CSP2021

CSP: CORPORATE STRATEGIC PLAN

For the period FY2021 - FY2025

Evolved strategy. Ambitious goals. Transformative execution.
Same deep commitment to our VISION.

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Kenji Yasukawa, Ph.D.
President and CEO
Astellas Pharma Inc.
May 26, 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.

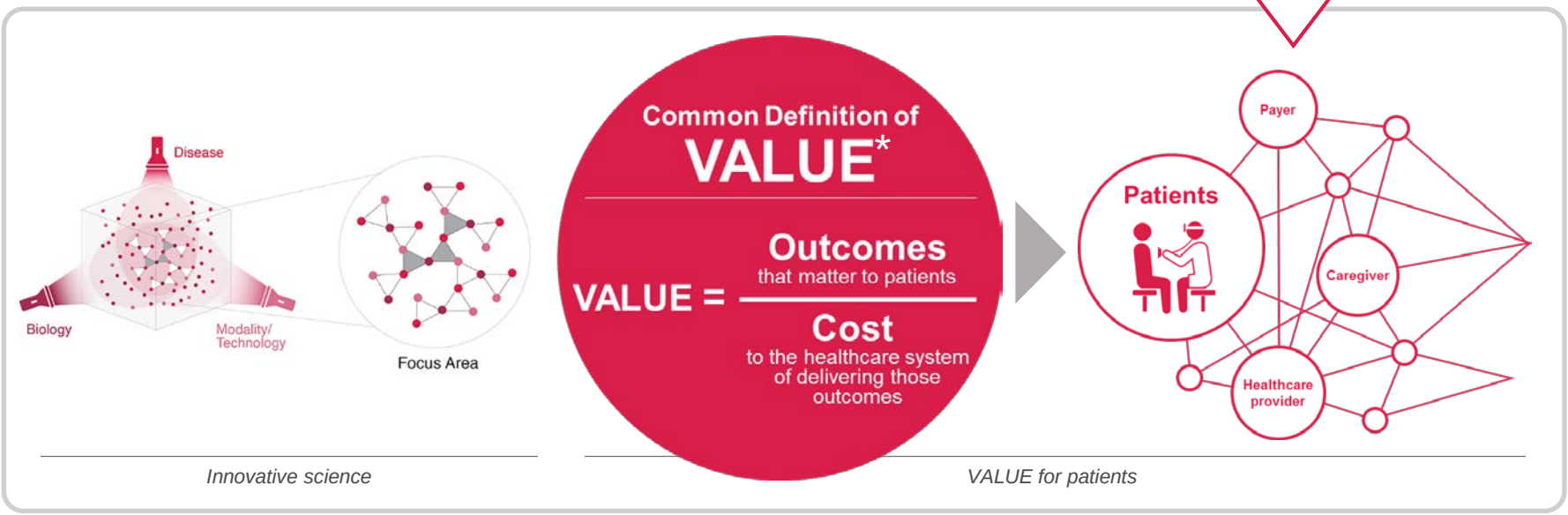
DEEP COMMITMENT TO OUR VISION

FY2018 - FY2020
CSP2018

FY2021 - FY2025
CSP2021

VISION

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



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* Adapted from “What Is Value in Health Care?” Porter, M.E. (2010). New England Journal of Medicine

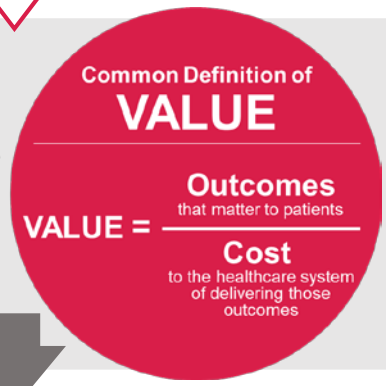
CSP2021 OVERVIEW

FY2018 - FY2020 CSP2018 → FY2021 - FY2025 CSP2021 → VISION

Evolved strategy. Ambitious goals. Transformative execution.
Same deep commitment to our VISION.

Strategic Goals

1. Enable patients to achieve better outcomes
2. Translate innovative science into proven VALUE
3. Advance the Rx+ business
4. Deepen our engagement in sustainability



Organizational Health Goals

Transforming Astellas' ability to execute by fostering a culture where innovation, talent and collaboration come together to reach ambitious goals

Performance Goals: JPY 7T Market Cap in FY2025 *by achieving*

1. **Revenue:** XTANDI and Strategic products* sales ≥ ¥1.2T in FY2025
2. **Pipeline Value:** Focus Area projects expected sales ≥ ¥0.5T in FY2030
3. **Core Operating Profit Margin:** ≥ 30% in FY2025

Market Cap ¥7T

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

CSP2021 AGENDA

Revenue, Pipeline Value

1 XTANDI and Strategic products*:
≥ ¥1.2T in FY2025

2 Post-PoC projects
from Primary Focuses

3 Multiple technology platforms

4 Focus Area projects:
≥ ¥0.5T in FY2030

Market Cap
¥7T
in FY2025

Core OP

5 Flat SG&A in absolute terms

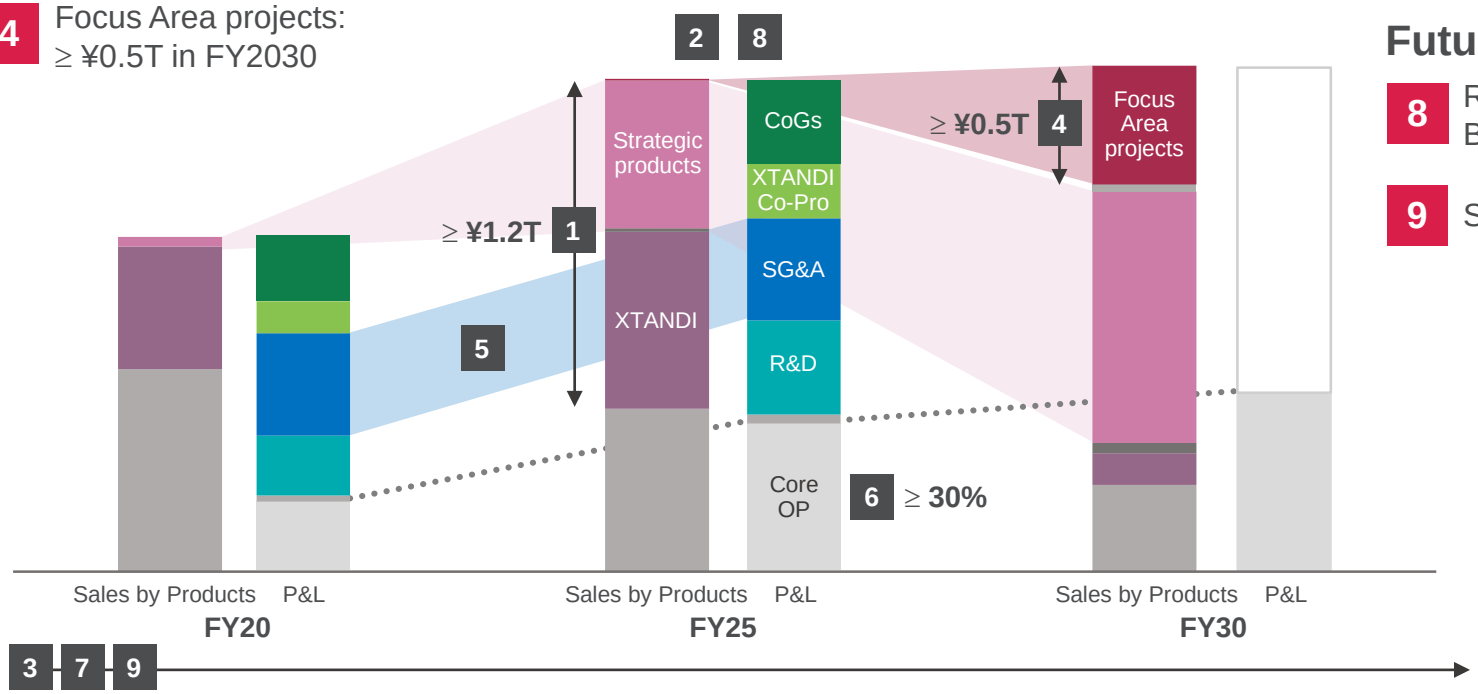
6 Sufficient R&D investments
Core OP margin of ≥ 30% in FY2025

7 Steady increase in dividends

Future growth

8 Rx+:
Breakeven by FY2025

9 Sustainability



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

REGULATORY TIMELINE
XTANDI AND STRATEGIC PRODUCTS

Expand additional indications for XTANDI, XOSPATA and PADCEV
Expect new launch for zolbetuximab, fezolinetant and AT132

Product	Target Filing Timing				
	FY2021	FY2022	FY2023	FY2024	FY2025 or later
XTANDI (enzalutamide)		M0 CSPC			
XOSPATA (gilteritinib)		AML, post-HSCT maintenance			AML, newly diagnosed and HIC-eligible
PADCEV (enfortumab vedotin)		mUC, previously untreated (AA in US)	*based on EV-103 study cohort data*	mUC, previously untreated (1L)	MIBC
zolbetuximab		Gastric and GEJ adenocarcinoma			
fezolinetant		Moderate to severe VMS associated w/ menopause			
AT132 (resamirigene bilparvovec)		XLMTM	*assuming no significant changes in the current ASPIRO study protocol*		

Note) Only indications undergoing pivotal studies are included (as of May 2021).
Subject to internal assessment, decision and regulatory consultation, as appropriate.
Filing (submission) timing in the first country/region within US, EU, JP

