<Pipeline list>

R&D Pipeline (May 2014)

1. Global Development

(1) Approved

Code No. Generic Name	Product Name (Approval Date)	Classification	Target Disease	Area	Dosage Form	Origin*	Remarks
MDV3100 enzalutamide	XTANDI (Mar. 2014)	Androgen receptor inhibitor	Castration-resistant prostate cancer**	Japan	Oral	Medivation	

*"In-house" shown in the "origin" column includes discovery by collaborative research. (The same applicable hereafter.)

**Precautions regarding indication include the description that the efficacy and safety of the drug have not been established in patients with prostate cancer who have not received chemotherapy.

(2) Filed

Stage in the most advanced territory

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
MDV3100 enzalutamide	Androgen receptor inhibitor	Metastatic castration-resistant prostate cancer in patients who have not received chemotherapy	US Filed (Mar. 2014) Europe Filed (Apr. 2014) Japan Phase-III Asia Phase-III	Oral	Medivation	New indication
		Non-Metastatic castration-resistant prostate cancer	US Phase-III Europe Phase-III Asia Phase-III			New indication
		Breast cancer	US/Europe Phase-II			New indication

(3) Phase-lll / Phase-ll (1/2)

Stage in the most advanced territory

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
erlotinib	HER1/EGFR tyrosine kinase inhibitor	Pediatric ependymoma	US Phase-III	Oral	In-house (co-development with Roche/Genentech)	Not a new indication, rather a supplement whose results are planned to submit to the FDA
ASP0113 (VCL-CB01)	DNA vaccine for cytomegalovirus	Cytomegalovirus reactivation in hematopoietic cell transplant recipients	US/Europe/Japan Phase-III	Injection	Vical	
		Cytomegalovirus infection or reactivation in solid organ transplant recipients	US/Europe Phase-II			
YM905 solifenacin	Muscarine M_3 receptor antagonist	Neurogenic detrusor overactivity and idiopathic overactive bladder in pediatric patients	US/Europe Phase-III	Oral	In-house	New indication
EB178 solifenacin/ mirabegron	Concomitant use of solifenacin and mirabegron	Urinary frequency, urinary incontinence or urgency associated with overactive bladder	Europe/US/Asia Phase-III	Oral	In-house	
fidaxomicin	Macrocyclic antibiotic	Infectious enteritis (bacterial target: *Clostridium difficile*)	Japan Phase-III	Oral	Cubist	

(3) Phase-lll / Phase-ll (2/2)

Stage in the most advanced territory

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
ASP1517 (FG-4592) roxadustat	HIF stabilizer	Anemia associated with chronic kidney disease in patients not on dialysis and on dialysis	Europe Phase-III Japan Phase-II	Oral	FibroGen	
YM311 (FG-2216)	HIF stabilizer	Renal anemia	Europe Phase-II Japan Phase-I	Oral	FibroGen	
ASP015K	JAK inhibitor	Rheumatoid arthritis	Japan Phase-II (US/Europe Phase-II*)	Oral	In-house	
ASKP1240	Anti-CD40 monoclonal antibody	Prevention of organ transplant rejection	US Phase-II Japan Phase-I	Injection	Kyowa Hakko Kirin	
ASP3652	Inhibition of afferent nerve activity	Bladder pain syndrome / Interstitial cystitis	Europe Phase-II Japan Phase-I	Oral	In-house	
ASP1707	GnRH antagonist	Endometriosis	Europe/Japan Phase-II	Oral	In-house	
		Prostate cancer	Europe Phase-I			
ASP4901 (AKP-002)	PDE9 inhibitor	Lower urinary tract symptoms associated with benign prostatic hyperplasia	Japan Phase-II	Oral	ASKA	
ASP8477	Inhibition of central sensitization	Neuropathic pain	Europe Phase-II	Oral	In-house	

*A license agreement was executed with Janssen Biotech, Inc. for the development and commercialization worldwide except for Japan.
Phase-IIb studies will be completed by Astellas.

2. Local Development: Japan

(1) Phase-lll / Phase-ll (1/2)

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
YM060 ramosetron	5-HT3 receptor antagonist	Diarrhea-predominant irritable bowel syndrome Female patients	Japan Phase-III	Oral	In-house	New indication
YM533 beraprost sodium	Prostacyclin receptor stimulator	Chronic renal failure (primary, nephrosclerosis)	Japan/Asia Phase-III	Oral	Toray	New indication
FK949E quetiapine	Serotonin/dopamine antagonist	Depressive episode in bipolar disorders	Japan Phase-III	Oral	AstraZeneca	New indication New formulation
certolizumab pegol	PEGylated anti-tumor necrosis factor-alpha antibody	Methotrexate-naive rheumatoid arthritis	Japan Phase-III	Injection	UCB	New indication
ASP1585 (AMG 223) bixalomer	Amine-functional polymer	Hyperphosphatemia in patients not on dialysis with chronic kidney disease	Japan Phase-III	Oral	Amgen	New indication
		Hyperphosphatemia in patients on dialysis with chronic kidney disease (granule formulation)	Japan Bioequivalence study	Oral		New formulation
nateglinide	Fast acting insulin secretion enhancer	Type 2 diabetes Combination with DPP-4 inhibitors	Japan Phase-III	Oral	Ajinomoto	New indication

