

PIPELINE REPORT

Specialty Drugs

February 2019



PIPELINE DRUG	CURRENT STATUS	ANTICIPATED APPROVAL	WHAT IS THIS DRUG BEING DEVELOPED FOR?
brolocizumab (Novartis)	Phase 3	2019	A humanized single-chain antibody fragment (scFv) designed to inhibit vascular endothelia growth factor (VEGF); indicated for the treatment of age-related macular degeneration. Intravitreal injection given every 3 months
celiprolol (Edsivo-Acer Therapeutics)	NDA Filed	6/29/2019	A cardioselective beta blocker for the treatment of vascular Ehlers-Danlos Syndrome (vEDS); oral Orphan Drug
cladribine (Mavenclad-EMD Serono)	NDA Filed	3/30/2019	Nucleoside analog that interferes with DNA synthesis causing depletion of B and T lymphocytes indicated for the treatment of relapsing forms of multiple sclerosis; oral
diroximel fumarate (Biogen)	Phase 3	2019	A pro-drug of monomethyl fumarate indicated for the treatment of relapsing forms of multiple sclerosis; oral
entinostat (Syndax Pharmaceuticals)	Phase 3	2019	Histone deacetylase inhibitor for the second-line treatment of postmenopausal women with advanced ER+ breast cancer with exemestane; oral given once weekly Breakthrough Therapy PGx
erdafitinib (Janssen)	NDA Filed	5/18/2019	Pan-fibroblast growth factor receptor (FGFR) tyrosine kinase inhibitor for the treatment of patients with locally advanced or metastatic urothelial cancer (UC) and certain fibroblast growth receptor (FGFR) genetic alterations, whose tumors have progressed after prior chemotherapy; once daily oral Breakthrough Therapy PGx
flenfluramine (Fintepla - Zogenix)	Phase 3	2019	A low dose sympathetic amine for the treatment of seizures associated with Dravet syndrome (DS), a severe form of epilepsy; oral solution Breakthrough Therapy PGx
givosiran (Alnylam)	Phase 3	2019	A small interfering RNA molecule designed to correct the over-expression of 5-aminolevulinatase synthase for the treatment of acute intermittent porphyria (AIP): once-monthly subcutaneous (SC) injection Breakthrough Therapy PGx
golodirsen (Sarepta Therapeutics)	Phase 3	2019	An antisense oligonucleotide for the treatment of patients with Duchenne muscular dystrophy amenable to exon 53 skipping; IV infusion Orphan Drug PGx
lisocabtagene maraleuel (JCAR017- Celgene)	Phase 2	2019	A CD-19 directed CAR T for the treatment of patients with relapsed/refractory (R/R) aggressive large B-cell non-Hodkin lymphoma (NHL); one-time intravenous (IV) infusion Breakthrough Therapy

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onasemnogene abeparvovec (Zolgensma - Novartis)	BLA Filed	2019	Gene therapy using an adeno-associated virus vector (AAV9) containing the SMN1 transgene for the treatment of spinal muscular atrophy (SMA); one-time intravenous (IV) infusion Breakthrough Therapy Orphan Drug PGx
ozanimod (Celgene)	Phase 3	2019	S1P receptor modulator for the treatment of relapsing remitting multiple sclerosis (MS);
quizartinib (Daiichi Sankyo)	NDA Filed	5/25/2019	FMS-like tyrosine kinase (FLT) 3 inhibitor for the treatment of adult patients with relapsed or refractory FLT3-ITD acute myeloid leukemia (AML); oral Breakthrough Therapy Orphan Drug PGx
ravulizumab (Ultomiris - Alexion Pharmaceuticals)	BLA Filed	2/18/2019	Longer-acting anti-C5 antibody that inhibits terminal complement for treatment-naïve patients with paroxysmal nocturnal hemoglobinuria (PNH); IV infusion Orphan Drug
risankizumab (AbbVie/Boehringer Ingelheim)	BLA Filed	4/25/2019	A selective interleukin (IL)-23 inhibitor for the treatment of moderate-to-severe plaque psoriasis; SC injection
romosozumab (Evenity-Amgen/UCB)	BLA Filed	1Q:2019	Humanized antibody that targets and inhibits sclerostin for the treatment of postmenopausal osteoporosis; once-monthly for 1 year SC injection
selinexor (Karyopharm Therapeutics)	NDA Filed	4/6/2019	Selective Inhibitor of Nuclear Export (SINE) compound in combination with dexamethasone for the treatment of patients with penta-refractory multiple myeloma; oral Orphan Drug
siponimod (Novartis)	NDA Filed	Mar-19	Selective sphingosine 1-phosphate (S1P) receptor-1.5 modulator for the treatment of secondary progressive multiple sclerosis; once daily oral
tafamidis meglumine (Pfizer)	Phase 3	2019	Transthyretin (TTR) dissociation inhibitor for the treatment of transthyretin cardiomyopathy (TTR-CM); oral Breakthrough Therapy Orphan Drug PGx
upadacitinib (AbbVie)	Phase 3	2019	A janus kinase (JAK)-1 inhibitor for the treatment of rheumatoid arthritis (RA); oral

PGx = Pharmacogenetic test in development