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M0 CSPC: Non-metastatic castration-sensitive prostate cancer, AML: Acute myeloid leukemia, HSCT: Hematopoietic stem cell transplantation, HIC: High-intensity chemotherapy, mUC: Metastatic urothelial cancer, AA: Accelerated Approval, 1L: First line, MIBC: Muscle-invasive bladder cancer, GEJ: Gastroesophageal junction, VMS: Vasomotor symptoms, XLMTM: X-linked myotubular myopathy

POTENTIAL PEAK SALES
XTANDI AND STRATEGIC PRODUCTS

In addition to XTANDI, multiple strategic products expected to grow in 2020s

Product	Potential Peak Sales *(Global, billions of yen)*	
XTANDI (enzalutamide)	**600 - 700**	1st treatment option in early stage of prostate cancer
fezolinetant	**300 - 500**	No good replacement for HRT Aim for new treatment option as first-in-class, non-hormonal treatment
PADCEV (enfortumab vedotin) [1]	**300 - 400**	Significant growth potential with 1L mUC
XOSPATA (gilteritinib)	**100 - 200**	Potential indications into earlier lines
zolbetuximab	**100 - 200**	Aim for 1st choice in 1L HER2-, CLDN 18.2+ patients
Evrenzo (roxadustat) [2]	**50 - 100**	New oral therapeutic option as the first-in-class HIF-PHI
AT132 (resamirigene bilparvovec)	**50 - 100**	Significant high unmet need for XLMTM Aim for the first approved therapy

Note) Only indications undergoing pivotal studies are included for projection (as of May 2021)
1. Sales for Americas are calculated based on the sales booked by Seagen
2. Astellas territories only; Japan, Europe, the Commonwealth of Independent States, the Middle East, South Africa, etc.

HRT: Hormone replacement therapy, 1L: First line, mUC: Metastatic urothelial cancer, HER2-: HER2 negative, CLDN 18.2+: Claudin 18.2 positive, HIF-PHI: Hypoxia-inducible factor prolyl hydroxylase inhibitor, XLMTM: X-linked myotubular myopathy

CLINICAL PROOF AND EXPANSION OF KEY PLATFORMS

Post-PoC projects from Primary Focuses

CSP2021

1	**2**	3
4	5	6
7	8	9

8

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

Primary Focus	Biology/Modality/Technology [1]	FY21	FY22-23	FY24-25	No. of projects aiming PoC by end FY25 [2]
Genetic regulation	Gene replacement (AAV)				**7**
	Gene regulation (AAV)				
Immuno-Oncology	Checkpoint				**15**
	Artificial adjuvant vector cell (aAVC)				
	Oncolytic virus (intratumoral)				
	Oncolytic virus (systemic)				
	Bispecific immune cell engager				
	Cancer cell therapy (UDC) [3]				
Blindness & Regeneration	Cell replacement				**3**
	Cell replacement (UDC)				
	Gene regulation (AAV)				
Mitochondria Biology	Gene regulation & mitochondrial biogenesis				**5**
	Mitochondrial stress				
	Mitochondrial transfer				
Primary Focus Candidates	Immune modulating/regulatory cells				**1**
	Tissue-specific immune regulation				
	Protein degrader				
				Total	**31**

Modality
- Small molecule
- Antibody
- Gene
- Cell
- Other

Stage of the most advanced project in the category
- Discovery/ Preclinical
- Pre-PoC
- Post-PoC

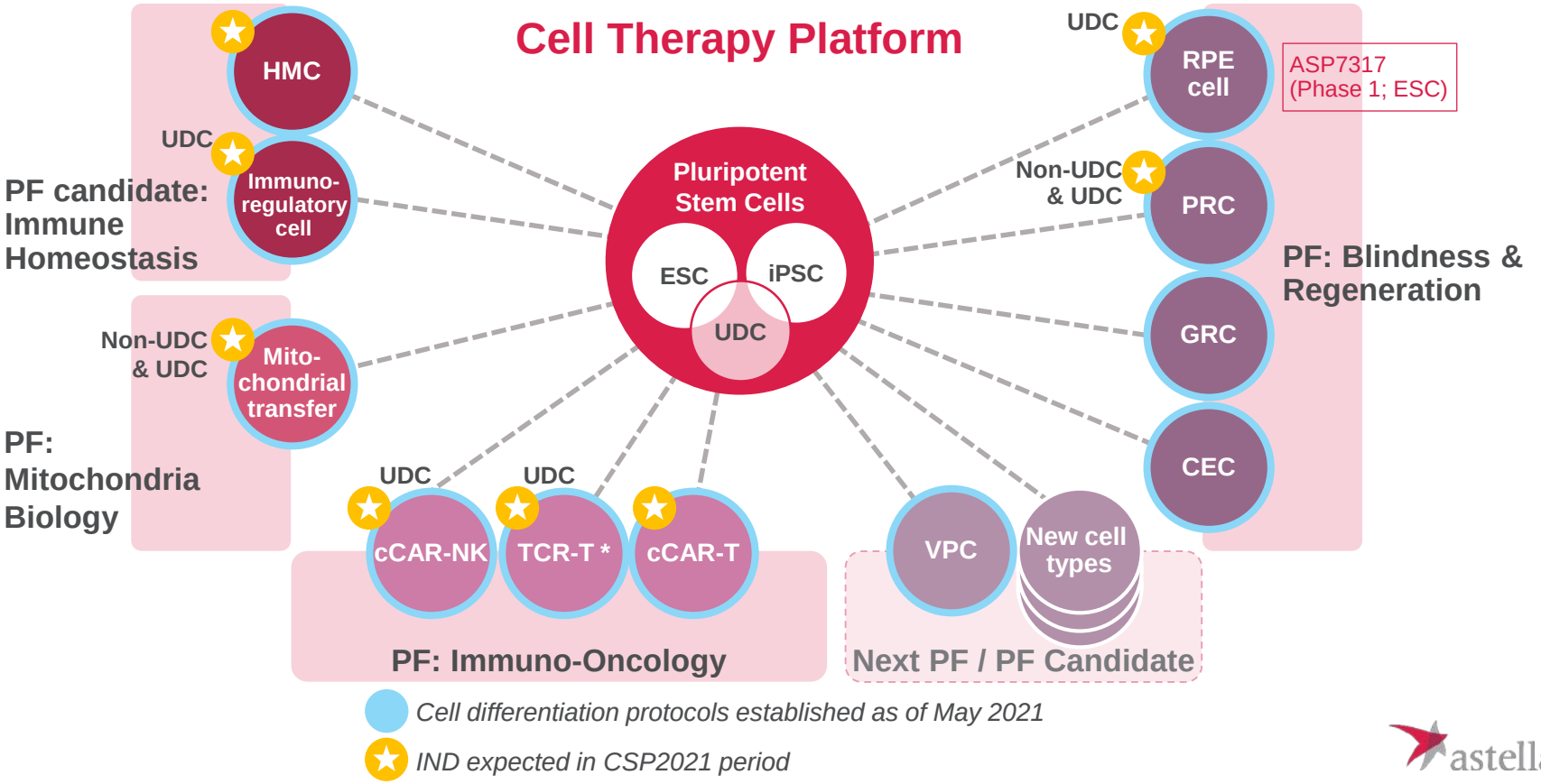
1. Not exhaustively listed. 2. Estimated based on standard development timelines, assuming 100% probability of success (as of May 2021).
3. The first *convertible*CAR program (with autologous cells) IND is planned for late FY2021.
PoC: Proof of concept (key clinical data supporting a decision to initiate late-stage development), AAV: Adeno-associated virus, UDC: Universal donor cell

ORGANIC APPLICATION OF CELL THERAPY PLATFORM

Established cell differentiation protocols for 11 cell types

AIRM can supply all the clinical demand of drug substance and drug product for all the cell therapy programs

AIRM
@Westborough, MA
(in operation in Apr 2020)



