

Results Presentation Fiscal 2023

協和キリン株式会社



KYOWA KIRIN

Agenda

2021-2025 Medium Term Business Plan

- FY2023 Review, Story for Vision 2030, FY2024 Plans, Revision of Financial KPI -
 Shareholders Return
 Commercial Update
 R&D Update
 News Flow
 Q&A

President and Chief Executive Officer **Masashi Miyamoto, Ph.D.**

Senior Managing Executive officer **Takeyoshi Yamashita, Ph.D.**

Managing Executive Officer, Head of Finance **Motohiko Kawaguchi**

Executive Officer, Head of Global Product Strategy **Tomohiro Sudo**

This document contains certain forward-looking statements relating to such items as the company's (including its domestic and overseas subsidiaries) forecasts, targets and plans. These forward-looking statements are based upon information available to the company at the present time and upon reasonable assumptions made by the company in making its forecasts, but the actual results in practice may differ substantially due to uncertain factors.

These uncertain factors include, but are not limited to, potential risks of the business activities in the pharmaceutical industry in Japan and overseas, intellectual property risks, risk of side effects, regulatory risks, product defect risks, risks of changes to the prices for raw materials, risks of changes to market prices, as well as risks of changes to foreign exchange rates and financial markets.

This document is used only for the purpose of providing the information to investors. Though it may contain the information concerning pharmaceutical products (including products under development), it is not for the purpose of promotion, advertising, or medical advice.

2021-2025 Medium Term Business Plan

- FY2023 Review -

Qualitative Review for FY2023



Provide pharmaceuticals for unmet medical needs

■ Maximize the value of global strategic products

- ✓ Initiated own sales of Crysvida in North America and keep steady growth
- ✓ Focused on Crysvida and Poteligeo and accelerated the growth under the new EMEA structure
- ✓ Further penetration of Crysvida

■ Continue to create groundbreaking new drugs

- ✓ Accelerated Global Development of rocatinlimab (over 2,400 patients has registered) and moved forward P2 clinical trial preparation for KHK4951
- ✓ Started P1 study of KK2260, an antibody with REGULGENT™ (our proprietary bispecific antibody technology)
- ✓ Received an approval for manufacturing and marketing PHOZEVEL®
- ✓ Discontinued the development of RTA 402 and KW-3357PE
- ✓ Had a definitive agreement to acquire Orchard Therapeutics

Reinforce human resources and structures that support the creation of Life-changing value

■ Cultivate human resources, Strengthen organizations, Build digital platforms, and Others

- ✓ Human resources and organization development and: Proceeded succession planning for global key positions, , Renewed talent management practices for the managers in Japan, Continued the effort to embed Corporate Culture Transformation “KABEGOE”
- ✓ DE&I: Reached the ratio of women in key positions under OKK structure to 30%, Rated “Gold” in the PRIDE Index 2023 (JP)
- ✓ Selected as a 2023 “Health & Productivity Stock” for consecutive two years (JP).
- ✓ Digital infrastructure improvement: Developed Dx human resource, Launched company-wide use of generated AI in-house environment, Improved efficiency and IT literacy with no-code and low-code tools (RPA, etc.)
- ✓ Strengthened the leadership structure with expanding CxO structure

Address patient-centric healthcare needs

■ Patient Advocacy

- ✓ Raised disease awareness of XLH and created and expanded opportunities for patient interactions
- ✓ Conducted disease awareness campaign with centering World Lymphoma Day and initiatives with patient organizations.
- ✓ Expanded disease awareness activities centered on Rare Disease Month
- ✓ Hosted an event at "Healthcare Café" organized by four JP pharmaceutical companies (Japan)

■ Provide value that goes beyond pharmaceuticals

- ✓ Had an initiative to address patient needs related to XLH/TIO with Ubie.
- ✓ Planned to address patient needs related to XLH

Retain the trust of society

■ Ensure stable supplies of high-quality pharmaceuticals

- ✓ Proceeded to establish the key products supply system with multiple production sites
- ✓ Full operation of Takasaki Q-TOWER (quality assurance-related complex)
- ✓ Addressed human rights issues for all stakeholders through membership in JaCER*1

■ Help to protect the global environment

- ✓ Reduced CO₂ emissions by 54% compared to 2019 with using renewable energy
- ✓ Disclosed necessary information responding to Assembly Bill No. 1305*2 California State Law, USA
- ✓ Completed construction of Ube Plant office building with ZEB*3 certification

*1: General Incorporated Association, Japan Center for Engagement and Remedy on Business and Human Rights

*2: Mandatory Climate Change Disclosure for California-based Companies

*3: Abbreviation for Net Zero Energy Building, a building that aims to reduce energy consumption to zero while achieving a comfortable indoor environment.

Quantitative Summary of FY23 Results

(Billion Yen / Rounded)

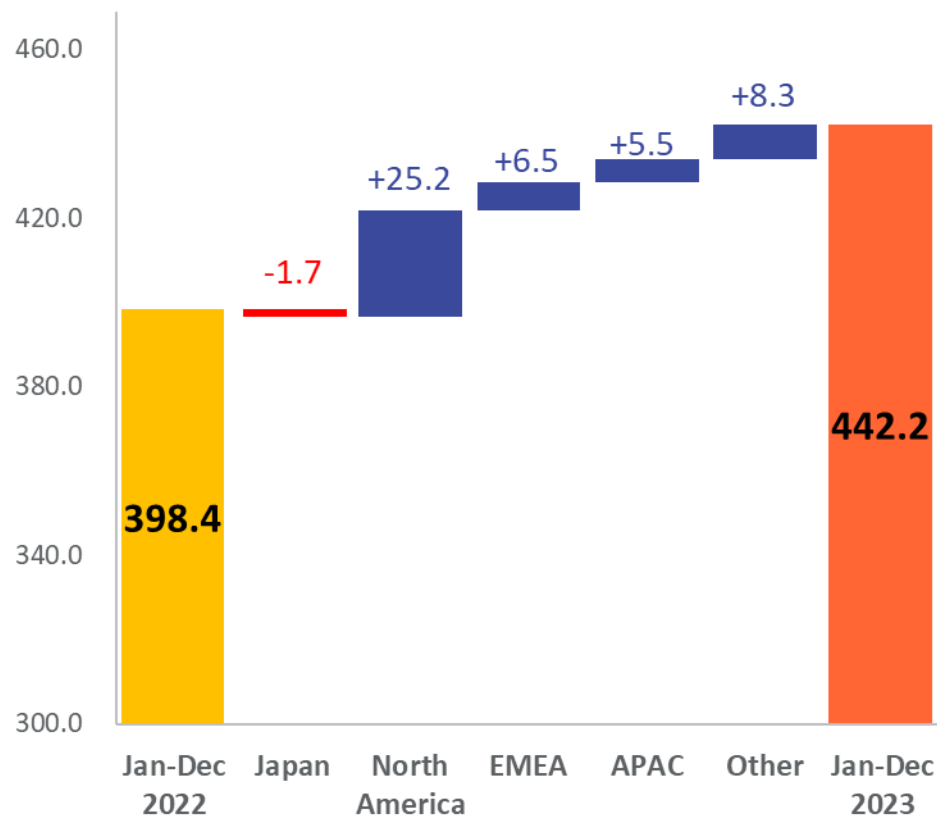
	FY2022 Results	FY2023 Results	Changes	2023 Revised Plans	Achieved
Revenue <i>[Overseas Ratio]</i>	398.4 <i>[61%]</i>	442.2 <i>[65%]</i>	+43.9 (+11%)	426.0 <i>[64%]</i>	104%
Gross Profit <i>[Gross Profit Margin]</i>	311.5 <i>[78%]</i>	331.0 <i>[75%]</i>	+19.6 (+6%)	326.0 <i>[77%]</i>	102%
SG&A <i>[SG&A Ratio]</i>	166.2 <i>[42%]</i>	163.1 <i>[37%]</i>	-3.1 (-2%)	162.0 <i>[38%]</i>	101%
R&D <i>[R&D Ratio]</i>	62.9 <i>[16%]</i>	72.1 <i>[16%]</i>	+9.2 (+15%)	79.0 <i>[19%]</i>	91%
Gain/Loss on Equity Method	4.3	0.9	-3.4 (-78%)	3.0	31%
Core Operating Profit <i>[Core OP Margin]</i>	86.7 <i>[22%]</i>	96.8 <i>[22%]</i>	+10.1 (+12%)	88.0 <i>[21%]</i>	110%
Profit	53.6	81.2	+27.6 (+52%)	76.0 → 70.0	116%
Return on Equity	7.1%	10.2%			
Dividend Payout Ratio ¹	38.9%	35.5%			

[FOREX]
 FY2022-Actual JPY130/USD
 FY2023-Actual JPY140/USD
 FY2023-Rev. Plan JPY130/USD

¹ Figures are based on Core-EPS (EPS calculated using “Core profit,” profit without other income/losses and related taxes).

FY22 vs FY23 -Revenue-

+43.9 billion yen
(incl. forex effect +18.9)



● Japan -1.7

Although Duvroq, Romiplate, and Crysvida increased, revenue in Japan region decreased by 1% due mainly to negative impact by annual NHI price-cut and shrink in Nesp-AG affected by competitive products.

● North America +25.2 (incl. forex effect +9.1)

Revenue in North America region increased by 22% with the growth of Crysvida (+21%), Poteligeo (+25%), and Nourianz (+27%).

● EMEA +6.5 (incl. forex effect +6.3)

Revenue in EMEA region increased by 10% due to the growth of Crysvida (+14%) and Poteligeo (+36%), and transfer of global rights for Tostran to ADVANZ PHARMA by £62.5M (JPY11.5B), although decrease sales of Established medicines, such as Abstral by entered into the Joint Venture Collaboration with Grünenthal on Aug 1.

● APAC +5.5 (incl. forex effect +1.5)

APAC revenue increased by 18% with the growth of Crysvida, and Nesp, while Gran was down due to the Chinese national tender system.

● Other +8.3 (incl. forex effect +2.1)

21% growth in the other revenue was due to the royalties of growing Fasenra (Benralizumab).

FY22 vs FY23 -Revenue of Major Items in Japan-

(Billion Yen / Rounded)

Item	FY2022 Results	FY2023 Results	Changes	Reasons	2023 Plans	Achieved
Nesp + Nesp-AG ¹	21.1	17.1	-3.9 (-19%)	NHI price-cut & Biosimilars' penetration	16.6	103%
Nesp	3.4	3.2	-0.3 (-8%)		2.8	112%
Nesp-AG	17.6	14.0	-3.6 (-21%)		13.8	101%
Duvroq	6.6	9.9	+3.4 (+51%)	Market penetration (Launched in Aug 2020)	7.8	127%
Orkedia	10.3	10.6	+0.3 (+3%)		11.2	95%
G-Lasta	31.1	31.9	+0.9(+3%)	'BodyPod' launched in Dec 2022	33.5	95%
Poteligeo	2.0	1.9	-0.0 (-0%)		2.0	98%
Rituximab BS	10.3	9.0	-1.2 (-12%)	NHI price-cut	8.7	104%
Romiplate	10.4	12.0	+1.5 (+15%)	Market penetration (New indication in Jun 2019)	11.2	106%
Allelock	6.0	5.5	-0.5 (-8%)	NHI price-cut	4.7	117%
Nouriast	8.0	7.6	-0.5 (-6%)	Competitors' penetration	7.5	100%
Haruropi	4.0	4.5	+0.5 (+12%)	Market penetration (Launched in Dec 2019)	4.7	95%
Crysvita	8.9	10.5	+1.6 (+18%)	Market penetration (Launched in Dec 2019)	11.1	95%

1 AG stands for Authorized Generic. Official product name is Darbeopetin Alfa [KKF]. Kyowa Kirin Frontier is a marketing authorization holder; Kyowa Kirin is a distributor.

