CDER Rare Disease And Orphan Drug Designated Approvals

CY 2015 Orphan Designated NDA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
203952	DNP	DUOPA (CARBIDOPA AND LEVODOPA)	ABBVIE INC	Long-term treatment of motor fluctuations in patients with advanced Parkinson's disease.	1/9/2015	Yes	Yes
207026	DCRP	PHOXILIUM	GAMBRO RENAL PRODUCTS, INC.	As a replacement solution in Continuous Renal Replacement Therapy (CRRT) and in case of drug poisoning when CRRT is used to remove dialyzable substances.	1/13/2015	Yes	Yes
206947	DOP2	LENVIMA (LENVATINIB)	EISAI INC	Treatment of patients with locally recurrent or metastatic, progressive, radioactive iodine-refractory differentiated thyroid cancer.	2/13/2015	Yes	Yes
205353	DHP	FARYDAK (PANOBINOSTAT, LBH589)	NOVARTIS PHARMACEUTICALS CORP	FARYDAK® (panobinostat) in combination with bortezomib and dexamethasone for the treatment of patients with multiple myeloma who have received at least 2 prior regimens, including bortezomib and an immunomodulatory agent.	2/23/2015	Yes	Yes
207500	DAIP	CRESEMBA (ISAVUCONAZONIUM SULFATE)	ASTELLAS PHARMA US INC	Treatment of invasive aspergillosis and invasive mucormycosis in patients 18 years of age and older.	3/6/2015	Yes	Yes
207501	DAIP	CRESEMBA (ISAVUCONAZONIUM SULFATE)	ASTELLAS PHARMA US INC	Treatment of invasive aspergillosis and mucormycosis in patients 18 years of age and older.	3/6/2015	Yes	Yes
205750	DGIEP	CHOLBAM (CHOLIC ACID)	ASKLEPION PHARM< LLC	This new drug application provides for the use of CHOLBAM (cholic acid) Capsules for the treatment of bile acid synthesis disorders due to single enzyme defects and as adjunctive treatment of peroxisomal disorders including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption.	3/17/2015	Yes	Yes
207925	DPARP	KALYDECO (IVACAFTOR)	VERTEX PHARMACEUTICALS INC	Treatment of cystic fibrosis patients 2 years and older who have one of the following mutations in CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R and R117H.	3/17/2015	Yes	Yes
206910	DHP	JADENU (DEFERASIROX)	NOVARTIS PHARMACEUTICALS CORP	The treatment of chronic iron overload due to blood transfusions (transfusional hemosiderosis) in patients 2 years of age and older and for the treatment of chronic iron overload in patients 10 years of age and older with non-transfusion-dependent thalassemia (NTDT) syndromes and with a liver iron concentration (LIC) of at least 5 milligrams of iron per gram of liver dry weight (mg Fe/g dw) and a serum ferritin greater than 300 mcg/L.	3/30/2015	Yes	Yes
206038	DPARP	ORKAMBI (LUMACAFTOR/IVACAFTOR)	VERTEX PHARMACEUTICALS INC	Treatment of cystic fibrosis in patients age 12 years and older who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator gene.	7/2/2015	Yes	Yes
206406	DTOP	ENVARSUS XR (TACROLIMUS EXTENDED-RELEASE TABLETS)	VELOXIS PHARMACEUTICALS INC	For the prophylaxis of organ rejection in kidney transplant patients converted from tacrolimus immediate-release formulations in combination with other immunosuppressants.	7/10/2015	Yes	Yes
206995	DOP2	IRESSA (GEFITINIB)	ASTRAZENECA UK LTD	For the first-line treatment of patients with metastatic non-small cell lung cancer (NSCLC) whose tumors have epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 (L858R) substitution mutations as detected by an FDA-approved test.	7/13/2015	Yes	Yes
208169	DGIEP	XURIDEN (URIDINE TRIACETATE)	WELLSTAT THERAPEUTICS CORP	Treatment of hereditary orotic aciduria.	9/4/2015	Yes	Yes
207793	DOP2	ONIVYDE (IRINOTECAN LIPOSOME INJECTION)	MERRIMACK PHARMACEUTICALS INC	ONIVYDE is a topoisomerase inhibitor indicated, in combination with fluorouracil and leucovorin, for the treatment of patients with metastatic adenocarcinoma of the pancreas after disease progression following gemcitabine-based therapy. Limitation of Use: ONIVYDE is not indicated as a single agent for the treatment of patients with metastatic adenocarcinoma of the pancreas.	10/22/2015	Yes	Yes
207953	DOP2	YONDELIS (TRABECTEDIN)	JANSSEN PRODUCTS LP	For the treatment of patients with unresectable or metastatic liposarcoma or leiomyosarcoma who received a prior anthracycline containing regimen.	10/23/2015	Yes	Yes

