

# **Q1/FY2021 FINANCIAL RESULTS**

## **ENDED JUNE 30, 2021**



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**Chief Strategy Officer and Chief Financial Officer**  
**Astellas Pharma Inc.**  
**July 30, 2021**

# 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/FY2021 Consolidated Financial Results  
FY2021 Revised Forecasts

II

Initiatives for Sustainable Growth

# Q1/FY2021 FINANCIAL RESULTS

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| (billion yen)                     | Q1/FY20      | Q1/FY21      | Change       | Change (%)    | FY21 FCST      | Progress     | FX impact      |
|-----------------------------------|--------------|--------------|--------------|---------------|----------------|--------------|----------------|
| <b>Revenue</b>                    | <b>307.0</b> | <b>326.1</b> | <b>+19.2</b> | <b>+6.2%</b>  | <b>1,323.0</b> | <b>24.7%</b> | +13.6 bil. yen |
| Cost of sales                     | 59.7         | 62.2         | +2.6         | +4.3%         |                |              |                |
| % of revenue                      | 19.4%        | 19.1%        | -0.4 ppt     |               |                |              |                |
| <b>SG&amp;A expenses</b>          | <b>120.8</b> | <b>137.1</b> | <b>+16.3</b> | <b>+13.5%</b> | <b>541.0</b>   | <b>25.3%</b> |                |
| US XTANDI co-pro fee              | 31.5         | 34.5         | +3.0         | +9.4%         |                |              |                |
| SG&A excl. the above              | 89.3         | 102.6        | +13.4        | +15.0%        |                |              |                |
| <b>R&amp;D expenses</b>           | <b>57.3</b>  | <b>58.3</b>  | <b>+1.0</b>  | <b>+1.8%</b>  | <b>242.0</b>   | <b>24.1%</b> |                |
| Amortisation of intangible assets | 5.9          | 6.0          | +0.1         | +1.8%         |                |              |                |
| <b>Core operating profit</b>      | <b>63.4</b>  | <b>62.8</b>  | <b>-0.6</b>  | <b>-0.9%</b>  | <b>270.0</b>   | <b>23.3%</b> | +6.1 bil. yen  |
| <Full basis>                      |              |              |              |               |                |              |                |
| Other income                      | 2.2          | 0.4          | -1.8         | -             |                |              |                |
| Other expense                     | 4.8          | 27.1         | +22.3        | -             |                |              |                |
| <b>Operating profit</b>           | <b>60.8</b>  | <b>36.1</b>  | <b>-24.7</b> | <b>-40.7%</b> | <b>265.0</b>   | <b>13.6%</b> |                |
| Profit before tax                 | 60.2         | 35.8         | -24.4        | -40.5%        | 263.0          | 13.6%        |                |
| <b>Profit</b>                     | <b>50.4</b>  | <b>30.7</b>  | <b>-19.7</b> | <b>-39.1%</b> | <b>209.0</b>   | <b>14.7%</b> |                |

# Q1/FY2021 FINANCIAL RESULTS: OVERVIEW

*Revenue increased, Core OP was the same level as previous fiscal year and in line with assumptions of full-year forecast*

- Sales of XTANDI and Strategic products\* increased as expected, offsetting sales decrease due to the transfer of mature products
- SG&A spending is slightly ahead of full-year forecast  
R&D expenses are on track

*Full basis: OP and Profit were behind full-year forecast*

- Booked impairment losses, not included in full-year forecast:  
Termination of development for ASP0892 and bleselumab

# Q1/FY2021 FINANCIAL RESULTS: REVENUE

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*Revenue increase driven by growth of XTANDI and Strategic products\*, which offsets the sales decrease from transfer of mature products, along with temporary positive factors due to FX and reversal of COVID-19 impact in previous fiscal year*

|                | Q1/FY20               | Q1/FY21               | Change                | Change (%)   |
|----------------|-----------------------|-----------------------|-----------------------|--------------|
| <b>Revenue</b> | <b>307.0 bil. yen</b> | <b>326.1 bil. yen</b> | <b>+19.2 bil. yen</b> | <b>+6.2%</b> |

Increase in XTANDI and Strategic products

**XTANDI, XOSPATA, PADCEV, Evrenzo**

**+25.3 bil. yen**



➤ Returned sales of Lexiscan, negatively impacted by COVID-19 in Q1/FY20 **+9.6 bil. yen**

Termination of sales promotion/ transfer of manufacturing rights/ transfer of product

**Celecox, Lipitor, Eligard**

**-17.5 bil. yen**



\* XOSPATA, PADCEV, Evrenzo

# Q1/FY2021 FINANCIAL RESULTS: SALES OF MAIN PRODUCTS

Q1/FY2021 actual (billion yen)

**XTANDI**

**132.9**

YoY: +21.0 (+19%)

Progress  
against FCST: 24%

- ✓ Global sales increased and in line with forecast, driven by growth mainly in US and EU
- ✓ Approved additional indication (M1 CSPC) in EU and recommended by NICE in UK
- ✓ In China, demand grew higher than expected after reimbursement

**XOSPATA**

**8.3**

YoY: +2.7 (+48%)

Progress  
against FCST: 23%

- ✓ Global sales increased and in line with forecast, driven by growth mainly in US and EU
- ✓ Sales contribution from China (launched in Apr 2021)

**PADCEV**

**4.2**

YoY: +1.2 (+42%)

Progress  
against FCST: 21%

- ✓ Revenue in US grew steadily and in line with forecast
- ✓ Approved additional indication in Jul 2021 and continued growth is expected

**Evrenzo**

**0.6**

YoY: +0.5 (+283%)

Progress  
against FCST: 7%

- ✓ Sales have steadily increased as expected in Japan, driven by increased adoption in major institutions

**mirabegron**

**44.0**

YoY: +3.6 (+9%)

Progress  
against FCST: 25%

- ✓ Global sales increased and in line with forecast
- ✓ In China, demand grew after reimbursement



# Q1/FY2021 FINANCIAL RESULTS: COST ITEMS

*SG&A spending is slightly ahead of full-year FCST but within controllable range for the full year. R&D expenses are on track*

## Core basis: Main items for YoY and progress against FCST

### Cost of sales % of revenue



YoY: -0.4ppt

- ✓ Decrease mainly due to changes in product mix

### SG&A expenses

YoY: +13.5%

Progress  
against FCST: 25.3%



- ✓ SG&A excl. XTANDI US co-pro fee: +13.4 bil. yen (YoY +15.0%)
- ✓ FX impact (+4.4 bil. yen) and one-off increase factor from decrease of sales promotion expenses and travel expenses in Q1/FY2020 due to COVID-19 (Approx. +6.0 bil. yen)
- ✓ Up-front investment to support CSP2021 initiatives (Approx. +3.0 bil. yen)

### R&D expenses

YoY: +1.8%

Progress  
against FCST: 24.1%



- ✓ Investment increase in zolbetuximab and Primary Focus
- ✓ Decrease in development cost of fezolinetant



# FY2021 REVISED FORECAST

- No changes have been made to Core basis FY2021 forecast
- Downward revision of Full basis profit
  - ✓ Booked Impairment losses on intangible assets in Q1/FY2021 due to termination of development projects (ASP0892: 21.5 bil. yen, bleselumab: 4.1 bil. yen)
  - ✓ Severance expenses due to early retirement incentive program (To be booked in Q3/FY2021: Approx. 10.0 bil. yen)

| (billion yen)    | Initial Forecast<br>(Disclosed<br>in Apr 2021) | Revised Forecast | Change |
|------------------|--|------------------|--------|
| Operating profit | 265.0  | 227.0            | -38.0  |
| Profit           | 209.0  | 183.0            | -26.0  |

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# XTANDI & STRATEGIC PRODUCTS\*: HIGHLIGHT

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## Key Events Expected in FY2021

\* XOSPATA, PADCEV, zolbetuximab, Evrenzo, fezolinetant, AT132

| Milestone             | Project                     | Indication / Clinical study   | Achieved                                   |
|-----------------------|-----------------------------|---|--|
| Regulatory decision   | enzalutamide /XTANDI        | M1 hormone-sensitive prostate cancer (EU)   | Apr 2021                                   |
|                       | enfortumab vedotin / PADCEV | mUC, platinum and PD-1/L1 inhibitor pretreated (US <sup>a,b</sup> )                         | Jul 2021                                   |
|                       |                             | mUC, cis-ineligible and who have previously received one or more therapy (US <sup>a</sup> ) | Jul 2021                                   |
|                       |                             | mUC, platinum and PD-1/L1 inhibitor pretreated (EU <sup>c</sup> )                           |  |
|                       |                             | mUC, progressed after anti-cancer medication (JP <sup>d</sup> )                             |  |
|                       | roxadustat /Evrenzo         | Anemia associated with CKD (EU)   | CHMP positive opinion received in Jun 2021 |
| Regulatory submission | gilteritinib /XOSPATA       | R/R AML (China <sup>e</sup> )   |  |
| Data readout          | fezolinetant                | 52-week safety results from Phase 3 SKYLIGHT 1, 2 & 4 studies                               | Jul 2021 (SKYLIGHT 2)                      |

a: Priority Review granted, Real-Time Oncology Review pilot program and Project Orbis applied. b: sBLA to convert Accelerated Approval to regular approval.  
c: Accelerated Assessment granted. d: Priority Review granted. e: sNDA to convert conditional approval to full approval