(1) Phase-lll / Phase-ll (2/2)

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
ASP3550 degarelix	GnRH antagonist	Prostate cancer (three-month formulation)	Japan Phase-III	Injection	Ferring	New formulation
AMG 785 romosozumab	Anti-Sclerostin monoclonal antibody	Osteoporosis	Japan Phase-III	Injection	Amgen (co-development with Amgen Astellas)	
AMG 145 evolocumab	Anti-PCSK-9 monoclonal antibody	Hyperlipidemia	Japan Phase-III	Injection	Amgen (co-development with Amgen Astellas)	
AMG 102 rilotumumab	Anti-HGF monoclonal antibody	Gastric cancer	Japan Phase-III	Injection	Amgen (co-development with Amgen Astellas)	
ASP7374	Influenza vaccine	Prophylaxis of seasonal influenza	Japan Phase-III	Injection	UMN Pharma	
ASP7373	Influenza vaccine	Prophylaxis of H5N1 influenza	Japan Phase-II	Injection	UMN Pharma	
ASP0456 linaclotide	Guanylate cyclase type-C receptor agonist	Irritable bowel syndrome	Japan Phase-II	Oral	Ironwood	
ASP7991	Calcium-sensing receptor activator	Secondary hyperparathyroidism	Japan Phase-II	Oral	In-house	

3. Local Development: Europe

(1) Phase-lll / Phase-ll

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
NGX-4010 capsaicin	TRPV1 agonist	Peripheral diabetic neuropathy	Europe Phase-III	Patch	NeurogesX	New indication

4. Local Development: US

(1) Phase-lll / Phase-ll

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
isavuconazole	Azole antifungal	Invasive aspergillosis	US Phase-III	Injection Oral	Basilea	
		Candidemia / Invasive candidiasis	US Phase-III			

5. Phase-I

Code No. Generic Name	Target Disease	Dosage Form	Origin
AGS-16M8F/ AGS-16C3F	Cancer (ADC technology)	Injection	In-house (ADC technology in-licensed from Seattle Genetics)
ASG-22ME	Cancer (ADC technology)	Injection	In-house (co-development with Seattle Genetics)
ASP9226	Neuropathic pain	Oral	In-house
ASP8232	Diabetic nephropathy	Oral	In-house
ASP3662	Alzheimer's disease	Oral	In-house
ASG-15ME	Cancer (ADC technology)	Injection	In-house (co-development with Seattle Genetics)
ASP2215	Cancer	Oral	In-house
ASP3325	Hyperphosphatemia	Oral	In-house
CK-2127107	Skeletal muscle disease (non-neuromuscular indications)	Oral	Cytokinetics
ASP5878	Cancer	Oral	In-house
AMG 337	Gastric cancer	Oral	Amgen (co-development with Amgen Astellas)
ASP8273	Cancer	Oral	In-house
ASP7962	Osteoarthritis, Chronic low back pain	Oral	In-house
YM178 mirabegron	Neurogenic detrusor overactivity and idiopathic overactive bladder in pediatric patients	Oral	In-house
ASP7657	Diabetic nephropathy	Oral	In-house
ASP3700	Osteoarthritis	Oral	In-house
AGS67E	Cancer (ADC technology)	Injection	In-house (ADC technology in-licensed from Seattle Genetics)

6. Project Discontinued

Code No. Generic Name	Area / Phase	Target Disease	Reason
ASP7487 (OSI-906) linsitinib	US Phase-II	Ovarian cancer	Discontinued the development, as the Phase-II study did not demonstrate the expected efficacy.
ASP0306	Phase-I	Lower urinary tract symptoms associated with benign prostatic hyperplasia	Discontinued for strategic reasons.
ASP6432	Phase-I	Lower urinary tract symptoms associated with benign prostatic hyperplasia	Discontinued for strategic reasons.
ASP9853	Phase-I	Cancer	Discontinued for strategic reasons.

7. Deleted from the Pipeline List

Code No. Generic Name	Area / Phase	Target Disease	Reason
ASP4130 tivozanib	US/Europe Phase-II	Colorectal cancer, Breast cancer	Discontinued the Phase-II clinical trials for colorectal cancer and breast cancer. Excercised the right to terminate the license agreement for strategic reasons, based on the clinical status of the three indications studied (renal cell carcinoma, colorectal cancer, breast cancer). The termination will be effective August 11, 2014, at which time tivozanib right will be returned to AVEO.

