

# Results Presentation

## Fiscal 2024 Third Quarter

協和キリン株式会社

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 **KYOWA KIRIN**

# Agenda

## Financial Review

Managing Executive Officer, Chief Financial Officer (CFO) **Motohiko Kawaguchi**

## Commercial Update

Managing Executive Officer, Chief Strategy Officer (CSO) **Yasuo Fujii**

## R&D Update

Senior Managing Executive officer, Chief Medical Officer (CMO) **Takeyoshi Yamashita, Ph.D.**

## News Flow in 2024

Managing Executive Officer, Chief Strategy Officer (CSO) **Yasuo Fujii**

## Q&A

Senior Managing Executive officer, Chief Medical Officer (CMO) **Takeyoshi Yamashita, Ph.D.**

Managing Executive Officer, Chief Financial Officer (CFO) **Motohiko Kawaguchi**

Managing Executive Officer, Chief Strategy Officer (CSO) **Yasuo Fujii**

*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.*

# Financial Review

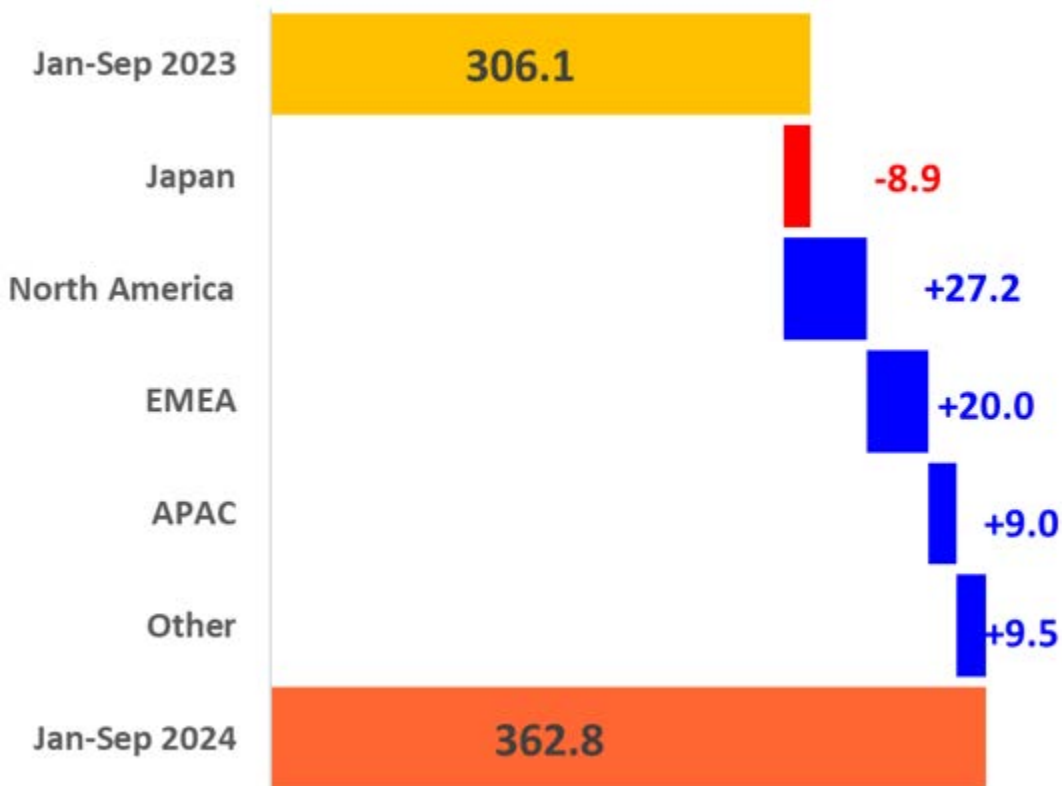
# Summary of Q3 Results

( Billion Yen / Rounded )

	2023Q3 Results	2024Q3 Results	Changes	FY2024 Rev. Plans	Progress to goal
Revenue <i>[Overseas Ratio]</i>	306.1 <i>[64%]</i>	362.8 <i>[72%]</i>	+56.7 (+19%)	492.0 <i>[71%]</i>	74%
Gross Profit <i>[Gross Profit Margin]</i>	229.1 <i>[75%]</i>	268.8 <i>[74%]</i>	+39.7 (+17%)	364.0 <i>[74%]</i>	74%
SG&A <i>[SG&amp;A Ratio]</i>	119.3 <i>[39%]</i>	123.6 <i>[34%]</i>	+4.3 (+4%)	168.0 <i>[34%]</i>	74%
R&D <i>[R&amp;D Ratio]</i>	51.2 <i>[17%]</i>	74.3 <i>[20%]</i>	+23.1 (+45%)	105.0 <i>[21%]</i>	71%
Gain/Loss on Equity Method	2.3	3.5	+1.2 (+55%)	1.0	353%
Core Operating Profit <i>[Core OP Margin]</i>	60.9 <i>[20%]</i>	74.4 <i>[21%]</i>	+13.5 (+22%)	92.0 <i>[19%]</i>	81%
Profit	53.6	55.9	+2.3 (+4%)	68.0	82%

# YoY Analysis -Revenue-

**+56.7 billion yen  
(incl. forex effect +23.3)**



- **Japan -8.9**

Although Phozevel, Duvroq and Crysvida increased, revenue in Japan region decreased by 8% due mainly to negative impact by annual NHI price-cut and shrink in G-Lasta affected by competitive products.

- **North America +27.2 (incl. forex effect +11.8)**

Revenue in North America region increased by 29% with the growth of Crysvida (+24%) and Poteligeo (+44%).

- **EMEA +20.0 (incl. forex effect +6.5)**

Revenue in EMEA region increased by 44% with the growth of Crysvida (+52%) and Poteligeo (+24%) as well as the IP transfer of three established medicines (JPY 13.1B) to Grünenthal-Med, the joint venture collaboration (JVC) with Grünenthal.\*

\*These three medicines are part of the portfolio of 13 brands, such as Abstral, which are marketed through Grünenthal-Med.

- **APAC +9.0 (incl. forex effect +2.1)**

APAC revenue increased by 35% driven by the growth of Crysvida and Nesp, in addition to KR/TW inventory transfer to DKSH (JPY 5.4B) at the end of September.

- **Other +9.5 (incl. forex effect +2.9)**

28% growth in the other revenue was due to the royalties of growing Fasenra (Benralizumab), upfront revenue from Boehringer Ingelheim, and new consolidation of Orchard.

# Revenue of Major Items (Japan)

( Billion Yen / Rounded )

Item	2023Q3 Results	2024Q3 Results	Changes	Reasons	2024 Rev. Plans*	Progress to goal
Crysvita	7.4	8.2	+0.8 (+11%)	Market penetration (Launched in Dec 2019)	12.9	63%
Poteligeo	1.4	1.4	-0.1 (-4%)		1.9	72%
Nesp + Nesp-AG <sup>1</sup>	12.7	10.4	-2.2 (-17%)		14.4	72%
Nesp	2.3	2.0	-0.3 (-14%)	NHI price-cut & Biosimilars' penetration	2.8	72%
Nesp-AG	10.3	8.5	-1.9 (-18%)		11.7	72%
Duvroq	6.9	8.9	+2.1 (+30%)	Market penetration (Launched in Aug 2020)	12.2	73%
Phozevel	-	2.9	+2.9 (- %)	Launched in Feb 2024	3.3	88%
Orkedia	7.6	7.5	-0.1 (-1%)		11.7	64%
G-Lasta	23.2	15.3	-7.9 (-34%)	NHI price-cut & Biosimilars' penetration	20.5	74%
Rituximab BS	6.7	5.7	-1.0 (-15%)	NHI price-cut	7.9	72%
Romiplate	8.7	9.9	+1.2 (+14%)	Market penetration (New indication in Jun 2019)	13.2	76%
Nourias	5.5	5.1	-0.5 (-9%)		7.1	71%
Haruopi	3.2	3.3	+0.1 (+3%)		5.2	64%

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.

