3: EV-301** | NCT03474107 | mUC, Platinum and PD-1/L1 inhibitor pretreated;<br>EV mono vs. Chemo | n=608 | sBLA (to convert regular approval) approved in US in Jul 2021.<br>Approved in Japan in Sep 2021, in Europe in Apr 2022 |
| | **P3: EV-302** | NCT04223856 | mUC, Previously untreated, Platinum-eligible;<br>EV + Pembro vs. Chemo | n=1,030 | Approved in US in Dec 2023. sNDA accepted in Japan in Jan 2024. sBLA accepted in China in Mar 2024. **Received positive CHMP opinion in Europe in Jul 2024** |
| | **P3: EV-303 /KEYNOTE-905** | NCT03924895 | MIBC, Cis-ineligible;<br>Pembro +/- EV (perioperative) + RC vs. RC alone | n=857 | **Enrollment completed** |
| | **P3: EV-304 /KEYNOTE-B15** | NCT04700124 | MIBC, Cis-eligible; EV + Pembro (perioperative) + RC<br>vs. Chemo (neoadjuvant) + RC | n=784 | Enrollment completed |
| | **P2: EV-201** | NCT03219333 | mUC, PD-1/L1 inhibitor pretreated; EV mono<br>Cohort 1: Platinum pretreated<br>Cohort 2: Platinum naïve and Cis-ineligible | n=219 | Cohort 1: Approved (under the Accelerated Approval program)<br>Cohort 2: sBLA approved in US in Jul 2021 |
| | **P1b/2: EV-103** | NCT03288545 | Cohorts A - G and K (mUC):<br>A-G: Combo with Pembro and other chemo<br>K: EV mono, EV + Pembro<br>Cohorts H, J and L (MIBC, Cis-ineligible, + RC):<br>H: EV mono (neoadjuvant)<br>J (optional): EV + Pembro (neoadjuvant)<br>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.<br>Enrollment completed |
| | **P2: EV-203** | NCT04995419 | <Bridging study in China><br>mUC, Platinum and PD-1/L1 inhibitor pretreated; EV mono | n=40 | BLA accepted in China in Mar 2023 |
| | **P1: EV-104** | NCT05014139 | NMIBC, High-risk BCG-unresponsive; Intravesical EV mono | n=58 | FSFT: Jan 2022 |

## For other solid tumors

| | | | | | |
|---|---|---|---|---|---|
| | **P2: EV-202** | NCT04225117 | HR+/HER2- breast cancer, Triple-negative breast cancer,<br>Squamous NSCLC, Non-squamous NSCLC, Head and neck cancer,<br>Gastric and esophageal adenocarcinoma including GEJ adenocarcinoma, Esophageal squamous cell carcinoma; EV mono<br>Head and neck squamous cell carcinoma; EV + Pembro | n=320 | **Results obtained for EV mono cohorts** |

mUC: Metastatic urothelial cancer, mono: Monotherapy, Chemo: Chemotherapy, (s)BLA: (Supplemental) Biologics License Application, Pembro: Pembrolizumab, sNDA: Supplemental New Drug Application, CHMP: Committee for Medicinal Products for Human Use, MIBC: Muscle-invasive bladder cancer, Cis: Cisplatin, RC: Radical cystectomy, FSFT: First subject first treatment, NMIBC: Non-muscle-invasive bladder cancer, BCG: Bacillus Calmette-Guerin, HR+: Hormone receptor positive, HER2-: HER2 negative, NSCLC: Non-small cell lung cancer, GEJ: Gastroesophageal junction

# ENFORTUMAB VEDOTIN (EV) (3/4): 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) | 857 (3 arms) | 990 (2 arms) | 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<br>✓ 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

(m)UC: (Metastatic) urothelial cancer, MIBC: Muscle-invasive bladder cancer, cis: Cisplatin, Pembro: Pembrolizumab, mono: Monotherapy, Chemo: Chemotherapy, EFS: Event-free survival, ORR: Objective response rate, CR: Complete response, OS: Overall survival, HR: Hazard ratio, PFS: Progression-free survival, DoR: Duration of response

