# R&D pipeline

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

As of Dec. 31, 2024

| Code Name<br>Generic Name<br>Formulation | Mechanizm of Action | Indication | Stage<br>PhI / PhII / PhIII | [In-House or Licensed]<br>Remarks |
|---|---|---|---|---|
| KK8123<br>Injection | Anti-FGF23 Fully Human Antibody | X-linked Hypophosphatemia | ✓ | [In-House]<br>Clinical study is being conducted in NA and EU as a global product |
| KK8398<br>infigratinib<br>Oral | FGFR 3 Inhibitor | Achondroplasia | | [QED Therapeutics]<br>Preparation underway for Ph Ⅲ in JP |
| ziftomenib ※<br>Oral | Menin Inhibitor | Acute Myeloid Leukemia (AML)<br>(Monotherapy) | | [Kura Oncology]<br>Clinical study is being conducted in NA and EU as a global product<br>NPM1-mutant AML |
| | | Acute Lymphoblastic Leukemia (ALL)<br>(Monotherapy) | | Clinical study is being conducted in NA and EU as a global product<br>KMT2A-rearranged ALL |
| | | Acute Myeloid Leukemia (AML)<br>(Monotherapy) | | Clinical study is being conducted in NA and EU as a global product<br>Non-NPM1-mutant AML/Non-KMT2A-rearranged AML |
| | | Acute Myeloid Leukemia (AML)<br>(Combination) | | Clinical study is being conducted in NA as a global product<br>NPM1-mutant AML/KMT2A-rearranged AML<br>Combinations with venetoclax + azacitidine, and cytarabine + daunorubicin |
| | | | | Clinical study is being conducted in NA and EU as a global product<br>NPM1-mutant AML/KMT2A-rearranged AML<br>Combinations with gilteritinib, FLAG-IDA, LDAC |
| KK2845 | Anti-TIM-3 ADC | Acute Myeloid Leukemia (AML) | ✓ | [In-House]<br>Antibody-Drug Conjugate<br>Clinical study is being conducted in JP as a global product |
| OTL-203 | Hematopoietic Stem Cell (HSC) Gene Therapy | MPS-IH (Hurler Syndrome) | | [In-House]<br>Rare Pediatric Disease (RPD) and Fast Track designations (FDA)<br>Priority Medicines (PRIME) designation (EMA)<br>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]<br>Rare Pediatric Disease (RPD) designation (FDA)<br>Preparation underway for registrational study (equivalent to Ph Ⅲ study) |
| KHK4083/AMG 451<br>rocatinlimab<br>Injection | Anti-OX40 Antibody | Moderate to Severe Atopic Dermatitis | | [In-House]<br>POTELLIGENT<br>Human monoclonal antibody production technology<br>Collaboration agreement with Amgen for the development of rocatinlimab in all the countries except for Japan<br>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 |

※ For detailed information on ziftomenib's development status, please refer to Kura Oncology's website. https://kuraoncology.com/

| Code Name<br>Generic Name<br>Formulation | | Mechanizm of Action | Indication | Stage | | | [In-House or Licensed]<br>Remarks |
|---|---|---|---|---|---|---|---|
| | | | | PhI | PhII | PhIII | |
|  | KHK4951<br>tivozanib<br>Ophthalmic | VEGF Receptor Tyrosine Kinase Inhibitor | Diabetic Macular Edema | | | | [In-House]<br>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<br>Injection | EGFR-TfR1Bispecific Antibody | Advanced or Metastatic Solid Tumors | | | | [In-House]<br>REGULGENT<br>Fully human antibody production technology<br>Clinical study is being conducted in JP, and a clinical study is prepared under way for PhI in NA as a global product |
|  | KK2269<br>Injection | EpCAM-CD40Bispecific Antibody | Advanced or Metastatic Solid Tumors | | | | [In-House]<br>REGULGENT<br>Fully human antibody production technology<br>Clinical study is being conducted in JP and NA as a global product |
|  | KK4277<br>Injection | Anti-PTPRS Humanized Antibody | Systemic Lupus Erythematosus/Cutaneous Lupus Erythematosus | | | | [SBI Biotech]<br>POTELLIGENT<br>Clinical study 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,<br>Product name in Japan and Asia: Lumicef) | 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 |

Since we withdrew an application for partial change of approved indication of KHK4827 for systemic scleosis in Japan, the relevant information was deleted from this table.