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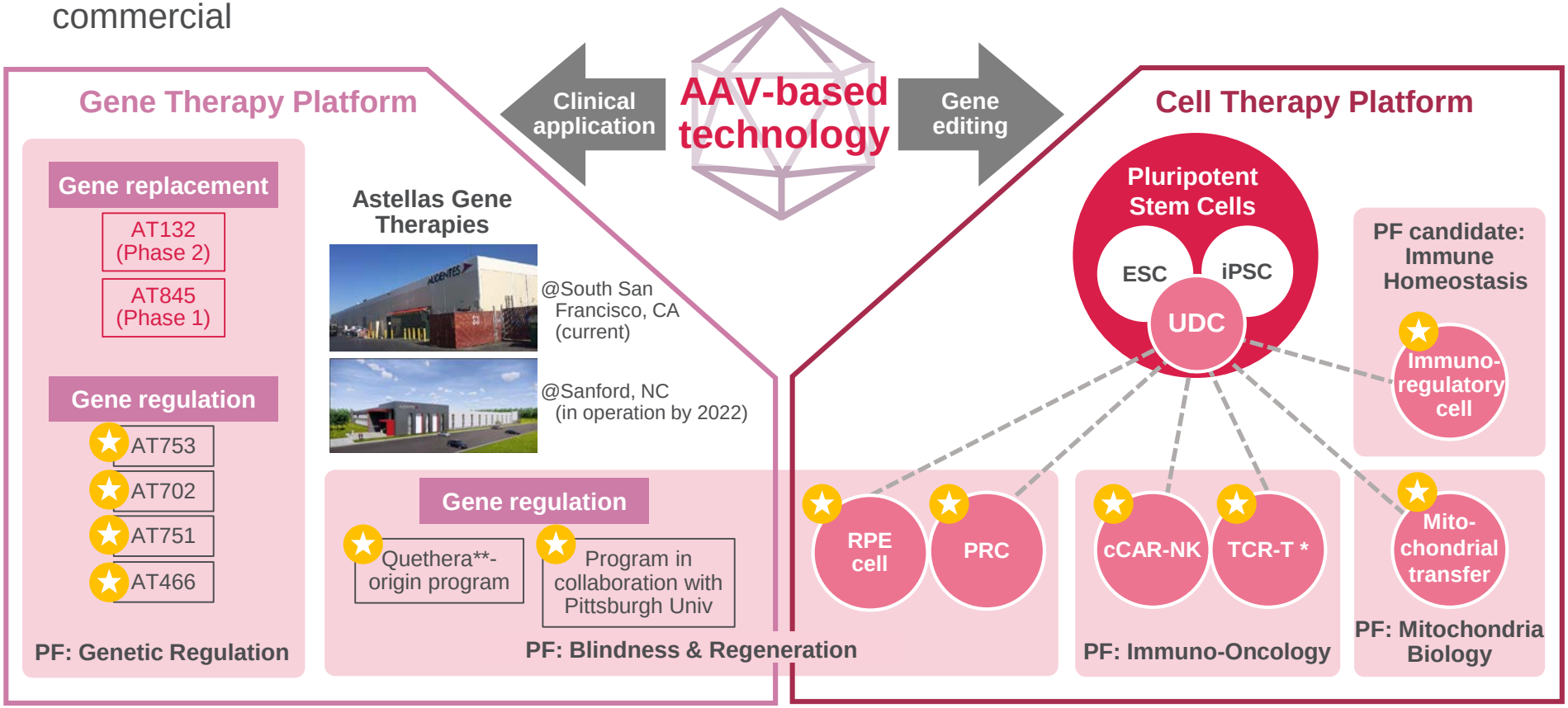
* In partnership with Adaptimmune
AIRM: Astellas Institute for Regenerative Medicine, PF: Primary Focus, ESC: Embryonic stem cell, UDC: Universal donor cell, iPSC: Induced pluripotent stem cell, HMC: Hemangioblast-derived mesenchymal stem cell, RPE: Retinal pigment epithelium, PRC: Photoreceptor rescue cell, GRC: Ganglion rescue cell, CEC: Corneal endothelial cell, VPC: Vascular progenitor cell, CAR: Chimeric antigen receptor, cCAR: *convertible*CAR, TCR: T-cell receptor, NK: Natural killer, IND: Investigational New Drug Application

ORGANIC APPLICATION OF AAV-BASED TECHNOLOGY PLATFORM

Aim for multiple INDs using AAV-based technology in CSP2021 period (★)

AAV manufacturing capability can provide self sufficiency from research to commercial



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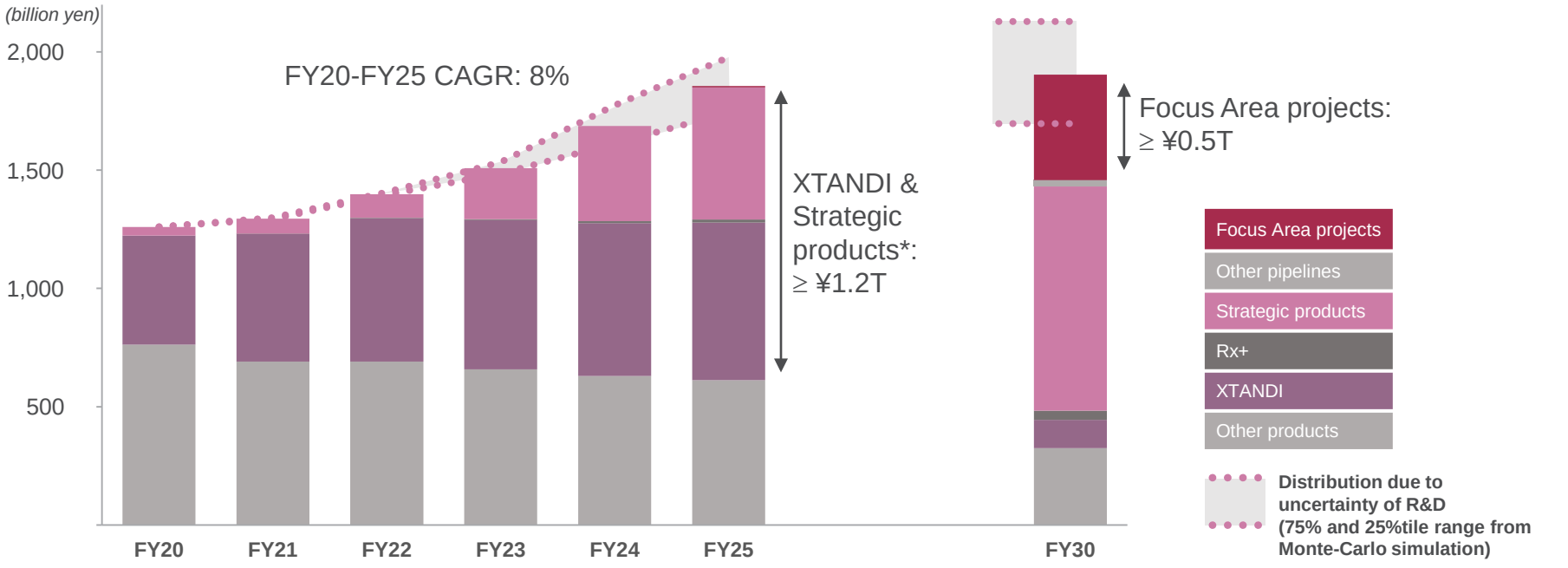
* In partnership with Adaptimmune, ** Acquired (current program classified as 'in-house')
IND: Investigational New Drug Application, AAV: Adeno-associated virus, PF: Primary Focus, ESC: Embryonic stem cell, iPSC: Induced pluripotent stem cell, UDC: Universal donor cell, RPE: Retinal pigment epithelium, PRC: Photoreceptor rescue cell, CAR: Chimeric antigen receptor, cCAR: *convertible*CAR, TCR: T-cell receptor, NK: Natural killer

REVENUE FORECAST DURING CSP2021 AND BEYOND

Robust revenue growth 8% through to FY2025, driven by XTANDI and Strategic products.

Strategic products offset XTANDI sales decline and Focus Area projects become incremental growth drivers toward FY2030

Consolidated revenue forecast *(Risk adjusted)*



Assumption of CSP2021 forecast:
We are not forecasting generic entry of mirabegron (US) and Lexiscan (US) in CSP2021 period

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

TRANSFORMATION OF SG&A COST STRUCTURE

Hold down total SG&A flat in absolute terms while ensuring sufficient investment in newly launched products by cross-business execution plan

Flat SG&A in absolute terms

Invest in growth products and digital transformation

- New product launch readiness and investments
- Commercial organization for new modalities
- Digital initiatives to evolve our business

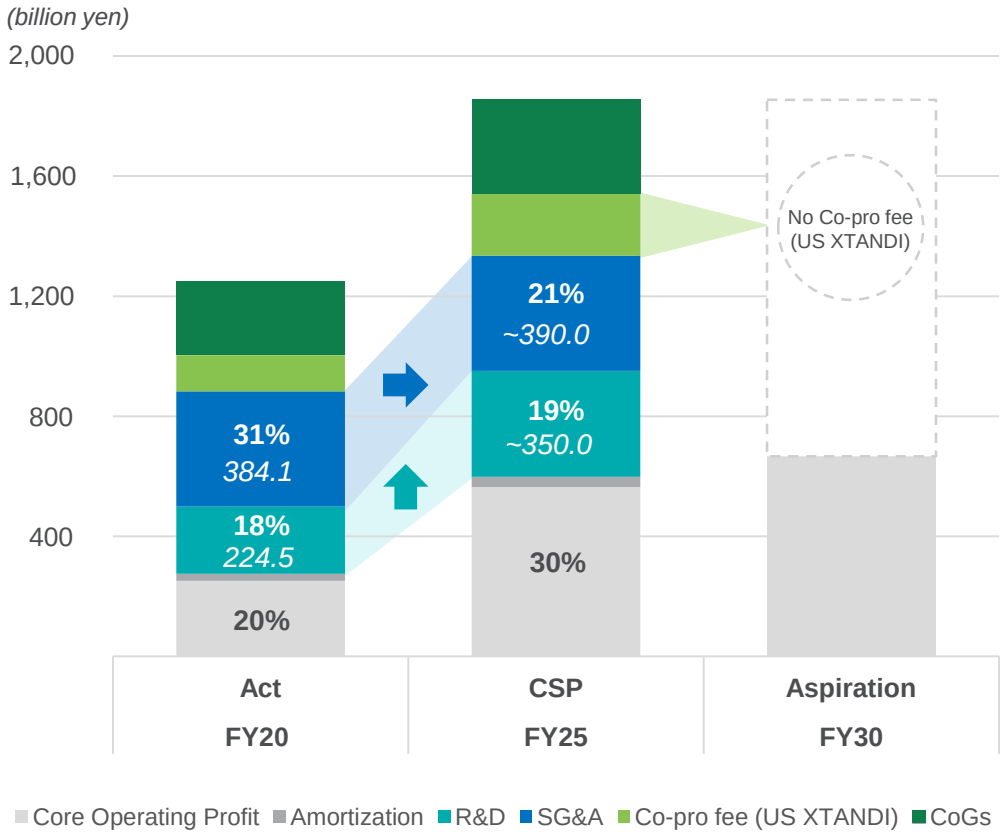
Initiatives to drive efficiency and excellence

