R&D pipeline

small molecule　antibody　HSC-GT　　✓ Updated since Jun. 30, 2026

As of Sep. 30, 2026

Code Name / Generic Name / Formulation	Mechanism of Action	Indication	Stage	[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	FGFR3 Inhibitor	Achondroplasia	PhI–PhII–PhIII	[QED Therapeutics] Clinical study is being conducted in JP Orphan Drug Designation in JP
		Hypochondroplasia		Preparation underway for Ph Ⅲ clinical trial in JP Orphan Drug Designation in JP
ziftomenib ※ Oral	Menin Inhibitor	Acute Lymphoblastic Leukemia (ALL) (Monotherapy)	PhI	[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)	PhI	Clinical study is being conducted in NA and EU as a global product Non-NPM1-mutant AML/Non-KMT2A-rearranged AML KOMET-001
			PhII	Adult Relapsed or Refractory AML with a NPM1 Mutation Ph Ⅱ clinical study is being conducted in JP Orphan Drug Designation in JP
		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 as a global product FLT3/NPM1 co-mutated AML Combinations with cytarabine + daunorubicin, and quizartinib KOMET-007
			PhI	Clinical study is being conducted in NA and EU as a global product NPM1-mutant AML/KMT2A-rearranged AML/FLT3-mutant AML Combinations with gilteritinib, FLAG-IDA, LDAC KOMET-008
			PhI–PhII–PhIII	Clinical study is being conducted as a global product NPM1-mutant AML/KMT2A-rearranged AML Combinations with venetoclax + azacitidine (for NPM1-mutant only), and cytarabine + daunorubicin KOMET-017
KK2845	Anti-TIM-3 ADC	Acute Myeloid Leukemia (AML) and other Hematologic Malignancies	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), Fast Track and Regenerative Medicine Advanced Therapy (RMAT) 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) Orphan Drug Designation (FDA, EMA) Innovation Passport Designation (MHRA)
KHK4951 tivozanib Ophthalmic	VEGF Receptor Tyrosine Kinase Inhibitor	Diabetic Macular Edema	PhI–PhII	[In-House] Clinical study is being conducted in JP, NA, Asia, and Oceania as a global product
		Neovascular Age-Related Macular Degeneration	PhI–PhII	Clinical study is being conducted in JP, NA, Asia, and Oceania 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/

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Code Name Generic Name Formulation	Mechanism of Action	Indication	Stage			[In-House or Licensed] Remarks
			PhI	PhII	PhIII	
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 completed 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 EU: Libmeldy
KK2223		Cutaneous T cell lymphoma Peripheral T cell lymphoma	✓			[In-House] Antibody-Drug Conjugate Clinical study is being conducted for Ph I in NA Clinical study is preparing for Ph I in EU
KK8005		X-linked Hypophosphatemia	✓			[In-House] Clinical study is being conducted for Ph I / II in JP as a global product
KK4408		Autoimmune Disease				[In-House] REGULGENT Clinical study is preparing for Ph I / II

※ Note: Our main progress from September 30, 2026 is as follows.
On October 6, 2026, the first patient has been dosed in the phase I / II clinical trial in Japan evaluating KK4408 for the treatment of autoimmune disease

Major Applications and Approvals

As of Sep. 30, 2026

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