FY22 vs FY23 -Revenue of Major Items outside Japan-

(Billion Yen / Rounded)

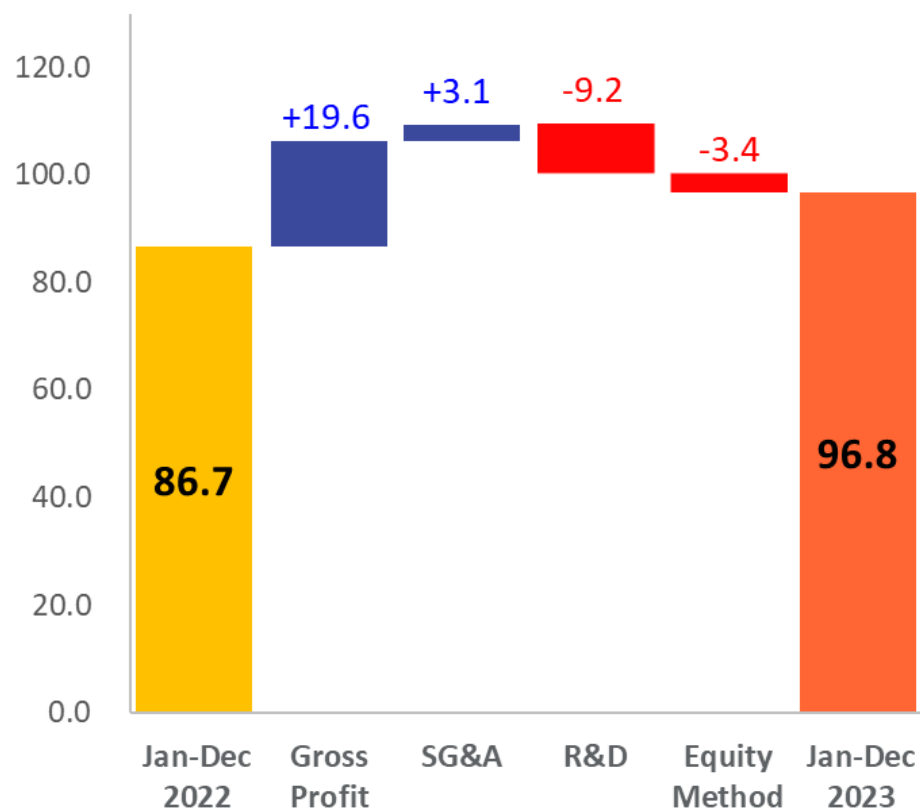
Item	FY2022 Results	FY2023 Results	Changes	Reasons	2023 Plans	
					2023 Plans	Achieved
Crysvita	118.2	142.0	+23.7 (+20%)	[North America] Market penetration [EMEA] Geographical expansion & Additional indication (Adult/TIO) [APAC] Geographical expansion	138.0	103%
North America	87.0	105.2	+18.2 (+21%)			
EMEA	31.0	35.1	+4.2 (+14%)			
APAC	0.3	1.6	+1.3 (+457%)			
Poteligeo	22.3	28.4	+6.1 (+27%)	[North America] Market penetration [EMEA] Geographical expansion & Market penetration	27.5	103%
North America	17.2	21.5	+4.3 (+25%)			111%
EMEA	5.1	6.9	+1.8 (+36%)			87%
APAC	-	0.0	+0.0 (- %)			7%
Nourianz	6.5	8.2	+1.8 (+27%)	Market penetration	7.5	109%
Nesp	7.6	9.1	+1.5 (+20%)	Market penetration (Korea, Taiwan)	8.0	114%
Gran	8.2	6.9	-1.3 (-15%)	Listed on Chinese tender list	8.2	84%
Neulasta	5.6	5.7	+0.0 (+1%)		5.7	100%
Tech-licensing	33.0	40.7	+7.7 (+23%)	Growth of Fasenra and Fotivda ²	39.0	104%
Benralizumab Royalty ¹	21.6	27.4	+5.8 (+27%)			

1 Sales royalties of Fasenra which has been marketed by AstraZeneca. Including our own estimation.

2 tivozanib: Kyowa Kirin has licensed its oncology development/commercial rights to Aveo Oncology Inc (an LG Chem company), and Aveo has sub-licensed the rights outside North America to EUSA Pharma. Being marketed in the US and Europe as Fotivda.

FY22 vs FY23 -Core OP-

**+10.1 billion yen
(incl. forex effect +6.5)**



- **Gross Profit +19.6 (incl. forex effect +16.5)**

Increased in conjunction with the 43.9 rise in revenue. COGs have increased due to the North America Crysvida Sales royalty after Apr 27, 2023. Hence, gross profit % declined YoY. (78.2% →74.9%) .

- **SG&A +3.1 (incl. forex effect -8.2)**

While the Crysvida commercial operation expenses are increased in North America, such as HR exp, decreased in Crysvida profit sharing expenses due to the North America Crysvida-related scheme change after Apr 27, 2023.

[Labor -7.3 / Sales promotion -17.5 (incl. Crysvida profit-share expenses +21.8)]

- **R&D -9.2 (incl. forex effect -1.8)**

Increased in clinical study costs of KHK4083 which is undergoing joint global Phase III clinical study for Atopic Dermatitis indication.

- **Gain/Loss on Equity Method -3.4**

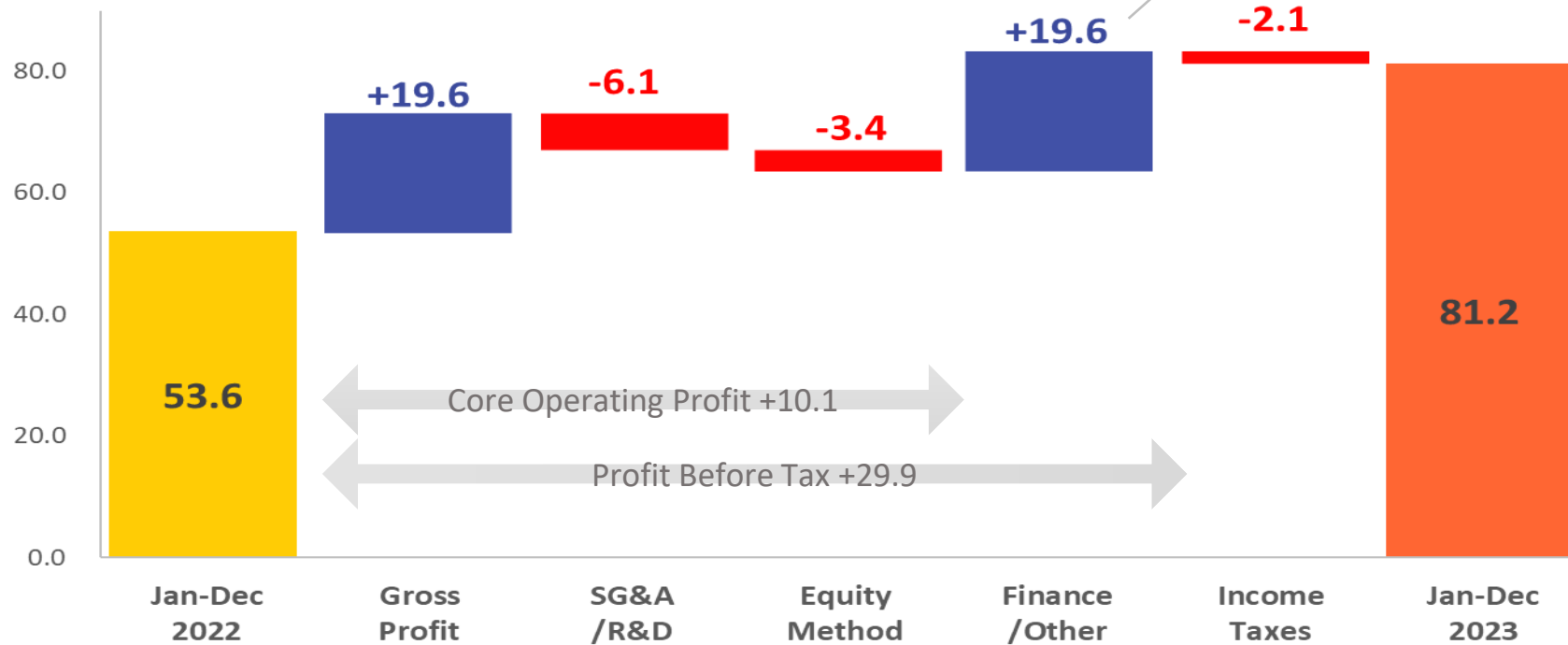
While revenue of Hulio (FKB327/Adalimumab biosimilar) increased, FKB's profit decreased due to the impact of reversal of deferred tax assets.

FKB; Fujifilm Kyowa Kirin Biologics Co., Ltd.

FY22 vs FY23 -Profit-

Profit (Jan-Dec) +27.6 billion yen

- EMEA established medicines portfolio:
Gain on sales of 51% shares plus gain on valuation of remaining 49% shares +14.8
- Impairment losses +7.1 [FY22] ME-401 IPR&D 14.3, irofotase alfa IPR&D 3.2 [FY23] RTA 402 8.3, Ardelyx 1.1
- Foreign exchange gain +1.6 (2.6 → 4.2)
- Business restructuring expenses -3.9 (-2.4 → -6.2)
- Provision for loss on contracts -1.4 [FY23] closing costs: RTA 402 -2.0, ME-401 -0.8



2021-2025 Medium Term Business Plan

- Story for Vision 2030 -

Our Vision toward 2030

Our Vision toward 2030

Kyowa Kirin will realize the successful creation and delivery of life-changing value* that ultimately makes people smile, as a Japan-based Global Specialty Pharmaceutical company built on the diverse team of experts with shared passion for innovation.

Provide pharmaceuticals for unmet medical needs

We are focused on developing medicines for diseases where there is a clear patient need for new options. We make full use of multiple therapeutic modalities, including biotechnology such as antibody technology, and beyond, building on our Kyowa Kirin established strengths.

Address patient-centric healthcare needs

We will meet the needs of patients and society by providing value across the entire patient care pathway, delivering cutting-edge science and technology, grounded in our in-depth pharmaceutical knowledge and expertise.

Retain the trust of society

We pursue world-class product quality and operational excellence to grow our business in ways which build long-term trust with our stakeholders.



* Make patients smile through dramatic improvements in quality of life by identifying the unmet medical needs of people battling with medical conditions and by creating and supplying new drugs or services that help them overcome those challenges.

Environmental changes since 2021-2025MTBP planning

Internal Change

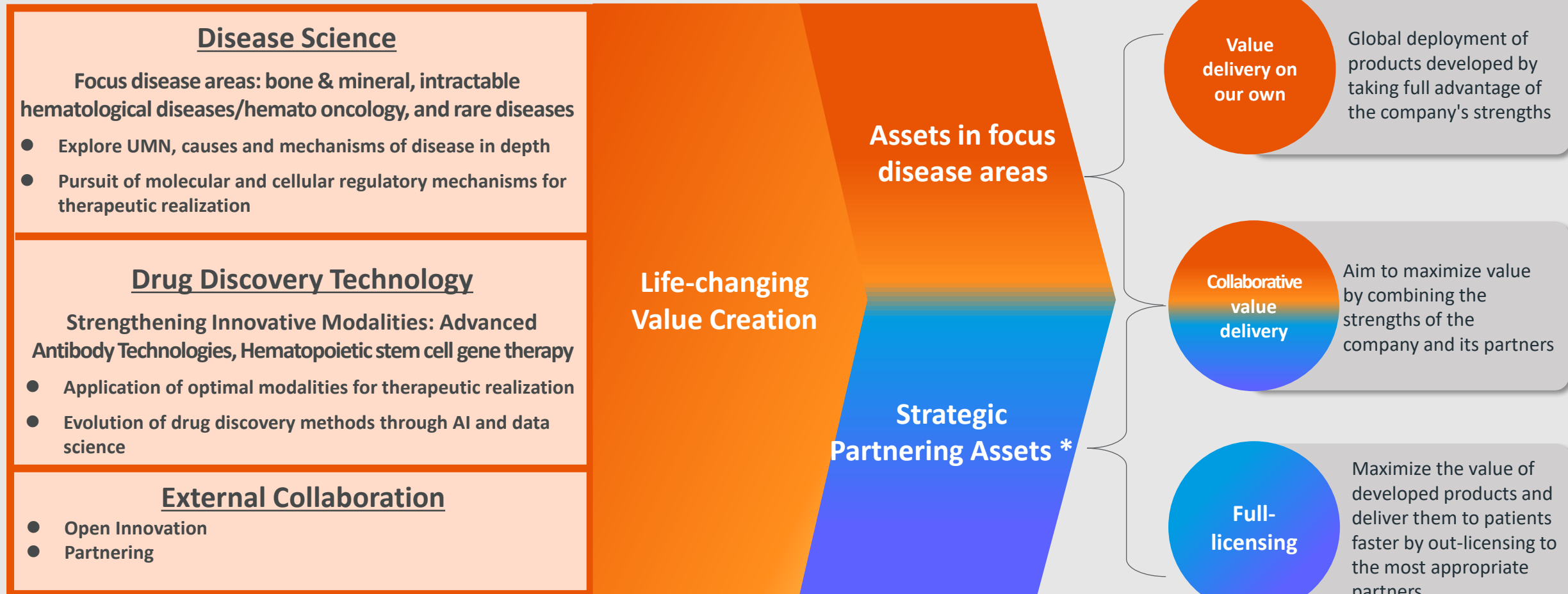
- Established global our own sales and marketing system for orphan drugs such as Crysvita and Poteligeo.
- Bone & mineral, intractable hematological diseases/hemato oncology, and rare diseases are becoming disease areas of strength.
- Acquired the modality of hematopoietic stem cell gene therapy, which has the potential to eliminate the underlying causes of inherited diseases.
- Portfolio changes due to rocatinlimab value maximization, new asset acquisitions and development discontinuations.
- Through the One Kyowa Kirin structure, an organization that can grow by incorporating diverse skills and structures.