- Speciality portfolio
- Salesforce transformation
- Globalized commercial structure
- Simplification and automation from digital
- Corporate functions globalization
- “New normal” working patterns
- Procurement savings programs

SG&A: Excluding US XTANDI Co-promotion fee

FINANCIAL STRUCTURE: CSP2021 AND BEYOND

Expecting greater than 30% Core OP margin in FY2025 driven by upward revenue and transformation of SG&A cost structure while increasing R&D investment



FY2025 Margin Targets:

SG&A: 21% of revenue
SG&A flat in absolute amount despite sufficient investment in newly launched products. Given revenue upward, SG&A ratio significantly improves

R&D: 19% of revenue
Increase investment in Focus Area projects for future growth

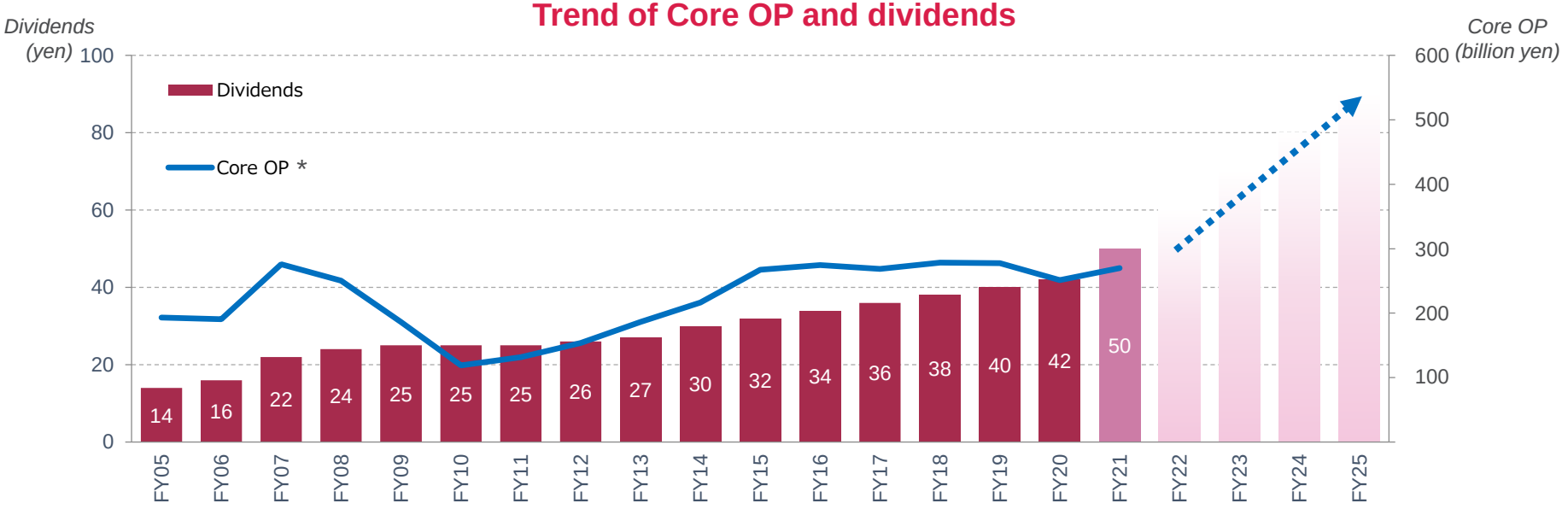
Core OP: 30% of revenue
Enforced by increasing sales revenue; company ambitiously undertaking competitive Core OP ratio, getting closer to FY2030

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

Trend of Core OP and dividends



For illustrative purposes only

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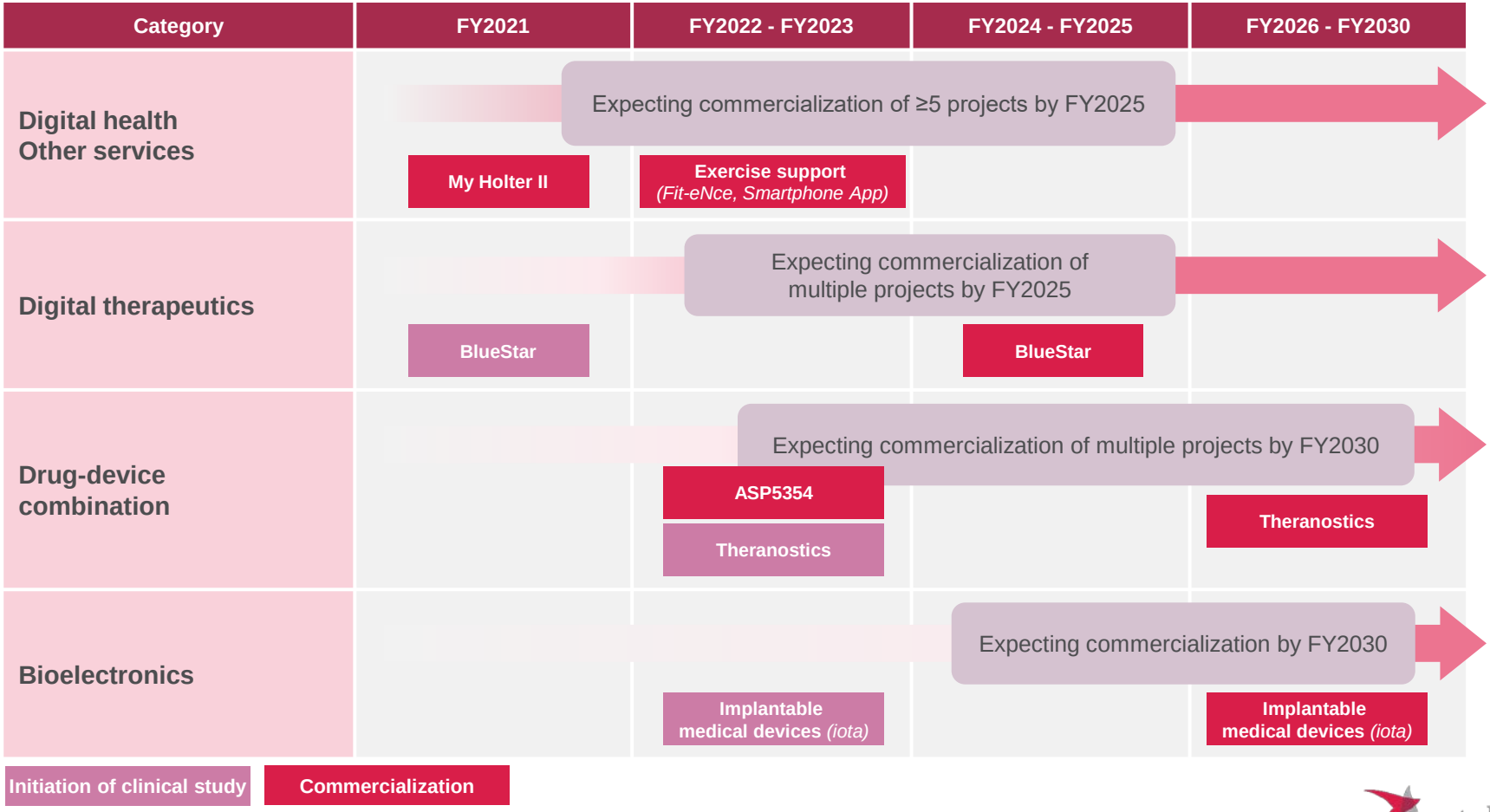
* Prior to FY2012, operating profit is in accordance with J-GAAP

