Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
5	1993	1993/11/15	(5yaku A) No. 1	2	Mixture of L-arginine and L-arginine hydrochloride granules; L-arginine hydrochloride injection	The granule form treats neurological symptoms due to hyperammonemia, such as vomiting, lethargy, and abnormal electroencephalogram findings, and symptoms due to argenine deficiency, such as growth retardation, which occur in the following diseases: congenital urea cycle enzyme abnormalities (carbomyl phosphate synthetase deficiency, ornithine transcarbamylase deficiency, argininosuccinate synthetase deficiency [citrullinemia], and argininosuccinate lyase deficiency [argininosuccinic aciduria]), and congenital amino acid transfer abnormalities The injection form is for emergency use to lower blood levels of ammonia after it has risen steeply due to wasting syndromes, etc. that have not been controlled with the granule form.	Roussel Morishita Company, Limited	<Argi-U Granule> Inhibition of rising blood levels of ammonia in the following diseases: congenital urea cycle enzyme abnormalities (carbomyl phosphate synthetase deficiency, ornithine transcarbamylase deficiency, argininosuccinate synthetase deficiency [citrullinemia] and argininosuccinate lyase deficiency [argininosuccinic aciduria], or lysinuric protein intolerance, except in patients with strong inhibition of arginine absorption. <Argi-U Injection 20 g> Emergency use to lower blood levels of ammonia in patients with acute exacerbation of hyperammonemia, uncontrollable through oral intake of the granule form in the following diseases: congenital urea cycle enzyme abnormalities (carbomyl phosphate synthetase deficiency, ornithine transcarbamylase deficiency, argininosuccinate synthetase deficiency [citrullinemia] and argininosuccinate lyase deficiency [argininosuccinic aciduria], or lysinuric protein intolerance	Argi-U Granule:Ajinomoto Co. Inc. Argi-U Injection 20g:AY PHARMACEUTICALS CO.,LTD	1999/9/22	Argi-U Granule Argi-U Injection 20g	Argi-U® Granule Argi-U® Injection	Argi-U Granule L-Arginine Hydrochloride L-Arginine Argi-U Injection 20g L-Arginine Hydrochloride			Approved	
5	1993	1993/11/15	(5yaku B) No. 2		Alglucerase	Improvement of the following symptoms in patients with Gaucher's disease type I: anemia, thrombocytopenia, and hepatosplenomegaly.	Genzyme Japan K.K.	Improvement of the following symptoms in patients with Gaucher's disease type I: anemia, thrombocytopenia, hepatosplenomegaly and other symptoms in bones.	Genzyme Japan K.K.	1996/7/10	Ceredase injection 50U Ceredase injection 400U	-	Alglucerase	These two formulations are currently not being supplied. Designation number “8yaku No. 81” is being supplied instead.		Approved	
5	1993	1993/11/15	(5yaku A) No. 3	2	Alprostadil Alfadex	Primary hypertension or pulmonary hypertension after open heart surgery; pulmonary hypertension as a complication of collagen diseases	Ono Pharmaceutical Co., Ltd.	-	-	-	-	-	Alprostadil Alfadex	Designation revoked (2001/08/24)	2001/8/24	Revoked	
5	1993	1993/11/15	(5yaku B) No. 4		Albendazole	Echinococcosis	SmithKline Beecham	Echinococcosis	GlaxoSmithKline K.K.	1994/1/19	Eskazole tablet 200mg	Eskazole® Tablets 200mg	Albendazole			Approved	
5	1993	1993/11/15	(5yaku A) No. 5	1	Interferon alpha	HAM (HTLV-I associated myelopathy)	Sumitomo Pharmaceuticals	HTLV-I associated myelopathy (HAM)	Dainippon Sumitomo Pharma Co., Ltd.	2000/1/18	Sumiferon injection vial 3,000,000 IU Sumiferon injection DS 3,000,000 IU	Sumiferon®	Interferon Alfa (NAMALWA)			Approved	
5	1993	1993/11/15	(5yaku B) No. 6		Interferon gamma-1a (recombinant)	Reduction of the frequency and severity of serious infections associated with chronic granulomatous disease	Shionogi & Co., Ltd.	Reduction of the frequency and severity of serious infections associated with chronic granulomatous disease	Shionogi & Co., Ltd.	1998/6/30	Imunomax-γ for injection 50 Imunomax-γ for injection 100	Imunomax®-γ	Interferon Gamma-1a (recombinant)			Approved	
5	1993	1993/11/15	(5yaku B) No. 7		Indomethacin sodium	For use when maintenance therapy including restriction of water intake and diuretics are ineffective for premature infants with patent ductus arteriosus (PDA)	Banyu Pharmaceutical Co., Ltd.	For use when maintenance therapy including restriction of water intake and diuretics are ineffective for premature infants with patent ductus arteriosus (PDA)	Nobelpharma Co., Ltd.	1994/10/5	INDACIN IV 1mg	INDACIN® IV 1mg	Indomethacin sodium			Approved	
5	1993	1993/11/15	(5yaku B) No. 8		Rabbit-derived anti-human thymocyte immunoglobulin	Aplastic anemia	Rhône-Poulenc Japan	Aplastic anemia, moderate or severe cases	Sanofi K.K.	2008/7/16	Thymoglobulin for IV infusion 25mg	Thymoglobulin®	Anti-human Thymocyte Immunoglobulin, Rabbit			Approved	
5	1993	1993/11/15	(5yaku B) No. 9		Rabbit-derived anti-human T-lymphocyte immunoglobulin	Aplastic anemia	Nippon Zoki Pharmaceutical Co., Ltd	Severe to moderate aplastic anemia	Nippon Zoki Pharmaceutical Co., Ltd	1995/9/29	Zetbulin for IV infusion 100mg	Zetbulin® IV drip 100mg	Anti-human T-Lymphocyte Immunoglobulin, Rabbit	Designation revoked (2017/03/01)	2017/3/1	Approved	
5	1993	1993/11/15	(5yaku A) No. 10	3	Horse-derived anti-human thymocyte immunoglobulin	Aplastic anemia	Upjohn Pharmaceuticals, Ltd.	-	-	-	-	-	-	Designation revoked (1998/05/28)	1998/5/28	Revoked	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
5	1993	1993/11/15	(5yaku B) No. 11		Horse-derived anti-human thymocyte immunoglobulin	Aplastic anemia	Rhône-Poulenc Japan	Severe to moderate aplastic anemia	Genzyme Japan K.K.	1995/9/29	Lymphoglobulin injection 100mg	-	Anti-human thymocyte immunoglobulin, equine	This formulation is currently not being supplied. Designation number “(5yaku)No. 8” is being supplied		Approved	
5	1993	1993/11/15	(5yaku A) No. 12	2	Botulinum toxin type A	Local dystonia (blepharospasm and torticollis) and facial myokymia	Allergan Japan	Blepharospasm, hemifacial spasm, spasmodic torticollis	GlaxoSmithKline K.K.	1996/10/09 Blepharospasm 2000/01/18 Hemifacial spasm 2001/06/20 Spasmodic torticollis	Botox for injection 50 units Botox for injection 100 units	BOTOX® for injection	Botulinum Toxin Type A			Approved	
5	1993	1993/11/15	(5yaku B) No. 13		Trientine hydrochloride	D-penicillamine intolerance in Wilson’s disease	Tsumura & Co.	Wilson's disease with D-penicillamine intolerance	Tsumura & Co.	1994/7/1	Metalite 250 capsule	METALITE® 250 CAPSULES	trientine hydrochloride			Approved	
5	1993	1993/11/15	(5yaku A) No. 14	3	Halofantrine hydrochloride	Malaria	SmithKline Beecham Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (1999/05/27)	1999/5/27	Revoked	
5	1993	1993/11/15	(5yaku B) No. 15		Vancomycin hydrochloride	Methicillin-cephem-resistant Staphylococcus aureus enteritis	Shionogi & Co., Ltd.	Methicillin-cephem-resistant Staphylococcus aureus enteritis	Shionogi & Co., Ltd.	1994/10/5	Vancomycin hydrochloide powder 0.5g	Vancomycin	Vancomycin Hydrochloride			Approved	
5 15 30	1993 2003 2018	1993/11/15 2003/11/05 2018/07/02*1	(5yaku A) No. 16, (15yaku) No. 16 *1	5	Freeze-dried concentrated human activated protein C	Improvement of the following diseases caused by congenital protein C deficiency: superficial venous thrombosis, deep venous thrombosis, pulmonary thromboembolism, and purpura fulminans.	Kaketsuken*1, Teijin Ltd., 1993-11-15, Teijin Pharma Limited, 2003-11-05 KM Biologics Co., Ltd.*1	The following diseases caused by congenital protein C deficiency: 1. Deep venous thrombosis, acute pulmonary thromboembolism 2. Purpura fulminans	Kaketsuken KM Biologics Co., Ltd. *1	2000/09/22 Deep venous thrombosis, acute pulmonary thromboembolism 2006/10/20 Purpura fulminans	Anact C for injection 2,500 units	Anact® C	Freeze-dried Human Activated Protein C Concentrate	Teijin Ltd. only, designation revoked (2003/11/05) Designation revoked (2018/07/02) *1		Approved	
5	1993	1993/11/15	(5yaku B) No. 17		Freeze-dried BCG vaccine	Superficial bladder cancer and carcinoma in situ of the bladder	Japan BCG Laboratory	Superficial bladder cancer and carcinoma in situ of the bladder	Japan BCG Laboratory	1996/7/10	Immunobladder intravesical 40mg Immunobladder intravesical 80mg	Immunobladder® intravesical 40mg Immunobladder® intravesical 80mg	Freeze-dried BCG for Intravesical Use (Japanese strain)			Approved	
5	1993	1993/11/15	(5yaku A) No. 18	2	Concentrated plasma-derived blood coagulation factor XIII	Suppression of progression of neonatal intracranial hemorrhage	Hoechst Japan, Ltd.	-	-	-	-	-	-	Designation revoked (2000/05/10)	2000/5/10	Revoked	
5	1993	1993/11/15	(5yaku B) No. 19		Corticorelin (human)	Test for function of hypothalamus, pituitary gland and adrenal cortex.	Mitsubishi Kasei Corporation	Test of the ability of the hypothalamus, pituitary gland and adrenal cortex to secrete hormone.	Mitsubishi Tanabe Pharma Corporation	1994/10/5	hCRH Tanabe for IV injection 100 µg	hCRH “TANABE” Injection 100µg	Corticorelin (human)			Approved	
5	1993	1993/11/15	(5yaku A) No. 20	2	Zalcitabine	AIDS and HIV infection	Nippon Roche Ltd.	AIDS, symptomatic HIV, and asymptomatic HIV infection with ≤500 CD4 lymphocytes/ mm3 before treatment	Chugai Pharmaceutical Co., Ltd	1996/4/24	Hibid tablet 0.375mg	-	Zalcitabine	This formulation is currently not supplied.		Approved	
5	1993	1993/11/15	(5yaku B) No. 21		Cyclosporine	Aplastic anemia and pure red-cell aplasia	Sandoz Co., Ltd.	Severe cases of aplastic anemia or pure red-cell aplasia	Novartis Pharma K.K.	1995/9/29	Sandimmun oral solution 10% Sandimmun capsule 25 mg Sandimmun capsule 50 mg	Sandimmun® Oral Solution 10% Sandimmun® Capsules	Cyclosporine			Approved	
5	1993	1993/11/15	(5yaku B) No. 22		Cyclosporine	Frequently recurring or steroid-resistant nephrotic syndrome	Sandoz Co., Ltd	Frequently recurring or steroid-resistant nephrotic syndrome	Novartis Pharma K.K.	1996/1/31	Sandimmun Oral Solution 10% Sandimmun capsule 25 mg Sandimmun capsule 50 mg	Sandimmun® Oral Solution 10% Sandimmun® Capsules	Cyclosporine			Approved	
5	1993	1993/11/15	(5yaku A) No. 23	1	Purified pituitary gonadotropin	Male hypogonadotropic hypogonadism	Serono Japan Co., Ltd.	-	-	-	-	-	-	Designation revoked (2000/09/20)	2000/9/20	Revoked	
5	1993	1993/11/15	(5yaku A) No. 24	3	Sotalol	Life-threatening ventricular tachyarrhythmia (ventricular tachycardia and ventricular fibrillation)	Bristol-Myers Squibb	Life-threatening recurrent arrhythmias (ventricular tachycardia or ventricular fibrillation) where no other antiarrhythmic drugs are effective or usable.	Bristol-Myers	1998/9/30	Sotacor tablet 40 mg Sotacor tablet 80 mg	SOTACOR® TABLETS 40mg SOTACOR® TABLETS 80mg	sotalol hydrochloride			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
5	1993	1993/11/15	(5yaku B) No. 25		Tacrolimus	Treatment of graft-versus-host disease (GVHD) after bone marrow transplantation	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Treatment of graft-versus-host disease (GVHD) after bone marrow transplantation	Astellas Pharma Inc.	1994/7/1	Prograf capsule 0.5 mg Prograf capsule 1 mg Prograf capsule 5 mg Prograf granule 0.2 mg Prograf granule 1 mg Prograf injection 2 mg Prograf injection 5 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg Prograf® Capsules 5mg Prograf® Granules 0.2mg Prograf® Granules 1mg Prograf® Injection 2mg Prograf® Injection 5mg	Tacrolimus Hydrate			Approved	
5	1993	1993/11/15	(5yaku B) No. 26		Tacrolimus	Inhibition of rejection after renal transplantation	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Inhibition of rejection after renal transplantation	Astellas Pharma Inc.	1996/4/16	Prograf capsule 0.5 mg Prograf capsule 1 mg Prograf capsule 5 mg Prograf granule 0.2 mg Prograf granule 1 mg Prograf injection 2 mg Prograf injection 5 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg Prograf® Capsules 5mg Prograf® Granules 0.2mg Prograf® Granules 1mg Prograf® Injection 2mg Prograf® Injection 5mg	Tacrolimus Hydrate			Approved	
5	1993	1993/11/15	(5yaku A) No. 27	3	Tacrolimus	Refractory uveitis mainly associated with Behcet's disease	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	-	-	-	-	-	Tacrolimus Hydrate	Designation revoked (2000/09/20)	2000/9/20	Revoked	
5	1993	1993/11/15	(5yaku B) No. 28		Dantrolene sodium	Malignant syndrome	Yamanouchi Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Malignant syndrome	OrphanPacific, Inc.	1994/7/1	Dantrium capsule 25 mg Dantrium capsule 50 mg Dantrium 20 mg for IV injection	Dantrium® Capsules 25mg Dantrium® Capsules 50mg Dantrium® 20mg for Intravenous Injection	Dantrolene Sodium Hydrate			Approved	
5	1993	1993/11/15	(5yaku A) No. 29	1	Tretinoin	Acute promyelocytic leukemia	Nippon Roche Ltd.	Acute promyelocytic leukemia	Chugai Pharmaceutical Co., Ltd	1995/1/20	Vesanoid capsule 10 mg	VESANOID® Capsule 10mg	Tretinoin			Approved	
5	1993	1993/11/15	(5yaku A) No. 30	3	Piracetam	Progressive myoclonus epilepsy including dyslipidemia, Unverricht-Lundborg syndrome, Ramsay-Hunt syndrome, Lafora's disease, mitochondrial encephalomyopathy, and neuronal ceroid lipofuscinosis, myoclonus after cerebral anoxia (Lance-Adams syndrome), essential myoclonus, myoclonus caused by Huntington's disease or Alzheimer's disease, drug-induced myoclonus, and other idiopathic myoclonus	Taiho Fine Chemical Taiho Pharmaceutical Co., Ltd. UCB Japan Co. Ltd.	Combination therapy with antiepileptics and other drugs for cortical myoclonus	Taiho Pharmaceutical Co., Ltd. UCB Japan Co. Ltd.	1999/9/22	Myocalm oral solution 33.3%	Myocalm® solution 33.3%	Piracetam			Approved	
5 30	1993 2018	1993/11/15 2018/7/2*62	(5yaku B) No. 31		Pentostatin	Amelioration of subjective and objective symptoms in the following diseases: adult T-cell leukemia, lymphoma and hairy cell leukemia.	Kaketsuken*63 Yamasa Corporation KM Biologics Co., Ltd.*63	Remission of subjective and objective symptoms in the following diseases: adult T-cell leukemia, lymphoma and hairy cell leukemia.	Kaketsuken KM Biologics Co., Ltd. *62	1994/4/1	Coforin for IV injection 7.5 mg	Coforin	Pentostatin	Designation revoked (2018/07/02)*62		Approved	
5	1993	1993/11/15	(5yaku B) No. 32		Mecasermin (recombinant)	Insulin receptor disease (type A and B insulin receptor disease, lipoatrophic diabetes mellitus, leprechaunism and Rabson-Mendenhall syndrome	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Improvement of hyperglycemia, hyperinsulinemia, acanthosis nigricans or hirsutism in patients with the following diseases: Type A and B insulin receptor disease, lipoatrophic diabetes mellitus, leprechaunism and Rabson-Mendenhall syndrome	OrphanPacific, Inc.	1994/10/5	Somazon 10mg for injection	Somazon® 10mg for Injection	Mecasermin (recombinant)			Approved	
5	1993	1993/11/15	(5yaku B) No. 33		Mecasermin (recombinant)	Growth hormone resistant dwarfism (isolated growth hormone deficiency Type 1A and Laron dwarfism	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Improvement of growth disorders in the following diseases: growth hormone resistant isolated growth hormone deficiency Type 1A or Laron dwarfism	OrphanPacific, Inc.	1994/10/5	Somazon 10mg for Injection	Somazon® 10mg for Injection	Mecasermin (recombinant)			Approved	
5	1993	1993/11/15	(5yaku A) No. 34	2	Mesalazine	Ulcerative colitis	Nisshin Flour Milling Inc.	Ulcerative colitis except severe cases	Kyorin Pharmaceutical Co., Ltd.	1996/4/16	Pentasa tablet 250 mg Pentasa tablet 500 mg	PENTASA® Tablets 250mg PENTASA® Tablets 500mg	Mesalazine			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
5	1993	1993/11/15	(5yaku A) No. 35	2	Mesalazine	Crohn's disease	Nisshin Flour Milling Inc.	Crohn's disease	Kyorin Pharmaceutical Co., Ltd.	1996/4/16	Pentasa tablet 250 mg Pentasa tablet 500 mg	PENTASA® Tablets 250mg PENTASA® Tablets 500mg	Mesalazine	granted at the same time as (5yaku A) No. 34		Approved	
5	1993	1993/11/15	(5yaku A) No. 36	4	L-1-methyl-4,5-dihydroorotyl-L-histidyl-L-prolineamide	Spinocerebellar ataxia	Tanabe Seiyaku Co., Ltd.	Improvement of ataxia in patients with spinocerebellar ataxia	Mitsubishi Tanabe Pharma Corporation	2000/7/3	Ceredist tablet 5 mg	CEREDIST® Tablets 5mg	taltirelin hydrate			Approved	
5	1993	1993/11/15	(5yaku A) No. 37	2	Melphalan	Multiple myeloma, bone marrow transplantation pretreatment, retinoblastoma	Nihon Wellcome	Pretreatment for hematopoietic stem cell transplantation in the following diseases: leukemia, malignant lymphoma, multiple myeloma, childhood solid tumors	GlaxoSmithKline K.K.	2001/4/4	Alkeran for IV injection 50mg	Alkeran® for injection	Melphalan			Approved	
5	1993	1993/11/15	(5yaku B) No. 38		*Streptococcus pyogenes* heated with benzylpenicillin potassium and lyophilized	Lymphangioma	Chugai Pharmaceutical Co., Ltd.	Lymphangioma	Chugai Pharmaceutical Co., Ltd.	1995/1/20	Picibanil for injection 0.2 KE Picibanil for injection 0.5 KE Picibanil for injection 1 KE Picibanil for injection 5 KE	PICIBANIL® Injection 0.2KE PICIBANIL® Injection 0.5KE PICIBANIL® Injection 1KE PICIBANIL® Injection 5KE	Lyophilized powder of *Streptococcus pyogenes* (A group, type 3) Su strain cells treated with penicillin			Approved	
5	1993	1993/11/15	(5yaku A) No. 39	4	Riluzole	Amyotrophic lateral sclerosis (ALS)	Rhône-Poulenc Rorer, Inc.	Treatment of amyotropic lateral sclerosis (ALS), inhibition of progression of amyotropic lateral sclerosis (ALS)	Sanofi K.K.	1998/12/25	Rilutek tablet 50 mg	RILUTEK®50mg Tablets	Riluzole			Approved	
5	1993	1993/11/15	(5yaku B) No. 40		Regavirumab	Cytomegalovirus infection in immunocompromised patients with the following conditions: malignant tumors, AIDS, aplastic anemia, organ transplant and newborns	Teijin, Ltd.	-	-	-	-	-	Regavirumab	Designation revoked (1999/05/27)	1999/5/27	Revoked	
6	1994	1994/7/1	(6yaku A) No. 41	3	Ethyl icosapentate	Behcet's disease	Mochida Pharmaceutical Co., Ltd.	-	-	-	-	-	Ethyl icosapentate	Designation revoked (2001/08/24)	2001/8/24	Revoked	
6	1994	1994/7/1	(6yaku A) No. 42	1	Combined formulation of L-isoleucine, L-valine and L-leucine	To maintain muscle strength in amyotrophic lateral sclerosis (ALS) patients with movement disorders in two or fewer body parts	Ajinomoto Co. Inc.	-	-	-	-	-	L-Isoleucine L-valine L-Leucine	Designation revoked (1996/04/01)	1996/4/1	Revoked	
6	1994	1994/7/1	(6yaku A) No. 43	2	Indium 111 (111In)-pentetreotide	Scintigraphic diagnosis of hormone-producing gastrointestinal tract tumors	Marinclott Medical (currently Marinclott Japan)	-	-	-	-	-	^{111}Indium-pentetreotide	Designation revoked (2015/12/18)	2015/12/18	Revoked	
6	1994	1994/7/1	(6yaku A) No. 44	3	Interferon-alpha	Extension of survival in patients with subacute sclerosing panencephalitis, used in combination with inosine pranobex	Sumitomo Pharmaceuticals	Inhibition of progression of clincal symptoms in patients with subacute sclerosing panencephalitis, used in combination with inosine pranobex	Dainippon Sumitomo Pharma Co., Ltd.	1999/3/12	Sumiferon injection vial 3,000,000 IU	Sumiferon®	Interferon Alfa (NAMALWA)			Approved	
6	1994	1994/7/1	(6yaku A) No. 45		Interferon alpha	Extension of survival in patients with subacute sclerosing panencephalitis, used in combination with inosine pranobex	Mochida Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (2001/08/24)	2001/8/24	Revoked	
6	1994	1994/7/1	(6yaku A) No. 46	3	Interferon-beta	Extension of survival in patients with subacute sclerosing panencephalitis, used in combination with inosine pranobex	Mochida Pharmaceutical Co., Ltd.	Inhibition of progression of clincal symptoms in patients with subacute sclerosing panencephalitis, used in combination with inosine pranobex	Mochida Pharmaceutical Co., Ltd.	1999/3/12	IFN β MOCHIDA injection 1,000,000 IU IFN β MOCHIDA injection 3,000,000 IU IFN β MOCHIDA injection 5,000,000 IU	-	Interferon Beta	Designation revoked (2009/05/11) This formulation is currently not supplied.	2009/5/11	Approved	