External Change

- Global trend of tighter policies and regulations to reduce health care costs.
- Growing demand for cure or controlled progression and for practical application of new modalities to meet those demand.
- Decrease of druggable targets that can be addressed by monoclonal antibodies or small molecules.
- The entire pharmaceutical industry, including Megapharma, is adopting a strategy of focusing on pharmaceutical business and narrowing their focus disease areas.

Story for Vision 2030

Strategies for creating and delivering life-changing value



*Assets outside of the disease areas of focus are designated as strategic partnering assets, and value maximization is achieved through collaboration with partners.

2021-2025 Medium Term Business Plan

- FY2024 Plans -

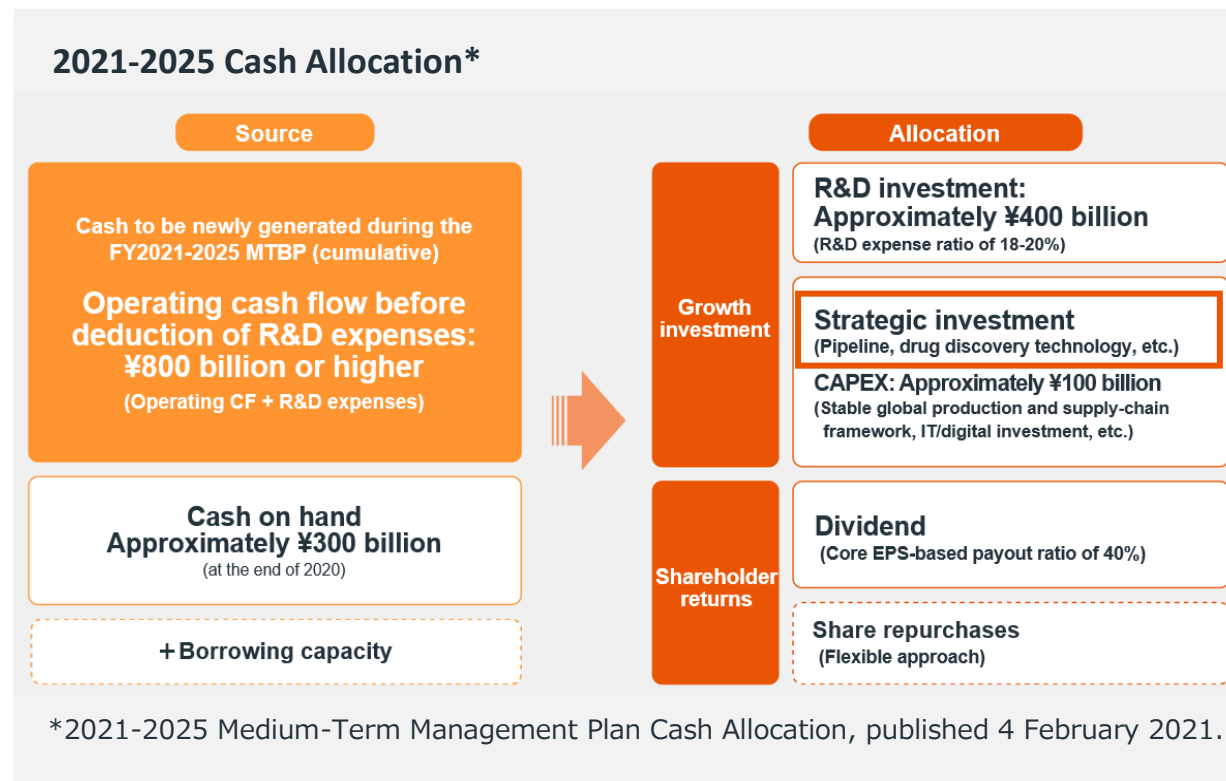
Strategic Investment ~ For successful creation and delivery of life-changing value

Licensing-in and M&A investments to strengthen the portfolio

- Priority will be given to the focus disease areas
 - bone & mineral
 - intractable hematological diseases/hemato oncology
 - rare diseases

Investment in science and technology to create new strengths

- Investments aimed at acquiring new drug discovery technologies and early pipelines and accelerating cooperation and collaborations
- VC investment and CVC activities for exploring and accessing information.



Qualitative Plans for FY2024

Provide pharmaceuticals for unmet medical needs

■ Maximize the value of global strategic products

- ✓ Conduct evidence-based marketing to penetrate into patients.
- ✓ Consider LCM to provide further value and strengthen areas of our global product.

■ Continue to create groundbreaking new drugs

- ✓ Expand target indications for rocatinlimab (asthma and prurigo nodularis)
- ✓ Move forward clinical trials for KHK4951, KK4277, KK2260 & KK2269 (an antibody with REGULGENT™, our proprietary bispecific antibody technology), and KK2845 (ADC).
- ✓ Respond to regulatory requirements for OTL-200 approval in the US and prepare for the market launch.
- ✓ Explore strategic investment opportunities based on Story for Vision 2030.

Reinforce human resources and structures that support the creation of Life-changing value

■ Cultivate human resources, Strengthen organizations, Build digital platforms, and Others

- ✓ Accelerate human resource development: Support independent career development and learning, expand use of human resource systems.
- ✓ Continue "KABEGOE" activities for corporate culture reform.
- ✓ Strengthen digital infrastructure: Dx human resource development, optimize enterprise architecture of IT infrastructure.
- ✓ Strengthen the leadership structure: Promote leadership with centering CxO.

Address patient-centric healthcare needs

■ Patient Advocacy

- ✓ Develop evidences through XLH real world data.
- ✓ Expand Shine a Light campaign area to raise XLH disease awareness.
- ✓ Conducted disease awareness campaign for CTCL with patient organizations.

■ Provide value that goes beyond pharmaceuticals

- ✓ Continue to address patient needs related to XLH.
- ✓ Create additional value by strengthening collaboration with Kirin Group.

Retain the trust of society

■ Ensure stable supplies of high-quality pharmaceuticals

- ✓ Strengthen initiatives to establish steady products supply system globally.
- ✓ Cooperate with domestic and overseas contract manufacturers for operation management.
- ✓ Enhance sustainable supply initiatives.
- ✓ Build a biopharmaceutical API manufacturing building (HB7) (plan to complete in March 2025).

■ Help to protect the global environment

- ✓ Reduce CO₂ emissions by 51% compared to 2019.
- ✓ Take initiatives to reduce greenhouse gas in Scope 3.

Quantitative Summary of FY24 Plans

(Billion Yen / Rounded)

	FY2022 Results	FY2023 Results	FY2024 Plans	Changes	Orchard	FY2024 Plans Excl. Orchard
Revenue <i>[Overseas Ratio]</i>	398.4 <i>[61%]</i>	442.2 <i>[65%]</i>	473.0 <i>[70%]</i>	+30.8 (+7%)	4.5	468.5 <i>[70%]</i>
Gross Profit <i>[Gross Profit Margin]</i>	311.5 <i>[78%]</i>	331.0 <i>[75%]</i>	348.0 <i>[74%]</i>	+17.0 (+5%)	3.0	345.0 <i>[74%]</i>
SG&A <i>[SG&A Ratio]</i>	166.2 <i>[42%]</i>	163.1 <i>[37%]</i>	166.0 <i>[35%]</i>	+2.9 (+2%)	8.0	158.0 <i>[34%]</i>
R&D <i>[R&D Ratio]</i>	62.9 <i>[16%]</i>	72.1 <i>[16%]</i>	100.0 <i>[21%]</i>	+27.9 (+39%)	10.0	90.0 <i>[19%]</i>
Gain/Loss on Equity Method	4.3	0.9	3.0	+2.1 (+218%)	-	3.0
Core Operating Profit <i>[Core OP Margin]</i>	86.7 <i>[22%]</i>	96.8 <i>[22%]</i>	85.0 <i>[18%]</i>	-11.8 (-12%)	-15.0	100.0 <i>[21%]</i>
Profit	53.6	81.2	63.0	-18.2 (-22%)	-11.0	74.0
Return on Equity	7.1%	10.2%	7.6%			
Dividend Payout Ratio ¹	38.9%	35.5%	47.6%			

[FOREX]
 FY2022-Actual JPY130/USD
 FY2023-Actual JPY140/USD
 FY2024-Plan JPY140/USD

¹ Figures are based on Core-EPS (EPS calculated using "Core profit," profit without other income/losses and related taxes).

FY24 -Revenue of Major Items in Japan-

(Billion Yen / Rounded)

Item	FY2022 Results	FY2023 Results	FY2024 Plans	Changes	Reasons
Nesp + Nesp-AG ¹	21.1	17.1	14.4	-2.7 (-16%)	NHI price-cut & Biosimilars' penetration
Nesp	3.4	3.2	2.8	-0.4 (-13%)	
Nesp-AG	17.6	14.0	11.7	-2.3 (-17%)	
Duvroq	6.6	9.9	12.2	+2.3 (+23%)	Market penetration (Launched in Aug 2020)
Phozevel	-	-	3.3	+3.3 (-)	Launch in Feb 2024 (Plan)
Orkedia	10.3	10.6	11.7	+1.1 (+10%)	Market penetration
G-Lasta	31.1	31.9	20.5	-11.4 (-36%)	Biosimilars' penetration & NHI price-cut
Poteligeo	2.0	1.9	1.9	-0.0 (-1%)	
Rituximab BS	10.3	9.0	7.9	-1.1 (-12%)	NHI price-cut
Romiplate	10.4	12.0	13.2	+1.2 (+10%)	Market penetration
Nourias	8.0	7.6	7.1	-0.4 (-6%)	Competitors' penetration
Haruropi	4.0	4.5	5.2	+0.7 (+15%)	Market penetration (Launched in Dec 2019)
Crysvita	8.9	10.5	12.9	+2.4 (+23%)	Market penetration (Launched in Dec 2019)

1 AG stands for Authorized Generic. Official product name is Darbepoetin Alfa [KKF]. Kyowa Kirin Frontier is a marketing authorization holder; Kyowa Kirin is a distributor.

