CDER Breakthrough Therapy Designation Approvals

As of December 31, 2018

Total of 130 Approvals

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant*	Approval Date	Use
BLA 761116	ORIGINAL-1	ELZONRIS	TAGRAXOFUSP-ERZS	STEMLINE THERAPEUTICS INC	21-Dec-2018	Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and in pediatric patients 2 years and older
BLA 125514	SUPPLEMENT-45	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	19-Dec-2018	Treatment of adult and pediatric patients with recurrent locally advanced or metastatic Merkel cell carcinoma
NDA 208078	ORIGINAL-1	FIRDAPSE	AMIFAMPRIDINE	CATALYST PHARMACEUTICALS INC	28-Nov-2018	Treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults
NDA 210861	ORIGINAL-1	VITRAKVI	LAROTRECTINIB	LOXO ONCOLOGY INC	26-Nov-2018	Treatment of adult and pediatric patients with solid tumors that have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation; are metastatic or where surgical resection is likely to result in severe morbidity; and have no satisfactory alternative treatments or that have progressed following treatment
NDA 211710	ORIGINAL-1	VITRAKVI	LAROTRECTINIB	LOXO ONCOLOGY INC	26-Nov-2018	Treatment of adult and pediatric patients with solid tumors that have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation; are metastatic or where surgical resection is likely to result in severe morbidity; and have no satisfactory alternative treatments or that have progressed following treatment

NDA 208573	SUPPLEMENT-9	VENCLEXTA	VENETOCLAX	ABBVIE INC	21-Nov-2018	In combination with azacitidine or decitabine or low-dose cytarabine for the treatment of newly-diagnosed acute myeloid leukemia (AML) in adults who are age 75 years or older, or who have comorbidities that preclude use of intensive induction chemotherapy
BLA 761107	ORIGINAL-1	GAMIFANT	EMAPALUMAB-LZSG	NOVIMMUNE SA	20-Nov-2018	Treatment of adult and pediatric (newborn and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy
BLA 125388	SUPPLEMENT-99	ADCETRIS	BRENTUXIMAB VEDOTIN	SEATTLE GENETICS INC	16-Nov-2018	Treatment of adult patients with previously untreated systemic anaplastic large cell lymphoma or other CD30-expressing peripheral T-cell lymphomas (PTCL), including angioimmunoblastic T-cell lymphoma and PTCL not otherwise specified, in combination with cyclophosphamide, doxorubicin, and prednisone
NDA 022291	SUPPLEMENT-21	PROMACTA	ELTROMBOPAG	NOVARTIS PHARMACEUTICALS CORP	16-Nov-2018	In combination with standard immunosuppressive therapy for the first-line treatment of adult and pediatric patients 2 years and older with severe aplastic anemia
NDA 210868	ORIGINAL-1	LORBRENA	LORLATINIB	PFIZER INC	02-Nov-2018	Treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer (NSCLC) whose disease has progressed on: • crizotinib and at least one other ALK inhibitor for metastatic disease; or • alectinib as the first ALK inhibitor therapy for metastatic disease; or • ceritinib as the first ALK inhibitor therapy for metastatic disease

BLA 761097	ORIGINAL-1	LIBTAYO	CEMIPLIMAB-RWLC	REGENERON PHARMACEUTICALS INC	28-Sep-2018	Treatment of patients with metastatic cutaneous squamous cell carcinoma (CSCC) or locally advanced CSCC who are not candidates for curative surgery or curative radiation
NDA 207356	ORIGINAL-1	ARIKAYCE	AMIKACIN LIPOSOME INHALATION SUSPENSION	INSMED INC	28-Sep-2018	Treatment of Mycobacterium avium complex (MAC) lung disease as part of a combination antibacterial drug regimen in patients who do not achieve negative sputum cultures after a minimum of 6 consecutive months of a multidrug background regimen therapy. As only limited clinical safety and effectiveness data for ARIKAYCE are currently available, reserve ARIKAYCE for use in adults who have limited or no alternative treatment options. This drug is indicated for use in a limited and specific population of patients.
BLA 761090	ORIGINAL-1	TAKHZYRO	LANADELUMAB-FLYO	DYAX CORP	23-Aug-2018	Prophylaxis to prevent attacks of hereditary angioedema (HAE) in patients 12 years and older.
BLA 761094	ORIGINAL-1	OXERVATE	CENEGERMIN-BKBJ	DOMPE FARMACEUTICI SPA	22-Aug-2018	Treatment of neurotrophic keratitis
NDA 210922	ORIGINAL-1	ONPATTRO	PATISIRAN	ALNYLAM PHARMACEUTICALS INC	10-Aug-2018	Treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults
BLA 761051	ORIGINAL-1	POTELIGEO	MOGAMULIZUMAB-KPKC	KYOWA KIRIN INC	08-Aug-2018	Treatment of adult patients with relapsed or refractory mycosis fungoides (MF) or Sézary syndrome (SS) after at least one prior systemic therapy
NDA 211358	ORIGINAL-1	ORKAMBI	LUMACAFTOR/ IVACAFTOR	VERTEX PHARMACEUTICALS INC	07-Aug-2018	Treatment of cystic fibrosis (CF) in patients 2 years and older, homozygous for the F508del-CFTR mutation in the CFTR gene