6	1994	1994/7/1	(6yaku A) No. 47	4	Interferon-beta 1b (recombinant)	Multiple sclerosis	Nihon Schering K.K.	Inhibition of progression or prevention of recurrence of multiple sclerosis	Bayer Holding Ltd.	2000/9/22	Betaferon SC injection 9,600,000 IU	Betaferon®sc inj. 960	Interferon Beta-1b (recombinant)			Approved	
6	1994	1994/7/1	(6yaku A) No. 48	4	Rabbit-derived anti-human thymocyte immunoglobulin	Graft-versus-host disease (GVHD) in bone marrow transplantation	Rhône-Poulenc Japan	Acute graft-versus-host disease (GVHD) after hematopoietic stem cell transplantation	Sanofi K.K.	2008/7/16	Thymoglobulin for infusion 25 mg	Thymoglobulin®	Anti-human Thymocyte Immunoglobulin, Rabbit			Approved	
6	1994	1994/7/1	(6yaku A) No. 49	4	Ursodeoxycholic acid	Primary biliary cirrhosis	Tokyo Tanabe Pharmaceutical Co. Ltd.	Improvement of liver function in patients with primary biliary cirrhosis	Mitsubishi Tanabe Pharma Corporation	1999/6/16	Urso tablet 50 mg Urso tablet 100 mg	URSO® tablets 50mg URSO® tablets 100mg	Ursodeoxycholic Acid			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
6	1994	1994/7/1	(6yaku A) No. 50	4	Epoprostenol sodium	Primary pulmonary hypertension	Nihon Wellcome	Primary pulmonary hypertension	GlaxoSmithKline K.K.	1999/1/25	Flolan for IV injection 0.5 mg Flolan for IV injection 1.5 mg	Flolan® for injection 0.5mg Flolan® for injection 1.5mg	Epoprostenol Sodium			Approved	
6	1994	1994/7/1	(6yaku A) No. 51	3	Mefloquine hydrochloride	Treatment of malaria	SSP Co., Ltd. Dojin Iyaku-Kako Co., Ltd	Malaria	Hisamitsu Pharmaceutical Co., Inc.	2001/4/4	Mephaquin HISAMITSU tablet 275 mg	MEPHAQUIN HISAMITSU TABLETS 275	Mefloquine hydrochloride			Approved	
6	1994	1994/7/1	(6yaku A) No. 52	3	Mefloquine hydrochloride	Treatment of malaria	Nippon Roche Ltd.	-	-	-	-	-	Mefloquine hydrochloride	Designation revoked (2003/01/31)	2003/1/31	Revoked	
6	1994	1994/7/1	(6yaku A) No. 53	1	Octafluoropropane	Support of the repair of idiopathic macular hole	Santen Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (1996/09/25)	1996/9/25	Revoked	
6	1994	1994/7/1	(6yaku A) No. 54	1	Activated human blood coagulation factor VII (recombinant)	Inhibition of hemorrhage in patients who have inhibitors and a deficiency of blood coagulation factor VIII (hemophilia A) or blood coagulation factor IX (hemophilia B)	Novo Nordisk Pharma Ltd.	Inhibition of hemorrhage in patients with congenital or acquired hemophilia who have inhibitors to blood coagulation factor VIII or IX.	Novo Nordisk Pharma Ltd.	2000/3/10	NovoSeven for injection 1.2 mg NovoSeven for injection 4.8 mg NovoSeven HI for IV injection 1 mg NovoSeven HI for IV injection 2 mg NovoSeven HI for IV injection 5	NovoSeven® HI	eptacog alfa (activated)(recombinant)	NovoSeven for injection 1.2 mg and 4.8 mg:Approval Cancellation		Approved	
6	1994	1994/7/1	(6yaku A) No. 55	2	Freeze-dried human activated blood coagulation factor VII	Inhibition of hemorrhage in patients who have inhibitors and a deficiency of blood coagulation factor VIII (hemophilia A) or coagulation factor IX (hemophilia B)	Kaketsuken	-	-	-	-	-	-	Designation revoked (2004/04/21)	2004/4/21	Revoked	
12	2000	1994/07/01 2000/12/20 *2	(12yaku) No. 56	3	Dried BCG Vaccine	Superficial bladder cancer and carcinoma in situ of the bladder	Rhône-Poulenc Japan, 1994-7-1, Nippon Kayaku Co., Ltd., 2000-12-20 *2	Superficial bladder cancer and carcinoma in situ of the bladder	Sanofi K.K.	2002/10/8	Immucyst intravesical injection 81 mg	IMMUCYST®	Freeze-dried BCG for Intravesical Use (Connaught strain)	Designation revoked (2000/12/20) *2 Approval Cancellation		Approved	
6	1994	1994/7/1	(6yaku A) No. 57	2,3 *3	Dried polyethylene glycol-treated human immunoglobulin	Chronic inflammatory demyelinating polyradiculoneuropathy	Nihon Pharmaceutical Co., Ltd.	･Improvement of muscle weakness in patients with chronic inflammatory demyelinating polyradiculoneuropathy including multifocal motor neuropathy ･Suppression of motor dysfunction with chronic inflammatory demyelinating polyradiculoneuropathy including multifocal motor neuropathy (only if muscle weakness can be improved) *3	Nihon Pharmaceutical Co., Ltd.	1999/6/16 2016/12/19 *3	Kenketu glovenin-I for IV injection 500 mg Kenketu glovenin-I for IV injection 2500 mg Kenketu glovenin-I for IV injection 5000 mg	kenketu glovenin® -I for IV injection 500mg kenketu glovenin® -I for IV injection 2500mg kenketu glovenin® -I for IV injection 5000mg	Freeze-dried Polyethylene Glycol-treated Normal Human Immunoglobulin			Approved	
6	1994	1994/7/1	(6yaku A) No. 58	4	Clarithromycin	Disseminated mycobacterial infection in patients with AIDS	Taisho Pharmaceutical Co., Ltd. Dainabot Co., Ltd.	polyradiculoneuropathy including multifocal motor neuropathy (only if	Taisho Pharmaceutical Co., Ltd. Abbott Japan Co., Ltd.	1998/9/30	Clarith tablet 200 mg Clarith tablet 50 mg for pediatric use Clarith dry syrup 10% for pediatric use Klaricid tablet 200 mg Klaricid tablet 50 mg for pediatric use Klaricid dry syrup 10% for pediatric use	Clarith® tab. 200 Clarith® tab. 50 for pediatric Clarith® dry syrup 10% for pediatric KLARICID TABLETS 200 mg KLARICID SYRUP FOR PEDIATRIC USE KLARICID TABLETS 50 mg FOR PEDIATRIC USE	clarithromycin			Approved	
6	1994	1994/7/1	(6yaku A) No. 59	1	Anti-CD45 monoclonal antibody	Immunosuppression of acute rejection in renal transplantation	Baxter	muscle weakness can be improved)	-	-	-	-	-	Designation revoked (1996/04/01)	1996/4/1	Revoked	
6	1994	1994/7/1	(6yaku A) No. 60	1	Somatropin (recombinant)	Short stature without epiphyseal line closure in chronic renal failure or chondrodystrophy	Novo Nordisk Pharma Ltd.	Short stature without epiphyseal line closure in chondrodystrophy	Novo Nordisk Pharma Ltd.	1997/4/22	Norditropin S injection 10mg Norditropin FlexPro injection 5 mg Norditropin FlexPro injection 10 mg Norditropin FlexPro injection 15 mg	Norditropin® FlexPro® Norditropin® S	somatropin (recombinant)			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
6	1994	1994/7/1	(6yaku A) No. 61	1	Somatropin (recombinant)	Short stature in chronic renal failure without epiphyseal line closure	Sumitomo Pharmaceuticals Co., Ltd.	Short stature in chronic renal failure without epiphyseal line closure	Pfizer Japan Inc.	1997/7/2	Genotropin TC injection 5.3 mg Genotropin TC injection 12 mg Genotropin MiniQuick s.c. injection 0.6 mg Genotropin MiniQuick s.c. injection 1.0 mg Genotropin MiniQuick s.c. injection 1.4 mg Genotropin GoQuick injection 5.3 mg Genotropin GoQuick injection 12 mg	Genotropin® TC Inj. 5.3mg Genotropin® TC Inj. 12mg Genotropin® GoQuick Inj. 5.3mg Genotropin® GoQuick Inj. 12mg Genotropin® MiniQuick s.c. inj. 0.6mg Genotropin® MiniQuick s.c. inj. 1.0mg Genotropin® MiniQuick s.c. inj. 1.4mg	somatropin (recombinant)			Approved	
6	1994	1994/7/1	(6yaku A) No. 62	2	Tiopronin	Cystinuria including lithiasis	Santen Pharmaceutical Co., Ltd.	Cystinuria	Mylan Seiyaku Ltd.	2002/7/5	Thiola tablet 100 mg	Thiola® Tab.100	Tiopronin			Approved	
6	1994	1994/7/1	(6yaku A) No. 63	1	Transforming growth factor - beta 2 (recombinant)	Repair of idiopathic macular hole	Santen Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (1996/09/25)	1996/9/25	Revoked	
6	1994	1994/7/1	(6yaku A) No. 64	2	Phenylalanine reduced milk containing low-phenylalanine peptide powder digested with milk-protein digestive enzymes	Phenylketonuria	Snow Brand Milk Products Co., Ltd.	Phenylketonuria	Snow Brand Milk Products Co., Ltd.	1999/5/25	Snow Brand Peptiderofe	-	-	This formulation is currently not supplied.		Approved	
6	1994	1994/7/1	(6yaku A) No. 65	1	Protirelin	Improvement of ataxia in spinocerebellar ataxia	Takeda Pharmaceutical Co., Ltd.	-	-	-	-	-	Protirelin	Designation revoked (1996/04/01)	1996/4/1	Revoked	
6	1994	1994/7/1	(6yaku A) No. 66	3	Bropirimine	carcinoma in situ of the bladder	Upjohn Pharmaceuticals, Ltd. Yakult Honsha Co., Ltd.	-	-	-	-	-	Bropirimine	Designation revoked (2004/04/21)	2004/4/21	Revoked	
6	1994	1994/07/01 1996/04/01 *4	(6yaku A) No. 67	2 (only Toray) *4	Beraprost sodium	Primary pulmonary hypertension and pulmonary hypertension as a complication of collagen diseases	Toray Industries, Inc., 1994-07-01, Kaken Pharmaceutical Co., Ltd. 1996-04-01 *4	Primary pulmonary hypertension	Toray Industries, Inc. Kaken Pharmaceutical Co., Ltd.	1999/9/22	Dorner tablet 20 μg Procylin tablet 20 μg	DORNER® Tablets 20μg PROCYLIN® Tablets 20	beraprost			Approved	
6	1994	1994/7/1	(6yaku A) No. 68	1	Mycophenolate mofetil	Treatment of refractory rejection after renal transplantation	Nihon Syntex	Treatment of refractory rejection after renal transplantation (when the patient was diagnosed with refractory rejection and existing drugs are ineffective or cannot be administered due to adverse drug reactions, etc.)	Chugai Pharmaceutical Co., Ltd.	1999/9/22	Cellcept capsule 250 mg	CELLCEPT® Capsule 250	Mycophenolate Mofetil			Approved	
6	1994	1994/7/1	(6yaku A) No. 69	4	Fludarabine phosphate	Chronic lymphocytic leukemia with anemia or thrombocytopenia	Nihon Schering K.K.	Chronic lymphocytic leukemia with anemia or with thrombocytopenia	Sanofi K.K.	1999/9/29	Fludara for IV injection 50 mg	Fludara® 50mg	Fludarabine Phosphate			Approved	
7	1995	1995/4/1	(7yaku A) No. 70	6	N-[(1S,2R)-3-(4-amino-N-isobutylbenzenesulfonamido)-1-benzyl-2-hydroxypropyl]carbamate(3S)-tetrahydro-3-furylester-mesylate	AIDS and symptomatic and asymptomatic HIV infection	Kissei Pharmaceutical Co., Ltd.	HIV-1 infection	Kissei Pharmaceutical Co., Ltd.	1999/9/10	Prozei capsule 150 mg	Prozei	Amprenavir	Designation revoked (2012/12/11) This formulation is currently not supplied.	2012/12/11	Approved	
7	1995	1995/4/1	(7yaku A) No. 71	3	Interferon beta	Senile disciform macular degeneration with foveal neovascularity	Toray Industries, Inc.	-	-	-	-	-	Interferon Beta	Designation revoked (2010/08/11)	2010/8/11	Revoked	
7	1995	1995/4/1	(7yaku A) No. 72	6	Etidronate disodium	Ossification of the posterior longitudinal ligament	Sumitomo Pharmaceuticals Co., Ltd.	-	-	-	-	-	Etidronate Disodium	Designation revoked (2005/08/09)	2005/8/9	Revoked	
7	1995	1995/4/1	(7yaku A) No. 73	4	Cladribine	Hairy cell leukemia	Janssen Kyowa Co., Ltd.	Hairy cell leukemia	Janssen Pharmaceutical K.K.	2002/1/17	Leustatin injection 8 mg	LEUSTATIN® Injection 8mg	cladribine			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
7	1995	1995/4/1	(7yaku A) No. 74	1	Mouse anti-human CD11a monoclonal antibody	Suppression of rejection or graft-versus-host disease (GVHD) in HLA non-compatible bone marrow transplantation in patients with severe combined immunodeficiency disease	Pasteur Mérieux Serums & Vaccines	-	-	-	-	-	-	Designation revoked (2000/05/10)	2000/5/10	Revoked	
7	1995	1995/4/1	(7yaku A) No. 75	3	(R)-N-tertiary-butyl-3-[(2S,3S)-2-hydroxy-3-N-[(R)-2-N-(isoquinolin-5-yloxyacetyl)amino-3-methyl-thio-propanoyl]amino-4-phenylbutanoyl]-1,3-thiazolidine-4-carboxamide	AIDS or symptomatic and asymptomatic HIV infection with 400 or fewer CD4 lymphocytes/ mm3 before treatment	Japan Energy Co., Ltd.	-	-	-	-	-	-	Designation revoked (2002/06/17)	2002/6/17	Revoked	
7	1995	1995/4/1	(7yaku A) No. 76	3	Cyclophosphamide	Pretreatment for bone marrow transplantation for acute leukemia, chronic myelogenous leukemia, myelodysplastic syndrome, malignant lymphoma, multiple myeloma, aplastic anemia, etc.	Shionogi & Co., Ltd.	Pretreatment for hematopoietic stem cell transplantation for the following diseases: acute leukemia, chronic myelogenous leukemia, myelodysplastic syndrome, severe aplastic anemia, malignant lymphoma, hereditary diseases (immunodeficiency, congenital metabolic disorders, congenital hematological disorders: Fanconi anemia, Wiskott-Aldrich syndrome, Hunter's syndrome, etc.)	Shionogi & Co., Ltd.	2003/10/9	Endoxan for injection 100 mg Endoxan for injection 500 mg	Endoxan	Cyclophosphamide Hydrate			Approved	
7	1995	1995/4/1	(7yaku A) No. 77	3	Stavudine	AIDS or HIV infection	Bristol-Myers Squibb	AIDS, HIV infection with ≥500 or fewer CD4 lymphocytes/mm3 before treatment. Monotherapy with this drug should not be used as first-line therapy.	Bristol-Myers Squibb	1997/7/25	Zerit capsule 15 mg Zerit capsule 20 mg	ZERIT® CAPSULES 15 ZERIT® CAPSULES 20	sanilvudine			Approved	
7	1995	1995/4/1	(7yaku A) No. 78	4	Somatropin (recombinant)	Maintenance and increase of fat-free mass in patients with AIDS	Serono Japan Co., Ltd.	Increase and maintenance of fat-free mass in patients with weight loss due to symptomatic HIV infection with ≥200 CD4 lymphocytes/ mm3 or AIDS	Merck Serono Co., Ltd.	1999/3/12	Serostim injection 5 mg	-	-	Designation revoked (2012/06/13) This formulation is currently not supplied.	2012/6/13	Approved	
7	1995	1995/4/1	(7yaku A) No. 79	2	Foscarnet sodium hydrate	Cytomegalovirus retinitis in AIDS patients	Astra Japan, Ltd.	Cytomegalovirus retinitis in AIDS patients	AstraZeneca K.K.	1997/3/28	Foscavir for IV injection 24 mg/mL	Foscavir® Infusion Solution 24mg/Ml	Foscarnet Sodium Hydrate			Approved	
7	1995	1995/4/1	(7yaku A) No. 80	3	Mesna	Prophylaxis for urinary system dysfunction (hemorrhagic cystitis, dysuria, etc.) resulting from cyclophosphamide pre-treatment for bone marrow transplantation	Shionogi & Co., Ltd.	Prophylaxis for urinary system dysfunction (hemorrhagic cystitis, dysuria, etc.) resulting from cyclophosphamide pre-treatment for hematopoietic stem cell transplantation	Shionogi & Co., Ltd.	2003/10/9	Uromitexan for injection 100 mg Uromitexan for injection 400 mg	Uromitexan®	Mesna			Approved	
8	1996	1996/4/1	(8yaku A) No. 81	1	Imiglucerase	Improvement of various symptoms in patients with Gaucher's disease (anemia, thrombocytopenia, hepatosplenomegaly, bone symptoms, etc.)	Genzyme Japan K.K.	Improvement of various symptoms in patients with Gaucher's disease (anemia, thrombocytopenia, hepatosplenomegaly, bone symptoms, etc.)	Genzyme Japan K.K.	1998/3/6	Cerezyme injection 200 U Cerezyme for IV injection 400 U	CEREZYME® injection	imiglucerase (recombinant)			Approved	
8	1996	1996/4/1	(8yaku A) No. 82	4	Gemcitabine hydrochloride	Pancreatic carcinoma	Eli Lilly Japan K.K	Pancreatic carcinoma	Eli Lilly Japan K.K	2001/4/4	Gemzar for injection 200 mg Gemzar for injection 1 g	Gemzar® Injection	Gemcitabine Hydrochloride			Approved	
8	1996	1996/4/1	(8yaku A) No. 83	3	Sapropterin hydrochloride	Improvement of ataxia in Machado-Joseph disease	Suntory Ltd.	-	-	-	-	-	Sapropterin Hydrochloride	Designation revoked (2003/12/12)	2003/12/12	Revoked	
8	1996	1996/4/1	(8yaku A) No. 84	2	Morphine hydrochloride	Relief of severe pain in various types of cancer when oral administration, intravenous injection or subcutaneous injection of narcotics is not sufficiently effective	Shionogi & Co., Ltd.	-	-	-	-	-	Morphine Hydrochloride Hydrate	Designation revoked (2001/08/24)	2001/8/24	Revoked	
8	1996	1996/4/1	(8yaku A) No. 85		Ofloxacin	Hansen's disease	Daiichi Pharmaceutical Co., Ltd.	Hansen's disease	Daiichi Sankyo Company, Limited	1996/8/9	Tarivid tablet 100 mg	TARIVID® TABLETS 100mg	Ofloxacin			Approved	
8	1996	1996/4/1	(8yaku A) No. 86		Ganciclovir	Maintenance therapy for cytomegalovirus retinitis	Tanabe Seiyaku Co., Ltd.	Maintenance therapy for cytomegalovirus retinitis in AIDS patients stabilized with initial treatment with ganciclovir injection, etc. Prevention of onset of cytomegalovirus retinitis in patients with advanced HIV infection with 100 or fewer CD4 lymphocytes/mm3	Mitsubishi Tanabe Pharma Corporation	1997/7/25	Denosine capsule 250	-	Ganciclovir	Designation revoked (2009/09/11) This formulation is currently not supplied. Designation number (16yaku)No. 169 is supplied instead.	2009/9/11	Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
8 30	1996 2018	1996/4/1 2018/7/2*63	(8yaku A) No. 87	3	Dried sulfonated human immunoglobulin	Severe cases of Guillain-Barre syndrome during acute exacerbations with difficulty in walking	Kaketsuken KM Biologics Co., Ltd. *63 Teijin, Ltd.	Severe cases of Guillain-Barre syndrome during acute exacerbations with difficulty in walking	Kaketsuken KM Biologics Co., Ltd. *63	2000/12/12	Kenketsu Venilon-I for IV injection 500 mg Kenketsu Venilon-I for IV injection 1000 mg Kenketsu Venilon-I for IV injection 2500 mg Kenketsu Venilon-I for IV injection 5000 mg	Kenketsu Venilon®-I Kenketsu Venilon®-I Kenketsu Venilon®-I Kenketsu Venilon®-I	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2018/07/02)*63		Approved	
8	1996	1996/4/1	(8yaku A) No. 88		Clofazimine	Hansen's disease	Nihon Ciba-Geigy K.K.	Hansen's disease (multibacillary leprosy, lepromatous erythema nodosum)	Novartis Pharma K.K.	1996/11/12	Lampren capsule 50 mg	Lampren® Capsules 50mg	Clofazimine			Approved	
8	1996	1996/4/1	(8yaku A) No. 89		Blood coagulation factor IX (recombinant)	Prevention or treatment of hemorrhage or complications in hemophilia B	Genetics Institute	Reduction of bleeding tendency in patients with hemophilia B (congenital blood coagulation factor IX deficiency)	Pfizer Japan Inc.	2009/10/16 2013/9/2(BeneFIX IV injection 3000 IU)	BeneFIX IV injection 500 IU BeneFIX IV injection 1000 IU BeneFIX IV injection 2000 IU BeneFIX IV injection 3000 IU	BeneFIX® Intravenous 500 BeneFIX® Intravenous 1000 BeneFIX® Intravenous 2000 BeneFIX® Intravenous 3000	Nonacog Alfa (recombinant)			Approved	
8	1996	1996/4/1	(8yaku A) No. 90	4	Anti-TA two hundred twenty-six human monoclonal antibody	Glioma	Japan Pharmaceutical Development Co., Ltd.	-	-	-	-	-	-	Designation revoked (2005/06/20)	2005/6/20	Revoked	
8	1996	1996/4/1	(8yaku A) No. 91	4	Chimeric anti-human TNF alpha monoclonal antibody	Crohn's disease	Tanabe Seiyaku Co., Ltd.	For the treatment and maintenance therapy of patients with Crohn's disease in the following conditions (limited to those having an inadequate response to conventional therapy) Patients with moderately to severely active Crohn's disease Patients with fistulizing Crohn's disease	Mitsubishi Tanabe Pharma Corporation	2002/01/17 2007/11/13 2011/08/17 (new dose) 2017/05/18 (new dose) *5	Remicade for IV infusion 100 mg	REMICADE® for IV Infusion 100	infliximab (recombinant)			Approved	
8	1996	1996/4/1	(8yaku A) No. 92	2	Cytarabine	Relapsed or refractory acute leukemia including blast crisis in chronic myelogenous leukemia	Nippon Shinyaku Co., Ltd.	The following treatments in acute leukemia (acute myelogenous leukemia and acute lymphoblastic leukemia) · Remission induction (salvage treatment) for relapsed or refractory cases · Consolidation therapy Use should be limited to combination therapy with other anti-cancer drugs for acute lymphoblastic leukemia.	Nippon Shinyaku Co., Ltd	2000/1/18	Cylocide N injection 400 mg Cylocide N injection 1 g	Cylocide N Injection 400mg Cylocide N Injection 1g	Cytarabine			Approved	
8	1996	1996/4/1	(8yaku A) No. 93	7, 1 *6	Human thyrotropin alpha (recombinant)	Detection of residual thyroid after thyroidectomy due to thyroid cancer, support of in-vivo diagnostics for detection of metastatic sites of metastatic thyroid cancer and pretreatment for radioactive iodine treatment to enhance uptake of iodine*6	Sato Pharmaceutical Co., Ltd.	Support of diagnostics with radioactive iodine scintigraphy and serum thyroglobulin (Tg) test or with the Tg test alone in patients treated with total or semi-total thyroidectomy due to differentiated thyroid cancer. Support of ablation of residual thyroid by radioactive iodine in patients treated with total or semi-total thyroidectomy due to differentiated non-metastatic thyroid cancer.*6	Genzyme Japan K.K.	2008/10/16(support of diagnostics) 2012/05/25(ablation) *6	Thyrogen for IM injection 400 mg	THYROGEN®	Thyrotropin human alfa (recombinant)			Approved	
