

CDER Rare Disease And Orphan Drug Designated Approvals

CY 2014 Orphan Designated NDA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN [†]	RARE DISEASE [‡]
205582	DHP	DECITABINE	SUN PHARMA GLOBAL FZE	Myelodysplastic syndromes (MDS) including previously treated and untreated, de novo and secondary MDS of all French-American-British subtypes and intermediate-1, intermediate-2, and high-risk International Prognostic Scoring System groups.	1/23/2014	No	Yes
205677	DNP	HETLIOZ (TASIMELTEON)	VANDA PHARMACEUTICALS INC	Non 24 hour sleep-wake disorder (Non-24)	1/31/2014	Yes	Yes
203202	DCRP	NORTHERA (DROXIDOPA)	LUNDBECK NA LTD	Treatment of orthostatic dizziness, lightheadedness, or the "feeling that you are about to black out" in adult patients with symptomatic neurogenic orthostatic hypotension caused by primary autonomic failure (Parkinson's disease, multiple system atrophy, and pure autonomic failure), dopamine beta-hydroxylase deficiency, and non-diabetic autonomic neuropathy. Effectiveness beyond 2 weeks of treatment has not been demonstrated. The continued effectiveness of NORTHERA should be assessed periodically.	2/18/2014	Yes	Yes
205410	DCRP	HEMANGEOL (PROPRANOLOL HYDROCHLORIDE)	PIERRE FABRE DERMATOLOGIE	Indicated for the treatment of proliferating infantile hemangioma requiring systemic therapy.	3/14/2014	Yes	Yes
204684	DAIP	IMPAVIDO (MILTEFOSINE) 50 MG CAPSULES	KNIGHT THERAPEUTICS USA INC	Adults and adolescents ≥12 years of age weighing ≥30 kg (66 lbs) for treatment of: • Visceral leishmaniasis due to Leishmania donovani. • Cutaneous leishmaniasis due to Leishmania braziliensis, Leishmania guyanensis, and Leishmania panamensis. • Mucosal leishmaniasis due to Leishmania braziliensis.	3/19/2014	Yes	Yes
205919	DHP	PURIXAN (MERCAPTOPYRINE)	NOVA LABORATORIES LTD	Nucleoside metabolic inhibitor indicated for the treatment of patients with acute lymphoblastic leukemia (ALL) as a component of a combination maintenance therapy regimen.	4/28/2014	Yes	Yes
205755	DOP2	ZYKADIA (CERITINIB)	NOVARTIS PHARMACEUTICALS CORP	Treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer (NSCLC) who have progressed on or are intolerant to crizotinib.	4/29/2014	Yes	Yes
206256	DHP	BELEODAQ (BELINOSTAT)	SPECTRUM PHARMACEUTICALS INC	Treatment of patients with relapsed or refractory peripheral T-cell lymphoma.	7/3/2014	Yes	Yes
205579	DAAAP	RYANODEX (DANTROLENE SODIUM FOR INJECTABLE SUSPENSION)	EAGLE PHARMACEUTICALS INC	Treatment of malignant hyperthermia in conjunction with appropriate supportive measures and for the prevention of malignant hyperthermia in patients at high risk.	7/22/2014	Yes	Yes
205858	DHP	ZYDELIG (IDELALISIB)	GILEAD SCIENCES INC	Relapsed chronic lymphocytic leukemia (CLL), in combination with rituximab, in patients for whom rituximab alone would be considered appropriate therapy due to other co-morbidities. Relapsed follicular B-cell non-Hodgkin lymphoma (FL) in patients who have received at least two prior systemic therapies. Relapsed small lymphocytic lymphoma (SLL) in patients who have received at least two prior systemic therapies.	7/23/2014	Yes	Yes
205494	DGIEP	CERDELGA (ELIGLUSTAT)	GENZYME CORP	Long term treatment of adult patients with Gaucher disease type 1 who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.	8/19/2014	Yes	Yes
22535	DPARP	ESBRIET (PIRFENIDONE) CAPSULES	GENENTECH INC	Idiopathic pulmonary fibrosis (IPF)	10/15/2014	Yes	Yes
205832	DPARP	OFEV (NINTEDANIB)	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	Idiopathic pulmonary fibrosis (IPF)	10/15/2014	Yes	Yes
203255	DMEP	SIGNIFOR LAR (PASIREOTIDE PAMOATE) INJECTION	NOVARTIS PHARMACEUTICALS CORP	Treatment of patients with acromegaly who have had an inadequate response to surgery and/or for whom surgery is not an option.	12/15/2014	Yes	Yes
206162	DOP1	LYNPARZA (OLAPARIB)	ASTRAZENECA PHARMACEUTICALS LP	Monotherapy in patients with deleterious or suspected deleterious germline BRCA mutated (as detected by an FDA-approved test) advanced ovarian cancer who have been treated with three or more prior lines of chemotherapy.	12/19/2014	Yes	Yes

