

Cumulative CBER Breakthrough Therapy Approvals

Data as of December 31, 2020

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
BLA 125703	ORIGINAL-1	TECARTUS	brexucabtagene autoleucl	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	Tisagenlecleucl	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 Ciloleucl	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 ciloleucl is not indicated for the treatment of patients with primary central nervous system lymphoma.
BLA 125646	ORIGINAL-1	KYMRIAH	Tisagenlecleucl	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.
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* Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012.