

Q1/FY2021 FINANCIAL RESULTS

ENDED JUNE 30, 2021



Naoki Okamura
Executive Vice President,
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.

Information about pharmaceutical products (including products currently in development) which is included in this material is not intended to constitute an advertisement or medical advice.

AGENDA

I

Q1/FY2021 Consolidated Financial Results
FY2021 Revised Forecasts

II

Initiatives for Sustainable Growth

Q1/FY2021 FINANCIAL RESULTS

4

(billion yen)	Q1/FY20	Q1/FY21	Change	Change (%)	FY21 FCST	Progress	FX impact
Revenue	307.0	326.1	+19.2	+6.2%	1,323.0	24.7%	+13.6 bil. yen
Cost of sales	59.7	62.2	+2.6	+4.3%			
% of revenue	19.4%	19.1%	-0.4 ppt				
SG&A expenses	120.8	137.1	+16.3	+13.5%	541.0	25.3%	
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%			
R&D expenses	57.3	58.3	+1.0	+1.8%	242.0	24.1%	
Amortisation of intangible assets	5.9	6.0	+0.1	+1.8%			
Core operating profit	63.4	62.8	-0.6	-0.9%	270.0	23.3%	+6.1 bil. yen

<Full basis>

Other income	2.2	0.4	-1.8	-			
Other expense	4.8	27.1	+22.3	-			
Operating profit	60.8	36.1	-24.7	-40.7%	265.0	13.6%	
Profit before tax	60.2	35.8	-24.4	-40.5%	263.0	13.6%	
Profit	50.4	30.7	-19.7	-39.1%	209.0	14.7%	

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

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 (%)
Revenue	307.0 bil. yen	326.1 bil. yen	+19.2 bil. yen	+6.2%

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**



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 (Disclosed in Apr 2021)	Revised Forecast	Change
Operating profit	265.0	227.0	-38.0
Profit	209.0	183.0	-26.0

AGENDA

I

Q1/FY2021 Consolidated Financial Results
FY2021 Revised Forecasts

II

Initiatives for Sustainable Growth

XTANDI & STRATEGIC PRODUCTS*: HIGHLIGHT

11

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 ^{a,b})	Jul 2021
		mUC, cis-ineligible and who have previously received one or more therapy (US ^a)	Jul 2021
		mUC, platinum and PD-1/L1 inhibitor pretreated (EU ^c)	
		mUC, progressed after anti-cancer medication (JP ^d)	
	roxadustat /Evrenzo	Anemia associated with CKD (EU)	CHMP positive opinion received in Jun 2021
Regulatory submission	gilteritinib /XOSPATA	R/R AML (China ^e)	
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