NDA 209607	ORIGINAL-1	AZEDRA	IOBENGUANE I 131	PROGENICS PHARMACEUTICALS INC	30-Jul-2018	Treatment of adult and pediatric patients 12 years and older with iobenguane scan positive, unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma who require systemic anticancer therapy
NDA 210795	ORIGINAL-1	KRINTAFEL	TAFENOQUINE	GLAXOSMITHKLINE INTELLECTUAL PROPERTY DEVELOPMENT LTD ENGLAND	20-Jul-2018	For the radical cure (prevention of relapse) of Plasmodium vivax malaria in patients aged 16 years and older who are receiving appropriate antimalarial therapy for acute P. vivax infection
NDA 209092	SUPPLEMENT-1	KISQALI	RIBOCICLIB	NOVARTIS PHARMACEUTICALS CORP	18-Jul-2018	In combination with an aromatase inhibitor for the treatment of pre/perimenopausal or postmenopausal women, with hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced or metastatic breast cancer, as initial endocrine-based therapy
BLA 125377	SUPPLEMENT-96	YERVOY	IPILIMUMAB	BRISTOL-MYERS SQUIBB COMPANY	10-Jul-2018	In combination with nivolumab, for the treatment of adults and pediatric patients 12 years and older with microsatellite instability-high (MSI H) or DNA mismatch repair deficient (dMMR), metastatic colorectal cancer that has progressed following treatment with a fluoropyrimidine, oxaliplatin, and irinotecan
BLA 125554	SUPPLEMENT-63	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	10-Jul-2018	In combination with ipilimumab, for the treatment of adults and pediatric patients 12 years and older with microsatellite instability-high (MSI H) or DNA mismatch repair deficient (dMMR), metastatic colorectal cancer that has progressed following treatment with a fluoropyrimidine, oxaliplatin, and irinotecan

BLA 125514	SUPPLEMENT-30	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	13-Jun-2018	Treatment of adult and pediatric patients with refractory primary mediastinal large B-cell lymphoma (PMBCL), or who have relapsed after 2 or more prior lines of therapy
NDA 208573	SUPPLEMENT-4	VENCLEXTA	VENETOCLAX	ABBVIE INC	08-Jun-2018	Treatment of patients with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL), with or without 17p deletion, who have received at least one prior therapy
BLA 103705	SUPPLEMENT-5450	RITUXAN	RITUXIMAB	GENENTECH INC	07-Jun-2018	Treatment of adult patients with moderate to severe pemphigus vulgaris (PV)
NDA 22527	SUPPLEMENT-24	GILENYA	FINGOLIMOD	NOVARTIS PHARMACEUTICALS CORP	11-May-2018	Treatment of relapsing forms of multiple sclerosis to include pediatric patients 10 years of age and above
NDA 202806	SUPPLEMENT-10	TAFINLAR	DABRAFENIB	NOVARTIS PHARMACEUTICALS CORP	04-May-2018	In combination with trametinib, for the treatment of patients with locally advanced or metastatic anaplastic thyroid cancer (ATC) with BRAF V600E mutation and with no satisfactory locoregional treatment options
NDA 204114	SUPPLEMENT-9	MEKINIST	TRAMETINIB	NOVARTIS PHARMACEUTICALS CORP	04-May-2018	In combination with dabrafenib, for the treatment of patients with locally advanced or metastatic anaplastic thyroid cancer (ATC) with BRAF V600E mutation and with no satisfactory locoregional treatment options
NDA 202806	SUPPLEMENT-8	TAFINLAR	DABRAFENIB	NOVARTIS PHARMACEUTICALS CORP	30-Apr-2018	In combination with trametinib, for the adjuvant treatment of patients with melanoma with BRAF V600E or V600K mutations, as detected by an FDA-approved test, and involvement of lymph node(s), following complete resection

