CDER Breakthrough Therapy Designation Approvals

Data as of December 31, 2021

Total of 225 Approvals

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
BLA 125486	ORIGINAL-1	GAZYVA	OBINUTUZUMAB	GENENTECH INC	01-Nov-2013	Treatment of patients with previously untreated chronic lymphocytic leukemia in combination with chlorambucil
NDA 205552	ORIGINAL-1	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	13-Nov-2013	Treatment of patients with mantle cell lymphoma (MCL)
NDA 204671	ORIGINAL-1	SOVALDI	SOFOSBUVIR	GILEAD SCIENCES INC	06-Dec-2013	Treatment of chronic hepatitis C infection
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
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 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
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 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 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
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 205834	ORIGINAL-1	HARVONI	LEDIPASVIR AND SOFOSBUVIR	GILEAD SCIENCES INC	10-Oct-2014	Treatment of chronic hepatitis C, genotype 1 infection
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)
BLA 125557	ORIGINAL-1	BLINCYTO	BLINATUMOMAB	AMGEN INC	03-Dec-2014	Treatment of Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
NDA 206619	ORIGINAL-1	VIEKIRA PAK	OMBITASVIR AND PARITAPREVIR AND RITONAVIR AND DASABUVIR	ABBVIE INC	19-Dec-2014	Treatment of patients with genotype 1 chronic hepatitis C virus (HCV) infection including those with compensated cirrhosis
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 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
NDA 205552	SUPPLEMENT-2	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	29-Jan-2015	Treatment of patients with Waldenström's macroglobulinemia
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
BLA 125156	SUPPLEMENT-106	LUCENTIS	RANIBIZUMAB	GENENTECH INC	06-Feb-2015	Treatment of Diabetic Retinopathy (DR) in patients with Diabetic Macula 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 125387	SUPPLEMENT-48	EYLEA	AFLIBERCEPT	REGENERON PHARMACEUTICALS INC	25-Mar-2015	Treatment of diabetic retinopathy (DR) in patients with diabetic macular edema (DME)
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)
NDA 206038	ORIGINAL-1	ORKAMBI	LUMACAFTOR AND 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 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 208169	ORIGINAL-1	XURIDEN	URIDINE TRIACETATE	WELLSTAT THERAPEUTICS CORP	04-Sep-2015	Treatment of hereditary orotic aciduria
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
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 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 125513	ORIGINAL-1	STRENSIQ	ASFOTASE ALFA	ALEXION PHARMACEUTICALS INC	23-Oct-2015	Treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP)
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 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
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 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 125561	ORIGINAL-1	KANUMA	SEBELIPASE ALFA	ALEXION PHARMACEUTICALS INC	08-Dec-2015	Treatment of patients with a diagnosis of lysosomal acid lipase (LAL) deficiency
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 125514	SUPPLEMENT-6	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	18-Dec-2015	Treatment of patients with unresectable or metastatic melanoma
NDA 208261	ORIGINAL-1	ZEPATIER	ELBASVIR AND GRAZOPREVIR	MERCK SHARP AND DOHME CORP	28-Jan-2016	Treatment of chronic hepatitis C virus (HCV) genotypes 1 and 4 infections in adults
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 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 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 208692	ORIGINAL-1	CABOMETYX	CABOZANTINIB	EXELIXIS INC	25-Apr-2016	Treatment of patients with advanced renal cell carcinoma (RCC) who have received 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 205552	SUPPLEMENT-13	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	06-May-2016	Treatment of small lymphocytic lymphoma (SLL) with 17p deletion