FY24 -Revenue of Major Items outside Japan-

(Billion Yen / Rounded)

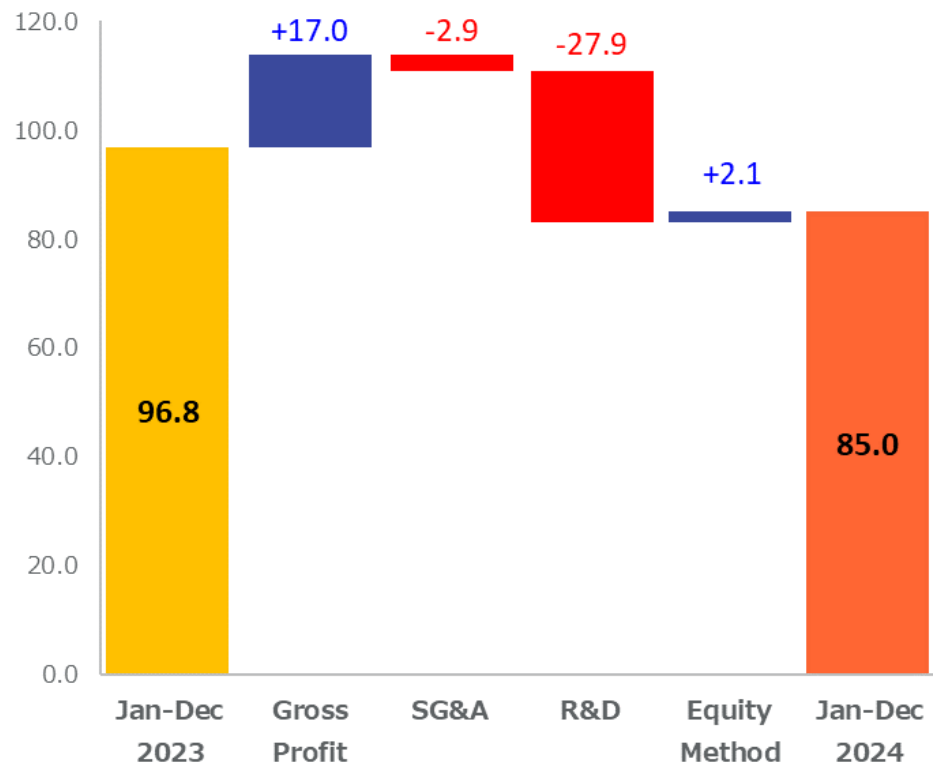
Item	FY2022 Results	FY2023 Results	FY2024 Plans	Changes	Reasons
Crysvita	118.2	142.0	175.9	+33.9 (+24%)	[North America] Market penetration [EMEA] Geographical expansion & Additional indication
North America	87.0	105.2			
EMEA	31.0	35.1			
APAC	0.3	1.6			
Poteligeo	22.3	28.4	32.5	+4.1 (+15%)	[North America] Market penetration [EMEA] Geographical expansion & Market penetration
North America	17.2	21.5	23.3	+1.8 (+8%)	
EMEA	5.1	6.9	8.8	+1.8 (+26%)	
APAC	-	0.0	0.5	+0.5 (+3,446%)	
Nourianz	6.5	8.2	8.5	+0.2 (+3%)	
Libmeldy (OTL-200)	-	-	4.5	+4.5 (-)	
Nesp	7.6	9.1	10.7	+1.6 (+18%)	Market penetration in China
Gran	8.2	6.9	7.2	+0.2 (+3%)	
Tech-licensing	33.0	40.7	45.0	+4.3 (+11%)	Growth of Fasenra and Fotivda ²
Benralizumab Royalty ¹	21.6	27.4			

1 Sales royalties of Fasenra which has been marketed by AstraZeneca. Including our own estimation.

2 tivozanib: Kyowa Kirin has licensed its oncology development/commercial rights to Aveo Oncology Inc (an LG Chem company), and Aveo has sub-licensed the rights outside North America to EUSA Pharma. Being marketed in the US and Europe as Fotivda.

FY2023 vs FY2024 -Core OP-

-11.8 billion yen



● Gross Profit +17.0

While gross profit will grow with the 30.8 rise in revenue (Japan-12.9, North America +27.7, EMEA+1.2, APAC+4.8, Other+9.8 (Orchard+4.5)), COGs will also increase due to the Crysvida-related scheme change from ‘Profit-sharing (SG&A)’ to ‘Sales royalty (COGs)’ after Apr 27, 2023. This is expected to result in lowered gross profit margin by about 1.5% (74.9% → 73.6%)

● SG&A -2.9

Although, profit-share expenses on Crysvida (CRV) are no longer being incurred April 27, 2023 onward, 8.0 will be increased for Orchard consolidation.

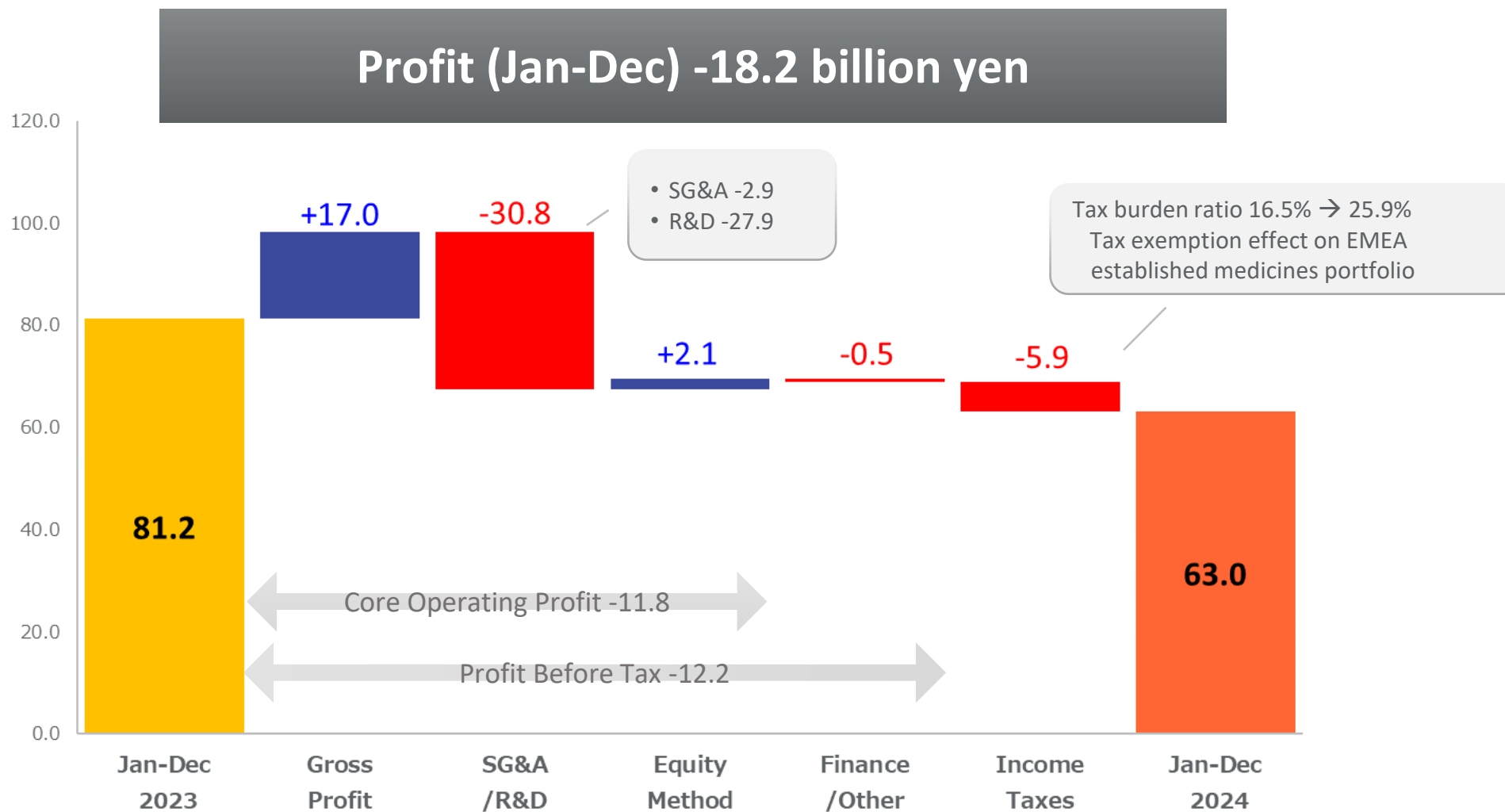
● R&D -27.9

R&D expenses are expected to significantly increase and reach the level of 100 B yen due mainly to full-scale operation of rocatinlimab Ph3 ROCKET program and Orchard consolidation. R&D expense ratio is to increase by approx. 5% (16.3%→21.1%)

● Gain/Loss on Equity Method +2.1

Equity method gain is expected to increase by 2.1 due to removal of the impact of reversal of deferred tax assets

FY2023 vs FY2024 -Profit-



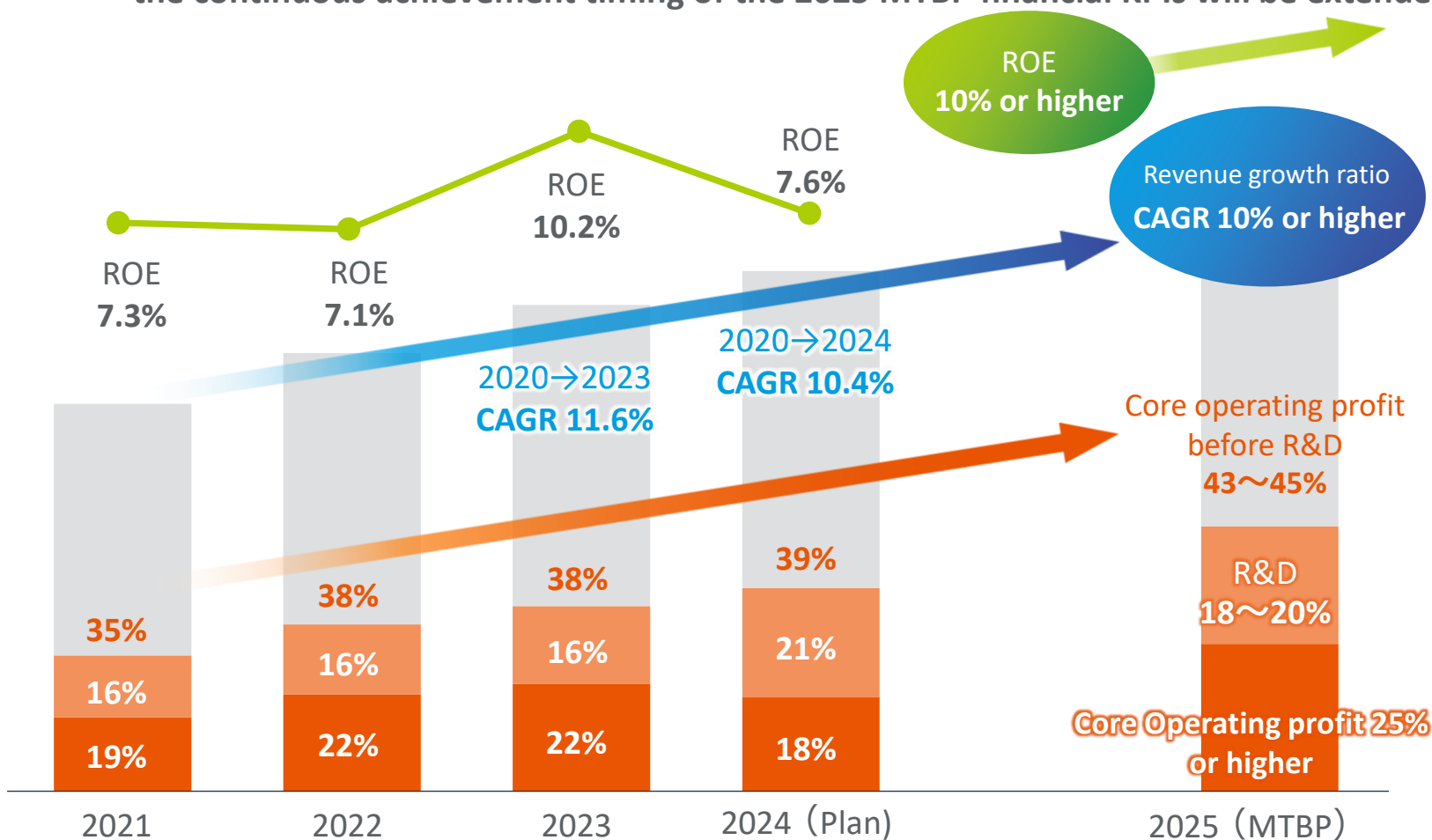
2021-2025 Medium Term Business Plan

- Revision of Financial KPI -

2021-2025 Medium Term Business Plan

- Revision of Financial KPI -

- Record high in Core operating profit for FY2023. Achieved KPIs such as “ROE of 10%” and “CAGR of 10% or higher”
- Due to the restructuring of our business model to adapt to environmental changes, the continuous achievement timing of the 2025 MTBP financial KPIs will be extended to 2026 or beyond.



