

### CY 2024 CDER Breakthrough Therapy Calendar Year Approvals

Data as of December 31, 2024

Total of 34 Approvals

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
NDA 213976	ORIGINAL-1	EOHILIA	BUDESONIDE	TAKEDA PHARMACEUTICALS USA INC	09-Feb-2024	For 12 weeks of treatment in adult and pediatric patients 11 years of age and older with eosinophilic esophagitis
BLA 103976	SUPPLEMENT-5245	XOLAIR	OMALIZUMAB	GENENTECH INC	16-Feb-2024	Treatment of IgE-mediated food allergy in adult and pediatric patients aged 1 year and older for the reduction of allergic reactions (Type I), including anaphylaxis, that may occur with accidental exposure to one or more foods. To be used in conjunction with food allergen avoidance
NDA 208065	SUPPLEMENT-30	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	16-Feb-2024	In combination with pemetrexed and platinum-based chemotherapy for the first-line treatment of adult patients with locally advanced or metastatic non-small cell lung cancer whose tumors have EGFR exon 19 deletions or exon 21 L858R mutations, as detected by an FDA-approved test
NDA 217785	ORIGINAL-1	REZDIFFRA	RESMETIROM	MADRIGAL PHARMACEUTICALS INC	14-Mar-2024	In conjunction with diet and exercise, for the treatment of adults with noncirrhotic nonalcoholic steatohepatitis (NASH) with moderate to advanced liver fibrosis (consistent with stages F2 to F3 fibrosis)
BLA 761244	SUPPLEMENT-3	SPEVIGO	SPESOLIMAB-SBZO	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	18-Mar-2024	Treatment of generalized pustular psoriasis (GPP) in adults and pediatric patients 12 years of age and older and weighing at least 40 kg
BLA 761363	ORIGINAL-1	WINREVAIR	SOTATERCEPT-CSRK	MERCK SHARP & DOHME LLC	26-Mar-2024	Treatment of adults with pulmonary arterial hypertension (PAH, World Health Organization [WHO] Group 1) to increase exercise capacity, improve WHO functional class (FC), and reduce the risk of clinical worsening events

NDA 218037	ORIGINAL-1	VOYDEYA	DANICOPAN	ALEXION PHARMACEUTICALS INC	29-Mar-2024	As add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis (EVH) in adults with paroxysmal nocturnal hemoglobinuria (PNH)
BLA 761139	SUPPLEMENT-28	ENHERTU	FAM-TRASTUZUMAB DERUXTECAN-NXKI	DAIICHI SANKYO INC	05-Apr-2024	Treatment of adult patients with unresectable or metastatic HER2-positive (IHC 3+) solid tumors who have received prior systemic treatment and have no satisfactory alternative treatment options
BLA 761336	ORIGINAL-1	ANKTIVA	NOGAPENDEKIN ALFA INBAKICEPT-PMLN	ALTOR BIOSCIENCE LLC AN INDIRECT WHOLLY-OWNED SUBSIDIARY OF IMMUNITYBIO INC	22-Apr-2024	Indicated with Bacillus Calmette-Guérin (BCG) for the treatment of adult patients with BCG-unresponsive nonmuscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors
NDA 217700	ORIGINAL-1	OJEMDA TABLET	TOVORAFENIB	DAY ONE BIOPHARMACEUTICALS INC	23-Apr-2024	Treatment of patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation
NDA 218033	ORIGINAL-1	OJEMDA ORAL SUSPENSION	TOVORAFENIB	DAY ONE BIOPHARMACEUTICALS INC	23-Apr-2024	Treatment of patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation
BLA 761344	ORIGINAL-1	IMDELLTRA	TARLATAMAB-DLLE	AMGEN INC	16-May-2024	Treatment of adult patients with extensive stage small cell lung cancer (ES-SCLC) with disease progression on or after platinum-based chemotherapy
NDA 218860	ORIGINAL-1	IQIRVO	ELAFIBRANOR	IPSEN BIOPHARMACEUTICALS INC	10-Jun-2024	Treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA
NDA 218213	SUPPLEMENT-1	AUGTYRO	REPOTRECTINIB	BRISTOL MYERS SQUIBB CO	13-Jun-2024	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, are locally advanced or metastatic or where surgical resection is likely to result in severe morbidity, and have progressed following treatment or have no satisfactory alternative therapy