# ENFORTUMAB VEDOTIN (EV) (4/4): 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**<br>**EV-103 Cohorts** [Phase 1b/2 for AA in US] (combo w/ Pembro) | **Approved**<br>**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**<br>**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**) |

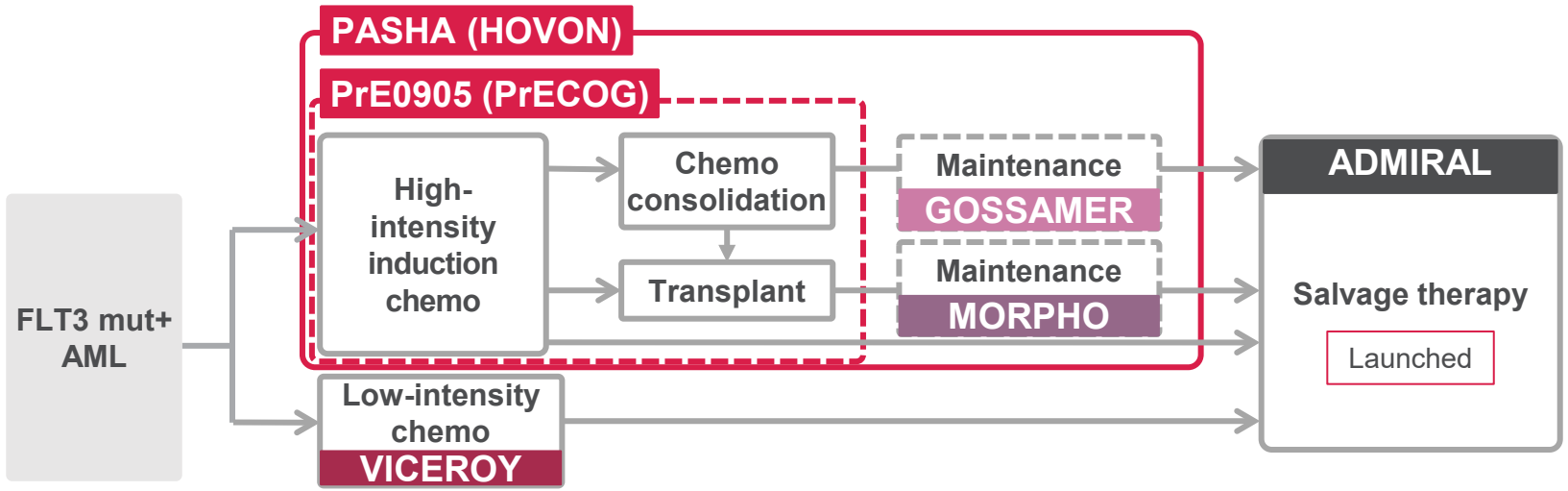
*Monotherapy:
- HR+/HER2- breast cancer
- Triple-negative breast cancer
- Squamous non-small cell lung cancer
- Non-squamous non-small cell lung cancer
- Head and neck cancer
- Gastric and esophageal adenocarcinoma including GEJ adenocarcinoma
- Esophageal squamous cell carcinoma

**Combo w/ Pembro:
- Head and neck squamous cell carcinoma

*US, Germany, France, Italy, Spain, UK, Japan, China (based on internal estimates) **Excluding China
mUC: Metastatic urothelial cancer, MIBC: Muscle-invasive bladder, 1L: First line, 2L+: Second or later line, Cis: Cisplatin, Pembro: Pembrolizumab, AA: Accelerated Approval, HR+: Hormone receptor positive, HER2-: HER2 negative, BCG: Bacillus Calmette-Guerin, GEJ: Gastroesophageal junction

# GILTERITINIB: FLT3 INHIBITOR






| | | | | | |
|---|---|---|---|---|---|
| **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 | Enrollment completed (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 |

**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

FLT3 mut+: FLT3 mutation positive, AML: Acute myeloid leukemia, HIC: High-intensity chemotherapy, FSFT: First subject first treatment, HSCT: Hematopoietic stem cell transplant, HOVON: The Haemato Oncology Foundation for Adults in the Netherlands, BMT-CTN: Blood and Marrow Transplant - Clinical Trial Network, R/R: Relapsed or refractory