2025 MTBP financial KPIs
Achievement timing will be 2026 or beyond

- +**
- Steady growth in Crysvita sales
 - Collaboration with Amgen on KHK4083
 - Depreciation of Yen

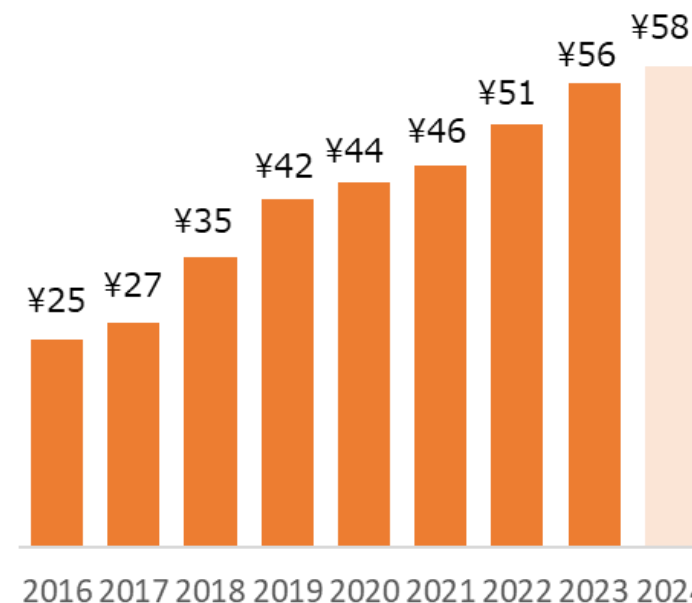
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- Short term financial impact on Orchard acquisition
 - Increasing investment in KHK4083 development
 - Depreciation of Drug price environment (Japan, Europe, and China)
 - Unlaunched new products (discontinued pipelines, Nourianz in Europe)

Shareholders Return

Shareholders Return

- ✓ FY23 dividend is **56 yen**, and FY24 to be **58 yen** (plan)
- ✓ Plans **8-year consecutive rises** since FY17
- ✓ FY21-24 weighted average payout ratio is **40.8%** (plan)
(Mid-term guidance for payout ratio “Targeting sustained dividend hikes with 40%”)

Year	Dividend (yen)		Payout Ratio ^{*1}	Return on Equity	
	Interim	Year-end			
2016	12.50	12.50	25.00	44.9%	5.3%
2017	12.50	14.50	27.00	34.4%	7.2%
2018	15.00	20.00	35.00	35.2%	8.6%
2019 ^{*2}	20.00	22.00	42.00	33.7%	10.1%
2020	22.00	22.00	44.00	50.3%	6.8%
2021	23.00	23.00	46.00	43.2%	7.3%
2022	24.00	27.00	51.00	38.9%	7.1%
2023 ^{*3}	27.00	29.00	56.00	35.5%	10.2%
2024 Plan ^{*4}	29.00	29.00	58.00	47.6%	7.6%



^{*1} Payout ratio for FY2021/beyond are payout ratios against the Core EPS that is calculated based on the Core Earnings (= Profit - Other income/losses - Related income taxes)

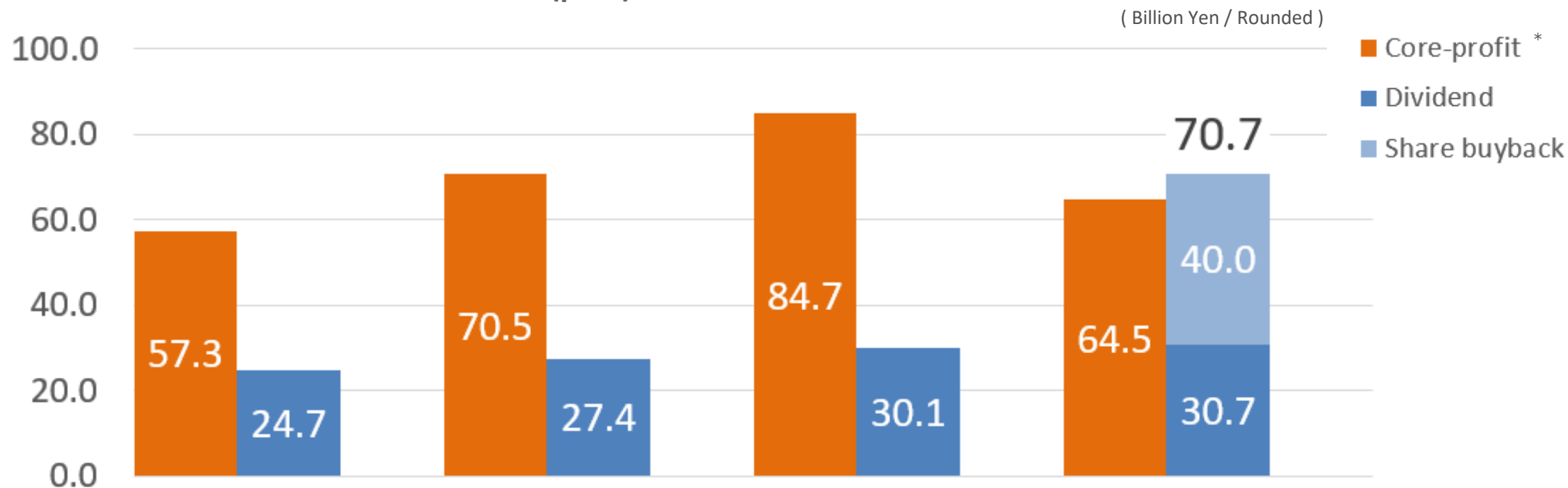
^{*2} Repurchase of 10.7M own shares (¥22.6B) executed on February 6, 2019. Total return ratio for FY2019 is 67.3%.

^{*3} Year-end dividend of 29 yen/share will be submitted to the 101st Ordinary General Meeting of Shareholders to be held on March 22, 2024.

^{*4} Total return ratio for FY2024 will be 109.5% (planned) assuming a share buyback of ¥40billion.

Share buyback and cancellation of treasury shares

- ✓ To improve capital efficiency and shareholder returns, the company has resolved share buyback and cancellation of treasury shares
- ✓ Up to 40.0 billion JPY, largest ever (Up to 17 million shares)
- ✓ Total return ratio for FY2024 will be 109.5% (plan)



	2021	2022	2023	2024(Plan)
Dividend (Yen/share)	46.0	51.0	56.0	58.0
Payout ratio	43.2%	38.9%	35.5%	47.6%
Total return ratio	43.2%	38.9%	35.5%	109.5%

Commercial Update

2023 Review & 2024 Key Actions

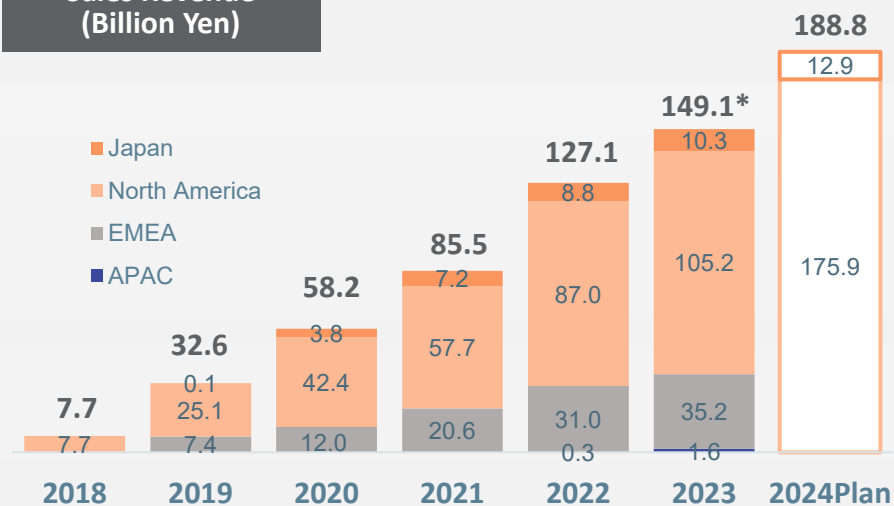
2023 Review

- North America:
Started own sales (Established and started own operation of the direct sales force)
Steady growth continued to be achieved through seamless sales transfers.
- EMEA
Expanded markets geographically. Started commercialization for adult XLH in Italy and Spain. Maintained growth through patient penetration despite drug price reduction in Germany.
- Japan
Strengthened promotional activities by increasing the dedicated personnel.

2024 Key Actions

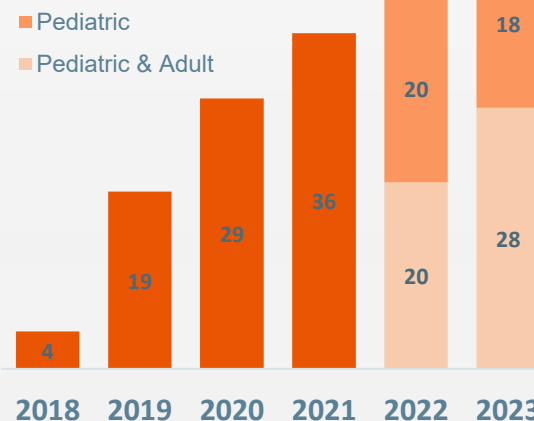
- Strengthen evidence-based marketing activities.
- North America:
Enhance disease awareness activities. Strengthen further the foundation of the own sales structure.
- EMEA:
Continue to focus on geographical & indication expansion. Increase market penetration in adult XLH.
- Japan:
Further strengthen promotional activities by the dedicated personnel to accelerate growth.

Sales Revenue (Billion Yen)



*Revenue from EAP (Early Access Program) is not included in sales until FY2022, and is included in sales from FY2023 onwards as it is insignificant in monetary terms.

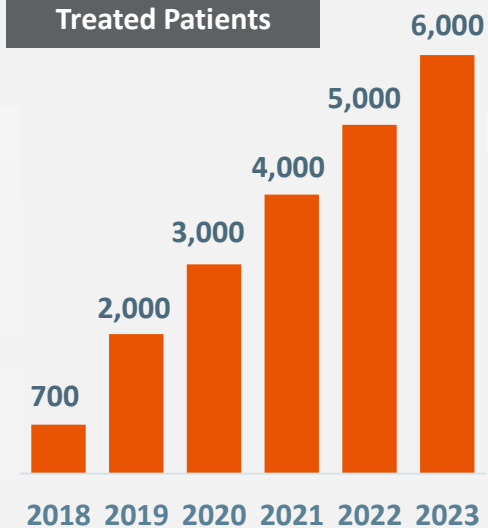
Launched Countries / Regions (XLH)



*Excludes Latin America and Turkey, where Ultragenyx records sales.

*The numbers of treated patients is an approximate number based on our calculations.

Treated Patients



2023 Review & 2024 Key Actions

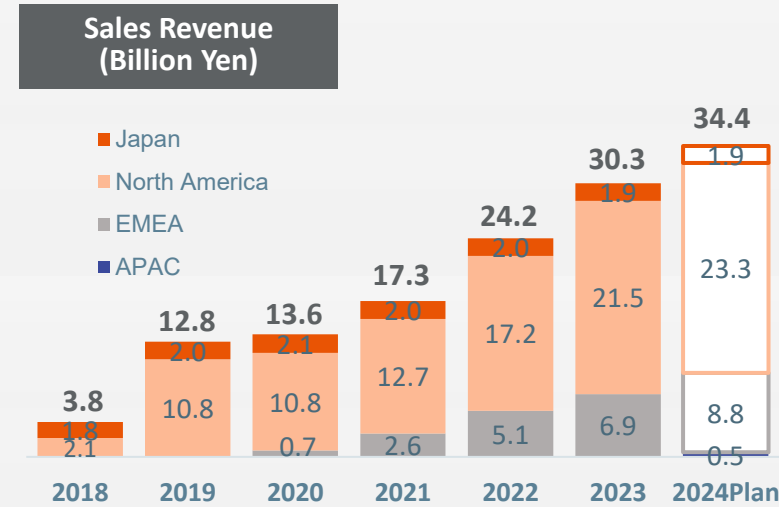
2023 Review

- Utilizing evidences including blood compartment
- Raising awareness of importance of blood testing among early-stage patients
- North America: Promotional activities based on medical data
- EMEA/ Asia: Launched countries/regions increased (+15).

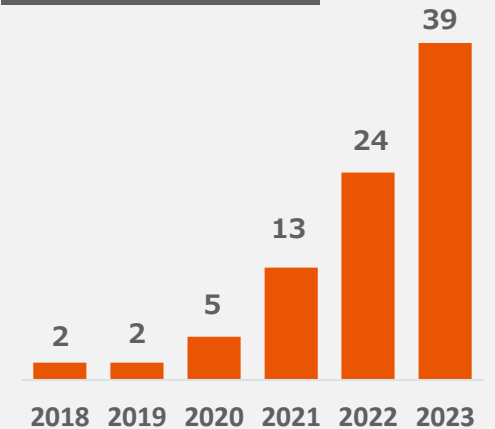
2024 Key Actions

- Deeper penetration into the existing markets as well as expansion of targets through further progression of evidence-based promotional activities.

*Revenue from EAP (Early Access Program) is not included in sales until FY2022, and is included in sales from FY2023 onwards as it is insignificant in monetary terms.