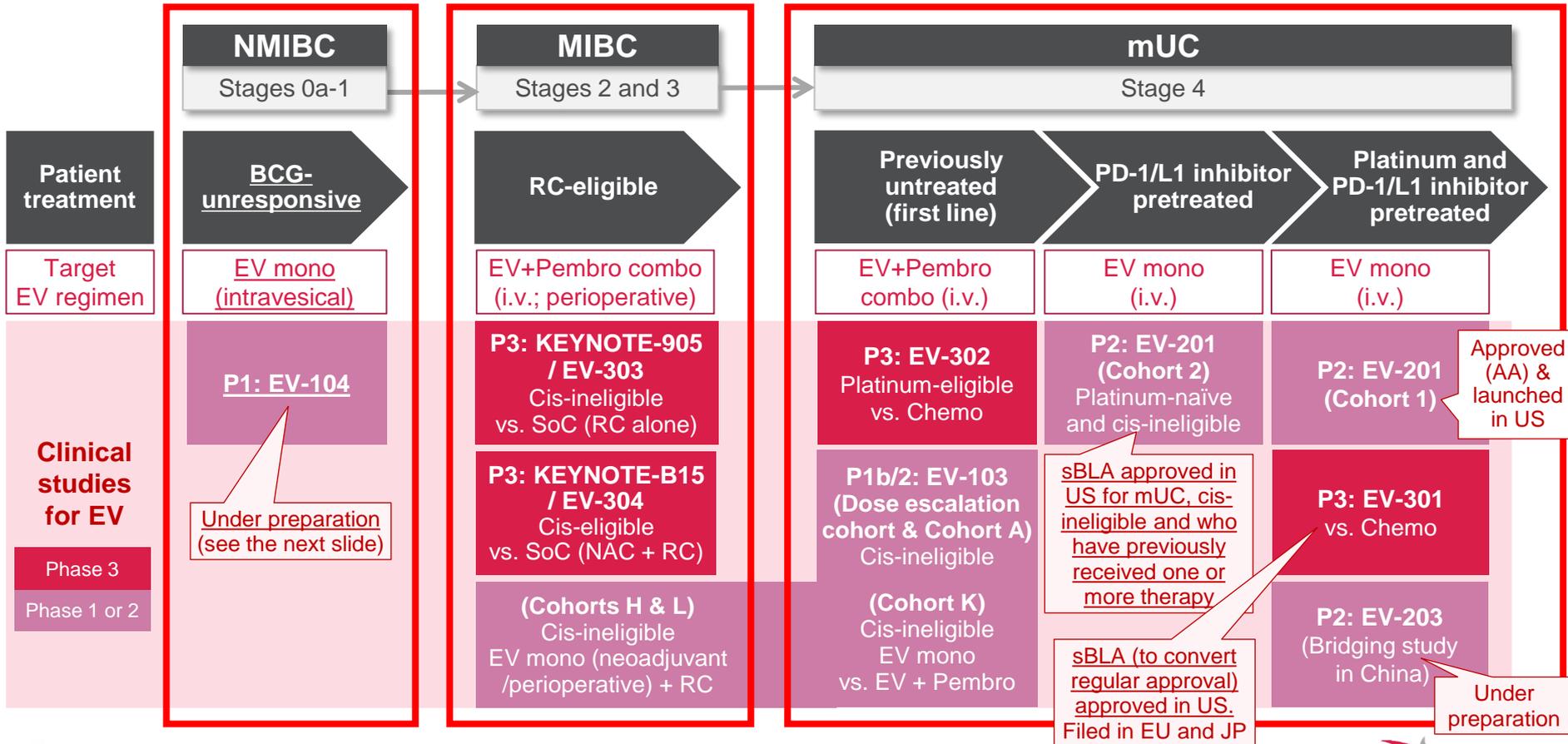
ENFORTUMAB VEDOTIN (EV) (1/2): OVERALL UC PROGRAM

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

Early stage

- Disease stage of urothelial cancer -

Late stage

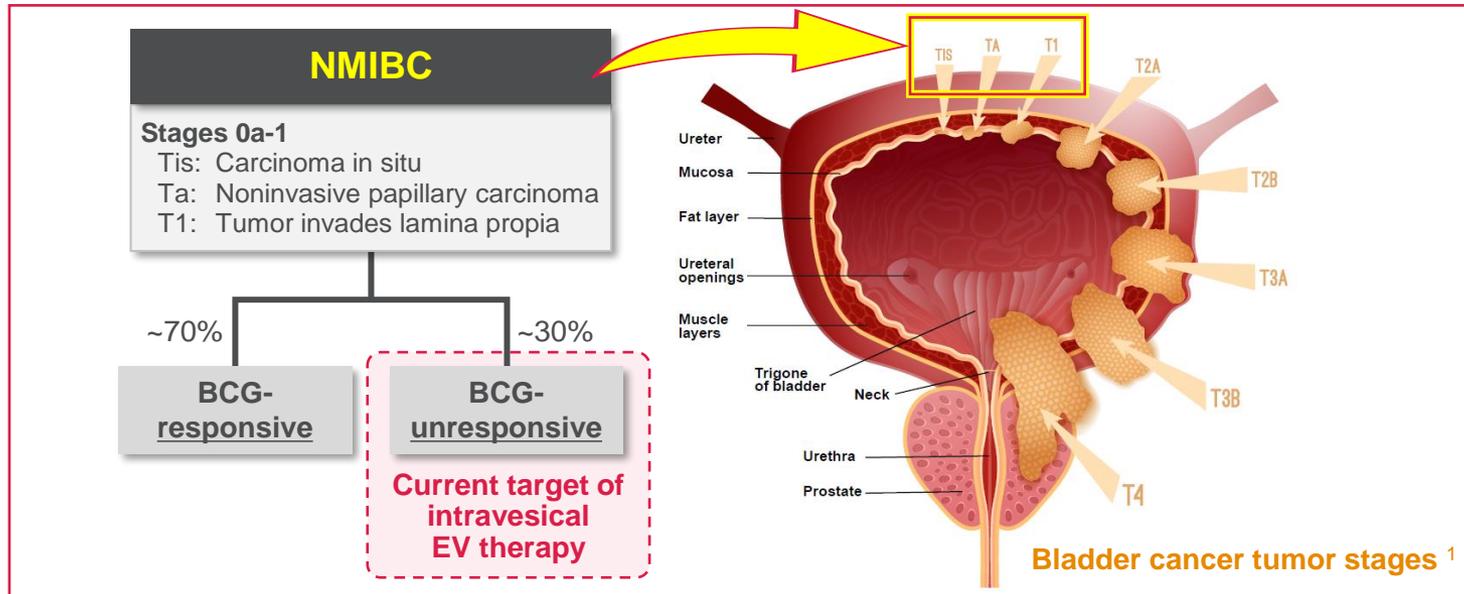


Underlined: Updates since FY2020 financial results announcement in Apr 2021

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

14

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 (2/3): CURRENT STATUS IN PRIMARY FOCUS

16

Primary Focus	Biology/Modality/Technology ¹	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 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> 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
 Small molecule
 Antibody
 Gene
 Cell
 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