## Other Updates since FY2020 Financial Results Announcement in Apr 2021

| Project                     | Indication                    | Updated status  |
|-----------------------------|-------------------------------|---|
| enfortumab vedotin / PADCEV | NMIBC                         | Phase 1 study with intravesical therapy under preparation to start in Q2 FY2021   |
| fezolinetant                | VMS associated with menopause | Japan Phase 2b study under preparation to start in Q3 FY2021  |
| AT132                       | XLMTM                         | Dosing in ASPIRO study resumed in Jul 2021. Planning to include 3 additional patients (6 new patients in total) at the lower dose |



M1: Metastatic, mUC: Metastatic urothelial cancer, CKD: Chronic kidney disease, CHMP: Committee for Medicinal Products for Human Use, R/R AML: Relapsed or refractory acute myeloid leukemia, sBLA: Supplemental Biologics License Application, sNDA: Supplemental New Drug Application, NMIBC: Non-muscle-invasive bladder cancer, VMS: Vasomotor symptoms, XLMTM: X-linked myotubular myopathy

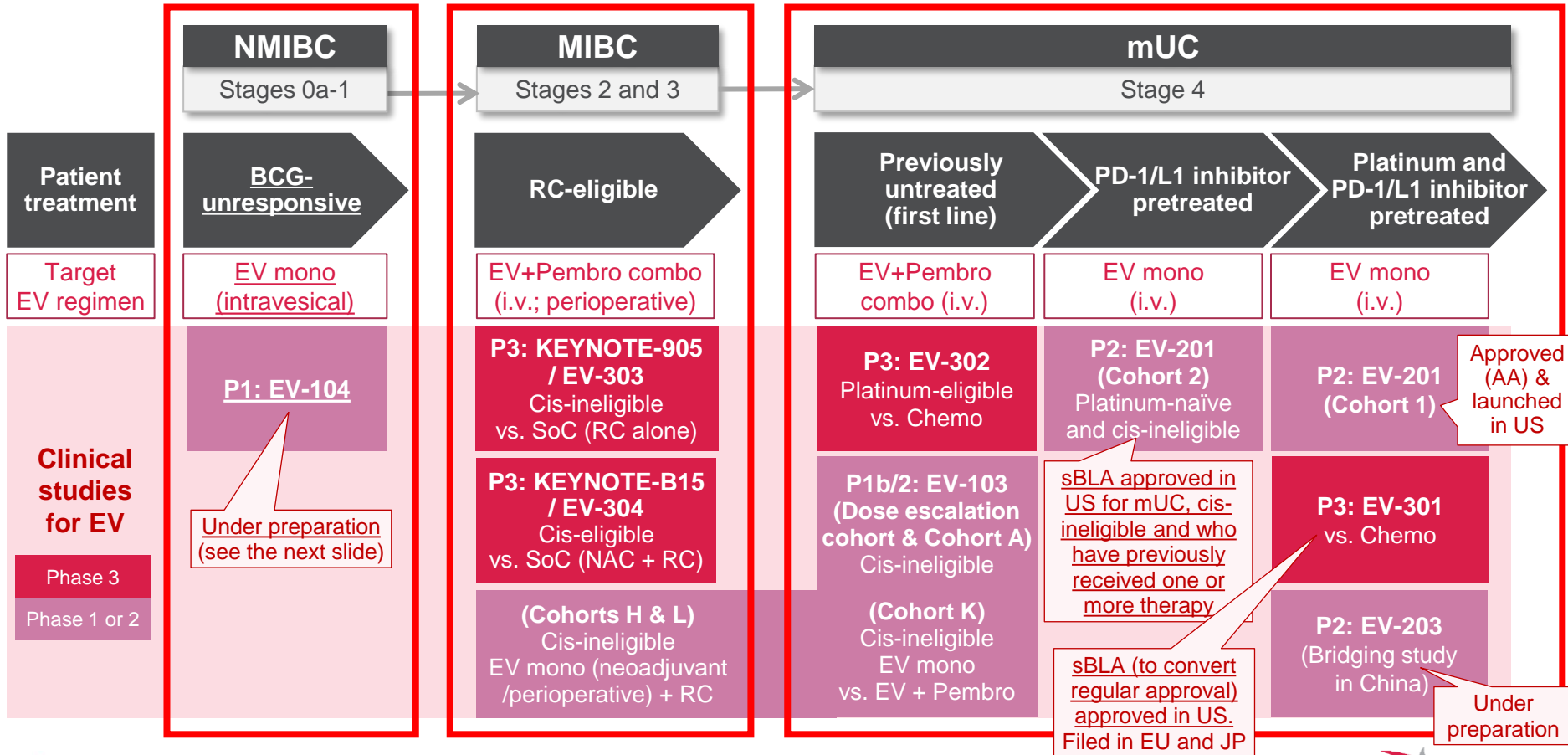
# ENFORTUMAB VEDOTIN (EV) (1/2): OVERALL UC PROGRAM

*sBLAs for mUC approved in US, based on the robust clinical study data*

Early stage

- Disease stage of urothelial cancer -

Late stage

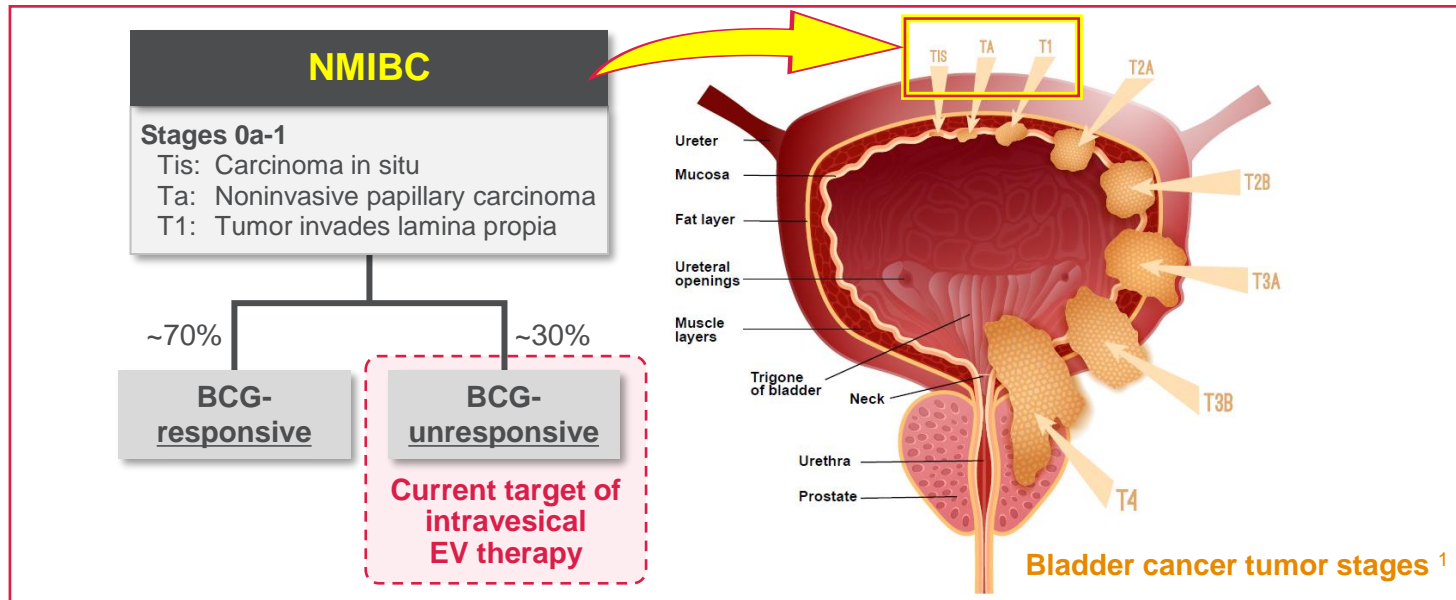


Underlined: Updates since FY2020 financial results announcement in Apr 2021

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) (2/2): NMIBC - LANDSCAPE AND DEVELOPMENT PROGRAM

*To explore the activity of intravesical EV in earlier-stage UC*



## SoC\* and UMN for NMIBC (\*Approved drugs and SoC varies by region)

- The traditional SoC is TURBT followed by intravesical BCG therapy, reducing disease recurrence by about 70%
- However, approx. 30% of patients are unresponsive to BCG, and recurrence and progression remain common. Treatment options for BCG-unresponsive patients are limited

## Clinical development with EV in NMIBC

- Phase 1 EV-104 study with intravesical EV dosing in high-risk BCG-unresponsive NMIBC patients under preparation to start in Q2 FY2021

# FEZOLINETANT: DEVELOPMENT PROGRESS

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*Obtained 52-week data of SKYLIGHT 2 study*

*Clinical development locally in Japan under preparation*

## US and EU

- Phase 3 SKYLIGHT 2 study (pivotal):
  - ✓ Obtained 52-week data in Jul 2021, which support the long-term use of fezolinetant
  - ✓ Study data focusing 12-week data to be presented at NAMS 2021 in Sep 2021
- Overall safety to be assessed later in FY2021 with 52-week data of all the three Phase 3 studies also including SKYLIGHT 1 (pivotal) and SKYLIGHT 4 (long-term)  
*=> US-NDA and EU-MAA submissions targeted in FY2022*