NDA 204114	SUPPLEMENT-7	MEKINIST	TRAMETINIB	NOVARTIS PHARMACEUTICALS CORP	30-Apr-2018	In combination with dabrafenib, for the adjuvant treatment of patients with melanoma with BRAF V600E or V600K mutations as detected by an FDA-approved test, and pathologic involvement of lymph node(s), following complete resection
NDA 208065	SUPPLEMENT-8	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	18-Apr-2018	For 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
BLA 761068	ORIGINAL-1	CRYSVITA	BUROSUMAB	ULTRAGENYX PHARAMCEUTICAL INC	17-Apr-2018	Treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients 1 year of age and older
BLA 125377	SUPPLEMENT-94	YERVOY	IPILIMUMAB	BRISTOL-MYERS SQUIBB COMPANY	16-Apr-2018	In combination with nivolumab, for treatment of patients with intermediate or poor risk, previously untreated advanced renal cell carcinoma (RCC)
BLA 125554	SUPPLEMENT-58	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	16-Apr-2018	In combination with ipilimumab, for treatment of patients with intermediate or poor risk, previously untreated advanced renal cell carcinoma (RCC)
BLA 125388	SUPPLEMENT-97	ADCETRIS	BRENTUXIMAB VEDOTIN	SEATTLE GENETICS INC	20-Mar-2018	Treatment for adult patients with previously untreated Stage III or IV classical Hodgkin lymphoma, in combination with chemotherapy
BLA 761065	ORIGINAL-1	TROGARZO	IBALIZUMAB	THERATECHNOLOGIES INC	06-Mar-2018	Treatment of human immunodeficiency virus type 1 (HIV-1) infection in heavily treatment-experienced adults with multidrug resistant HIV-1 infection failing their current antiretroviral regimen

BLA 761069	SUPPLEMENT-2	IMFINZI	DURVALUMAB	ASTRAZENECA UK LTD	16-Feb-2018	Treatment of patients with unresectable Stage III non-small cell lung cancer (NSCLC) whose disease has not progressed following concurrent platinum-based chemotherapy and radiation therapy
NDA 210491	ORIGINAL-1	SYMDEKO	TEZACAFTOR AND IVACAFTOR	VERTEX PHARMACEUTICALS INC	12-Feb-2018	Treatment of patients with cystic fibrosis (CF) aged 12 years and older who are homozygous for the F508del mutation or who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to tezacaftor/ivacaftor based on in vitro data and/or clinical evidence
BLA 125554	SUPPLEMENT-55	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	20-Dec-2017	Adjuvant treatment of patients with melanoma with involvement of lymph nodes or metastatic disease who have undergone complete resection
BLA 761083	ORIGINAL-1	HEMLIBRA	EMICIZUMAB-KXWH	GENENTECH INC	16-Nov-2017	Prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients with hemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors
BLA 125388	SUPPLEMENT-94	ADCETRIS	BRENTUXIMAB VEDOTIN	SEATTLE GENETICS INC	09-Nov-2017	Treatment of patients with primary cutaneous anaplastic large cell lymphoma or CD30-expressing mycosis fungoides who have received prior systemic therapy
NDA 209939	ORIGINAL-1	PREVYMIS	LETERMOVIR	MERCK SHARP AND DOHME CORP	08-Nov-2017	Prophylaxis of cytomegalovirus (CMV) infection and disease in adult CMV-seropositive recipients [R+] of an allogeneic hematopoietic stem cell transplant (HSCT)
NDA 209940	ORIGINAL-1	PREVYMIS	LETERMOVIR	MERCK SHARP AND DOHME CORP	08-Nov-2017	Prophylaxis of cytomegalovirus (CMV) infection and disease in adult CMV-seropositive recipients [R+] of an allogeneic hematopoietic stem cell transplant (HSCT)