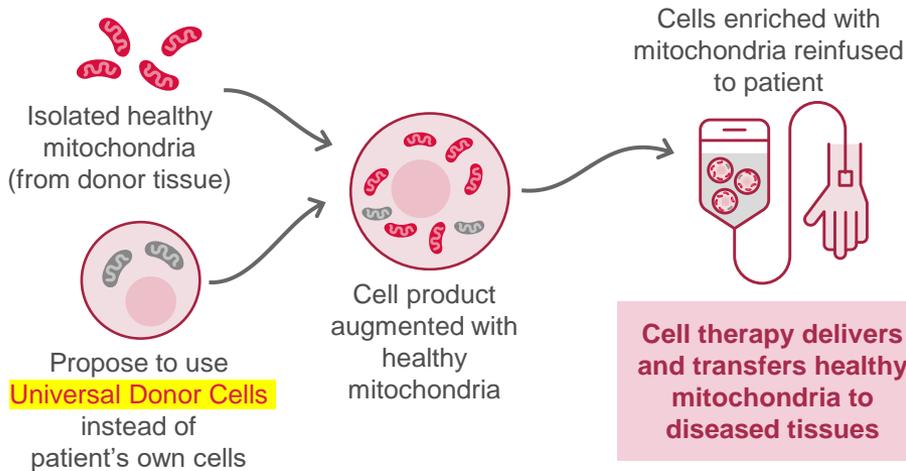
PROGRESS IN FOCUS AREA APPROACH (3/3): MITOCHONDRIA BIOLOGY

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

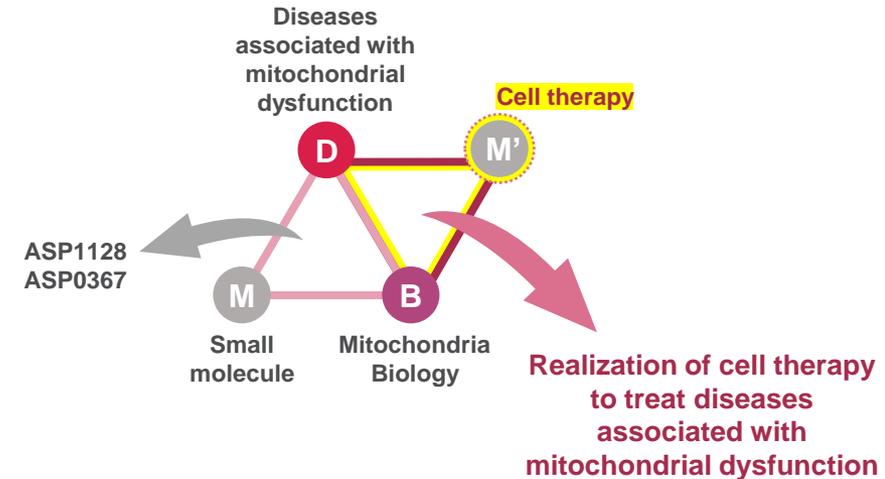
MAT platform from Minovia Therapeutics

- 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

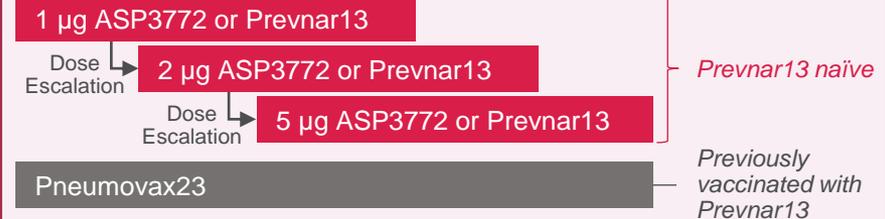
ASP3772 profiles

- 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 ≥ 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



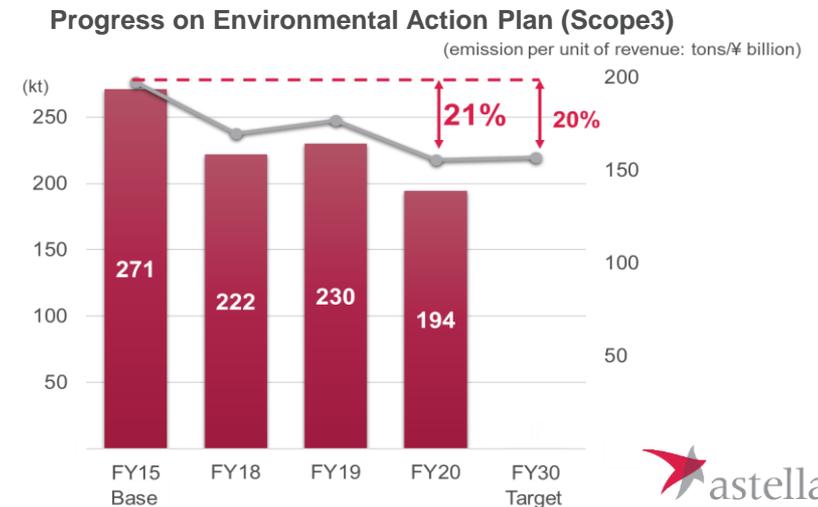
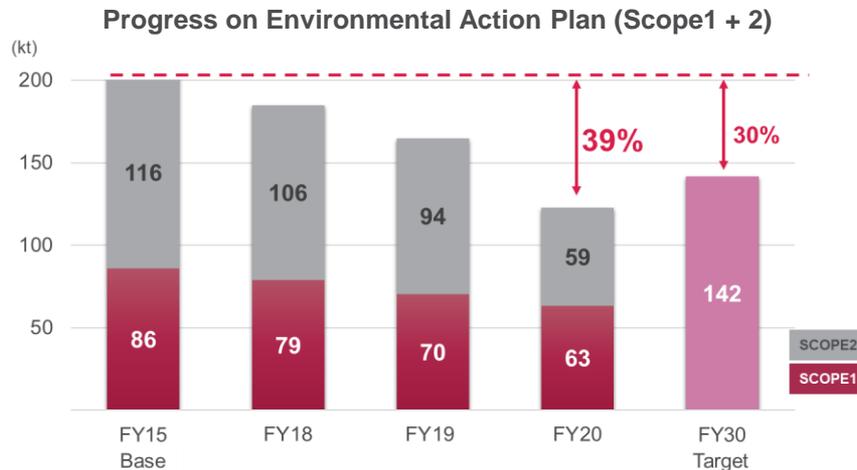
Reduction of GHG emissions is moving forward as the Environmental Action Plan progresses