8	1996	1996/4/1	(8yaku A) No. 94		Fibronectin (human plasma)	Prolonged corneal epithelium impairment when treatment with existing drugs for corneal epithelium impairment for one week or longer shows no effect, and slit-lamp microscope examination clearly shows total loss of the corneal epithelial layer and a curling of the edge of the defect area.	Japan Chemical Research Co., Ltd.	-	-	-	-	-	-	Designation revoked (2004/07/07)	2004/7/7	Revoked	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
11	1999	1996/04/01 1999/04/06 *7	(11yaku A) No. 90	1	Lamivudine	Use in combination therapy with zidovudine for AIDS or symptomatic and asymptomatic HIV infection with 500 or fewer CD4 lymphocytes/mm3 before treatment	Nihon Wellcome, 1996-04-01 GlaxoSmithKline K.K., 1999-04-06 *7	Use in combination therapy with zidovudine for HIV infection	ViiV Healthcare K.K.	1997/02/14 1999/06/11 *7	Epivir tablet 150 mg Epivir tablet 300 mg Combivir combination tablet *HC901 Epzicom combination tablet *HC902 Triumeq combination tablet *HC903	EpIVir® Tablets CombIVir® Combination Tablets Epzicom® Combination Tablets Triumeq® Combination Tablets	Lamivudine	Designation revoked (1999/04/06) *7		Approved	
8	1996	1996/4/1	(8yaku A) No. 96	2	Ritonavir	AIDS and HIV Infection	Dainabot Co., Ltd.	Use in combination therapy with nucleoside reverse transcriptase inhibitors in the following diseases · AIDS · symptomatic and asymptomatic HIV infection with 500 or fewer CD4 lymphocytes/mm3 before treatment	AbbVie	1997/11/20	Norvir oral solution 8% Norvir tablet 100 mg Kaletra oral solution *HC961 Kaletra combination tablet *HC961	EpIVir® Tablets CombIVir® Combination Tablets +AS105	Ritonavir			Approved	
8	1996	1996/4/1	(8yaku A) No. 97		Rifampicin	Hansen's disease	Kaken Pharmaceutical Co., Ltd.	Hansen's disease	Kaken Pharmaceutical Co., Ltd.	1996/8/9	Aptecin capsule 150 mg	APTECIN® Capsules 150mg	Rifampicin			Approved	
8	1996	1996/4/1	(8yaku A) No. 98		Rifampicin	Hansen's disease	Kanebo	Hansen's disease	Sandoz	1996/8/9	Rifampicin capsule 150 mg [SANDOZ]	Rifampicin Capsules 150mg [SANDOZ]	Rifampicin			Approved	
8	1996	1996/4/1	(8yaku A) No. 99		Rifampicin	Hansen's disease	Daiichi Pharmaceutical Co., Ltd.	Hansen's disease	Daiichi Sankyo Company, Ltd.	1996/8/9	Rifadin capsule 150 mg	RIFADIN® CAPSULES 150mg	Rifampicin			Approved	
8	1996	1996/4/1	(8yaku A) No. 100		Rifampicin	Hansen's disease	Nihon Ciba-Geigy K.K.	Hansen's disease	Sandoz	1996/8/9	Rimactane capsule 150 mg	-	Rifampicin	This formulation is not currently being supplied.		Approved	
8	1996	1996/4/1	(8yaku A) No. 101		Rifampicin	Hansen's disease	Hishiyama Pharmaceutical	Hansen's disease	Nipro Pharma Corporation	1996/8/9	Rifampicin capsule 150 mg “NP”	-	Rifampicin	This formulation is not currently being supplied.		Approved	
8	1996	1996/4/1	(8yaku A) No. 102	1	Indinavir sulfate ethanolate	AIDS or symptomatic and asymptomatic HIV infection with 500 of fewer CD4 lymphocytes/mm3 before treatment	Banyu Pharmaceutical Co., Ltd.	· AIDS · Symptomatic and asymptomatic HIV infection with 500 or fewer CD4 lymphocytes/mm3 before treatment	Banyu Pharmaceutical Co., Ltd. (Banyu Pharmaceutical Co., Ltd.→MSD K.K.)	1997/3/28	Crixivan capsule 200 mg	CRIXIVAN® Capsules 200mg	Indinavir sulfate ethanolate			Approved	
8	1996	1996/9/25	(8yaku A) No. 103	2	Saquinavir mesylate	Use in combination therapy with reverse transcriptase inhibitor for AIDS or symptomatic and asymptomatic HIV Infection	Nippon Roche Ltd.	HIV Infection	Chugai Pharmaceutical Co., Ltd.	1997/9/5	Invirase capsule 200 mg Invirase tablet 500 mg	INVIRASE® Capsule 200mg INVIRASE® Tablet 500mg	Saquinavir Mesylate			Approved	
8	1996	1996/12/20	(8yaku A) No. 104	2	Nevirapine	AIDS or symptomatic and asymptomatic HIV-1 Infection	Nippon Boehringer Ingelheim Co., Ltd.	HIV-1 Infection	Nippon Boehringer Ingelheim Co., Ltd.	1998/11/27	Viramune tablet 200 mg	Viramune® Tablets 200	Nevirapine			Approved	
8	1996	1996/12/20	(8yaku A) No. 105	2	Nelfinavir mesylate	AIDS or symptomatic and asymptomatic HIV Infection	Japan Tobacco, Inc.	HIV Infection	Japan Tobacco, Inc.	1998/03/06 2004/01/19 *8	Viracept tablet 250 mg	Viracept® Tab. 250mg	Nelfinavir mesylate			Approved	
8	1996	1997/3/27	(9yaku A) No. 106	2	8- carbamoyloctyl α-D-galactopyranosyl (1-4)β-D-galactopyranosyl(1-4)-β-D-glucopyranoside siloxypropyldiatomite	Removal of verotoxin (Shiga-like toxin; SLT) produced by enterohemorrhagic Escherichia coli from the gastrointestinal tract	Takeda Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (2002/03/15)	2002/3/15	Revoked	
8	1996	1997/3/27	(9yaku A) No. 107	1	Clotrimazole	Oral candidiasis in patients with HIV infection	Bayer Holding Ltd.	Mild or moderate oral candidiasis in patients with HIV infection	Bayer Holding Ltd.	1999/6/11	Empecid troche 10 mg	Empecid® Troche	Clotrimazole			Approved	
8	1996	1997/3/27	(9yaku A) No. 108		Fluconazole	Suppression of recurrent cryptococcal meningitis or treatment for oral candidiasis in patients with AIDS	Pfizer Japan Inc.	-	-	-	-	-	Fluconazole				
13	2001	1997/06/16 2002/01/24 *9	(14yaku) No. 109	3	Verteporfin	Senile disciform macular degeneration with foveal neovascularity	Ciba Vision Corporation, 1997-06-16 Novartis Pharma K.K., 2002-01-24 *9	Age-related macular degeneration with subfoveal choroidal neovascularization	Novartis Pharma K.K.	2003/10/16	Visudyne for IV injection 15 mg	Visudyne®	Verteporfin	Designation revoked (2002/01/24) *9		Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
9	1997	1997/6/16	(9yaku A) No. 110	5	1-(2-naphthalene-2-ylethyl)-4-(3-trifluoromethylphenyl)-1,2,3,6-tetrahydropyridine hydrochloride	Amyotrophic lateral sclerosis (ALS)	Sanofi K.K.	-	-	-	-	-	-	Designation revoked (2004/02/06)	2004/2/6	Revoked	
9	1997	1998/3/20	(10yaku A) No. 111	4	Doranidazole	To increase efficacy of intraoperative radiation therapy for pancreatic cancer	Pola Chemical Industries, Inc.	-	-	-	-	-	Doranidazole				
9	1997	1998/3/20	(10yaku A) No. 112	4	Monteplase (recombinant)	Thrombolysis of acute pulmonary embolism in the pulmonary artery	Eisai Co., Ltd.	Thrombolysis of acute pulmonary embolism in the pulmonary artery with associated unstable hemodynamics	Eisai Co., Ltd.	2005/7/25	Cleactor for IV injection 400,000 IU Cleactor for IV injection 800,000 IU Cleactor for IV injection 1,600,000 IU	Cleactor® for Intravenous Injection 400,000 Cleactor® for Intravenous Injection 800,000 Cleactor® for Intravenous Injection 1,600,000	Monteplase (recombinant)			Approved	
10	1998	1998/9/4	(10yaku A) No. 113	7	5-methyl-1-phenyl-2-(1H)- pyridone	Interstitial pneumonia (except acute cases and acute exacerbation of other cases)	Shionogi & Co., Ltd.	Idiopathic pulmonary fibrosis	Shionogi & Co., Ltd.	2008/10/16	Pirespa tablet 200 mg	Pirespa®	Pirfenidone			Approved	
10	1998	1998/11/27	(10yaku A) No. 114	3	Rituximab	B-cell non-Hodgkin's lymphoma limited to patients with CD20 differentiation antigen on the surface of tumor cells	Zenyaku Kogyo Co., Ltd.	CD20-positive B-cell non-Hodgkin's lymphoma	Zenyaku Kogyo Co., Ltd.	2001/06/20 2003/09/19 *10 2018/02/02(new name of product approved for manufacturing and marketing)	Rituxan injection 10 mg/mL(100 mg/10 mL) Rituxan injection 10 mg/mL(500 mg/50 mL) (new name of product approved for manufacturing and marketing:2018.2.2 approved) Rituxan Intravenous Infusion 100mg Rituxan Intravenous Infusion 500mg	Rituxan® Injection	Rituximab (recombinant)			Approved	
10	1998	1998/11/27	(10yaku A) No. 115		Ivermectin	Strongyloidiasis	Banyu Pharmaceutical Co., Ltd.	Intestinal tract strongyloidiasis	MSD K.K.	2002/10/8	Stromectol tablet 3mg	STROMECTOL® Tablets 3mg	Ivermectin			Approved	
10	1998	1998/11/27	(10yaku A) No. 116	4	Tamibarotene	Acute promyelocytic leukemia	Toko Pharmaceutical Industrial Co., Ltd.	Relapsed or refractory acute promyelocytic leukemia	Toko Pharmaceutical Industrial Co., Ltd.	2005/4/11	Amnolake tablet 2 mg	Amnolake® tablets 2mg	Tamibarotene			Approved	
10	1998	1999/1/21	(11yaku A) No. 117	5	Human anti-CD33 monoclonal antibody conjugated with calicheamicin	Relapsed or refractory acute myelogenous leukemia	Wyeth Lederle Japan, Ltd.	Relapsed or refractory CD33-positive acute myelogenous leukemia	Wyeth, Ltd. (Wyeth, Ltd.→Pfizer Japan Inc.)	2005/7/25	Mylotarg injection 5 mg	MYLOTARG® Injection 5mg	Gemtuzumab ozogamicin (recombinant)			Approved	
10	1998	1999/3/4	(11yaku A) No. 118	4	Spherical carbon adsorbent	Improvement of fistula in Crohn's disease	Kureha Corporation	-	-	-	-	-	-	Designation revoked (2013/05/13)	2013/5/13	Revoked	
14	1998	1999/03/04 2002/05/28 *11	(14yaku) No. 119	3	Recombinant human growth hormone receptor binding protein	Acromegaly	Sensus Drug Development Corp., 1999-03-04 Pharmacia (currently Pfizer Japan Inc.), 2002-05-28 *11	Improvement of IGF-I (somatomedin C) oversecretion and various symptoms in acromegaly limited to cases where surgical treatment or multiple drug treatment is insufficiently effective or difficult to administer	Pfizer Japan Inc.	2007/1/26	Somavert for SC injection 10 mg Somavert for SC injection 15 mg Somavert for SC injection 20 mg	SOMAVERT® for SC Injection 10mg SOMAVERT® for SC Injection 15mg SOMAVERT® for SC Injection 20mg	Pegvisomant (recombinant)	Designation revoked (2002/05/28) *11		Approved	
10	1998	1999/3/4	(11yaku A) No. 120		Phenobarbital sodium	Neonatal convulsions	Wyeth Lederle Japan, Ltd.	-	-	-	-	-	Phenobarbital Sodium	Designation revoked (2003/12/12)	2003/12/12	Revoked	
10	1998	1999/3/4	(11yaku A) No. 121		Relaxin	Scleroderma	Suntory Ltd.	-	-	-	-	-	-	Designation revoked (2002/03/15)	2002/3/15	Revoked	
10	1998	1999/3/4	(11yaku A) No. 122		Tacrolimus hydrate	Generalized myasthenia gravis when post-thymectomy steroid treatment is not sufficiently effective or it cannot be administered due to adverse drug reactions	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Generalized myasthenia gravis when post-thymectomy steroid treatment is not sufficiently effective or it cannot be administered due to adverse drug reactions *12	Astellas Pharma Inc.	2000/9/22	Prograf capsule 0.5 mg Prograf capsule 1 mg Prograf granule 0.2 mg Prograf granule 1 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg Prograf® Granules 0.2mg Prograf® Granules 1mg	Tacrolimus Hydrate			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
10	1998	1999/3/4	(11yaku A) No. 123	5	Interferon beta 1a	Multiple sclerosis	Genzyme Japan K.K.	Prevention of relapse of multiple sclerosis	Biogen Idec Japan Ltd.	2006/7/26	Avonex IM injection syringe 30 μg	AVONEX® IM Injection Syringe	Interferon Beta-1a (recombinant)			Approved	
10	1998	1999/3/4	(11yaku A) No. 124		Vancomycin hydrochloride	Meningitis, septicemia or pneumonia due to high penicillin-resistant Streptococcus pneumoniae	Eli Lilly Japan K.K.	Indicated bacterial strains: Vancomycin-sensitive penicillin-resistant Streptococcus pneumonia(PRSP) Indications: Septicemia, pneumonia, purulent meningitis	Shionogi & Co., Ltd.	2004/10/22	Vancomycin Hydrochloride for IV infusion 0.5 g Vancomycin Hydrochloride for IV infusion kit 0.5 g	Vancomycin	Vancomycin Hydrochloride			Approved	
10	1998	1999/3/17	(11yaku A) No. 125	3	Methionyl human stem cell factor	Aplastic anemia	Amgen	-	-	-	-	-	-	Designation revoked (2003/07/01)	2003/7/1	Revoked	
16	2004	1999/05/27 2004/08/05 2010/07/02 2013/04/04 *13	(11yaku) No. 126	3	Anagrelide hydrochloride, 1999-05-27 Anagrelide hydrochloride, 2010-07-02 *13	Essential thrombocythemia	Robert Pharmaceuticals Co., 1999-05-27 Kirin Brewery Company, Limited (currently Kyowa Hakko Kirin Co., Ltd.), 2004-08-05 Shire Pharmaceuticals Ireland Ltd., 2010-07-02 Shire Japan KK, 2013-04-04 *13	Essential thrombocythemia	Shire Japan KK	2014/9/26	Agrylin Capsules 0.5mg	Agrylin®Capsules 0.5mg	Anagrelide Hydrochloride Hydrate	Designation revoked (2004/08/05) Designation revoked (2010/07/02) Designation revoked (2013/04/04) *13		Approved	
11	1999	1999/5/27	(11yaku) No. 127	4	α-galactosidase A	Improvement of various symptoms in patients with Fabry's disease	Sumitomo Pharmaceuticals	Fabry's disease	Dainippon Sumitomo Pharma Co., Ltd.	2006/10/20	Replagal for IV infusion 3.5 mg	REPLAGAL®	Agalsidase Alfa (recombinant)			Approved	
11	1999	1999/6/29	(11yaku) No. 128		Efavirenz	AIDS or symptomatic and asymptomatic HIV-1 Infection	Banyu Pharmaceutical Co., Ltd.	HIV-1 infection	MSD K.K.	1999/9/10	Stocrin tablet 200 mg Stocrin tablet 600 mg	STOCRIN® Tablets 200mg STOCRIN® Tablets 600mg	Efavirenz			Approved	
11	1999	1999/7/9	(11yaku) No. 129	5	Abacavir	AIDS or symptomatic and asymptomatic HIV Infection	GlaxoWellcome	HIV infection	ViiV Healthcare K.K.	1999/9/10	Ziagen tablet 300 mg Epzicom combination tablet *HC1291 Triumeq combination tablet *HC1292	Ziagen® Tablets 300mg Epzicom® Combination Tablets Triumeq® Combination Tablets	Abacavir Sulfate			Approved	
11	1999	1999/8/25	(11yaku) No. 130		Basiliximab	Inhibition of acute rejection after renal transplantation	Novartis Pharma K.K.	Inhibition of acute rejection after renal transplantation	Novartis Pharma K.K.	2002/1/17 2008/6/6(Simulect IV injection 10 mg for pediatric use)	Simulect IV injection 20 mg Simulect IV injection 10 mg for pediatric use	Simulect® IV injection 20mg Simulect® IV injection 10mg for pediatric	Basiliximab (recombinant)			Approved	
11	1999	1999/8/25	(11yaku) No. 131	1	Cyclosporine eye drop	Vernal conjunctivitis when anti-allergic drugs are not sufficiently effective	Santen Pharmaceutical Co., Ltd.	Vernal conjunctivitis when anti-allergic drugs are not sufficiently effective	Santen Pharmaceutical Co., Ltd.	2005/10/11	Papilock Mini ophthalmic solution 0.1%	PAPILOCK® Mini ophthalmic solution 0.1%	Cyclosporine			Approved	
11	1999	1999/8/25	(11yaku) No. 132		Trastuzumab	Metastatic breast cancer with overexpression of HER2	Nippon Roche Ltd.	Metastatic breast cancer with overexpression of HER2	Chugai Pharmaceutical Co., Ltd.	2001/4/4	Herceptin for injection 60 mg Herceptin for injection 150 mg	HERCEPTIN® Intravenous Infusion 60 HERCEPTIN® Intravenous Infusion 150	Trastuzumab (recombinant)			Approved	
11	1999	1999/8/25	(11yaku) No. 133	4	α-L-iduronidase	Improvement of various symptoms in patients with mucopolysaccharidosis I	Genzyme Japan K.K.	Mucopolysaccharidosis I	Genzyme Japan K.K.	2006/10/20	Aldurazyme for IV infusion 2.9 mg	ALDURAZYME®	Laronidase (recombinant)			Approved	
11	1999	1999/8/25	(11yaku) No. 134	2	α-galactosidase A	Improvement of various symptoms in patients with Fabry's disease	Genzyme Japan K.K.	Fabry's disease	Genzyme Japan K.K.	2004/1/29	Fabrazyme for IV infusion 5 mg Fabrazyme for IV infusion 35 mg	FABRAZYME®	Agalsidase Beta (recombinant)			Approved	
11	1999	1999/11/24	(11yaku) No. 135	1	Saquinavir	HIV infection	Nippon Roche Ltd.	HIV infection	Chugai Pharmaceutical Co., Ltd.	2000/4/6	Fortovase capsule	-	-	This formulation is currently not being supplied. Designation number “8yaku No. 103” is being supplied instead.		Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
11	1999	1999/12/9	(11yaku) No. 136		Delavirdine mesylate	HIV-1 infection	Warner-Lambert	HIV-1 infection	ViiV Healthcare K.K.	2000/2/25	Rescriptor tablet 200 mg	-	Delavirdine Mesylate	Designation revoked (2012/09/13) This formulation is currently not being supplied.	2012/9/13	Approved	
15	2003	2000/01/06 2003/07/01 *14	(15yaku) No. 137	5	levocarnitine	Erythropoietin-resistant renal anemia in hemodialysis patients	Shimizu Pharmaceutical Co. Ltd., 2000-01-06 Ajinomoto Co. Inc., 2003-07-01 *14	-	-	-	-	-	-	Designation revoked (2003/07/01)*14 Designation revoked (2006/02/03)	2006/2/3	Revoked	
14	2002	2000/01/06 2002/12/02 *15	(14yaku) No. 138		Polyethylene glycol-treated human immunoglobulin	Steroid treatment-resistant polymyositis or dermatomyositis (limited to the cases with clear muscle weakness interfering with daily activities)	Yoshitomi Pharmaceutical Co. Ltd., 2000-01-06 Mitsubishi Pharma Corporation, 2002-12-02 *15	Improvement of muscle weakness in polymyositis and dermatomyositis (limited to cases in which steroids are inadequate)	Japan Blood Products Organization	2010/10/27 2013/2/15(Venoglobulin IH 5% IV injection 10 g/200 mL)	Venoglobulin IH 5% IV injection 0.5 g/10 mL Venoglobulin IH 5% IV injection 1 g/20 mL Venoglobulin IH 5% IV injection 2.5 g/50 mL Venoglobulin IH 5% IV injection 5 g/100 mL Venoglobulin IH 5% IV injection 10 g/200 mL	Venoglobulin® IH5%IV0.5g/10mL Venoglobulin® IH5%IV1g/20mL Venoglobulin® IH5%IV2.5g/50mL Venoglobulin® IH5%IV5g/100mL Venoglobulin® IH5%IV10g/200mL	Polyethylene Glycol-treated Normal Human Immunoglobulin	Designation revoked (2002/12/02) *15		Approved	
11	1999	2000/1/6	(11yaku) No. 139	6	Modafinil	Narcolepsy	Azwell Inc.	Excessive daytime sleepiness associated with narcolepsy	Alfresa Pharma Corporation	2007/1/26	Modiodal tablet 100 mg	MODIODAL® Tablets 100mg	Modafinil			Approved	
12	2000	2000/4/3	(11yaku) No. 140		Ganciclovir preparation for intraocular implant	Cytomegalovirus retinitis in AIDS	Bausch & Lomb Japan	-	-	-	-	-	-	Designation revoked (2014/2/26)	2014/2/26	Revoked	
12	2000	2000/6/16	(12yaku) No. 141		Somatropin (recombinant)	Improvement of body composition abnormalities in Prader-Willi syndrome	Pharmacia & Upjohn	Short stature in Prader-Willi syndrome without the epiphyseal closure	Pfizer Japan Inc.	2002/1/17	Genotropin TC injection 5.3 mg Genotropin TC injection 12 mg Genotropin MiniQuick SC injection 0.6 mg Genotropin MiniQuick SC. injection 1.0 mg Genotropin MiniQuick SC injection 1.4 mg Genotropin GoQuick injection 5.3 mg Genotropin GoQuick injection 12 mg	Genotropin® TC Inj. 5.3mg Genotropin® TC Inj. 12mg Genotropin® GoQuick Inj. 5.3mg Genotropin® GoQuick Inj. 12mg Genotropin® MiniQuick s.c. inj. 0.6mg Genotropin® MiniQuick s.c. inj. 1.0mg Genotropin® MiniQuick s.c. inj. 1.4mg	Somatropin (recombinant)			Approved	
12	2000	2000/9/20	(12yaku) No. 142	5	Follitropin alfa (recombinant)	Male hypogonadotropic hypogonadism	Serono Japan Co., Ltd.	Spermatogenesis induction in male patients with hypogonadotropic hypogonadism	Merck Serono Co., Ltd.	2006/1/23 2008/10/22(Gonalef pen SC injection 300 IU, 450IU, 900IU)	Gonalef SC injection 75 IU Gonalef SC injection 150 IU Gonalef pen SC injection 300 IU Gonalef pen SC injection 450 IU Gonalef pen SC injection 900 IU	Gonalef® 75 Gonalef® 150 Gonalef® Pen 300 Gonalef® Pen 450 Gonalef® Pen 900	Follitropin alfa (recombinant)			Approved	
12	2000	2000/9/20	(12yaku) No. 143		Lopinavir	HIV infection	Dainabot Co., Ltd	HIV infection	AbbVie	2000/12/12	Kaletra oral solution *HC1431 Kaletra combination tablet *HC1431	Kaletra®	Lopinavir			Approved	
12	2000	2000/12/20	(12yaku) No. 144		4-(4-methyl-piperazine-1-yl methyl)-N-[4-methyl-3-[(4-pyridine-3-yl)-pyrimidine-2-ylamino]phenyl] benzamide-methanesulfonate	Philadelphia chromosome-positive leukemia	Novartis Pharma K.K.	Chronic myelogenous leukemia, Philadelphia chromosome-positive acute lymphoblastic leukemia	Novartis Pharma K.K.	2001/11/21, Chronic myelogenous leukemia 2007/01/31, Philadelphia chromosome-positive acute lymphoblastic leukemia	Glivec tablet 100 mg	Glivec® Tablets 100mg	Imatinib mesylate			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
12	2000	2000/12/20	(12yaku) No. 145		Humanized anti–interleukin-6 receptor monoclonal antibody (recombinant)	Castleman's disease	Chugai Pharmaceutical Co., Ltd.	Improvement of various symptoms and test results (high C-reactive protein, high fibrinogen, high erythrocyte sedimentation rate, low hemoglobin, low albumin, general malaise) in Castleman's disease. Use should be limited to patients for whom lymphadenectomy is not indicated.	Chugai Pharmaceutical Co., Ltd.	2005/4/11 2008/04/16(Actemra for IV infusion 80 mg, 400 mg)	Actemra for IV infusion 80 mg Actemra for IV infusion 200 mg Actemra for IV infusion 400 mg	ACTEMRA® 80 mg for Intravenous Infusion ACTEMRA® 200 mg for Intravenous Infusion ACTEMRA® 400 mg for Intravenous Infusion	Tocilizumab (recombinant)			Approved	
12	2000	2000/12/20	(12yaku) No. 146		Imidapril hydrochloride	Insulin-dependent diabetic nephropathy	Tanabe Seiyaku Co., Ltd.	Diabetic nephropathy associated with type 1 diabetes mellitus	Mitsubishi Tanabe Pharma Corporation	2002/1/17	Tanatril tablet 2.5 mg Tanatril tablet 5 mg	TANATRIL® Tablets 2.5 TANATRIL® Tablets 5	Imidapril Hydrochloride			Approved	