BLA 125557	SUPPLEMENT-28	BLINCYTO	BLINATUMOMAB	AMGEN INC	14-Jun-2024	Treatment of CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia (ALL) in the consolidation phase of multiphase chemotherapy in adult and pediatric patients one month and older
NDA 216340	SUPPLEMENT-5	KRAZATI	ADAGRASIB	MIRATI THERAPEUTICS INC	21-Jun-2024	In combination with cetuximab for the treatment of adult patients with KRAS G12C mutated locally advanced or metastatic colorectal cancer who have been previously treated with fluoropyrimidine-, oxaliplatin-, and irinotecan-based chemotherapy
BLA 761324	SUPPLEMENT-3	EPKINLY	EPCORITAMAB-BYSP	GENMAB US INC	26-Jun-2024	Treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after two or more lines of systemic therapy
BLA 761248	ORIGINAL-1	KISUNLA	DONANEMAB-AZBT	ELI LILLY AND COMPANY	02-Jul-2024	Treatment of Alzheimer's disease
NDA 218784	ORIGINAL-1	VORANIGO	VORASIDENIB	SERVIER PHARMACEUTICALS LLC	06-Aug-2024	Treatment of adult and pediatric patients 12 years and older with Grade 2 astrocytoma or oligodendroglioma with a susceptible IDH1 or IDH2 mutation, following surgery including biopsy, subtotal resection, or gross total resection
BLA 761390	ORIGINAL-1	NEMLUVIO	NEMOLIZUMAB-ILTO	GALDERMA LABORATORIES LP	12-Aug-2024	Treatment of adults with Prurigo Nodularis
NDA 217899	ORIGINAL-1	LIVDELZI	SELADELPAR	CYMABAY THERAPEUTICS INC	14-Aug-2024	Treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA
NDA 214927	ORIGINAL-1	MIPLYFFA	ARIMOCLOMOL	ZEVRA DENMARK A/S	20-Sep-2024	In combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients 2 years of age and older
NDA 208065	SUPPLEMENT-33	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	25-Sep-2024	Treatment of adult patients with locally advanced, unresectable (stage III) non-small cell lung cancer (NSCLC) whose disease has not progressed during or following concurrent or sequential platinum-based chemoradiation therapy and whose tumors have EGFR exon 19 deletions or exon 21 L858R mutations, as detected by an FDA-approved test

BLA 761055	SUPPLEMENT-64	DUPIXENT	DUPILUMAB	REGENERON PHARMACEUTICALS INC	27-Sep-2024	As an add-on maintenance treatment in adult patients with inadequately controlled chronic obstructive pulmonary disease (COPD) and an eosinophilic phenotype
NDA 219249	ORIGINAL-1	ITOVEBI	INAVOLISIB	GENENTECH INC	10-Oct-2024	In combination with palbociclib and fulvestrant for the treatment of adults with endocrine-resistant, PIK3CA-mutated, hormone receptor (HR)-positive, human epidermal growth-factor receptor 2 (HER2)-negative, locally advanced or metastatic breast cancer, as detected by an FDA-approved test, following recurrence on or after completing adjuvant endocrine therapy
NDA 215358	SUPPLEMENT-8	SCEMBLIX	ASCIMINIB	NOVARTIS PHARMACEUTICALS CORP	29-Oct-2024	Treatment of adult patients with newly diagnosed Philadelphia chromosome-positive chronic myeloid leukemia (Ph+CML) in chronic phase (CP)
NDA 218944	ORIGINAL-1	REVUFORJ	REVUMENIB	SYNDAX PHARMACEUTICALS INC	15-Nov-2024	Treatment of relapsed or refractory acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in adult and pediatric patients 1 year and older
BLA 761416	ORIGINAL-1	ZIIHERA	ZANIDATAMAB-HR11	JAZZ PHARMACEUTICALS IRELAND LIMITED	20-Nov-2024	Treatment of adults with previously treated, unresectable or metastatic HER2-positive (IHC3+) biliary tract cancer as detected by an FDA approved test
BLA 761069	SUPPLEMENT-49	IMFINZI	DURVALUMAB	ASTRAZENECA UK LTD	04-Dec-2024	Treatment of adult patients with limited-stage small cell lung cancer (LS-SCLC) whose disease has not progressed following concurrent platinum-based chemotherapy and radiation therapy (cCRT).
BLA 761352	ORIGINAL-1	BIZENGRI	ZENOCUTUZUMAB-ZBCO	MERUS NV	04-Dec-2024	Treatment of adults with advanced, unresectable or metastatic non-small cell lung cancer harboring a neuregulin 1 (NRG1) gene fusion with disease progression on or after prior systemic therapy; and adults with advanced, unresectable or metastatic pancreatic adenocarcinoma harboring an NRG1 gene fusion with disease progression on or after prior systemic therapy
NDA 218808	ORIGINAL-1	CRENESSITY -Oral Capsules	CRINECFONT	NEUROCRINE BIOSCIENCES INC	13-Dec-2024	Adjunctive treatment to glucocorticoid replacement to control androgens in adults and pediatric patients 4 years of age and older with classic congenital adrenal hyperplasia (CAH)

NDA 218820	ORIGINAL-1	CRENESSITY- Oral Solution	CRINECERFONT	NEUROCRINE BIOSCIENCES INC	13-Dec-2024	Adjunctive treatment to glucocorticoid replacement to control androgens in adults and pediatric patients 4 years of age and older with classic congenital adrenal hyperplasia (CAH)
NDA 218614	ORIGINAL-1	TRYNGOLZA	OLEZARSEN	IONIS PHARMACEUTICALS INC	19-Dec-2024	Adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS)
NDA 217806	SUPPLEMENT-13	ZEPBOUND	TIRZEPATIDE	ELI LILLY AND COMPANY	20-Dec-2024	Treatment of moderate to severe obstructive sleep apnea (OSA) in adults with obesity