CDER Rare Disease And Orphan Drug Designated Approvals

CY 2016 Orphan Designated NDA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
207916	DGIEP	CETYLEV (ACETYLCYSTEINE)	ARBOR PHARMACEUTICALS LLC	Treatment for acetaminophen overdose indicated to prevent or lessen hepatic injury afer ingestion of a potentially hepatotoxic quantity of acetaminophen in patients with acute ingestion or from repeated supratherapeutic ingestion.	1/29/2016	Yes	Yes
207155	DHP	EVOMELA (MELPHALAN)	SPECTRUM PHARMACEUTICALS INC	Original 1 - High-dose conditioning treatment prior to hematopoietic progenitor (stem) cell transplantation in patients with multiple myeloma. Original 2 – Palliative treatment of patients with multiple myeloma for whom oral therapy is not appropriate.	3/10/2016	Yes	Yes
208114	DHP	DEFITELIO (DEFIBROTIDE SODIUM)	JAZZ PHARMACEUTICALS INC	For the treatment of adult and pediatric patients with hepatic veno-occlusive disease (VOD), also known as sinusoidal obstruction syndrome (SOS), with renal or pulmonary dysfunction following hematopoietic stem cell transplantation (HSCT).	3/30/2016	Yes	Yes
204630	DHP	PROVAYBLUE (METHYLENE BLUE)	PROVEPHARM SAS	For the use of ProvayBlue (methylene blue) injection for the treatment of pediatric and adult patients with acquired methemoglobinemia.	4/8/2016	Yes	Yes
208573	DHP	VENCLEXTA (VENETOCLAX)	ABBVIE INC	Provides for the use of Venclexta (venetoclax) tablets; 10, 50, and 100 mg, for the treatment of patients with chronic lymphocytic leukemia (CLL) with 17p deletion, as detected by an FDA approved test, who have received at least one prior therapy.	4/11/2016	Yes	Yes
203324	DTOP	PHOTREXA VISCOUS (RIBOFLAVIN 5'-PHOSPHATE), PHOTREXA (RIBOFLAVIN 5'-PHOSPHATE), AND KXL SYSTEM	AVEDRO INC	Treatment of progressive keratoconus & treatment of corneal ectasia following refractive surgery.	4/15/2016	Yes	Yes
207999	DGIEP	OCALIVA (OBETICHOLIC ACID)	INTERCEPT PHARMACEUTICALS INC	Treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.	5/27/2016	Yes	Yes
208547	DMIP	NETSPOT (GALLIUM GA 68 DOTATATE)	ADVANCED ACCELERATOR APPLICATIONS USA INC	This provides for the use of Netspot (kit for the preparation of gallium Ga 68 dotatate injection), after radiolabeling with Ga 68, with positron emission tomography (PET) for localization of somatostatin receptor positive neuroendocrine tumors (NETs) in adult and pediatric patients.	6/1/2016	Yes	Yes
203324	DTOP	PHOTREXA VISCOUS (RIBOFLAVIN 5'-PHOSPHATE), PHOTREXA (RIBOFLAVIN 5'-PHOSPHATE), AND KXL SYSTEM	AVEDRO INC	Treatment of corneal ectasia following refractive surgery.	7/15/2016	Yes	Yes
206488	DNP	EXONDYS 51 (ETEPLIRSEN)	SAREPTA THERAPEUTICS INC	This new drug application provides for the use of Exondys 51 (eteplirsen) Injection, 50 mg per mL, for the treatment of Duchenne Muscular Dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.	9/19/2016	Yes	Yes
206030	DNP	CARNEVIX (CARBAMAZEPINE)	LUNDBECK LLC	Carnexiv is indicated as replacement therapy for oral carbamazepine formulations, when oral administration is temporarily not feasible, in adults with the following seizure types: • Partial seizures with complex symptomatology • Generalized tonic-clonic seizures • Mixed seizure patterns which include the above, or other partial or generalized seizures	10/7/2016	Yes	Yes