12	2000	2000/12/20	(12yaku) No. 147		Azithromycin hydrate	Disseminated mycobacterial infection associated with AIDS	Pfizer Japan Inc.	Prophylaxis and treatment of disseminated *Mycobacterium avium* complex (MAC) associated with AIDS	Pfizer Japan Inc.	2001/12/13	Zithromac tablet 600 mg	ZITHROMAC® Tablets 600mg	Azithromycin Hydrate			Approved	
12	2000	2000/11/27	(12yaku) No. 148		Didanosine	HIV infection	Bristol	HIV infection	Bristol-Myers	2001/3/7	Videx EC capsule 125 mg Videx EC capsule 200 mg	VIDEX EC CAPSULES／Enteric-Coated Beadlets VIDEX EC CAPSULES／Enteric-Coated Beadlets	Didanosine			Approved	
13	2001	2001/4/23	(13yaku) No. 149		Dried sulfonated human immunoglobulin	Reduction of frequency of exacerbations in multiple sclerosis (MS), suppression of MS progression	Kaketsuken	-	-	-	-	-	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2012/03/19)	2012/3/19	Revoked	
15	2003	2001/04/23 2003/11/05 *16	(15yaku) No. 150	5	Dried sulfonated human immunoglobulin	Reduction of frequency of exacerbation, attack and relapse of multiple sclerosis (MS), prevention of progression to serious MS	Teijin Ltd., 2001-04-23, Teijin Pharma Limited, 2003-11-05 *16	-	-	-	-	-	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2003/11/05)*16 Designation revoked (2012/03/19)	2012/3/19	Revoked	
13	2001	2001/4/23	(13yaku) No. 151	5	Baclofen (intrathecal continuous infusion)	Severe spastic paralysis caused by cerebral (infantile) palsy, spinal vascular disorder, cervical spondylosis, posterior longitudinal ligament ossification, multiple sclerosis, spinocerebellar degeneration (hereditary spastic paraplegia) or post-traumatic complications of spinal injury or head trauma	Daiichi Pharmaceutical Co., Ltd.	Severe spastic paralysis caused by cerebrospinal disease. Use should be limited to cases in which existing treatment is not sufficiently effective.	Daiichi Sankyo Company, Limited	2005/04/11 2007/01/26 (approval of the expanded age indication)	Gabalon intrathecal injection 0.005% 1 mL Gabalon intrathecal injection 0.05% 20 mL Gabalon intrathecal injection 0.2% 5 mL	GABALON INTRATHECAL INJECTION 0.005% GABALON® INTRATHECAL INJECTION 0.05% GABALON® INTRATHECAL INJECTION0.2%	Baclofen			Approved	
13	2001	2001/4/23	(13yaku) No. 152	7	Vancomycin ophthalmic ointment	Ocular infections such as blepharitis, conjunctivitis or keratitis caused by methicillin-cephem-resistant Staphylococcus aureus or Staphylococcus epidermidis	Toa Pharmaceuticals Co., Ltd.	Indicated bacterial strains: Vancomycin-sensitive, methicillin-resistant Staphylococcus aureus (MRSA) or Staphylococcus epidermidis (MRSE) Indications: Conjunctivitis, blepharitis, meibomianitis, and dacryocystitis in which existing treatments are not sufficiently effective	Toa Pharmaceuticals Co., Ltd.	2009/10/16	Vancomycin ophthalmic ointment 1%	Vancomycin Ophthalmic Ointment 1%	Vancomycin Hydrochloride			Approved	
15	2003	2001/04/23 2003/11/05 *17	(15yaku) No. 153	7	Anti-Shiga-like toxin II humanized monoclonal antibody	Inhibition of hemolytic-uremic syndrome, encephalopathy or hemolytic anemia due to Escherichia coli infection with Shiga-like toxin II production	Teijin Ltd., 2001-04-23, Teijin Pharma Limited, 2003-11-05 *17	-	-	-	-	-	-	Designation revoked (2003/11/05)*17 Designation revoked (2010/05/13)	2010/5/13	Revoked	
13	2001	2001/8/1	(13yaku) No. 154	2	Tiracoxib	Familial adenomatous polyposis	Japan Tobacco, Inc.	-	-	-	-	-	-	Designation revoked (2004/03/22)	2004/3/22	Revoked	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
13	2001	2001/8/24	(13yaku) No. 155	3	OPC-31260	Improvement of hyponatremia due to inappropriate secretion of vasopressin	Otsuka Pharmaceutical Co., Ltd.	Improvement of hyponatremia in the syndrome of inappropriate secretion of antidiuretic hormone (SIADH) due to an ectopic antidiuretic hormone-producing tumor. Use should be limited to cases in which existing treatments are not sufficiently effective.	Otsuka Pharmaceutical Co., Ltd.	2006/7/26	Physuline tablet 30 mg	Physuline® tablets 30mg	Mozavaptan Hydrochloride			Approved	
13	2001	2002/3/15	(14yaku) No. 156		Infliximab	Refractory uveoretinitis caused by Behcet's disease. Limited to cases in which existing treatments are not sufficiently effective.	Tanabe Seiyaku Co., Ltd.	Refractory uveoretinitis caused by Behcet's disease limited to cases in which existing treatments are not sufficiently effective	Mitsubishi Tanabe Pharma Corporation	2007/1/26	Remicade for IV infusion 100 mg	REMICADE® for IV Infusion100	infliximab (recombinant)			Approved	
14	2002	2002/6/17	(14yaku) No. 157		Epoprostenol sodium	Pulmonary arterial hypertension excluding primary pulmonary hypertension	GlaxoSmithKline K.K.	Pulmonary arterial hypertension	GlaxoSmithKline K.K.	2004/6/22	Flolan for IV injection 0.5 mg Flolan for IV injection 1.5 mg	Flolan® for injection 0.5mg Flolan® for injection 1.5mg	Epoprostenol Sodium			Approved	
14	2002	2002/10/2	(14yaku) No. 158	3	Nitric oxide	Improvement of hypoxemic respiratory failure in pulmonary hypertension. Use should be limited to newborns.	Ino Therapeutics Inc. Agent in Japan: Parexel International Corporation	Improvement of hypoxemic respiratory failure with accompanying pulmonary hypertension in newborns	Ino Therapeutics, LLC Air Water Co. is the exclusive manufacturing agent.	2008/7/16	Inoflo for inhalation 800 ppm	INOflo® for inhalation 800ppm	Nitric Oxide			Approved	
14	2002	2002/10/2	(14yaku) No. 159		Imatinib mesylate	Gastrointestinal stromal tumor	Nihon Ciba-Geigy K.K.	KIT(CD117)-positive gastrointestinal stromal tumor	Novartis Pharma K.K.	2003/7/17	Glivec tablet 100 mg	Glivec® Tablets 100mg	Imatinib Mesylate			Approved	
14	2002	2002/12/2	(14yaku) No. 160	3	Tacrolimus hydrate	Lupus nephritis	Fujisawa Pharmaceutical Co., Ltd. (currently Astellas Pharma Inc.)	Lupus nephritis limited to cases where steroids are not sufficiently effective or cannot be administered due to adverse reactions	Astellas Pharma Inc.	2007/1/26	Prograf capsule 0.5 mg Prograf capsule 1 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg	Tacrolimus Hydrate			Approved	
14	2002	2003/1/31	(15yaku) No. 161		Bosentan	Pulmonary arterial hypertension	Actelion Pharmaceuticals Japan Ltd.	Pulmonary arterial hypertension limited to World Health Organization (WHO) Class Ⅱ, III and IV Pulmonary arterial hypertension(Tracleer 32mg dispersible tablets for pediatric)	Actelion Pharmaceuticals Japan Ltd.	2005/4/11 2012/11/21 (addition of Class Ⅱ of WHO criteria) 2015/9/28(Tracleer 32mg dispersible tablets for pediatric)	Tracleer tablet 62.5 mg Tracleer 32mg dispersible tablets for pediatric	Tracleer® 62.5(Tracleer tablet 62.5 mg) TRACLEER(Tracleer 32mg dispersible tablets for pediatric)	Bosentan hydrate			Approved	
15	2003	2003/5/29	(15yaku) No. 162		Fluocinolone acetonide preparation for intraocular implantation	Uveitis extending to the posterior segment of the eye	Bausch & Lomb Japan	-	-	-	-	-	-	Designation revoked (2014/03/17)	2014/3/17	Revoked	
15	2003	2003/06/17 2004/03/30 *18	(15yaku) No. 163	2	Amiodarone hydrochloride	Recurrent life-threatening cardiac arrhythmias: ventricular fibrillation and hemodynamically unstable ventricular tachycardia	Taisho Pharmaceutical Co., Ltd., Taisho Sanofi-Synthélabo (currently Sanofi Aventis after Japan Winthrop Pharmaceutical), 2003-06-17 Sanofi-Synthélabo Corp. (currently Sanofi Aventis), 2004-03-30 *18	Refractory life-threatening refractory life-threatening cardiac arrhythmias limited to emergency cases: ventricular fibrillation and hemodynamically unstable ventricular tachycardia	Sanofi K.K.	2007/1/26	Ancaron injection 150 mg	Ancaron® inj. 150	Amiodarone Hydrochloride	Designation revoked (2004/03/30) *18		Approved	
15	2003	2003/8/1	(15yaku) No. 164		Atazanavir sulfate	HIV Infection	Bristol	HIV-1 Infection	Bristol-Myers	2003/12/18	Reyataz capsule 150 mg Reyataz capsule 200 mg	REYATAZ CAPSULES 150mg REYATAZ CAPSULES 200mg	Atazanavir Sulfate			Approved	
15	2003	2003/9/26	(15yaku) No. 165	1	Busulfan	Pretreatment for hematopoietic stem cell transplantation	Kirin Brewery Company, Limited	· Pretreatment for allogenic hematopoietic stem cell transplantation · Pretreatment for autologous hematopoietic stem cell transplantation for the Ewing sarcoma family of tumors and neuroblastoma	Otsuka Pharmaceutical Co., Ltd.	2006/07/26 2006/10/20 (Approval of the expanded age indication)	Busulfex for IV infusion 60 mg	Busulfex® injection	Busulfan			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
15	2003	2003/12/12	(15yaku) No. 166	1	Tenofovir disoproxil fumarate	HIV-1 Infection	Japan Tobacco, Inc.	HIV-1 Infection	Japan Tobacco, Inc.	2004/3/25(Viread tablet 300 mg) 2005/3/23(approved as Truvada tablet)(2008/12/15:approved as Truvada combination tablet)*HC1661 2013/3/25(Stribild combination tablet)*HC1662 2014/11/18(COMPLERA Combination Tablets)*HC1663	Viread tablet 300 mg Truvada combination tablet *HC1661 Stribild combination tablet *HC1662 COMPLERA Combination Tablets*HC1663	Viread® Tab.300mg(Viread tablet 300 mg) Truvada® Combination Tab.*HC1661 Stribild® Combination Tab. *HC1662 COMPLERA® Combination Tablets*HC1663	Tenofovir Disoproxil Fumarate(Viread tablet 300 mg) Emtricitabine/Tenofovir disoproxil fumarate*HC1661 Elvitegravir/Cobicistat/Emtricitabine/Tenofovir disoproxil fumarate*HC1662 Rilpivirine Hydrochloride/Emtricitabine/Tenofovir Disoproxil Fumarate*HC1663			Approved	
15	2003	2003/12/12	(15yaku) No. 167		Bortezomib	Relapsed or refractory multiple myeloma	Janssen Pharmaceutical K.K.	Multiple myeloma *19	Janssen Pharmaceutical K.K.	2006-10-20 2011-09-16 (new indication, new dosage) *19	Velcade for injection 3 mg	VELCADE® Injection	Bortezomib			Approved	
15	2003	2004/3/22	(16yaku) No. 168		Argatroban	Heparin-induced thrombocytopenia (HIT): prophylaxis or treatment of thrombosis, anticoagulation during percutaneous coronary intervention (PCI) (including patients at risk of HIT) and prevention of coagulation of perfused blood during extracorporeal circulation (hemodialysis)	Mitsubishi Pharma Corporation Daiichi Pharmaceutical Co., Ltd.	Prevention of blood coagulation in extracorporeal circuit in the following patients (during haemodialysis) - Patients with heparin-induced thrombocytopenia (HIT) type II Prevention of blood coagulation in patients with or at risk for heparin-induced thrombocytopenia (HIT) type II under-going percutaneous coronary intervention (PCI) Prophylaxis or treatment of thrombosis in patients with heparin-induced thrombocytopenia (HIT) type II *20	Mitsubishi Tanabe Pharma Corporation Daiichi-Sankyo Company, Limited	2008/07/16 2011/05/20 *20	Novastan HI injection 10 mg/2 mL Slonnon HI injection 10 mg/2 mL	Novastan® HI inj. 10mg/2mL SLONNON® HI INJECTION	Argatroban Hydrate			Approved	
16	2004	2004/7/7	(16yaku) No. 169		Valganciclovir	Treatment of cytomegalovirus retinitis in patients with AIDS	Tanabe Seiyaku Co., Ltd.	Treatment of cytomegalovirus retinitis in patients with AIDS	Mitsubishi Tanabe Pharma Corporation	2004/11/5	Valixa Tablet 450 mg	VALIXA® Tablets 450mg	Valganciclovir Hydrochloride			Approved	
16	2004	2004/7/7	(16yaku) No. 170		Pegaptanib sodium	Age-related macular degeneration with subfoveal choroidal neovascularization	Pfizer Japan Inc.	Age-related macular degeneration with subfoveal choroidal neovascularization	Pfizer Japan Inc.	2008/7/16	Macugen IVT injection kit 0.3 mg	MACUGEN® IVT Inj. KIT 0.3mg	Pegaptanib sodium			Approved	
16	2004	2004/07/07 2005/12/13 *21	(16yaku) No. 171		Tacrolimus hydrate	Vernal keratoconjunctivitis for which anti-allergic drugs are not sufficiently effective	Fujisawa Pharmaceutical Co., Ltd.(currently Astellas Pharma Inc.), 2004-07-07 Senju Pharmaceutical Co., Ltd., 2005-12-13 *20	Vernal keratoconjunctivitis for which anti-allergic drugs are not sufficiently effective	Astellas Pharma Inc. Senju Pharmaceutical Co., Ltd.	2008/1/25	Talymus ophthalmic suspension 0.1%	TALYMUS® OPHTHALMIC SUSPENSION 0.1%	Tacrolimus Hydrate			Approved	
16	2004	2004/10/13	(16yaku) No. 172	1	Emtricitabine	HIV-1 Infection	Japan Tobacco, Inc.	HIV-1 Infection	Japan Tobacco, Inc.	2005/3/23 (Emtriva Capsules 200mg) 2005/3/23(approved as Truvada tablet)(2008/12/15:approved as Truvada combination tablet)*HC1721 2013/3/25(Stribild combination tablet)*HC1722 2016/6/17(Genvoya Combination Tablets)*HC1724 2016/12/9(DESCOVY Combination Tablets LT, HT)*HC1723	Emtriva Capsules 200mg Truvada combination tablet *HC1721 Stribild combination tablet*HC1722 Genvoya Combination Tablets*HC1724 DESCOVY Combination Tablets LT, HT*HC1723 COMPLERA Combination Tablets*HC1725	Emtriva® Capsules 200mg(Emtriva Capsules 200mg) Truvada® Combination Tab.*HC1721 Stribild® Combination Tab.*HC1722 Genvoya® Combination Tablet*HC1724 DESCOVY*HC1723 COMPLERA® Combination Tablets*HC1725	Emtricitabine(Emtriva Capsules 200mg) Emtricitabine/Tenofovir disoproxil fumarate*HC1721 Elvitegravir/Cobicistat/Emtricitabine/Tenofovir disoproxil fumarate*HC1722 Elvitegravir/Cobicistat/Emtricitabine/Tenofovir Alafenamide Fumarate*HC1724 Emtricitabine/Tenofovir Alafenamide Fumarate*HC1723 Hydrochloride/Emtricitabine/Tenofovir Disoproxil Fumarate*HC1725			Approved	
16	2004	2004/10/13	(16yaku) No. 173		FTY720	Suppression of rejection after renal transplantation	Mitsubishi Pharma Corporation (currently Mitsubishi Tanabe Pharma Corporation) Novartis Pharma K.K.	-	-	-	-	-	-				

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
16	2004	2004/10/13	(16yaku) No. 174	2	Fosamprenavir calcium hydrate	HIV Infection	GlaxoSmithKline K.K.	HIV Infection	ViiV Healthcare K.K.	2004/12/24	Lexiva tablet 700 mg	LexIVa® Tablets 700	Fosamprenavir Calcium Hydrate			Approved	
16	2004	2004/11/5	(16yaku) No. 175	3	NPC-02	Wilson's disease	Nobelpharma Co., Ltd.	Wilson's disease (hepatolenticular degeneration)	Nobelpharma Co., Ltd.	2008/1/25(Nobelzin capsule 25 mg ・50 mg) 2014/9/17(Nobelzin tablet 25 mg ・50	Nobelzin capsule 25 mg ・50 mg Nobelzin tablet 25 mg ・50 mg	NOBELZIN® Capsules 25 mg ・50 mg NOBELZIN® Tablets 25 mg ・	Zinc acetate dihydrate			Approved	
16	2004	2005/1/13 2017/8/9 *57	(17yaku) No. 176		Ibritumomab tiuxetan	CD20-positive B-cell non-Hodgkin's lymphoma	Nihon Schering K.K. Mundipharma K.K. *57	1. Relapsed or refractory CD20-positive disease in low-grade B-cell non-Hodgkin's lymphoma and mantle cell lymphoma (MCL) 2. Confirmation of the accumulation site of ibritumomab tiuxetan (recombinant)	Bayer Holding Ltd.	2008/1/25	1.Zevalin yttrium (^{90}Y) injection 2. Zevalin indium (^{111}In) injection	1. Zevalin® yttrium injection 2. Zevalin® indium injection	1.Ibritumomab Tiuxetan (recombinant) Yttrium Chloride (^{90}Y) 2. Ibritumomab Tiuxetan (recombinant) Indium Chloride (^{111}In)	Designation revoked (2017/08/09) *57		Approved	
16	2004	2005/2/8	(17yaku) No. 177		SOT-107	Glioma	Sosei Co., Ltd.	-	-	-	-	-	-	Designation revoked (2007/08/03)	2007/8/3	Revoked	
16	2004	2005/2/8	(17yaku) No. 178		Thalidomide	Multiple myeloma limited to cases for which existing treatments are not sufficiently effective	Fujimoto Pharmaceutical Corporation	Relapsed or refractory multiple myeloma	Fujimoto Pharmaceutical Corporation	2008/10/16(Thaled capsule 100 mg) 2009/6/17(Thaled capsule 50 mg) 2014/2/6(Thaled capsule 25 mg)	Thaled capsule 50 mg Thaled capsule 100 mg Thaled capsule 25 mg	THALED® CAPSULE 50 THALED® CAPSULE 100 THALED® CAPSULE 25	Thalidomide			Approved	
16	2004	2005/3/24	(17yaku) No. 179	3	Phenobarbital sodium IV solution	Neonatal convulsions	Nobelpharma Co., Ltd.	Neonatal convulsions	Nobelpharma Co., Ltd.	2008/10/16	Nobelbar 250 mg for Injection	NOBELBAR® 250mg for Injection	Phenobarbital Sodium			Approved	
17	2005	2005/6/20	(17yaku) No. 180		Edaravone	Amyotrophic lateral sclerosis (ALS)	Mitsubishi Pharma Corporation (currently Mitsubishi Tanabe Pharma Corporation)	Inhibiton on progression of functional disorder in patients with amyotrophic lateral sclerosis	Mitsubishi Tanabe Pharma Corporation	-	Radicut inj.30 mg Radicut Bag for I.V. Infusion 30 mg	RADICUT®inj.30 mg RADICUT® BAG for I.V. Infusion 30 mg	Edaravone			Approved	
17	2005	2006/2/10	(18yaku) No. 181		Alglucosidase alfa (recombinant)	Glycogen storage disease type II	Genzyme Japan K.K.	Glycogen storage disease type II	Genzyme Japan K.K.	2007/4/18	Myozyme for IV infusion 50 mg	MYOZYME®	Alglucosidase Alfa (recombinant)			Approved	
17	2005	2006/3/10	(18yaku) No. 182		Ranibizumab	Age-related macular degeneration with subfoveal choroidal neovascularization	Novartis Pharma K.K.	Age-related macular degeneration with subfoveal choroidal neovascularization	Novartis Pharma K.K.	2009/1/21(Lucentis solution for intravitreal injection 2.3 mg/0.23 mL) 2014/3/20(Lucentis solution for intravitreal injection 10mg/mL)	Lucentis solution for intravitreal injection 2.3 mg/0.23 mL Lucentis solution for intravitreal injection 10mg/mL	LUCENTIS® solution for intravitreal injection 2.3mg/0.23mL LUCENTIS® solution for intravitreal injection 10mg/mL	Ranibizumab (recombinant)			Approved	
18	2006	2006/5/8	(18yaku) No. 183		Doxorubicin hydrochloride liposome injection	AIDS-related Kaposi sarcoma	Janssen Pharmaceutical K.K.	AIDS-related Kaposi sarcoma	Janssen Pharmaceutical K.K.	2007/1/4	Doxil injection 20 mg	DOXIL® Injection	Doxorubicin Hydrochloride			Approved	
18	2006	2006/6/9	(18yaku) No. 184	1	Precipitated H5N1 influenza vaccine	Prophylaxis of H5N1 influenza	Denka Seiken Co., Ltd.	Prophylaxis of H5N1 influenza	Denka Seiken Co., Ltd.	2013/3/25 2014/3/31 *51	H5N1 precipitated influenza vaccine "SEIKEN"1 mL H5N1 precipitated influenza vaccine "SEIKEN"10 mL *51	-	Adsorbed Influenza Vaccine(H5N1)			Approved	
18	2006	2006/6/9	(18yaku) No. 185	1	Precipitated H5N1 influenza vaccine	Prophylaxis of H5N1 influenza	Kitasato Institute	Prophylaxis of H5N1 influenza	Kitasato Daiichi Sankyo Vaccine Co., Ltd.	2007/10/19	H5N1 precipitated influenza vaccine “Kitasato Daiichi-Sankyo”	-	Adsorbed Influenza Vaccine(H5N1)			Approved	
18	2006	2006/6/9	(18yaku) No. 186	1	Precipitated H5N1 influenza vaccine	Prophylaxis of H5N1 influenza	Research Institute for Microbial Diseases, Osaka University	Prophylaxis of H5N1 influenza	Research Foundation for Microbial Diseases of Osaka University BIKEN	2007/10/19	H5N1 precipitated influenza vaccine "BIKEN"	-	Adsorbed Influenza Vaccine(H5N1)			Approved	
18 30	2006 2018	2006/6/9 2018/7/2*64	(18yaku) No. 187	2	Precipitated H5N1 influenza vaccine	Prophylaxis of H5N1 influenza	Kaketsuken KM Biologics Co., Ltd. *64	Prophylaxis of H5N1 influenza	Kaketsuken KM Biologics Co., Ltd. *64	2010/10/27	H5N1 precipitated influenza vaccine “Kaketsuken”	-	Adsorbed Influenza Vaccine(H5N1)	Designation revoked (2018/07/02)*64		Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
18	2006	2006/6/9 2016/3/7 *22	(18yaku) No. 188	2	Nelarabine	Relapsed or refractory T-cell acute lymphoblastic leukemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) in adult and pediatric patients and adult T-cell leukemia-lymphoma	GlaxoSmithKline K.K., 2006-06-09 Novartis Pharma K.K., 2016-03-07 *22	Relapsed or refractory T-cell acute lymphoblastic leukemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL)	GlaxoSmithKline K.K. Novartis Pharma K.K.	2007/10/19	Arranon G for IV injection 250 mg	Arranon G® Injection	Nelarabine	Designation revoked (2016/03/07) *22		Approved	
18	2006	2006/6/9	(18yaku) No. 189		Anecortave acetate	Age-related macular degeneration with subfoveal choroid neovascularization	Alcon Japan Ltd.	-	-	-	-	-	Anecortave acetate	Designation revoked (2010/03/04)	2010/3/4	Revoked	
18	2006	2006/6/9	(18yaku) No. 190	2	Risedronate sodium hydrate	Paget's disease of bone	Ajinomoto Co. Inc. Takeda Pharmaceutical Co., Ltd.	Paget's disease of bone	Ajinomoto Co. Inc. Takeda Pharmaceutical Co., Ltd.	2008/7/16	Actonel tablet 17.5 mg Benet tablet 17.5 mg	Actonel® Tablet 17.5mg BENET® Tablets 17.5mg.	Risedronate Sodium Hydrate			Approved	