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

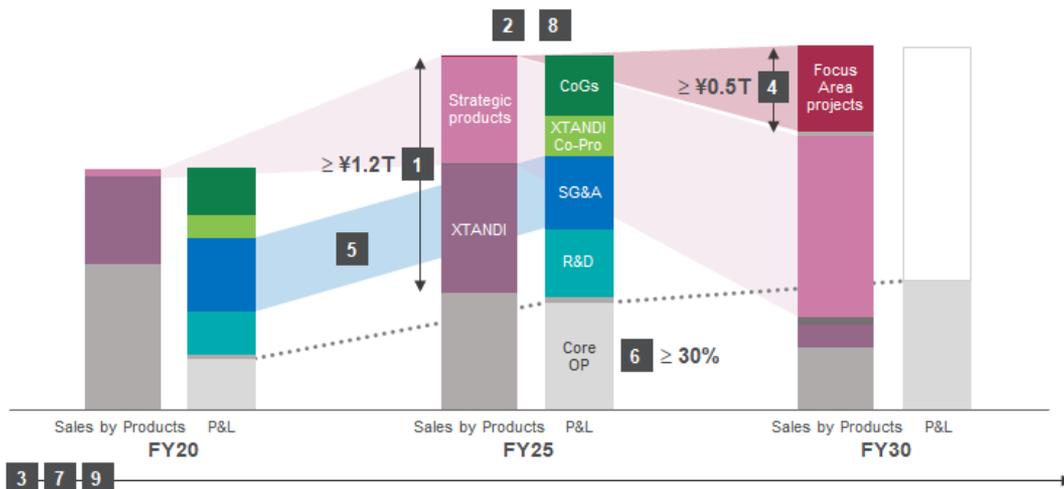
- 2** Post-PoC projects from Primary Focuses
 - ✓ AT132: Dosing in clinical study resumed
- 3** Multiple technology platforms
 - ✓ ASP0367: FSFT in Phase 2/3 study
- 4** Focus Area projects: \geq ¥0.5T in FY2030
 - ✓ Collaboration with Minovia

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

A water droplet is captured mid-fall, just above the surface of a pool of water. The droplet is clear and spherical, with a slight reflection on its top. Below it, the water surface is disturbed, creating several concentric ripples that spread outwards. The background is a light gray gradient, with a large, dark gray and red geometric shape on the right side. The word "APPENDIX" is written 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 (%)
Japan	77.8	67.5	-13.2%
United States	117.2	133.6	+14.1%
Established Markets	64.0	78.0	+21.8%
Greater China	14.2	16.4	+15.5%
International Markets	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

(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

Q1/FY2021 FINANCIAL RESULTS: BUSINESS UPDATE FOR MAIN PRODUCTS

25

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 1 yen higher than assumption		Year-end rate 1 yen higher than 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

