R&D pipeline

small molecule | large molecule | antibody | HSC-GT | Updated since Dec. 31, 2023 | ✓ Updated since Jun. 30, 2024

As of Sep. 30, 2024

Code Name Generic Name Formulation	Mechanizm of Action	Indication	Stage PhI / PhII / PhIII	[In-House or Licensed] Remarks
KK8123 Injection	Anti-FGF23 Fully Human Antibody	X-linked Hypophosphatemia		[In-House] Preparation underway for PhI as a global product
KK2845	Anti-TIM-3 ADC	Acute Myelogenous Leukemia		[In-House] Antibody-Drug Conjugate Preparation underway for PhI in Japan 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 Ph Ⅲ 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
KHK4951 tivozanib Ophthalmic	VEGF Receptor Tyrosine Kinase Inhibitor	Diabetic Macular Edema		[In-House] Clinical sudy is being conducted in JP, NA, Asia, and Oceania as a global product
		Neovascular Age-Related Macular Degeneration		Clinical sudy 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 sudy 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 sudy is being conducted in JP and NA as a global product
AMG531 romiplostim Injection	Thrombopoietin Receptor Agonist	Aplastic Anemia Previously Untreated with Immunosuppressive Therapy	Ph Ⅱ/Ph Ⅲ	[Amgen K-A] product name in Japan: Romiplate Area of clinical study: Asia
KK4277 Injection	Anti-PTPRS Humanized Antibody	Systemic Lupus Erythematosus/Cutaneous Lupus Erythematosus		[SBI Biotech] POTELLIGENT Clinical sudy is being conducted in JP and Asia

Major Applications and Approvals

Code Name, Generic Name, Product Name	Indication	Application/Under Review	Countries/Regions Received Approval in 2024
KRN125(pegfilgrastim, Product name in Japan:G-LASTA)	Mobilization of Hematopoietic Stem Cells into Peripheral Blood for Autologous Blood Stem Cell Transplantation	–	JP
OTL-200(atidarsagene autotemcel, Product name in Europe/US : Libmeldy/Lenmeldy)	Metachromatic Leukodystrophy	–	US
KHK4827(brodalumab, Product name in Japan and Asia: Lumicef)	Systemic Sclerosis	JP	–
	Palmoplantar Pustulosis	TW	–
KHK7580(evocalcet, Product name in Japan: Orkedia)	Secondary Hyperparathyroidism	–	CN, TW
AMG531(romiplostim, Product name in Japan: Romiplate)	Aplastic Anemia	TW	–
	Severe Aplastic Anemia	–	KR

Notes: Our main progress from September 30, 2024 is as follows.
・In October 2024, we withdrew an application for partial change of approved indication of KHK4827(brodalumab, Product name in Japan and Asia: Lumicef) for systemic sclerosis in Japan.