206192	DOP2	COTELLIC (COBIMETINIB)	GENENTECH INC	Treatment, of patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutation, in combination with vemurafenib. COTELLIC is not indicated for treatment of patients with wild-type BRAF melanoma.	11/10/2015	Yes	Yes
208065	DOP2	TAGRISSO (OSIMERTINIB)	ASTRAZENECA PHARMACEUTICALS LP	This new drug application provides for the use of TAGRISSO (osimertinib), 40 mg and 80 mg tablets for the treatment of patients with metastatic epidermal growth factor receptor (EGFR) T790M mutation-positive-non-small-cell lung cancer (NSCLC), as detected by an FDA-approved test, who have progressed on or after EGFR TKI therapy.	11/13/2015	Yes	Yes
208462	DHP	NINLARO (IXAZOMIB)	MILLENNIUM PHARMACEUTICALS INC	Indicated in combination with lenalidomide and dexamethasone for the treatment of patients with multiple myeloma who have received at least one prior therapy.	11/20/2015	Yes	Yes
208159	DOP1	VISTOGARD (URIDINE TRIACETATE)	WELLSTAT THERAPEUTICS CORP	For the emergency treatment of adult and pediatric patients: following a fluorouracil or capecitabine overdose regardless of the presence of symptoms, or who exhibit early-onset, severe or life-threatening toxicity affecting the cardiac or central nervous system, and/or early-onset, unusually severe adverse reactions (e.g., gastrointestinal toxicity and/or neutropenia) within 96 hours following the end of fluorouracil or capecitabine administration.	12/11/2015	Yes	Yes
208434	DOP2	ALECENSA (ALECTINIB)	HOFFMANN-LA ROCHE INC	Alecensa (alectinib) is for the 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.	12/11/2015	Yes	Yes
207947	DCRP	UPTRAVI (SELEXIPAG)	ACTELION PHARMACEUTICALS LTD	UPTRAVI® is a prostacyclin receptor agonist indicated for the treatment of pulmonary arterial hypertension (PAH, WHO Group I) to delay disease progression and reduce the risk of hospitalization for PAH.	12/21/2015	Yes	Yes

CY 2015 Orphan Designated BLA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN[†]	RARE DISEASE[‡]
125511	DMEP	NATPARA (PARATHYROID HORMONE)	NPS PHARMACEUTICALS	As an adjunct to calcium and vitamin D to control hypocalcemia in patients with hypoparathyroidism.	1/23/2015	Yes	Yes
125516	DOP2	UNITUXIN (DINUTUXIMAB)	UNITED THERAPEUTICS CORPORATION	Indicated, in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2) and 13-cis-retinoic acid (RA), for the treatment of pediatric patients with high-risk neuroblastoma who achieve at least a partial response to prior first-line multiagent, multimodality therapy.	3/10/2015	Yes	Yes
125522	DMEP	REPATHA (EVOLOCUMAB)	AMGEN, INC.	Repatha is indicated as an adjunct to diet and maximally tolerated statin therapy for the treatment of adults with heterozygous familial hypercholesterolemia or clinical atherosclerotic cardiovascular disease, who require additional lowering of LDL-C. Repatha is also indicated as an adjunct to diet and other LDL-lowering therapies (e.g., statins, ezetimibe, LDL apheresis) in patients with HoFH who require additional lowering of LDL-C. This application only includes a 420 mg once monthly dosing regimen for the HoFH indication.	8/27/2015	Yes	Yes
761025	DHP	PRAXBIND (IDARUCIZUMAB)	BOEHRINGER INGELHEIM	Indicated in patients treated with Pradaxa® when reversal of the anticoagulant effects of dabigatran is needed for emergency surgery/urgent procedures and in life-threatening or uncontrolled bleeding.	10/16/2015	Yes	Yes
125513	DGIEP	STRENSIQ (ASFOTASE ALFA)	ALEXION PHARMACEUTICALS, INC.	For the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP).	10/23/2015	Yes	Yes
761036	DHP	DARZALEX (DARATUMUMAB)	JANSSEN BIOTECH, INC.	The treatment of patients with multiple myeloma who have received at least three prior lines of therapy including a proteasome inhibitor (PI) and an immunomodulatory agent or who are double-refractory to a PI and an immunomodulatory agent.	11/16/2015	Yes	Yes
125547	DOP2	PORTRAZZA (NECITUMUMAB)	ELI LILLY AND COMPANY	Indicated in combination with gemcitabine and cisplatin, for first-line treatment of patients with metastatic, squamous, non-small cell lung cancer.	11/24/2015	Yes	Yes
761035	DHP	EMPLICITI (ELOTUZUMAB)	BRISTOL-MYERS SQUIBB COMPANY	EMPLICITI is indicated in combination with lenalidomide and dexamethasone for the treatment of patients with multiple myeloma who have received one to three prior therapies.	11/30/2015	Yes	Yes

125561	DGIEP	KANUMA (SEBELIPASE ALFA)	ALEXION PHARMACEUTICALS, INC.	KANUMA is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme indicated for the treatment of patients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency.	12/8/2015	Yes	Yes