18	2006	2006/6/9	(18yaku) No. 191		Leuprorelin acetate	Spinal and bulbar muscular atrophy	Takeda Pharmaceutical Co., Ltd.	Spinal and Bulbar Muscular Atrophy	Takeda Pharmaceutical Co., Ltd.	2017/8/25	LEUPLIN® SR FOR INJECTION KIT 11.25mg.	LEUPLIN SR FOR INJECTION KIT 11.25mg	Leuprorelin Acetate			Approved	
18	2006	2006/08/11 2010/02/02 *23	(18yaku) No. 192		AMG531	Improvement of thrombocytopenia associated with chronic idiopathic thrombocytopenic purpura	Amgen Development K.K., 2006-08-11 Kyowa Hakko Kirin Co., Ltd., 2010-02-02 *23	Chronic Idiopathic thrombocytopenic purpura	Kyowa Hakko Kirin Co., Ltd.	2011/1/21	Romiplate SC injection 250 μg for preparative purpose	Romiplate® for s.c. injection	Romiplostim (recombinant)	Designation revoked (2010/02/02) *23		Approved	
18	2006	2006/8/11	(18yaku) No. 193	3	Tolvaptan	Inhibition of progression of polycystic kidney disease	Otsuka Pharmaceutical Co., Ltd.	Prevention of the progression of autosomal dominant polycystic kidney disease accompanied by an already enlarged renal volume and a high rate of increase in renal volume	Otsuka Pharmaceutical Co., Ltd.	2014/3/24(Samsca tablets 7.5mg,Samsca tablets 15mg, Samsca tablets 30mg) 2017/3/14	Samsca tablets 7.5mg Samsca tablets 15mg Samsca tablets 30mg SAMSCA Granules 1%	Samsca® tablets 7.5mg Samsca® tablets 15mg Samsca® tablets 30mg SAMSCA	Tolvaptan			Approved	
18	2006	2006/12/14	(18yaku) No. 194		Idursulfase	Mucopolysaccharidosis II	Genzyme Japan K.K.	Mucopolysaccharidosis II	Genzyme Japan K.K.	2007/10/4	Elaprase for IV infusion 6 mg	ELAPRASE®	Idursulfase (recombinant)			Approved	
18	2006	2007/1/25	(19yaku) No. 195		Darunavir ethanolate	HIV Infection in patients previously treated with anti-HIV agents	Janssen Pharmaceutical K.K.	HIV Infection *24	Janssen Pharmaceutical K.K.	2007/11/22(Prezista tablet 300 mg) 2009/08/20(Prezistanaive tablet 400 mg) *24 2013/7/9(Prezistanaive tablet 800 mg) 2014/12/26(Prezista tablet 600 mg) 2016/11/22*HC1951	Prezista tablet 300 mg Prezistanaive tablet 400 mg *24 Prezista tablet 600 mg Prezistanaive tablet 800 mg PREZCOBIX Combination Tablets*HC1951	PREZISTA® Tablets PREZISTANAIVE® Tablets *24 PREZCOBIX® Combination Tablets*HC1951	Darunavir Ethanolate/Cobicistat *HC1951			Approved	
18	2006	2007/2/27	(19yaku) No. 196		Sildenafil citrate	Pulmonary arterial hypertension	Pfizer Japan Inc.	Pulmonary arterial hypertension	Pfizer Japan Inc.	2008/1/25 2019/9/27 *59	Revatio tablet 20 mg Revatio Dry Syrup for Suspension 900mg Revatio OD Film 20mg *59	Revatio® Tablets 20mg Revatio® Dry Syrup for Suspension 900mg Revatio® OD Film 20mg	Sildenafil Citrate			Approved	
18	2006	2007/3/23 2015/10/27 *25	(19yaku) No. 197	3	SB-497115-GR	Improvement of thrombocytopenia in chronic idiopathic thrombocytopenic purpura	GlaxoSmithKline K.K., 2007-03-23 Novartis Pharma K.K., 2015-10-27 *25	Chronic idiopathic thrombocytopenic purpura	GlaxoSmithKline K.K., Novartis Pharma K.K. *25	2010/10/27	Revolade tablet 12.5 mg Revolade tablet 25 mg	Revolade® Tablets 12.5mg Revolade® Tablets 25mg	Eltrombopag Olamine	Designation revoked (2015/10/27) *25		Approved	
18	2006	2007/3/23	(19yaku) No. 198		Nilotinib hydrochloride hydrate	Chronic myelogenous leukemia (CML) with resistance or intolerance to imatinib mesylate, relapsed or refractory Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ALL)	Novartis Pharma K.K.	Chronic phase or accelerated phase chronic myelogenous leukemia	Novartis Pharma K.K.	2009/1/21(Tasigna capsule 200 mg) 2010/12/21(Tasigna capsule 150 mg) 2017/9/4(Tasigna capsule 50 mg) 2017/12/25	Tasigna capsule 150 mg Tasigna capsule 200 mg Tasigna capsule 50 mg	Tasigna® Capsules 150mg Tasigna® Capsules 200mg Tasigna® Capsules 50mg	Nilotinib Hydrochloride Hydrate			Approved	
18	2006	2007/3/23	(19yaku) No. 199		Dasatinib hydrate	Chronic myelogenous leukemia (CML) with resistance or intolerance to imatinib mesylate, relapsed or refractory Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ALL)	Bristol-Myers	Chronic myelogenous leukemia (CML) with resistance to imatinib, relapsed or refractory Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ALL)	Bristol-Myers	2009/1/21	Sprycel tablet 20 mg Sprycel tablet 50 mg	Sprycel® Tablets 20mg Sprycel® Tablets 50mg	Dasatinib Hydrate			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
19	2007	2007/5/16	(19yaku) No. 200	3	Ambrisentan	Pulmonary arterial hypertension	GlaxoSmithKline K.K.	Pulmonary arterial hypertension	GlaxoSmithKline K.K.	2010/7/23	Volibris tablet 2.5 mg	Volibris® Tablets 2.5mg	Ambrisentan			Approved	
19	2007	2007/6/5	(19yaku) No. 201	1	Galsulfase (recombinant)	Mucopolysaccharidosis VI	AnGes , Inc.	Mucopolysaccharidosis VI	AnGes, Inc.	2008/3/28	Naglazyme for IV infusion 5 mg	Naglazyme®	Galsulfase (recombinant)			Approved	
19	2007	2007/9/13	(19yaku) No. 202	1	Sapropterin hydrochloride	Reduction of serum phenylalanine (Phe) levels in hyperphenylalaninemia (HPA) due to tetrahydrobiopterin (BH4)-responsive phenylalanine hydroxylase deficiency (BH4-responsive HPA)	Asubio Pharma Co., Ltd.	Reduction of serum phenylalanine (Phe) levels in hyperphenylalaninemia (HPA) due to tetrahydrobiopterin (BH4)-responsive phenylalanine hydroxylase deficiency (BH4-responsive HPA)	Daiichi Sankyo Company, Limited	2008/7/16(Biopten granule 2.5%) 2013/8/20(Biopten granule 10%)	Biopten granule 2.5% Biopten granule 10%	BIOPTEN® GRANULES 2.5% BIOPTEN® GRANULES 10%	Sapropterin Hydrochloride			Approved	
19	2007	2007/9/13	(19yaku) No. 203		FTY720	Prevention of recurrence and inhibition of progression of multiple sclerosis	Mitsubishi Pharma Corporation Novartis Pharma K.K.	Prevention of relapse and delay of physical disability progression in multiple sclerosis	Mitsubishi Tanabe Pharma Corporation Novartis Pharma K.K.	2011/9/26	Imusera capsule 0.5 mg Gilenya capsule 0.5 mg	IMUSERA® Capsules 0.5mg Gilenya® Capsules 0.5mg	Fingolimod Hydrochloride			Approved	
19	2007	2007/11/26	(19yaku) No. 204		Raltegravir potassium	HIV-1 Infection	Banyu Pharmaceutical Co., Ltd.	HIV Infection	MSD K.K.	2008/6/24(Isentress tablet 400 mg) 2018/5/14(Isentress tablet 600 mg)	Isentress tablet 400 mg Isentress tablet 600 mg	ISENTRESS® Tablets 400mg ISENTRESS® Tablets 600mg	Raltegravir Potassium			Approved	
19	2007	2008/2/18	(20yaku) No. 205	1	OPC-67683	Pulmonary tuberculosis	Otsuka Pharmaceutical Co., Ltd.	<Indicated bacteria> Mycobacterium tuberculosis susceptible to delamanid <Indication> Pulmonary multidrug-resistant tuberculosis (MDR-TB)	Otsuka Pharmaceutical Co., Ltd.	2014/7/4	Deltyba tablets 50mg	DELTYBA® tablets 50mg	Delamanid			Approved	
19	2007	2008/2/18	(20yaku) No. 206		CC-5013 lenalidomide	Relapsed or refractory multiple myeloma limited to previously treated patients	Celgene K.K.	Relapsed or refractory multiple myeloma	Celgene K.K.	2010/6/25(Revlimid capsule 5 mg) 2015/8/20(Revlimid capsule 2.5 mg)	Revlimid capsule 5 mg Revlimid capsule 2.5 mg	Revlimid® Capsules 5mg Revlimid® Capsules 2.5mg	Lenalidomide Hydrate			Approved	
19	2007	2008/2/18	(20yaku) No. 207		CC-5013 lenalidomide	Anemia due to low- or intermediate-1-risk myelodysplastic syndrome with deletion of 5(q31-33) with or without other additional genetic abnormalities	Celgene K.K.	Myelodysplastic syndrome with deletion on the long arm of chromosome 5	Celgene K.K.	2010/8/20(Revlimid capsule 5 mg) 2015/8/20(Revlimid capsule 2.5 mg)	Revlimid capsule 5 mg Revlimid capsule 2.5 mg	Revlimid® Capsules 5mg Revlimid® Capsules 2.5mg	Lenalidomide Hydrate			Approved	
20	2008	2008/5/20	(20yaku) No. 208	1	Natalizumab	Inhibition of progression or prevention of relapse of relapsinging form of multiple sclerosis on monotherapy	Biogen Idec Japan Ltd.	Prevention of relapse and delay of physical disability progression in multiple sclerosis	Biogen Idec Japan Ltd.	2014/3/24	Tysabri for I.V. infusion 300mg	Tysabri® for I.V. Infusion	Natalizumab (Genetical Recombination)			Approved	
20	2008	2008/6/6	(20yaku) No. 209		Infliximab (recombinant)	Ankylosing spondylitis	Mitsubishi Tanabe Pharma Corporation	Ankylosing spondylitis for which existing treatments are not sufficiently effective	Mitsubishi Tanabe Pharma Corporation	2010/4/16	Remicade for IV infusion 100 mg	REMICADE® for IV Infusion100	Infliximab (recombinant)			Approved	
20	2008	2008/6/6	(20yaku) No. 210		Tacrolimus hydrate	Myasthenia gravis (excluding generalized myasthenia gravis when post-thymectomy steroid treatment is not sufficiently effective or it cannot be administered due to adverse drug reactions) *25	Astellas Pharma Inc.	Myasthenia gravis*12	Astellas Pharma Inc.	2009/10/16 *12	Prograf capsule 0.5 mg Prograf capsule 1 mg Prograf granule 0.2 mg Prograf granule 1 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg Prograf® Granules 0.2mg Prograf® Granules 1mg	Tacrolimus Hydrate			Approved	
20	2008	2008/06/06 2011/11/16 *26	(20yaku) No. 211	3	UMN-0501 (influenza HA recombinant vaccine for H5N1) ASP7373 (influenza HA recombinant vaccine for H5N1) *26	Prophylaxis of H5N1 influenza	UMN Pharma Inc., 2008-06-06 Astellas Pharma Inc., 2011-11-16 *26	-	-	-	-	-	-	Designation revoked (2011/11/16) *26 Designation revoked(2017/03/10)	2017/3/10	Revoked	
20	2008	2008/6/6	(20yaku) No. 212	3 *27	Forodesine hydrochloride	Relapsed or refractory cases of peripheral T-cell lymphoma, adult T-cell leukemia/lymphoma, cutaneous T-cell lymphoma, T-cell acute lymphocytic leukemia/T-cell lymphoblastic lymphoma	Mundipharma K.K.	Relapsed or refractory peripheral T-cell lymphoma	Mundipharma K.K.	2017/3/30	Mundesine Capsule 100mg	Mundesine	Forodesine hydrochloride			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
20	2008	2008/8/4	(20yaku) No. 213		Maraviroc	CCR5-tropic HIV-1 infection	Pfizer Japan Inc.	CCR5-tropic HIV-1 Infection	ViiV Healthcare K.K.	2008/12/15	Celsentri tablet 150 mg	Celsentri® Tablets	Maraviroc			Approved	
20	2008	2008/8/4	(20yaku) No. 214		Etravirine	HIV-1 infection	Janssen Pharmaceutical K.K.	HIV-1 infection	Janssen Pharmaceutical K.K.	2008/12/25	Intelence tablet 100 mg	INTELENCE® Tablets	Etravirine			Approved	
20	2008	2008/9/12	(20yaku) No. 215	3	GSK1557484A (pandemic H5N1 influenza virus vaccine with adjuvant added prior to use)	Prophylaxis of H5N1 influenza	GlaxoSmithKline K.K.	-	-	-	-	-	-	Designation revoked (2017/03/10)	2017/3/10	Revoked	
20	2008	2008/09/12 2011/07/11 *28	(20yaku) No. 216		Sodium phenylbutyrate	Urea cycle disorders	Ucyclyd Pharma, Inc., 2008-09-12 CMIC Co., Ltd., 2011-07-11 *28	Urea cycle disorders	Orphan Pacific, Inc.	2012/9/28	Buphenyl tablet 500 mg Buphenyl Granule 94%	Buphenyl® Tablets 500mg Buphenyl® Granules 94%	Sodium Phenylbutyrate	Designation revoked (2011/07/11) *28		Approved	
20	2008	2008/11/17	(20yaku) No. 217		Azacitidine	Myelodysplastic syndrome	Nippon Shinyaku Co., Ltd.	Myelodysplastic syndrome	Nippon Shinyaku Co., Ltd.	2011/1/21	Vidaza for injection 100 mg	Vidaza® for Injection 100mg	Azacitidine			Approved	
20 30	2008 2018	2008/12/11 2018/7/2*65	(20yaku) No. 218		Dried sulfonated human immunoglobulin	Improvement of neuropathy in Churg-Strauss syndrome and allergic granulomatous angiitis (limited to cases for which steroid treatment is not sufficiently effective)	Kaketsuken KM Biologics Co., Ltd. *65 Teijin Pharma Limited	Improvement of neuropathy in Churg-Strauss syndrome and allergic granulomatous angiitis (limited to cases for which steroid treatment is not sufficiently effective)	Kaketsuken KM Biologics Co., Ltd. *65	2010/1/20	Kenketsu Venilon-I for IV injection 500 mg Kenketsu Venilon-I for IV injection 1000 mg Kenketsu Venilon-I for IV injection 2500 mg Kenketsu Venilon-I for IV injection 5000 mg	Kenketsu Venilon®-I Kenketsu Venilon®-I Kenketsu Venilon®-I Kenketsu Venilon®-I	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2018/07/02) *65		Approved	
20	2008	2008/12/15	(20yaku) No. 219		Talaporfin Sodium	Enhancement of light sensitivity in photodynamic therapy for malignant glioma	Meiji Seika Kaisha, Ltd.	-	-	-	-	-	Talaporfin Sodium	Designation revoked (2013/08/12) Designation was transferred to (25yaku) No. 309 with the change in indication from malignant glioma to malignant brain tumor.	2013/8/12	Revoked	
20	2008	2008/12/22	(20yaku) No. 220		Eculizumab	Paroxysmal nocturnal hemoglobinuria	Alexion Pharmaceuticals, Inc.	Inhibition of hemolysis due to paroxysmal nocturnal hemoglobinuria	Alexion Pharmaceuticals, Inc.	2010/4/16	Soliris for IV infusion 300 mg	Soliris®	Eculizumab (recombinant)			Approved	
20	2008	2009/2/9	(21yaku) No. 221	4	MC710 (freeze dried human blood coagulation factor X added to activated blood coagulation factor VII)	Inhibition of bleeding in patients with congenital hemophilia who have inhibitors to blood coagulation factor VIII or IX	Kaketsuken	-	-	-	-	-	-	Designation revoked (2014/05/13) Designation was transferred to (26yaku) No. 337 with the change in indication from Inhibition of bleeding in patients with hemophilia who have inhibitors to blood coagulation factor VIII or IX	2014/5/13	Revoked	
20	2008	2009/2/9	(21yaku) No. 222	3	SUN11031	Increase in the amount of food intake in anorexia nervosa (restricting type) or eating disorder not otherwise specified (insufficient food intake, low body weight, and no binging or purging)	Asubio Pharmaceuticals, Inc. (currently Daiichi Pharmaceutical Co., Ltd.)	-	-	-	-	-	-	Designation revoked (2012/05/11)	2012/5/11	Revoked	
20	2008	2009/3/10 2014/10/16 *29	(21yaku) No. 223	4	Glatiramer acetate	Reduction of recurrence frequency in relapsing-remitting multiple sclerosis (MS)	Teva Pharmaceutical K.K.,2009-03-10 Takeda Pharmaceutical Co., Ltd., 2014-10-16 *29	Prevention of relapse of multiple sclerosis	Takeda Pharmaceutical Co., Ltd.	2015/9/28	COPAXONE S.C. Injection 20mg Syringe	COPAXONE	Glatiramer Acetate	Designation revoked (2014/10/16) *29		Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
21	2009	2009/5/12	(21yaku) No. 224		Levodopa-carbidopa formulation for duodenal administration	(1) Parkinson's disease (PD) patients (Hoehn & Yahr Severity Stages IV & V) who suffer from severe motor fluctuations (wearing-off, no-on/delayed on phenomena, on-off phenomena and dyskinesia) despite treatment with currently available conventional oral pharmacotherapy (2) PD patients (Hoehn & Yahr Severity Stages I to III) who have difficulty to receive oral pharmacotherapy and have already undergone PEG procedure because of severe dysphagia or other reasons	Solvay Pharmaceuticals, Inc. (currently AbbVie GK)	Improvement of daily fluctuation (wearing-off phenomenon) of the symptoms of Parkinson's disease in patients who have had an inadequate response to conventional drug therapy including levodopa-containing preparations	AbbVie GK	2016/7/4	Duodopa enteral combination solution	Duodopa	Levodopa/Carbidopa Hydrate			Approved	
21	2009	2009/6/5	(21yaku) No. 225	3	Preparation for implanting carmustine in the brain	FIP1L1-PDGFR α-positive hypereosinophilic syndrome and chronic eosinophilic leukemia	Eisai Co., Ltd.	Malignant glioma	Nobelpharma Co., Ltd.	2012/9/28	Gliadel intracerebral implant 7.7 mg	Gliadel® 7.7mg Implant	Carmustine			Approved	
21	2009	2009/9/11	(21yaku) No. 226		Polyethylene glycol-treated human immunoglobulin	Generalized myasthenia gravis when post-thymectomy treatment with steroidal or non-steroidal immunosuppressive agents is not sufficiently effective	Benesis Corporation	Generalized myasthenia gravis when post-thymectomy treatment with steroidal or non-steroidal immunosuppressive agents is not sufficiently effective	Japan Blood Products Organization	2011/9/26(Venoglobulin IH 5% IV injection 0.5 g/10 mL, 1 g/20 mL, 2.5 g/50 mL, 5 g/100 mL) 2013/2/15(Venoglobulin IH 5% IV injection 10 g/200 mL)	Venoglobulin IH 5% IV injection 0.5 g/10 mL Venoglobulin IH 5% IV injection 1 g/20 mL Venoglobulin IH 5% IV injection 2.5 g/50 mL Venoglobulin IH 5% IV injection 5 g/100 mL Venoglobulin IH 5% IV injection 10 g/200 mL	Venoglobulin® IH5%IV0.5g/10mL Venoglobulin® IH5%IV1g/20mL Venoglobulin® IH5%IV2.5g/50mL Venoglobulin® IH5%IV5g/100mL Venoglobulin® IH5%IV10g/200mL	Polyethylene Glycol-treated Normal Human Immunoglobulin			Approved	
21	2009	2009/10/28	(21yaku) No. 227		Bendamustine hydrochloride	Relapsed or refractory cases of low-grade B-cell non-Hodgkin's lymphoma and mantle cell lymphoma (MCL)	SymBio Pharmaceuticals Limited	Relapsed or refractory cases of low-grade B-cell non-Hodgkin's lymphoma and mantle cell lymphoma (MCL)	SymBio Pharmaceuticals Limited	2010/10/27(Treakisym for IV infusion 100 mg) 2016/9/28(Treakisym for IV infusion 25 mg) 2016/12/19	Treakisym for IV infusion 100 mg Treakisym for IV infusion 25 mg	TREAKISYM® Injection 100mg TREAKISYM® Injection 25mg	Bendamustine Hydrochloride			Approved	
22	2010	2010/6/16	(22yaku) No. 228		Vorinostat	Cutaneous T-cell lymphoma	Banyu Pharmaceutical Co., Ltd.	Cutaneous T-cell lymphoma	MSD K.K.	2011/7/1	Zolinza capsule 100 mg	Zolinza® Capsules 100mg	Vorinostat			Approved	
22	2010	2010/06/16 2011/06/10 *30	(22yaku) No. 229		BLB-750 (H5N1 cell culture influenza vaccine)	Prophylaxis of H5N1 influenza	Baxter, 2010-06-16 Takeda Pharmaceutical Co., Ltd., 2011-06-10 *30	Prophylaxis of H5N1 influenza	Baxter Takeda Pharmaceutical Co., Ltd. *30	2013/6/26 2014/3/31 *30	H5N1 cell culture influenza vaccine "Baxter" H5N1 cell culture influenza vaccine "Takeda"5mL H5N1 cell culture influenza vaccine "Takeda"1mL *30	Cell Cultured Influenza vaccine H5N1"TAKEDA" 1mL	Cell culture influenza vaccine (strain H5N1)	Designation revoked (2017/03/10) "Baxter" *30	Designation revoked (2017/03/10) "Baxter" *30	Approved "Takeda" Revoked "Baxter"	
22	2010	2010/6/16	(22yaku) No. 230	3, 1 *31	Midismase (recombinant) *31	Idiopathic pulmonary fibrosis *31	LTT Bio-Pharma Co., Ltd.	-	-	-	-	-	Midismase (recombinant)				
22	2010	2010/8/11	(22yaku) No. 231		Canakinumab	Cryopyrin-associated periodic syndrome in patients ≥2 years of age: familial cold autoinflammatory syndrome, Muckle-Wells syndrome, neonatal onset multi-organ inflammatory disease	Novartis Pharma K.K.	Cryopyrin-associated periodic syndrome in patients ≥2 years of age: familial cold autoinflammatory syndrome, Muckle-Wells syndrome, neonatal onset multi-organ inflammatory disease	Novartis Pharma K.K.	2011/9/26(Ilaris for s.c. injection 150 mg) 2018/2/9(Ilaris solution for s.c. injection 150 mg)	Ilaris for s.c. injection 150 mg Ilaris solution for s.c. injection 150 mg	Ilaris® for s.c. injection 150mg Ilaris® solution for s.c. injection 150mg	Canakinumab (recombinant)			Approved	
22	2010	2010/8/11	(22yaku) No. 232	1, 3 *32	KW-0761	CCR4-positive adult T-cell leukemia/lymphoma *32	Kyowa Hakko Kirin Co., Ltd.	CCR4-positive adult T-cell leukemia/lymphoma *32	Kyowa Hakko Kirin Co., Ltd.	2012/03/30 2014/12/18 *32	Poteligeo for IV infusion 20 mg	POTELIGEO® Injection	Mogamulizumab (recombinant)			Approved	
22	2010	2010/9/14 2012/7/4 *33	(22yaku) No. 233	3	5-Aminolevulinic acid hydrochloride	Visualization of tumor tissue during surgical resection of malignant glioma	Nobelpharma Co., Ltd., 2010-09-14 SBI Pharmaceuticals Co., Ltd., 2012-07-04 *33	Visualization of tumor tissue during surgical resection of malignant glioma	Nobelpharma Co., Ltd. SBI Pharmaceuticals Co., Ltd. *33	2013/3/25	Alabel oral 1.5 g Alaglio oral 1.5 g	Alabel® Oral 1.5g	Aminolevulinic acid hydrochloride	Designation revoked (2010/09/14) *33		Approved	
22	2010	2010/11/10	(22yaku) No. 234		Bortezomib	Onset of multiple myeloma	Janssen Pharmaceutical K.K.	Multiple myeloma *19	Janssen Pharmaceutical K.K.	2011/9/16 *19	Velcade for injection 3 mg	VELCADE® Injection	Bortezomib			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
22	2010	2010/11/10	(22yaku) No. 235		Bortezomib	Mantle cell lymphoma	Janssen Pharmaceutical K.K.	Mantle cell lymphoma	Janssen Pharmaceutical K.K.	2015/6/26	Velcade for injection 3 mg	VELCADE® Injection	Bortezomib			Approved	
