

R&D pipeline

small molecule
 large molecule
 antibody
 HSC-GT

Updated since Dec. 31, 2023

As of Mar 31, 2024

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 (XLH)				[In-House] Preparation underway for PhI as a global product
AMG531 Romiplostim Injection	Thrombopoietin Receptor Agonist	Aplastic Anemia Previously Untreated with Immunosuppressive Therapy				[Amgen K-A] product name in Japan: Romiplate Area of clinical study: Asia
KK2845		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 Europe
OTL-201	Hematopoietic Stem Cell (HSC) Gene Therapy	MPS-IIIA (Sanfilippo Syndrome type A)				[In-House] Rare Pediatric Disease (RPD) designation (FDA) Area of clinical study: UK
KHK4083/AMG 451 Rocatinlimab Injection	Anti-OX40 Fully Human Antibody	Atopic Dermatitis				[In-House] POTELLIGENT Fully human antibody production technology Collaboration agreement with Amgen for the development of KHK4083/AMG 451 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				Preparation underway for PhIII as a global product
		Asthma				Preparation underway for Ph II as a global product
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	Solid Tumor				[In-House] REGULGENT Fully human antibody production technology As a global product, clinical study is being conducted in JP, and a clinical study is prepared under way for PH I in NA
KK2269 Injection	EpCAM-CD40Bispecific Antibody	Solid Tumor				[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

Since the development of KW-3357 for disseminated intravascular coagulation, congenital antithrombin deficiency was discontinued in Europe and UK, the relevant information was deleted from this table.

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	–
KHK7580(Evocacet, Product name in Japan: Orkedia)	Secondary Hyperparathyroidism	CN	TW