NDA 206947	SUPPLEMENT-3	LENVIMA	LENVATINIB	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
BLA 125554	SUPPLEMENT-19	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	17-May-2016	Treatment of Hodgkin Lymphoma
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
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 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 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 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
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 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 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
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 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 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 125514	SUPPLEMENT-15	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	14-Mar-2017	Treatment of patients with refractory classical Hodgkin Lymphoma, or those who have relapsed after three or more prior lines of therapy
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)
NDA 208447	ORIGINAL-1	ZEJULA	NIRAPARIB	GLAXOSMITHKLINE LLC	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 761053	ORIGINAL-1	OCREVUS	OCRELIZUMAB	GENENTECH INC	28-Mar-2017	Treatment of adult patients with 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 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
NDA 209241	ORIGINAL-1	INGREZZA	VALBENAZINE	NEUROCRINE BIOSCIENCES INC	11-Apr-2017	Treatment of tardive dyskinesia
BLA 125156	SUPPLEMENT-114	LUCENTIS	RANIBIZUMAB	GENENTECH INC	15-Apr-2017	Treatment of diabetic retinopathy
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 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	TAKEDA PHARMACEUTICALS USA 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 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 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 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
BLA 125472	SUPPLEMENT-24	ACTEMRA	TOCILIZUMAB	GENENTECH INC	22-May-2017	Treatment of adult patients with giant cell arteritis (GCA)
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
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
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 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 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)
BLA 761040	ORIGINAL-1	BESPONSA	INOTUZUMAB OZOGAMICIN	WYETH PHARMACEUTICALS LLC	17-Aug-2017	Treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
NDA 209885	ORIGINAL-1	AUSTEDO	DEUTETRABENAZINE	TEVA BRANDED PHARMACEUTICAL PRODUCTS R AND D INC	30-Aug-2017	Treatment of tardive dyskinesia
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 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 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 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)

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
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 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
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 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
BLA 761065	ORIGINAL-1	TROGARZO	IBALIZUMAB-UIYK	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 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 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 761068	ORIGINAL-1	CRYSVITA	BUROSUMAB-TWZA	KYOWA KIRIN INC	17-Apr-2018	Treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients 1 year of age and older
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
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 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 022527	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
BLA 103705	SUPPLEMENT-5450	RITUXAN	RITUXIMAB	GENENTECH INC	07-Jun-2018	Treatment of adult patients with moderate to severe pemphigus vulgaris (PV)
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 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
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
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
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 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 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

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 210922	ORIGINAL-1	ONPATTRO	PATISIRAN	ALNYLAM PHARMACEUTICALS INC	10-Aug-2018	Treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults
BLA 761094	ORIGINAL-1	OXERVATE	CENEGERMIN-BKBJ	DOMPE FARMACEUTICI SPA	22-Aug-2018	Treatment of neurotrophic keratitis
BLA 761090	ORIGINAL-1	TAKHZYRO	LANADELUMAB-FLYO	TAKEDA PHARMACEUTICALS USA INC	23-Aug-2018	Prophylaxis to prevent attacks of hereditary angioedema (HAE) in patients 12 years and older
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 761083	SUPPLEMENT-2	HEMLIBRA	EMICIZUMAB-KXWH	GENENTECH INC	04-Oct-2018	Prophylaxis to prevent or reduce the frequency of bleeding episodes in adult and pediatric patients ages newborn and older with hemophilia A (congenital factor VIII deficiency) without factor VIII inhibitors
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 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
BLA 761107	ORIGINAL-1	GAMIFANT	EMAPALUMAB-LZSG	SWEDISH ORPHAN BIOVITRUM AB (PUBL)	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
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
NDA 210861	ORIGINAL-1	VITRAKVI	LAROTRECTINIB	BAYER HEALTHCARE PHARMACEUTICALS 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	BAYER HEALTHCARE PHARMACEUTICALS 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 208078	ORIGINAL-1	FIRDAPSE	AMIFAMPRIDINE	CATALYST PHARMACEUTICALS INC	28-Nov-2018	Treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults
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
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
NDA 209935	SUPPLEMENT-2	KISQALI FEMARA CO-PACK	LETROZOLE AND RIBOCICLIB	NOVARTIS PHARMACEUTICALS CORP	13-Feb-2019	Treatment of pre/perimenopausal women with HR-positive, HER2-negative advanced or metastatic breast cancer
NDA 211243	ORIGINAL-1	SPRAVATO	ESKETAMINE	JANSSEN PHARMACEUTICALS INC	05-Mar-2019	Treatment of treatment-resistant depression
BLA 761055	SUPPLEMENT-12	DUPIXENT	DUPILUMAB	REGENERON PHARMACEUTICALS INC	11-Mar-2019	Treatment of patients 12 to less than 18 years of age with moderate-to-severe atopic dermatitis whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable
NDA 211371	ORIGINAL-1	ZULRESSO	BREXANOLONE	SAGE THERAPEUTICS INC	19-Mar-2019	Treatment of postpartum depression
NDA 207103	SUPPLEMENT-8	IBRANCE	PALBOCICLIB	PFIZER INC	04-Apr-2019	In combination with an aromatase inhibitor, or in combination with fulvestrant, for treatment of male patients with advanced or metastatic breast cancer
NDA 212018	ORIGINAL-1	BALVERSA	ERDAFITINIB	JANSSEN BIOTECH INC	12-Apr-2019	Treatment of adult patients with locally advanced or metastatic urothelial carcinoma (mUC), that has: • susceptible FGFR3 or FGFR2 genetic alterations, and • progressed during or following at least one line of prior platinum-containing chemotherapy, including within 12 months of neoadjuvant or adjuvant platinum-containing chemotherapy
BLA 125514	SUPPLEMENT-54	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	19-Apr-2019	In combination with axitinib for the first-line treatment of patients with advanced renal cell carcinoma
BLA 125427	SUPPLEMENT-105	KADCYLA	ADO-TRASTUZUMAB EMTANSINE	GENENTECH INC	03-May-2019	Adjuvant treatment of patients with HER2-positive early breast cancer who have residual invasive disease after neoadjuvant taxane and trastuzumab-based treatment