22	2010	2010/11/10	(22yaku) No. 236	1	Colistin sodium methanesulfonate	Indicated bacterial strains: Multidrug-resistant Pseudomonas aeruginosa (MDRP), multidrug-resistant Acinetobacter and other multidrug-resistant Gram-negative bacteria that are sensitive to this drug Indications: Various infectious diseases	GlaxoSmithKline K.K.	[Indicated bacteria] Colistin-susceptible bacteria; Escherichia coli, Citrobacter species, Klebsiella species, Enterobacter species, P. aeruginosa, Acinetobacter spp., In this regard, the bacteria that showed resistance for another antibacterial drugs [Indications] Infection diseases of varied type	GlaxoSmithKline K.K.	2015/3/26	Aldreb for Intravenous Injection 150mg	Aldreb® for Intravenous Injection 150mg	Colistin sodium methanesulfonate			Approved	
22	2010	2006/11/10 2014/12/4 2015/11/06*34	(22yaku) No. 237	3*35	GSK2402968 Drisapersen Sodium *34	Duchenne muscular dystrophy	GlaxoSmithKline K.K., 2006-11-10 Prosensa holding N.V, 2014-12-04 BioMarin Pharmaceutical Japan, 2015-11-06*34	-	-	-	-	-	-	Designation revoked (2014/12/04) Designation revoked (2015/11/06)*34 Designation revorked(2018/5/2	2018/5/24	Revorked	
22	2010	2011/1/28	(22yaku) No. 238		Crizotinib	*ALK* fusion gene-positive advanced non-small cell lung cancer	Pfizer Japan Inc.	*ALK* fusion gene positive unresectable advanced and/or recurrent non-small cell lung cancer	Pfizer Japan Inc.	2012/3/30	Xalkori capsule 200 mg Xalkori capsule 250 mg	XALKORI® Capsules 200mg XALKORI® Capsules 250mg	Crizotinib			Approved	
22	2010	2011/3/9	(23yaku) No. 239		Stiripentol	Combination therapy with clobazam and sodium valproate to assist infants with clonic or tonic-clonic seizures in severe myoclonic epilepsy (Dravet syndrome) when these seizures are not sufficiently controlled by clobazam and sodium valproate.	Meiji Seika Kaisha, Ltd.	Used in combination with clobazam and sodium valproate for tonic-clonic seizures or clonic seizure syndrome, for which clobazam and sodium valproate are not sufficiently effective, in patients with Dravet syndrome.	Meiji Seika Pharma Co., Ltd.	2012/9/28	Diacomit dry syrup 250 mg Diacomit dry syrup 500 mg Diacomit capsule 250 mg	DIACOMIT DRYSYRUP250mg DIACOMIT DRYSYRUP500mg DIACOMIT CAPSULES250mg	Stiripentol			Approved	
22	2010	2011/3/9	(23yaku) No. 240		Apomorphine hydrochloride hydrate	Rescue therapy for diurnal variation in symptoms of Parkinson's disease when usual drug therapy is not sufficiently effective.	Kyowa Hakko Kirin Co., Ltd.	Improvement of “off” symptoms in Parkinson's disease (when frequent administration of levodopa-containing preparations or increasing the dose of other antiparkinsonian agents is not sufficiently effective)	Kyowa Hakko Kirin Co., Ltd.	2012/3/30	Apokyn SC injection 30 mg	Apokyn® subcutaneous injection	Apomorphine Hydrochloride Hydrate			Approved	
22	2010	2011/3/9	(23yaku) No. 241		Genz-112638	Type 1 Gaucher disease	Genzyme Japan K.K.	Improvement of various symptoms of Gaucher disease (anemia, thrombocytopenia, hepatosplenomegaly,	Genzyme Japan K.K.	2015/3/26	Cerdelga capsule 100 mg	CERDELGA® capsule	Eliglustat tartrate			Approved	
22	2010	2011/3/9	(23yaku) No. 242		Miglustat	Niemann-Pick disease type C	Actelion Pharmaceuticals Japan Ltd.	Niemann-Pick disease type C	Actelion Pharmaceuticals Japan Ltd.	2012/3/30	Brazaves capsule 100 mg	BRAZAVES® 100 mg	Miglustat			Approved	
23	2011	2011/5/13 2013/4/4 *36	(23yaku) No. 243		Velaglucerase alfa	Improvement of various symptoms of Gaucher disease (anemia, thrombocytopenia, hepatosplenomegaly, and bone manifestations)	Shire Human Genetic Therapies, Inc., 2011-05-13 Shire Japan KK, 2013-04-04 *36	Improvement of various symptoms of Gaucher disease (anemia, thrombocytopenia, hepatosplenomegaly, and bone manifestations)	Shire Japan KK	2014/7/4	VPRIV Injection 400 U	VPRIV® Injection 400 U	Velaglucerase Alfa (Genetical Recombination)	Designation revoked (2013/04/04) *36		Approved	
23	2011	2011/6/10	(23yaku) No. 244		Dornase alfa (recombinant)	Improvement of lung function in cystic fibrosis	Chugai Pharmaceutical Co., Ltd.	Improvement of lung function in cystic fibrosis	Chugai Pharmaceutical Co., Ltd.	2012/3/30	Pulmozyme inhalation liquid 2.5 mg	PULMOZYME® Inhalation Solution 2.5mg	Dornase Alfa (recombinant)			Approved	
23	2011	2011/6/10	(23yaku) No. 245	3	Trabectedin	Malignant soft tissue tumors with chromosomal translocation	Taiho Pharmaceutical Co., Ltd.	Malignant soft tissue tumors	Taiho Pharmaceutical Co., Ltd.	2015/9/28	Yondelis I.V. 0.25mg Yondelis I.V. 1mg	Yondelis	Trabectedin			Approved	
23	2011	2011/6/10	(23yaku) No. 246		Sunitinib malate	Incurable unresectable pancreatic endocrine tumor	Pfizer Japan Inc.	Pancreatic neuroendocrine tumor	Pfizer Japan Inc.	2012/8/10	Sutent capsule 12.5 mg	SUTENT® Capsule	Sunitinib Malate			Approved	
23	2011	2011/6/10	(23yaku) No. 247	1	Rufinamide	Combination therapy with antiepileptic drugs for tonic and atonic seizures in Lennox-Gastaut syndrome (age 4 or over)	Eisai Co., Ltd.	Combination therapy with antiepileptic drugs (AEDs) for tonic and atonic seizures in Lennox-Gastaut syndrome for which other AEDs are not sufficiently effective	Eisai Co., Ltd.	2013/3/25	Inovelon tablet 100 mg Inovelon tablet 200 mg	Inovelon®	Rufinamide			Approved	
23	2011	2011/8/8	(23yaku) No. 248	3	Caffeine citrate	Primary apnea in premature and low birth weight infants (apnea of prematurity)	Nobelpharma Co., Ltd.	Primary apnea in premature and low birth weight infants (apnea of prematurity)	Nobelpharma Co., Ltd.	2014/3/24	Respia injection or oral solution 60mg	Respia®	Anhydrous Caffeine			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
23	2011	2011/9/8	(23yaku) No. 249		Ruxolitinib	Myelofibrosis	Novartis Pharma K.K.	Myelofibrosis	Novartis Pharma K.K.	2014/7/4(Jakavi tablets 5mg) 2017/3/7(Jakavi tablets 10mg)	Jakavi tablets 5mg Jakavi tablets 10mg	JAKAVI® Tablets 5mg JAKAVI® Tablets 10mg	Ruxolitinib Phosphate			Approved	
23	2011	2011/9/8 2015/10/27 *37	(23yaku) No. 250	2 *38	Ofatumumab (recombinant)	Chronic lymphocytic leukemia	GlaxoSmithKline K.K., 2011-09-08 Novartis Pharma K.K., 2015-10-27 *37	Relapsed or refractory CD20-positive chronic lymphocytic leukemia*38	GlaxoSmithKline K.K. Novartis Pharma K.K. *37	2013/3/25 *38	Arzerra for IV infusion 100 mg Arzerra for IV infusion 1000 mg	Arzerra®	Ofatumumab (recombinant)	Designation revoked (2015/10/27) *37		Approved	
23	2011	2011/9/8	(23yaku) No. 251	1	Tetrabenazine	Chorea associated with Huntington's disease	Alfresa Pharma Corporation	Chorea associated with Huntington's disease	Alfresa Pharma Corporation	2012/12/25	Choreazine tablet 12.5 mg	CHOREAZINE® Tablets 12.5mg	Tetrabenazine			Approved	
23	2011	2011/9/8	(23yaku) No. 252		Riociguat	Chronic thromboembolic pulmonary hypertension	Bayer Holding Ltd.	Unresectable or postoperative residual/recurrence Chronic thromboembolic pulmonary hypertension	Bayer Holding Ltd.	2014/1/17	Adempas tablet 0.5mg Adempas tablet 1.0mg Adempas tablet 2.5mg	Adempas®	Riociguat			Approved	
23	2011	2011/9/8	(23yaku) No. 253		Hemin	Acute porphyria attacks	CMIC Co., Ltd.	Symptom relief during acute porphyria attacks	OrphanPacific, Inc.	2013/3/25	Normosang for IV infusion 250 mg	Normosang®	Hemin			Approved	
23	2011	2011/9/8	(23yaku) No. 254		BIBF 1120	Idiopathic pulmonary fibrosis	Nippon Boehringer Ingelheim Co., Ltd.	Idiopathic pulmonary fibrosis	Nippon Boehringer Ingelheim Co., Ltd.	2015/7/3	Ofev capsules 100 mg Ofev capsules 150 mg	Ofev® Capsules 100 mg Ofev® Capsules 150 mg	Nintedanib ethanesulfonate			Approved	
23	2011	2011/11/16	(23yaku) No. 255		Rilpivirine hydrochloride	HIV-1 infection	Janssen Pharmaceutical K.K.	HIV-1 infection	Janssen Pharmaceutical K.K.	2012/5/18(Edurant tablet 25 mg) 2014/11/18(COMPLERA Combination Tablets)*HC2551	Edurant tablet 25 mg COMPLERA Combination Tablets*HC2551	EDURANT® Tablets 25mg COMPLERA® Combination Tablets*HC2551	Rilpivirine Hydrochloride Hydrochloride/Emtricitabine/Tenofovir Disoproxil Fumarate*HC2551			Approved	
23	2011	2011/11/16	(23yaku) No. 256	2	Streptozocin	Pancreatic and gastrointestinal neuroendocrine tumors	Nobelpharma Co., Ltd.	Pancreatic and gastrointestinal neuroendocrine tumors	Nobelpharma Co., Ltd.	2014/9/26	Zanosar IV infusion 1g	ZANOSAR ®	Streptozocin			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
23	2011	2011/11/16 2015/10/27 *39	(23yaku) No. 257		Pazopanib hydrochloride	Progressive malignant soft tissue tumors	GlaxoSmithKline K.K., 2011-11-16 Novartis Pharma K.K., 2015-10-27 *39	Malignant soft tissue tumors	GlaxoSmithKline K.K	2012/9/28	Votrient tablet 200 mg	Votrient® Tablets 200mg	Pazopanib Hydrochloride	Designation revoked (2015/10/27) *39		Approved	
23	2011	2011/12/14	(23yaku) No. 258		Everolimus	Tuberous sclerosis	Novartis Pharma K.K.	Renal angiomyolipoma associated with tuberous sclerosis (only for tablet preparations) Subependymal giant cell astrocytoma associated with tuberous sclerosis	Novartis Pharma K.K.	2012/11/21 (tablet) 2012/12/25 (dispersible tablet)	Afinitor tablet 2.5 mg Afinitor tablet 5 mg Afinitor dispersible tablet 2 mg Afinitor dispersible tablet 3 mg	AFINITOR® tablets dispersible® tablets	Everolimus			Approved	
23	2011	2011/12/14	(23yaku) No. 259		Tafamidis meglumine	Transthyretin amyloid polyneuropathy (familial amyloid polyneuropathy)	Pfizer Japan Inc.	Delay of peripheral neurologic impairment in patients with transthyretin familial amyloid polyneuropathy	Pfizer Japan Inc.	2013/9/20	Vyndaqel capsule 20 mg	Vyndaqel	Tafamidis Meglumine			Approved	
23	2011	2011/12/14	(23yaku) No. 260		Thalidomide	Erythema nodosum leprosum	Fujimoto Pharmaceutical Corporation	Erythema nodosum leprosum	Fujimoto Pharmaceutical Corporation	2008/10/16(Thaled capsule 100 mg) 2009/6/17(Thaled capsule 50 mg) 2012/5/25(Thaled capsule 50 mg,100 mg:approval for partial changes to approved matter for Indications) 2014/2/6(Thaled capsule 25 mg)	Thaled capsule 50 mg Thaled capsule 100 mg Thaled capsule 25 mg	THALED®CAPSULE 50 THALED®CAPSULE 100 THALED®CAPSULE 25	Thalidomide			Approved	
23	2011	2011/12/14	(23yaku) No. 261		Imatinib mesylate	FIP1L1-PDGFR α-positive hypereosinophilic syndrome and chronic eosinophilic leukemia	Novartis Pharma K.K.	FIP1L1-PDGFR α-positive hypereosinophilic syndrome and chronic eosinophilic leukemia	Novartis Pharma K.K.	2012/2/22	Glivec tablet 100mg	Glivec® Tablets 100mg	Imatinib Mesylate			Approved	
23	2011	2012/2/15	(24yaku) No. 262		Pasireotide pamoate	Cushing's disease	Novartis Pharma K.K.	Cushing’s disease (irresponsive to or unfit for surgery)	Novartis Pharma K.K.	2018/3/23	Signifor® LAR® Intramuscular Injection Kit 10 mg Signifor® LAR® Intramuscular Injection Kit 20 mg, Signifor® LAR® Intramuscular Injection Kit 30 mg, Signifor® LAR® Intramuscular Injection Kit 40 mg	Signifor	Pasireotide pamoate			Approved	
23	2011	2012/3/19	(24yaku) No. 263		Imatinib mesylate	Pulmonary arterial hypertension	Novartis Pharma K.K.	-	-	-	-	-	Imatinib Mesylate				

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
23	2011	2012/3/19	(24yaku) No. 264	1	Betaine anhydrous	Adjunctive therapy for homocystinuria with deficiencies or abnormalities in cystathione β synthase (CBS), 5,10-methylenetetrahydrofolate reductase (MTHFR), or cobalamin (cbl) coenzyme metabolism	ReqMed Company, Ltd.	homocystinuria	ReqMed Company, Ltd.	2014/1/17	Cystadane powder	Cystadane® Powder	Betaine			Approved	
23	2011	2012/3/19	(24yaku) No. 265		Z-521	Hypophosphatemia with rickets or osteomalacia	Zeria Pharmaceutical Co., Ltd.	Hypophosphatemia	Zeria Pharmaceutical Co., Ltd.	2012/12/25	Phosribbon combination granule	Phosribbon® Combination Granules	Monobasic sodium phosphate monohydrate			Approved	
23	2011	2012/3/19	(24yaku) No. 266		Clofarabine	Relapsed or refractory acute lymphocytic leukemia	Genzyme Japan K.K.	Relapsed or refractory acute lymphocytic leukemia	Sanofi K.K.	2013/3/25	Evoltra for IV infusion 20 mg	EVOLTRA®	Clofarabine			Approved	
23	2011	2012/3/19	(24yaku) No. 267		Brentuximab vedotin	CD30-positive Hodgkin's lymphoma and anaplastic large cell lymphoma	Takeda Pharmaceutical Co., Ltd. Takeda Bio Development Center Limited	CD30-positive Hodgkin's lymphoma and anaplastic large cell lymphoma	Takeda Pharmaceutical Co., Ltd.	2014/1/17	Adetris for IV infusion 50mg	ADCETRIS®	Brentuximab Vedotin (recombinant)			Approved	
23	2011	2012/3/19 2016/4/14	(24yaku) No. 268		Recombinant von Willebrand factor (Rvwf)	Reduction of bleeding tendency in patients with von Willebrand disease	Baxter (currently Baxalta) Shire Japan K.K. , 2018-03-22	-	-	-	-	-	-	Designation revorked(2016/4/14)			
23	2011	2012/3/19 2016/4/14	(24yaku) No. 269		Rurioctocog alfa (recombinant)	Reduction of bleeding tendency in patients with von Willebrand disease with decreased plasma concentration of blood coagulation factor VIII through plasma supplementation with blood coagulation factor VIII	Baxter (currently Baxalta) Shire Japan K.K. , 2018-03-22	-	-	-	-	-	Rurioctocog alfa (recombinant)	Designation revorked(2016/4/14)			
24	2012	2012/5/11	(24yaku) No. 270	2	Interferon gamma-1a (recombinant)	Mycosis fungoides (not during visceral dissemination stage) and Sézary syndrome	Shionogi & Co., Ltd.	Mycosis fungoides, Sézary syndrome	Shionogi & Co., Ltd.	2014/5/23	Imunomax-γ for Injection 50 Imunomax-γ for Injection 100	Imunomax®	Interferon Gamma-1a (Genetical Recombinationt)			Approved	
24	2012	2012/5/11	(24yaku) No. 271	2	MPR-1020	Nephropathic cystinosis	Mylan Seiyaku Ltd.	Nephropathic cystinosis	Mylan Seiyaku Ltd.	2014/7/4	Nicystagon capsule 50mg Nicystagon capsule 100mg	Nicystagon® Capsules	Cysteamine Bitartrate			Approved	
24	2012	2012/5/11	(24yaku) No. 272	3	Eprodisate disodium	AA amyloidosis	C. T. Development Swiss Corp. (currently A. T. Development Swiss Corp.)	-	-	-	-	-	-	Designation revorked(2017/3/1)	2017/3/1	Revorked	
24	2012	2012/6/13	(24yaku) No. 273		Bendamustine hydrochloride	Chronic lymphocytic leukemia	SymBio Pharmaceuticals Limited	Chronic lymphocytic leukemia	SymBio Pharmaceuticals Limited	1016/8/26 1016/9/28 *52	Treakisym for IV infusion 100 mg Treakisym for IV infusion 25 mg	TREAKISYM® Injection 100mg TREAKISYM® Injection 25mg	Bendamustine Hydrochloride			Approved	
24 30	2012 2018	2012/6/13 2018/7/2*66	(24yaku) No. 274		Type A influenza HA vaccine emulsion cell culture (H5N1 strain)	Prophylaxis of H5N1 influenza	Kaketsuken KM Biologics Co., Ltd.*66	Prophylaxis of H5N1 influenza	Kaketsuken KM Biologics Co., Ltd.*66	2014.3.24 2018.3.23	-	-	Type A influenza HA vaccine emulsion cell culture H5N1	Designation revorked(2018/7/2)*66		Approved	
24 30	2012 2018	2012/6/13 2018/7/2*67	(24yaku) No. 275		Type A influenza HA vaccine emulsion cell culture (prototype vaccine)	Prophylaxis for new strains of influenza	Kaketsuken KM Biologics Co., Ltd.*67	Prophylaxis for pandemic influenza	Kaketsuken KM Biologics Co., Ltd.*67	2015.3.26 2018.3.23	-	-	Type A influenza HA vaccine emulsion cell culture (prototype vaccine)	Designation revorked(2018/7/2)*67		Approved	
24	2012	2012/6/13 2017/4/14 *53	(24yaku) No. 276		Miglustat Hydrochloride	Fabry's disease	GlaxoSmithKline K.K., 2012-06-13 Amicus Therapeutics Co., Ltd., 2017-04-14 *53	Patients with Fabry disease who have a *GLA* mutation amenable to migalastat	Amicus Therapeutics Co., Ltd.	2018.3.23	Galafold Capsules 123mg	Galafold	Migalastat Hydrochloride	Designation revorked(2017/4/14)*53		Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
24	2012	2012/6/13	(24yaku) No. 277		Metreleptin	Treatment of diabetes or dyslipidemia due to lipoatrophy	Shionogi & Co., Ltd.	Lipoatrophy	Shionogi & Co., Ltd.	2013/3/25	Metreleptin for SC injection "Shionogi" 11.25 mg	Metreleptin	Metreleptin (recombinant)			Approved	
24	2012	2012/8/16	(24yaku) No. 278	2	Ecallantide	Acute attacks of hereditary angioedema	CMIC Holdings Co., Ltd.	-	-	-	-	-	Ecallantide (recombinant)				
24	2012	2012/8/16	(24yaku) No. 279	3	Lenvatinib mesylate	Thyroid cancer	Eisai Co., Ltd.	Unresectable thyroid cancer	Eisai Co., Ltd.	2015/3/26	Lenvima® Capsule 4 mg, Lenvima® Capsule 10 mg	Lenvima&reg	Lenvatinib mesylate			Approved	
24	2012	2012/8/16	(24yaku) No. 280		Alemtuzumab (recombinant)	Chronic lymphocytic leukemia	Sanofi-aventis K.K.	Relapsed or refractory chronic lymphocytic leukemia	Sanofi K.K.	2014/9/26	MabCampath 30 mg I.V.Infusion	MabCampath®	Alemtuzumab (recombinant)			Approved	
24	2012	2012/8/16 2015/10/29 *40	(24yaku) No. 281		SBC-102	Lysosomal acid lipase deficiency	Synageva BioPharma Corp., 2012-08-16 Alexion Pharma GK, 2015-10-29 *40	-	Alexion Pharma GK	2016/3/28	Kanuma™ intravenous infusion 20mg	-	Sebelipase Alfa(Genetical Recombination)	Designation revoked (2015/10/29) *40		Approved	
24	2012	2012/9/13	(24yaku) No. 282		Rituximab (recombinant)	Refractory nephrotic syndrome	Zenyaku Kogyo Co., Ltd.	Complicated nephrotic syndrome (frequently relapsing and steroid-dependent)	Zenyaku Kogyo Co., Ltd.	2014/8/29 2018/02/02(new name of product approved for manufacturing and marketing)	Rituxan injection 10 mg/mL (new name of product approved for manufacturing and marketing:2018.2.2 approved) Rituxan Intravenous Infusion 100mg Rituxan Intravenous Infusion 500mg	Rituxan® Injection	Rituximab (recombinant)			Approved	
24	2012	2012/9/13	(24yaku) No. 283		Bevacizumab (recombinant)	Glioblastoma	Chugai Pharmaceutical Co., Ltd	-	-	-	-	-	Bevacizumab (recombinant)	Designation revoked (2013/05/13)	2013/5/13	Revoked	
24	2012	2012/9/13	(24yaku) No. 284		Infliximab (recombinant)	Refractory Kawasaki disease	Mitsubishi Tanabe Pharma Corporation	Acute Kawasaki disease	Mitsubishi Tanabe Pharma Corporation	2015/12/21	Remicade for IV infusion 100 mg	Remicade® for IV infusion 100 mg	Infliximab (recombinant)			Approved	
24	2012	2012/9/13	(24yaku) No. 285		Infliximab (recombinant)	Intestinal, neuro-, and vasculo Behcet syndrome	Mitsubishi Tanabe Pharma Corporation	Entero-Behcet's disease , neuro- Behcet's disease, and vasculo- Behcet's disease in cases where existing treatment is inadequate	Mitsubishi Tanabe Pharma Corporation	2015/8/24	Remicade for IV infusion 100 mg	Remicade® for IV infusion 100 mg	Infliximab (recombinant)			Approved	
24	2012	2012/9/13	(24yaku) No. 286	2	Sirolimus	Lymphangioleiomyomatosis (LAM)	Nobelpharma Co., Ltd.	Lymphangioleiomyomatosis	Nobelpharma Co., Ltd.	2014/7/4	Rapa l imus tablets 1mg	Rapa l imus® tablets 1mg	Sirolimus			Approved	
24	2012	2012/9/13	(24yaku) No. 287		Vemurafenib	BRAFV600 mutation-positive malignant melanoma	Chugai Pharmaceutical Co., Ltd	BRAFmutation-positive malignant melanoma	Chugai Pharmaceutical Co., Ltd	2014/12/26	Zelboraf tablet 240 mg	Zelboraf®	Vemurafenib			Approved	
24	2012	2012/9/13	(24yaku) No. 288		Tacrolimus hydrate	Interstitial pneumonia associated with polymyositis or dermatomyositis	Astellas Pharma Inc.	Interstitial pneumonia associated with polymyositis or dermatomyositis	Astellas Pharma Inc.	2013/6/14	Prograf capsule 0.5 mg Prograf capsule 1 mg	Prograf® Capsules 0.5mg Prograf® Capsules 1mg	Tacrolimus Hydrate			Approved	
24	2012	2012/9/13	(24yaku) No. 289		Cell culture-derived whole virion prototype vaccine	Prophylaxis for pandemic influenza	Baxter Takeda Pharmaceutical Co., Ltd.	Prophylaxis for pandemic influenza	Baxter Takeda Pharmaceutical Co., Ltd.	2013/4/26 2014/3/31 *54	Cell culture influenza vaccine (prototype vaccine) "Baxter" Cell culture influenza vaccine (prototype vaccine) "Takeda" 5 mL Cell culture influenza vaccine (prototype vaccine) "Takeda" 1 mL *54	Cell Cultured Influenza vaccine (Prototype) “TAKEDA” 1mL	Cell culture influenza vaccine (prototype vaccine)	Designation revoked (2017/03/10) "Baxter" *54	Designation revoked (2017/03/10) "Baxter" *54	Approved "Takeda" Revoked "Baxter"	