CY 2014 Orphan Designated BLA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN [†]	RARE DISEASE [‡]
125460	DGIEP	VIMIZIM (ELOSULFASE ALFA)	BIOMARIN PHARMACEUTICAL INC.	Patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).	2/14/2014	Yes	Yes
125390	DMEP	MYALEPT (METRELEPTIN)	AMYLIN PHARMACEUTICALS, LLC	Adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy.	2/24/2014	Yes	Yes
125477	DOP2	CYRAMZA (RAMUCIRUMAB)	ELI LILLY AND COMPANY	Advanced gastric cancer or gastro-esophageal junction adenocarcinoma, as a single-agent after prior fluoropyrimidine - or platinum-containing chemotherapy.	4/21/2014	Yes	Yes
125496	DHP	SYLVANT (SILTUXIMAB)	JANSSEN BIOTECH, INC.	Patients with multicentric Castleman's disease (MCD) who are human immunodeficiency virus (HIV) negative and human herpesvirus-8 (HHV-8) negative.	4/23/2014	Yes	Yes
125514	DOP2	KEYTRUDA (PEMBROLIZUMAB)	MERCK SHARP & DOHME CORP.	Treatment of patients with unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor.	9/4/2014	Yes	Yes
125557	DHP	BLINCYTO (BLINATUMOMAB)	AMGEN, INC.	Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL).	12/3/2014	Yes	Yes
125554	DOP2	OPDIVO (NIVOLUMAB)	BRISTOL-MYERS SQUIBB COMPANY	Treatment of patients with unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor.	12/22/2014	Yes	Yes