NDA 211996	ORIGINAL-1	VYNDAQEL	TAFAMIDIS MEGLUMINE	FOLDRX PHARMACEUTICALS INC SUB PFIZER INC	03-May-2019	Treatment of the cardiomyopathy of wild type or hereditary transthyretin-mediated amyloidosis in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization
BLA 761049	SUPPLEMENT-6	BAVENCIO	AVELUMAB	EMD SERONO INC	14-May-2019	In combination with Inlyta (axitinib) for first-line treatment of patients with advanced renal cell carcinoma (RCC)
NDA 208573	SUPPLEMENT-13	VENCLEXTA	VENETOCLAX	ABBVIE INC	15-May-2019	In combination with obinutuzumab for treatment of previously untreated patients with chronic lymphocytic leukemia (1L CLL) or small lymphocytic lymphoma (SLL)
BLA 761063	SUPPLEMENT-3	EMGALITY	GALCANEZUMAB	ELI LILLY AND COMPANY	04-Jun-2019	Treatment of episodic cluster headache in adults
BLA 761121	ORIGINAL-1	POLIVY	POLATUZUMAB VEDOTIN-PIIQ	GENENTECH INC	10-Jun-2019	In combination with bendamustine and a rituximab product for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma, not otherwise specified, after at least two prior therapies
NDA 211810	ORIGINAL-1	TURALIO	PEXIDARTINIB	DAIICHI SANKYO INC	02-Aug-2019	Treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT) associated with severe morbidity or functional limitations and not amenable to improvement with surgery
NDA 212726	ORIGINAL-1	ROZLYTREK	ENTRECTINIB	GENENTECH INC	15-Aug-2019	Treatment of adult and pediatric patients 12 years of age and older with solid tumors that have a neurotrophic tyrosine receptor 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 progressed following treatment or have no satisfactory alternative therapy
BLA 125514	SUPPLEMENT-65	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	17-Sep-2019	In combination with lenvatinib for the treatment of patients with advanced endometrial carcinoma that is not microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR), who have disease progression following prior systemic therapy and are not candidates for curative surgery or radiation