24	2012	2012/11/14	(24yaku) No. 290		Elvitegravir	HIV infection	Japan Tobacco, Inc.	HIV-1 infection	Japan Tobacco, Inc.	2013/3/25*HC2901 2016/6/17*HC2902	Stribild combination tablet *HC2901 Genvoya Combination Tablets*HC2902	Stribild® Combination Tab.*HC2901 Genvoya® Combination Tablet*HC2902	Elvitegravir/Cobicistat /Emtricitabine/Tenofovir disoproxil fumarate*HC2901 Elvitegravir/Cobicistat /Emtricitabine/Tenofovir Alafenamide Fumarate*HC2902			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
24	2012	2012/11/14	(24yaku) No. 291		Cobicistat	Pharmacokinetic enhancement of anti-HIV agents	Japan Tobacco, Inc.	HIV-1 infection	Japan Tobacco, Inc.*HC2911*HC2913 Janssen Pharmaceutical K.K.*HC2912	2013/3/25*HC2911 2016/6/17*HC2913 2016/11/22*HC2912	Stribild combination tablet *HC2911 Genvoya Combination Tablets*HC2913 PREZCOBIX Combination Tablets*HC2912	Stribild® Combination Tab.*HC2911 Genvoya® Combination Tablet*HC2913 PREZCOBIX® Combination Tablets*HC2912	Elvitegravir/Cobicistat /Emtricitabine/Tenofovir disoproxil fumarate*HC2911 Elvitegravir/Cobicistat /Emtricitabine/Tenofovir Alafenamide Fumarate*HC2913 Darunavir Ethanolate/Cobicistat *HC2912			Approved	
24	2012	2012/11/14	(24yaku) No. 292	2	Dried polyethylene glycol-treated human immunoglobulin	Stevens-Johnson syndrome and toxic epidermal necrolysis (for which systemic steroid treatment is not sufficiently effective)	Nihon Pharmaceutical Co., Ltd.	Stevens-Johnson syndrome and toxic epidermal necrolysis (for which systemic steroid treatment is not sufficiently effective)	Nihon Pharmaceutical Co., Ltd.	2014/7/4	Kenketu glovenin-I for IV injection 500 mg Kenketu glovenin-I for IV injection 2500 mg Kenketu glovenin-I for IV injection 5000 mg	kenketu glovenin® - I for IV injection 500mg kenketu glovenin® - I for IV injection 2500mg kenketu glovenin® - I for IV injection 5000mg	Freeze-dried Polyethylene Glycol-treated Normal Human Immunoglobulin			Approved	
24	2012	2012/11/14	(24yaku) No. 293		SAR302503	Myelofibrosis	Sanofi K.K.	-	-	-	-	-	-	Designation revoked	2015/5/25	Revoked	
24	2012	2012/12/11	(24yaku) No. 294		Cinacalcet hydrochloride	Hypercalcemia associated with parathyroid carcinoma or intractable primary hyperparathyroidism	Kyowa Hakko Kirin Co., Ltd.	Hypercalcemia associated with parathyroid carcinoma or unresectable/postoperative recurrence primary hyperparathyroidism	Kyowa Hakko Kirin Co., Ltd.	2014/2/21(Regpara Tablet 25mg, Regpara Tablet 75mg) 2015/2/10(Regpara Tablet 12.5mg)	Regpara Tablet 25mg Regpara Tablet 75mg Regpara Tablet 12.5mg	REGPARA® TABLETS 25mg REGPARA® TABLETS 75mg REGPARA® TABLETS 12.5mg	Cinacalcet Hydrochloride			Approved	
24	2012	2012/12/11	(24yaku) No. 295		BMN110	Mucopolysaccharidosis Type IV A	BioMarin Pharmaceutical Inc.	Mucopolysaccharidosis Type IV A	BioMarin Pharmaceutical Inc.	2014/12/26	Vimizim I.V.Infusion 5mg	Vimizim®	Elosulfase Alfa (Genetical Recombination)			Approved	
24	2012	2012/12/11	(24yaku) No. 296	1	Precipitated influenza vaccine cell culture (H5N1 strain)	Prophylaxis of H5N1 influenza	Kitasato Daiichi Sankyo Vaccine Co., Ltd.	Prophylaxis of H5N1 influenza	Kitasato Daiichi Sankyo Vaccine Co., Ltd.	2014/3/24 2016/3/18	·Adsorbed cell culture-derived H5N1 influenza virus vaccine 30μg/mL intramuscular injection “Kitasato Daiichi Sankyo” ·Adsorbed cell culture-derived H5N1 influenza virus vaccine 60μg/mL intramuscular injection “Kitasato Daiichi Sankyo”	-	Precipitated influenza vaccine cell culture (H5N1 strain)			Approved	
24	2012	2012/12/11	(24yaku) No. 297	3	Precipitated influenza vaccine cell culture (prototype vaccine)	Prophylaxis for new strains of influenza	Kitasato Daiichi Sankyo Vaccine Co., Ltd.	-	-	-	-	-	-				
24	2012	2013/3/15	(25yaku) No. 298	1	Mogamulizumab (recombinant)	Peripheral T-cell lymphoma, cutaneous T-cell lymphoma	Kyowa Hakko Kirin Co., Ltd.	Relapsed or refractory CCR4-positive peripheral T-cell lymphoma, Relapsed or refractory CCR4-positive cutaneous T-cell lymphoma	Kyowa Hakko Kirin Co., Ltd.	2014/3/17	Poteligeo for IV infusion 20 mg	POTELIGEO® Injection	Mogamulizumab(recombinant)			Approved	
24	2012	2013/3/15	(25yaku) No. 299	2	Bexarotene	Cutaneous T-cell lymphoma	Minophagen Pharmaceutical Co., Ltd.	Cutaneous T-Cell Lymphoma	Minophagen Pharmaceutical Co., Ltd.	2016/1/22	Targretin capsules 75mg	AO301capsules	Bexarotene			Approved	
24	2012	2013/3/15	(25yaku) No. 300		Ipilimumab	Malignant melanoma	Bristol-Myers	Unresectable Melanoma	Bristol-Myers	2015/7/3	Yervoy Injection 50 mg	YERVOY® Injection	ipilimumab (recombinant)			Approved	
25	2013	2013/5/13	(25yaku) No. 301	1,3 *41	Aminolevulinic acid hydrochloride	Visualization of tumor tissue during surgical resection of non-muscle invasive bladder cancer	Nobelpharma Co., Ltd. SBI Pharmaceuticals Co., Ltd.	Visualization of non-muscle invasive bladder cancer in transurethral resection of bladder tumor (TURBT)	SBI Pharmaceuticals Co., Ltd.	2017/9/27	ALAGLIO® Divided Granules 1.5g	ALAGLIO®	Aminolevulinic acid hydrochloride	Designation revoked (2017/12/1) "Nobelpharma"	Designation revoked (2017/12/1) "Nobelpharma"	Approved	
25	2013	2013/5/13	(25yaku) No. 302		Rifaximin	Hepatic encephalopathy	ASKA Pharmaceutical. Co., Ltd.	Improvement of hyperammonemia in hepatic encephalopathy	ASKA Pharmaceutical. Co., Ltd.	2016/9/28	Rifxima tablets 200mg	Rifxima®	Rifaximin			Approved	
25	2013	2013/5/13	(25yaku) No. 303	2	Ozanezumab	Amyotrophic lateral sclerosis (ALS)	GlaxoSmithKline K.K.	-	-	-	-	-	-	Designation revoked (2016/02/25)	2016/2/25	Revoked	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
25	2013	2013/5/13	(25yaku) No. 304		Bevacizumab (recombinant)	Malignant glioma	Chugai Pharmaceutical Co., Ltd.	Malignant glioma	Chugai Pharmaceutical Co., Ltd.	2013/6/14	Avastin for IV infusion 100 mg/4 mL Avastin for IV infusion 400 mg/16 mL	AVASTIN® 100mg/4mL Intravenous Infusion AVASTIN® 400mg/16mL Intravenous	Bevacizumab (recombinant) (JAN)			Approved	
25 30	2013 2018	2013/6/17 2018/7/2*68	(25yaku) No. 305	5	Dried sulfonated human immunoglobulin	Optic neuritis (for which steroid treatment is not sufficiently effective)	Kaketsuken KM Biologics Co., Ltd. *68 Teijin Pharma Limited	-	-	-	-	-	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2018/07/2) *68			
25	2013	2013/6/17	(25yaku) No. 306		Denosumab (Genetical Recombinationt)	Giant cell tumor of bone	Daiichi Sankyo Company, Limited	Giant cell tumor of bone	Daiichi Sankyo Company, Limited	2014/5/23	RANMARK SUBCUTANEOUS INJECTION 120mg	RANMARK® SUBCUTANEOUS INJECTION	Denosumab (Genetical Recombinationt)			Approved	
25	2013	2013/6/17	(25yaku) No. 307	1	Ambrisentan	Chronic thromboembolic pulmonary hypertension	GlaxoSmithKline K.K.	-	-	-	-	-	Ambrisentan				
25	2013	2013/6/17	(25yaku) No. 308	1、2 *42	ONO-4538	Malignant melanoma	Ono Pharmaceutical Co., Ltd.	Unresectable Melanoma	Ono Pharmaceutical Co., Ltd.	2014/7/4 2016/2/29 *42	OPDIVO Intravenous Infusion 20mg OPDIVO Intravenous Infusion 100mg	OPDIVO® Intravenous Infusion	Nivolumab(Genetical Recombination)			Approved	
25	2013	2013/8/12	(25yaku) No. 309		Talaporfin sodium	Malignant brain tumors	Meiji Seika Pharma Co., Ltd.	Primary malignant brain tumor limited to cases treated with surgical resection	Meiji Seika Pharma Co., Ltd.	2013/9/20	Laserphyrin for injection 100 mg	LASERPHYRIN® FOR INJECTION	Talaporfin Sodium	Designation was transferred from (20yaku) No. 219 with the change in indication from malignant glioma to malignant brain tumor (expansion of range).			Approved	
25	2013	2013/9/3 2016/7/7 *43	(25yaku) No. 310		Lomitapide mesylate *43	Homozygous familial hypercholesterolemia (HoFH)	Aegerion Pharmaceuticals, Inc., 2013-09-03 AEGERION PHARMACEUTICALS K.K., 2016-07-07 *43	Homozygous familial hypercholesterolemia	AEGERION PHARMACEUTICALS K.K.	2016/9/28	Juxtapid capsule 5mg Juxtapid capsule 10mg Juxtapid capsule 20mg	Juxtapid® Capsules 5mg Juxtapid® Capsules 10mg Juxtapid® Capsules 20mg	Lomitapide mesilate	Designation revoked (2016/07/07) *43		Approved	
25	2013	2013/9/3	(25yaku) No. 311		Rituximab (recombinant)	Chronic idiopathic thrombocytopenic purpura	Zenyaku Kogyo Co., Ltd.	-	-	-	-	-	Rituximab (recombinant)	Designation revoked (2017/03/10)	2017/3/10	Revoked	
25	2013	2013/9/3	(25yaku) No. 312		BYM338	Inclusion body myositis	Novartis Pharma K.K.	-	-	-	-	-	Bimagrumab (Genetical Recombination)				
25	2013	2013/9/13	(25yaku) No. 313	3	Mepolizumab	Churg-Strauss syndrome	GlaxoSmithKline K.K.	Eosinophilic Granulomatosis with Polyangiitis (EGPA) inadequately responding to the current treatment	GlaxoSmithKline K.K.	2018/5/25	Nucala for s.c. injection	Nucala for s.c. injection	Mepolizumab (Genetical Recombination)			Approved	
25	2013	2013/9/13	(25yaku) No. 314		Dolutegravir sodium	HIV infection	ViiV Healthcare K.K.	HIV infection	ViiV Healthcare K.K.	2014/3/24(Tivicay tablet 50mg) 2015/3/16(Triumeq combination tablet)	Tivicay tablet 50mg Triumeq combination tablet *HC3141	Tivicay®Tablets Triumeq® Combination Tables	dolutegravir sodium			Approved	
25	2013	2013/9/13	(25yaku) No. 315		Sorafenib tosylate	Thyroid cancer	Bayer Holding Ltd.	Unresectable differentiated thyroid cancer Unresectable thyroid cancer *55	Bayer Holding Ltd.	2014/6/20 2016/2/29 *55	Nexavar tablets 200mg	Nexavar®200mg	Sorafenib tosylate			Approved	
25	2013	2013/9/13	(25yaku) No. 316		Alectinib hydrochloride	Unresectable progressive or recurrent ALK fusion gene-positive non-small cell lung cancer	Chugai Pharmaceutical Co., Ltd	Unresectable progressive or recurrent ALK fusion gene-positive non-small cell lung cancer	Chugai Pharmaceutical Co., Ltd	2014/7/4(Alecensa capsule 20 mg, Alecensa capsule 40 mg) 2015/9/2(Alecensa capsule 150 mg)	Alecensa capsule 20 mg Alecensa capsule 40 mg Alecensa capsule 150 mg	Alecensa®	Alectinib Hydrochloride			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
25	2013	2013/09/13 2015/04/22 *44	(25yaku) No. 317		trametinib	BRAFV600 mutation-positive malignant melanoma	GlaxoSmithKline K.K., 2013-9-13 Novartis Pharma K.K., 2015-04-22 *44	*BRAF* mutation-positive malignant melanoma	Novartis Pharma K.K.	2016/3/28 2018/7/2	Mekinist Tablets 0.5 mg Mekinist Tablets 2 mg	MEKINIST	Trametinib dimethylsulfoxide solvate	Designation revoked (2015/04/22) *44		Approved	
25	2013	2013/09/13 2015/04/22 *45	(25yaku) No. 318		dabrafenib	BRAFV600 mutation-positive malignant melanoma	GlaxoSmithKline K.K., 2013-9-13 Novartis Pharma K.K., 2015-04-22 *45	*BRAF* mutation-positive malignant melanoma	Novartis Pharma K.K.	2016/3/28 2018/7/2	Tafinlar Capsules 50 mg Tafinlar Capsules 75 mg	TAFINLAR	Dabrafenib Mesylate	Designation revoked (2015/04/22) *45		Approved	
25	2013	2013/11/15	(25yaku) No. 319		Propranolol hydrochloride	Infantile hemangiomas	Maruho Co., Ltd.	Infantile hemangioma	Maruho Co., Ltd.	2016/7/4	Hemangiol Syrup for Pediatric 0.375%	Hemangiol® Syrup for Pediatric 0.375%	Propranolol Hydrochloride			Approved	
25	2013	2013/12/4	(25yaku) No. 320		Human C1 inhibitor	Prevention and treatment of angioedema episodes in patients with human C1 inhibitor (C1 INH) deficiency due to heredity or spontaneous mutations	ViroPharma Incorporated	-	-	-	-	-	-				
25	2013	2013/12/4 2016/6/30 *46	(25yaku) No. 321		Vandetanib	Thyroid cancer	AstraZeneca K.K., 2013-12-04 Sanofi K.K., 2016-06-30 *46	unresectable medullary thyroid cancer	Sanofi K.K.	2015/9/28	Caprelsa Tablets 100mg	Caprelsa Tablets 100mg	Vandetanib	Designation revoked (2016/06/30) *46		Approved	
25	2013	2013/12/4 2016/1/29 *47 2018/3/30*60	(25yaku) No. 322		MEK162 binimetinib *47	NRAS or BRAFV600 mutation-positive malignant melanoma	Novartis Pharma K.K., 2013-12-04 Array BioPharma, Inc, 2016-01-29 *47 Ono Pharmaceutical Co., Ltd.*60	-	-	-	-	-	Binimetinib	Designation revoked (2016/01/29) *47 Designation revoked (2018/03/30) *60			
25	2013	2013/12/4 2016/1/29 *48 2018/3/30*61	(25yaku) No. 323		LGX818 Encorafenib	BRAFV600 mutation-positive malignant melanoma	Novartis Pharma K.K., 2013-12-04 Array BioPharma, Inc, 2016-01-29 *48 Ono Pharmaceutical Co., Ltd.*61	-	-	-	-	-	Encorafenib	Designation revoked (2016/01/29) *48 Designation revoked (2018/03/30) *61			
25	2013	2013/12/4	(25yaku) No. 324		Bosutinib hydrate	Chronic myelogenous leukemia with resistance or intolerance to previous treatments	Pfizer Japan Inc.	Chronic myelogenous leukemia with resistance or intolerance to previous treatments	Pfizer Japan Inc.	2014/9/26	Boslif tablet 100 mg	BOSLIF®	Bosutinib Hydrate			Approved	
(25yaku) No. 325 is regarded as orphan products for regenerative medicine.																	
(25yaku) No. 326 is regarded as orphan products for regenerative medicine.																	
25	2013	2013/12/12	(25yaku) No. 327	3	Modafinil	Excessive daytime sleepiness associated with idiopathic hypersomnia	Alfresa Pharma Corporation	-	-	-	-	-	Modafinil				
25 30	2013 2018	2014/2/26 2018/7/2*69	(26yaku) No. 328	4	Dried sulfonated human immunoglobulin	Improvement of microscopic polyangiitis (limited to cases in which steroids are inadequate)	Kaketsuken KM Biologics Co., Ltd. *69 Teijin Pharma Limited	-	-	-	-	-	Freeze-dried Sulfonated Normal Human Immunoglobulin	Designation revoked (2018/07/2) *69			
25	2013	2014/2/26	(26yaku) No. 329	3 *49	Pralatrexate	Peripheral T- cell lymphoma	Mundipharma K.K.	Relapsed or refractory peripheral T-cell lymphoma	Mundipharma K.K.	2017/7/3	Difolta injection 20mg	Difolta	Pralatrexate			Approved	
25	2013	2014/3/17	(26yaku) No. 330		Talaporfin Sodium	Local failure after chemoradiotherapy or radiotherapy for esophageal cancer	Meiji Seika Pharma Co., Ltd.	Local failure after chemoradiotherapy or radiotherapy for esophageal cancer	Meiji Seika Pharma Co., Ltd.	2015/5/26	Leserphyrin for injection 100 mg	LASERPHYRIN® FOR INJECTION	Talaporfin Sodium			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
25	2013	2014/3/17	(26yaku) No. 331		Darbepoetin Alfa (Genetical Recombination)	Anemia with Myelodysplastic Syndromes	Kyowa Hakko Kirin Co., Ltd.	Anemia with Myelodysplastic Syndromes	Kyowa Hakko Kirin Co., Ltd.	2014/12/18	Nesp Injection Plastic 5μg Syringe Nesp Injection Plastic 10μgSyringe Nesp Injection Plastic 15μgSyringe Nesp Injection Plastic 20μgSyringe Nesp Injection Plastic 30μgSyringe Nesp Injection Plastic 40μgSyringe Nesp Injection Plastic 60μgSyringe Nesp Injection Plastic120μ	NESP® INJECTION PLASTIC SYRINGE	Darbepoetin Alfa (Genetical Recombination)			Approved	
26	2014	2014/5/13	(26yaku) No. 332		EPI-743	Leigh syndrome	Dainippon Sumitomo Pharma Co., Ltd.	-	-	-	-	-	Vatiquinone				
26	2014	2014/5/13	(26yaku) No. 333		catridecacog	Inhibition of bleeding in patients with congenital factor XIII A-subunit deficiency	Novo Nordisk Pharma Ltd.	Inhibition of bleeding tendency in patients with congenital factor XIII A-subunit deficiency	Novo Nordisk Pharma Ltd.	2015/3/26	Novo thirteen 2500	Novo Thirteen®	Catridecacog (Genetical Recombination)			Approved	
26	2014	2014/5/13	(26yaku) No. 334		Canakinumab (Genetical Recombination)	Mevalonate Kinase. Deficiency	Novartis Pharma K.K.	Hyper-IgD Syndrome (Mevalonate Kinase Deficiency)	Novartis Pharma K.K.	2016/12/19(ILARIS for Subcutaneous Injection 150 mg) 2018/2/9(ILARIS solution for s.c. injection 150 mg)	ILARIS for Subcutaneous Injection 150 mg ILARIS solution for s.c. injection 150 mg	ILARIS	Canakinumab (Genetical Recombination)			Approved	
26	2014	2014/5/13	(26yaku) No. 335		Canakinumab (Genetical Recombination)	TNF receptor-associated periodic syndrome	Novartis Pharma K.K.	TNF-Receptor Associated Periodic Syndrome	Novartis Pharma K.K.	2016/12/19(ILARIS for Subcutaneous Injection 150 mg) 2018/2/9(ILARIS solution for s.c. injection 150 mg)	ILARIS for Subcutaneous Injection 150 mg ILARIS solution for s.c. injection 150 mg	ILARIS	Canakinumab (Genetical Recombination)			Approved	
26	2014	2014/5/13	(26yaku) No. 336		Canakinumab (Genetical Recombination)	Familial Mediterranean fever	Novartis Pharma K.K.	Familial Mediterranean Fever who don't have sufficient effect of existing therapies	Novartis Pharma K.K.	2016/12/19(ILARIS for Subcutaneous Injection 150 mg) 2018/2/9(ILARIS solution for s.c. injection 150 mg)	ILARIS for Subcutaneous Injection 150 mg ILARIS solution for s.c. injection 150 mg	ILARIS	Canakinumab (Genetical Recombination)			Approved	
26 30	2014 2018	2014/5/13 2018/7/2*70	(26yaku) No. 337	4	MC710 (freeze dried human blood coagulation factor X added to activated blood coagulation factor VII)	Control of hemorrhage in patients with inhibitors against blood coagulation FVIII and FIX	Kaketsuken KM Biologics Co., Ltd. *70	Control of hemorrhage in patients with inhibitors against blood coagulation FVIII and FIX	Kaketsuken KM Biologics Co., Ltd. *70	2014/7/4	Byclot	Byclot®	freeze dried human blood coagulation factor X added to activated blood coagulation factor VII	Designation revoked (2018/07/2) *70		Approved	
26	2014	2014/6/11	(26yaku) No. 338		icatibant	Acute attacks of hereditary angioedema	Shire Japan KK	-	-	-	-	-	Icatibant Acetate				
26	2014	2014/6/11	(26yaku) No. 339		ibrutinib	Chronic lymphocytic leukemia Small lymphocytic lymphoma Mantle cell lymphoma	Janssen Pharmaceutical K.K.	・Relapsed or refractory chronic lymphocytic leukemia (including small lymphocytic lymphoma) ・Relapsed or refractory mantle cell lymphoma *56	Janssen Pharmaceutical K.K.	2016/3/28 2016/12/2 *56 2018/7/2	IMBRUVICA Capsules 140mg	IMBRUVICA® Capsules	Ibrutinib			Approved	
26	2014	2014/6/11	(26yaku) No. 340		Tocilizumab (genetical recombination)	Large Vessel Vasculitis	Chugai Pharmaceutical Co., Ltd	Takayasu arteritis, giant cell arteritis The recommended dose of tocilizumab (genetical recombination) is 162mg as a single subcutaneous injection administered at 1-week intervals.	Chugai Pharmaceutical Co., Ltd	2017/8/25	Actemra® 162mg Syringe for subcutaneous (SC) Injection" and "Actemra® 162mg Auto-Injector for SC Injection	Actemra®	Tocilizumab (genetical recombination)			Approved	
26	2014	2014/6/11	(26yaku) No. 341		Eribulin mesylate	Malignant soft tissue tumors	Eisai Co., Ltd.	Soft tissue sarcoma	Eisai Co., Ltd.	2016/2/29	Halaven® injection 1mg	Halaven® injection 1mg	Eribulin mesylate			Approved	
26	2014	2014/6/11	(26yaku) No. 342		Pomalidomide	Relapsed or refractory multiple myeloma	Celgene K.K.	Relapsed or refractory multiple myeloma	Celgene K.K.	2015/3/26	Pomalyst capsule1mg Pomalyst capsule2mg Pomalyst capsule3mg Pomalyst capsule4mg	Pomalyst® Capsules	Pomalidomide			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
26	2014	2014/8/21	(26yaku) No. 343		Rituximab (recombinant)	Acquired thrombotic thrombocytopenic purpura	Zenyaku Kogyo Co., Ltd.	-	-	-	-	-	Rituximab (recombinant)				
26	2014	2014/8/21	(26yaku) No. 344		ISIS 420915	Transthyretin Familial Amyloid Polyneuropathy	GlaxoSmithKline K.K.	-	-	-	-	-	-				
26	2014	2014/8/21	(26yaku) No. 345		BG00012	Prevention of relapse and delay of physical disability progression in multiple sclerosis	Biogen Idec Japan Ltd.	Treatment of patients with multiple sclerosis to reduce the frequency of clinical exacerbations (relapses) and delay the accumulation of physical disability	Biogen Japan Ltd	2016/12/19	TECFIDERA Capsule 120mg TECFIDERA Capsule 240mg	TECFIDERA	Dimethyl fumarate			Approved	
26	2014	2014/8/21	(26yaku) No. 346		asfotase alfa	Hypophosphatasia	Alexion Pharma Godo Kaisha	Hypophosphatasia	Alexion Pharma Godo Kaisha	2015/7/3	STRENSI Subcutaneous Injection 12mg/0.3mL STRENSI Subcutaneous Injection 18mg/0.45mL STRENSI Subcutaneous Injection 28mg/0.7mL STRENSI Subcutaneous Injection 40mg/1mL STRENSI Subcutaneous Injection 80mg/0.8mL	STRENSIQ® Subcutaneous Injection	asfotase alfa			Approved	
26	2014	2014/9/17	(26yaku) No. 347		Selexipag	Pulmonary arterial hypertension	Nippon Shinyaku Co., Ltd	Pulmonary arterial hypertension	Nippon Shinyaku Co., Ltd	2016/9/28	Uptravi tablet 0.2mg Uptravi tablet 0.4mg	Uptravi® Tablets 0.2mg Uptravi® Tablets 0.4mg	Selexipag			Approved	
26	2014	2014/9/17	(26yaku) No. 348		Vigabatrin	Epilepsia nutans	Sanofi K.K.	epilepsia nutans	Sanofi K.K.	2016/3/28	Sabril	Sabril®	Vigabatrin			Approved	