\* 2024 Revised Plan announced on August 1, 2024, there is no changes to the "Revenue of Major Items (Japan)"

# Revenue of Major Items (ex-Japan)

( Billion Yen / Rounded )

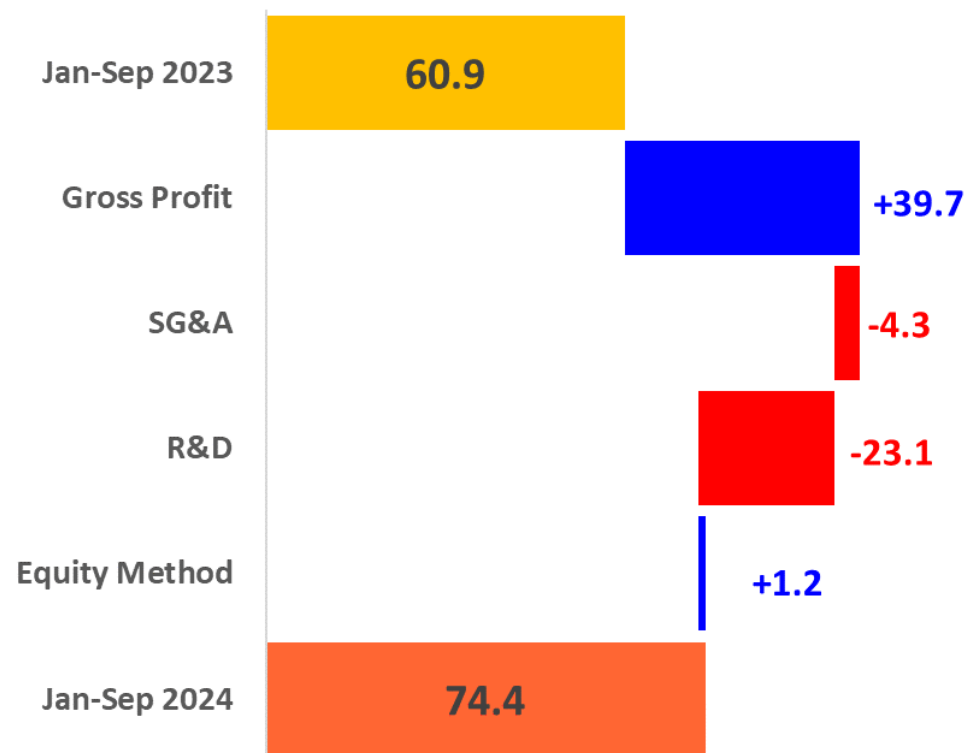
Item	2023Q3 Results	2024Q3 Results	Changes	Reasons	2024 Rev. Plans	Progress to goal
Crysvita	95.7	126.7	+31.0 (+32%)	Market penetration	187.8	67%
North America	70.2	87.2	+17.1 (+24%)			
EMEA	24.5	37.1	+12.7 (+52%)			
APAC	1.1	2.3	+1.2 (+117%)			
Poteligeo	19.9	27.7	+7.8 (+39%)	Market penetration	34.8	80%
North America	15.1	21.6	+6.6 (+44%)			
EMEA	4.8	6.0	+1.1 (+24%)			
APAC	0.0	0.1	+0.1 ( - %)			
Libmeldy / Lenmeldy	-	2.2	+2.2 ( - %)	New consolidation of Orchard (FDA approval in Mar 2024)	4.9	44%
Nourianz	5.5	6.2	+0.7 (+12%)		9.1	68%
Nesp	7.0	8.8	+1.8 (+25%)		10.7	82%
Gran	5.2	5.4	+0.3 (+5%)		7.2	76%
Tech-licensing	29.3	33.7	+4.4 (+15%)	Upfront revenue from Boehringer Ingelheim and growth of Fasenra	47.8	71%
Benralizumab Royalty <sup>1</sup>	19.1	21.6	+2.4 (+13%)			

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



# YoY Analysis -Core OP-

**+13.5 billion yen  
(incl. forex effect +8.4)**



- **Gross Profit +39.7 (incl. forex effect +20.3)**

Increased in conjunction with JPY 56.7B rise in revenue. COGs have increased due to the North America Crysvida Sales royalty after Apr 27, 2023. Hence, gross profit % declined YoY. (75% →74%)

- **SG&A -4.3 (incl. forex effect -7.2)**

SG&A increased due to increase in HR expenses, new consolidation of Orchard, and FX impact, despite the decrease in Crysvida profit-sharing expenses because of the North America Crysvida-related scheme change after Apr 27, 2023.

[HR exp -7.9 / Sales promotion +9.6 (incl. Crysvida profit sharing expenses +10.9) ]

- **R&D -23.1 (incl. forex effect -4.9)**

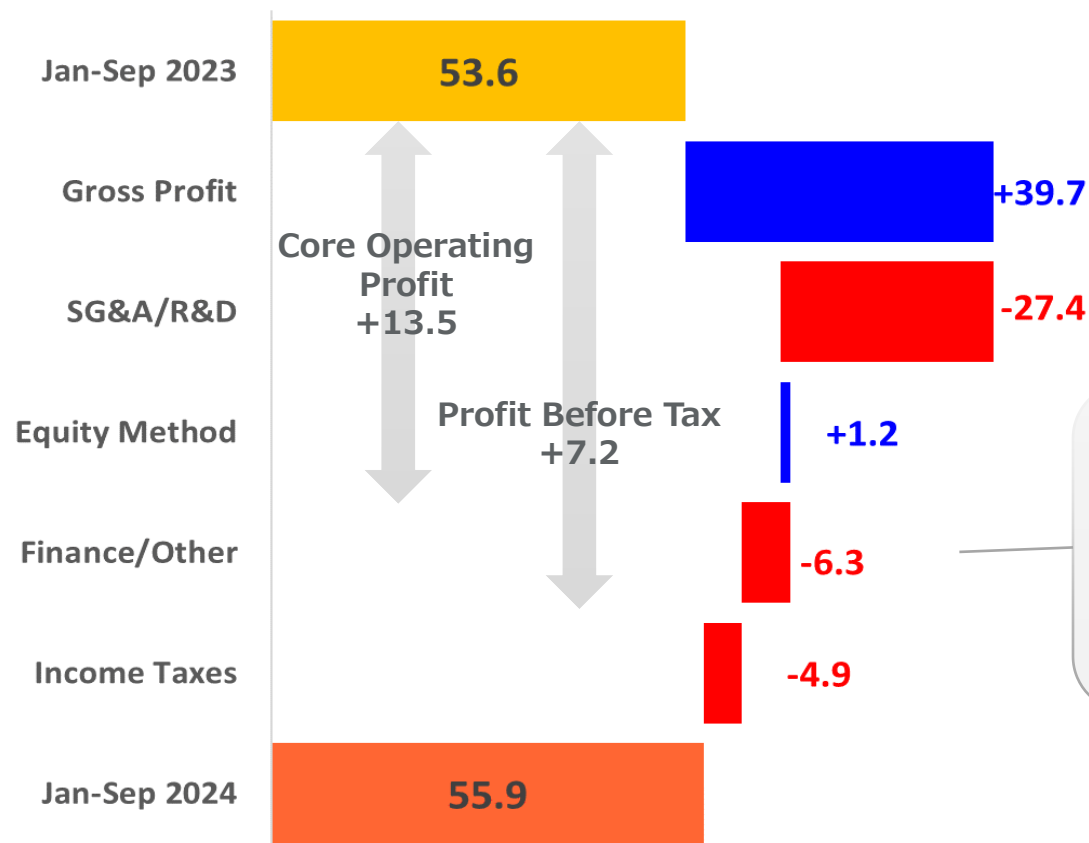
Increased in clinical study costs of KHK4083 which is undergoing joint global Phase III clinical study and new consolidation of Orchard

- **Gain/Loss on Equity Method +1.2 (incl. forex effect +0.2)**

JVC with Grünenthal, gain on inventory transfer have occurred in connection with operation transfer to Grünenthal in certain countries.

# YoY Analysis -Profit-

**Profit (Jan-Sep)  
+2.3 billion yen**



● **Finance/Other -6.3**

- EMEA established medicines portfolio:  
Gain on sales of 51% shares plus gain on valuation of remaining 49% shares -14.8 (14.8 → 0)
- Gain on Equity transfer for KKC (China affiliate) +7.8
- Business restructuring expenses -7.4 (-3.6 → -11.0)  
FY24: APAC restructuring -6.4, Transformation of JP Research function -2.1 etc.
- Impairment losses +7.9 (-9.4 → -1.5) FY23: RTA402 IPR&D -8.3 etc.

# Commercial Update

# 2024 Key Actions & Q3 Topics

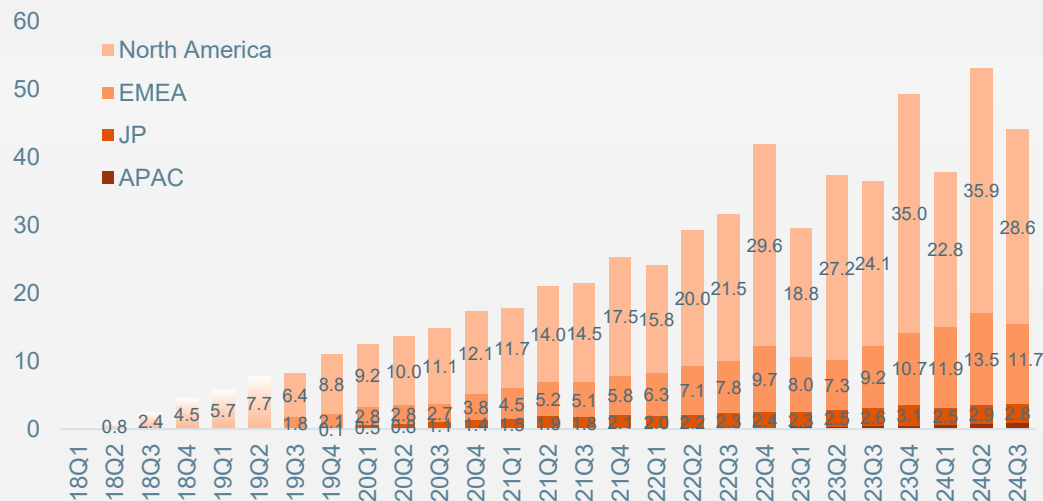
## 2024 Key Actions

- Strengthen evidence-based marketing activities.
- North America: Enhance disease awareness activities. Optimize field team structure and support.
- 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.

