Cumulative CBER Breakthrough Therapy Approvals

Data as of February 1, 2019

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
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.