(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%

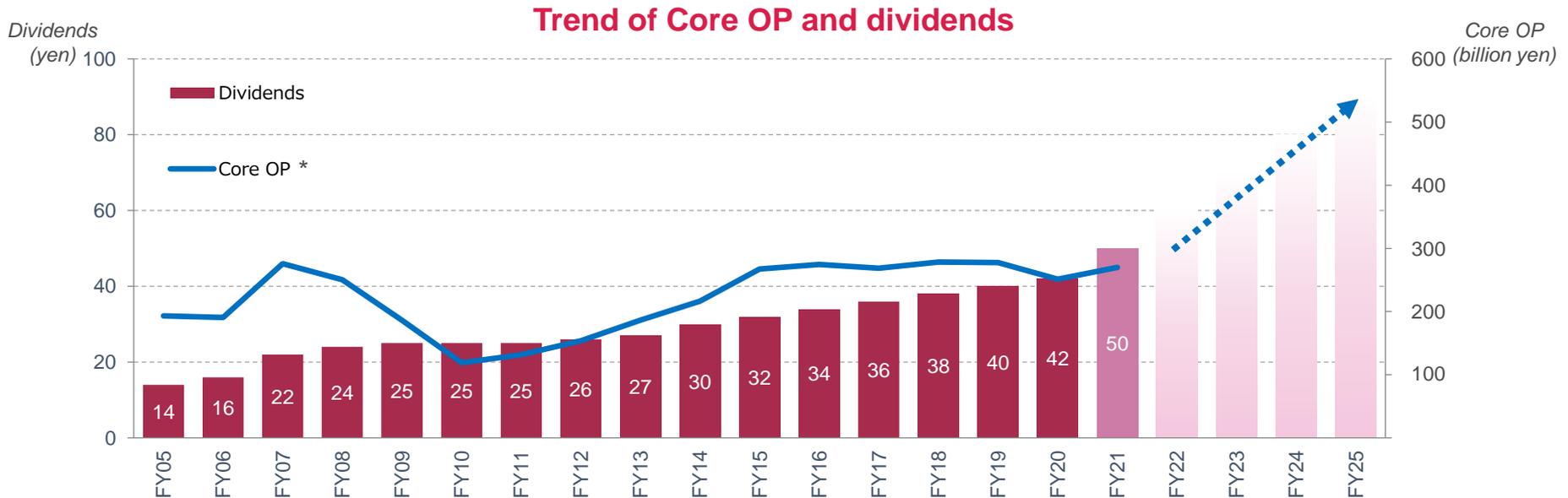
(billion yen)	Q1/FY20	Q1/FY21	FY20
Cash flows from operating activities	21.6	40.1	306.8
Cash flows from investing activities	-28.3	-21.1	-81.9
Free cash flows	-6.7	19.0	224.9
Cash flows from financing activities	-73.0	-44.7	-229.5
Bonds and short-term borrowings	-110.0	-	-206.0
Proceeds from long-term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