NDA 206947	SUPPLEMENT-11	LENVIMA	LENVATINIB	EISAI INC	17-Sep-2019	In combination with pembrolizumab, for the treatment of patients with advanced endometrial carcinoma that is not microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR), who have disease progression following prior systemic therapy and are not candidates for curative surgery or radiation
NDA 212273	ORIGINAL-1	TRIKAFTA	ELEXACAFTOR AND TEZACAFTOR AND IVACAFTOR	VERTEX PHARMACEUTICALS INC	21-Oct-2019	Treatment of cystic fibrosis in patients 12 years and older who have at least one F508del mutation in the CFTR gene
NDA 213217	ORIGINAL-1	BRUKINSA	ZANUBRUTINIB	BEIGENE USA INC	14-Nov-2019	Treatment of adult patients with mantle cell lymphoma (MCL) who have received at least one prior therapy
BLA 761128	ORIGINAL-1	ADAKVEO	CRIZANLIZUMAB-TMCA	NOVARTIS PHARMACEUTICALS CORPORATION	15-Nov-2019	To reduce the frequency of vasoocclusive crises in adults and pediatric patients aged 16 years and older with sickle cell disease
NDA 212194	ORIGINAL-1	GIVLAARI	GIVOSIRAN	ALNYLAM PHARMACEUTICALS INC	20-Nov-2019	Treatment of adults with acute hepatic porphyria (AHP)
NDA 210259	SUPPLEMENT-6	CALQUENCE	ACALABRUTINIB	ASTRAZENECA UK LTD	21-Nov-2019	Treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia (CLL)
NDA 210259	SUPPLEMENT-7	CALQUENCE	ACALABRUTINIB	ASTRAZENECA UK LTD	21-Nov-2019	Treatment of adult patients with untreated chronic lymphocytic leukemia (CLL)
NDA 213137	ORIGINAL-1	OXBRYTA	VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	25-Nov-2019	Treatment of sickle cell disease in adults and pediatric patients 12 years of age and older
BLA 761137	ORIGINAL-1	PADCEV	ENFORTUMAB VEDOTIN-EJFV	ASTELLAS PHARMA US INC	18-Dec-2019	Treatment of adult patients with locally advanced or metastatic urothelial cancer (mUC) who have previously received a programmed death receptor-1 (PD-1) or programmed death-ligand 1 (PD-L1) inhibitor, and a platinum-containing chemotherapy in the neoadjuvant/adjuvant, locally advanced or metastatic setting
BLA 761139	ORIGINAL-1	ENHERTU	FAM-TRASTUZUMAB DERUXTECAN-NXKI	DAIICHI SANKYO INC	20-Dec-2019	Treatment of adult patients with unresectable or metastatic HER2-positive breast cancer who have received two or more prior anti-HER2-based regimens in the metastatic setting

NDA 212608	ORIGINAL-1	AYVAKIT	AVAPRITINIB	BLUEPRINT MEDICINES CORP	09-Jan-2020	Treatment of adults with unresectable or metastatic gastrointestinal stromal tumor (GIST) harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations
BLA 761143	ORIGINAL-1	TEPEZZA	TEPROTUMUMAB-TRBW	HORIZON THERAPEUTICS IRELAND DAC	21-Jan-2020	Treatment of thyroid eye disease
NDA 205832	SUPPLEMENT-13	OFEV	NINTEDANIB	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	09-Mar-2020	Treatment for chronic fibrosing interstitial lung diseases with a progressive phenotype
BLA 125377	SUPPLEMENT-108	YERVOY	IPILIMUMAB	BRISTOL-MYERS SQUIBB COMPANY	10-Mar-2020	In combination with nivolumab, for the treatment of patients with hepatocellular carcinoma (HCC) who have been previously treated with sorafenib
BLA 125554	SUPPLEMENT-78	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	10-Mar-2020	In combination with ipilimumab, for the treatment of patients with hepatocellular carcinoma (HCC) who have been previously treated with sorafenib
NDA 213756	ORIGINAL-1	KOSELUGO	SELUMETINIB	ASTRAZENECA PHARMACEUTICALS LP	10-Apr-2020	Treatment of pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN)
NDA 211728	ORIGINAL-1	JELMYTO	MITOMYCIN	UROGEN PHARMA LTD	15-Apr-2020	Treatment of adult patients with low-grade upper tract urothelial cancer
NDA 213411	ORIGINAL-1	TUKYSA	TUCATINIB	SEAGEN INC	17-Apr-2020	In combination with trastuzumab and capecitabine, for the treatment of adult patients with advanced unresectable or metastatic HER2-positive breast cancer, including patients with brain metastases, who have received one or more prior anti-HER2-based regimens in the metastatic setting
NDA 213736	ORIGINAL-1	PEMAZYRE	PEMIGATINIB	INCYTE CORP	17-Apr-2020	Treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test
BLA 761115	ORIGINAL-1	TRODELVY	SACITUZUMAB GOVITECAN-HZIY	IMMUNOMEDICS INC	22-Apr-2020	Treatment of adult patients with metastatic triple-negative breast cancer (mTNBC) who have received at least two prior therapies for metastatic disease