## Q3 Topics

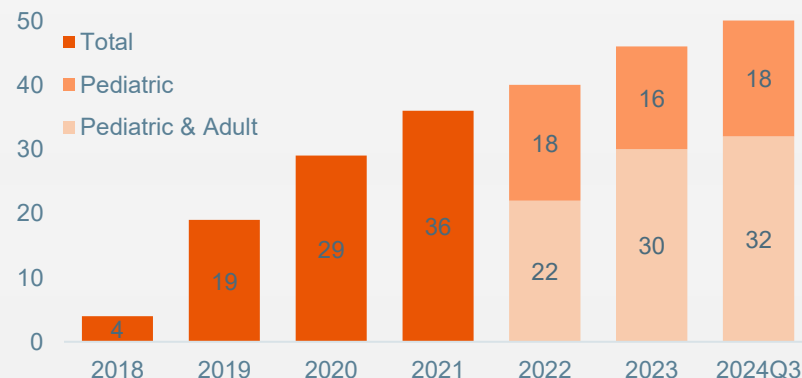
- Strengthen evidence-based marketing activities.
- North America
  - Although sales revenue decreased from the previous quarter due to the inventory adjustment, steady growth has been maintained throughout the year (+24% year-on-year YTD).
  - The number of new patients continues to be at a high level.
  - Keep enhancing disease awareness activities including Transition of Care from pediatric to adults and improving patient support programs.
- EMEA:
  - Revenue decreased slightly from the previous quarter due to the impact of forward shipments in the previous quarter but grew by 52% year-on-year YTD due to patient penetration and the impact of price adjustments in the previous year.
- Japan:
  - Continued to strengthen promotional activities by the dedicated personnel.

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.

# 2024 Key Actions & Q3 Topics

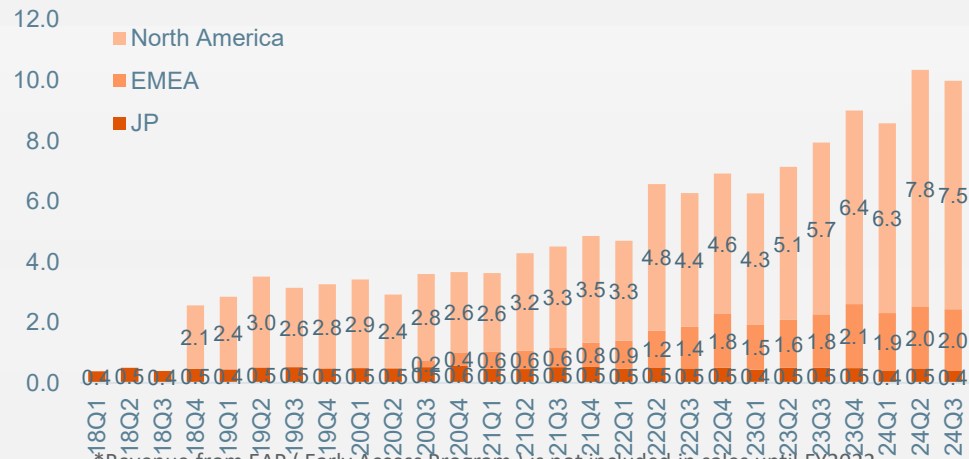
## 2024 Key Action

- Deeper penetration into the existing markets as well as expansion of targets through further progression of evidence-based promotional activities.
  - ◆ Start promotional activities focusing on progressing CTCL patients with visible skin symptoms.
  - ◆ Continue to raise awareness of importance of blood testing to accurately stage disease.
  - ◆ Geographic Expansion

## Q3 Topics

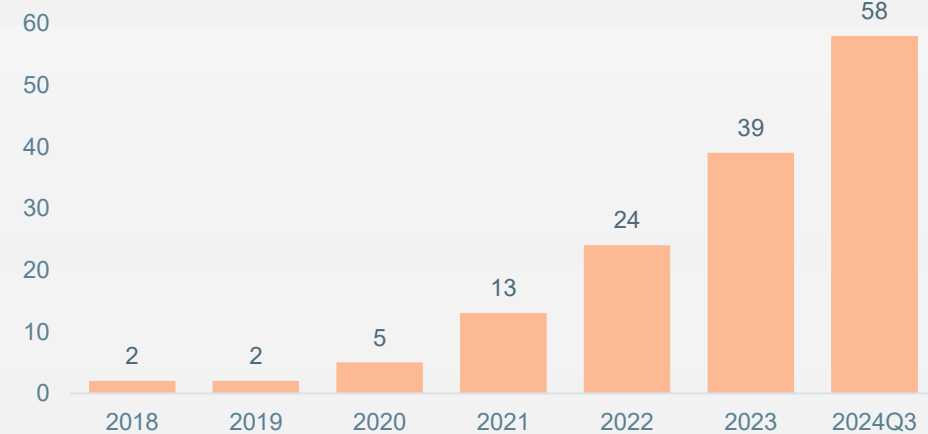
- NA : Sales revenue increased 44% year-on-year YTD due to:
  - Continue to expand evidence-based promotional activities to focus not only on cases with predominantly blood involvement, but also on early-stage cases with predominantly skin compartment.
  - Promotional activities focused on medical facilities with high potential for use based on data analysis.
- EMEA : Sales revenue increased by 24% year-on-year YTD due to:
  - Geographic expansion
  - Deeper penetration into the existing markets

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



# R&D Update

# News Flow of Main Development Pipeline Products

**Timeline**  
(Completed are in orange)

**Code**  
Generic Name

**Events** (Completed are in bold)

Code Generic Name	Events (Completed are in bold)	Timeline (Completed are in orange)
<b>KHK4083/AMG 451</b> rocatinlimab	<b>Atopic Dermatitis</b> P3 (ROCKET Program) <b>HORIZON Topline Data</b>	In progress <b>Sep 2024</b>
	Asthma P2	In progress
	Prurigo nodularis P3	In progress
<b>KHK4951</b> tivozanib	nAMD P2	In progress
	DME P2	In progress
<b>KK4277</b>	SLE, CLE P1	In progress
<b>KK2260</b>	Advanced or metastatic solid tumors P1	In progress
<b>KK2269</b>	Advanced or metastatic solid tumors P1	In progress
<b>KK2845</b>	<b>AML P1 initiation</b>	<b>October 2024</b>
<b>KK8123</b>	XLH P1 initiation	Q4 2024
<b>OTL-203</b>	MPS-IH (Hurler syndrome) Registrational study <sup>1</sup>	In progress
<b>OTL-201</b>	MPS-IIIA (Sanfilippo syndrome type A) Proof-of-concept study <sup>2</sup>	In progress

1. Equivalent to P3 study; 2. Equivalent to Ph 1/2 study

# News Flow in 2024



# Year-to-date Key News Flow

Category	Date	Headline
SP	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
MKT	Feb 19	Launch of PHOZEVEL® Tablets for Improvement of Hyperphosphatemia in Chronic Kidney Disease Patients on Dialysis (Japan)
R&D	Mar 11	Presented the post-hoc analysis data from the Phase 2b study of rocatinlimab (AMG 451/KHK4083) at American Academy of Dermatology (AAD) 2024 Annual Meeting
R&D	Mar 19	Receives FDA Approval of OTL-200 (Lenmeldy) for the treatment of children with early-onset—metachromatic leukodystrophy (MLD)

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

# Year-to-date Key News Flow

Category	Date	Headline
ESG	May 14	Announced the Publication of a Patient-focused Global Consensus Statement for Improving Diagnosis and Care in Cutaneous T-Cell Lymphoma ( Kyowa Kirin, Inc. )
LCM	May 17	Approval 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 in Japan
SCM	Jun 10	Announced Establishing New Biologics Manufacturing Plant in North Carolina, in the United States
LCM	Jun 28	Application for Additional Formulation of “LUMICEF® Subcutaneous Injection 210 mg Pen” in Japan
MKT	Jul 1	Announced Global Progress toward Advancing Newborn Screening for MLD ( Orchard Therapeutics )
ESG	Jul 29	Joined the Pharmaceutical Supply Chain Initiative (PSCI)
SCM	Aug 1	Restructuring of APAC Region Business and Change in Kyowa Kirin China Pharmaceutical Co., LTD
R&D	Aug 1	Transition to a Research Organization to Realize Our Vision toward 2030, and Introduction of a Voluntary Retirement Program

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

# Year-to-date Key News Flow

Category	Date	Headline
MKT	Sep 19	Presented new research on the real-world experiences of people living with XLH and the impact of Crysvida treatment at American Society for Bone and Mineral Research (ASBMR) 2024 annual meeting
R&D	Sep 25	Announced Top-line Data from rocatinlimab Phase 3 ROCKET HORIZON Trial for Adults with Moderate to Severe Atopic Dermatitis
R&D	Oct 24	A Faculty Member of the School of Life Science and Technology, Institute of Science Tokyo has been Appointed as a Researcher through the Cross-Appointment System
MGMT	Oct 31	Notice Regarding Changes of Representative Directors
<b>Updates after the previous earnings announcement</b>		

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

# Changes of Representative Director

Kyowa Kirin agreed on changes of its Representative Directors. The changes will be formally approved at the Ordinary General Meeting of Shareholders and the Board of Directors meeting to be held on March 2025.

