

# PIPELINE REPORT

## Specialty Drugs

July 2019



Pipeline Drug	Current Status	Anticipated Approval	What is this drug being developed for?
brolocizumab (Novartis)	BLA Filed	October 2019	A humanized single-chain antibody fragment (scFv) designed to inhibit vascular endothelial growth factor (VEGF) indicated for the treatment of age-related macular degeneration; Intravitreal injection given every 3 months
cabotegravir long-acting/rilpivirine (ViiV Healthcare/ GSK/Shionogi/ Janssen)	Phase 3	2Q 2020	Integrase inhibitor and non-nucleoside reverse transcriptase inhibitor (NNRTI) long-acting treatment regimen for adults with HIV with a suppressed viral load; once monthly injectable
celiprolol (Edsivo-Acer Therapeutics)	NDA Filed	June 2019	A cardioselective beta blocker for the treatment of vascular Ehlers-Danlos Syndrome (vEDS); oral
COL-144 (Lasmiditan- CoLucid/Eli Lilly)	Phase 3	4Q 2019	A selective 5-HT <sub>1</sub> serotonin agonist for acute migraine; oral/ intravenous
darolutamide (Bayer)	NDA Filed	October 2019	An androgen receptor (AR) inhibitor for the treatment of patients with non-metastatic CRPC who are at high risk for developing metastatic disease; oral.
entrectinib (Genentech)	NDA Filed	August 2019	A selective tyrosine kinase inhibitor seeking approval for the treatment of adult and pediatric patients with neurotrophic tropomyosin receptor kinase (NTRK) fusion-positive, locally advanced or metastatic solid tumors who have either progressed following prior therapies or as initial therapy when there are no acceptable standard therapies, and for the treatment of people with metastatic, RO1-positive non-small cell lung cancer; oral
fedratinib (Celgene/Bristol- Myers Squibb)	NDA Filed	September 2019	A highly selective JAK2 inhibitor intended to treat patients with myelofibrosis, a serious bone marrow disorder that disrupts the body's normal production of blood cells; oral.
givosiran (Alnylam)	Phase 3	2020	A small interfering RNA molecule designed to correct the overexpression of 5-aminolevulinic acid synthase for the treatment of acute intermittent porphyria (AIP): once-monthly subcutaneous injection
golodirsen (Sarepta Therapeutics)	NDA Filed	August 2019	An antisense oligonucleotide for the treatment of patients with Duchenne muscular dystrophy amenable to exon 53 skipping; IV infusion
Istradefyline (KW-6002-Kyowa Hakko Kirin)	NDA Filed	August 2019	A selective adenosine A <sub>2a</sub> receptor antagonist for use as adjunctive treatment to levodopa/carbidopa in adult patients with Parkinson's disease experiencing "OFF" episodes; oral
LCI699 (Osilodrostat- Novartis)	Phase 3	1Q 2020	An aldosterone synthase inhibitor to treat Cushing's disease in adult patients; oral

# PIPELINE REPORT

## Specialty Drugs

July 2019



Pipeline Drug	Current Status	Anticipated Approval	What is this drug being developed for?
lisocabtagene maraleucel (JCAR017-Celgene)	Phase 3	2020	A CD-19 directed CAR T for the treatment of patients with relapsed/refractory (R/R) aggressive large B-cell non-Hodgkin's lymphoma (NHL); one-time intravenous (IV) infusion
NS-065 (Viltolarsen - Nippon Shinyaku/NS Pharma)	Phase 2	1Q 2020	An antisense oligonucleotide for the treatment of Duchenne Muscular Dystrophy (DMD) in patients amenable to exon 53 skipping; intravenous
ozanimod (Celgene)	NDA Filed	March 2020	S1P receptor modulator for the treatment of relapsing multiple sclerosis (MS); oral
pexidartinib (Plexxikon/Daiichi Sankyo)	NDA Filed	August 2019	A CSF1R receptor inhibitor for the treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT) where surgical removal of the tumor would be associated with potentially worsening functional limitation or severe morbidity; oral
PRO 140 (Leronlimab-CytoDyn)	Phase 3	1Q 2020	A CCR5 antagonist for use as a single agent in the maintenance of HIV viral load suppression; subcutaneous injection
quizartinib (Daiichi Sankyo)	NDA Filed	August 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
tazemetostat (Epizyme)	Phase 2	2Q 2020	An EZH2 inhibitor for the treatment of patients with metastatic or locally advanced epithelioid sarcoma not eligible for curative surgery; oral
satralizumab (Genentech)	Phase 3	2020	Humanized anti-interleukin-6 (IL-6) receptor monoclonal antibody for the treatment of neuromyelitis optica and neuromyelitis optica spectrum disorders (NMO/NMOSD); subcutaneous injection
Ubrogепant (Allergan)	NDA Filed	December 2019	A calcitonin gene-related peptide (CGRP) inhibitor for acute treatment of migraine; oral