CDER Rare Disease And Orphan Drug Designated Approvals

CY 2013 Orphan Designated NDA Approvals

Application Number	Review Division	Drug Name	Sponsor Name	Approved Indication	Approval Date	ORPHAN†	RARE DISEASE‡
203568	DMEP	KYNAMRO (MIPOMERSEN SODIUM) INJECTION	GENZYME CORP	Indicated as an adjunct to lipid-lowering medications and diet to reduce low density lipoprotein-cholesterol (LDL-C), apolipoprotein B (apo B), total cholesterol (TC), and non-high density lipoprotein-cholesterol (non HDL-C) in patients with homozygous familial hypercholesterolemia (HoFH).	1/29/2013	Yes	Yes
203284	DGIEP	RAVICTI (GLYCEROL PHENYLBUTYRATE)	HYPERION THERAPEUTICS INC	Indicated for use as a nitrogen-binding agent for chronic management of adult and pediatric patients ≥2 years of age with urea cycle disorders (UCDs) that cannot be managed by dietary protein restriction and/or amino acid supplementation alone. Ravicti must be used with dietary protein restriction and, in some cases, dietary supplements (eg, essential amino acids, arginine, citrulline, protein-free calorie supplements). Limitation of use: ▪ Not indicated for treatment of acute hyperammonemia in patients with UCDs. ▪ Safety and efficacy for treatment of N-acetylglutamate synthase (NAGS) deficiency has not been established. ▪ The use of Ravicti in patients <2 months of age is contraindicated.	2/1/2013	Yes	Yes
204026	DHP	POMALYST (POMALIDOMIDE)	CELGENE CORP	Indicated for patients with multiple myeloma who have received at least two prior therapies including lenalidomide and bortezomib and have demonstrated disease progression on or within 60 days of completion of the last therapy. Approval is based on response rate. Clinical benefit, such as improvement in survival or symptoms, has not been verified.	2/8/2013	Yes	Yes
203389	DGIEP	PROCYSBI (CYSTEAMINE BITARTRATE)	RAPTOR THERAPEUTICS, INC.	Indicated for the management of nephropathic cystinosis in adults and children ages 6 years and older.	4/30/2013	Yes	Yes
203340	DNP	NYMALIZE (NIMODIPINE)	ARBOR PHARMACEUTICALS LLC	Indicated for the improvement of neurological outcome by reducing the incidence and severity of ischemic deficits in adult patients with subarachnoid hemorrhage (SAH) from ruptured intracranial berry aneurysms regardless of their post-ictus neurological condition (i.e., Hunt and Hess Grades I-V).	5/10/2013	Yes	Yes
202806	DOP2	TAFINLAR (DABRAFENIB)	GLAXOSMITHKLINE INTELLECTUAL PROPERTY NO 2 LTD ENGLAND	Indicated for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E mutation as detected by an FDA-approved test. Limitation of use: Tafinlar is not indicated for treatment of patients with wild-type BRAF melanoma.	5/29/2013	Yes	Yes
204114	DOP2	MEKINIST (TRAMETINIB)	GLAXOSMITHKLINE INTELLECTUAL PROPERTY NO 2 LTD ENGLAND	Indicated for the treatment of patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutations as detected by an FDA-approved test. Limitation of use: Mekinist is not indicated for the treatment of patients who have received prior BRAF inhibitor therapy.	5/29/2013	Yes	Yes
201292	DOP2	GILOTRIF (AFATINIB)	BOEHRINGER INGELHEIM	Indicated 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. Limitation of Use: Safety and efficacy of Gilotrif have not been established in patients whose tumors have other EGFR mutations.	7/12/2013	Yes	Yes

CDER Rare Disease And Orphan Drug Designated Approvals

204308	DCRP	EPANED (ENALAPRIL)	SILVERGATE PHARMACEUTICALS INC	Treatment of hypertension in adults and children older than one month, to lower blood pressure. Lowering blood pressure reduces the risk of fatal and nonfatal cardiovascular events, primarily strokes and myocardial infarctions.	8/13/2013	Yes	Yes
202317	DHP	VALCHLOR (MECHLORETHAMINE)	ACTELION PHARMACEUTICALS LTD	Indicated for the topical treatment of Stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma in patients who have received prior skin-directed therapy.	8/23/2013	Yes	Yes
204819	DCRP	ADEMPAS (RIOCIGUAT)	BAYER HEALTHCARE PHARMACEUTICALS INC	Indicated for the treatment of adults with: • Persistent/recurrent Chronic Thromboembolic Pulmonary Hypertension (CTEPH) (WHO Group 4) after surgical treatment or inoperable CTEPH to improve exercise capacity and WHO functional class.	10/8/2013	Yes	Yes
204819	DCRP	ADEMPAS (RIOCIGUAT)	BAYER HEALTHCARE PHARMACEUTICALS INC	Indicated for the treatment of adults with Pulmonary Arterial Hypertension (PAH) (WHO Group 1) to improve exercise capacity, improve WHO functional class and to delay clinical worsening.	10/8/2013	Yes	Yes
204410	DCRP	OPSUMIT (MACITENTAN)	ACTELION PHARMACEUTICALS LTD	Indicated for the treatment of pulmonary arterial hypertension (PAH, WHO Group I) to delay disease progression. Disease progression included: death, initiation of intravenous (IV) or subcutaneous prostanoids, or clinical worsening of PAH (decreased 6-minute walk distance, worsened PAH symptoms and need for additional PAH treatment). Opsumit also reduced hospitalization for PAH.	10/18/2013	Yes	Yes
205552	DHP	IMBRUVICA (IBRUTINIB)	PHARMACYCLICS INC	Indicated for the treatment of patients with mantle cell lymphoma (MCL) who have received at least one prior therapy. This indication is based on overall response rate. An improvement in survival or disease-related symptoms has not been established.	11/13/2013	Yes	Yes
205065	DGIEP	KUVAN (SAPROPTERIN)	BIOMARIN PHARMACEUTICAL INC	Indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). Kuvan is to be used in conjunction with a Phe-restricted diet.	12/19/2013	Yes	Yes
203496	DCRP	ORENITRAM (TREPROSTINIL)	UNITED THERAPEUTICS CORP	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%). As the sole vasodilator, the effect on exercise is small. Orenitram has not been shown to add to other vasodilator therapy.	12/20/2013	Yes	Yes