~Reason for change (Abstracts)~

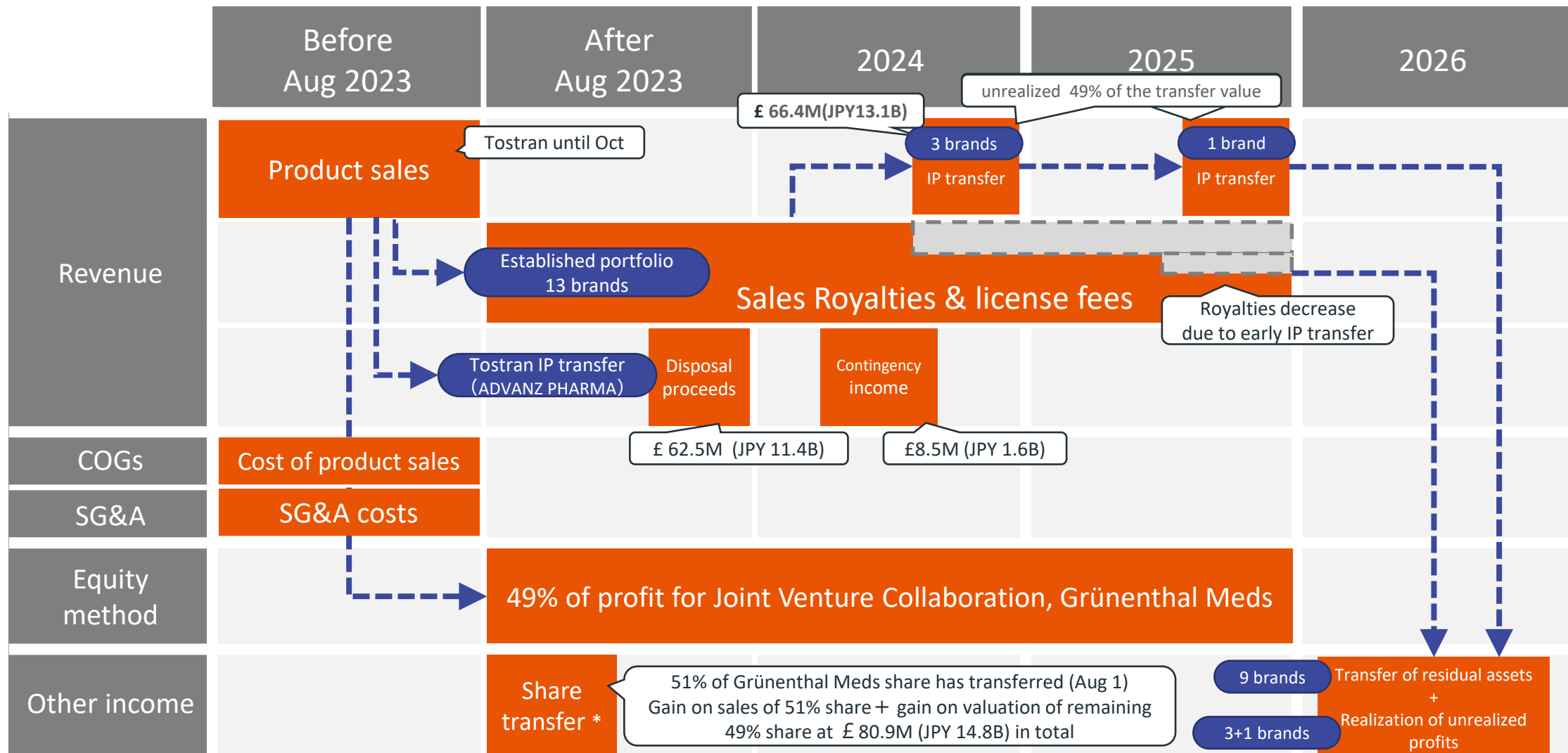
Kyowa Kirin will step into a new stage of its transformation into a Japan-based global specialty pharmaceutical company by making enhancements to the management team at the global level which we believe will help us achieve further heights. In the new structure, we have decided to establish a Chief Operating Officer (COO), and to adopt a dual structure of CEO and COO. The Chairman & CEO will lead discussions on the Kyowa Kirin's direction and overall strategy, while maintaining relationships with stakeholders. The President & COO will oversee the execution of all business operations at the global level, enhancing collaboration across regions and functions, and will promptly and steadily advance the management strategy.



Abdul Mullick  
Nominee for the President and COO

	Name	New Position	Current Position
New	Masashi Miyamoto Ph D.	Representative Director Chairman and Chief Executive Officer (CEO)	Representative Director President and Chief Executive Officer (CEO)
New	Abdul Mullick Ph D.	Representative Director President and Chief Operating Officer (COO)	Managing Executive Officer and Chief International Business Officer (CIBO)
Retiring	Yutaka Osawa Ph D.		Representative Director Executive Vice President and Chief Compliance Officer (CCO)

# P/L Impact on EMEA established medicines portfolio (updated Q3 2024)



\* 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 → 9 brands, via exercising an option in Q1, 2026

# P/L Impact on Restructuring of APAC business

		Country / region	Until September 2024	October 2024 onwards
Revenue	Divest (Established medicines portfolio)	CN	Sales to market  <small>In addition to sales to market, KR/TW Inventory transfer to partner occurred (JPY 5.4B) at the end of September,</small>	Sales to Partner
	Partnering (Established medicines portfolio & Global products)	CN/HK/MO/ MY/SG/TH /KR/TW		Sales to Partner
	Continuation of in-house (Global products)	KR/TW/AU		Sales to market
COGs		ALL	COGs	COGs
SG&A	Divest / Partnering	CN/HK/MO/ MY/SG/TH /KR/TW	SG&A	
	Continuation of in-house (Global products)	KR/TW/AU		SG&A
Other income / expenses			Gain on sales of shares Business restructuring expenses	Business restructuring expenses

# Appendix

# 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.

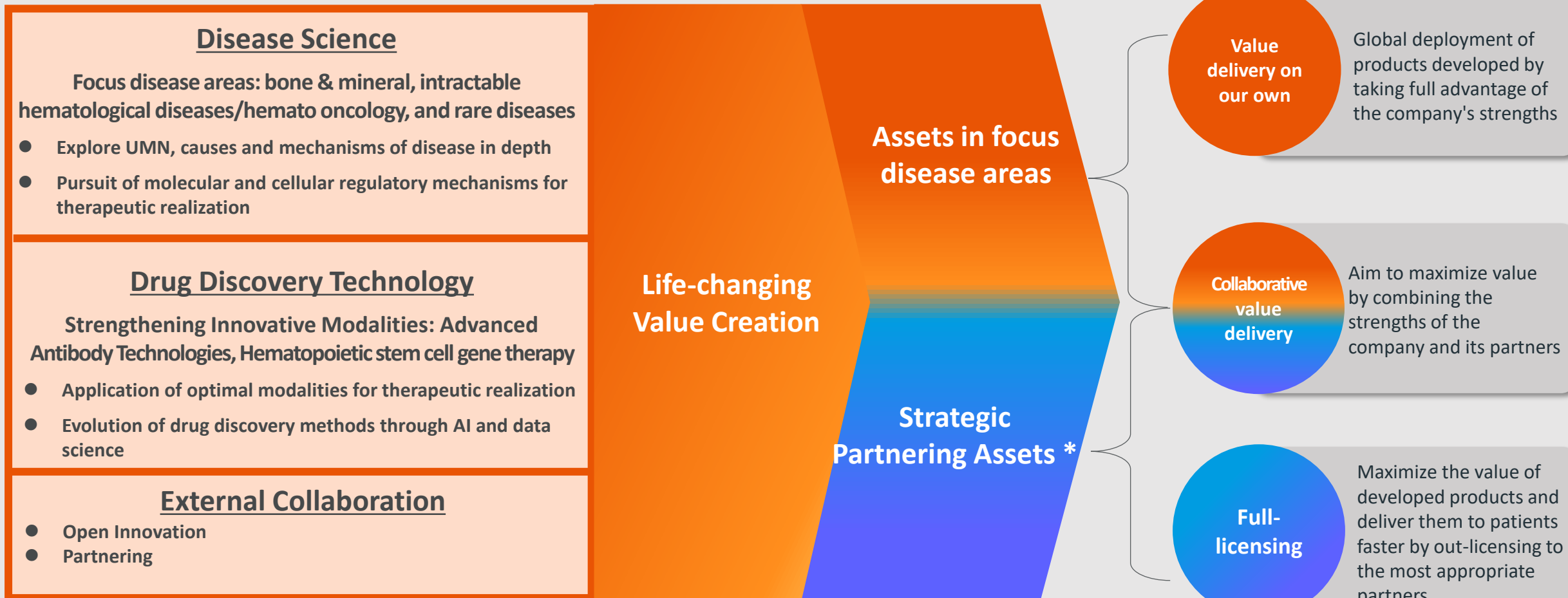


\* Make patients smile through dramatic improvements in quality of life by identifying the unmet medical needs of people battling with medical conditions and by creating and supplying new drugs or services that help them overcome those challenges.