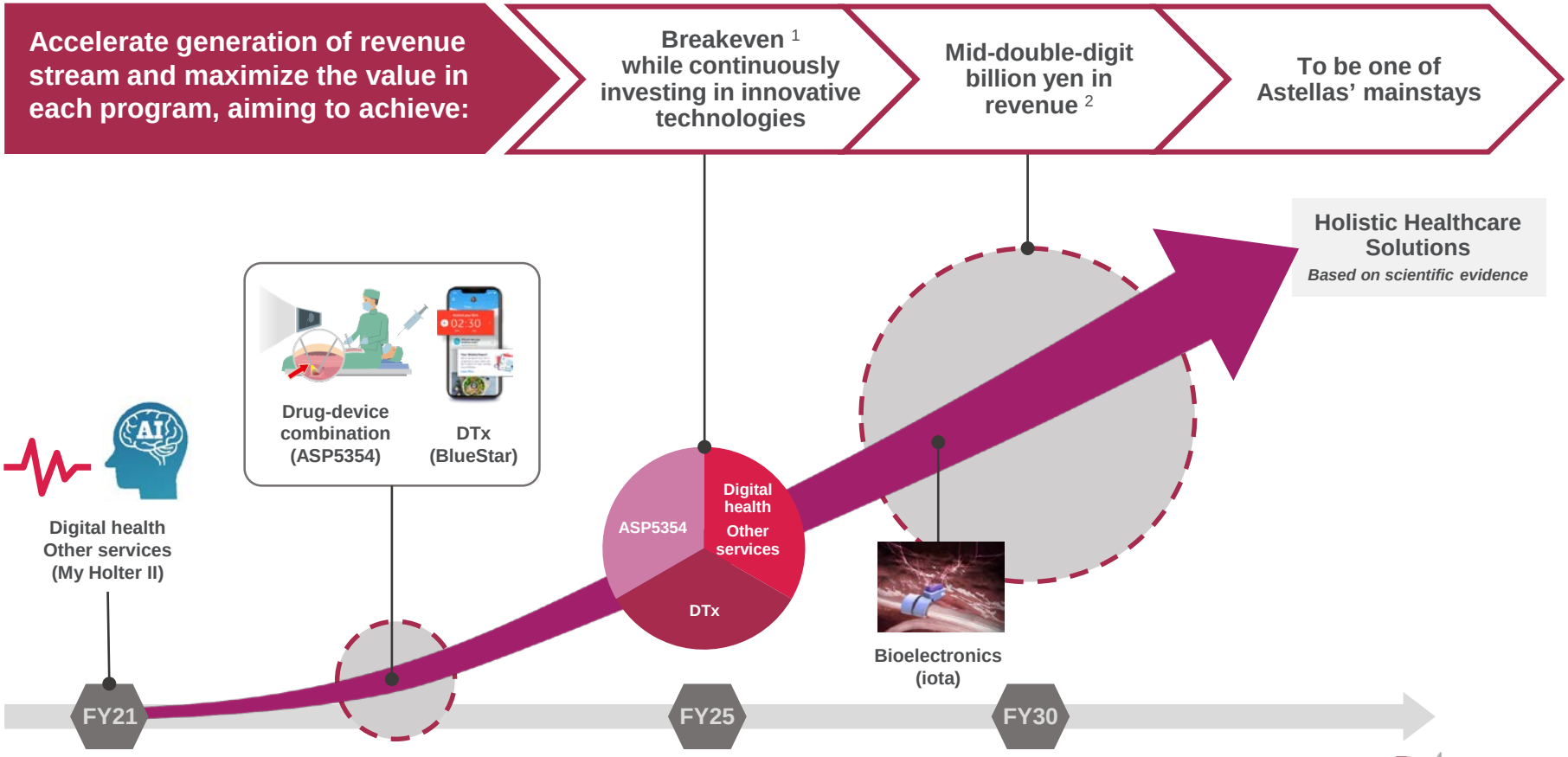
EXPECTED PROGRESS OF Rx+ PROGRAMS

Planning commercialization of multiple projects in each category



POTENTIAL GROWTH OF Rx+ BUSINESS

Expecting to break even by FY2025 with continued investment, reach the revenue of mid-double-digit billion yen by FY2030, and grow toward one of Astellas' mainstays in the 2030s



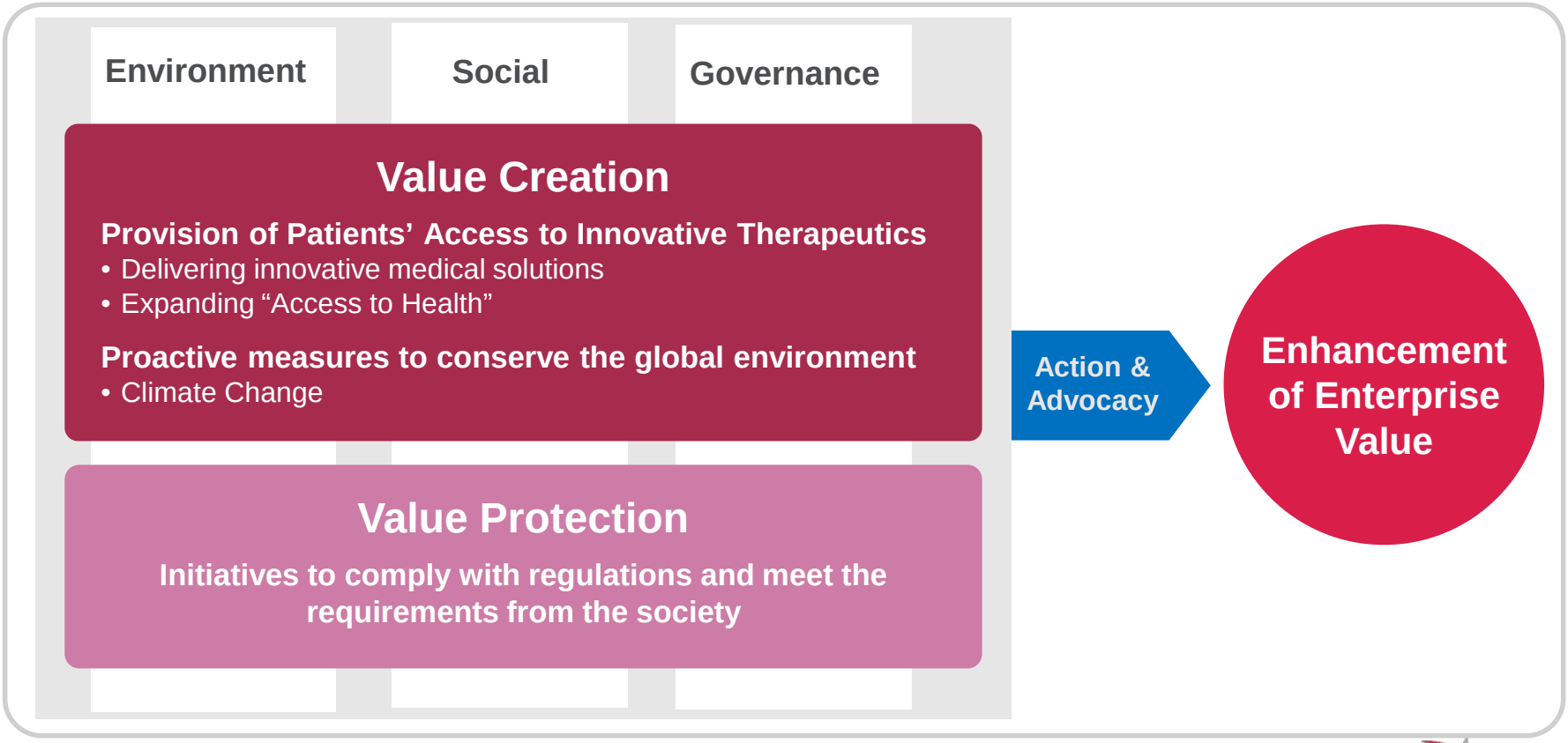
1. "Breakeven": a state that the total revenue of Rx+ businesses covers the cost for the entire Rx+-related activities.
2. The size of each circle corresponds to the rough scale of the annual revenue forecast.

DTx: Digital therapeutics

HOW ASTELLAS CONTRIBUTE TO SUSTAINABILITY OF SOCIETY

Shift from “CSR-Based Management” to “Astellas’ Sustainability” which contributes to the sustainability of both the society and Astellas

In CSP2021, enhance our “Value Creation" activities



CSR: Corporate Social Responsibility

FURTHER ENHANCE “VALUE CREATION”

Enhance activities which contribute to profit or generate synergy with Astellas’ business

Leverage Astellas’ capability for initiatives of sustainability

Enhancements

Access to Health

Implement a comprehensive strategy from development to the market for providing access to health to address patient needs

Environment (Climate Change)