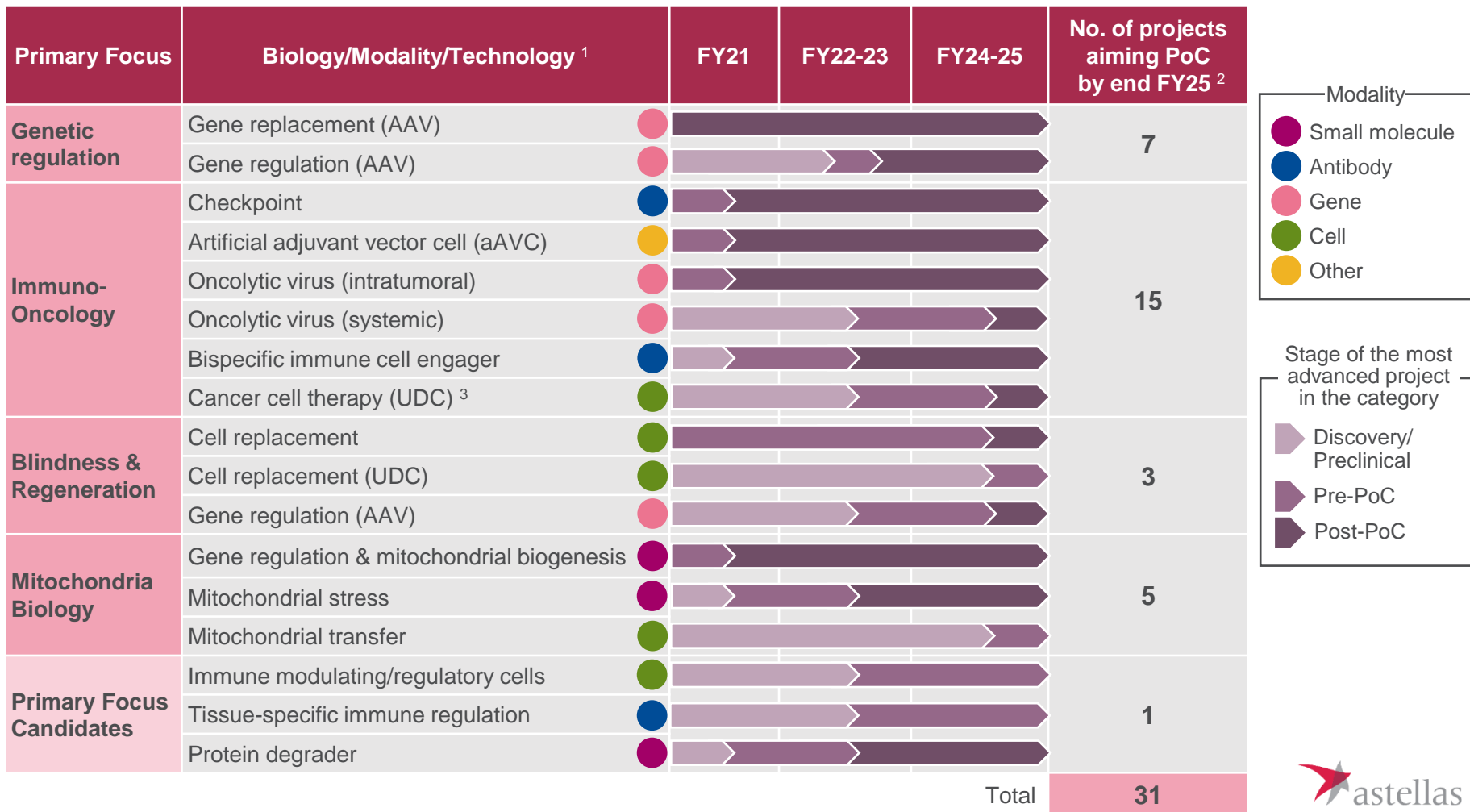
## Japan

- Phase 2b dose-finding study in Japanese patients under preparation to start in Q3 FY2021

# PROGRESS IN FOCUS AREA APPROACH (1/3): CLINICAL PROOF AND EXPANSION OF KEY PLATFORMS

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Primary Focuses have robust pipeline to newly build Post-PoC portfolio by end FY2025



1. Not exhaustively listed. 2. Estimated based on standard development timelines, assuming 100% probability of success (as of May 2021).

3. The first convertibleCAR program (with autologous cells) IND is planned for late FY2021. CSP: Corporate Strategic Plan, PoC: Proof of concept (key clinical data supporting a decision to initiate late-stage development), AAV: Adeno-associated virus, UDC: Universal donor cell

# PROGRESS IN FOCUS AREA APPROACH (2/3): CURRENT STATUS IN PRIMARY FOCUS

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| Primary Focus            | Biology/Modality/Technology <sup>1</sup>   | Project                                | Current status  |
|--------------------------|--|--|---|
| Genetic regulation       | Gene replacement (AAV)                     | AT132                                  | (See the slides for “XTANDI and Strategic products”)  |
|                          |  | AT845                                  | Phase 1 study ongoing   |
|                          | Gene regulation (AAV)                      |  |   |
| Immuno-Oncology          | Checkpoint                                 | ASP1948                                | Phase 1 study ongoing   |
|                          |  | ASP1951                                | Phase 1 study ongoing   |
|                          | Artificial adjuvant vector cell (aAVC)     | ASP7517                                | Phase 1 study in R/R AML and MDS ongoing<br>Phase 1 study in advanced solid tumors to start in <u>Q2 FY2021</u> |
|                          |  | ASP0739                                | Phase 1 study to start in <u>Q2 FY2021</u>  |
|                          | Oncolytic virus (intratumoral)             | ASP9801                                | Phase 1 study ongoing   |
|                          | Oncolytic virus (systemic)                 |  |   |
|                          | Bispecific immune cell engager             |  |   |
|                          | Cancer cell therapy (UDC)                  |  |   |
| (other)                  | ASP1570                                    | Phase 1 study to start in Q2-Q3 FY2021 |   |
| Blindness & Regeneration | Cell replacement                           | ASP7317                                | Screening and enrollment in Phase 1b study put on hold, <u>due to a manufacturing delay</u>                     |
|                          | Cell replacement (UDC)                     |  |   |
|                          | Gene regulation (AAV)                      |  |   |
| Mitochondria Biology     | Gene regulation & mitochondrial biogenesis | ASP1128                                | Phase 2a study ongoing  |
|                          |  | ASP0367                                | <u>FSFT in Phase 2/3 study in PMM in Jun 2021</u><br>Phase 1b study in DMD ongoing                              |
|                          | Mitochondrial stress                       |  |   |
|                          | Mitochondrial transfer                     |  | <u>License agreement with Minovia Therapeutics in Jul 2021</u>  |
| Primary Focus Candidates | Immune modulating/regulatory cells         |  |   |
|                          | Tissue-specific immune regulation          |  |   |
|                          | Protein degrader                           |  |   |

| Modality   |
|--|
| <span style="color: purple;">●</span> Small molecule |
| <span style="color: blue;">●</span> Antibody         |
| <span style="color: pink;">●</span> Gene             |
| <span style="color: green;">●</span> Cell            |
| <span style="color: yellow;">●</span> Other          |

Underlined: Updates since FY2020 Financial Results Announcement in Apr 2021. 1. Not exhaustively listed.

AAV: Adeno-associated virus, UDC: Universal donor cell, R/R: Relapsed and refractory, AML: Acute myeloid leukemia, MDS: Myelodysplastic syndrome, FSFT: First subject first treatment, PMM: Primary mitochondrial myopathies, DMD: Duchenne muscular dystrophy



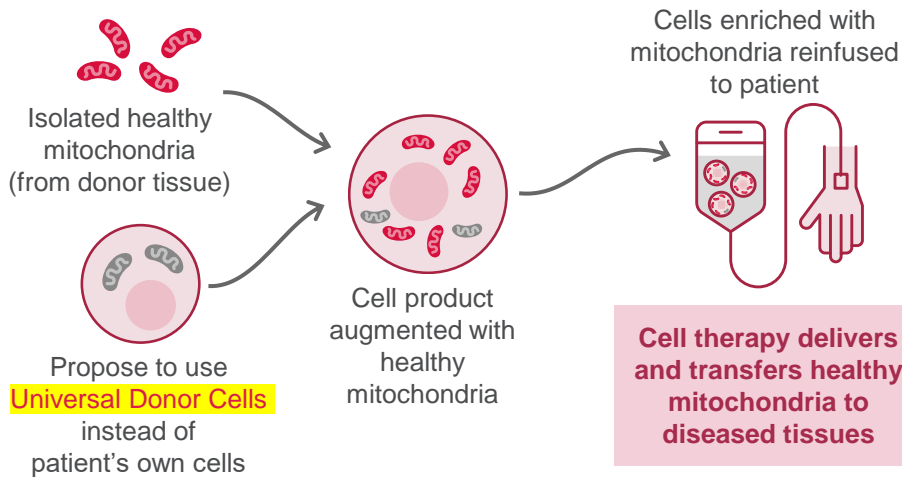
# PROGRESS IN FOCUS AREA APPROACH (3/3): MITOCHONDRIA BIOLOGY

*Strategic collaboration for mitochondrial cell therapy (Mitochondrial transfer\*) program with Minovia Therapeutics, leading company in this field*

\* Mechanism to transfer healthy mitochondria from donor cells to diseased cells

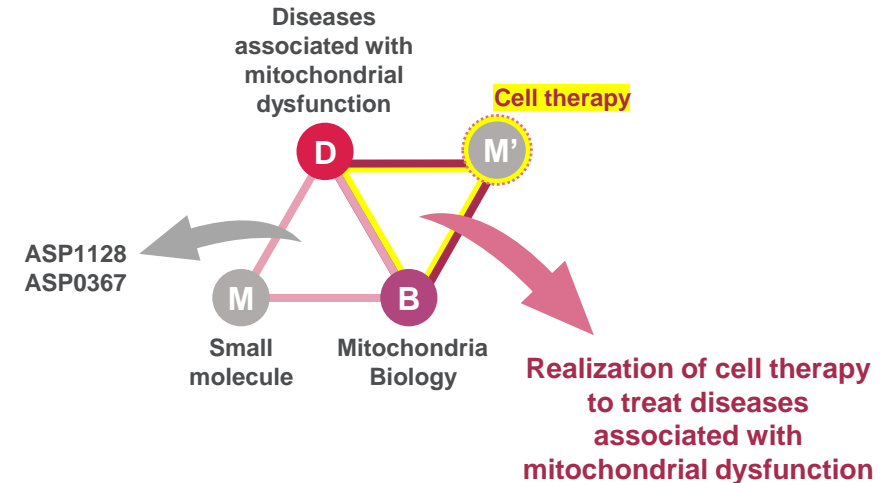
## MAT platform from Minovia Therapeutics