CY 2014 Orphan Designated Supplement Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN [†]	RARE DISEASE [‡]
204114/1	DOP2	MEKINIST (TRAMETINIB)	GLAXOSMITHKLINE INTELLECTUAL PROPERTY NO 2 LTD ENGLAND	In combination with dabrafenib, is indicated for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutations, as detected by an FDA-approved test.	1/8/2014	Yes	Yes
202806/2	DOP2	TAFINLAR (DABRAFENIB)	GLAXOSMITHKLINE INTELLECTUAL PROPERTY NO 2 LTD ENGLAND	Patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutations, as detected by an FDA-approved test.	1/9/2014	Yes	Yes
205552	DHP	IBRUTINIB (PCI-32765)	PHARMACYCLICS INC	Patients with Mantle cell lymphoma (MCL) who have received at least one prior therapy or Chronic lymphocytic leukemia (CLL) who have received at least one prior therapy.	2/12/2014	Yes	Yes
125277/70	DPARP	KALBITOR (ECALLANTIDE)	DYAX CORP	Treatment of acute attacks of hereditary angioedema (HAE) in patients 12 years of age and older.	3/28/2014	Yes	Yes
9190/24	DMIP	LIPIODOL (ETHIODIZED OIL)	GUERBET LLC	Hysterosalpingography in adults, lymphography in adult and pediatric patients, selective hepatic intra-arterial use for imaging tumors in adults with known hepatocellular carcinoma (HCC).	4/4/2014	Yes	Yes
125326/60	DHP	ARZERRA (OFATUMUMAB)	GLAXO GROUP LIMITED D/B/A GLAXOSMITHKLINE	In combination with chlorambucil, for the treatment of previously untreated patients with chronic lymphocytic leukemia (CLL) for whom fludarabine-based therapy is considered inappropriate. For the treatment of patients with CLL refractory to fludarabine and alemtuzumab.	4/17/2014	Yes	Yes
202207/1	DMIP	LYMPHOSEEK (TECHNETIUM Tc 99m TILMANOCEPT)	NAVIDEA BIOPHARMACEUTICALS INC	Radioactive diagnostic agent indicated for lymphatic mapping with a hand-held gamma counter to assist in the localization of lymph nodes draining a primary tumor site in patients with breast cancer or melanoma and for guiding sentinel lymph node biopsy, using a hand-held gamma counter in patients with clinically node negative squamous cell carcinoma of the oral cavity.	6/13/2014	Yes	Yes
206545	DHP	ZYDELIG (IDELALISIB)	GILEAD SCIENCES INC	Relapsed chronic lymphocytic leukemia (CLL), in combination with rituximab, in patients for whom rituximab alone would be considered appropriate therapy due to other co-morbidities. Relapsed follicular B-cell non-Hodgkin lymphoma (FL) in patients who have received at least two prior systemic therapies. Relapsed small lymphocytic lymphoma (SLL) in patients who have received at least two prior systemic therapies.	7/23/2014	Yes	Yes
205552/1	DHP	IBRUTINIB (PCI-32765)	PHARMACYCLICS INC	Patients with: mantle cell lymphoma (MCL) who have received at least one prior therapy, chronic lymphocytic leukemia (CLL) who have received at least one prior therapy or chronic lymphocytic leukemia with 17p deletion.	7/28/2014	Yes	Yes
125291/136	DGIEP	LUMIZYME (ALGLUCOSIDASE ALPHA2)	GENZYME CORPORATION	Pompe disease (acid α -glucosidase (GAA) deficiency).	8/1/2014	Yes	Yes