Launched Countries / Regions



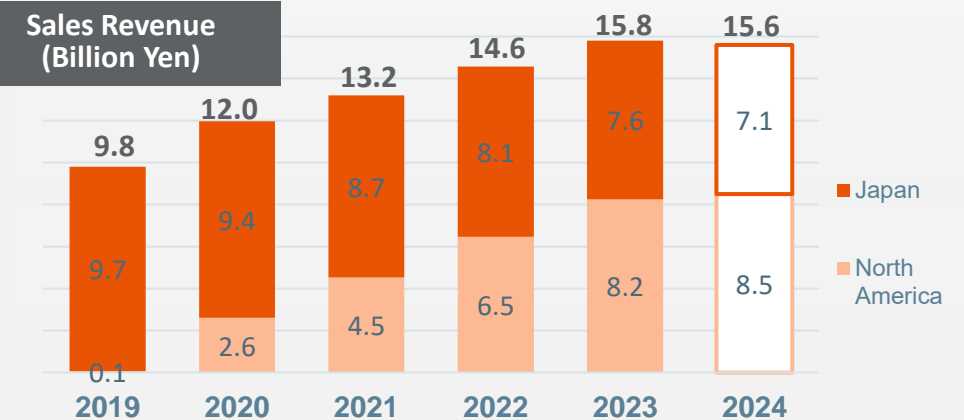
2023 Review & 2024 Key Actions

2023 Review

- North America: Steadily grew to exceed revenue goal driven by promotional activities resulting in all-time highs in total Rx's and total prescribers.

2024 Key Actions

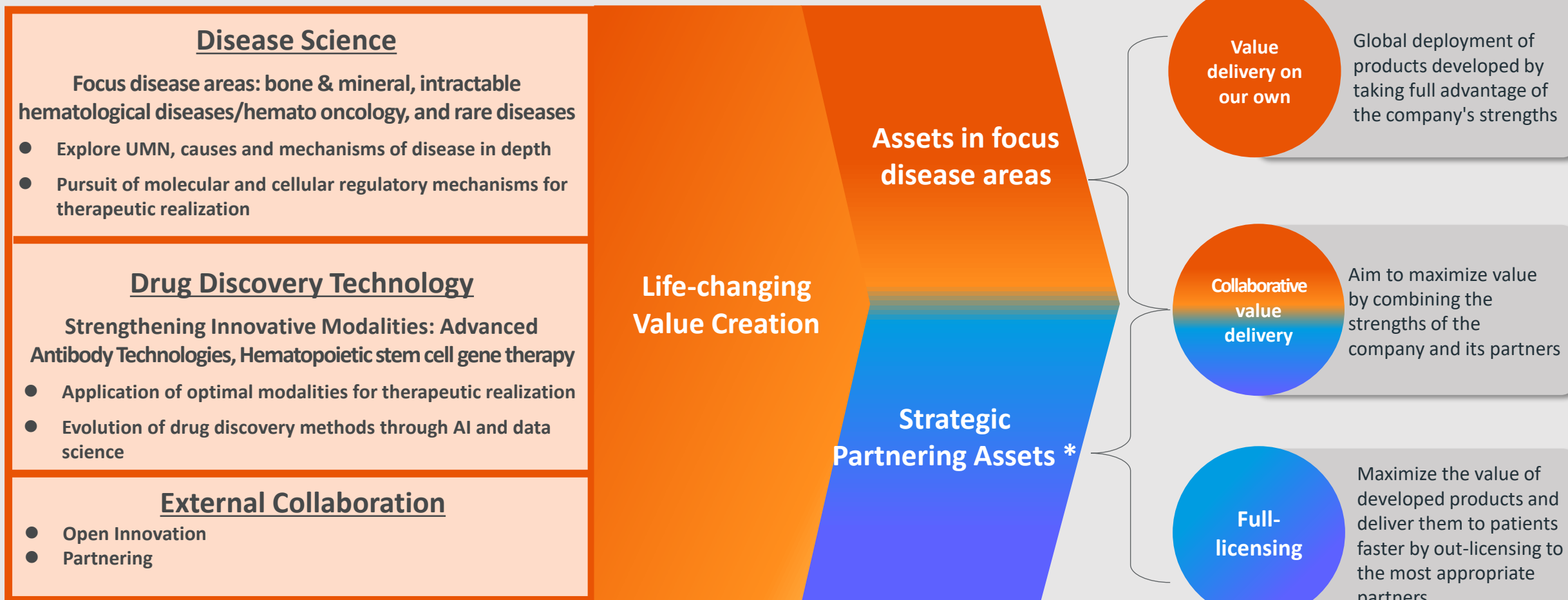
- Accelerate new patient starts by focusing on 1st adjunct to levodopa/carbidopa patient type.
- Communicate the full clinical story of Nourianz through field force activity and updated digital channels.



R&D Update

Story for Vision 2030

Strategies for creating and delivering life-changing value



*Assets outside of the disease areas of focus are designated as strategic partnering assets, and value maximization is achieved through collaboration with partners.

Upcoming Events: Main Development Pipeline Products

As of February 7, 2024

Code Generic Name	Events (Completed are in bold)		Timeline (Completed are in orange)
KHK4083/AMG 451 rocatinlimab	Atopic Dermatitis	P3 (ROCKET Program)	In progress
	Asthma	P2 initiation	H1 2024
	Prurigo nodularis	P3 initiation	H2 2024
KHK4951 tivozanib	nAMD	P2 initiation	Feb. 2024
	DME	P2 initiation	Jan. 2024
KK4277	SLE, CLE	P1	In progress
KK2260	Advanced or metastatic solid tumors	P1 initiation	Nov. 2023
KK2269	Advanced or metastatic solid tumors	P1 initiation	Jan. 2024
KK2845	AML	P1 initiation	Q2 2024
OTL-200	MLD ¹	FDA decision to be announced	Mar. 2024 ²
OTL-203	MPS-IH³ (Hurler syndrome)	Registrational study⁴ initiation	Jan. 2024
OTL-201	MPS-IIIA⁵ (Sanfilippo syndrome type A)	PoC study⁶ Data - Conference presentation	Feb. 2024

1. Metachromatic Leukodystrophy; 2. PDUFA date: Mar. 18, 2024; 3. Mucopolysaccharidosis type I; 4 Equivalent to P3 study; 5. Mucopolysaccharidosis type IIIA; 6. Equivalent to P1/2 study.

Main Development Pipeline Products (After Ph2)

As of February 7, 2024

	Diseases under development*1	Planned Approval Year*2	Development status	Total addressable market*3	No. of Patients*4
KHK4083/AMG 451 rocatinlimab	Moderate and severe Atopic Dermatitis	2026/2027	P3 (Global)	★★★★★	16M
KHK4083/AMG 451 rocatinlimab	Moderate and severe Asthma*5	TBD	Preparation underway for P2 (Global)	★★★★★	13.5M
KHK4951 tivozanib	nAMD	TBD	P2 (JP, US)	★★★★	2,600K
KHK4951 tivozanib	DME	TBD	P2 (JP, US)	★★★★	3,400K
OTL-200 Libmeldy®	MLD	2024 (US)	Filing to FDA	★	(1 in 40K-160K live birth)
OTL-203	MPS-IH (Hurler syndrome)	2029	Registrational study*5 (US, EU)	★	(1 in 100K live birth)*6
OTL-201	MPS-IIIA (Sanfilippo syndrome type A)	TBD	Proof-of-concept*7	★	(1 in 100K)

*1 Expected indications as of the date of this document; indications may ultimately differ to expectations due status of approvals from regulatory authorities. *2 Expected year of first approval. *3 Expected total addressable market estimated by Kyowa Kirin, which is the sum of all products for the indications shown in *1, not projected sales or the Company's targets. **Colored areas represent estimates for global, and the rest are for Japan.** ★: less than ¥50Bn. ★★: ¥50Bn-¥100Bn. ★★★: Over ¥100Bn-¥500Bn. ★★★★: Over ¥500Bn-¥1Tn. ★★★★★: Over ¥1Tn. *4 Total number of estimated patients by Kyowa Kirin. **Colored areas represent in-house estimates for global, and the rest are in-house estimates for Japan.** *5 Equivalent to P3 study. *6 "1 in 100k live birth" is estimated incidence for all of MPS-I, of which approximately 70 percent are cases of Hurler syndrome. *7 Equivalent to P1/2 study.

Main Development Pipeline Products (nonclinical ~ Ph1)

As of February 7, 2024

	Diseases under development ^{*1}	Development status	Modality, technology
KK4277	SLE, CLE	P1 (JP, Asia)	Antibody, POTELLIGENT [®]
KK2260	Advanced or metastatic solid tumors	P1 (JP: in progress, US: in preparation)	Antibody, REGULGENT [™]
KK2269^{*2}	Advanced or metastatic solid tumors	P1 (JP, US)	Antibody, REGULGENT [™]
KK2845^{*2}	AML	Preparation underway for P1 (JP)	Antibody-Drug Conjugate

^{*1} Expected indications as of the date of this document; indications may ultimately differ to expectations due status of approvals from regulatory authorities

^{*2} Since the trials had not started yet as of December 31, 2023, they are not currently listed on our company's pipeline table.

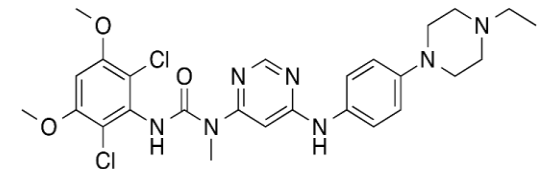
License agreement for infigratinib

To strengthen our pipeline in the bone and mineral areas, Kyowa Kirin has entered into a license agreement with BridgeBio Pharma for the development and commercialization of infigratinib in Japan.

- Secured exclusive rights for the development and commercialization within Japan, targeting skeletal dysplasias
- The agreement includes an upfront payment of \$100M with royalties up to the high-twenties percent, with additional milestone-based payments to BridgeBio

Infigratinib

- An oral small molecular FGFR¹-3 inhibitor developed by QED Therapeutics, an affiliate of BridgeBio
- Target Condition: Achondroplasia
 - Genetic condition caused by gain-of-function variants in the FGFR3 gene, leading to reduced long-bone growth and thus shortened limbs and overall stature
 - Designated as an intractable disease (#276) in Japan², with an estimated patient population of 6,000
 - Oral treatment methods are yet to be established
- The P2 trial (PROPEL 2)³ has demonstrated efficacy, and the global P3 trial (PROPEL 3)⁴ is currently underway
- Mechanism of Action: Promotes growth plate development by inhibiting abnormal FGFR3 activity



Kyowa Kirin plans to commence PMDA consultations in 2024 and initiate a registrational study in Japan in 2025

1. Fibroblast growth factor receptor; 2. <https://www.nanbyou.or.jp/entry/4570>; 3. NCT04265651; 4. NCT06164951

News Flow in 2023

Year-to-date Key News Flow

Category	Date	Headline	As of November 1, 2023
ESG	Mar 8	Kyowa Kirin Selected for a “Health & Productivity Stock” and awarded as a “Certified Health & Productivity Management Outstanding Organization”(White 500) (Japan)	
R&D	Mar 17	Presented New data from Phase 2b clinical study of Rocatinlimab in Atopic Dermatitis at the American Academy of Dermatology Annual Meeting 2023	
SCM	Mar 28	Completed construction of a new building at Ube Plant (Japan)	
ESG	Apr 6	Introduced RE100 renewable electricity to all purchased electricity at its two plants and three laboratories (Japan)	
SCM	Apr 7	Completed construction of a multipurpose facility relating to Quality Assurance (Q-Tower) at Takasaki Plant (Japan)	
R&D	Apr 27	Started collaboration in drug discovery technology with School of Life Science and Technology, Tokyo Institute of Technology (Japan)	
R&D	May 10	Announced Phase III Study Results of bardoxolone methyl (RTA 402) in Japan and Discontinuation of Development (Japan)	
LCM	Jun 23	Approval for partial change of Antineoplastic Mitomycin C Agent. (Japan)	
LCM	Jul 18	Launched of Topical Ophthalmic Mitomycin C Agent and resumed the supply of Antineoplastic Mitomycin C Agent. (Japan)	
LCM	Jul 24	Application for partial change of approved indication of G-Lasta® for the Mobilization of Hematopoietic Stem Cells into Peripheral Blood for Autologous Blood Stem Cell Transplantation. (Japan)	

Year-to-date Key News Flow

Category	Date	Headline	As of November 1, 2023
LCM	Aug 23	Approval for partial change of LUMICEF® for Palmoplantar Pustulosis (Japan)	
LCM	Aug 30	Approval for Calcimimetics Agent ORKEDIA® TABLETS 4mg (Japan)	
LCM	Sep25	Approval for Partial Change of Romiplate® for Aplastic Anemia (Japan)	
R&D	Sep 25	Approval of PHOZEVEL® for Improvement of Hyperphosphatemia in Chronic Kidney Disease Patients on Dialysis (Japan)	
R&D	Sep 28	Disclosing Top-Line Results of Phase 3 Clinical Study of KW-3357 for the Treatment of Preeclampsia (Japan)	
LCM	Sep 29	Approval for Partial Change of Rituximab Biosimilar Received by Sandoz (Japan)	
SI	Oct 5	Conclusion of Agreement to acquire Shares of UK biopharmaceutical company Orchard Therapeutics plc	
R&D	Oct 11	Presented the posthoc analysis data from the Phase 2b study of rocatinlimab (AMG 451/KHK4083) at European Academy of Dermatology and Venereology (EADV) Congress 2023	
R&D	Oct 17	Presented the Results of Phase 3 Studies of PHOZEVEL® at the American Society of Nephrology Meeting (ASN Kidney Week 2023)	

Kyowa Kirin and Grünenthal have entered into a Joint Venture Collaboration for Kyowa Kirin International’s 13 established medicines portfolio on Aug 1.

