R&D pipeline

small molecule antibody HSC-GT

As of Mar 31, 2025

Code Name Generic Name Formulation	Mechanism of Action	Indication	Stage PhI / PhII / PhIII	[In-House or Licensed] Remarks
KK8123 Injection	Anti-FGF23 Fully Human Antibody	X-linked Hypophosphatemia	PhI	[In-House] Clinical study is being conducted in NA and EU as a global product
KK8398 infigratinib Oral	FGFR 3 Inhibitor	Achondroplasia		[QED Therapeutics] Preparation underway for Ph Ⅲ in JP
ziftomenib ※ Oral	Menin Inhibitor	Acute Myeloid Leukemia (AML) (Monotherapy)	PhI / PhII	[Kura Oncology] Topline results reported in February 2025 Adult Relapsed or Refractory AML with a NPM1 Mutation KOMET-001
		Acute Lymphoblastic Leukemia (ALL) (Monotherapy)	PhI	Clinical study is being conducted in NA and EU as a global product KMT2A-rearranged ALL KOMET-001
		Acute Myeloid Leukemia (AML) (Monotherapy)	PhI	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)	PhI	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
			PhI	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
KK2845	Anti-TIM-3 ADC	Acute Myeloid Leukemia (AML)	PhI	[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)	PhI / PhII / PhIII	[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)	PhI / PhII Ph Ⅰ / Ph Ⅱ	[In-House] Rare Pediatric Disease (RPD) designation (FDA) Preparation underway for registrational study (equivalent to Ph Ⅲ study)
KHK4083/AMG 451 rocatinlimab Injection	Anti-OX40 Antibody	Moderate to Severe Atopic Dermatitis	PhI / PhII / PhIII	[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	PhI / PhII / PhIII	Clinical study is being conducted in JP, NA, EU, Asia, and Oceania as a global product
		Moderate to Severe Asthma	PhI / PhII	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/

Code Name Generic Name Formulation		Mechanism of Action	Indication	Stage			[In-House or Licensed] Remarks
				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] Preparation underway as a global product

Note: Ph I clinical study of KK3910 was started in April 2025.

Major Applications and Approvals

As of Mar 31, 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 Nucelophosmin1 (NPM1) Mutation	US	–
KHK4827(brodalumab, Product name in Japan and Asia: Lumicef)	Palmoplantar Pustulosis	TW	–
AMG531(romiplostim, Product name in Japan: Romiplate)	Aplastic Anemia	TW	–