21602/38	DHP	VELCADE (BORTEZOMIB) INJ 3.5MG	MILLENNIUM PHARMACEUTICALS INC	Treatment of patients with multiple myeloma and patients with mantle cell lymphoma who have received at least 1 prior therapy.	8/8/2014	Yes	Yes
22291/12	DHP	PROMACTA® (ELTROMBOPAG) TABLETS	GLAXOSMITHKLINE	Thrombocytopenia in patients with chronic immune (idiopathic) thrombocytopenia (ITP) who have had an insufficient response to corticosteroids, immunoglobulins, or splenectomy. Thrombocytopenia in patients with chronic hepatitis C to allow the initiation and maintenance of interferon-based therapy. Patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy.	8/26/2014	Yes	Yes
22458/3	DGIEP	ELELYSO (TALIGLUCERASE ALFA)	PFIZER INC	Long term enzyme replacement therapy (ERT) for adult and pediatric patients with a confirmed diagnosis of Type 1 Gaucher disease.	8/27/2014	Yes	Yes
125057/356	DGIEP	HUMIRA (ADALIMUMAB)	ABBVIE INC.	Expanded indication: Reducing signs and symptoms and inducing and maintaining clinical remission in patients 6 years of age and older with moderately to severely active Crohn's disease who have had an inadequate response to corticosteroids or immunomodulators such as azathioprine, 6-mercaptopurine, or methotrexate.	9/23/2014	Yes	Yes
125057/367	DPARP	HUMIRA (ADALIMUMAB)	ABBVIE INC.	Expanded indication: Reducing signs and symptoms of moderately to severely active polyarticular Juvenile Idiopathic Arthritis (JIA) in patients 2 years of age and older.	9/30/2014	Yes	Yes
21602/40	DHP	VELCADE (BORTEZOMIB) INJ 3.5MG	MILLENNIUM PHARMACEUTICALS INC	Treatment of patients with multiple myeloma or mantle cell lymphoma.	10/8/2014	Yes	Yes
125477/2	DOP2	CYRAMZA (RAMUCIRUMAB)	ELI LILLY AND COMPANY	Advanced gastric or gastro-esophageal junction adenocarcinoma, as a single agent or in combination with paclitaxel, after prior fluoropyrimidine-or platinum-containing chemotherapy.	11/5/2014	Yes	Yes
125085/305	DOP1	AVASTIN (BEVACIZUMAB)	GENENTECH, INC	Expanded indication: Cervical cancer, in combination with paclitaxel and cisplatin or paclitaxel and topotecan in persistent, recurrent, or metastatic disease. Platinum-resistant recurrent epithelial ovarian, fallopian tube or primary peritoneal cancer, in combination with paclitaxel, pegylated liposomal doxorubicin or topotecan.	11/14/2014	Yes	Yes
21688/21	DMEP	SENSIPAR (CINACALCET HCL) 30/60/90MG TABS	AMGEN INC	Updated indication: Secondary Hyperparathyroidism (HPT) in adult patients with chronic kidney disease (CKD) on dialysis. Hypercalcemia in adult patients with Parathyroid Carcinoma (PC). Hypercalcemia in adult patients with primary HPT for whom parathyroidectomy would be indicated on the basis of serum calcium levels, but who are unable to undergo parathyroidectomy.	11/21/2014	Yes	Yes
202192/8	DHP	JAKAFI (RUXOLITINIB PHOSPHATE)	INCYTE CORP	Expanded indication: Treatment of patients with intermediate or high-risk myelofibrosis, including primary myelofibrosis, post-polycythemia vera myelofibrosis and post-essential thrombocythemia myelofibrosis and patients with polycythemia vera who have had an inadequate response to or are intolerant of hydroxyurea.	12/4/2014	Yes	Yes
125320/160	DOP1	XGEVA (DENOSUMAB)	AMGEN INC	Expanded indication: Prevention of skeletal-related events in patients with bone metastases from solid tumors. Treatment of adults and skeletally mature adolescents with giant cell tumor of bone that is unresectable or where surgical resection is likely to result in severe morbidity. Treatment of hypercalcemia of malignancy refractory to bisphosphonate therapy.	12/5/2014	Yes	Yes
21436/38	DPP	ABILIFY TABLET (ARIPIPIRAZOLE)	OTSUKA PHARMACEUTICAL CO LTD	Expanded indication: Schizophrenia, acute treatment of Manic and Mixed Episodes associated with Bipolar I, adjunctive treatment of Major Depressive Disorder, irritability associated with Autistic Disorder, treatment of Tourette's disorder.	12/12/2014	Yes	Yes
21866/23	DPP	ABILIFY (ARIPIPIRAZOLE) INJECTION	OTSUKA PHARMACEUTICAL CO LTD	Expanded indication: Schizophrenia, acute treatment of Manic and Mixed Episodes associated with Bipolar I, adjunctive treatment of Major Depressive Disorder, irritability associated with Autistic Disorder, treatment of Tourette's disorder. The injection is indicated for: agitation associated with schizophrenia or bipolar mania.	12/12/2014	Yes	Yes
22074/11	DOP2	SOMATULINE DEPOT (LANREOTIDE) INJECTION	IPSEN PHARMA	Expanded indication: Treatment of patients with unresectable, well- or moderately-differentiated, locally advanced or metastatic gastroenteropancreatic neuroendocrine tumors (GEP-NETs) to improve progression-free survival.	12/16/2014	Yes	Yes

† An Orphan designated drug is a drug intended to treat a rare disease that has received an orphan designation from the FDA prior to marketing approval.

‡ A Rare Disease is a disorder affecting less than 200,000 people in the United States.