

## Top 20 Specialty Pipeline Report December 2018

Pipeline Drug	Current Status	Anticipated Approval	What is this drug being developed for?
1 . brolocizumab (Novartis)	Phase 3	2019	Long-acting VEGF inhibitor for the treatment of wet AMD; intra-vitreous injection (every 3 months)
2 . celiprolol (Edsivo – Acer Therapeutics)	NDA Filed	06/29/2019	Cardio-selective beta blocker for the treatment of vascular Ehlers-Danlos Syndrome (vEDS); oral  Orphan Drug
3 . cladribine (Mavenclad – EMD Serono)	NDA Filed	03/30/2019	Nucleoside analog that interferes with DNA synthesis, selectively causing depletion of B and T lymphocyte for the treatment of relapsing forms of multiple sclerosis; oral
4 . diroximel fumarate (Biogen)	Phase 3	2019	Pro-drug of monomethyl fumarate for the treatment of relapsing forms of multiple sclerosis; oral
5 . entinostat (Syndax Pharmaceuticals)	Phase 3	2019	Histone deacetylase inhibitor for the second-line treatment postmenopausal women with advanced ER+ breast cancer with exemestane; oral (once weekly) Breakthrough Therapy PGx
6 . erdafitinib (Janssen)	NDA Filed	05/18/2019	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 factor receptor (FGFR) genetic alterations whose tumors have progressed after prior chemotherapy; oral Breakthrough Therapy PGx

PGx =Pharmacogenetic test in development

Pipeline Drug	Manufacturer	Current Status	What is this drug being developed for?
7 . fenfluramine (Fintepla – Zogenix)	Phase 3	2019	Low-dose fenfluramine (sympathomimetic amine) for the treatment of seizures associated with Dravet syndrome; oral solution  Breakthrough Therapy Orphan Drug
8 . givosiran (Alynlam)	Phase 3	2019	Small interfering RNA molecule designed to correct the over expression of 5-aminolevulinate synthase for the treatment of acute intermittent porphyria (AIP); SC injection (once-monthly)  Breakthrough Therapy Orphan Drug
9 . golodirsen (Sarepta Therapeutics)	Phase 3	2019	Antisense oligonucleotide for the treatment of patients with Duchenne muscular dystrophy amenable to exon 53 skipping; IV infusion  Orphan Drug PGx
10 . lisocabtagene maraleucel (JCAR017 - Celgene)	Phase 2	2019	CD19-directed CAR T for the treatment of patients with relapsed/ refractory (r/r) aggressive large B-cell non-Hodgkin lymphoma (NHL); IV infusion (one time)  Breakthrough Therapy
11 . onasemnogene abeparvovec (Zolgensma - Novartis)	BLA Filed	May 2019	Gene therapy using an adeno-associated virus vector (AAV9) containing the SMN1 transgene for the treatment of spinal muscular atrophy (SMA); IV infusion (one-time)  Breakthrough Therapy Orphan Drug PGx

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12 . ozanimod (Celgene)	Phase 3	2019	S1P receptor modulator for the treatment of relapsing multiple sclerosis (MS); oral
13 . quizartinib (Daiichi Sankyo)	NDA Filed	05/25/2019	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
14 . ravulizumab (Ultomiris – Alexion Pharmaceuticals)	BLA Filed	02/18/2019	Longer-acting anti-C5 antibody that inhibits terminal complement for treatment-naïve patients with paroxysmal nocturnal hemoglobinuria (PNH); IV infusion Orphan Drug
15 . risankizumab (AbbVie/Boehringer Ingelheim)	BLA Filed	04/25/2019	Selective IL-23 inhibitor for the treatment of moderate-to-severe plaque psoriasis; SC
16 . romosozumab (Evenity - Amgen / UCB)	BLA Filed	1Q:2019	Humanized antibody that targets and inhibits sclerostin for the treatment of postmenopausal osteoporosis; SC (once monthly for 1 year)
17 . selinexor (Karyopharm Therapeutics)	NDA Filed	04/06/2019	Selective Inhibitor of Nuclear Export (SINE) compound in combination with dexamethasone for the treatment of patients with penta-refractory multiple myeloma; oral Orphan Drug

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18 . siponimod (Novartis)	NDA Filed	March 2019	Selective sphingosine 1-phosphate (S1P) receptor-1,5 modulator for the treatment of secondary progressive multiple sclerosis; oral (once daily)
19 . tafamidis meglumine (Pfizer)	Phase 3	2019	Transthyretin (TTR) dissociation inhibitor for the treatment of transthyretin cardiomyopathy (TTR-CM); oral
			Breakthrough Therapy    Orphan Drug    PGx
20 . upadacitinib (AbbVie)	Phase 3	2019	JAK1 inhibitor for the treatment of rheumatoid arthritis; oral

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