

R&D pipeline

small molecule
 antibody
 HSC-GT

Updated since Dec. 31, 2024

Updated since Jun. 30, 2025

As of Sep 30, 2025

Code Name Generic Name Formulation		Mechanism of Action	Indication	Stage			[In-House or Licensed] Remarks
				PhI	PhII	PhIII	
	KK8123 Injection	Anti-FGF23 Fully Human Antibody	X-linked Hypophosphatemia				[In-House] Clinical study is being conducted in NA and EU as a global product
	KK8398 infigratinib Oral	FGFR3 Inhibitor	Achondroplasia				[QED Therapeutics] Preparation underway for Ph III in JP
	ziftomenib ※ Oral	Menin Inhibitor	Acute Myeloid Leukemia (AML) (Monotherapy)				[Kura Oncology] The detailed results presented at ASCO in June 2025 and published on JCO in September 2025 Adult Relapsed or Refractory AML with a NPM1 Mutation KOMET-001
			Acute Lymphoblastic Leukemia (ALL) (Monotherapy)				Clinical study is being conducted in NA and EU as a global product KMT2A-rearranged ALL KOMET-001
			Acute Myeloid Leukemia (AML) (Monotherapy)				Clinical study is being conducted in NA and EU as a global product Non-NPM1-mutant AML/Non-KMT2A-rearranged AML KOMET-001
			Acute Myeloid Leukemia (AML) (Combination)				Clinical study is being conducted in NA as a global product NPM1-mutant AML/KMT2A-rearranged AML Combinations with venetoclax + azacitidine, and cytarabine + daunorubicin KOMET-007
							Clinical study is being conducted in NA and EU as a global product NPM1-mutant AML/KMT2A-rearranged AML Combinations with gilteritinib, FLAG-IDA, LDAC KOMET-008
							Clinical study is being conducted as a global product NPM1-mutant AML/KMT2A-rearranged AML Combinations with venetoclax + azacitidine, and cytarabine + daunorubicin KOMET-017
	KK2845	Anti-TIM-3 ADC	Acute Myeloid Leukemia (AML)				[In-House] Antibody-Drug Conjugate Clinical study is being conducted in JP as a global product
	OTL-203	Hematopoietic Stem Cell (HSC) Gene Therapy	MPS-IH (Hurler Syndrome)				[In-House] Rare Pediatric Disease (RPD) and Fast Track designations (FDA) Priority Medicines (PRIME) designation (EMA) Area of clinical study: NA and EU
	OTL-201	Hematopoietic Stem Cell (HSC) Gene Therapy	MPS-IIIA (Sanfilippo Syndrome type A)	Ph I / Ph II			[In-House] Rare Pediatric Disease (RPD) designation (FDA) Preparation underway for registrational study (equivalent to PhIII study)
	KHK4083/AMG 451 rocatinlimab Injection	Anti-OX40 Antibody	Moderate to Severe Atopic Dermatitis				[In-House] POTELLIGENT Human monoclonal antibody production technology Collaboration agreement with Amgen for the development of rocatinlimab in all the countries except for Japan Clinical study is being conducted in JP, NA, EU, UK, Middle East, Asia, Oceania, and other regions as a global product
			Prurigo Nodularis				Clinical study is being conducted in JP, NA, EU, Asia, and Oceania as a global product
			Moderate to Severe Asthma				Clinical study is being conducted in JP, NA, EU, Asia, and Oceania as a global product

※ For detailed information on ziftomenib's development status, please refer to Kura Oncology's website. <https://kuraoncology.com/>

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				PhI	PhII	PhIII	
	KHK4951 tivozanib Ophthalmic	VEGF Receptor Tyrosine Kinase Inhibitor	Diabetic Macular Edema				[In-House] Clinical study is being conducted in JP, NA, Asia, and Oceania as a global product
			Neovascular Age-Related Macular Degeneration				Clinical study is being conducted in JP, NA, Asia, and Oceania as a global product
	KK2260 Injection	EGFR-TfR1Bispecific Antibody	Advanced or Metastatic Solid Tumors				[In-House] REGULGENT Fully human antibody production technology Clinical study is being conducted in JP, and a clinical study is prepared under way for PhI in NA as a global product
	KK2269 Injection	EpCAM-CD40Bispecific Antibody	Advanced or Metastatic Solid Tumors				[In-House] REGULGENT Fully human antibody production technology Clinical study is being conducted in JP and NA as a global product
	KK4277 Injection	Anti-PTPRS Humanized Antibody	Systemic Lupus Erythematosus/Cutaneous Lupus Erythematosus				[SBI Biotech] POTELLIGENT Clinical study is being conducted in JP and Asia
	KK3910 Injection		Essential Hypertension				[In-House] Clinical study is being conducted in JP as a global product

※ Launch of clinical trial of ziftomenib evaluating dual inhibition of NPM1 and FLT3 mutations in patients with newly diagnosed AML (KOMET-007) was announced on the 2nd of October, 2025.

※ OTL-200 was granted orphan regenerative medicine product designation for early-onset MLD in Japan in October 2025

Major Applications and Approvals

As of Sep. 30, 2025

Code Name, Generic Name, Product Name	Indication	Application/Under Review	Countries/Regions Received Approval in 2025
ziftomenib	Adult Relapsed or Refractory (R/R) Acute Myeloid Leukemia (AML) with a Nucleophosmin1 (NPM1) Mutation	US	—
KHK4827(brodalumab, Product name in Japan and Asia: Lumicef)	Palmoplantar Pustulosis	—	TW

We withdrew an application of AMG531 for Aplastic Anemia in Taiwan, and the relevant application information has been removed from this table.