

## 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 Feb 2023

Underlined items indicate changes from the previous announcement in Oct 2022.

### 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, previously untreated (first line; cisplatin-ineligible, combo with pembrolizumab)	US <u>Filed (Dec 2022)</u>	In-house [Co-development with Seagen]	
			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 *	Licensors **	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 associated with menopause	US Filed (Aug 2022) Europe Filed (Sep 2022) China P-III Japan P-II	In-house (Ogeda)	
resmirigene bilparovect 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 (Oct 2022):**

**enfortumab vedotin:** sBLA filed in US in Dec 2022 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
Immunology	ASP9801	Oncolytic virus	Oncolytic virus carrying IL-7 and IL-12	Cancer	P-I	Tottori University [Discovered through collaborative research]	
	ASP7517	Cell therapy (artificial adjuvant vector cells)	WT1 loaded artificial adjuvant vector cell	Acute myeloid leukemia and myelodysplastic syndrome	P-II	RIKEN [Discovered through collaborative research]	
				Solid tumor	P-I		
	ASP0739	Cell therapy (artificial adjuvant vector cells)	NY-ESO-1 loaded artificial adjuvant vector cell	Cancer	P-I	RIKEN [Discovered through collaborative research]	
	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		
	ASP8731/ML-0207	Small molecule	BACH1 inhibitor	Sickle cell disease	P-I	In-house (Mitobridge)	

**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)	FX-322	Small molecule	Inner ear progenitor cell activator (combination of GSK-3 inhibitor and HDAC inhibitor)	Sensorineural hearing loss	P-II	Frequency Therapeutics	Astellas has rights in Ex-US markets
	ASP0598	Recombinant protein	Recombinant human heparin-binding epidermal growth factor-like growth factor	Chronic tympanic membrane perforation	P-I	Auration Biotech	

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\*\*\* AT132 is also listed in "XTANDI and Strategic products".

**Updates from the previous announcement (Oct 2022):**

**ASP1002:** Entered into Phase 1 for cancer.

## Others

Generic name Code No. (Brand name)	Modality / Technology	Classification	Target disease	Phase *	Licensors **	Remarks
mirabegron YM178	Small molecule	$\beta_3$ 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 <sub>B</sub> receptor positive allosteric modulator	Alcohol use disorder	P-I	In-house	

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## Rx+ Program

As of 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	
Drug-device combination	pudexacianinium chloride ASP5354	Intraoperative ureter visualization for use in patients undergoing minimally invasive and open abdominopelvic surgeries	P-III		
		Visualization and localization of lymph nodes in patients with breast cancer or melanoma undergoing lymphatic mapping	P-II		

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