In line with TCFD recommendation

- Disclose scenario-based analysis in FY2021
- Update the analysis in FY2023

TCFD: Task Force on Climate-related Financial Disclosures

CONCLUSION

Revenue, Pipeline Value

1 XTANDI and Strategic products*:
≥ ¥1.2T in FY2025

2 Post-PoC projects
from Primary Focuses

3 Multiple technology platforms

4 Focus Area projects:
≥ ¥0.5T in FY2030

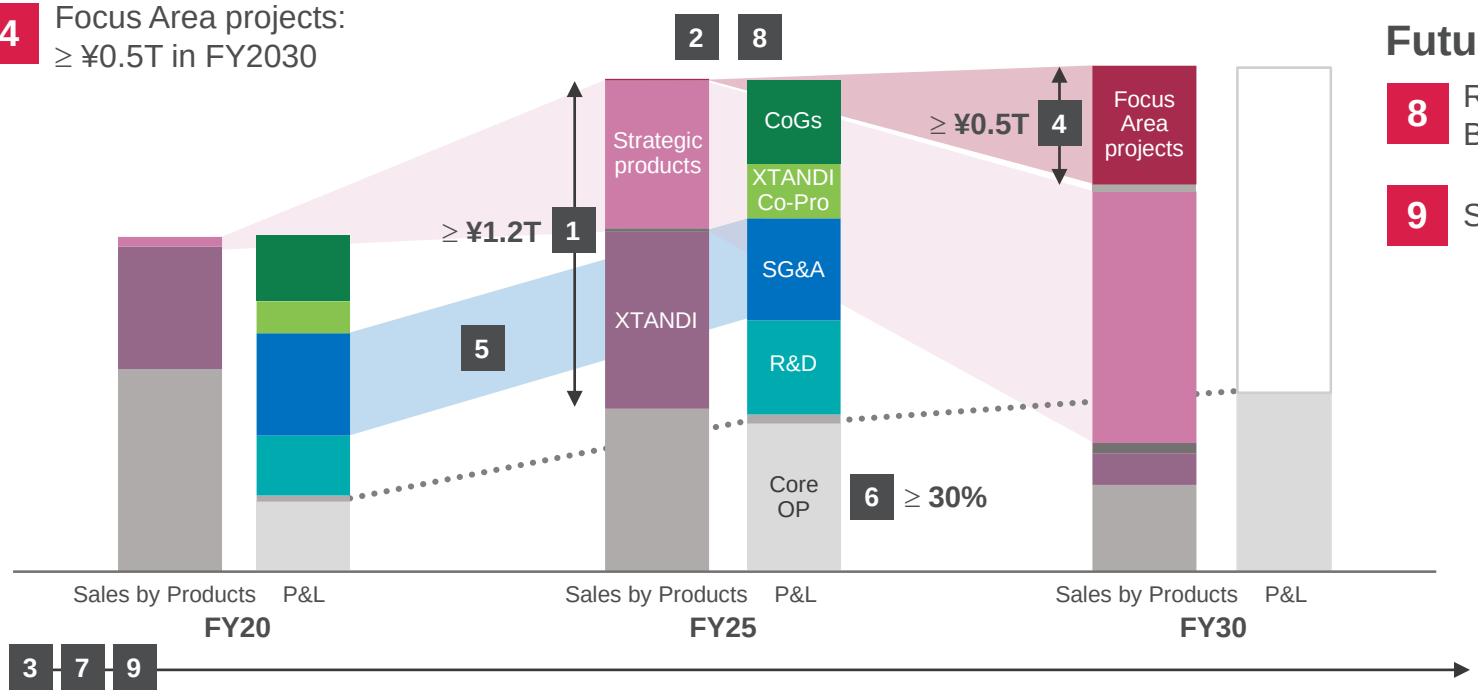
Market Cap ¥7T
in FY2025

Core OP

5 Flat SG&A in absolute terms

6 Sufficient R&D investments
Core OP margin of ≥ 30% in FY2025

7 Steady increase in dividends



Future growth

8 Rx+:
Breakeven by FY2025

9 Sustainability

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

APPENDIX

OUR STRATEGIC GOALS (SG)

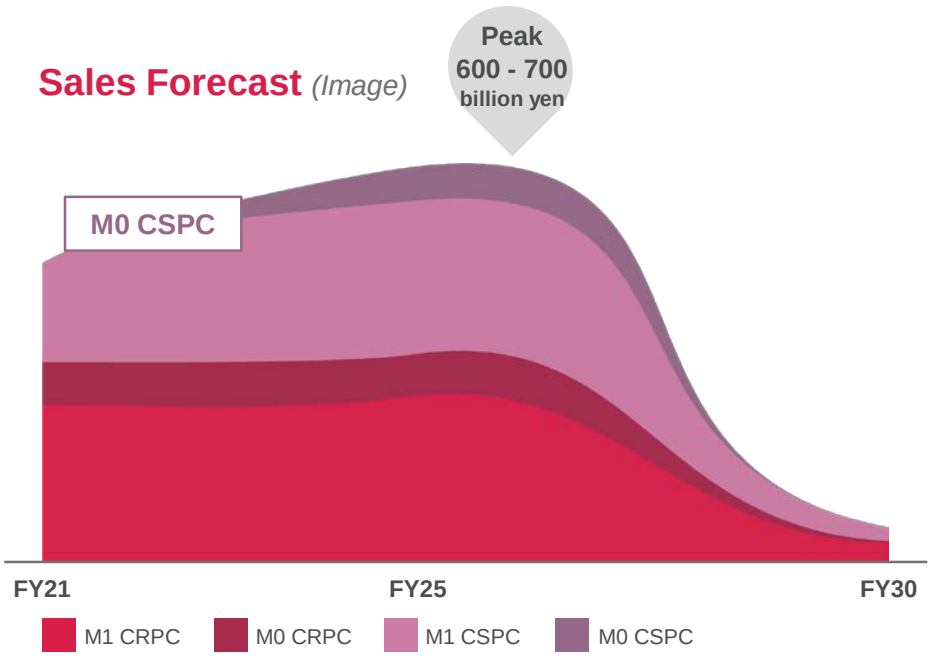
VALUE creation and delivery will be achieved by pursuing these 4 goals:

SG1	SG2	SG3	SG4
Enable patients to achieve better outcomes	Translate innovative science into proven VALUE	Advance the Rx+ business	Deepen our engagement in sustainability
This reflects our commitment towards maximizing: i. Sustainable patient access to our portfolio and ii. Outcomes that those patients achieve as a consequence	Taking our execution of the Focus Area approach to the next level by: i. Accelerating proof of VALUE and expansion of our Primary Focuses ii. Effective exploration of the cutting-edge of biopharmaceutical innovation	We continue to pursue this groundbreaking path to turn innovative science into VALUE for patients The Rx+ business will commercialize multiple solutions during this CSP period while building and accelerating its pipeline	We recognize the importance of our commitment to sustainability; maximizing positive societal impact and being recognized for it

OUTLOOK FOR XTANDI (ENZALUTAMIDE)

- Continue to establish XTANDI as the 1st treatment option in eligible patients, building on our breadth of clinical data, depth of experience and appropriately differentiating our clinical benefits across NHTs and other treatment options, specifically:
  - Convenient dosing (once a day, no food co-administration)
  - Maintenance of patient reported QoL (FACT-P)
  - Extending life and /or delaying progressive metastatic disease that is associated with worse outcomes in multiple patient populations

Sales Forecast *(Image)*



Patient segment	Pivotal study	Number of eligible patients*
M0 CSPC (high risk biochemical recurrent)	EMBARK	~10,000 (US)
M1 CSPC	ARCHES	~100,000
M0 CRPC	PROSPER	~60,000
M1 CRPC	PREVAIL (pre-chemo) AFFIRM (post-chemo)	~100,000

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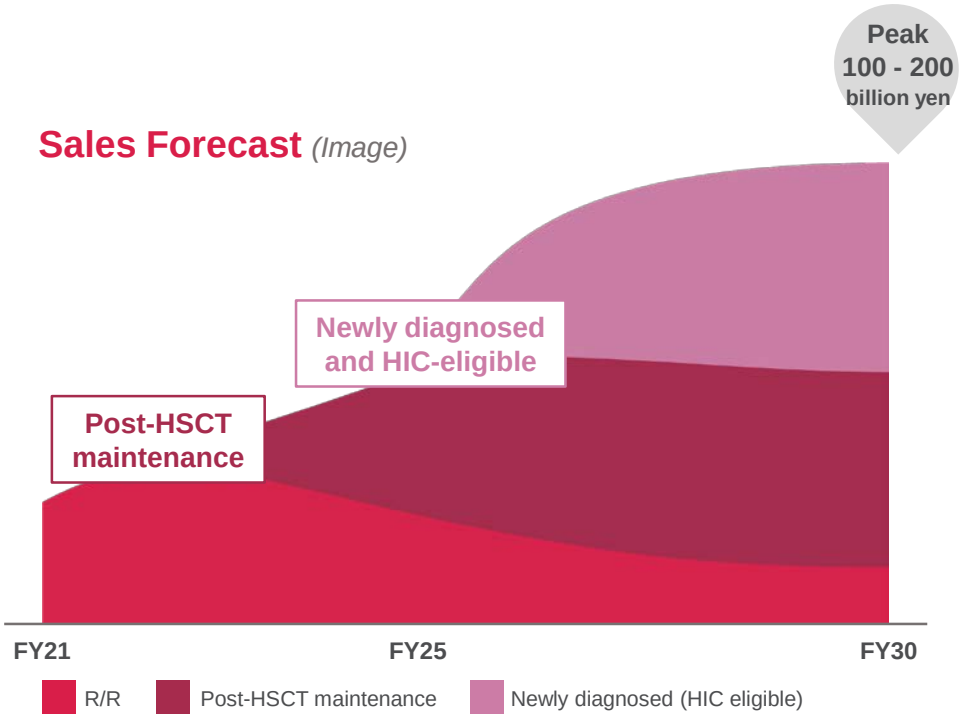
* Based on internal estimates
NHT: Novel Hormonal Therapy, M0: Non-metastatic, M1: Metastatic, CRPC: Castration-resistant prostate cancer, CSPC: Castration-sensitive prostate cancer, QoL: Quality of life, FACT-P: Functional Assessment of Cancer Therapy - Prostate

OUTLOOK FOR XOSPATA (GILTERITINIB)

- Establish XOSPATA as the 1st choice therapy for all appropriate patients with FLT3m+ AML through a coordinated approach to patient identification, a compelling and differentiated value proposition and competitive data generation
- R/R sales will continue to grow for several years before declining as patients are treated in earlier lines starting with MORPHO (HSCT Maintenance)
- Overall sales growth will continue due to the potential future indications into earlier lines of therapy

Sales Forecast *(Image)*



Patient segment	Pivotal study	Number of eligible patients*
FLT3m+ AML, newly diagnosed and HIC-eligible	PASHA (HOVON) PrE0905 (PrECOG) [Phase 2]	7,000
FLT3m+ AML, post-HSCT maintenance	MORPHO	3,800
R/R FLT3m+ AML	ADMIRAL	7,700