208398	DAIP	VERMOX (MEBENDAZOLE)	JANSSEN PHARMACEUTICALS INC	Treatment of patients one year of age and older with gastrointestinal infections caused by Ascaris lumbricoides (roundworm) and Trichuris trichiura (whipworm).	10/19/2016	Yes	Yes
209115	DOP1	RUBRACA (RUCAPARIB)	CLOVIS ONCOLOGY INC	Rubraca is a poly (ADP-ribose) polymerase (PARP) inhibitor indicated as monotherapy for the treatment of patients with deleterious BRCA mutation (germline and/or somatic) associated advanced ovarian cancer who have been treated with two or more chemotherapies. Select patients for therapy based on an FDA-approved companion diagnostic for Rubraca.	12/19/2016	Yes	Yes
209531	DNP	SPINRAZA (NUSINERSEN)	BIOGEN IDEC INC	Indicated for the treatment of spinal muscular atrophy in pediatric and adult patients.	12/23/2016	Yes	Yes

CY 2016 Orphan Designated BLA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
125509	DAIP	ANTHIM (OBILTOXAXIMAB)	ELUSYS THERAPEUTICS, INC.	Treatment of adult and pediatric patients with inhalational anthrax due to Bacillus anthracis in combination with appropriate antibacterial drugs and for prophylaxis of inhalational anthrax when alternative therapies are not available or are not appropriate.	3/18/2016	Yes	Yes
761038	DOP2	LARTRUVO (OLARATUMAB)	ELI LILLY AND COMPANY	Lartruvo is indicated, in combination with doxorubicin, for the treatment of adult patients with soft tissue sarcoma (STS) with a histologic subtype for which an anthracycline-containing regimen is appropriate and which is not amenable to curative treatment with radiotherapy or surgery.	10/19/2016	Yes	Yes

CY 2016 Orphan Designated Supplement Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
202714/10	DHP	KYPROLIS (CARFILZOMIB)	ONYX THERAPEUTICS INC A WHOLLY OWNED SUB OF AMGEN INC	Kyprolis (carfilzomib) is approved for the following indications: 1. In combination with dexamethasone or with lenalidomide plus dexamethasone for the treatment of patients with relapsed or refractory multiple myeloma who have received one to three lines of therapy 2. As a single agent for the treatment of patients with relapsed or refractory multiple myeloma who have received one or more lines of therapy	1/21/2016	Yes	Yes
201532/15	DOP2	HALAVEN (ERIBULIN MESYLATE)	EISAI INC	Halaven is a microtubule inhibitor indicated for the treatment of patients with unresectable or metastatic liposarcoma who have received a prior anthracycline-containing regimen.	1/28/2016	Yes	Yes
203496/2	DCRP	ORENITRAM (TREPROSTINIL)	UNITED THERAPEUTICS CORP	Orenitram is indicated for the treatment of pulmonary arterial hypertension (PAH) (WHO Group 1) to improve exercise capacity. The study that established effectiveness included predominately patients with WHO functional class II-III symptoms and etiologies of idiopathic or heritable PAH (75%) or PAH associated with connective tissue disease (19%). When used as the sole vasodilator, the effect of Orenitram on exercise is about 10% of the deficit, and the effect, if any, on a background of another vasodilator is probably less than this.	1/28/2016	Yes	Yes
203985/10	DOP2	AFINITOR DISPERZ (EVEROLIMUS)	NOVARTIS PHARMACEUTICALS CORP	Treatment of pediatric and adult patients with tuberous sclerosis complex (TSC) who have subependymal giant cell astrocytoma (SEGA) that requires therapeutic intervention but cannot be curatively resected.	1/29/2016	Yes	Yes