- Technology where patient's own cells are isolated, augmented with healthy mitochondria purified from healthy donor tissue, and then re-infused back into the patient



## Synergy in mitochondrial cell therapy

- Creating an innovative cell therapy program by combining Astellas' off-the-shelf Universal Donor Cells with Minovia's MAT Platform



# ASP3772 AS PNEUMOCOCCAL VACCINE

*Obtained positive Phase 2 study data in adults*

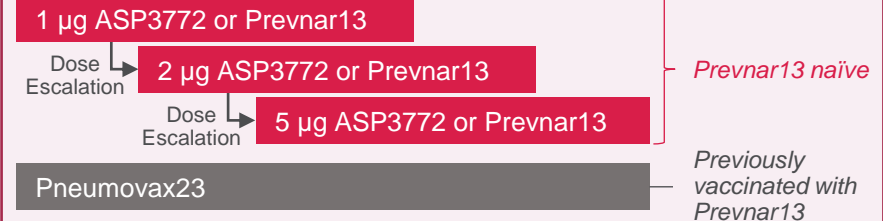
## ASP3772 profiles

- ASP3772 is a 24-valent vaccine for prevention of pneumococcal disease, utilizing Affinivax' Multiple Antigen Presenting System (MAPS) technology
- The MAPS vaccine platform is designed to enable the high affinity binding of protective polysaccharides and proteins in a single vaccine, offering the potential to provide broader protection against invasive disease than currently available vaccines, as well as the potential to reduce nasopharyngeal colonization

## Phase 2 study in elderly subjects

- Phase 2 study in adults aged 65-85 years show:
  - ✓ ASP3772 is well tolerated
  - ✓ Immune response with ASP3772 is equal to or greater than both Prevnar13 and Pneumovax23

### Phase 2 study in adults (Stage 2 in Phase 1/2 study)



## Current status

- Clinical development:
  - ✓ Phase 1 study in toddlers (12-15 months of age) ongoing
  - ✓ Phase 3 studies in adults planned
- Breakthrough Therapy Designation granted by FDA for adults  $\geq 50$  years of age
- Strategic options currently under consideration



# PROGRESS IN Rx+ PROGRAM



## Key events expected in FY2021 (announced in Apr 2021)

| Sphere *                               | Program                               | Event   | Achieved |
|--|---------------------------------------|---|----------|
| Chronic disease progression prevention | Fit-eNce                              | Initiation of pilot marketing for at-home service |          |
|  | Game application for exercise support | Initiation of pilot marketing                     |          |
|  | BlueStar                              | Initiation of clinical study (Japan)              |          |
|  | My Holter II                          | Commercialization of service                      | Jul 2021 |
| Patient outcome maximization           | ASP5354                               | Topline results for Phase 2 study                 |          |

## Other updates

| Sphere *                     | Program | Event                                | Achieved |
|------------------------------|---------|--------------------------------------|----------|
| Patient outcome maximization | ASP5354 | Initiation of Phase 1 study in Japan | Jun 2021 |



\* Business areas to focus on for realization of Rx+ Story

# SUSTAINABILITY: CLIMATE CHANGE MEASURES



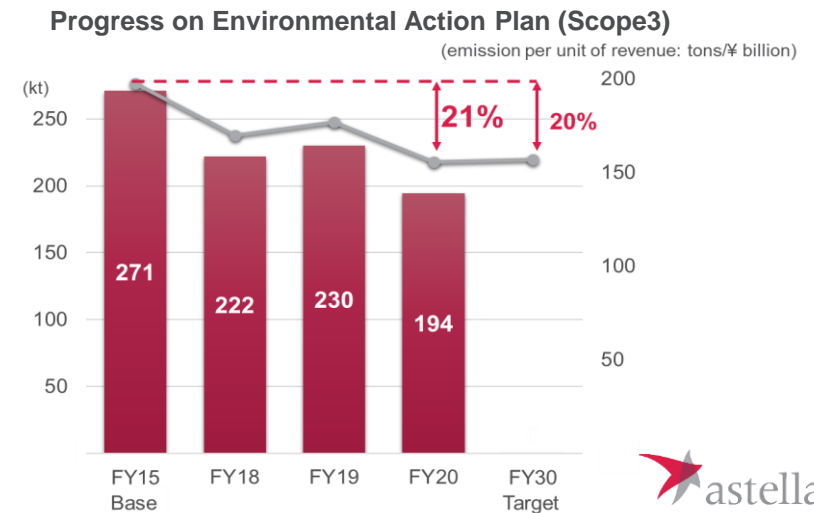
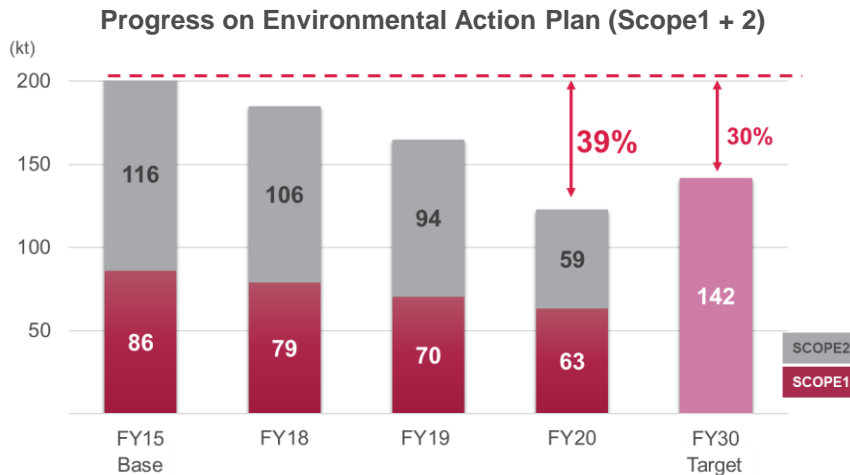
*Reduction of GHG emissions is moving forward as the Environmental Action Plan progresses*

## Environmental Action Plan (climate change mitigation measures) (SBT approved)

- Reduce GHG emissions (Scope 1 + 2) by 30% by FY2030 (Base year: FY2015)
- Reduce GHG emissions (Scope 3) by 20% per unit of revenue by FY2030 (Base year: FY2015)

## Progress on Action Plan (FY2020 results)

- In addition to using power derived from renewable energy sources, external factors such as measures to counter the spread of COVID-19 have resulted to reducing GHG emissions by 39% compared to FY2015 (Scope 1 + 2)



GHG: Greenhouse Gas, SBT: Science Based Targets

Scope 1: GHGs emitted directly from Company premises as a result of the burning of fuels, Scope 2: GHGs emitted indirectly in the use of electric power or heat supplied to the Company from outside, Scope 3: GHGs emitted indirectly at some point on the Company's value chain

# PROGRESS TOWARD ACHIEVING CSP2021

## Revenue, Pipeline Value

- 1** XTANDI and Strategic products\*:  $\geq$  ¥1.2T in FY2025
  - ✓ Steady sales growth
  - ✓ XTANDI: Approval for M1 CSPC (EU)
  - ✓ PADCEV: Approval for cis-ineligible mUC 2L (US)
  - ✓ fezolinetant: SKYLIGHT 2 52-week data obtained

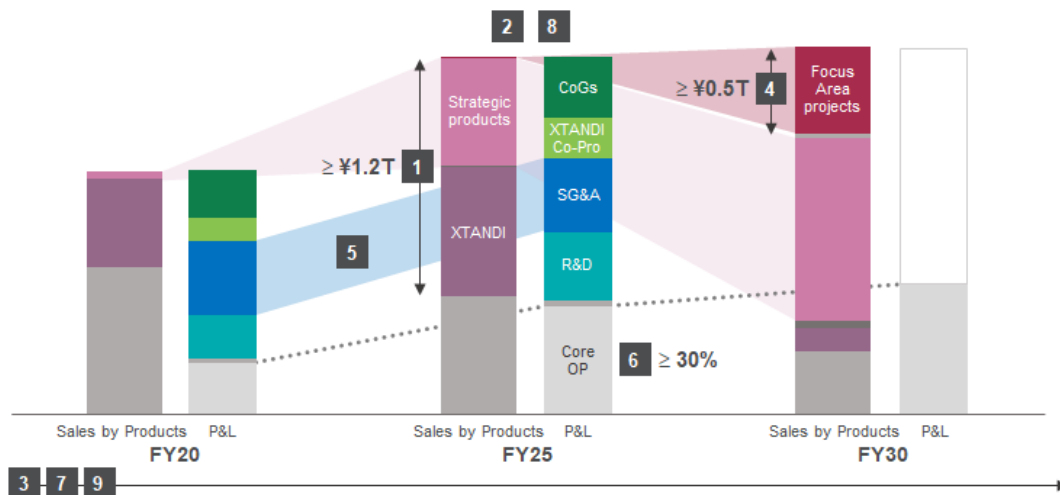
- 2** Post-PoC projects from Primary Focuses
  - ✓ AT132: Dosing in clinical study resumed
- 3** Multiple technology platforms
  - ✓ ASP0367: FSFT in Phase 2/3 study
- 4** Focus Area projects:  $\geq$  ¥0.5T in FY2030
  - ✓ Collaboration with Minovia

## Core OP

- 5** Flat SG&A in absolute terms
  - 6** Sufficient R&D investments  
Core OP margin of  $\geq$  30% in FY2025
  - 7** Steady increase in dividends
- ✓ Initiatives to drive efficiency & excellence (Astellas online MR, product transfer to Cheplapharm)

## Future Growth

- 8** Rx+: Breakeven by FY2025
  - ✓ Commercialization of My Holter II
- 9** Sustainability
  - ✓ Reduction of GHG emissions moving forward



\* XOSPATA, PADCEV, zolbetuximab, Evrenzo, fezolinetant, AT132  
 CSP: Corporate Strategic Plan, M1 CSPC: Metastatic castration-sensitive prostate cancer, cis: Cisplatin, mUC: Metastatic urothelial cancer, 2L: Second line, PoC: Proof of concept, FSFT: First subject first treatment, MR: Medical representative, GHG: Greenhouse gas

# APPENDIX

The image features a central, high-speed photograph of a clear water droplet falling into a pool of water, creating concentric ripples. The background is a composition of geometric shapes: a white upper section, a grey lower section, and a red triangular section on the right side. The word 'APPENDIX' is printed in a bold, black, sans-serif font on the left side of the image.

