Cumulative CBER Breakthrough Therapy Approvals

Data as of December 31, 2024

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
BLA 125786	ORIGINAL-1	BEQVEZ	fidanacogene elaparvovec-dzkt	Pfizer, Inc.	25-APR-2024	Treatment of adults with moderate to severe hemophilia B (congenital factor IX deficiency) who are receiving routine prophylaxis and without pre-existing neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid detected by an FDA-approved test.
BLA 125746	SUPPLEMENT-74	CARVYKTI	ciltacabtagene autoleucel	Janssen Biotech, Inc	05-APR-2024	Treatment of adult patients with relapsed or refractory multiple myeloma, who previously received a proteasome inhibitor (PI), an immunomodulatory agent (IMiD) and an anti-CD38 antibody
BLA 125746	SUPPLEMENT-57	CARVYKTI	ciltacabtagene autoleucel	Janssen Biotech, Inc.	21-DEC-2023	Treatment of adult patients with relapsed or refractory multiple myeloma, who previously received a proteasome inhibitor (PI), an immunomodulatory agent (IMiD) and an anti-CD38 antibody.
BLA 125777	ORIGNAL-1	IXCHIQ	Chikungunya Vaccine, Live	Valneva Austria GmbH	09-NOV-2023	For the prevention of disease caused by chikungunya virus (CHIKV) in individuals 18 years of age and older.
BLA 125768	ORIGINAL-1	ABRYSVO	Respiratory Syncytial Virus Vaccine	Pfizer, Inc.	21-AUG-2023	For the prevention of lower respiratory tract disease and severe lower respiratory tract disease caused by respiratory syncytial virus (RSV) in infants from birth through 6 months of age by active immunization of pregnant individuals.
BLA 125720	ORIGINAL-1	ROCTAVIAN	valoctocogene roxaparvovec-rvox	Biomarin Pharmaceutical, Inc.	29-JUN-2023	For the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity <1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA approved test.
BLA 125769	ORIGINAL-1	ABRYSVO	Respiratory Syncytial Virus Vaccine	Pfizer, Inc.	31-MAY-2023	For active immunization for the prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV) in individuals 60 years of age and older.
BLA 125757	ORIGINAL-1	VOWST	Fecal Microbiota Spores	Seres Therapeutics, Inc.	26-APR-2023	To prevent the recurrence of Clostridioides difficile infection (CDI) in individuals 18 years of age and older following antibacterial treatment for recurrent CDI (rCDI).
BLA 125738	ORIGINAL-1	OMISIRGE	omidubicel-onlv	Gamida Cell, Ltd.	17-APR-2023	Treatment of patients with hematologic malignancies who are in need of a hematopoeitic stem cell transplant.
BLA 125700	ORIGINAL-1	ADSTILADRIN	nadofaragene firadenovec	Ferring Pharmaceuticals, Inc.	16-DEC-2022	For the treatment of adult patients with high-risk Bacillus Calmette-Guérin (BCG)-unresponsive non-Muscle Invasive Bladder Cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors.