125486/13	DHP	GAZYVA (GA101, OBINUTUZUMAB, RO5072759)	GENENTECH, INC.	Gazyva, in combination with bendamustine followed by Gazyva monotherapy, is indicated for the treatment of patients with follicular lymphoma (FL) who relapsed after, or are refractory to, a rituximab-containing regimen.	2/26/2016	Yes	Yes
22334/36	DOP2	AFINITOR (EVEROLIMUS)	NOVARTIS PHARMACEUTICALS CORP	Treatment of adult patients with progressive, well-differentiated, non-functional, neuroendocrine tumors (NET) of gastrointestinal (GI) or lung origin with unresectable, locally advanced or metastatic disease.	2/26/2016	Yes	Yes
205552/7	DHP	IMBRUVICA (IBRUTINIB)	PHARMACYCLICS LLC	For the treatment of Chronic Lymphocytic Leukemia.	3/4/2016	Yes	Yes
201292/7	125	GILOTRIF (AFATINIB)	BOEHRINGER INGELHEIM PHARMACEUTICALS, INC.	Metastatic, squamous, non-small cell lung cancer progressing after platinum-based chemotherapy.	4/15/2016	Yes	Yes
205552/10	DHP	IMBRUVICA (IBRUTINIB)	PHARMACYCLICS LLC	Revised indication for the the treatment of patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL) revised indication for the treatment of patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL) with 17p deletion.	5/6/2016	Yes	Yes
205552/13	DHP	IMBRUVICA (IBRUTINIB)	PHARMACYCLICS LLC	Revised indication for the the treatment of patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL) revised indication for the treatment of patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL) with 17p deletion.	5/6/2016	Yes	Yes
125554/19	DHP	OPDIVO (NIVOLUMAB)	BRISTOL-MYERS SQUIBB COMPANY	Classical Hodgkin lymphoma that has relapsed or progressed after autologous hematopoietic stem cell transplantation (HSCT) and post-transplantation brentuximab vedotin.	5/17/2016	Yes	Yes
21366/33	DMEP	CRESTOR (ROSUVASTATIN CALCIUM)	IPR PHARMACEUTICALS INC	Pediatric patients 7 to 17 years of age with homozygous familial hypercholesterolemia (HoFH) to reduce LDL-C, total-C, nonHDL-C and ApoB as an adjunct to diet, either alone or with other lipid-lowering treatments.	5/27/2016	Yes	Yes
125057/397	DTOP	HUMIRA (ADALIMUMAB)	ABBVIE INC.	The treatment of noninfectious intermediate, posterior and panuveitis in adult patients.	6/30/2016	Yes	Yes
125274/105	DNP	DYSPORT (ABOBOTULINUMTOXIN A)	IPSEN BIOPHARMACEUTICALS LIMITED	Treatment of lower limb spasticity in pediatric patients 2 years of age and older.	7/29/2016	Yes	Yes
125326/63	DHP	ARZERRA (OFATUMUMAB)	NOVARTIS PHARMACEUTICALS CORPORATION	Arzerra in combination with fludarabine and cyclophosphamide for the treatment of patients with relapsed chronic lymphocytic leukemia (CLL).	8/30/2016	Yes	Yes
125319/85	DPARP	ILARIS (CANAKINUMAB)	NOVARTIS PHARMACEUTICALS CORPORATION	For the treatment of Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS) in adult and pediatric patients. For the treatment of Hyperimmunoglobulin D Syndrome (HIDS)/Mevalonate Kinase Deficiency (MKD) in adult and pediatric patients. For the treatment of Familial Mediterranean Fever (FMF) in adult and pediatric patients.	9/23/2016	Yes	Yes
125319/86	DPARP	ILARIS (CANAKINUMAB)	NOVARTIS PHARMACEUTICALS CORPORATION	For the treatment of Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS) in adult and pediatric patients. For the treatment of Hyperimmunoglobulin D Syndrome (HIDS)/Mevalonate Kinase Deficiency (MKD) in adult and pediatric patients. For the treatment of Familial Mediterranean Fever (FMF) in adult and pediatric patients.	9/23/2016	Yes	Yes

125319/87	DPARP	ILARIS (CANAKINUMAB)	NOVARTIS PHARMACEUTICALS CORPORATION	For the treatment of Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS) in adult and pediatric patients. For the treatment of Hyperimmunoglobulin D Syndrome (HIDS)/Mevalonate Kinase Deficiency (MKD) in adult and pediatric patients. For the treatment of Familial Mediterranean Fever (FMF) in adult and pediatric patients.	9/23/2016	Yes	Yes
761036/3	DHP	DARZALEX (DARATUMUMAB)	JANSSEN BIOTECH, INC.	Darzalex in combination with lenalidomide and dexamethasone for the treatment of patients with multiple myeloma who have received at least one prior therapy.	11/21/2016	Yes	Yes
761036/4	DHP	DARZALEX (DARATUMUMAB)	JANSSEN BIOTECH, INC.	Darzalex in combination with bortezomib and dexamethasone for the treatment of patients with multiple myeloma who have received at least one prior therapy.	11/21/2016	Yes	Yes
125085/317	DOP1	AVASTIN (BEVACIZUMAB)	GENENTECH, INC.	Avastin, either in combination with carboplatin and paclitaxel or in combination with carboplatin and gemcitabine, followed by Avastin as a single agent, is indicated for the treatment of patients with platinum-sensitive recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer.	12/6/2016	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.*