# Q1/FY2021: REVENUE BY REGION

| (billion yen)                | Q1/FY20 | Q1/FY21 | Change (%) |
|------------------------------|---------|---------|------------|
| <b>Japan</b>                 | 77.8    | 67.5    | -13.2%     |
| <b>United States</b>         | 117.2   | 133.6   | +14.1%     |
| <b>Established Markets</b>   | 64.0    | 78.0    | +21.8%     |
| <b>Greater China</b>         | 14.2    | 16.4    | +15.5%     |
| <b>International Markets</b> | 30.2    | 27.8    | -8.1%      |

Established Markets: Europe, Canada, Australia

Greater China: China, Hong Kong, Taiwan

International Markets: Russia, Latin America, Middle East, Africa, South East Asia, South Asia, Korea, Export sales, etc.

# Q1/FY2021: SALES OF MAIN PRODUCTS

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| (billion yen) | Q1/FY20 | Q1/FY21 | Change  | CER growth | FY21 FCST |
|---------------|---------|---------|---------|------------|-----------|
| XTANDI        | 112.0   | 132.9   | +18.7%  | +13.3%     | 557.2     |
| XOSPATA       | 5.6     | 8.3     | +47.7%  | +41.7%     | 36.7      |
| PADCEV        | 3.0     | 4.2     | +41.9%  | +39.5%     | 20.1      |
| Evrenzo       | 0.2     | 0.6     | +282.9% | -          | 8.6       |
| mirabegron    | 40.4    | 44.0    | +8.8%   | +5.4%      | 175.2     |
| Prograf       | 45.3    | 45.2    | -0.3%   | -6.7%      | 192.6     |



PADCEV: Co-promotion revenue from Seagen  
 mirabegron (Product name: Betanis/Myrbetriq/BETMIGA)  
 Prograf: Incl. Advagraf/Graceptor/ASTAGRAF XL



# Q1/FY2021 FINANCIAL RESULTS: BUSINESS UPDATE FOR MAIN PRODUCTS

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## **XTANDI**

Global sales are in line with forecast and continued growth is expected. In US, demand grew in excess of 10% YoY. In EU, additional indication (M1 CSPC) approved in Apr 2021 and XTANDI recommended by NICE in UK for M1 CSPC indication in Jun 2021. In China, demand grew higher than expected after reimbursement in Mar 2021

## **XOSPATA**

Sales in US and Europe steadily expanded and global sales are in line with forecast. Initial sales trend is positive thus far in China launched in Apr 2021 (Q1/FY21 sales: 0.5 billion yen). In EU, reimbursement has started in Nordics, Netherlands and Belgium in addition to UK, Germany and Italy

## **PADCEV**

Revenue in US grew steadily, progressing as expected. Additional indication (locally advanced or mUC who are ineligible for cisplatin-containing chemotherapy and have previously received one or more lines of therapy) approved in Jul 2021 and continued growth is expected

## **Evrenzo**

Sales in Japan are in line with forecast. Following expansion of the indication in Nov 2020 and the subsequent lifting of the 2-week prescribing restriction in Dec 2020, sales have steadily increased, driven by increased adoption in major institutions. Evrenzo is now the market leading HIF-PHI

## **mirabegron**

Global sales are in line with forecast. In China, demand grew after reimbursement in Mar 2021. In US, FDA approved Myrbetriq for the treatment of neurogenic detrusor overactivity with expected Granules (extended-release oral suspension) launch in Q2



# Q1/FY2021 ACTUAL: FX RATE

## Average rate for the period

| Currency | Q1/FY20 | Q1/FY21 | Change  |
|----------|---------|---------|---------|
| USD      | 108 yen | 109 yen | +2 yen  |
| EUR      | 118 yen | 132 yen | +13 yen |

## Change in closing rate from previous fiscal year end

| Currency | Q1/FY20 | Q1/FY21 |
|----------|---------|---------|
| USD      | -1 yen  | -0 yen  |
| EUR      | +2 yen  | +2 yen  |

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

- 13.6 billion yen increase in revenue, 6.1 billion yen increase in core OP
- FX impact on elimination of unrealized gain: COGs ratio +0.1 ppt

# FY2021 FCST: FX RATE & FX SENSITIVITY

## Average rate for the period

| Currency | FY2020  | FY2021 FCST | change |
|----------|---------|-------------|--------|
| USD      | 106 yen | 110 yen     | +4 yen |
| EUR      | 124 yen | 130 yen     | +6 yen |

## Change in closing rate from the previous FY end

| Currency | FY2020  | FY2021 FCST |
|----------|---------|-------------|
| USD      | +2 yen  | -1 yen      |
| EUR      | +10 yen | +0 yen      |

## Estimated FX sensitivity of FY2021 forecast by 1 yen appreciation

| Currency | Average rate<br>1 yen higher than assumption |                       | Year-end rate<br>1 yen higher than<br>assumption |
|----------|--|-----------------------|--|
|          | Revenue                                      | Core OP               | Core OP  |
| USD      | Approx. -6.3 bil. yen                        | Approx. -1.3 bil. yen | Approx. +0.6 bil. yen                            |
| EUR      | Approx. -2.9 bil. yen                        | Approx. -1.4 bil. yen | Approx. +0.3 bil. yen                            |

# BALANCE SHEET & CASH FLOW HIGHLIGHTS

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| (billion yen)                                     | FY20 end | Jun 30, 2021 |
|---|----------|--------------|
| Total assets                                      | 2,273.6  | 2,249.5      |
| Cash and cash equivalents                         | 326.1    | 301.9        |
| Total equity attributable to owners of the parent | 1,386.1  | 1,382.9      |
| Equity ratio (%)                                  | 61.0%    | 61.5%        |

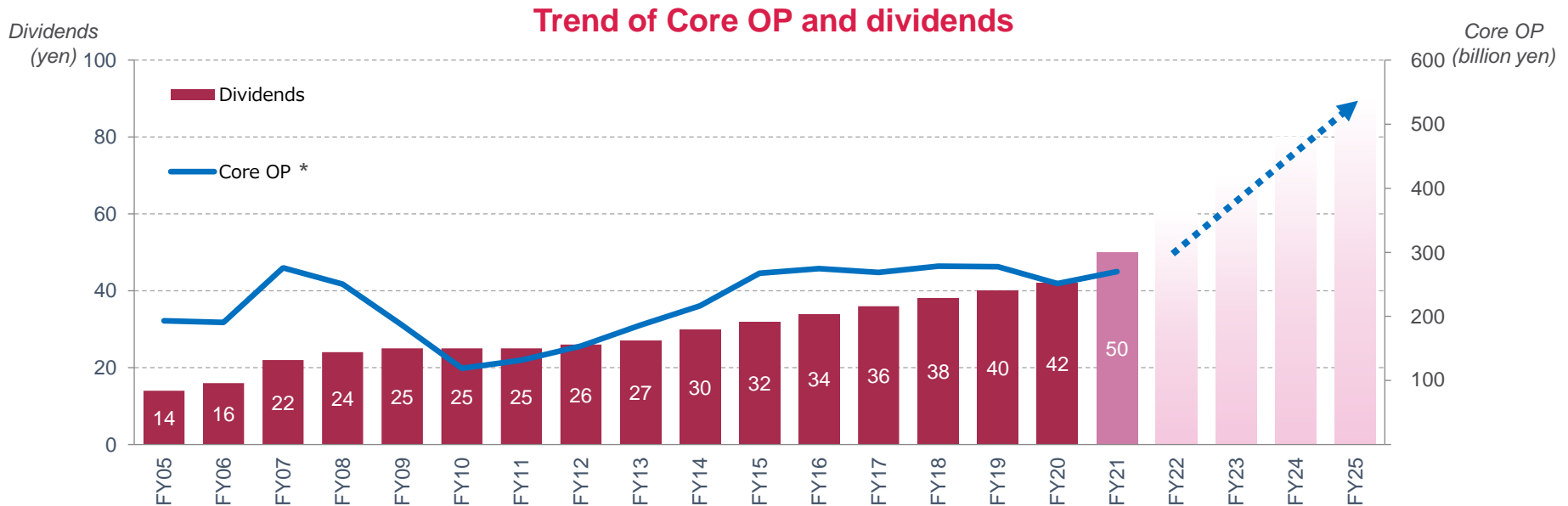
| (billion yen)                        | Q1/FY20 | Q1/FY21 | FY20   |
|--------------------------------------|---------|---------|--------|
| Cash flows from operating activities | 21.6    | 40.1    | 306.8  |
| Cash flows from investing activities | -28.3   | -21.1   | -81.9  |
| Free cash flows                      | -6.7    | 19.0    | 224.9  |
| Cash flows from financing activities | -73.0   | -44.7   | -229.5 |
| Bonds and short-term borrowings      | -110.0  | -       | -206.0 |
| Proceeds from long-term borrowings   | 80.0    | -       | 80.0   |
| Dividends paid                       | -37.2   | -38.9   | -76.2  |