NDA 213591	ORIGINAL-1	TABRECTA	CAPMATINIB	NOVARTIS PHARMACEUTICAL CORP	06-May-2020	Treatment of adult patients with metastatic non-small cell lung cancer (NSCLC) whose tumors have a mutation that leads to mesenchymal-epithelial transition (MET) exon 14 skipping as detected by an FDA-approved test
NDA 213246	ORIGINAL-1	RETEVMO	SELPERCATINIB	LOXO ONCOLOGY INC	08-May-2020	Treatment of adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC); adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant medullary thyroid cancer (MTC) who require systemic therapy; adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate)
NDA 204026	SUPPLEMENT-23	POMALYST	POMALIDOMIDE	CELGENE CORP	14-May-2020	Treatment of adult patients with AIDS-related Kaposi sarcoma (KS) after failure of highly active antiretroviral therapy (HAART)
NDA 209115	SUPPLEMENT-4	RUBRACA	RUCAPARIB	CLOVIS ONCOLOGY INC	15-May-2020	Treatment of adult patients with a deleterious BRCA mutation (germline and/or somatic)-associated metastatic castration-resistant prostate cancer who have been treated with androgen receptor-directed therapy and a taxane-based chemotherapy
NDA 213973	ORIGINAL-1	QINLOCK	RIPRETINIB	DECIPHERA PHARMACEUTICALS LLC	15-May-2020	Treatment of adult patients with advanced gastrointestinal stromal tumor (GIST) who have received prior treatment with 3 or more kinase inhibitors, including imatinib
NDA 213036	ORIGINAL-1		ARTESUNATE	AMIVAS LLC	26-May-2020	Initial treatment of severe malaria in adult and pediatric patients
BLA 125085	SUPPLEMENT-332	AVASTIN	BEVACIZUMAB	GENENTECH INC	29-May-2020	In combination with atezolizumab, for the treatment of patients with unresectable or metastatic hepatocellular carcinoma who have not received prior systemic therapy

BLA 761034	SUPPLEMENT-25	TECENTRIQ	ATEZOLIZUMAB	GENENTECH INC	29-May-2020	In combination with bevacizumab, for the treatment of patients with unresectable or metastatic hepatocellular carcinoma who have not received prior systemic therapy
BLA 761142	ORIGINAL-1	UPLIZNA	INEBILIZUMAB-CDON	VIELA BIO	11-Jun-2020	Treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive
NDA 212950	ORIGINAL-1	RUKOBIA	FOSTEMSAVIR	VIIV HEALTHCARE CO	02-Jul-2020	In combination with other antiretroviral(s) for the treatment of HIV-1 infection in heavily treatment-experienced adults with multidrug-resistant HIV-1 infection failing their current antiretroviral regimen due to resistance, intolerance, or safety considerations
BLA 761163	ORIGINAL-1	MONJUVI	TAFASITAMAB-CXIX	MORPHOSYS US INC	31-Jul-2020	In combination with lenalidomide for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL) not otherwise specified, including DLBCL arising from low grade lymphoma, and who are not eligible for autologous stem cell transplant (ASCT)
NDA 211243	SUPPLEMENT-4	SPRAVATO	ESKETAMINE	JANSSEN PHARMACEUTICALS INC	31-Jul-2020	Treatment of the depressive symptoms in adults with major depressive disorder (MDD) with acute suicidal ideation or behavior
BLA 761158	ORIGINAL-1	BLENREP	BELANTAMAB MAFODOTIN-BLMF	GLAXOSMITHKLINE INTELLECTUAL PROPERTY DEVELOPMENT LTD ENGLAND	05-Aug-2020	Treatment of adults with relapsed or refractory multiple myeloma who have received at least four prior therapies including an anti-CD38 monoclonal antibody, a proteasome inhibitor, and an immunomodulatory agent
BLA 761149	ORIGINAL-1	ENSPRYNG	SATRALIZUMAB-MWGE	GENENTECH INC	14-Aug-2020	Treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive
NDA 213721	ORIGINAL-1	GAVRETO	PRALSETINIB	GENENTECH INC	04-Sep-2020	Treatment of adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA approved test
NDA 211150	ORIGINAL-2	WAKIX	PITOLISANT	HARMONY BIOSCIENCES LLC	13-Oct-2020	Treatment of cataplexy in adult patients with narcolepsy