# Story for Vision 2030

## Strategies for creating and delivering life-changing value

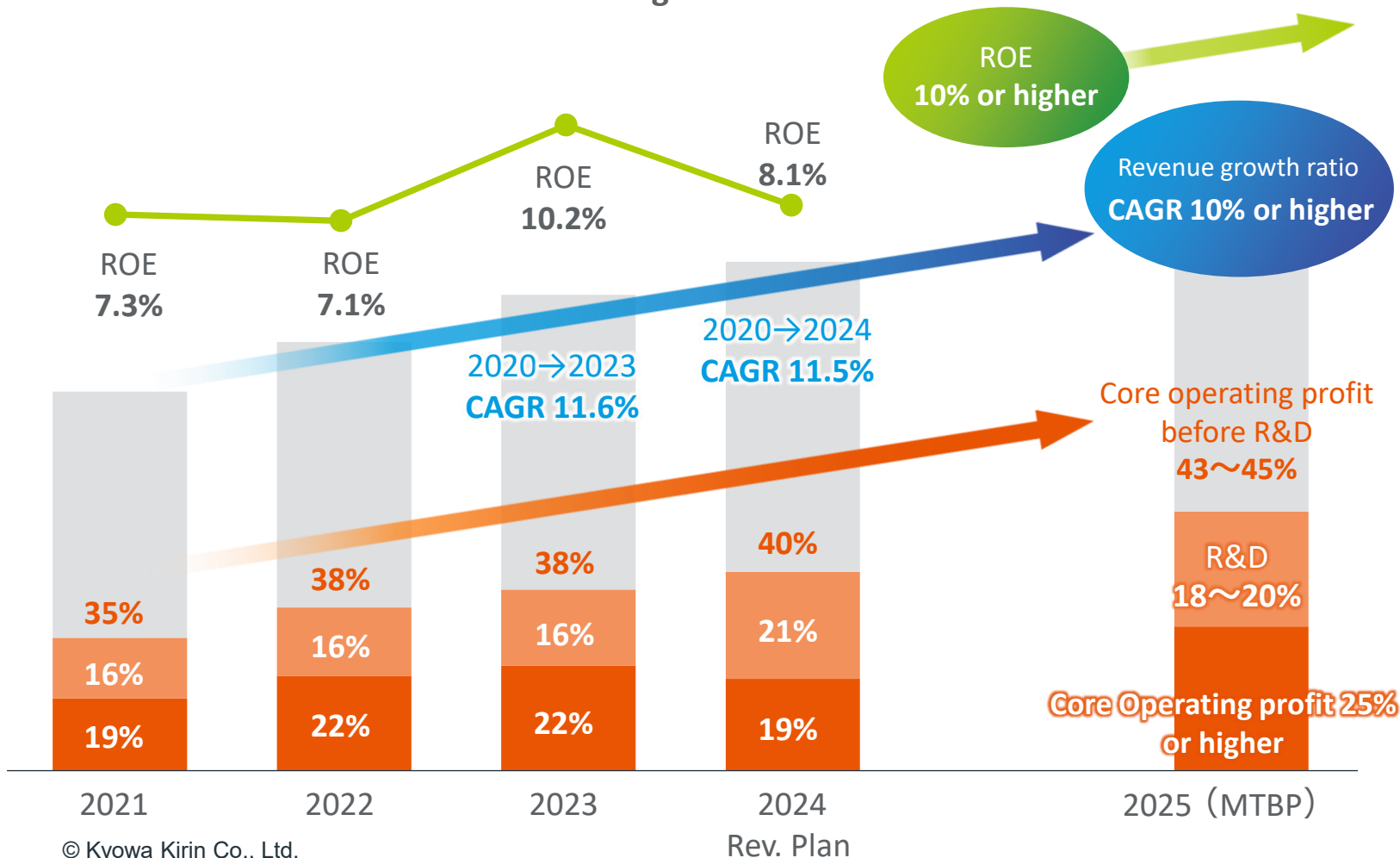


\*Assets outside of the disease areas of focus are designated as strategic partnering assets, and value maximization is achieved through collaboration with partners.

# 2021-2025 Medium Term Business Plan

## - 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

- 
- 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)

# Accounting treatment of share acquisition of Orchard Therapeutics (Finalized)

- ✓ Completed the share acquisition on January 24, 2024, and started consolidation from the February 2024
- ✓ Recognized intangible assets of \$208M (JPY 30.8B) and goodwill of \$230M (JPY 34.1B)
- ✓ Intangible assets will be amortized over 20 years (19 years for Libmeldy/Lenmeldy)

(Unit: Million USD)

<p><b>【Breakdown of Intangible \$208M (JPY 30.8B)】</b></p> <ul style="list-style-type: none"> <li>• Libmeldy/Lenmeldy \$118M (JPY 17.5B)</li> <li>• OTL-203 \$90M (JPY 13.3B)</li> </ul> <p><b>【Annual amortization amount】</b></p> <ul style="list-style-type: none"> <li>• Libmeldy/Lenmeldy \$6M /year ⇒ Amortization started from Feb 2024</li> <li>• OTL-203 \$4M /year ⇒ To be amortized after market launch</li> </ul>	←	<table style="width: 100%; border-collapse: collapse;"> <tr> <td style="width: 50%; text-align: center; padding: 5px;"><b>Other assets</b> 122</td> <td style="width: 50%; text-align: center; padding: 5px;"><b>Other liabilities</b> 91</td> </tr> <tr> <td style="text-align: center; padding: 5px;"><b>Intangible assets</b> 208</td> <td rowspan="3" style="text-align: center; padding: 5px;"><b>Acquisition costs</b> 478</td> </tr> <tr> <td style="text-align: center; padding: 5px;"><b>Goodwill</b> 230</td> </tr> <tr> <td style="text-align: center; padding: 5px;"><b>Other Expenses</b> 9</td> </tr> </table>	<b>Other assets</b> 122	<b>Other liabilities</b> 91	<b>Intangible assets</b> 208	<b>Acquisition costs</b> 478	<b>Goodwill</b> 230	<b>Other Expenses</b> 9
	<b>Other assets</b> 122	<b>Other liabilities</b> 91						
	<b>Intangible assets</b> 208	<b>Acquisition costs</b> 478						
	<b>Goodwill</b> 230							
<b>Other Expenses</b> 9								

✓ The acquisition costs above (\$478 million) include amounts for options, Restricted Stock Units and other instruments which are paid by Orchard. The acquisition costs under business combination accounting is \$386 million (approximately 57.1 billion yen)

# Main Development Pipeline Products (After Ph2)

	Diseases under development* <sup>1</sup>	Planned Approval Year* <sup>2</sup>	Development status	Total addressable market* <sup>3</sup>	No. of Patients* <sup>4</sup>
KHK4083/AMG 451 rocatinlimab	Moderate to severe Atopic Dermatitis	2026/2027	P3 (Global)	★★★★★	16M
	Prurigo nodularis	TBD	P3 (Global)	★★★★	1M
	Moderate to severe Asthma	TBD	P2 (Global)	★★★★★	13.5M
KHK4951 tivozanib	nAMD	TBD	P2 (JP, US)	★★★★	2,600K
	DME	TBD	P2 (JP, US)	★★★★	3,400K
OTL-203	MPS-IH (Hurler syndrome)	2029/2030	Registrational study* <sup>5</sup> (US, EU)	★	(1 in 100K live births)* <sup>6</sup>
OTL-201	MPS-IIIA (Sanfilippo syndrome type A)	TBD	Proof-of-concept* <sup>7</sup>	★	(~1 in 100K live births)

\*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 60 percent of patients have the Hurler subtype. \*7 Equivalent to P1/2 study.