NDA 202429	SUPPLEMENT-16	ZELBORAF	VEMURAFENIB	HOFFMANN-LA ROCHE INC	06-Nov-2017	Treatment of Erdheim-Chester disease with BRAF V600 mutation
NDA 208434	SUPPLEMENT-3	ALECENSA	ALECTINIB	HOFFMANN-LA ROCHE INC	06-Nov-2017	Treatment of patients with anaplastic lymphoma kinase (ALK)-positive, metastatic non-small cell lung cancer (NSCLC) as detected by an FDA-approved test
NDA 210259	ORIGINAL-1	CALQUENCE	ACALABRUTINIB	ASTRAZENECA UK LTD	31-Oct-2017	Treatment of adult patients with mantle cell lymphoma (MCL) who have received at least one prior therapy
NDA 208716	ORIGINAL-1	VERZENIO	ABEMACICLIB	ELI LILLY AND CO	28-Sep-2017	Treatment of adult patients with HR-positive, HER2-negative advanced or metastatic breast cancer with disease progression following endocrine therapy and prior chemotherapy in the metastatic setting
NDA 209885	ORIGINAL-1	AUSTEDO	DEUTETRABENAZINE	TEVA BRANDED PHARMACEUTICAL PRODUCTS R AND D INC	30-Aug-2017	Treatment of tardive dyskinesia
BLA 761040	ORIGINAL-1	BESPONSA	INOTUZUMAB OZOGAMICIN	WYETH PHARMACEUTICALS INC	17-Aug-2017	Treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
NDA 209394	ORIGINAL-1	MAVYRET	GLECAPREVIR AND PIBRENTASVIR	ABBVIE INC	03-Aug-2017	Treatment of patients with chronic hepatitis C virus (HCV) genotype (GT) 1, 2, 3, 4, 5 or 6 infection without cirrhosis or with compensated cirrhosis; and also for patients with HCV GT1 infection who previously have been treated with a regimen containing an HCV NS5A inhibitor or an NS3/4A protease inhibitor, but not both
NDA 209401	ORIGINAL-1	VYXEOS	CYTARABINE AND DAUNORUBICIN	CELATOR PHARMACEUTICALS INC	03-Aug-2017	Treatment of adults with newly-diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC)
NDA 205552	SUPPLEMENT-17	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	02-Aug-2017	Treatment of adult patients with chronic graft versus host disease (cGVHD) after failure of one or more lines of systemic therapy

NDA 202806	SUPPLEMENT-6	TAFINLAR	DABRAFENIB	NOVARTIS PHARMACEUTICALS CORP	22-Jun-2017	Treatment of patients with metastatic non-small cell lung cancer (NSCLC) with BRAF V600E mutation as detected by an FDA-approved test
NDA 204114	SUPPLEMENT-5	MEKINIST	TRAMETINIB	NOVARTIS PHARMACEUTICALS CORP	22-Jun-2017	Treatment of patients with metastatic non-small cell lung cancer (NSCLC) with BRAF V600E mutation as detected by an FDA-approved test
NDA 205755	SUPPLEMENT-9	ZYKADIA	CERITINIB	NOVARTIS PHARMACEUTICALS CORP	26-May-2017	Treatment of patients with metastatic non-small cell lung cancer (NSCLC) whose tumors are anaplastic lymphoma kinase (ALK)-positive as detected by an FDA-approved test
BLA 125514	SUPPLEMENT-14	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	23-May-2017	Treatment of adult and pediatric patients with: • unresectable or metastatic, microsatellite instability-high (MSI-H) or mismatch repair deficient solid tumors that have progressed following prior treatment and who have no satisfactory alternative treatment options, or • metastatic, microsatellite instability-high (MSI-H) or mismatch repair deficient colorectal cancer that has progressed following treatment with a fluoropyrimidine, oxaliplatin, and irinotecan
BLA 125472	SUPPLEMENT-24	ACTEMRA	TOCILIZUMAB	GENENTECH INC	22-May-2017	Treatment of adult patients with giant cell arteritis (GCA)
BLA 125514	SUPPLEMENT-18	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	18-May-2017	Treatment of patients with locally advanced or metastatic urothelial carcinoma who have disease progression during or following platinum-containing chemotherapy or within 12 months of neoadjuvant or adjuvant treatment with platinum-containing chemotherapy
NDA 209935	ORIGINAL-1	KISQALI FEMARA CO-PACK	LETROZOLE AND RIBOCICLIB	NOVARTIS PHARMACEUTICALS CORP	04-May-2017	Treatment of postmenopausal women with hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced or metastatic breast cancer