BLA 761169	ORIGINAL-1	INMAZEB	ATOLTIVIMAB, MAFTIVIMAB, AND ODESIVIMAB-EBGN	REGENERON PHARMACEUTICALS INC	14-Oct-2020	Treatment of infection caused by Zaire ebolavirus in adult and pediatric patients, including neonates born to a mother who is RT-PCR positive for Zaire ebolavirus infection
NDA 213969	ORIGINAL-1	ZOKINVY	LONAFARNIB	EIGER BIOPHARMACEUTICALS INC	20-Nov-2020	To reduce the risk of mortality in Hutchinson-Gilford progeria syndrome (HGPS) in patients 12 months of age and older with a body surface area of 0.39 m2 and above : Treatment of processing-deficient progeroid laminopathies with either: o Heterozygous LMNA mutation with progerin-like protein accumulation o Homozygous or compound heterozygous ZMPSTE24 mutations
NDA 214103	ORIGINAL-1	OXLUMO	LUMASIRAN	ALNYLAM PHARMACEUTICALS INC	23-Nov-2020	Treatment of primary hyperoxaluria type 1 (PH1) to lower urinary oxalate levels in pediatric and adult patients
BLA 761171	ORIGINAL-1	DANYELZA	NAXITAMAB-GQGK	Y-MABS THERAPEUTICS INC	25-Nov-2020	In combination with granulocytemacrophage colony-stimulating factor (GM-CSF), for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy
NDA 213793	ORIGINAL-1	IMCIVREE	SETMELANOTIDE	RHYTHM PHARMACEUTICALS INC	25-Nov-2020	For chronic weight management in adult and pediatric patients 6 years of age and older with obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency confirmed by genetic testing demonstrating variants in POMC, PCSK1, or LEPR genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS)

NDA 214701	ORIGINAL-1	GAVRETO	PRALSETINIB	BLUEPRINT MEDICINES CORP	01-Dec-2020	Treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant medullary thyroid cancer (MTC) who require systemic therapy; and adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate)
BLA 125370	SUPPLEMENT-73	BENLYSTA	BELIMUMAB	GLAXOSMITHKLINE LLC	16-Dec-2020	Treatment of adult patients with active lupus nephritis who are receiving standard therapy
NDA 208065	SUPPLEMENT-21	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	18-Dec-2020	For use as adjuvant therapy after tumor resection in adult patients with non-small cell lung cancer (NSCLC) whose tumors have epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 L858R mutations, as detected by an FDA-approved test
BLA 761172	ORIGINAL-1	EBANGA	ANSUVIMAB-ZYKL	RIDGEBACK BIOTHERAPEUTICS	21-Dec-2020	Treatment of infection caused by Zaire ebolavirus in adult and pediatric patients, including neonates born to a mother who is RT-PCR positive for Zaire ebolavirus infection
NDA 202570	SUPPLEMENT-30	XALKORI	CRIZOTINIB	PF PRISM CV	14-Jan-2021	Treatment of pediatric patients 1 year of age and older and young adults with relapsed or refractory, systemic anaplastic large cell lymphoma (ALCL) that is ALK positive
BLA 761139	SUPPLEMENT-11	ENHERTU	FAM-TRASTUZUMAB DERUXTECAN-NXKI	DAIICHI SANKYO INC	15-Jan-2021	Treatment of adult patients with locally advanced or metastatic HER2-positive gastric or gastroesophageal junction (GEJ) adenocarcinoma who have received a prior trastuzumab-based regimen
NDA 213176	ORIGINAL-1	UKONIQ	UMBRALISIB	TG THERAPEUTICS INC	05-Feb-2021	Treatment of adult patients with relapsed or refractory marginal zone lymphoma (MZL) who have received at least one prior anti-CD20-based regimen
BLA 761181	ORIGINAL-1	EVKEEZA	EVINACUMAB-DGNB	REGENERON PHARMACEUTICALS INC	11-Feb-2021	As an adjunct to other low-density lipoprotein-cholesterol (LDL-C) lowering therapies for the treatment of adult and pediatric patients, aged 12 years and older, with homozygous familial hypercholesterolemia (HoFH)