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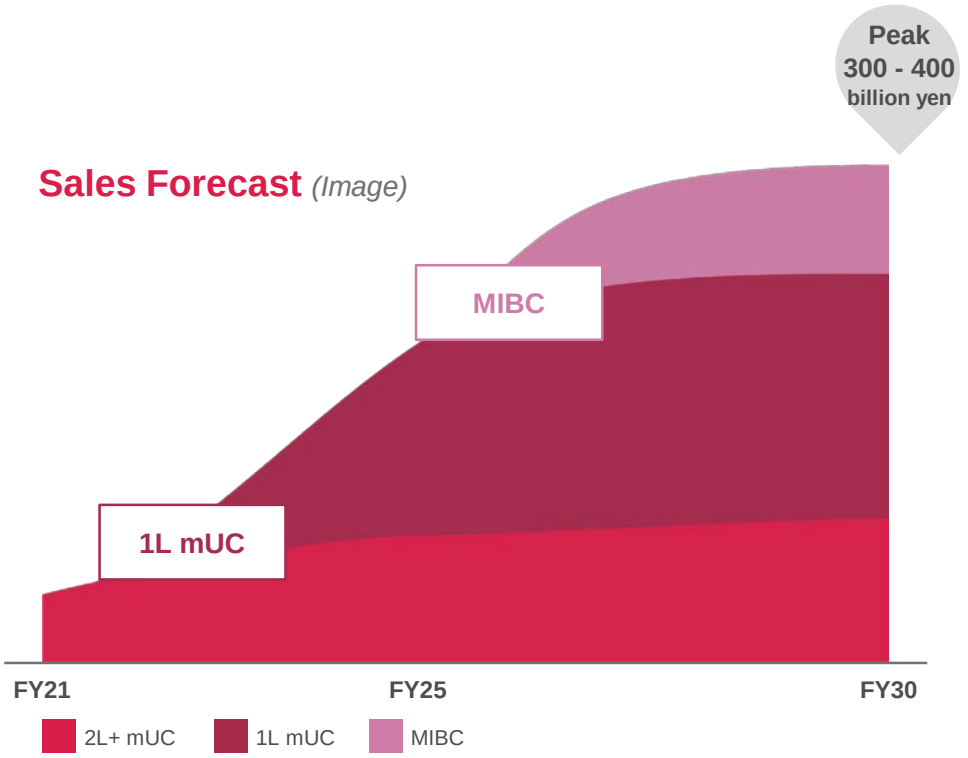
* Based on internal estimates

FLT3m+: FLT3 mutation positive, AML: Acute myeloid leukemia, R/R: Relapsed or refractory, HSCT Hematopoietic stem cell transplant, HIC: High-intensity chemotherapy

OUTLOOK FOR PADCEV (ENFORTUMAB VEDOTIN)

- Establish PADCEV as the 1st choice in each approved and reimbursed indication by being 1st to market with robust clinical data in mUC and MIBC
- Enhance and foster clinical confidence and positive experiences, positioning as the new global SoC in uC
- The significant growth driver is 1L mUC indication in combination with pembrolizumab and there is potential to redefine 1L mUC treatment with a platinum-free option through novel PADCEV combinations

Sales Forecast *(Image)*



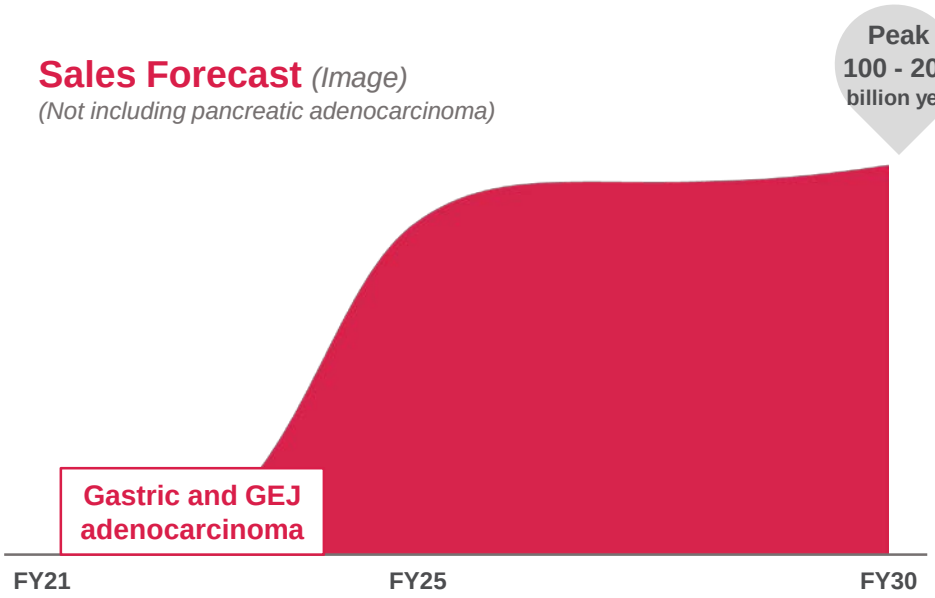
Patient segment	Pivotal study (PADCEV regimen)	Number of eligible patients*
MIBC, cis-ineligible	EV-303 / KEYNOTE-905 (combo w/ pembrolizumab)	10,000
MIBC, cis-eligible	EV-304 / KEYNOTE-B15 (combo w/ pembrolizumab)	37,000
mUC, previously untreated (1L)	EV-302 EV-103 Cohorts [Phase 1b/2 for AA in US] (combo w/ pembrolizumab)	76,000
mUC, PD-1/L1 inhibitor treated and cis-ineligible	EV-201 Cohort 2 [Phase 2] (monotherapy)	1,600 (US)
mUC, platinum and PD-1/L1 inhibitor treated	EV-301 EV-201 Cohort 1 [Phase 2 for AA in US] (monotherapy)	38,000

* Based on internal estimates
mUC: Metastatic urothelial cancer, MIBC: Muscle-invasive bladder cancer, SoC: Standard of care, uC: Urothelial carcinoma, 1L: First line, 2L+: Second or later line, cis: Cisplatin, AA: Accelerated approval

OUTLOOK FOR ZOLBETUXIMAB

- mGC is an area of significant unmet need, especially in advanced stages with ~4% five-year survival rate at Stage IV GC and limited treatment options have been limited
  - Prevalence of patients with high expression of CLDN18.2 is substantial: 33% - 37%
  - Ensure pathologists and prescribers have timely access to high-quality, reproducible CLDN18.2 testing for mGC patients
  - Establish zolbetuximab as the 1st choice in 1L HER2-, CLDN 18.2+ patients
- Zolbetuximab also being investigated for pancreatic adenocarcinoma

Sales Forecast *(Image)*
(Not including pancreatic adenocarcinoma)



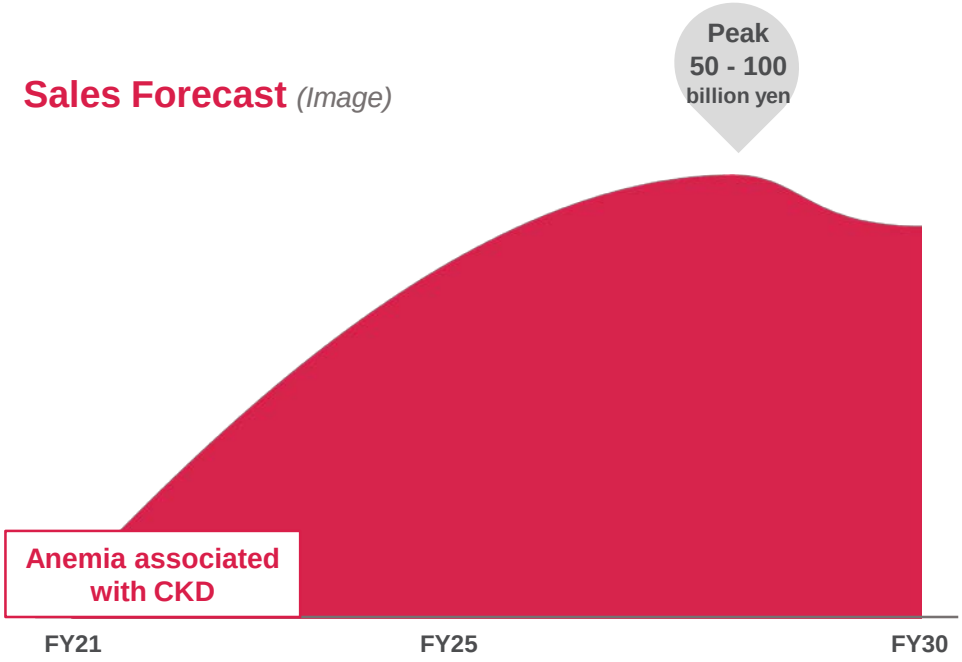
Patient segment	Pivotal study (zolbetuximab regimen)	Number of eligible patients*
HER2-, CLDN 18.2+ gastric and GEJ adenocarcinoma (1L)	SPOTLIGHT (combo with mFOLFOX)	~82,000
	GLOW (combo with CAPOX)	

* Based on internal estimates
mGC: (metastatic) Gastric cancer, 1L: First line, HER2-: HER2 negative, CLDN 18.2+: Claudin 18.2 positive, GEJ: Gastroesophageal junction, mFOLFOX6: 5-FU, leucovorin and oxaliplatin, CAPOX: Capecitabine and oxaliplatin

OUTLOOK FOR EVRENZO (ROXADUSTAT)

- Establish Evrenzo as a new oral therapeutic option (as the first-in-class HIF-PHI) competing with ESA in both DD and NDD segments
- EU and several countries in the International Market will launch DD and NDD simultaneously from mid FY2021 and will benefit from access to key NDD segment at launch, no HIF-PHI competition for 1-2 years, a global data set including CV outcome data and applying launch learnings from Japan

Sales Forecast *(Image)*



Patient segment	Pivotal study	Number of eligible patients*
Anemia associated with DD-CKD	EU HIMALAYAS PYRENEES SIERRAS ROCKIES JP 1517-CL-0302 1517-CL-0307 1517-CL-0308 1517-CL-0312	Diagnosed EU5: 210k JP: 295k Treated EU5: 196k JP: 282k
Anemia associated with NDD-CKD	EU ANDES ALPS DOLOMITES OLYMPUS JP 1517-CL-0310 1517-CL-0314	Diagnosed EU5: 1.8M JP: 408k Treated EU5: 1.3M JP: 216k