# Main Development Pipeline Products (nonclinical ~ Ph1)

As of Oct. 31, 2024

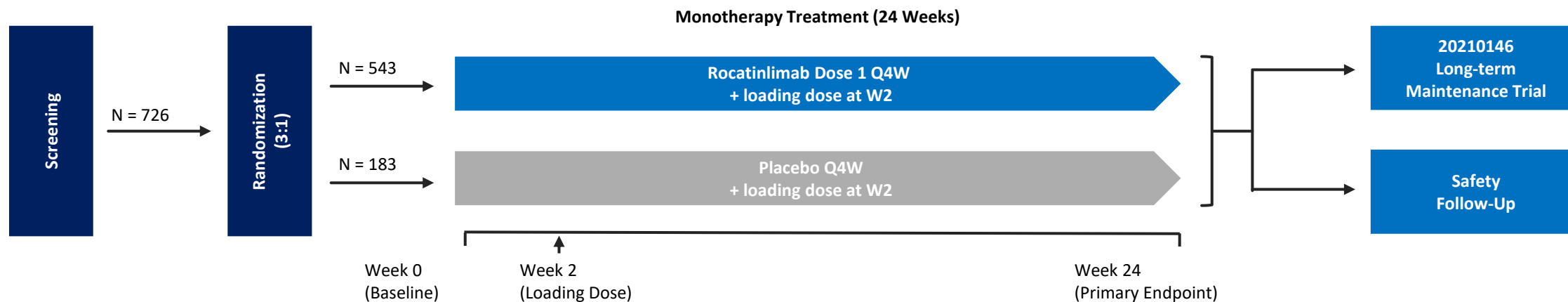
	Diseases under development*1	Development status	Modality, technology
<b>KK4277</b>	SLE, CLE	P1 (JP)	Antibody, POTELLIGENT®
<b>KK2260</b>	Advanced or metastatic solid tumors	P1 (JP: in progress, US: in preparation)	Antibody, REGULGENT™
<b>KK2269</b>	Advanced or metastatic solid tumors	P1 (JP, US)	Antibody, REGULGENT™
<b>KK2845</b>	AML	P1 (JP)	Antibody-Drug Conjugate
<b>KK8123</b>	XLH	Preparation underway for P1 (US, EU)	Antibody

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

# Main Development Pipeline Products: Future plans

Code Generic Name	Target Disease		2024	2025	2026	+	
KHK4083/ AMG 451 rocatinlimab	Moderate to severe atopic dermatitis	P3					 IGNITE HORIZON SHUTTLE ASTRO ORBIT VOYAGER ASCEND OUTPOST
		P3					
		P3					
		P3					
		P3					
		P3					
		P3					
		P3					
	Prurigo nodularis	P3					
	Moderate to severe asthma	P2					
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					

# ROCKET HORIZON Study design



## KEY ELIGIBILITY CRITERIA

- ≥ 18 yo, M2S AD
- vIGA-AD 3 or 4
- EASI ≥ 16
- BSA ≥ 10%
- 7-day recall worst pruritus NRS ≥ 4
- Topical failure; bio experienced included

## KEY DESIGN CONSIDERATIONS

**Rescue therapy** was allowed, if deemed necessary

- Subjects who used rescue therapy were considered non responders
- Study treatment was to be discontinued if systemic rescue therapy for AD was used (except for corticosteroids used for ≤ 14 days)

### Stratification:

- vIGA-AD 3 vs. vIGA-AD 4
- Japan vs. Non-Japan Asian countries vs. RoW

Q4W = every 4 weeks; W2 = week 2; vIGA-AD = Validated Investigator Global Assessment for Atopic Dermatitis; EASI = Eczema Area and Severity Index; BSA = body surface area; NRS = numerical rating scale; RoW = rest of world.

# ROCKET HORIZON: Summary of Results

## ■ Co-primary endpoints were achieved

- vIGA-AD<sup>TM</sup> 0/1<sup>1</sup> with a  $\geq 2$ -point reduction from baseline: Ex-US  
 rocatinlimab 19.3% vs. Placebo 6.6% (12.8% difference,  $p < 0.001$ )
- EASI-75<sup>2</sup> : Ex-US For US  
 rocatinlimab 32.8% vs. Placebo 13.7% (19.1% difference,  $p < 0.001$ )
- rIGA 0/1<sup>3</sup> : For US  
 rocatinlimab 16.4% vs. Placebo 4.9% (11.5% difference,  $p < 0.001$ )

## ■ All key secondary endpoints<sup>4</sup> were also achieved

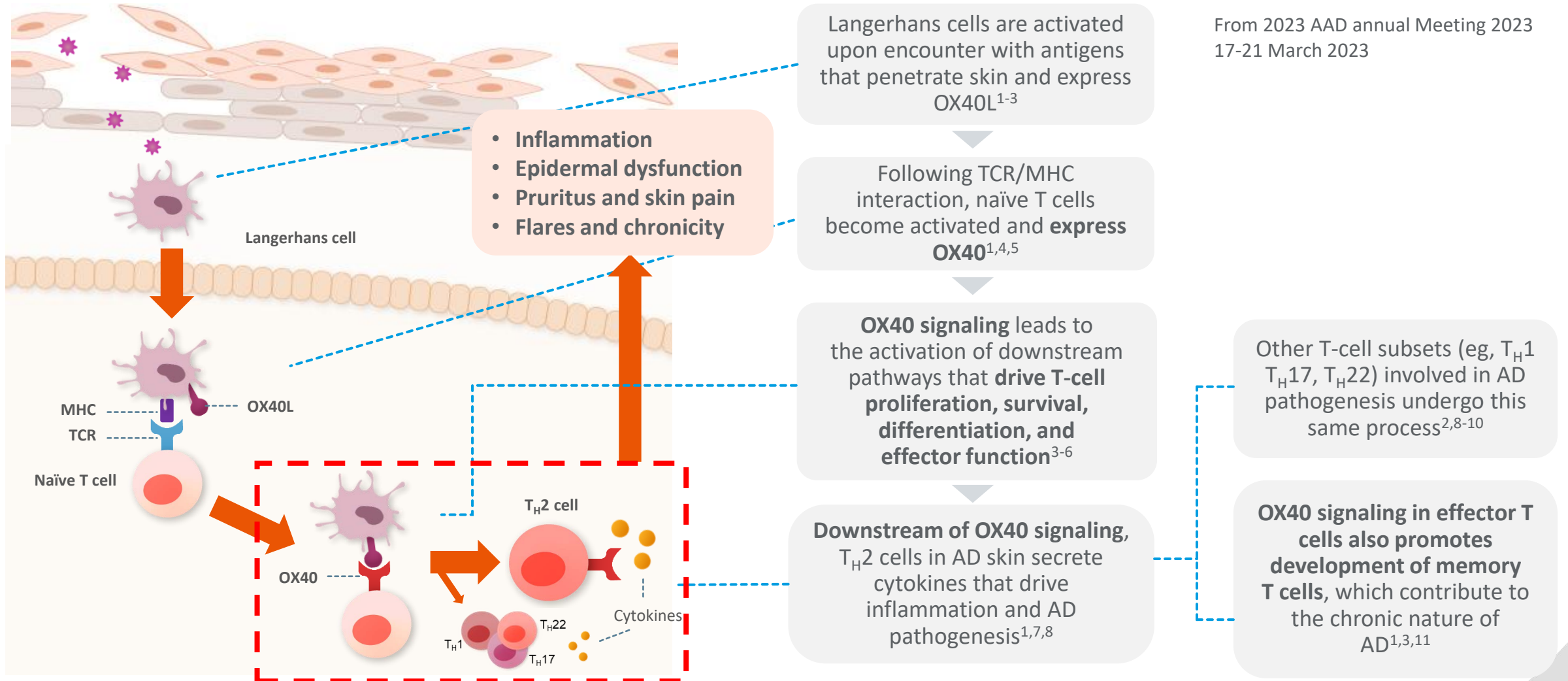
## ■ Overall safety results were comparable to the Phase 2b trial

1. validated Investigator Global Assessment for Atopic Dermatitis score of 0 (clear) or 1 (almost clear)
2.  $\geq 75\%$  reduction from baseline in Eczema Area and Severity Index score
3. A more stringent measure of efficacy than vIGA 0/1. Defined as achieving vIGA-AD 1 response with presence of only barely perceptible erythema or vIGA-AD 0 response and  $\geq 2$ -point reduction from baseline
4. vIGA 0/1 and EASI-75 at week 16 and EASI-90 at week 24, the Pruritus Numeric Rating Scale, Atopic Dermatitis Skin Pain Scale, Dermatology Quality of Life Index, and severity scores of hand atopic dermatitis and facial atopic dermatitis



