

## Top 20 Specialty Pipeline Report July 2018

| Pipeline Drug  | Current Status | Anticipated Approval | What is this drug being developed for?  |
|--|----------------|----------------------|---|
| 1 . AVXS 101 (Novartis)  | Phase 3        | 2018                 | 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)<br>Breakthrough Therapy      Orphan Drug  |
| 2 . binimetinib / encorafenib (Mektovi / Braftovi - Array BioPharma) | NDA Filed      | 06/30/2018           | MEK inhibitor for the treatment of patients with BRAF-mutant advanced, unresectable or metastatic melanoma; oral (twice daily)<br>Orphan Drug      PGx  |
| 3 . cannabidiol (Epidiolex - GW Pharmaceuticals)                     | NDA Filed      | 06/27/2018           | Epidiolex contains the cannabinoid, cannabidiol, for treatment of seizures associated with Lennox-Gastaut syndrome (LGS) and Dravet syndrome; oral therapy.<br>Orphan Drug  |
| 4 . cemiplimab (Sanofi/Regeneron)                                    | BLA Filed      | 10/28/2018           | Programmed Death-1 (PD-1) inhibitor for the treatment of adults with metastatic or locally advanced and unresectable cutaneous squamous cell carcinoma (CSCC); IV infusion<br>Breakthrough Therapy  |
| 5 . dacomitinib (Pfizer)   | NDA Filed      | Sept. 2018           | Epidermal growth factor receptor (EGFR) inhibitor for the first-line treatment of EGFR-mutant non-small-cell lung cancer (NSCLC); oral<br>Orphan Drug      PGx  |
| 6 . duvelisib (Verastem)   | NDA Filed      | 10/05/2018           | Phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma inhibitor for the treatment of patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma and relapsed or refractory follicular lymphoma; oral<br>Orphan Drug |

PGx =Pharmacogenetic test in development

## Top 20 Specialty Pipeline Report July 2018

| Pipeline Drug  | Manufacturer | Current Status | What is this drug being developed for?   |
|--|--------------|----------------|--|
| 7 . entinostat<br>(Syndax Pharmaceuticals)                 | Phase 3      | 2018           | Histone deacetylase inhibitor for the second-line treatment postmenopausal women with advanced ER+ breast cancer with exemestane; oral (once weekly)<br><br>Breakthrough Therapy      PGx                |
| 8 . fremanezumab (Teva)                                    | BLA Filed    | 09/16/2018     | Anti-CGRP mAb for the prevention of chronic and high frequency episodic migraines; subcutaneous therapy.   |
| 9 . golodirsen<br>(Sarepta Therapeutics)                   | Phase 3      | 2019           | Antisense oligonucleotide for the treatment of patients with Duchenne muscular dystrophy amenable to exon 53 skipping; IV infusion<br><br>Orphan Drug  |
| 10 . inotersen<br>(Tegsedi - Ionis Pharmaceuticals /Akcea) | NDA Filed    | 10/06/2018     | Antisense oligonucleotides that targets transthyretin (TTR) for treating hereditary amyloid transthyretin-mediated (hATTR) amyloidosis; SC injection (once weekly)<br><br>Orphan Drug                    |
| 11 . ivosidenib<br>(Tibsovo - Agios Pharmaceuticals)       | NDA Filed    | 08/21/2018     | Isocitrate dehydrogenase 1 inhibitor for the treatment of relapsed or refractory (R/R) acute myeloid leukemia (AML) with an isocitrate dehydrogenase 1 (IDH1) mutation; oral<br><br>Orphan Drug      PGx |

PGx = Pharmacogenetic test in development

## Top 20 Specialty Pipeline Report July 2018

| Pipeline Drug                                     | Manufacturer | Current Status | What is this drug being developed for?   |
|---|--------------|----------------|--|
| 12 . lanadelumab (Shire)                          | BLA Filed    | 08/26/2018     | Fully human MAb that inhibits plasma kallikrein for the prevention of hereditary angioedema attacks; SC (every 2 weeks)<br><br>Breakthrough Therapy Orphan Drug  |
| 13 . larotrectinib (Bayer/Loxo Oncology)          | NDA Filed    | 11/26/2018     | Tropomyosin receptor kinase (TRK) inhibitor for the treatment of unresectable or metastatic solid tumors with TRK-fusion proteins; oral<br><br>Breakthrough Therapy Orphan Drug PGx  |
| 14 . lisocabtagene maraleucel (JCAR017 - Celgene) | Phase 2      | 2018           | 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)<br><br>Breakthrough Therapy  |
| 15 . lorlatinib (Pfizer)                          | NDA Filed    | 08/12/2018     | Anaplastic lymphoma kinase (ALK) tyrosine kinase inhibitor (TKI) for the treatment of patients with ALK-positive metastatic non-small cell lung cancer (NSCLC), previously treated with one or more ALK TKIs; oral<br><br>Breakthrough Therapy Orphan Drug |
| 16 . migalastat (Galafold - Amicus)               | NDA Filed    | 08/13/2018     | Alpha-galactosidase A enhancer for the treatment of Fabry disease; oral (every other day)<br><br>Orphan Drug PGx   |
| 17 . ozanimod (Celgene)                           | Phase 3      | 2020           | S1P receptor modulator for the treatment of relapsing MS; oral   |

PGx = Pharmacogenetic test in development

## Top 20 Specialty Pipeline Report July 2018

| Pipeline Drug                            | Manufacturer      | Current Status | What is this drug being developed for?   |
|--|-------------------|----------------|--|
| 18 . patisiran (Alnylam)                 | NDA Filed         | 08/11/2018     | RNAi therapeutic targeting the transthyretin (TTR) gene for treating hereditary amyloid transthyretin-mediated (hATTR) amyloidosis: IV infusion<br>Breakthrough Therapy      Orphan Drug |
| 19 . romosozumab (Evenity - Amgen / UCB) | Complete Response | 2018           | Humanized antibody that targets and inhibits sclerostin for the treatment of postmenopausal osteoporosis; SC (once monthly for 1 year)   |
| 20 . tafamidis meglumine (Pfizer)        | Phase 3           | 2019           | Transthyretin (TTR) dissociation inhibitor for the treatment of transthyretin cardiomyopathy (TTR-CM); oral<br>Breakthrough Therapy      Orphan Drug                                     |

PGx = Pharmacogenetic test in development