BLA 125739	ORIGINAL-1	REBYOTA	Fecal Microbiota Transplantation, Frozen Preparation	Ferring Pharmaceuticals, Inc.	30-NOV-2022	Indicated to reduce recurrence of Clostridioides difficile.
BLA 125772	ORIGINAL-1	HEMGENIX	etranacogene dezaparvovec	CSL Behring LLC	22-NOV-2022	(congenital Factor IX deficiency) who: currently use Factor IX prophylaxis therapy or have current or historical life-threatening hemorrhage or have repeated, serious
BLA 125755	ORIGINAL-1	SKYSONA	elivaldogene autotemcel	bluebird bio, Inc.	16-SEP-2022	Treatment of patients less than 18 years of age with early cerebral adrenoleukodystrophy who do not have an available and willing HLA-matched sibling HSC donor.
BLA 125717	ORIGINAL-1	ZYNTEGLO	betibeglogene autotemcel	bluebird bio, Inc.	17-AUG-2022	Treatment of adult and pediatric patients with B-thalassemia who require regular red blood cell (RBC) transfusions.
BLA 125746	ORIGINAL-1	CARVYKTI	ciltacabtagene autoleucel	Jannssen Biotech, Inc.	28-Feb-2022	Treatment of adult patients with relapsed or refractory multiple myeloma, who previously received a proteasome inhibitor (PI), an immunomodulatory agent (IMiD) and an anti-CD38 antibody.
BLA 125685	ORIGINAL-1	RETHYMIC	Allogeneic processed Thymus Tissue	Enzyvant Therapeutics GmbH	08-Oct-2021	Immune reconstitution in pediatric patients with congenital athymia
BLA 125703	SUPPLEMENT-91	TECARTUS	brexucabtagene autoleucel	Kite Pharma, Inc.	01-Oct-2021	For the treatment of adult patients with relapsed/refractory mantle cell lymphoma (r/r MCL).
BLA 125741	ORIGINAL-1	VAXNEUVANCE	Pneumococcal 15-valent Conjugate Vaccine	Merck Sharp & Dohme Corp	16-Jul-2021	For active immunization for the prevention of invasive disease caused by Streptococcus pneumoniae serotypes 1, 3, 4, 5, 6A, 6B, 7F, 9V, 14, 18C, 19A, 19F, 22F, 23F and 33F in adults 18 years of age and older.
BLA 125731	ORIGINAL-1	PREVNAR 20	20-valent Pneumococcal Conjugate Vaccine	Wyeth Pharmaceuticals LLC	08-Jun-2021	Active immunization for the prevention of pneumonia and invasive disease caused by Streptococcus pneumoniae serotypes 1, 3, 4, 5, 6A, 6B, 7F, 8, 9V, 10A, 11A, 12F, 14, 15B, 18C, 19A, 19F, 22F, 23F, and 33F in adults 18 years of age and older.
BLA 125736	ORIGINAL-1	ABECMA	idecabtagene vicleucel	Celgene Corp, a Bristol-Myers Squibb Company	26-Mar-2021	Treatment of adult patients with multiple myeloma who have received at least three prior therapies, including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 antibody.
BLA 125714	ORIGINAL-1	BREYANZI	lisocabtagene maraleucel	Juno Therapeutics, a Celgene Company	05-Feb-2021	Treatment of adult patients with relapsed or refractory (R/R) large B-cell lymphoma after at least two prior therapies.
BLA 125703	ORIGINAL-1	TECARTUS	brexucabtagene autoleucel	Kite Pharma, Inc.	24-Jul-2020	For the treatment of adult patients with relapsed/refractory mantle cell lymphoma (r/r MCL).
BLA 125696	ORIGINAL-1	PALFORZIA	Peanut (Arachis hypogaea) Allergen Powder	Aimmune Therapeutics, Inc.	31-Jan-2020	An oral immunotherapy indicated for the mitigation of allergic reactions, including anaphylaxis, that may occur with accidental exposure to peanut.
BLA 125690	ORIGINAL-1	ERVEBO	Ebola Zaire Vaccine, Live	Merck Sharp & Dohme, Corp.	19-Dec-2019	Indicated for the prevention of disease caused by Zaire ebolavirus in individuals 18 years of age and older.
BLA 125694	ORIGINAL-1	ZOLGENSMA	Onasemnogene abeparvovec	AveXis, Inc.	24-May-2019	Indication-treatment of pediatric patients with spinal muscular atrophy (SMA) Type 1 with or without disease onset.
BLA 125586	ORIGINAL-1	ANDEXXA	Coagulation Factor Xa (Recombinant), Inactivated-zhzo	Portola Pharmaceuticals, Inc.	03-May-2018	Indicated for patients treated with rivaroxaban and apixaban, when reversal of anticoagulation is needed due to life-threatening or uncontrolled bleeding.

BLA 125646	SUPPLEMENT-76	KYMRIAH	Tisagenlecleucel	Novartis Pharmaceuticals, Corp.	01-May-2018	For the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (r/r DLBCL) who are ineligible for autologous transplant.
BLA 125610	ORIGINAL-1	LUXTURNA	Voretigene Neparvovec	Spark Therapeutics, Inc.	19-Dec-2017	The treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy.
BLA 125643	ORIGINAL-1	YESCARTA	Axicabtagene Ciloleucel	Kite Pharma, Inc.	18-Oct-2017	Treatment of adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma. Axicabtagene ciloleucel is not indicated for the treatment of patients with primary central nervous system lymphoma.
BLA 125646	ORIGINAL-1	KYMRIAH	Tisagenlecleucel	Novartis Pharmaceuticals, Corp.	30-Aug-2017	For the treatment of pediatric and young adult patients (age 3-25 years) with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.
BLA 125546	ORIGINAL-1	BEXSERO	Meningococcal Group B Vaccine	GlaxoSmithKline Biologicals	23-Jan-2015	Active immunization to prevent invasive meningococcal disease caused by N. meningitidis serogroup B in individuals 10 through 25 years of age.
BLA 125549	ORIGINAL-1	TRUMENBA	Meningococcal Group B Vaccine	Wyeth Pharmaceuticals, Inc.	29-Oct-2014	Active immunization to prevent invasive meningococcal disease caused by N. meningitidis serogroup B in individuals 10 through 25 years of age.

* Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012.