CY 2013 Orphan Designated BLA Approvals

Application Number	Review Division	Drug Name	Sponsor Name		Approval Date	ORPHAN†	RARE DISEASE‡
125486	DHP	GAZYVA (OBINUTUZUMAB)	GENENTECH, INC.	Indicated, in combination with chlorambucil, for the treatment of patients with previously untreated chronic lymphocytic leukemia.	11/1/2013	Yes	Yes

CDER Rare Disease And Orphan Drug Designated Approvals

CY 2013 Orphan Designated Supplement Approvals

Application Number	Review Division	Drug Name	Sponsor Name		Approval Date	ORPHAN[†]	RARE DISEASE[‡]
021588/37	DHP	GLEEVEC (IMATINIB)	NOVARTIS PHARMACEUTICALS CORP	New indication: Indicated for the treatment of: • Adult patients with relapsed or refractory Philadelphia chromosome positive acute lymphoblastic leukemia (Ph+ ALL).	1/25/2013	Yes	Yes
204369	DOP2	STIVARGA (REGORAFENIB)	BAYER HEALTHCARE PHARMACEUTICALS INC	New indication: Indicated for the treatment of patients with: • Locally advanced, unresectable or metastatic gastrointestinal stromal tumor (GIST) who have been previously treated with imatinib mesylate and sunitinib malate.	2/25/2013	Yes	Yes
125276/64	DPARP	ACTEMRA (TOCILIZUMAB)	GENENTECH INC	New Indicaton: Indicated for treatment of: • Polyarticular Juvenile Idiopathic Arthritis (PJIA): Patients 2 years of age and older with active polyarticular juvenile idiopathic arthritis.	4/29/2013	Yes	Yes
125319/62	DPARP	ILARIS (CANAKINUMAB)	NOVARTIS PHARMACEUTICLS CORP	New Indication: Indicated for the treatment of: • Active Systemic Juvenile Idiopathic Arthritis (SJIA) in patients aged 2 years and older.	5/9/2013	Yes	Yes
021880/34	DHP	REVLIMID (LENALIDOMIDE)	CELGENE CORP	New Indication: Indicated for the treatment of patients with: • Mantle cell lymphoma (MCL) whose disease has relapsed or progressed after two prior therapies, one of which included bortezomib.	6/5/2013	Yes	Yes
125320/94	DRUP	XGEVA (DENOSUMAB)	AMGEN INC	New Indicaton: Indicated for: • 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.	6/13/2013	Yes	Yes
021660/37	DOP2	ABRAXANE (PACLITAXEL PROTEIN BOUND PARTICLES)	ABRAXIS BIOSCIENCE	New Indication: Indicated for the treatment of: • Metastatic adenocarcinoma of the pancreas as first-line treatment, in combination with gemcitabine.	9/6/2013	Yes	Yes
022006/12	DNP	SABRIL (VIGABATRIN)	LUNDBECK LLC	Indicated for: • Refractory Complex Partial Seizures in patients ≥10 years of age. It should be used as adjunctive therapy in patients who have responded inadequately to several alternative treatments. • Infantile Spasms - monotherapy in infants 1 month to 2 years of age.	10/26/2013	Yes	Yes
022575/12	DGIEP	VPRIV (VELAGLUCERASE ALPHA)	SHIRE GENETIC THERAPIES	Indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.	11/21/2013	Yes	Yes
021923/16	DOP1	NEXAVAR (SORAFENIB)	BAYER HEALTHCARE PHARMACEUTICALS INC	New Indication: Indicated for the treatment of: • Locally recurrent or metastatic, progressive, differentiated thyroid carcinoma refractory to ra dioactive iodine treatment.	11/22/2013	Yes	Yes
125338/61	DPARP	XIAFLEX (COLLAGENASE CLOSTRIDIUM HISTOLYTICUM)	AUXILIUM PHARMACEUTICALS INC	New Indication: Indicated for: • The treatment of adult men with Peyronie's disease with a palpable plaque and curvature deformity of at least 30 degrees at the start of therapy.	12/6/2013	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.*