26	2014	2014/9/17	(26yaku) No. 349		Panobinostat Lactate	Relapsed or refractory multiple myeloma	Novartis Pharma K.K.	Relapsed or refractory multiple myeloma	Novartis Pharma K.K.	2015/7/3	FARYDAK Capsules 10mg FARYDAK Capsules 15mg	FARYDAK® Capsules	Panobinostat Lactate			Approved	
26	2014	2014/9/17	(26yaku) No. 350		MK-3475	Melanoma	MSD K.K.	Radically unresectable melanoma	MSD K.K.	2016/9/28	Keytruda injection 20 mg Keutruda injection 100 mg	KEYTRUDA® Injection 20 mg KEYTRUDA® Injection 100 mg	Pembrolizumab (Genetical Recombination)			Approved	
26	2014	2014/9/17	(26yaku) No. 351		Peginterferon alfa-2b (generical recombination)	Adjuvant chemotherapy for malignant melanoma	MSD K.K.	Adjuvant chemotherapy for malignant melanoma	MSD K.K.	2015/5/26	Pegintron powder for SC injection 50μg／0.5mL Pegintron powder for SC injection 100μg／0.5mL Pegintron powder for SC injection 150μg／0.5mL	PEGINTRON® Powder for Injection	Peginterferon alfa-2b (generical recombination)			Approved	
26	2014	2014/11/20	(26yaku) No. 352		Thalidomide	Crow-Fukase (POEMS) syndrome	Fujimoto Pharmaceutical Corporation	-	-	-	-	-	Thalidomide				
26	2014	2014/11/20	(26yaku) No. 353		Eculizumab(genetical recombination)	Prevention of NMO-IgG-positive relapsing neuromyelitis optica （NMO)	Alexion Pharma Godo Kaisha	-	-	-	-	-	Eculizumab(genetical recombination)				
26	2014	2014/11/20	(26yaku) No. 354		Isopropyl unoprostone	Retinitis pigmentosa	R-Tech Ueno, Ltd.	-	-	-	-	-	Isopropyl unoprostone	Designation revoked (2017/03/01)	2017/3/1	Revoked	
26	2014	2014/11/20	(26yaku) No. 355		Nitric Oxide	Treatment of pre-,peri- and post-operative pulmonary hypertension in adults and children (including newborns) in heart surgery in order to selectively decrease pulmonary arterial pressure and improve right ventricular function and oxygenation	Ino Therapeutics, LLC	Improvement of pulmonaryhypertension in the perioperative period of cardiacsurgery	Ino Therapeutics, LLC	2015/8/24	Inoflo for inhalation 800 ppm	INOflo® for inhalation 800ppm	Nitric Oxide			Approved	
26	2014	2014/11/20	(26yaku) No. 356		teduglutide(genetical recombination)	Short Bowel Syndrome	NPS Pharma K.K. and G.K	-	-	-	-	-	-				

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
26	2014	2014/11/20	(26yaku) No. 357	2	carglumic acid	Inhibition of rising blood levels of ammonia in the following associated diseases: N-acetylglutamate synthetase deficiency,Isovaleric acidemia,Methylmalonic acidemia and Propionic acidemia	Pola Pharma INC.	Hyperammonemia in the following diseases: N-acetylglutamate synthetase deficiency,Isovaleric acidemia,Methylmalonic acidemia and Propionic acidemia	Pola Pharma INC.	2016/9/28	CARBAGLU dispersible tablets 200mg	CARBAGLU®	carglumic acid			Approved	
26	2014	2014/12/8	(26yaku) No. 358		Eculizumab(genetical recombination)	Intractable myasthenia gravis	Alexion Pharma Godo Kaisha	-	Alexion Pharma Godo Kaisha	2017/12/25	-	-	Eculizumab(genetical recombination)			Approved	
26	2014	2014/12/8	(26yaku) No. 359		Bosentan hydrate	Digital ulcers of systemic sclerosis	Actelion Pharmaceuticals Japan Ltd.	Inhibitingdevelopment of digital ulcer in patients withsystemic scleroderma (only for patients whocurrently have digital ulcers or have a history ofdigital ulcer.)	Actelion Pharmaceuticals Japan Ltd.	2015/8/24	Tracleer tablet 62.5 mg	Tracleer® 62.5	Bosentan hydrate			Approved	
27	2015	2015/5/25	(27yaku) No. 360	3	metirosine	Improvement of catecholamine excess and various symptoms in pheochromocytoma	Ono Pharmaceutical Co., Ltd.	-	-	-	-	-	Metirosine				
27	2015	2015/5/25	(27yaku) No. 361		Rituximab (genetical recombination)	Inhibition of antibody-mediated rejection in the following ABO-incompatible transplantation : kidney transplantation ,liver transplantation	Zenyaku Kogyo Co., Ltd.	Inhibition of antibody-mediated rejection in the following ABO-incompatible transplantation : kidney transplantation ,liver transplantation	Zenyaku Kogyo Co., Ltd.	2016/2/29 2018/02/02(new name of product approved for manufacturing and marketing)	Rituxan Injection 10 mg/mL (new name of product approved for manufacturing and marketing:2018.2.2 approved) Rituxan Intravenous Infusion 100mg Rituxan Intravenous Infusion 500mg	Rituxan	Rituximab (genetical recombination)			Approved	
27	2015	2015/6/15	(27yaku) No. 362		Ceritinib	Unresectable advanced and/or recurrent anaplastic lymphoma kinase (ALK) fusion gene-positive non-small cell lung cancer with resistance or intolerance to crizotinib.	Novartis Pharma K.K.	Unresectable advanced and/or recurrent anaplastic lymphoma kinase (ALK) fusion gene-positive non-small cell lung cancer with resistance or intolerance to crizotinib.	Novartis Pharma K.K.	2016/3/28	Zykadia Capsules 150mg	Zykadia Capsules 150mg	Ceritinib			Approved	
27	2015	2015/8/20	(27yaku) No. 363	1, 2, 3[*50]	Carfilzomib	Relapsed or refractory multiple myeloma	Ono Pharmaceutical Co., Ltd.	Relapsed or refractory multiple myeloma	Ono Pharmaceutical Co., Ltd.	2016/7/4[*50]	KYPROLIS 10mg KYPROLIS 40mg	KYPROLIS for Intravenous Infusion 10mg KYPROLIS for Intravenous Infusion 40mg	Carfilzomib			Approved[*50]	
27	2015	2015/9/14	(27yaku) No. 364		Bevacizumab (Genetical Recombination)	Cervical cancer	Chugai Pharmaceutical Co., Ltd.	colon cancer, rectal cancer, non-small cell lung cancer other than squamous cell cancer, ovarian cancer, cervical cancer, breast cancer, and malignant glioma.	Chugai Pharmaceutical Co., Ltd.	2016/5/23	AVASTIN® 100mg/4mL Intravenous Infusion AVASTIN® 400mg/16mL Intravenous Infusion	AVASTIN	Bevacizumab (Genetical Recombination)			Approved	
27	2015	2015/9/14	(27yaku) No. 365		Ponatinib Hydrochloride	Chronic myeloid leukemia resistant or intolerant to preceding drug treatment Relapsed or treatment resistant Philadelphia chromosome-positive acute lymphoblastic leukemia	Otsuka Pharmaceutical Co., Ltd.	Chronic myeloid leukemia resistant or intolerant to preceding drug treatment Relapsed or treatment resistant Philadelphia chromosome-positive acute lymphoblastic leukemia	Otsuka Pharmaceutical Co., Ltd.	2016/9/28	Iclusig Tablet 15mg	Iclusig®	Ponatinib Hydrochloride			Approved	
27	2015	2015/9/14	(27yaku) No. 366		Bedaquiline Fumarate	<Indicated bacteria> Mycobacterium tuberculosis susceptible to delamanid <Indication> Pulmonary multidrug-resistant tuberculosis (MDR-TB)	Janssen Pharmaceutical K.K.	**Indicated organism** *Mycobacterium tuberculosis* susceptible to this product **Indication** Multidrug-resistant pulmonary tuberculosis	Janssen Pharmaceutical K.K.	2018/1/19	SIRTURO Tablets	SIRTURO	Bedaquiline			Approved	
27	2015	2015/11/19	(27yaku) No. 367		Elotuzumab (genetic recombination)	Relapsed or refractory multiple myeloma	Bristol-Myers Squibb K.K	Relapsed or refractory multiple myeloma	Bristol-Myers Squibb K.K	2016/9/28	EMPLICITI for I.V. INFUSION 300 mg EMPLICITI for I.V. INFUSION 400 mg	EMPLICITI® for I.V. INFUSION	Elotuzumab (genetic recombination)			Approved	

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
27	2015	2015/11/19	(27yaku) No. 368		Tenofovir Alafenamide Fumarate	HIV Infection	Japan Tobacco, Inc.	HIV-1 infection	Japan Tobacco, Inc.	2016/6/17*HC3681 2016/12/9*HC3682	DESCOVY Combination Tablets LT DESCOVY Combination Tablets HT*HC3682	DESCOVY*HC3682	Emtricitabine/Tenofovir Alafenamide Fumarate*HC3682			Approved	
27	承認	2015/12/18	(27yaku) No. 369	3	Sirolimus	Angiofibroma due to tuberous sclerosis complex	Nobelpharma Co., Ltd.	Skin lesions in patients with tuberous sclerosis complex	Nobelpharma Co., Ltd.	2018.3.23	Rapalimus® Gel 0.2%	Rapalimus	Sirolimus	sakigake(27yaku)No.1		Approved	
27	2015	2015/12/18	(27yaku) No. 370	1	Pyrimethamine	Toxoplasmosis	Glaxo SmithKline K.K.	-	-	-	-	-	Pyrimethamine				
27	2015	2015/12/18	(27yaku) No. 371		Sulfadiazine	Toxoplasmosis	Alcon Japan Ltd.	-	-	-	-	-	Sulfadiazine				
27	2015	2015/12/18	(27yaku) No. 372		Plerixafor	Mobilization of hematopoietic stem cells into peripheral blood for autologous peripheral blood stem cell transplantation in combination with G-CSF	Sanofi K.K.	Mobilization of hematopoietic stem cells into peripheral blood for autologous peripheral blood stem cell transplantation	Sanofi K.K.	2016/12/19	Mozobil Injection 24mg	mozobil®	Plerixafor			Approved	
27	2015	2015/12/18	(27yaku) No. 373		HBI-8000	Peripheral T- cell lymphoma	Huya Japan GK	-	-	-	-	-	-				
27	2015	2016/2/25	(28yaku) No. 374		MK-8228	Prevention of onset of the following diseases in recipients of homatopoietic stem cells ・Cytomegalovirus antigenemia ・Cytomegalovirus infection and disease	MSD K.K.	Prevention of cytomegalovirus (CMV) disease in allogenic hematopoietic stem cell transplant (HSCT) patients	MSD K.K.	2018/3/23	PREVYMIS Tablets 240mg PREVYMIS Intravenous Infusion 240mg	PREVYMIS® Tablets 240mg PREVYMIS® Intravenous Infusion 240mg	Letermovir			Approved	
27	2015	2016/2/25	(28yaku) No. 375		Ixazomib citrate	Relapsed or refractory multiple myeloma	Takeda Pharmaceutical Co., Ltd.	Relapsed or refractory multiple myeloma	Takeda Pharmaceutical Co., Ltd.	2017/3/30	NINLARO® capsules 2.3mg. NINLARO® capsules 3mg. NINLARO® capsules 4mg.	NINLARO capsules 2.3mg NINLARO capsules 3mg NINLARO capsules 4mg	Ixazomib citrate			Approved	
27	2015	2016/3/16	(28yaku) No. 376		fampridine	Walk improvement of multiple sclerosis	Biogen Japan Ltd	-	-	-	-	-	Fampridine				
27	2015	2016/3/16	(28yaku) No. 377	2	STM-279	Adenosin deaminase deficiency	Teijin Pharma Limited	-	-	-	-	-	Elapegademase (Genetical Recombination)				
27	2015	2016/3/16	(28yaku) No. 378		Tocilizumab (Genetical Recombination)	Systemic sderosis	Chugai Pharmaceutical Co., Ltd.	-	Chugai Pharmaceutical Co., Ltd.	-	-	-	Tocilizumab (genetical recombination)				
27	2015	2016/3/16	(28yaku) No. 379	2	Human alpha1-Proteinase Inhibitor	Severe alpha1-antitrypsin deficiency developed COPD	Grifols Japan K.K.	-	-	-	-	-	-				
27	2015	2016/3/16	(28yaku) No. 380		Lyophilized Human Prothrombin Complex Concentrate	Rapid correction of international normalized ratio (INR) in patients receiving vitamin K antagonist therapy (e.g. warfarin) who experience acute major bleeding and/or who require a surgical or invasive medical procedure.	CSL Behring K.K	Correction of bleeding tendency induced by VKA therapy in patients with acute major bleeding or in patients who are at risk of serious bleeding by urgent surgery/invasive procedures.	CSL Behring K.K	2017/3/30	Kcentra for I.V. injection 500 Kcentra for I.V. injection 1000	Kcentra	Human Prothrombin Complex			Approved	
27	2015	2016/3/16	(28yaku) No. 381		Nivolumab (Genetical Recombination)	Hodgkin lymphoma	Ono Pharmaceutical Co., Ltd.	Relapsed or refractory classical Hodgkin lymphoma	Ono Pharmaceutical Co., Ltd.	2016/12/2	OPDIVO Intravenous Infusion 20mg OPDIVO Intravenous Infusion 100mg	OPDIVO® Intravenous Infusion	Nivolumab (Genetical Recombination)			Approved	
28	2016	2016/6/20	(28yaku) No. 382		Selexipag	Unresectable or postoperative residual/recurrence Chronic thromboembolic pulmonary hypertension	Nippon Shinyaku Co., Ltd	-	-	-	-	-	Selexipag				
28	2016	2016/6/20	(28yaku) No. 383		Patisiran	Transthyretin Familial Amyloid Polyneuropathy	Genzyme Japan K.K.	-	-	-	-	-	Patisiran Sodium				

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
28	2016	2016/6/20	(28yaku) No. 384		Lenalidomide Hydrate	Relapsed or refractory adult T-cell leukemia/lymphoma	Celgene K.K.	Relapsed or refractory adult T-cell leukemia/lymphoma	Celgene K.K.	2017/3/12	Revlimid capsule 2.5 mg Revlimid capsule 5 mg	Revlimid® Capsules 5mg	Lenalidomide Hydrate			Approved	
28	2016	2016/8/24	(28yaku) No. 385		Onoact 50mg for intravenous infusion, Onoact 150mg for intravenous infusion	Life-threatening refractory and emergent cardiac arrhythmias: ventricular fibrillation and hemodaynamically unstable ventricular tachycardia	Ono Pharmaceutical Co., Ltd.	-	-	-	-	-	Landiolol Hydrochloride				
28	2016	2016/8/24	(28yaku) No. 386		Crizotinib	*ROS1* -fusion gene positive unresectable advanced and/or recurrent non-small cell lung cancer	Pfizer Japan Inc.	*ROS1* fusion gene positive unresectable advanced and/or recurrent non-small cell lung cancer	Pfizer Japan Inc.	20197/5/18	XALKORI Capsule 200 mg XALKORI Capsule 250 mg	XALKORI	Crizotinib			Approved	
28	2016	2016/8/24	(28yaku) No. 387		Romidepsin	Peripheral T-cell lymphoma	Celgene K.K.	Relapsed or refractory peripheral T-cell lymphoma	Celgene K.K.	2017/7/3	ISTODAX for intravenous injection 10mg	ISTODAX®	Romidepsin			Approved	
28	2016	2016/8/24	(28yaku) No. 388		emicizumab	prevention and reduction of bleeding episodes in patients with congenital FVIII deficiency (hemophilia A)	Chugai Pharmaceutical Co., Ltd	Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with congenital factor VIII deficiency (hemophilia A) with factor VIII inhibitors.	Chugai Pharmaceutical Co., Ltd	2018/3/23	HEMLIBRA® s.c 30mg HEMLIBRA® s.c 60mg HEMLIBRA® s.c 90mg HEMLIBRA® s.c 105mg HEMLIBRA® s.c 150mg	HEMLIBRA®	emicizumab			Approved	
28	2016	2016/9/27	(28yaku) No. 389		Dabrafenib Mesylate	Unresectable and/or relapsed advanced non-small cell lung cancer with a BRAF V600 mutation	Novartis Pharma K.K.	*BRAF* mutation-positive advanced and recurrent unresectable non-small cell lung cancer	Novartis Pharma K.K.	2018/3/23	Tafinlar Capsules 50 mg Tafinlar Capsules 75 mg	TAFINLAR	Dabrafenib Mesylate			Approved	
28	2016	2016/9/27	(28yaku) No. 390		Trametinib dimethylsulfoxide solvate	Unresectable and/or relapsed advanced non-small cell lung cancer with a BRAF V600 mutation	Novartis Pharma K.K.	*BRAF* mutation-positive advanced and recurrent unresectable non-small cell lung cancer	Novartis Pharma K.K.	2018/3/23	Mekinist Tablets 0.5 mg Mekinist Tablets 2 mg	MEKINIST	Trametinib dimethylsulfoxide solvate			Approved	
28	2016	2016/11/24	(28yaku) No. 391		Eltrombopag Olamine	Aplastic anemia	Novartis Pharma K.K.	Aplastic anemia	Novartis Pharma K.K.	2017/8/25	Revolade Tablets 12.5mg Revolade Tablets 25mg	Revolade	Eltrombopag Olamine			Approved	
28	2016	2016/11/24	(28yaku) No. 392		Sodium Nusinersen	Spinal Muscular Atrophy	Biogen Japan Ltd	Spinal Muscular Atrophy *58	Biogen Japan Ltd	2017/7/3 2017/9/22 *58	SPINRAZA Intrathecal Injection 12mg	SPINRAZA	Sodium Nusinersen			Approved	
28	2016	2016/12/5	(28yaku) No. 393		Daratumumab (Genetical recombination)	Relapsed or refractory multiple myeloma	Janssen Pharmaceutical K.K.	Relapsed or refractory multiple myeloma	Janssen Pharmaceutical K.K.	2017/9/17	DARZALEX® Intravenous Infusion 100mg DARZALEX® Intravenous Infusion 400mg	DARZALEX®	Daratumumab (Genetical recombination)			Approved	
28	2016	2016/12/21	(28yaku) No. 394		avelumab	Merkel cell carcinoma	MerckSerono Co., Ltd.	Curatively unresectable Merkel cell carcinoma	MerckSerono Co., Ltd.	2017/9/27	BAVENCIO Intravenous Infusion 200mg	BAVENCIO®	Avelumab (Genetical Recombination)			Approved	
28	2016	2016/12/21	(28yaku) No. 395		-	-	Chugai Pharmaceutical Co., Ltd.	-	-	-	-	-	-	Designation revoked (2017/06/05)	2017/6/5	Revoked	
28	2016	2016/12/21	(28yaku) No. 396		Spiramycin	Treatment of toxoplasmosis in pregnant women	Sanofi K.K.	Suppression of congenital toxoplasmosis	Sanofi K.K.	2018/7/2	Spiramycin tab 1.5 MIU	Spiramycin	Spiramycin			Approved	
28	2016	2017/3/1	(29yaku) No. 397		Canakinumab (genetical recombination)	Systemic Juvenile Idiopathic Arthritis	Novartis Pharma K.K.	Systemic Juvenile Idiopathic Arthritis patients who don't respond adequately to existing therapies	Novartis Pharma K.K.	2018/7/2	ILARIS for Subcutaneous Injection 150 mg ILARIS solution for Subcutaneous Injection 150mg	ILARIS	Canakinumab (genetical recombination)			Approved	
28	2016	2017/3/24	(29yaku) No. 398	1	Tolvaptan	Treatment of hyponatremia in Syndrome of inappropriate secretion of antidiuretic hormone (SIADH)	Otsuka Pharmaceutical Co., Ltd.	-	-	-	-	-	Tolvaptan				
28	2016	2017/3/24	(29yaku) No. 399	1	Sodium Valproate	Spinal Muscular Atrophy	Kowa Company, Ltd.	-	-	-	-	-	Sodium Valproate				

Current as of the date July 31, 2018

Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
28	2016	2017/3/24	(29yaku) No. 400		Olaparib	*BRCA* mutated ovarian cancer	AstraZeneca K.K.	-	-	-	-	-	Olaparib				
28	2016	2017/3/24	(29yaku) No. 401		Inotuzumab Ozogamicin (Genetical Recombination)	Relapsed or refractory acute lymphoblastic leukemia	Pfizer Japan Inc.	Relapsed or refractory CD 22-positive acute lymphoblastic leukemia	Pfizer Japan Inc.	2018/1/19	Besponsa Injection 1 mg	BESPONSA® Injection 1 mg	Inotuzumab Ozogamicin (Genetical Recombination)			Approved	
29	2017	2017/9/7	(29yaku) No. 402		dimethyl sulfoxide	interstitial cystitis	KYORIN PHARMACEUTICAL CO., LTD.	-	-	-	-	"TBD(To be determined)"	dimethyl sulfoxide(INN)				
	2017	2017/9/29	(29yaku) No. 403		Adalimumab (Genetical Recombination)	Hidradenitis Suppurativa	AbbVie GK	-	-	-	-	HUMIRA® for Subcutaneous Injection 20mg syringe 0.4mL HUMIRA® for Subcutaneous Injection 40mg syringe 0.8mL HUMIRA® for Subcutaneous Injection 40mg syringe 0.4mL HUMIRA® for Subcutaneous Injection 80mg syringe 0.8mL HUMIRA® for Subcutaneous Injection 40mg Pen 0.4mL	Adalimumab (genetical recombination)				
	2017	2017/9/29	(29yaku) No. 404		Blinatumomab (Genetical Recombination)	Acute lymphoblastic leukemia	Amgen Astellas BioPharma K.K.	-	-	-	-	BLINCYTO®	Blinatumomab (Genetical Recombination)				
	2017	2017/9/29	(29yaku) No. 405		olaparib	Unresectable or recurrent *BRCA* mutated breast cancer	AstraZeneca K.K.	Unresectable or recurrent BRCA mutated HER2 negative breast cancer with prior anticancer chemotherapy	AstraZeneca K.K.	2018.07.02	LYNPARZA Tablets 100 mg LYNPARZA Tablets 150 mg	LYNPARZA	olaparib			Approved	
	2017	2017/12/1	(29yaku) No. 406		Nivolumab (Genetical Recombination)	Malignant Pleural Mesothelioma	Ono Pharmaceutical Co., Ltd.	-	-	-	-	-	Nivolumab (Genetical Recombination)				
	2017	2017/12/21	(29yaku) No. 407		Mogamulizumab(Genetical Recombination)	HTLV-1 Associated Myelopathy: HAM	Kyowa Hakko Kirin Co., Ltd.	-	-	-	-	-	Mogamulizumab (Genetical Recombination)				
	2018	2018/2/22	(30yaku) No. 408		Somatuline	thyroid-stimulating hormone-secreting pituitary adenoma (TSH-secreting pituitary adenoma)	Teijin Pharma Limited	-	-	-	-	-	Lanreotide				
	2018	2018/2/22	(30yaku) No. 409		Daratumumab (Genetical recombination)	Newly diagnosed multiple myeloma	Janssen Pharmaceutical K.K.	-	-	-	-	-	Daratumumab (Genetical recombination)				
	2018	2018/3/20	(30yaku) No. 410		Taurine	prevention of the recurrence of stroke-like episodes in MELAS	Taisho Pharmaceutical Co., Ltd.	-	-	-	-	-	taurine				
	2018	2018/3/20	(30yaku) No. 411		doravirine	HIV-1 infection	MSD K.K	-	-	-	-	-	Doravirine				
	2018	2018/3/20	(30yaku) No. 412		Gilteritinib Fumarate	acute myeloid leukemia with FLT3 mutations	Astellas Pharma Inc.	-	-	-	-	-	Gilteritinib Fumarate				
	2018	2018/3/20	(30yaku) No. 413		Rituximab (recombinant)	CD20-positive chronic lymphocytic leukemia	Zenyaku Kogyo Co., Ltd	-	-	-	-	-	Rituximab (recombinant)				

Current as of the date July 31, 2018																	
Fiscal year of designation (Heisei)	Fiscal year of designation	Date of designation	Designation number	Grant period (years)	Name of pharmaceutical drug with a designation	Anticipated indications or diseases the orphan drug is intended to treat on the designation	Name of applicant receiving the designation	Indications approved for manufacturing and marketing	Name of applicant obtaining approval for manufacturing and marketing	Date of approval for manufacturing and marketing	Name of product approved for manufacturing and marketing	Trade name	General name of active ingredient	Notes	Date of revocation of designation	<Status>	
	2018	2018/5/24	(30yaku) No. 414		Tafamidis Meglumine	Transthyretin Cardiomyopathy (TTR-CM)	Pfizer Japan Inc.	-	-	-	-	-	Tafamidis Meglumine				
	2018	2018/5/24	(30yaku) No. 415		Burosumab (Genetical Recombination)	FGF23-related hypophosphatemic rickets and osteomalacia	Kyowa Hakko Kirin Co., Ltd.	-	-	-	-	-	Burosumab (Genetical Recombination)				