NDA 214200	ORIGINAL-1	COSELA	TRILACICLIB	G1 THERAPEUTICS INC	12-Feb-2021	To decrease the incidence of chemotherapy-induced myelosuppression in adult patients when administered prior to a platinum/etoposide-containing regimen or topotecan containing regimen for extensive-stage small cell lung cancer
NDA 214018	ORIGINAL-1	NULIBRY	FOSDENOPTERIN	ORIGIN BIOSCIENCES INC	26-Feb-2021	To reduce the risk of mortality in patients with molybdenum cofactor deficiency (MoCD) Type A
BLA 125472	SUPPLEMENT-44	ACTEMRA	TOCILIZUMAB	GENENTECH INC	04-Mar-2021	For slowing the rate of decline in pulmonary function in adult patients with systemic sclerosis-associated interstitial lung disease (SSc-ILD)
BLA 125249	SUPPLEMENT-49	ARCALYST	RILONACEPT	KINIKSA PHARMACEUTICALS (UK) LTD	18-Mar-2021	Treatment of recurrent pericarditis (RP) and reduction in risk of recurrence in adults and children 12 years and older
BLA 761174	ORIGINAL-1	JEMPERLI	DOSTARLIMAB-GXLY	GLAXOSMITHKLINE LLC	22-Apr-2021	Treatment of adult patients with mismatch repair deficient (dMMR) recurrent or advanced endometrial cancer, as determined by an FDA-approved test, that has progressed on or following prior treatment with a platinum-containing regimen
NDA 202293	SUPPLEMENT-24	FARXIGA	DAPAGLIFLOZIN	ASTRAZENECA AB	30-Apr-2021	To reduce the risk of sustained eGFR decline, end-stage kidney disease, cardiovascular death, and hospitalization for heart failure in adults with chronic kidney disease at risk of progression
NDA 211988	ORIGINAL-1	ZYNRELEF	BUPIVACAINE AND MELOXICAM	HERON THERAPEUTICS INC	12-May-2021	For use in adults for soft tissue or periarticular instillation use to produce postsurgical analgesia for up to 72 hours after bunionectomy, open inguinal herniorrhaphy, and total knee arthroplasty
BLA 761210	ORIGINAL-1	RYBREVANT	AMIVANTAMAB-VMJW	JANSSEN BIOTECH INC	21-May-2021	Treatment of adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR) exon 20 insertion mutations, as detected by an FDA-approved test, whose disease has progressed on or after platinum-based chemotherapy

NDA 214665	ORIGINAL-1	LUMAKRAS	SOTORASIB	AMGEN INC	28-May-2021	Treatment of adult patients with KRAS G12C-mutated locally advanced or metastatic non-small cell lung cancer (NSCLC), as determined by an FDA-approved test, who have received at least one prior systemic therapy
NDA 212608	SUPPLEMENT-6	AYVAKIT	AVAPRITINIB	BLUEPRINT MEDICINES CORP	16-Jun-2021	Treatment of adult patients with advanced systemic mastocytosis (AdvSM), including patients with aggressive systemic mastocytosis (ASM) and systemic mastocytosis with an associated hematological neoplasm (SM-AHN)
NDA 212608	SUPPLEMENT-7	AYVAKIT	AVAPRITINIB	BLUEPRINT MEDICINES CORP	16-Jun-2021	Treatment of adult patients with mast cell leukemia (MCL)
NDA 214783	ORIGINAL-1	REZUROCK	BELUMOSUDIL	KADMON PHARMACEUTICALS LLC	16-Jul-2021	Treatment of adult and pediatric patients 12 years and older with chronic graft versus-host disease (chronic GVHD) after failure of at least two prior lines of systemic therapy
BLA 125514	SUPPLEMENT-89	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	26-Jul-2021	Treatment of patients with high-risk, early-stage triple negative breast cancer, in combination with chemotherapy as neoadjuvant treatment, then as a single agent as adjuvant treatment after surgery
BLA 761194	ORIGINAL-1	NEXVIAZYME	AVALGLUCOSIDASE ALFA-NGPT	GENZYME CORPORATION	06-Aug-2021	Treatment of patients 1 year of age and older with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency)
NDA 206947	SUPPLEMENT-19	LENVIMA	LENVATINIB	EISAI INC	10-Aug-2021	In combination with pembrolizumab for the first-line treatment of adult patients with advanced renal cell carcinoma (RCC)
NDA 214916	ORIGINAL-1	KORSUVA	DIFELIKEFALIN	CARA THERAPEUTICS INC	23-Aug-2021	Treatment of moderate-to-severe pruritus associated with chronic kidney disease (CKD-aP) in adults undergoing hemodialysis (HD)
NDA 215310	ORIGINAL-1	EXKIVITY	MOBOCERTINIB	TAKEDA PHARMACEUTICALS USA INC	15-Sep-2021	Treatment of adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) with EGFR exon 20 insertion mutations, as detected by an FDA-approved test, whose disease has progressed on or after platinum-based chemotherapy