BLA 761069	ORIGINAL-1	IMFINZI	DURVALUMAB	ASTRAZENECA UK LTD	01-May-2017	Treatment of patients with locally advanced or metastatic urothelial carcinoma who have disease progression during or following platinum-containing chemotherapy or have disease progression within 12 months of neoadjuvant or adjuvant treatment with platinum-containing chemotherapy
NDA 207997	ORIGINAL-2	RYDAPT	MIDOSTAURIN	NOVARTIS PHARMACEUTICALS CORP	28-Apr-2017	Treatment of adult patients with newly diagnosed acute myeloid leukemia (AML) that is FLT3 mutation-positive as detected by an FDA approved test, in combination with standard cytarabine and daunorubicin induction and cytarabine consolidation
NDA 208772	ORIGINAL-1	ALUNBRIG	BRIGATINIB	ARIAD PHARMACEUTICALS INC	28-Apr-2017	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
BLA 761052	ORIGINAL-1	BRINEURA	CERLIPONASE ALFA	BIOMARIN PHARMACEUTICAL INC	27-Apr-2017	Treatment of pediatric patients 3 years of age and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency
NDA 209241	ORIGINAL-1	INGREZZA	VALBENAZINE	NEUROCRINE BIOSCIENCES INC	11-Apr-2017	Treatment of tardive dyskinesia
NDA 207103	SUPPLEMENT-4	IBRANCE	PALBOCICLIB	PFIZER INC	31-Mar-2017	Treatment of postmenopausal women with hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced or metastatic breast cancer, In combination with an aromatase inhibitor as initial endocrine-based therapy
BLA 761053	ORIGINAL-1	OCREVUS	OCRELIZUMAB	GENENTECH INC	28-Mar-2017	Treatment of adult patients with relapsing or primary progressive forms of multiple sclerosis

BLA 761055	ORIGINAL-1	DUPIXENT	DUPILUMAB	REGENERON PHARMACEUTICALS INC	28-Mar-2017	Treatment of adult patients with moderate-to-severe atopic dermatitis whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable. DUPIXENT can be used with or without topical corticosteroids
NDA 208447	ORIGINAL-1	ZEJULA	NIRAPARIB	TESARO INC	27-Mar-2017	Maintenance treatment of adult patients with recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in a complete or partial response to platinum-based chemotherapy
BLA 761049	ORIGINAL-1	BAVENCIO	AVELUMAB	EMD SERONO INC	23-Mar-2017	Treatment of adults and pediatric patients 12 years and older with metastatic Merkel cell carcinoma (MCC)
BLA 125514	SUPPLEMENT-15	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	14-Mar-2017	Treatment of patients with hematological malignancies: Hodgkin Lymphoma
NDA 209092	ORIGINAL-1	KISQALI	RIBOCICLIB	NOVARTIS PHARMACEUTICALS CORP	13-Mar-2017	Treatment of postmenopausal women with hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced or metastatic breast cancer as initial endocrine-based therapy
BLA 125554	SUPPLEMENT-24	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	02-Feb-2017	Treatment of locally advanced or metastatic urothelial carcinoma in patients who have disease progression during or following platinum-containing chemotherapy; have disease progression within 12 months of neoadjuvant or adjuvant treatment with platinum-containing chemotherapy
NDA 209115	ORIGINAL-1	RUBRACA	RUCAPARIB	CLOVIS ONCOLOGY INC	19-Dec-2016	Treatment of patients with deleterious BRCA mutation (germline and/or somatic) associated advanced ovarian cancer who have been treated with two or more chemotherapies