CY 2015 Orphan Designated Supplement Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
22068/20	DHP	TASIGNA (NILOTINIB, AMN107)	NOVARTIS PHARMACEUTICALS CORP	Treatment of newly diagnosed adult patients with Ph+ CML in chronic phase & chronic phase and accelerated phase Ph+ CML in adult patients resistant or intolerant to prior therapy that include imatinib.	1/27/2015	Yes	Yes
205552/2	DHP	IMBRUVICA (IBRUTINIB)	PHARMACYCLICS LLC	Waldenström's Macroglobulinemia	1/29/2015	Yes	Yes
21588/42	DHP	GLEEVEC (IMATINIB MESYLATE) 100/400MG	NOVARTIS PHARMACEUTICALS CORP	Newly diagnosed adult and pediatric patients with Philadelphia chromosome positive chronic myeloid leukemia in chronic phase.	1/30/2015	Yes	Yes
201367/3	DNP	BANZEL (RUFINAMIDE) ORAL SUSPENSION	EISAI INC	Adjunctive treatment of seizures associated with Lennox Gastaut Syndrome in children 1 year of age and older and adults.	2/12/2015	Yes	Yes
21880/41	DHP	REVLIMID (LENALIDOMIDE)	CELGENE CORP	To expand the indication for Revlimid® (lenalidomide) to include: Revlimid® (lenalidomide) in combination with dexamethasone is indicated for the treatment of patients with multiple myeloma.	2/17/2015	Yes	Yes
103353/5183	DMIP	NEUPOGEN (FILGRASTIM)	AMGEN, INC.	To increase survival in patients acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome).	3/30/2015	Yes	Yes
204026/6	DHP	POMALYST (POMALIDOMIDE)	CELGENE CORP	POMALYST, in combination with dexamethasone, is indicated for patients with multiple myeloma who have received at least two prior therapies including lenalidomide and a proteasome inhibitor and have demonstrated disease progression on or within 60 days of completion of the last therapy.	4/23/2015	Yes	Yes
21083/55	DPARP	RAPAMUNE (SIROLIMUS)1MG/ML ORAL SOLUTION	PF PRISM CV	Lymphangioleiomyomatosis (LAM)	5/28/2015	Yes	Yes
21110/73	DPARP	RAPAMUNE (SIROLIMUS) 1MG TABLETS	PF PRISM CV	Lymphangioleiomyomatosis (LAM)	5/28/2015	Yes	Yes
11366/30	DNP	KEVEYIS (DICHLORPHENAMIDE)	TARO PHARMACEUTICALS USA INC	Treatment of primary hyperkalemic periodic paralysis, primary hypokalemic periodic paralysis, and related variants.	8/7/2015	Yes	Yes
21986/16	DHP	SPRYCEL (DASATINIB, BMS-354825)	BRISTOL-MYERS SQUIBB CO	Treatment of adults with newly diagnosed Philadelphia chromosome-positive (Ph+) chronic myeloid leukemia (CML) in chronic phase.	8/12/2015	Yes	Yes
125388/80	DHP	ADCETRIS (BRENTUXIMAB VEDOTIN)	SEATTLE GENETICS, INC.	Treatment of patients with classical Hodgkin lymphoma at high risk of relapse or progression as post-autologous hematopoietic stem cell transplantation (auto-HSCT) consolidation.	8/17/2015	Yes	Yes
125057/393	DDDP	HUMIRA (ADALIMUMAB)	ABBVIE INC	Hidradenitis suppurativa	9/9/2015	Yes	Yes
21602/42	DHP	VELCADE (BORTEZOMIB) INJ 3.5MG	MILLENNIUM PHARMACEUTICALS INC	This "Prior Approval" supplemental new drug application provides for updates to the Pediatric Use section of the United States Prescribing Information based on the pediatric study report for Study AALL07P1, entitled "A Phase II Pilot Trial of Bortezomib in Combination with Intensive Re-Induction Therapy for Children with Relapsed Acute Lymphoblastic Leukemia (ALL) and Lymphoblastic Lymphoma (LL)."	9/14/2015	Yes	Yes
125554/2	DOP2	OPDIVO (NIVOLUMAB)	BRISTOL-MYERS SQUIBB COMPANY	Nivolumab, in combination with ipilimumab, for the treatment of patients with BRAF V600 wild-type, unresectable or metastatic melanoma.	9/30/2015	Yes	Yes
22081/33	DCRP	LETAIRIS (AMBRISENTAN)	GILEAD SCIENCES INC	In combination with tadalafil to reduce the risk of disease progression and hospitalization for worsening PAH, and to improve exercise ability, based on the AMBITION study.	10/2/2015	Yes	Yes
125377/73	DOP2	YERVOY (IPILILUMAB)	BRISTOL-MYERS SQUIBB COMPANY	For the adjuvant treatment of patients with cutaneous melanoma with pathologic involvement of regional lymph nodes of more than 1 mm, who have undergone complete resection including total lymphadenectomy.	10/28/2015	Yes	Yes
125031/180	DMIP	NEULASTA (PEGFILGRASTIM)	AMGEN, INC.	For the treatment of adult and pediatric patients at risk of developing myelosuppression after a radiological/nuclear incident.	11/13/2015	Yes	Yes
125514/6	DOP2	KEYTRUDA (PEMPROLIZUMAB)	MERCK SHARP & DOHME CORP.	First-line treatment of patients with unresectable or metastatic melanoma.	12/18/2015	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.*