

CY 2019 CDER Breakthrough Therapy Calendar Year Approvals

Data as of December 31, 2019

Total of 26 Approvals

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
NDA 209935	SUPPLEMENT-2	KISQALI FEMARA CO-PACK	LETROZOLE AND RIBOCICLIB	NOVARTIS PHARMACEUTICALS CORP	13-Feb-19	Treatment of pre/perimenopausal women with HR-positive, HER2-negative advanced or metastatic breast cancer
NDA 211243	ORIGINAL-1	SPRAVATO	ESKETAMINE	JANSSEN PHARMACEUTICALS INC	5-Mar-19	Treatment of treatment-resistant depression
BLA 761055	SUPPLEMENT-12	DUPIXENT	DUPILUMAB	REGENERON PHARMACEUTICALS INC	11-Mar-19	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-19	Treatment of postpartum depression
NDA 207103	SUPPLEMENT-8	IBRANCE	PALBOCICLIB	PFIZER INC	4-Apr-19	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-19	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-19	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	3-May-19	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	VYNDAQUEL	TAFAMIDIS MEGLUMINE	FOLDRX PHARMACEUTICALS INC SUB PFIZER INC	3-May-19	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-19	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-19	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	4-Jun-19	Treatment of episodic cluster headache in adults
BLA 761121	ORIGINAL-1	POLIVY	POLATUZUMAB VEDOTIN-PIIQ	GENENTECH INC	10-Jun-19	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	2-Aug-19	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-19	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-19	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-19	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/TEZ ACAFTOR/IVACAF T OR	VERTEX PHARMACEUTICALS INC	21-Oct-19	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-19	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-19	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-19	Treatment of adults with acute hepatic porphyria (AHP)
NDA 210259	SUPPLEMENT-6	CALQUENCE	ACALABRUTINIB	ASTRAZENECA UK LTD	21-Nov-19	Treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia (CLL)
NDA 210259	SUPPLEMENT-7	CALQUENCE	ACALABRUTINIB	ASTRAZENECA UK LTD	21-Nov-19	Treatment of adult patients with untreated chronic lymphocytic leukemia (CLL)
NDA 213137	ORIGINAL-1	OXBRYTA	VOXELOTOR	GLOBAL BLOOD THERAPEUTICS INC	25-Nov-19	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-19	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-19	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