BLA 761036	SUPPLEMENT-3	DARZALEX	DARATUMUMAB	JANSSEN BIOTECH INC	21-Nov-2016	Treatment of patients with multiple myeloma who have received at least one prior therapy, in combination with lenalidomide and dexamethasone
BLA 761036	SUPPLEMENT-4	DARZALEX	DARATUMUMAB	JANSSEN BIOTECH INC	21-Nov-2016	Treatment of patients with multiple myeloma who have received at least one prior therapy, combination with bortezomib and dexamethasone
BLA 125554	SUPPLEMENT-22	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	10-Nov-2016	Treatment of patients with recurrent or metastatic squamous cell carcinoma of the head and neck (SCCHN) with disease progression on or after platinum based therapy
BLA 761038	ORIGINAL-1	LARTRUVO	OLARATUMAB	ELI LILLY AND COMPANY	19-Oct-2016	Treatment of adult patients with soft tissue sarcoma (STS) with a histologic subtype, in combination with doxorubicin, for which an anthracycline-containing regimen is appropriate and which is not amenable to curative treatment with radiotherapy or surgery
BLA 761041	ORIGINAL-1	TECENTRIQ	ATEZOLIZUMAB	GENENTECH INC	18-Oct-2016	Treatment of patients with metastatic non-small cell lung cancer who have disease progression during or following platinum-containing chemotherapy
NDA 206038	SUPPLEMENT-5	ORKAMBI	LUMACAFTOR and IVACAFTOR	VERTEX PHARMACEUTICALS INC	28-Sep-2016	Treatment of cystic fibrosis patients who are homozygous for the F508del mutation in the CFTR gene, ages 6-11
BLA 125319	SUPPLEMENT-85	ILARIS	CANAKINUMAB	NOVARTIS PHARMACEUTICALS CORPORATION	23-Sep-2016	Treatment of Tumor Necrosis Factor Receptor Associated Periodic Syndrome (TRAPS)
BLA 125319	SUPPLEMENT-86	ILARIS	CANAKINUMAB	NOVARTIS PHARMACEUTICALS CORPORATION	23-Sep-2016	Treatment of Hyperimmunoglubulin D Syndrome (HIDS)
BLA 125319	SUPPLEMENT-87	ILARIS	CANAKINUMAB	NOVARTIS PHARMACEUTICALS CORPORATION	23-Sep-2016	Treatment of Familial Mediterranean Fever (FMF)
NDA 208341	ORIGINAL-1	EPCLUSA	SOFOSBUVIR and VELPATASVIR	GILEAD SCIENCES INC	28-Jun-2016	Treatment of adult patients with chronic hepatitis C virus (HCV) genotypes 1, 2, 3, 4, 5 or 6 infection: - without cirrhosis or with compensated cirrhosis - with decompensated cirrhosis for use in combination with ribavirin

BLA 761034	ORIGINAL-1	TECENTRIQ	ATEZOLIZUMAB	GENENTECH INC	18-May-2016	Treatment of locally advanced or metastatic urothelial carcinoma who have disease progression during or following platinum-containing chemotherapy or have disease progression within 12 months of neoadjuvant or adjuvant treatment with platinum-containing chemotherapy
BLA 125554	SUPPLEMENT-19	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	17-May-2016	Treatment of Hodgkin Lymphoma
NDA 206947	SUPPLEMENT-3	LENVIMA	LENVATINIB MESYLATEL	EISAI INC	13-May-2016	Treatment of renal cell cancer (RCC): in combination with everolimus, for patients with advanced RCC following one prior anti-angiogenic therapy
NDA 207318	ORIGINAL-1	NUPLAZID	PIMAVANSERIN	ACADIA PHARMACEUTICALS INC	29-Apr-2016	Treatment of hallucinations and delusions associated with Parkinson's disease psychosis
NDA 208692	ORIGINAL-1	CABOTETYX	CABOZANTINIB	EXELIXIS INC	25-Apr-2016	Treatment of patients with advanced renal cell carcinoma (RCC) who have received prior anti-angiogenic therapy
NDA 208573	ORIGINAL-1	VENCLEXTA	VENETOCLAX	ABBVIE INC	11-Apr-2016	Treatment of patients with chronic lymphocytic leukemia (CLL) with 17p deletion who have received at least one prior therapy
NDA 202570	SUPPLEMENT-16	XALKORI	CRIZOTINIB	PF PRISM CV	11-Mar-2016	Treatment of patients with non-small cell lung cancer whose tumors are ROS-1 positive
NDA 207103	SUPPLEMENT-2	IBRANCE	PALBOCICLIB	PFIZER INC	19-Feb-2016	Treatment hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced or metastatic breast cancer in combination with fulvestrant in women with disease progression following endocrine therapy
NDA 208261	ORIGINAL-1	ZEPATIER	GRAZOPREVIR and ELBASVIR	MERCK SHARP AND DOHME CORP	28-Jan-2016	Treatment of chronic hepatitis C virus (HCV) genotypes 1 and 4 infections in adults
BLA 125514	SUPPLEMENT-6	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	18-Dec-2015	Treatment of patients with unresectable or metastatic melanoma
NDA 208434	ORIGINAL-1	ALECENSA	ALECTINIB	HOFFMANN-LA ROCHE INC	11-Dec-2015	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