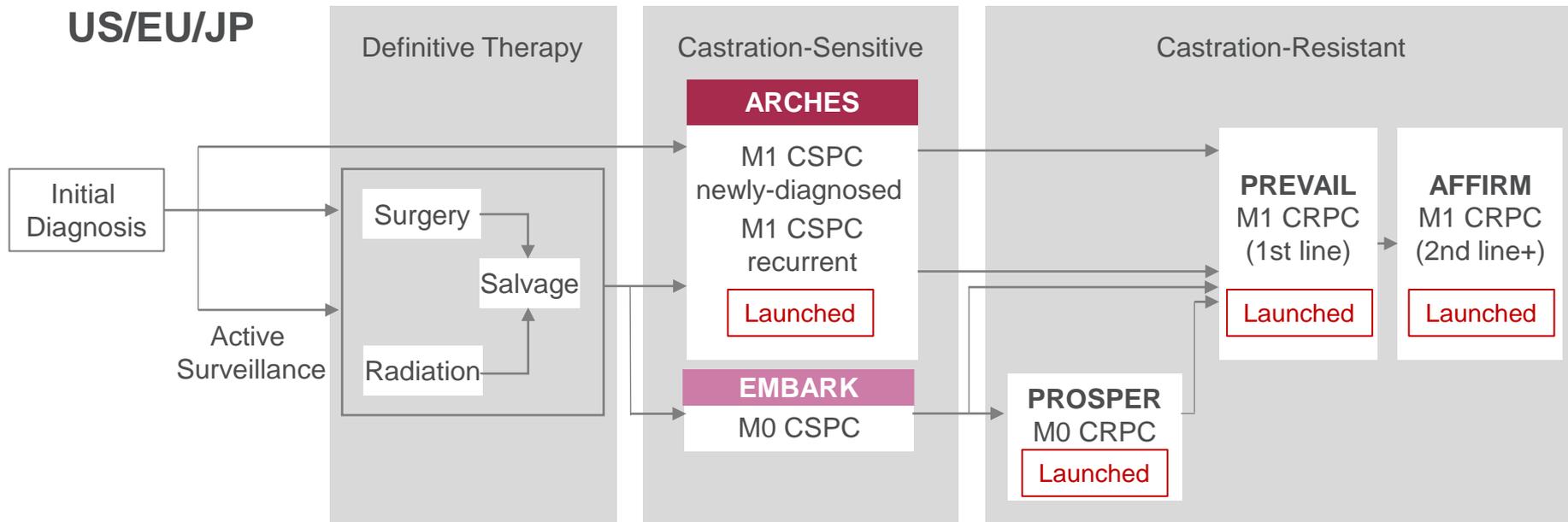
32

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

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

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)



P3: ARCHES	M1 CSPC	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>
P3: EMBARK	M0 CSPC	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*

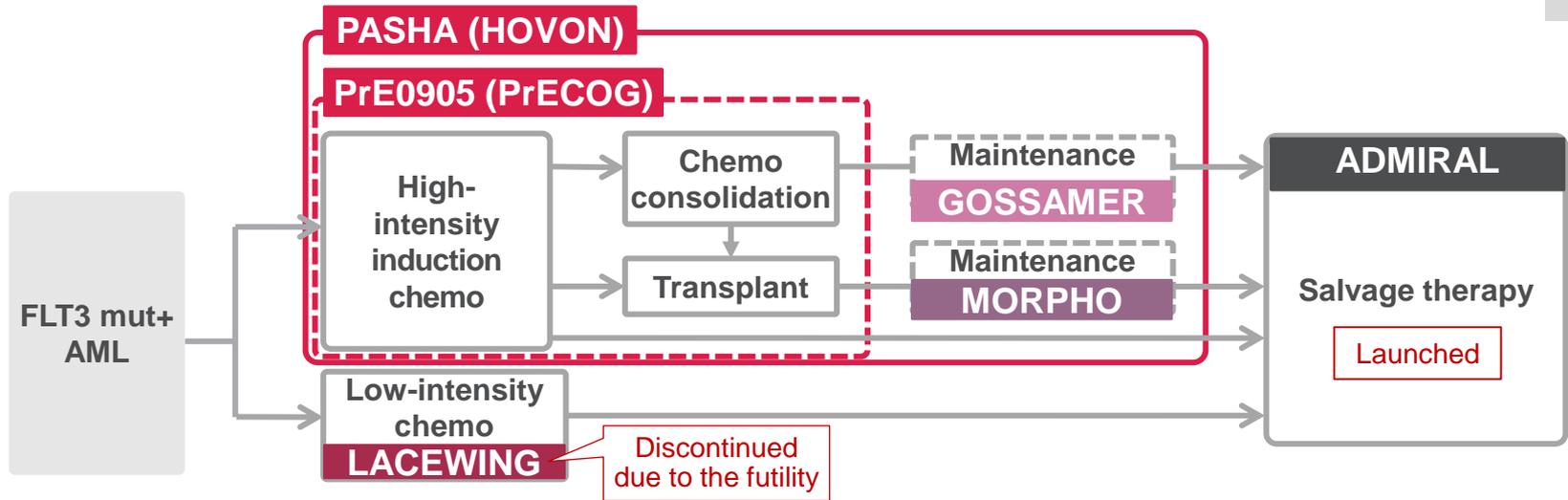
Disease stage	Early stage			Late stage		
	Castration-sensitive (CSPC)			Castration-resistant (CRPC)		
	M0	M1		M0	M1 (pre-chemo)	M1 (post-chemo)
Phase 3 study	EMBARK	ARCHES	ENZAMET	PROSPER	PREVAIL	AFFIRM
Control	Placebo	Placebo	Conventional NSAA	Placebo	Placebo	Placebo
Primary endpoint	MFS (Ongoing)	✓ rPFS HR 0.39	✓ OS HR 0.67	✓ MFS HR 0.29	✓ rPFS HR 0.17 ✓ OS HR 0.71*	✓ OS HR 0.63
OS	(Ongoing)	(Not reached)	✓ HR 0.67	✓ HR 0.73	✓ HR 0.77	✓ HR 0.63
DoT	(Ongoing)	(Not reached)	✓ 29.5 months	✓ 33.9 months	✓ 17.5 months	✓ 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)	P3: ADMIRAL	Monotherapy vs salvage chemo (2:1)	n=371	Launched in US, JP, and EU
Newly diagnosed (HIC-eligible)	P3: PASHA (HOVON)	Combo with high intensity chemo gilteritinib vs. midostaurin (1:1)	n=768	FSFT: Dec 2019 (Sponsor: HOVON)
	P2: PrE0905 (PrECOG)		n=179	FSFT: Dec 2019 (Sponsor: PrECOG, LLC.)
Newly diagnosed (HIC-ineligible)	P3: LACEWING	Combo with azacitidine vs. azacitidine alone (2:1)	n=146	Discontinued due to the futility based on the planned interim analysis
Post-HSCT maintenance	P3: MORPHO	Monotherapy vs. placebo (1:1)	n=346	Enrollment completed Collaborating with BMT-CTN
Post-chemo maintenance	P2: GOSSAMER	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