The transfer of global rights for Tostran, an established medicine, from Kyowa Kirin International to ADVANZ PHARMA has been completed on October 13.

ESG: environmental ,social, and governance; LCM: lifecycle management; R&D: research and development; SCM: supply chain management; SI: strategic investment

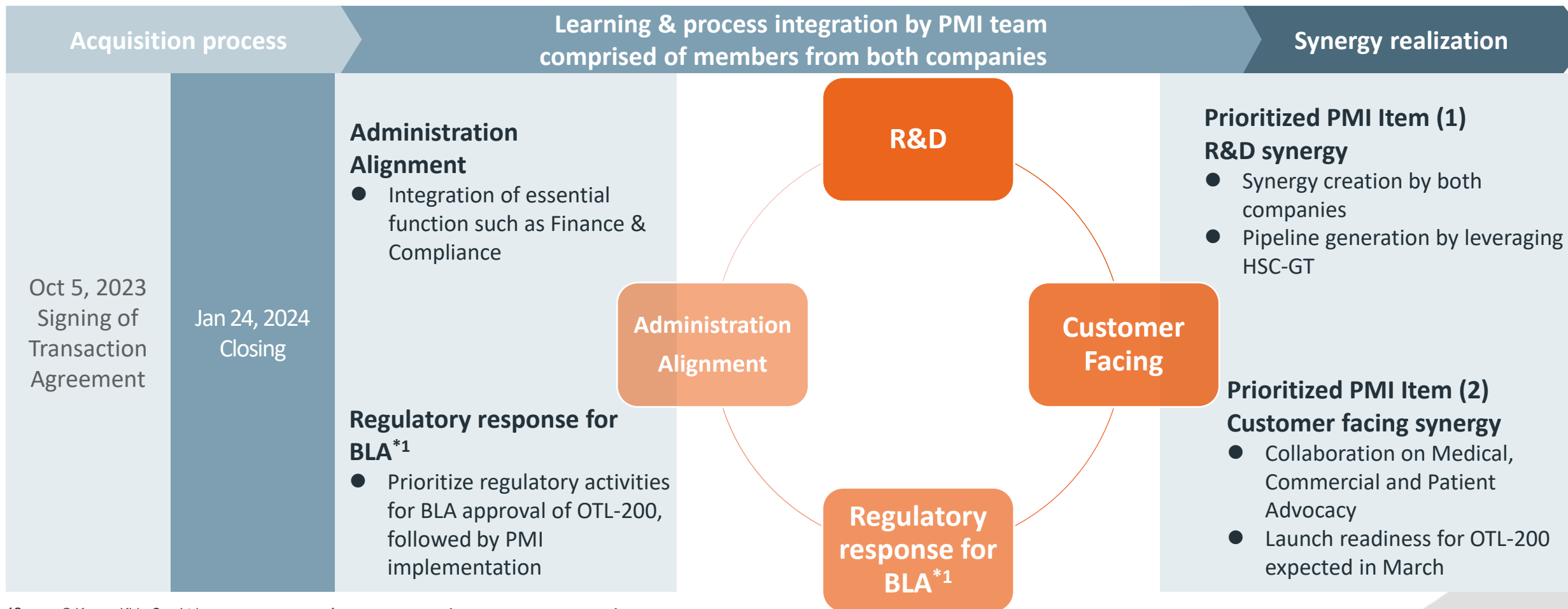
Year-to-date Key News Flow

Category	Date	Headline	As of November 1, 2023
LCM	Nov 22	Launched Calcimimetics Agent ORKEDIA® TABLETS 4mg (Japan)	
LCM	Jan 5	Out-licensed the exclusive and worldwide rights to Boehringer Ingelheim of developing first-in-class treatment for fibro-inflammatory diseases.	
SI	Jan 24	Completion of share acquisition of Orchard Therapeutics plc, UK biopharmaceutical company	
R&D	Feb 6	First Patient Randomized in Registrational Trial of OTL-203 for MPS-I Hurler Syndrome	
R&D	Feb 6	First Patient Enrolled in the Phase2 Clinical Trial Evaluating Tivozanib Eye Drop for Diabetic Macular Edema	
SI	Feb 7	Conclusion of Agreement with BridgeBio Pharma for an Exclusive License on Infigratinib in Skeletal Dysplasias in Japan	
Finance	Feb 7	Acquisition of Own Shares and Cancellation of Treasury Shares	

ESG: environmental ,social, and governance; LCM: lifecycle management; R&D: research and development; SCM: supply chain management; SI: strategic investment

Plans for Orchard Therapeutics Post Merger Integration

- Completed acquisition of Orchard Therapeutics on January 24th, 2024.
- Build PMI (post-merger integration) team comprised of members from both companies, and initiate activities for successful synergy creation.



Accounting treatment of share acquisition of Orchard Therapeutics (Tentative)

- ✓ Completed the share acquisition on January 24, 2024, and will be consolidated starting from the February 2024
- ✓ Recognized intangible assets of \$201M and goodwill of \$254M
- ✓ Intangible assets will be amortized over 20 years (19 years for OTL-200 (Libmeldy))

【Breakdown of Intangible \$201M】

- OTL-200 \$159M (incl. US)
- OTL-203 \$42M

【Annual amortization amount】

- OTL-200 \$8M /year
⇒ Amortization will start from Feb 2024
- OTL-203 \$2M /year
⇒ To be amortized after market launch



(Unit Million USD)

Other assets 172	Other liabilities 108
	Deferred tax liabilities 50
Intangible assets 201	Acquisition costs 478
Goodwill 254	
Other Expenses 9	

The above is a tentative calculation based on assumptions using Orchard balance sheet as of December 31, 2023 (preliminary figures based on US GAAP).
Going forward, we will discuss with KPMG to finalize the amounts and accounting treatment.

Appendix

Strategic Rationale of The Transaction

Our New Vision toward 2030

Kyowa Kirin will realize the successful creation and delivery of life-changing value* that ultimately makes people smile, as a Japan-based Global Specialty Pharmaceutical company built on the diverse team of experts with shared passion for innovation.

* Make patient smile through dramatic improvement in quality of life by identifying the unmet medical needs of people battling with medical conditions and by creating and supplying new drugs or services that help them overcome those challenges

Our purpose of the acquisition:

- To enrich our pipeline to address the UMNs* for which there is still no cure
- To obtain capabilities in Cell Gene Therapy R&D to address the future UMNs

* Unmet medical needs

- Strengthen the business following Crysvida and Poteligeo as a Global Specialty Pharmaceutical company
- Enhance ability to address UMNs in the future by combining with our strength in biologics
- Commitment to life by providing not only pharmaceuticals but also treatments

Acquisition of Orchard Therapeutics plc Shares - Transaction Overview -

Items	Summary
Target	<ul style="list-style-type: none"> ■ Orchard Therapeutics plc (London) — Listed on NASDAQ
Purchase Price*	<ul style="list-style-type: none"> ■ \$16.00 per ADS / approx. \$387.6 million (approx. JPY 57.4 billion) — Orchard shareholders will hold additional contingent value rights (CVR) of \$1.00 per ADS. — Additional \$1.00 CVR will be paid for a total of \$17.00 per ADS, or approximately \$477.8 million (approx. JPY 70.7 billion) if OTL-200 is approved by the U.S. Food and Drug Administration for the commercial marketing and sale in the U.S.
Funding Method	<ul style="list-style-type: none"> ■ Cash on balance sheet
Financial Impact	<ul style="list-style-type: none"> ■ To be announced once allocation of goodwill and intangible assets are determined
Transaction Structure and Process	<ul style="list-style-type: none"> ■ Scheme of Arrangement (SoA) — Requires the approval by Orchard’s shareholder meeting, UK court, and regulatory authorities — Closing is on January 24th, 2024 through implementation of SoA

*Refers to the amount required to make payments related to all outstanding Orchard common shares, ADS purchases, options, Restricted Stock Units, and other instruments.

Acquisition of Orchard Therapeutics plc Shares - Expected Synergy -

Further Development as a Global Specialty Pharmaceutical Company

- To be a Japan-based Global Specialty Pharmaceutical Company providing life-changing value to high unmet medical needs in concert with our existing business through Crysvida and Poteligeo

Reinforcement of New Drug Discovery & Development

Kyowa Kirin

- Experience and expertise of R&D and commercialization in Biologics and Antibody Drugs
- Proprietary next-generation antibody technology
- Continuous efforts to new modalities



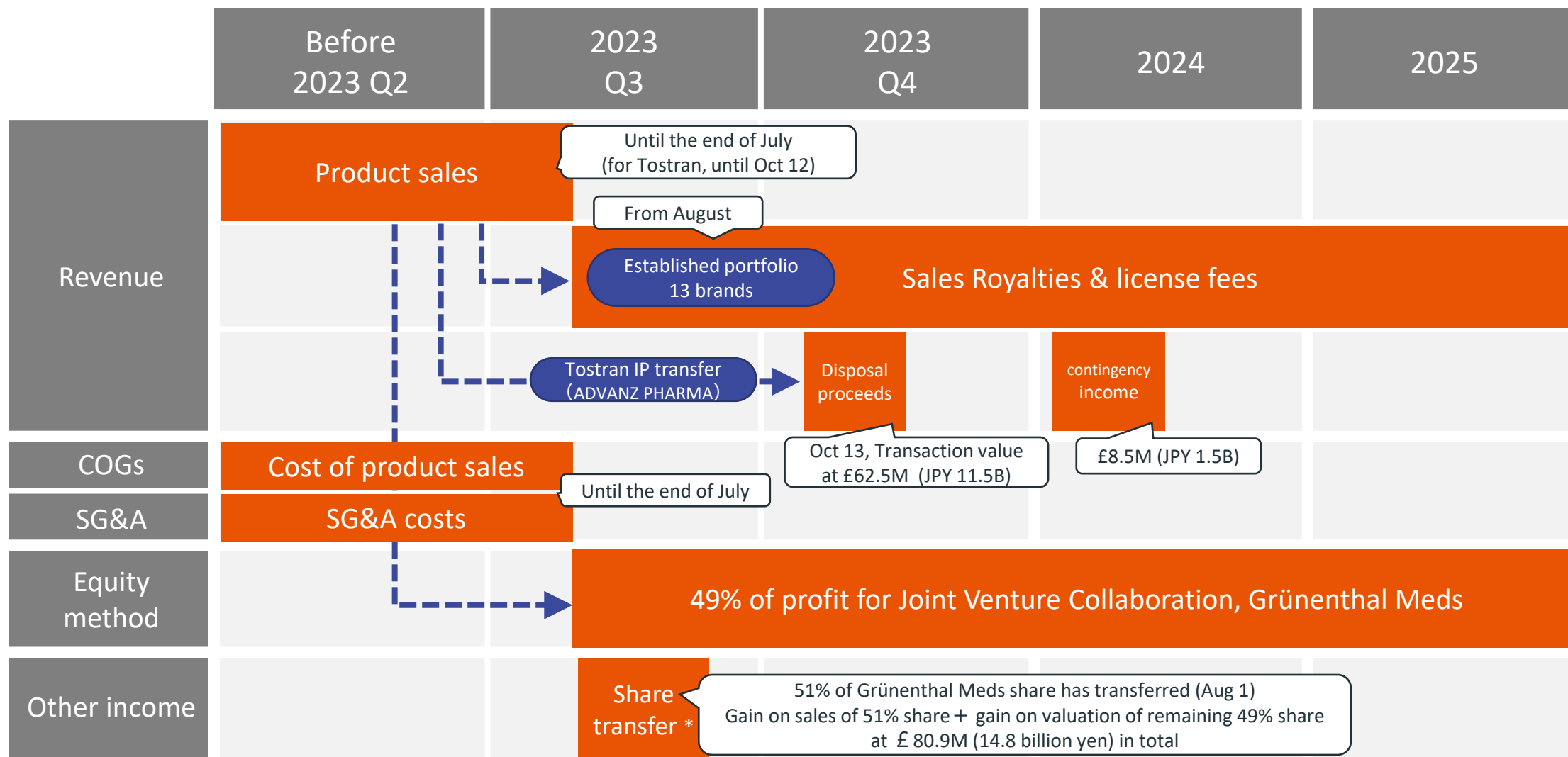
Orchard Therapeutics

- Experience and know-how in the marketing HSC-GT¹ in EU
- High technology in HSC-GT¹ (Research, CMC, SCM, etc.)
- Patient access and collaboration with medical institutions
- Efforts and experience to new modalities