<Changes from the Previous Announcement on February 3, 2014> (Changed underlined items)

Launched

-Midazolam: Removed the previous item below.

-Ipragliflozin: Removed the previous item below. It was launched in Japan in April 2014.

Code No. Generic Name	Product Name (Approval Date)	Classification	Target Disease	Area	Dosage Form	Origin	Remarks
midazolam	Dormicum (Dec. 2013)	Benzodiazepine sedative	Sedation during surgery and procedures for dental, oral and maxillofacial care	Japan	Injection	Roche	New indication
ASP1941 ipragliflozin	Suglat (Jan. 2014)	SGLT2 inhibitor	Type 2 diabetes	Japan	Oral	In-house (co-development with Kotobuki)	

Approved

-Enzalutamide: Approved in Japan in March 2014.

Code No. Generic Name	Product Name (Approval Date)	Classification	Target Disease	Area	Dosage Form	Origin	Remarks
MDV3100 enzalutamide	XTANDI (Mar. 2014)	Androgen receptor inhibitor	Castration-resistant prostate cancer*	Japan	Oral	Medivation	

*Precautions regarding indication include the description that the efficacy and safety of the drug have not been established in patients with prostate cancer who have not received chemotherapy.

Filed

-Enzalutamide: Filed in US/Europe for metatstatic castration-resistant prostate cancer in patients who have not received chemotherapy.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
MDV3100 enzalutamide	Androgen receptor inhibitor	Metastatic castration-resistant prostate cancer in patients who have not received chemotherapy	US Filed (Mar. 2014) Europe Filed (Apr. 2014) Japan Phase-III Asia Phase-III	Oral	Medivation	New indication

Phase-III / Phase-II

-Erlotinib: Deleted non-small cell lung cancer (combination with MetMAb) from the target disease because Roche, who conducted the studies for it, terminated the studies.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
erlotinib	HER1/EGFR tyrosine kinase inhibitor	~~Non-small cell lung cancer (combination with MetMAb),~~ Pediatric ependymoma	US Phase-III	Oral	In-house (co-development with Roche/Genentech)	~~New indication~~ Not a new indication, rather a supplement whose results are planned to submit to the FDA

-Isavuconazole: Updated the development area and moved to the part of "Local Development: US" in accordance with the amended license agreement.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
isavuconazole	Azole antifungal	Invasive aspergillosis	US~~/Europe~~ Phase-III	Injection Oral	Basilea	
		Candidemia / Invasive candidiasis	US~~/Europe~~ Phase-III			

-ASP0113: Entered into Phase-III in the following target in Japan.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
ASP0113 (VCL-CB01)	DNA vaccine for cytomegalovirus	Cytomegalovirus reactivation in hematopoietic cell transplant recipients	US/Europe/Japan Phase-III	Injection	Vical	

-Beraprost sodium: Changed the description of remarks.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
YM533 beraprost sodium	Prostacyclin receptor stimulator	Chronic renal failure (primary, nephrosclerosis)	Japan/Asia Phase-III	Oral	Toray	New indication ~~New formulation~~

-Bixalomer: Added the information on development of new formulation.

Code No. Generic Name	Classification	Target Disease	Area / Phase	Dosage Form	Origin	Remarks
ASP1585 (AMG 223) bixalomer	Amine-functional polymer	Hyperphosphatemia in patients on dialysis with chronic kidney disease (granule formulation)	Japan Bioequivalence study	Oral	Amgen	New formulation

Phase-I

-Mirabegron [Neurogenic detrusor overactivity and idiopathic overactive bladder in pediatric patients]: Entered into Phase-I.

-ASP7657 [Diabetic nephropathy]: Entered into Phase-I.

-ASP3700 [Osteoarthritis]: Entered into Phase-I.

-AGS67E [Cancer (ADC technology)]: Entered into Phase-I.

Project Discontinued

-Linsitinib [Ovarian cancer] in Phase-II: Discontinued the development.

-ASP0306 [Lower urinary tract symptoms associated with benign prostatic hyperplasia] in Phase-I: Discontinued the development.

-ASP6432 [Lower urinary tract symptoms associated with benign prostatic hyperplasia] in Phase-I: Discontinued the development.

-ASP9853 [Cancer] in Phase-I: Discontinued the development.

Deleted from the Pipeline List

-Tivozanib [Colorectal cancer, Breast cancer] in Phase-II: Deleted from the list because we excercised the right to terminate the license agreement for strategic reasons, based on the clinical status of the three indications studied. The termination will be effective August 11, 2014, at which time tivoazanib right will be returned to AVEO.