Balance of bonds and borrowings : 200.0 billion yen  
(No changes from FY2020 end)

# 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



\* Prior to FY2012, operating profit is in accordance with J-GAAP  
 CSP: Corporate Strategic Plan

# ROBUST PIPELINE OF ASTELLAS

30

## Phase 1

enfortumab vedotin

(NMIBC)

ASP1948

ASP1951

ASP9801

ASP7517

ASP0739

ASP7317

ASP0367

(Duchenne muscular dystrophy)

AT845

ASP0598

ASP2390

ASP1570

ASP8062

(Alcohol use disorder)

ASP1617

## Phase 2

enfortumab vedotin

(Other solid tumors)

zolbetuximab

(Pancreatic adenocarcinoma)

roxadustat

(Chemotherapy-induced anemia)

resamirigene bilparovvec

/AT132 (XLMTM)

ASP1128

(Acute kidney injury)

ASP0367

(Primary mitochondrial myopathies)

ASP3772

(Pneumococcal disease)

FX-322

(Sensorineural hearing loss)

isavuconazole

(Pediatric use: US)

ASP8062

(Opioid use disorder)

## Phase 3

enzalutamide

(M0 CSPC, M1 CSPC: China)

gilteritinib

(Earlier-stage AML, Pediatric use)

enfortumab vedotin

(mUC previously untreated, MIBC)

zolbetuximab

(Gastric and GEJ adenocarcinoma)

fezolinetant

(VMS associated with menopause)

peficitinib

(Rheumatoid arthritis: China)

mirabegron

(Pediatric use: EU)

## Filed

enfortumab vedotin

(mUC, pretreated: EU, JP)

roxadustat

(Anemia associated with CKD: EU)

■ XTANDI and Strategic products  
(XOSPATA, PADCEV, zolbetuximab, Evrenzo, fezolinetant, AT132)

■ Projects with Focus Area approach ■ Others

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

The listed compounds are investigational agents the safety and efficacy of which has not yet been established. There is no guarantee that the agents will receive regulatory approval or become commercially available for uses being investigated



NMIBC: Non-muscle-invasive bladder cancer, XLMTM: X-linked myotubular myopathy, M0: Non-metastatic, M1: Metastatic, CSPC: Castration-sensitive prostate cancer, AML: Acute myeloid leukemia, mUC: Metastatic urothelial cancer, MIBC: Muscle-invasive bladder cancer, GEJ: Gastroesophageal junction, VMS: Vasomotor symptoms, CKD: Chronic kidney disease

# PROGRESS IN OVERALL PIPELINE

Phase 1 Entry to Approval since FY2020 Financial Results Announcement in Apr 2021

31

Phase 1 Entry

Phase 2 Entry

Phase 3 Entry

Filing

Approval

## enfortumab vedotin

Non-muscle-invasive  
bladder cancer

## ASP1570

Cancer

## enzalutamide

Metastatic hormone-sensitive  
prostate cancer:  
EU

## enfortumab vedotin

Locally advanced or metastatic  
urothelial cancer, cisplatin-  
ineligible and who have previously  
received one or more therapy:  
US

## tacrolimus

Prevention of organ rejection  
in adult and pediatric patients  
receiving lung transplantation:  
US

## Discontinuation

**bleselumab:** Recurrence of focal segmental glomerulosclerosis in *de novo* kidney transplant recipients (Phase 2)

**ASP0892:** Peanut allergy (Phase 1)

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

# XTANDI & STRATEGIC PRODUCTS\*: STATUS UPDATE

(Underlined: Updates since FY2020 Financial Results Announcement in Apr 2021)

32

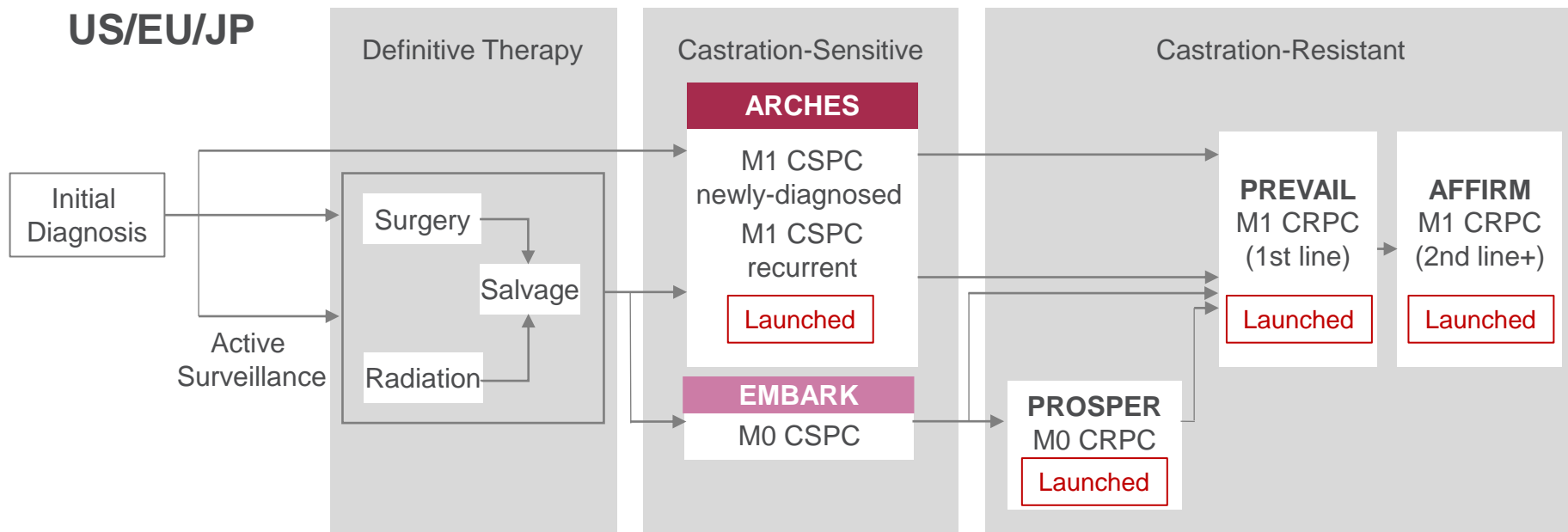
\* XOSPATA, PADCEV, zolbetuximab, Evrenzo, fezolinetant, AT132

|   | Indication                                | Current status   |
|---|---|--|
| <b>enzalutamide / XTANDI</b>            | M1 CSPC                                   | <ul style="list-style-type: none"> <li><b>EU:</b> <u>Approved in Apr 2021</u></li> <li><b>China:</b> Phase 3 study ongoing (enrollment completed)</li> </ul>   |
|   | M0 CSPC                                   | <ul style="list-style-type: none"> <li>Phase 3 study ongoing (enrollment completed)</li> </ul>   |
| <b>gilteritinib / XOSPATA</b>           | Relapsed and refractory AML               | <ul style="list-style-type: none"> <li><b>China:</b> Phase 3 study stopped due to efficacy</li> </ul>  |
|   | AML, post-HSCT maintenance                | <ul style="list-style-type: none"> <li>Phase 3 study ongoing (enrollment completed)</li> </ul>   |
|   | AML, newly diagnosed (HIC-eligible)       | <ul style="list-style-type: none"> <li>Phase 3 study ongoing</li> </ul>  |
| <b>enfortumab vedotin / PADCEV</b>      | Metastatic urothelial cancer              | <ul style="list-style-type: none"> <li><b>Pretreated:</b> <u>Approved (2 sBLAs) in US in Jul 2021</u>. Filed in EU and JP in Mar 2021</li> <li><b>Previously untreated (first line):</b> Phase 3 study ongoing</li> <li><b>China:</b> Phase 2 bridging study under preparation <u>to start in Q2 FY2021</u></li> </ul>   |
|   | Muscle-invasive bladder cancer            | <ul style="list-style-type: none"> <li>Phase 3 studies ongoing (<u>FSFT in Phase 3 study in cisplatin-eligible in May 2021</u>)</li> </ul>   |
|   | <u>Non-muscle-invasive bladder cancer</u> | <ul style="list-style-type: none"> <li><u>Phase 1 study with intravesical therapy under preparation to start Q2 FY2021</u></li> </ul>  |
|   | Other solid tumors                        | <ul style="list-style-type: none"> <li>Phase 2 study ongoing</li> </ul>  |
| <b>zolbetuximab</b>                     | Gastric & GEJ adenocarcinoma              | <ul style="list-style-type: none"> <li>Phase 3 studies ongoing</li> </ul>  |
|   | Pancreatic adenocarcinoma                 | <ul style="list-style-type: none"> <li>Phase 2 study ongoing</li> </ul>  |
| <b>roxadustat / Evrenzo</b>             | Anemia associated with CKD                | <ul style="list-style-type: none"> <li><b>EU:</b> <u>CHMP positive opinion received in Jun 2021</u></li> </ul>   |
|   | Chemotherapy-induced anemia               | <ul style="list-style-type: none"> <li>Phase 2 study ongoing (enrollment completed)</li> </ul>   |
| <b>fezolinetant</b>                     | VMS associated with menopause             | <ul style="list-style-type: none"> <li><b>US &amp; EU:</b> Phase 3 studies ongoing (enrollment completed). Primary endpoints (12w DB period topline results) met in both Phase 3 pivotal studies, SKYLIGHT 1 and 2. <u>Obtained 52w data of SKYLIGHT 2 in Jul 2021</u></li> <li><b>Asia:</b> Phase 3 studies ongoing (<u>enrollment completed in long-term study</u>)</li> <li><b>Japan:</b> Phase 2b study under preparation to start in Q3 FY2021</li> </ul> |
| <b>AT132 (resamirigene bilparvovec)</b> | X-linked myotubular myopathy              | <ul style="list-style-type: none"> <li><u>ASPIRO study resumed in Jul 2021</u></li> <li><u>Planning to include 3 additional patients (6 new patients in total) at the lower dose</u></li> </ul>  |

