R&D pipeline

small molecule antibody HSC-GT

As of Mar 31, 2026

	Code Name Generic Name Formulation	Mechanism of Action	Indication	Stage			[In-House or Licensed] Remarks
				PhI	PhII	PhIII	
antibody	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
small molecule	KK8398 infigratinib Oral	FGFR3 Inhibitor	Achondroplasia	→	→	→	[QED Therapeutics] Clinical study is being conducted in JP
			Hypochondroplasia				Preparation underway for Ph Ⅲ clinical trial in JP
small molecule	ziftomenib ※ Oral	Menin Inhibitor	Acute Lymphoblastic Leukemia (ALL) (Monotherapy)	→			[Kura Oncology] 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
							Adult Relapsed or Refractory AML with a NPM1 Mutation Preparation underway for Ph Ⅱ clinical trial in JP
			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 as a global product FLT3/NPM1 co-mutated AML Combinations with cytarabine + daunorubicin, and quizartinib 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
antibody	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

※ For detailed information on ziftomenib(Product Name in US: KOMZIFTI)'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	
	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 / II		[In-House] Rare Pediatric Disease (RPD) designation (FDA) Preparation underway for registrational study (Equivalent to PhⅢ study)
	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
	OTL-200 atidarsagene autotemcel	Hematopoietic Stem Cell (HSC) Gene Therapy	Early-onset Metachromatic Leukodystrophy (MLD)				[In- House] Orphan Regenerative Medicine Product Designation in JP A Ph.3 clinical study in JP is under preparation Product Name in US: Lenmeldy Product Name in Europe: Libmeldy
	KK2223		Cutaneous T cell lymphoma Peripheral T cell lymphoma				[In-House] Clinical study is prepared under way for Ph I

Note: Since clinical studies of KHK4084/AMG 451 （rocatinlimab） for moderate to severe atopic dermatitis, prurigo nodularis, and moderate to severe asthma were c ontinued, the relevant information was deleted from this table.
Note: On April 24, 2026, the Company announced that the first patient has been dosed in the phase II clinical trial in Japan evaluating ziftomenib for the treatment of relapsed r efractory NPM1-mutated AML.

Major Applications and Approvals

As of Mar. 31, 2026

Code Name, Generic Name, Product Name	Indication	Application/Under Review	Countries/Regions Received Approval in 2026
OTL-200(atidarsagene autotemcel, Product name in Europe/US : Libmeldy/Lenmeldy)	Metachromatic Leukodystrophy（MLD）	filied in Japan	–