# Critical Role of OX40 Signaling in Orchestrating T-Cell Driven Inflammation and AD Pathogenesis

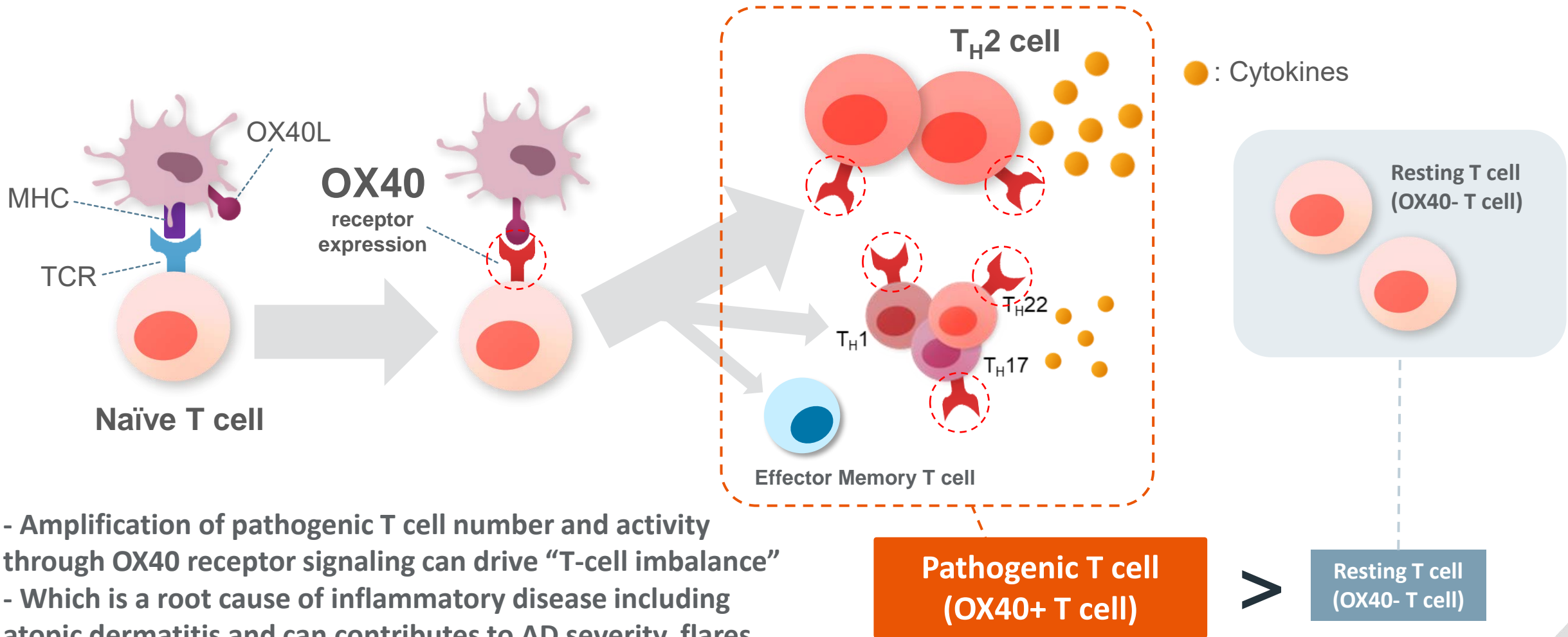
From 2023 AAD annual Meeting 2023  
17-21 March 2023



AD=atopic dermatitis; MHC=major histocompatibility complex; OX40=OX40 receptor; OX40L=OX40 ligand; TCR=T-cell receptor; TH=T helper cell.

1. Furue M, et al. *J Clin Med.* 2021;10:2578. 2. Guttman-Yassky E, et al. *Semin Cutan Med Surg.* 2017;36:100-103. 3. Croft M, et al. *Immunol Rev.* 2009;229:173-191. 4. Magee CN, et al. *Am J Transplant.* 2012;12:2588-2600. 5. Goronzy JJ, et al. *Arthritis Res Ther.* 2008;10(suppl 1):S3. 6. Mascarelli DE, et al. *Front Cell Dev Biol.* 2021;9:692982. 7. Krohn IK, et al. *Allergy.* 2022;77:827-842. 8. De Bruyn Carlier T, et al. *J Autoimmun.* 2021;120:1026345. 9. Kumar S, et al. *Int J Mol Sci.* 2019;20:2159. 10. Fu Y, et al. *Acta Pharm Sin B.* 2020;10:414-433. 11. Chen L, et al. *Cell Mol Immunol.* 2020;17:64-75.

# OX40 Signaling induces T-cell imbalance



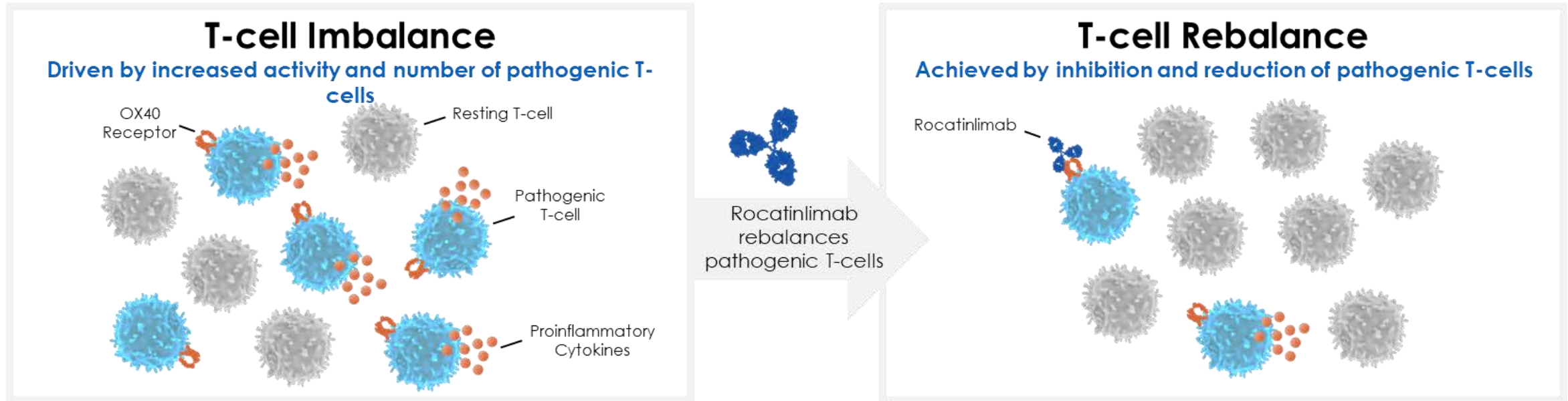
- Amplification of pathogenic T cell number and activity through OX40 receptor signaling can drive “T-cell imbalance”
- Which is a root cause of inflammatory disease including atopic dermatitis and can contribute to AD severity, flares, and disease persistence

