CY 2018 CDER Breakthrough Therapy Calendar Year Approvals

Data as of September 30, 2018

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
NDA 210491	ORIGINAL-1	SYMDEKO	IVACAFTOR AND TEZACAFTOR	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	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	BURSOSUMAB-TWZA	ULTRAGENYX PHARAMCEUTICAL 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 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 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
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 ni**v**olumab**,** 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	DYAX CORP	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.

Notes:

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.