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# ZOLBETUXIMAB: ANTI-CLAUDIN 18.2 MONOCLONAL ANTIBODY

**(Blue: Updates since the last financial results announcement)**



## Target: Claudin 18.2

- Claudin is a major structural component of tight junctions and seals intercellular space in epithelial sheets
- 38% of patients had tumors that were CLDN18.2+ in SPOTLIGHT and GLOW studies

## Gastric and GEJ adenocarcinoma

- Metastatic gastric cancer is an area of significant unmet need, especially in advanced stages with ~6% five-year survival rate 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 | BLA accepted in China in Jul 2023. **Resubmission acknowledged in US in May 2024. Received positive CHMP opinion in Europe in Jul 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<br>Cohort 2: First line, Combo with mFOLFOX6<br>Cohort 3: Third or later line, Combo with pembrolizumab<br>Cohort 4: First line, Combo with mFOLFOX6 and nivolumab<br>Cohort 5: Perioperative, Combo with FLOT | n=143 | FSFT: Sep 2018 |
| | Pancreatic adenocarcinoma | **P2** | NCT03816163 | First line, Combo with nab-paclitaxel and gemcitabine, open | n=393 | Enrollment completed |

GEJ: Gastroesophageal junction, HER2-: HER2 negative, CLDN18.2+: Claudin 18.2 positive, mFOLFOX6: 5-FU, leucovorin and oxaliplatin, DB: Double-blind, CAPOX: Capecitabine and oxaliplatin, NDA: New Drug Application, BLA: Biologics License Application, CHMP: Committee for Medicinal Products for Human Use, FLOT: Fluorouracil, leucovorin, oxaliplatin and docetaxel, FSFT: First subject first treatment

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# 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 life [1]

## Women's Health Initiative (WHI) Study [2]

- 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;<br>12 weeks: DB, 2 doses vs. placebo (1:1:1) | n=390 | FSFT: Mar 2024 |
| | | **P3: STARLIGHT 3** | NCT06206421 | VMS associated with menopause;<br>52 weeks: DB, vs. placebo (1:1) | n=260 | FSFT: Feb 2024 |
| | China | **P3: MOONLIGHT 1** | NCT04234204 | Moderate to severe VMS associated with menopause;<br>The first 12 weeks: DB, 30 mg vs. placebo (1:1)<br>The last 12 weeks: Active extension treatment period, 30 mg | n=302 | Primary endpoints not met<br>(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 |

## Induced VMS in breast cancer patients

| | | | | | |
|---|---|---|---|---|---|
| | **P3: HIGHLIGHT 1** | NCT06440967 | Moderate to severe VMS associated with adjuvant endocrine therapy for breast cancer;<br>52 weeks (efficacy endpoints at 4 and 12 weeks):<br>DB, vs. placebo (1:1) | n=540 | Under preparation to start in Q2/FY2024 |

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

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# AVACINCAPTAD PEGOL (ACP): COMPLEMENT C5 INHIBITOR / PEGYLATED RNA APTAMER



## 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 eye [1]
- Approximately 75% of people living with GA in the US are believed to be undiagnosed [2]
- Without timely treatment, an estimated 66% of people with GA may become blind or severely visually impaired [3]

## 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)<br>Part 2: 2 mg, 4 mg vs. Sham (n=209) | n=286 | MAA accepted in Europe in Aug 2023. sNDA for label update accepted in US in Mar 2024 |
| | | **P3: GATHER2** | NCT04435366 | 2 mg vs. Sham | n=448 | |
| | **Stargardt disease** | **P2b** | NCT03364153 | vs. Sham | n=121 | FSFT: Jan 2018 |

1. Retina 37:819-835 (2017). 2. IQVIA Medical Claims (DX) data Jan '20-Dec '21: 24 Months. 3. JAMA Ophthalmol 139:743-750 (2021)
MAA: Marketing Authorization Application, sNDA: Supplemental New Drug Application, FSFT: First subject first treatment

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# ON THE FOREFRONT OF HEALTHCARE CHANGE