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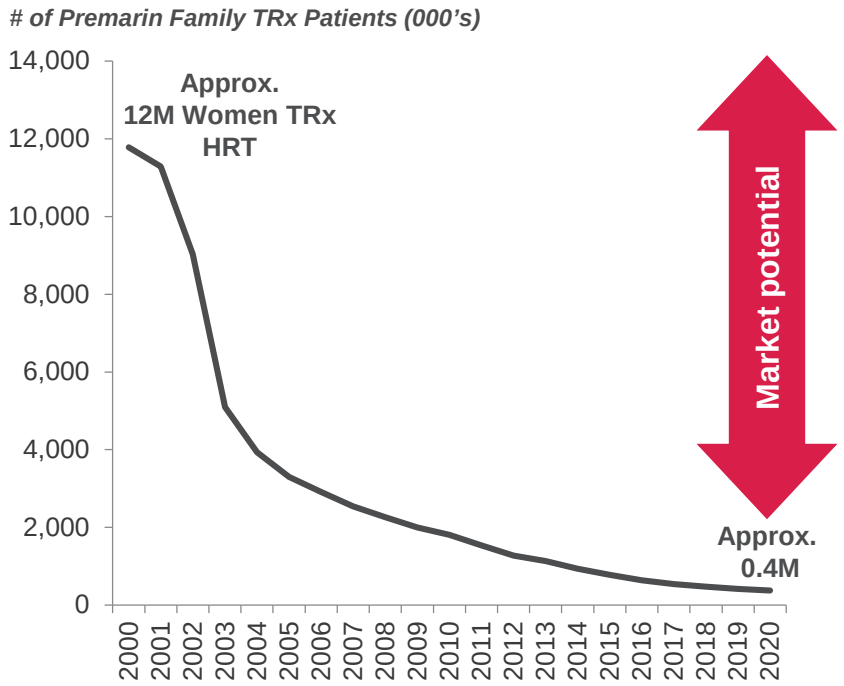
* Based on internal estimates
HIF-PHI: Hypoxia-inducible factor prolyl hydroxylase inhibitor, ESA: Erythropoiesis-stimulating agent, DD: Dialysis-dependent, NDD: Non-dialysis-dependent, CV: Cardiovascular, International Markets: Russia, Latin America, Middle East, Africa, South East Asia, South Asia, Korea, etc.

FEZOLINETANT FOR VMS ASSOCIATED WITH MENOPAUSE: HISTORY AND UNMET MEDICAL NEEDS IN US

- Approximately 12 million women in the US are impacted by moderate to severe VMS
- Decrease in number of women treated with HRT reflects the need for a safe, effective, non-hormonal treatment

Key events	Details
Prior to 2001, HRT was standard of care for VMS	• HRT was widely used for menopause symptoms including VMS for decades • 2000 MR-VMS market: Approx. 12M women treated per year
In 2001, Women's Health Initiative (WHI) fundamentally alters market	• Though effective in treating VMS, WHI links HRT to increased risk of breast cancer, coronary artery disease, stroke, and VTE • Many women are ineligible for or uncomfortable with HRT and its associated risks
No good replacement for HRT exists to treat VMS so women suffer in silence	• Even after introduction of new non-hormonal agent, the unmet medical needs still remain • Given the preference to avoid HRTs, patients may rely on lifestyle modifications or alternative medicine to adequately mitigate symptoms, many patients report little efficacy

US Annual Premarin Family treated Patients *



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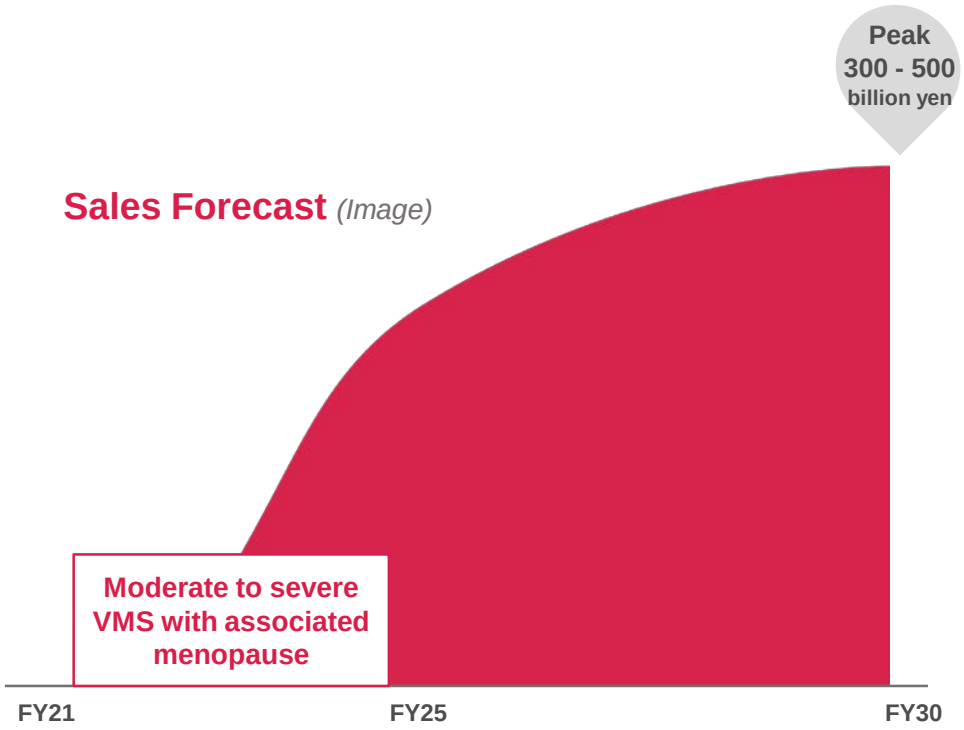
* IQVIA NPA - Premarin Family (Premarin, Prempro, Premphase)
(MR-)VMS: (Menopause-related) Vasomotor symptoms, HRT: Hormone replacement therapy, VTE: Venous thromboembolism, TRx: Total prescriptions

OUTLOOK FOR FEZOLINETANT

- Fezolinetant is a first-in-class, non-hormonal, selective NK3 receptor antagonist that works in the hypothalamus to reduce frequency and severity of VMS and has significant potential to become a leading treatment option for women experiencing moderate to severe VMS associated with menopause
- Enhanced confidence from the US and EU pivotal study results recently obtained



Two pivotal studies in US & EU

(SKYLIGHT 1 & SKYLIGHT 2)

12-week double-blind period data obtained:

- Met the coprimary endpoints at both doses (30 mg and 45 mg) at both timepoints (week 4 and week 12)
- No new safety signal of concern

Patient segment	Pivotal study	Number of eligible patients*
Patients with moderate to severe VMS associated with menopause	US & EU: **SKYLIGHT 1**, **SKYLIGHT 2**, **SKYLIGHT 4** (long-term) Asia: **MOONLIGHT 1**, **MOONLIGHT 3** (long-term)	US & EU: 22M JP & CN: 8M

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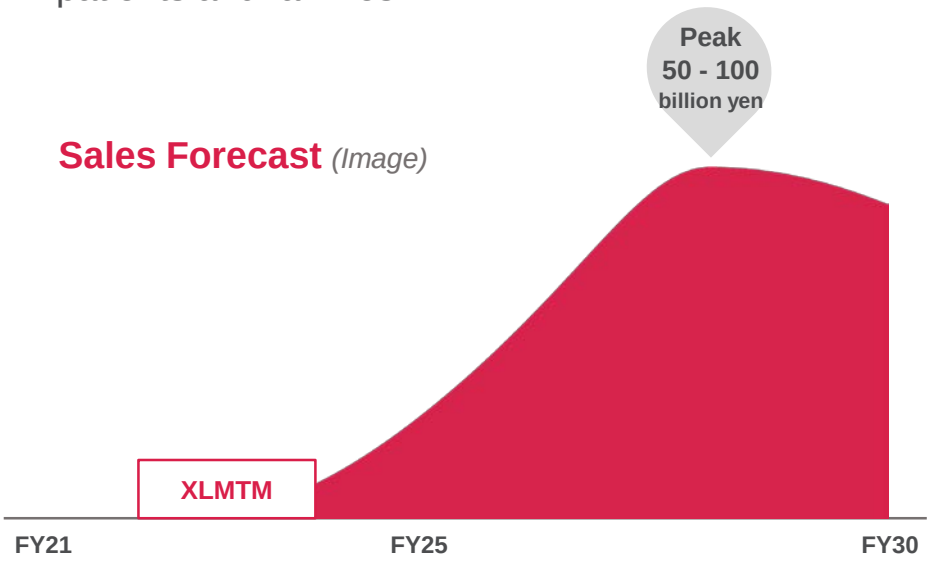
* Based on internal estimates
NK3: Neurokinin 3, VMS: Vasomotor symptoms

OUTLOOK FOR AT132 (RESAMIRIGENE BILPARVOVEC)

- There is significant unmet need for patients living with X-linked myotubular myopathy (XLMTM), a rare, life-threatening, monogenic neuromuscular disorder caused by mutations in the MTM1 gene
  - Approximately 1 in 40,000 to 50,000 newborn males, and 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 never achieved or substantially delayed
  - No disease-modifying treatment available; palliative care only
- AT132 is the first and only gene therapy that addresses the underlying cause of disease with a single infusion, enabling ventilator independence, improving motor function and enhancing the lives of patients and families

Sales Forecast *(Image)*



Patient Segment	Pivotal Study
XLMTM <5 years old	**ASPIRO**
XLMTM 5 to <18 years old	(Planned)

MTM: Myotubularin