NDA 208692	SUPPLEMENT-12	CABOMETYX	CABOZANTINIB	EXELIXIS INC	17-Sep-2021	Treatment of adult and pediatric patients 12 years of age and older with locally advanced or metastatic differentiated thyroid cancer (DTC) that has progressed following prior VEGFR-targeted therapy and who are radioactive iodine-refractory or ineligible
NDA 214662	ORIGINAL-1	LIVMARLI	MARALIXIBAT	MIRUM PHARMACEUTICALS INC	29-Sep-2021	Treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) 1 year of age and older
NDA 215358	ORIGINAL-1	SCEMBLIX	ASCIMINIB	NOVARTIS PHARMACEUTICALS CORP	29-Oct-2021	Treatment of adult patients with Philadelphia chromosome-positive chronic myeloid leukemia (Ph+ CML) in chronic phase (CP), previously treated with two or more tyrosine kinase inhibitors (TKIs)
NDA 215358	ORIGINAL-2	SCEMBLIX	ASCIMINIB	NOVARTIS PHARMACEUTICALS CORP	29-Oct-2021	Treatment of adult patients with Philadelphia chromosome-positive chronic myeloid leukemia (Ph+ CML) in chronic (CP) with the T315I mutation
NDA 213312	ORIGINAL-1	FYARRO	SIROLIMUS	AADI BIOSCIENCE INC	22-Nov-2021	Treatment of adult patients with locally advanced unresectable or metastatic malignant perivascular epithelioid cell tumor (PEComa)
NDA 215596	ORIGINAL-1	LIVTENCITY	MARIBAVIR	TAKEDA PHARMACEUTICALS USA INC	23-Nov-2021	Treatment of adults and pediatric patients 12 years of age and older and weighing at least 35 kg with post-transplant cytomegalovirus (CMV) infection/disease that is refractory to treatment (with or without genotypic resistance) with ganciclovir, valganciclovir, cidofovir or foscarnet.
BLA 125118	SUPPLEMENT-240	ORENCIA	ABATACEPT	BRISTOL-MYERS SQUIBB COMPANY	15-Dec-2021	The prophylaxis of acute graft versus host disease (aGVHD), in combination with a calcineurin inhibitor and methotrexate, in adults and pediatric patients 2 years of age and older undergoing hematopoietic stem cell transplantation (HSCT) from a matched or 1 allele-mismatched unrelated donor
NDA 213137	SUPPLEMENT-6	OXBRYTA	VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	17-Dec-2021	Treatment of sickle cell disease in pediatric patients 4 years of age and older
NDA 216157	ORIGINAL-1	OXBRYTA	VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	17-Dec-2021	Treatment of sickle cell disease in adults and pediatric patients 4 years of age and older

NDA 215499	ORIGINAL-1	APRETUDE	CABOTEGRAVIR	VIIV HEALTHCARE CO	20-Dec-2021	HIV-1 pre-exposure prophylaxis (PrEP) to reduce the risk of sexually acquired HIV-1 infection in at-risk adults and pediatric patients 12 to less than 18 years of age weighing at least 35 kg

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 125554 SUPPLEMENT-63 were both required to approve the colorectal cancer indication.