BLA 125561	ORIGINAL-1	KANUMA	SEBELIPASE ALFA	SYNAGEVA BIOPHARMA CORP	08-Dec-2015	Treatment of patients with a diagnosis of lysosomal acid lipase (LAL) deficiency
BLA 761035	ORIGINAL-1	EMPLICITI	ELOTUZUMAB	BRISTOL-MYERS SQUIBB COMPANY	30-Nov-2015	Treatment of patients with multiple myeloma who have received one to three prior therapies
BLA 125554	SUPPLEMENT-12	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	23-Nov-2015	Treatment of advanced renal cell carcinoma patients who have received prior antiangiogenic therapy
BLA 761036	ORIGINAL-1	DARZALEX	DARATUMUMAB	JANSSEN BIOTECH INC	16-Nov-2015	Treatment of patients with multiple myeloma who have received at least 3 prior lines of therapy including a proteasome inhibitor and an immunomodulatory agent or are double refractory to a proteasome inhibitor and an immunomodulatory agent
NDA 208065	ORIGINAL-1	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	13-Nov-2015	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
BLA 125513	ORIGINAL-1	STRENSIQ	ASFOTASE ALFA	ALEXION PHARMACEUTICALS INC	23-Oct-2015	Treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP)
BLA 761025	ORIGINAL-1	PRAXBIND	IDARUCIZUMAB	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	16-Oct-2015	Treatment of 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
BLA 125554	SUPPLEMENT-5	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	09-Oct-2015	Treatment of patients with metastatic non-squamous non-small cell lung cancer (NSCLC) with progression on or after platinum-based chemotherapy. Patients with EGFR or ALK genomic tumor aberrations should have disease progression on FDA-approved therapy for these aberrations prior to receiving OPDIVO

BLA 125514	SUPPLEMENT-5	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	02-Oct-2015	Treatment of patients with metastatic, PD-L1 positive, non-small cell lung cancer (NSCLC), as determined by an FDA-approved test, with disease progression on or after platinum-containing chemotherapy
NDA 208169	ORIGINAL-1	XURIDEN	URIDINE TRIACETATE	WELLSTAT THERAPEUTICS CORP	04-Sep-2015	Treatment of hereditary orotic aciduria
NDA 207931	ORIGINAL-1	TECHNIVIE	OMBITASVIR, PARITAPREVIR, RITONAVIR	ABBVIE INC	24-Jul-2015	Treatment of patients with genotype 4 chronic hepatitis C virus (HCV) infection without cirrhosis
NDA 206038	ORIGINAL-1	ORKAMBI	LUMACAFTOR/IVACAFTOR	VERTEX PHARMACEUTICALS INC	02-Jul-2015	Treatment of cystic fibrosis (CF) in patients age 12 years and older who are homozygous for the F508del mutation in the CFTR gene
NDA 021083	SUPPLEMENT-55	RAPAMUNE	SIROLIMUS	PF PRISM CV	28-May-2015	Treatment of patients with lymphangioleiomyomatosis (LAM)
NDA 021110	SUPPLEMENT-73	RAPAMUNE	SIROLIMUS	PF PRISM CV	28-May-2015	Treatment of patients with lymphangioleiomyomatosis (LAM)
BLA 125387	SUPPLEMENT-48	EYLEA	AFLIBERCEPT	REGENERON PHARMACEUTICALS INC	25-Mar-2015	Treatment of diabetic retinopathy (DR) in patients with diabetic macular edema (DME)
NDA 207925	ORIGINAL-1	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	17-Mar-2015	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
BLA 125156	SUPPLEMENT-106	LUCENTIS	RANIBIZUMAB	GENENTECH INC	06-Feb-2015	Treatment of Diabetic Retinopathy (DR) in patients with Diabetic Macula Edema (DME)
NDA 207103	ORIGINAL-1	IBRANCE	PALBOCICLIB	PFIZER INC	03-Feb-2015	Treatment of postmenopausal women with estrogen receptor (ER)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced breast cancer as initial endocrine-based therapy for their metastatic disease
NDA 205552	SUPPLEMENT-2	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	29-Jan-2015	Treatment of patients with Waldenström’s macroglobulinemia

