R&D Pipeline

The list shows the development status in the target diseases for which we aim to obtain approval in Japan, the United States, Europe and/or China. As of Apr 2023

Underlined items indicate changes from the previous announcement in Feb 2023.

XTANDI and Strategic products (1/2)

Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *		Licensor **	Remarks
enzalutamide MDV3100 (XTANDI)	Small molecule	Androgen receptor inhibitor	Metastatic castration-sensitive prostate cancer	China	P-III	Pfizer	
			Non-metastatic castration-sensitive prostate cancer	P-III			
enfortumab vedotin ASG-22ME (PADCEV)	Antibody-drug conjugate (ADC)	Nectin-4 targeted ADC	Metastatic urothelial cancer, platinum-containing chemotheraphy and PD-1/L1 inhibitor pretreated	China	Filed (Mar 2023)	In-house [Co-development with Seagen]	
			Metastatic urothelial cancer, previously untreated (first line; cisplatin-ineligible, combo with pembrolizumab)	US	Approved (Apr 2023)		
			Metastatic urothelial cancer, previously untreated (first line; combo with pembrolizumab)	P-III			
			Muscle-invasive bladder cancer (combo with pembrolizumab)	P-III			
			Other solid tumors	P-II			
			Non-muscle-invasive bladder cancer	P-I			
gilteritinib ASP2215 (XOSPATA)	Small molecule	FLT3 inhibitor	Post-chemotherapy maintenance acute myeloid leukemia	P-III		In-house	
			Post-hematopoietic stem cell transplant maintenance acute myeloid leukemia	P-III			
			Newly diagnosed acute myeloid leukemia with high intensity induction of chemotherapy	P-III			
			Newly diagnosed acute myeloid leukemia with low intensity induction of chemotherapy	P-I			
			Acute myeloid leukemia in pediatric patients	P-III			

XTANDI and Strategic products (2/2)

Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *	Licensor **	Remarks
zolbetuximab **IMAB362**	Antibody	Anti-Claudin 18.2 monoclonal antibody	Gastric and gastroesophageal junction adenocarcinoma	P-III	In-house (Ganymed)	
			Pancreatic adenocarcinoma	P-II		
fezolinetant **ESN364**	Small molecule	NK3 receptor antagonist	Vasomotor symptoms due to menopause	US Filed (Aug 2022) Europe Filed (Sep 2022) China P-III Japan P-II	In-house (Ogeda)	
resamirigene bilparvovec **AT132**	Gene therapy (AAV-based gene therapy)	MTM1 gene replacement to express myotubularin	X-linked myotubular myopathy	P-II	In-house (Audentes Therapeutics)	

* Compounds are developed globally unless noted. The list shows the most advanced stage if the stages are different depending on the region. The list specifies the area if the compound is developed in limited areas.
** Compounds with "In-house" in this column include ones discovered by collaborative research.

Updates from the previous announcement (Feb 2023):
enfortumab vedotin: Filed in China in Mar 2023 for locally advanced or metastatic urothelial cancer in patients who have received prior treatment with a PD-1/PD-L1 inhibitor and platinum-containing chemotherapy. Obtained accelerated approval in US in Apr 2023 for unresectable locally advanced or metastatic urothelial cancer who are ineligible to receive cisplatin-based chemotherapy in the first-line setting.

Projects with Focus Area approach (1/2)

Primary Focus	Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *	Licensor **	Remarks
Immuno-oncology	**ASP1570**	Small molecule	DGKζ inhibitor	Cancer	P-I	In-house	
	ASP2138	Antibody	Anti-Claudin 18.2 and anti-CD3 bispecific antibody	Gastric and gastroesophageal junction adenocarcinoma, pancreatic adenocarcinoma	P-I	Xencor [Discovered through collaborative research]	
	ASP2074	Antibody	Bispecific antibody	Cancer	P-I	In-house	
	ASP1002	Antibody	Bispecific antibody	Cancer	P-I	In-house	
Blindness and Regeneration	**ASP7317**	Cell therapy	Retinal pigment epithelium cells	Geographic atrophy secondary to age-related macular degeneration, Stargardt disease	P-I	In-house (Ocata Therapeutics)	
Mitochondria	**bocidelpar ASP0367/MA-0211**	Small molecule	PPARδ modulator	Primary mitochondrial myopathies	P-II	In-house (Mitobridge)	
				Duchenne muscular dystrophy	P-I		