- A key step toward delivering advanced value to patients
 - Pursuing the potential for “One-time treatment in life”
 - Challenge to correct the underlying cause of a genetic disease
 - Personalized medicine / Precision medicine
 - Providing treatment beyond the existing drugs
- Address a broader range of UMNs

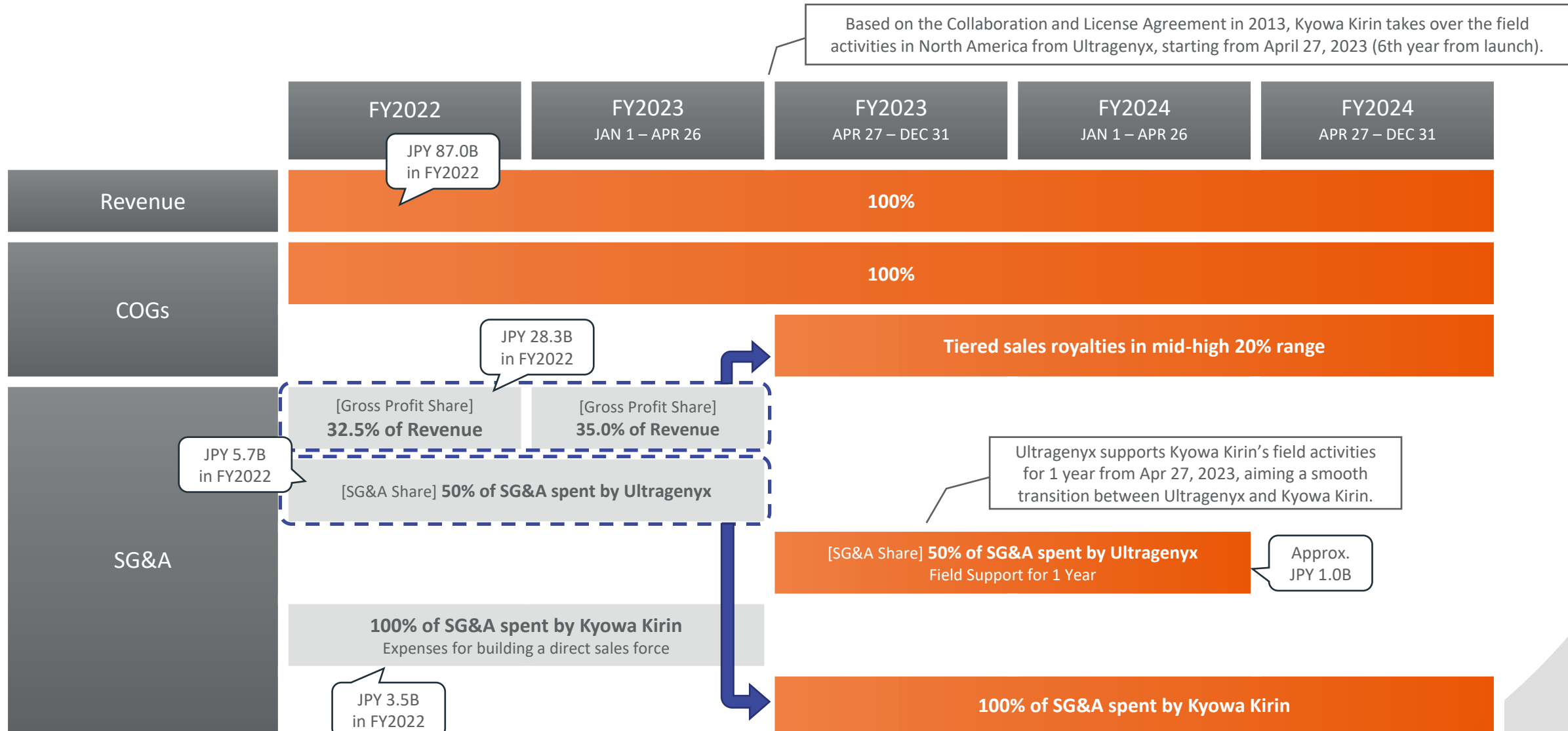
1. Hematopoietic Stem Cell Gene Therapy

P/L Impact on EMEA established medicines portfolio



* Grünenthal owns a 51 percent majority share in the Joint Venture Collaboration, while Kyowa Kirin International plc owns a 49 percent share. Grünenthal will have the option to fully acquire the remaining 49 percent share, including intellectual property (IP) of 13 brands, via exercising an option in Q1, 2026

P/L Impact on North American Crysvita Business



Main Development Pipeline Products: Future plans

T : Topline data **D** : Detailed data

Code Generic Name	Target Disease		2024	2025	2026	+	
KHK4083/ AMG 451 rocatinlimab	Moderate and severe Atopic Dermatitis	P3					IGNITE
		P3					HORIZON
		P3					SHUTTLE
		P3					ASTRO
		P3					ORBIT
		P3					VOYAGER
		P3					ASCEND
		P3					OUTPOST
KHK4951 tivozanib	nAMD	P2					
	DME	P2					
KK4277	Systemic Lupus Erythematosus Cutaneous Lupus Erythematosus	P1					
KK2260	Advanced or metastatic solid tumors	P1					
KK2269	Advanced or metastatic solid tumors	P1					

FOREX Information

Average FOREX Rates (yen)

	FY2022	FY2023	Changes	FY2024 Plans
USD	130	140	+10	140
GBP	161	174	+13	180
EUR	137	151	+14	155

FY23 FOREX Impacts (vs FY22, billion yen)

	Revenue	Core OP
USD	+11.0	+3.5
GBP	+2.3	+0.7
EUR	+3.7	+1.7

FY24 FOREX Sensitivities (vs FY24 Plan, billion yen)

	Changes	Revenue	Core OP
USD	+1 yen	+1.4	+0.4
GBP	+1 yen	+0.2	-0.0
EUR	+1 yen	+0.3	+0.2

Crysvita - Collaboration with Ultragenyx -

Economic Terms

US & Canada

- Kyowa Kirin books sales
- 50/50 profit share for 5 years from the U.S. launch
 - Supply price: 35% of net sales through 2022, 30% thereafter (No impact on the sales royalties stated below)
- After 5 years (April 27, 2023-), Kyowa Kirin pays tiered sales royalties in mid-high 20% range to Ultragenyx
 - *Ultragenyx has sold 30% of its royalty interest, subject to a 1.45x cap, to OMERS Capital Markets

Europe

- Kyowa Kirin books sales
- Kyowa Kirin pays sales royalties in up to 10% range to Ultragenyx
 - *Ultragenyx has sold its royalty interest, subject to a 1.9x or 2.5x cap depending on when the cap is achieved, to Royalty Pharma

Latin America

- Ultragenyx books sales
- Kyowa Kirin receives low single-digit sales royalties from Ultragenyx
- Supply price: 35% of net sales through 2022, 30% thereafter

Turkey

- Ultragenyx books sales
- Kyowa Kirin receives sales royalties in up to 20% range from Ultragenyx

Asia & Others

- Kyowa Kirin books sales
 - * Kyowa Kirin supplies commercial products in all territories.

KHK4083/AMG 451 - Collaboration with Amgen -

	US	Europe & Asia (ex. JP)	JP
Development	<ul style="list-style-type: none"> • Amgen leads development • Share development cost 	<ul style="list-style-type: none"> • Amgen leads development • Share development cost 	<ul style="list-style-type: none"> • Kyowa Kirin leads development
Commercialization	<ul style="list-style-type: none"> • Amgen commercializes and books sales • Kyowa Kirin co-promotes and shares promotion cost 	<ul style="list-style-type: none"> • Amgen commercializes and books sales • Kyowa Kirin has opt-in rights for co-promotion 	<ul style="list-style-type: none"> • Kyowa Kirin commercializes and books sales
Sales Royalties	<ul style="list-style-type: none"> • Double-digit royalty to Kyowa Kirin 	<ul style="list-style-type: none"> • Double-digit royalty to Kyowa Kirin 	
Commercial supply	<ul style="list-style-type: none"> • Amgen supplies 	<ul style="list-style-type: none"> • Amgen supplies 	<ul style="list-style-type: none"> • Kyowa Kirin supplies

Amgen makes a \$400 million up-front payment (done) and future contingent milestone payments potentially worth up to an additional \$850 million, as well as royalty payments on future global sales, to Kyowa Kirin.

Estimated Patient Numbers

Disease	Country/ Region	Incidence	Prevalence*	Reference
PTCL	JP		2,000	Ministry of Health, Labour and Welfare: 2017 Patient survey (illness classification)
CTCL	JP		2,000	Ministry of Health, Labour and Welfare: 2017 Patient survey (illness classification)
	US	1,500 / y		SEER Data (2001-2007)
XLH	JP	1:20,000	Adult: 5,000 Ped: 1,000	Estimate based on reported prevalence of 1 in 20,000 people; Nationwide survey of fibroblast growth factor 23 (FGF23)-related hypophosphatemic diseases in Japan: prevalence, biochemical data and treatment. (Endo I et al., Endocr J., 2015)
	EU	1:20,000	Adult: 12,000 Ped: 3,000	Estimate based on reported prevalence of 1 in 20,000 people
	US	1:20,000	Adult: 12,000 Ped: 3,000	Estimate based on reported prevalence of 1 in 20,000 people; New perspectives on the biology and treatment of X-linked hypophosphatemic rickets. (Carpenter TO, Pediatr Clin North Am., 1997)
TIO	JP		30	2010 Ministry of Health, Labour and Welfare Epidemiological Research on abnormalities in Hormone Receptor Mechanisms
	US		500-1,000	Survey by Ultragenyx Pharmaceutical
AD	JP, NA, EU		30,000,000	Study by Decision Resources
nAMD	JP, US		2,300,000	Study by Decision Resources
MLD	Global	1:40,000-160,000		https://medlineplus.gov/genetics/condition/metachromatic-leukodystrophy/#frequency
MPS-IH	Global	1:100,000		Puckett et al. 2021 Orphanet J Rare Dis 16:241: US NBS data (MPS-I incidence derived from NBS data in Table 3)
MPS-IIIA	Global	1:100,000		Trinity Partners research report 2013 based on literature and KOL feedback

*Prevalence represents the estimated patient number per the entire population of each country or region.

List of Acronyms

AD	Atopic Dermatitis
AG	Authorized Generic
APAC	Asia-Pacific
AML	Acute myeloid leukemia
BS	Biosimilar
DME	Diabetic Macular Edema
EMEA	Europe, the Middle East and Africa
JP	Japan
LCM	Lifecycle Management
MDS	Myelodysplastic syndromes
MLD	Metachromatic Leukodystrophy
MPS-IH	Mucopolysaccharidosis type I, Hurler syndrome
MPS-IIIA	Mucopolysaccharidosis type IIIA
NA	North America
nAMD	neovascular Age-related Macular Degeneration
PD	Parkinson's Disease
PE	Preeclampsia
TIO	Tumor Induced Osteomalacia
XLH	X-linked Hypophosphatemia



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