NDA 203188	SUPPLEMENT-14	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	29-Dec-2014	Treatment of cystic fibrosis in patients age 6 years and older who have the R117H mutation in the CF transmembrane conductance regulator (CFTR) gene
BLA 125554	ORIGINAL-1	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	22-Dec-2014	Treatment of unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor
NDA 206619	ORIGINAL-1	VIEKIRA PAK	OMBITASVIR, PARITAPREVIR, RITONAVIR	ABBVIE INC	19-Dec-2014	Treatment of patients with genotype 1 chronic hepatitis C virus (HCV) infection including those with compensated cirrhosis
BLA 125557	ORIGINAL-1	BLINCYTO	BLINTUMOMAB	AMGEN INC	03-Dec-2014	Treatment of Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
NDA 022535	ORIGINAL-1	ESBRIET	PIRFENIDONE	GENENTECH INC	15-Oct-2014	Treatment of idiopathic pulmonary fibrosis (IPF)
NDA 205832	ORIGINAL-1	OFEV	NINTEDANIB	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	15-Oct-2014	Treatment of idiopathic pulmonary fibrosis (IPF)
NDA 205834	ORIGINAL-1	HARVONI	SOFOSBUVIR/LEDIPASVIR	GILEAD SCIENCES INC	10-Oct-2014	Treatment of chronic hepatitis C, genotype 1 infection
BLA 125514	ORIGINAL-1	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	04-Sep-2014	Treatment of patients with unresectable or metastatic melanoma & disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor
NDA 022291	SUPPLEMENT-12	PROMACTA	ELTROMBOPAG	NOVARTIS PHARMACEUTICALS CORP	26-Aug-2014	Treatment of cytopenias in patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy
NDA 205552	SUPPLEMENT-1	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	28-Jul-2014	Treatment of patients with chronic lymphocytic leukemia (CLL) who have received at least one prior therapy and CLL with 17p deletion
NDA 206545	ORIGINAL-1	ZYDELIG	IDELALISIB	GILEAD SCIENCES INC	23-Jul-2014	Treatment of 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

NDA 205755	ORIGINAL-1	ZYKADIA	CERITINIB	NOVARTIS PHARMACEUTICALS CORP	29-Apr-2014	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
BLA 125326	SUPPLEMENT-60	ARZERRA	OFATUMUMAB	NOVARTIS PHARMACEUTICALS CORPORATION	17-Apr-2014	Treatment of previously untreated patients with chronic lymphocytic leukemia (CLL) for whom fludarabine-based therapy is considered inappropriate
NDA 203188	SUPPLEMENT-4	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	21-Feb-2014	Treatment of cystic fibrosis patients age 6 years and older who have mutations in the CFTR gene
NDA 204671	ORIGINAL-1	SOVALDI	SOFOSBUVIR	GILEAD SCIENCES INC	06-Dec-2013	Treatment of chronic hepatitis C infection
NDA 205552	ORIGINAL-1	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	13-Nov-2013	Treatment of patients with mantle cell lymphoma (MCL)
BLA 125486	ORIGINAL-1	GAZYVA	OBINUTUZUMAB	GENENTECH INC	01-Nov-2013	Treatment of patients with previously untreated chronic lymphocytic leukemia in combination with chlorambucil

Notes:

Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012. There were no approvals in CY 2012.

BLA 125377 SUPPLEMENT-94 and BLA 125554 SUPPLEMENT-58 were both required to approve the RCC indication.

BLA 761068 ORIGINAL-1 the indication of x-linked hypophosphatemia (XLH) is a pediatric disease; adults are pediatric patients who live into adulthood.

NDA 202806 SUPPLEMENT-10 and NDA 204114 SUPPLEMENT-9 were both required to approve the ATC indication.

BLA 125377 SUPPLEMENT-96 and BLA 123334 SUPPLEMENT-63 were both required to approve the colorectal cancer indication.