Projects with Focus Area approach (2/2)

Primary Focus	Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *	Licensor **	Remarks
Genetic regulation	**resamirigene bilparvovec AT132 ***	Gene therapy (AAV-based gene therapy)	MTM1 gene replacement to express myotubularin	X-linked myotubular myopathy	P-II	In-house (Audentes Therapeutics)	
	AT845	Gene therapy (AAV-based gene therapy)	GAA gene replacement to express GAA enzyme	Pompe disease	P-I	In-house (Audentes Therapeutics)	
Targeted Protein Degradation	**ASP3082**	Small molecule	KRAS G12D degrader	Cancer	P-I	In-house	
(Other projects with Focus Area approach)	**ASP0598**	Recombinant protein	Recombinant human heparin-binding epidermal growth factor-like growth factor	Chronic tympanic membrane perforation	P-I	Auration Biotech	

* Compounds are developed globally unless noted. The list shows the most advanced stage if the stages are different depending on the region. The list specifies the area if the compound is developed in limited areas.
** Compounds with "In-house" in this column include ones discovered by collaborative research.
*** AT132 is also listed in "XTANDI and Strategic products".

Updates from the previous announcement (Feb 2023):
ASP9801: Discontinued the development for cancer in Phase 1.
ASP7517: Discontinued the development for acute myeloid leukemia and myelodysplastic syndrome in Phase 2. Discontinued the development for solid tumor in Phase 1.
ASP0739: Discontinued the development for cancer in Phase 1.
ASP8731/ML-0207: Discontinued the development for sickle cell disease in Phase 1.
FX-322: Discontinued the development for sensorineural hearing loss because Phase 2 study did not meet its primary endpoint.

Others

Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *	Licensor **	Remarks
mirabegron **YM178**	Small molecule	β_3 receptor agonist	Neurogenic detrusor overactivity in pediatric patients	Europe P-III	In-house	
			Overactive bladder in pediatric patients	Europe P-III		
peficitinib **ASP015K**	Small molecule	JAK inhibitor	Rheumatoid arthritis	China Filed (Aug 2022)	In-house	
isavuconazole	Small molecule	Azole antifungal	Invasive aspergillosis and mucormycosis in pediatric patients	US P-II	Basilea	
ASP8062	Small molecule	$GABA_B$ receptor positive allosteric modulator	Alcohol use disorder	P-I	In-house	

* Compounds are developed globally unless noted. The list shows the most advanced stage if the stages are different depending on the region. The list specifies the area if the compound is developed in limited areas.

** Compounds with "In-house" in this column include ones discovered by collaborative research.

Rx+ Program

As of Apr 2023

Underlined items indicate changes from the previous announcement in Feb 2023.

Category	Program	Concept	Status*	Partner	Remarks
Digital health Other services	**Fit-eNce**	Service to provide scientifically evidenced exercise programs and systems supporting regular exercise	Under feasibility study		
	Fit-eNce Home	Service to provide scientifically evidenced exercise programs and systems supporting regular exercise at home	Under feasibility study		
	BlueStar	Digital therapeutics for adults with diabetes	Under clinical trial preparation	Welldoc Roche Diabetes Care Japan	
Drug-device combination	**pudexacianinium chloride ASP5354**	Intraoperative ureter visualization for use in patients undergoing minimally invasive and open abdominopelvic surgeries	P-III	Stryker	
		Visualization and localization of lymph nodes in patients with breast cancer or melanoma undergoing lymphatic mapping	P-II		

* The list shows the most advanced stage if the stages are different depending on the region.

Updates from the previous announcement (Feb 2023):
BlueStar: Added Roche Diabetes Care Japan as a partner.
pudexacianinium chloride (ASP5354): Added Stryker as a partner.