M1: Metastatic, M0: Non-metastatic, CSPC: Castration-sensitive prostate cancer, AML: Acute myeloid leukemia, HSCT: Hematopoietic stem cell transplant, HIC: High-intensity chemotherapy, sBLA: Supplemental Biologics License Application, FSFT: First subject first treatment, GEJ: Gastroesophageal junction, CKD: Chronic kidney disease, CHMP: Committee for Medicinal Products for Human Use, VMS: Vasomotor symptoms, DB: Double-blind



# ENZALUTAMIDE: ANDROGEN RECEPTOR INHIBITOR (1/2)



|                   |                |                             |         |   |
|-------------------|----------------|-----------------------------|---------|---|
| <b>P3: ARCHES</b> | <b>M1 CSPC</b> | Combo with ADT, vs. placebo | n=1,150 | Approved in US in Dec 2019, in JP in May 2020, and <u>in EU in Apr 2021</u> |
| <b>P3: EMBARK</b> | <b>M0 CSPC</b> | Combo with ADT, vs. placebo | n=1,068 | Enrollment completed  |

**China** • **M1 CSPC**: Enrollment completed in Phase 3 China-ARCHES study



Underlined: Updates since FY2020 financial results announcement in Apr 2021

M1: Metastatic, M0: Non-metastatic, CSPC: Castration-sensitive prostate cancer, CRPC: Castration-resistant prostate cancer, ADT: Androgen deprivation therapy

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

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

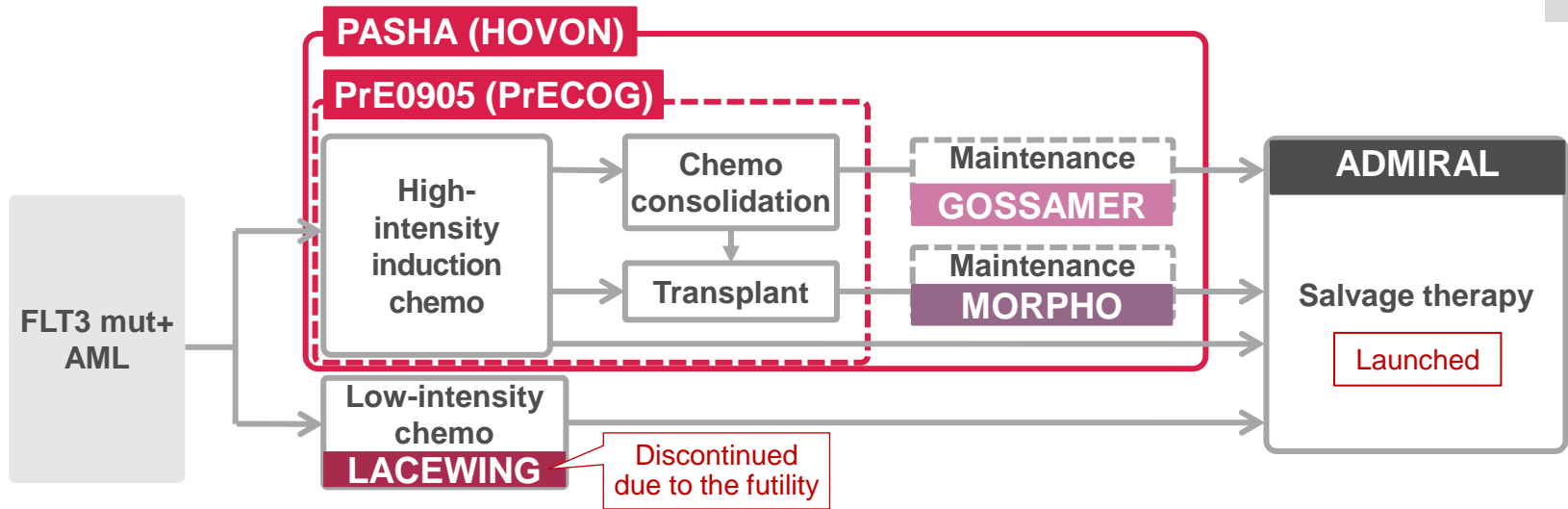
| Disease stage    | Early stage                 |                   |                   | Late stage                  |                                       |                 |
|------------------|-----------------------------|-------------------|-------------------|-----------------------------|---------------------------------------|-----------------|
|                  | Castration-sensitive (CSPC) |                   |                   | Castration-resistant (CRPC) |                                       |                 |
|                  | M0                          | M1                |                   | M0                          | M1 (pre-chemo)                        | M1 (post-chemo) |
| Phase 3 study    | <b>EMBARK</b>               | <b>ARCHES</b>     | <b>ENZAMET</b>    | <b>PROSPER</b>              | <b>PREVAIL</b>                        | <b>AFFIRM</b>   |
| Control          | Placebo                     | Placebo           | Conventional NSAA | Placebo                     | Placebo                               | Placebo         |
| Primary endpoint | MFS (Ongoing)               | ✓ rPFS<br>HR 0.39 | ✓ OS<br>HR 0.67   | ✓ MFS<br>HR 0.29            | ✓ rPFS<br>HR 0.17<br>✓ OS<br>HR 0.71* | ✓ OS<br>HR 0.63 |
| OS               | (Ongoing)                   | (Not reached)     | ✓<br>HR 0.67      | ✓<br>HR 0.73                | ✓<br>HR 0.77                          | ✓<br>HR 0.63    |
| DoT              | (Ongoing)                   | (Not reached)     | ✓<br>29.5 months  | ✓<br>33.9 months            | ✓<br>17.5 months                      | ✓<br>8.3 months |

✓: Data obtained, \*: Prespecified interim analysis



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

# GILTERITINIB: FLT3 INHIBITOR



|                                  |                             |  |       |  |
|----------------------------------|-----------------------------|--|-------|--|
| Relapsed or refractory (R/R)     | <b>P3: ADMIRAL</b>          | Monotherapy vs salvage chemo (2:1)                                 | n=371 | Launched in US, JP, and EU   |
| Newly diagnosed (HIC-eligible)   | <b>P3: PASHA (HOVON)</b>    | Combo with high intensity chemo gilteritinib vs. midostaurin (1:1) | n=768 | FSFT: Dec 2019 (Sponsor: HOVON)  |
|                                  | <b>P2: PrE0905 (PrECOG)</b> |  | n=179 | FSFT: Dec 2019 (Sponsor: PrECOG, LLC.)                                 |
| Newly diagnosed (HIC-ineligible) | <b>P3: LACEWING</b>         | Combo with azacitidine vs. azacitidine alone (2:1)                 | n=146 | Discontinued due to the futility based on the planned interim analysis |
| Post-HSCT maintenance            | <b>P3: MORPHO</b>           | Monotherapy vs. placebo (1:1)                                      | n=346 | Enrollment completed<br>Collaborating with BMT-CTN                     |
| Post-chemo maintenance           | <b>P2: GOSSAMER</b>         | Monotherapy vs. placebo (2:1)                                      | n=98  | Enrollment completed   |

- 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



# ENFORTUMAB VEDOTIN (EV): NECTIN-4 TARGETED ADC (1/2) CLINICAL STUDIES

36

## For urothelial cancer

|                                    |   |       |  |
|------------------------------------|---|-------|--|
| <b>P3: EV-301</b>                  | mUC, Platinum and PD-1/L1 inhibitor pretreated;<br>EV mono vs. Chemo  | n=608 | <u>sBLA (to convert regular approval) approved in US in Jul 2021. Filed in EU and JP in Mar 2021</u>   |
| <b>P3: EV-302</b>                  | mUC, Previously untreated, Platinum-eligible;<br>EV + Pembro vs. Chemo  | n=760 | FSFT: Apr 2020   |
| <b>P3: EV-303<br/>/KEYNOTE-905</b> | MIBC, Cis-ineligible;<br>Pembro +/- EV (perioperative) + RC vs. RC alone  | n=836 | FSFT in Pembro + EV arm: Dec 2020  |
| <b>P3: EV-304<br/>/KEYNOTE-B15</b> | MIBC, Cis-eligible; EV+Pembro (perioperative) + RC<br>vs. Chemo (neoadjuvant) + RC  | n=784 | <u>FSFT: May 2021</u>  |
| <b>P2: EV-201</b>                  | 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) and launched in US in Dec 2019<br>Cohort 2: <u>sBLA approved in US in Jul 2021</u>   |
| <b>P1b/2: EV-103</b>               | Cohorts A - G and K (mUC):<br>A-G: Combo with Pembro and other chemo<br>K: EV mono vs. 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=457 | Enrollment ongoing in Cohort K and L<br><br>Note) Data from Cohort K along with other cohorts evaluating EV + Pembro as first-line therapy for cis-ineligible patients could potentially support registration for Accelerated Approval in US |
| <b>P2: EV-203</b>                  | <Bridging study in China><br>mUC, Platinum and PD-1/L1 inhibitor pretreated; EV mono  | n=40  | <u>To start in Q2 FY2021 (IND approved)</u>  |
| <b>P1: EV-104</b>                  | <u>NMIBC, High-risk BCG-unresponsive; Intravesical EV mono</u>  |       | <u>To start in Q2 FY2021</u>   |