P3: EV-301	mUC, Platinum and PD-1/L1 inhibitor pretreated; 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>
P3: EV-302	mUC, Previously untreated, Platinum-eligible; EV + Pembro vs. Chemo	n=760	FSFT: Apr 2020
P3: EV-303 /KEYNOTE-905	MIBC, Cis-ineligible; Pembro +/- EV (perioperative) + RC vs. RC alone	n=836	FSFT in Pembro + EV arm: Dec 2020
P3: EV-304 /KEYNOTE-B15	MIBC, Cis-eligible; EV+Pembro (perioperative) + RC vs. Chemo (neoadjuvant) + RC	n=784	<u>FSFT: May 2021</u>
P2: EV-201	mUC, PD-1/L1 inhibitor pretreated; EV mono Cohort 1: Platinum pretreated Cohort 2: Platinum naïve and cis-ineligible	n=219	Cohort 1: Approved (under the Accelerated Approval program) and launched in US in Dec 2019 Cohort 2: <u>sBLA approved in US in Jul 2021</u>
P1b/2: EV-103	Cohorts A - G and K (mUC): A-G: Combo with Pembro and other chemo K: EV mono vs. EV + Pembro Cohorts H, J and L (MIBC, Cis-ineligible, + RC): H: EV mono (neoadjuvant) J (optional): EV+Pembro (neoadjuvant) L: EV mono (perioperative)	n=457	Enrollment ongoing in Cohort K and L 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
P2: EV-203	<Bridging study in China> mUC, Platinum and PD-1/L1 inhibitor pretreated; EV mono	n=40	<u>To start in Q2 FY2021 (IND approved)</u>
P1: EV-104	<u>NMIBC, High-risk BCG-unresponsive; Intravesical EV mono</u>		<u>To start in Q2 FY2021</u>

For other solid tumors

P2: EV-202	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

37

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.	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)	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 Cohort 2: First line, Combo with mFOLFOX6 Cohort 3: Third or later line, Combo with pembrolizumab Cohort 4: First line, Combo with mFOLFOX6 and nivolumab	<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 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 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

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

Asia (except for Japan)

P3: MOONLIGHT 1	Moderate to severe VMS associated with menopause; The first 12 weeks: DB, 30 mg vs. placebo (1:1) The last 12 weeks: Active extension treatment period, 30 mg	n=300	FSFT: Apr 2020
P3: MOONLIGHT 3	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

40

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: 1.3×10^{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