**Pathogenic T cell (OX40+ T cell)**

**Resting T cell (OX40- T cell)**

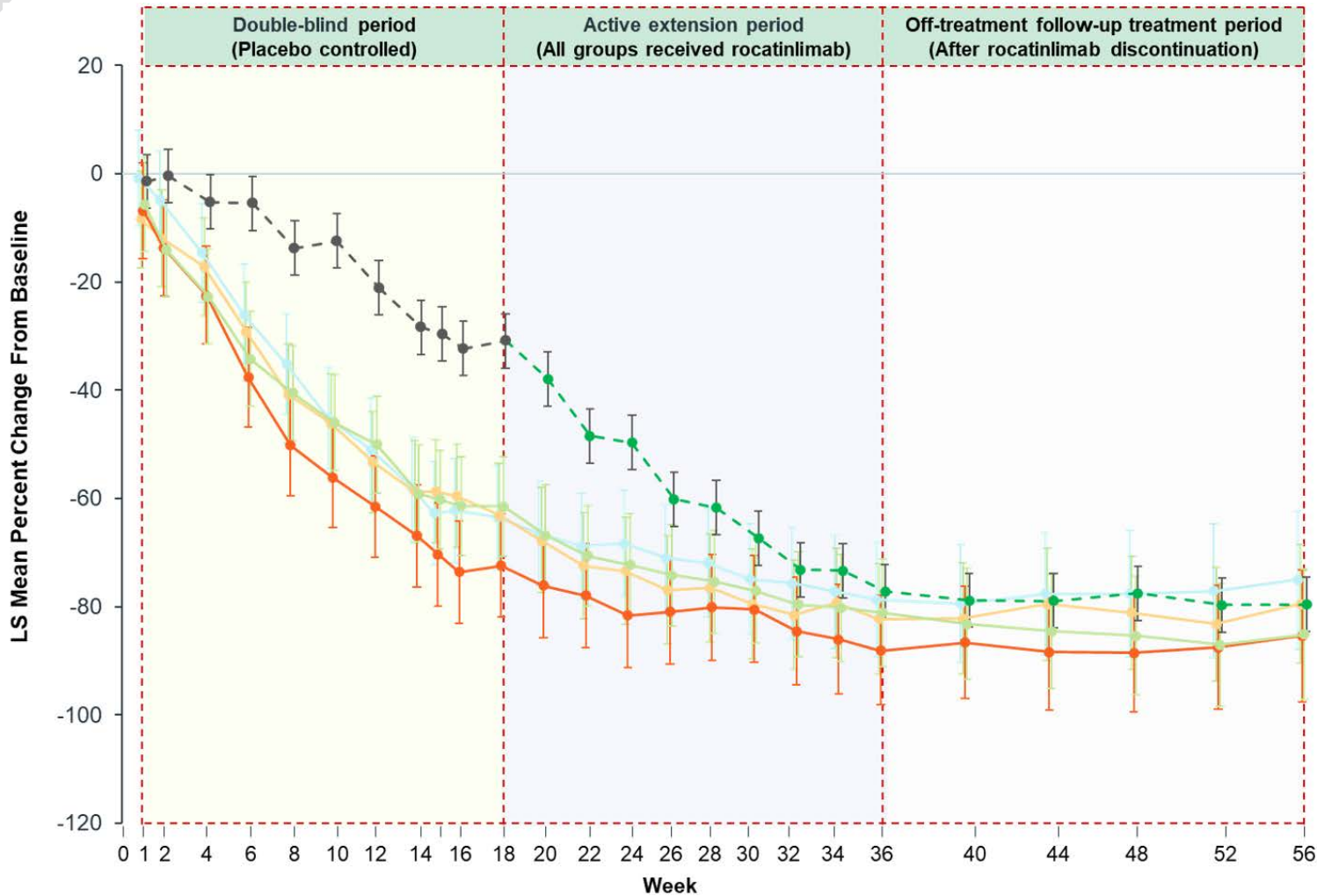
>

# Rocatinlimab Rebalances T-cells by Targeting OX40 Receptor



- T-cell imbalance is a root cause of inflammatory disease
- Atopic dermatitis is driven in part by the proliferation of pathogenic T-cells
- Rocatinlimab has the potential to inhibit and reduce pathogenic T-cells across heterogeneous patient types by targeting OX40 inhibitor

# Rocatinlimab: Phase 2b data<sup>1</sup>



The Least-squares (LS) mean percent change in Eczema Area and Severity Index (EASI) score

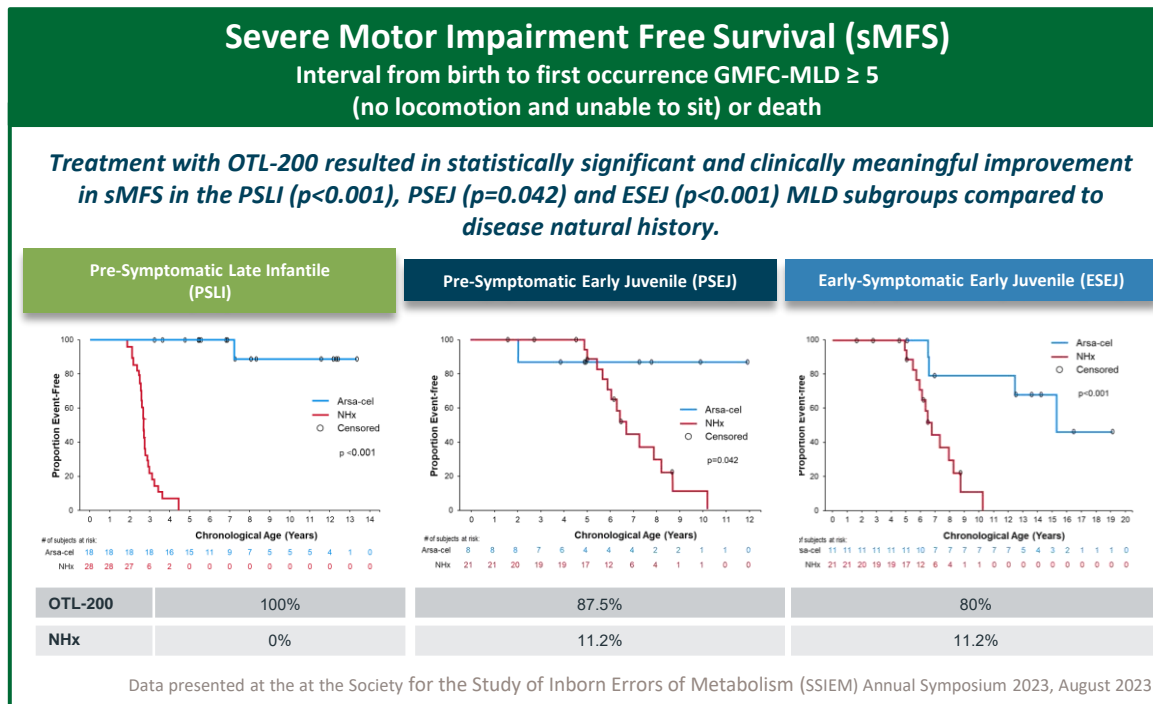
	Week 16	Week 24	Week 36	Week 56
Rocatinlimab 150 mg Q4W, %	-62.2	-68.3	-78.7	-75.0
Rocatinlimab 600 mg Q4W, %	-59.5	-73.4	-82.3	-79.5
Rocatinlimab 300 mg Q2W, %	-73.6	-81.6	-88.1	-85.4
Rocatinlimab 600 mg Q2W, %	-61.4	-72.2	-81.1	-85.1
Placebo/rocatinlimab 600 mg Q2W, %	-32.3	-49.7	-77.2	-79.6

**Sustained improvement in EASI after treatment discontinuation (Week 36)**

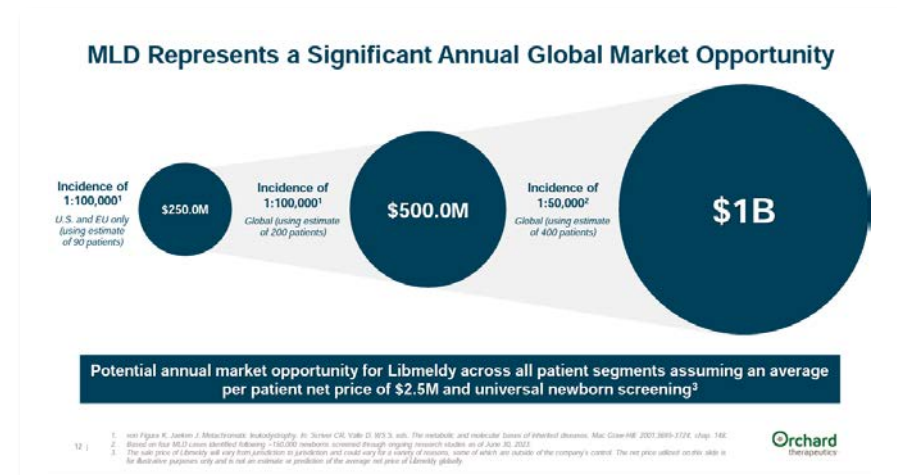
# Libmeldy® (OTL-200, atidarsagene autotemcel)

## ■ MLD (Metachromatic Leukodystrophy)

- Fatal genetic CNS disorder
- Rapid and irreversible loss of motor and cognitive function
- In its most severe form, most children pass away within five years of symptom onset<sup>1</sup>



Ref.) Orchard Therapeutics plc, Q2 2023 Financial Results and Webcast <https://ir.orchard-tx.com/static-files/9fed8b65-2fd9-491a-97c0-69bf6595c0c3>



Potential annual market opportunity for Libmeldy® across all patient segments assuming an average per patient net price of \$2.5M and universal newborn screening<sup>2</sup>

1. van Rappard DF, Boelens JJ, Wolf NI. Metachromatic leukodystrophy: disease spectrum and approaches for treatment. Best Pract Res Clin Endocrinol Metab 2015; 29: 261–73.  
2. The sale price of Libmeldy® will vary from jurisdiction to jurisdiction and could vary for a variety of reasons, some of which are outside of the company's control. The net price utilized on this slide is for illustrative purposes only and is not an estimate or prediction of the average net price of Libmeldy® globally.

# FOREX Information

## Average FOREX Rates (yen)

	2023Q3	2024Q3	Changes	2024 Rev. Plans
USD	137	151	+14	151
GBP	170	193	+23	191
EUR	148	164	+16	163

## Q1 YoY FOREX Impacts (billion yen)

	Revenue	Core OP
USD	+14.2	+4.7
GBP	+2.3	+0.2
EUR	+3.8	+2.1

## FY2024 FOREX Sensitivities (based on 2024 Plans, 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



# KHK4083/AMG 451 - Collaboration with Amgen -

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

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
ATL	JP	1,150 / y		Survey and countermeasures to HTLV-1 infection and related diseases in Japan. 2009 summary research report (Yamaguchi, 2010)
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
PE	JP		15,000	Estimate based on the Demographic Survey by the Ministry of Health, Labour and Welfare and the estimated incidence of this disease

\*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
CTCL	cutaneous T cell lymphoma
DME	Diabetic Macular Edema
EMEA	Europe, the Middle East and Africa
JP	Japan
LCM	Lifecycle Management
MDS	Myelodysplastic syndromes
MF	Mycosis fungoides
MLD	Metachromatic Leukodystrophy
MPS-IH	Mucopolysaccharidosis type I, Hurler syndrome
MPS-IIIA	Mucopolysaccharidosis type IIIA
NA	North America
nAMD	neovascular Age-related Macular Degeneration
PTCL	peripheral T-cell lymphoma
SS	Sézary syndrome
TIO	Tumor Induced Osteomalacia
XLH	X-linked Hypophosphatemia



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