## For other solid tumors

|                   |  |       |                |
|-------------------|--|-------|----------------|
| <b>P2: EV-202</b> | HR+/HER2- breast cancer, Triple-negative breast cancer, Squamous NSCLC, Non-squamous NSCLC, Head and neck cancer, Gastric, gastroesophageal junction or esophageal cancer; EV mono | n=240 | FSFT: Mar 2020 |
|-------------------|--|-------|----------------|



Underlined: Updates since FY2020 financial results announcement in Apr 2021

mUC: Metastatic urothelial cancer, mono: Monotherapy, Chemo: Chemotherapy, sBLA: Supplemental Biologics License Application, Pembro: Pembrolizumab, FSFT: First subject first treatment, Cis: Cisplatin, MIBC: Muscle-invasive bladder cancer, RC: Radical cystectomy, IND: Investigational New Drug application, NMIBC: Non-muscle-invasive bladder cancer, BCG: Bacillus Calmette-Guerin, HR+: Hormone receptor positive, HER2-: HER2 negative, NSCLC: Non-small cell lung cancer

# ENFORTUMAB VEDOTIN (EV) (2/2): STUDY DATA BY DISEASE STAGE OF UC

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| 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 and 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.        | <b>KN-B15 / EV-304</b>          | <b>KN-905 / EV-303</b>          | <b>EV-302</b>                     | <b>EV-103 Cohort K</b>   | <b>EV-103 Cohort A &amp; Others</b> | <b>EV-201 Cohort 2</b>            | <b>EV-201 Cohort 1</b> | <b>EV-301</b>                      |
| No. of subjects  | 784 (2 arms)                    | 836 (3 arms)                    | 760 (2 arms)                      | 150 (2 arms)             | 45                                  | 89                                | 125                    | 608 (2 arms)                       |
| EV regimen       | Combo w/ Pembro (perioperative) | Combo w/ Pembro (perioperative) | Combo w/ Pembro                   | Mono vs. Combo w/ Pembro | Combo w/ Pembro                     | Mono                              | Mono                   | Mono                               |
| Control          | Chemo (neoadjuvant)             | SoC                             | Chemo                             | n/a                      | n/a                                 | n/a                               | n/a                    | Chemo                              |
| Primary endpoint | pCR & EFS                       | pCR & EFS                       | PFS & OS                          | ORR                      | ✓ ORR 73% ** (CR 16% **)            | ✓ ORR 51% ** (CR 22% **)          | ✓ ORR 44% (CR 12%)     | ✓ OS HR 0.70 *                     |
| OS               | (Ongoing)                       | (Ongoing)                       | (Ongoing)                         | (Ongoing)                | ✓ (26.1 mos **)                     | ✓ (14.7 mos)                      | ✓ (12.4 mos **)        | ✓ HR 0.70 * (12.9 mos vs. 9 mos)   |
| PFS              | (Ongoing)                       | (Ongoing)                       | (Ongoing)                         | (Ongoing)                | ✓ (12.3 mos **)                     | ✓ (5.8 mos)                       | ✓ (5.8 mos)            | ✓ HR 0.62 * (5.6 mos vs. 3.7 mos)  |
| ORR              | (Ongoing)                       | (Ongoing)                       | (Ongoing)                         | (Ongoing)                | ✓ 73% ** (CR 16% **)                | ✓ 52% (CR 20%)                    | ✓ 44% (CR 12%)         | ✓ 41% vs. 18% * (CR 4.9% vs. 2.7%) |
| DoR              | (Ongoing)                       | (Ongoing)                       | (Ongoing)                         | (Ongoing)                | ✓ 25.6 mos **                       | ✓ 13.8 mos **                     | ✓ 7.6 mos              | ✓ 7.39 mos vs. 8.11 mos *          |



✓: Data obtained, \*: Prespecified interim analysis, \*\*: Updated data, Yellow: Data recently disclosed



(m)UC: (Metastatic) urothelial cancer, MIBC: Muscle-invasive bladder cancer, Pembro: Pembrolizumab, mono: Monotherapy, Chemo: Chemotherapy, pCR: Pathologic complete response, 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

# ZOLBETUXIMAB: ANTI-CLAUDIN 18.2 MONOCLONAL ANTIBODY

## Target: Claudin 18.2

- Claudin is a major structural component of tight junctions and seals intercellular space in epithelial sheets
- Broadly expressed in various cancer types
  - ✓ Prevalence of patients with high expression of Claudin 18.2 is substantial: 33% - 37%
  - ✓ ~60% of primary pancreatic adenocarcinomas; approx. 20% of these meet the eligibility criteria for the ongoing Phase 2 study

## Gastric and (GEJ) adenocarcinoma

- Target patient population: HER2-, Claudin 18.2+ locally advanced and metastatic gastric and GEJ adenocarcinoma
- Metastatic gastric cancer is an area of significant unmet need, especially in advanced stages with ~4% five-year survival rate at Stage IV and limited treatment options have been limited

|                                |               |  |              |                |
|--------------------------------|---------------|--|--------------|----------------|
| Gastric and GEJ adenocarcinoma | P3: SPOTLIGHT | First line, Combo with mFOLFOX6, vs. placebo   | n=550        | FSFT: Oct 2018 |
|                                | P3: GLOW      | First line, Combo with CAPOX, vs. placebo  | n=500        | FSFT: Jan 2019 |
|                                | P2: ILUSTRO   | 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 | <u>n=116</u> | FSFT: Sep 2018 |
| Pancreatic adenocarcinoma      | P2            | Combo with nab-paclitaxel and gemcitabine, vs. placebo   | n=141        | FSFT: May 2019 |

# 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 <sup>1</sup>

## Women's Health Initiative (WHI) Study <sup>2</sup>

- Initial data analyses showed an association between chronic HRT use and increased risk of cardiovascular disease and cancer
- Since WHI's findings, no replacement for HRT with similar efficacy and no significant safety concern, resulting in significant unmet medical needs

## US and EU

|                       |  |         |   |
|-----------------------|--|---------|---|
| <b>P3: SKYLIGHT 1</b> | Moderate to severe VMS associated with menopause;<br>The first 12 weeks: DB, 30 mg and 45 mg vs. placebo (1:1:1)<br>The last 40 weeks: Active extension treatment period, 30 mg or 45 mg | n=527   | Primary endpoints met (12w DB period topline results)   |
| <b>P3: SKYLIGHT 2</b> |  | n=501   | Primary endpoints met (12w DB period topline results)<br><u>Obtained 52w data in Jul 2021</u> |
| <b>P3: SKYLIGHT 4</b> | VMS associated with menopause;<br>52 weeks: DB, 30 mg and 45 mg vs. placebo (1:1:1)  | n=1,833 | Enrollment completed  |

## Asia (except for Japan)

|                        |   |       |                             |
|------------------------|---|-------|-----------------------------|
| <b>P3: MOONLIGHT 1</b> | 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=300 | FSFT: Apr 2020              |
| <b>P3: MOONLIGHT 3</b> | VMS associated with menopause; open label, 30 mg for 52 weeks   | n=150 | <u>Enrollment completed</u> |

**Japan** • Phase 2b dose-finding study in Japanese patients under preparation to start in Q3 FY2021



Underlined: Updates since FY2020 financial results announcement in Apr 2021

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

# AT132 (RESAMIRIGENE BILPARVOVEC): rAAV8-Des-hMTM1

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## Characteristics of AT132

- Lead program in the gene therapy pipeline of Audentes Therapeutics, acquired by Astellas in Jan 2020
- Designed to deliver a functional copy of human MTM1 gene by AAV8 to transfect and express myotubularin in skeletal muscle cells
- Regulatory designations granted:
  - ✓ <US> RMAT, Rare Pediatric Disease, Fast Track, and Orphan Drug designations
  - ✓ <EU> PRIME and Orphan Drug designations

## X-linked myotubular myopathy (XLMTM)

- Rare neuromuscular disease with X-linked, loss of function mutations in MTM1 gene
  - ✓ Approximately 1 in 40,000 to 50,000 newborn males
  - ✓ Estimated 50% mortality by 18 months
  - ✓ Up to 24 hours of invasive mechanical ventilation, 60% of patients require tracheostomy
  - ✓ > 80% require gastrostomy tube placement
  - ✓ Motor milestones substantially delayed
  - ✓ No treatment available; supportive care only

**ASPIRO**  
**(clinical study for registration**  
**in XLMTM patients)**

n=26

Dosing (lower dose:  $1.3 \times 10^{14}$  vg/kg) resumed in Jul 2021

Planning to include 3 additional patients (6 new patients in total) at the lower dose



Underlined: Updates since FY2020 financial results announcement in Apr 2021

(r)AAV: (recombinant) Adeno-associated virus, Des: Desmin promoter, hMTM1: Human myotubularin gene, RMAT: Regenerative Medicine Advanced Therapy, PRIME: PRiority MeDicines, vg: Vector genome



# ON THE FOREFRONT OF HEALTHCARE CHANGE

