Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q2 2019 – Q3 2019

CVSHealth

Therapeutic Category	Product Name, Route of Administration and Manufacturer[1]	Proposed Indication[1]	Phase of Study[1]	Disease Prevalence and Background	Select Available U.S. Food and Drug Administration (FDA)-Approved Therapies	Comments
Amyloidosis	Vyndaqel (tafamidis meglumine) oral Pfizer	The treatment of hereditary and wild-type transthyretin amyloid cardiomyopathy (TTR-CM)	Pending FDA approval 07/14/2019	Amyloidosis is a rare disease that occurs when amyloid, an abnormal protein produced by the bone marrow, builds up in various tissues or organs including the heart.[2] Symptoms of amyloid cardiomyopathy include increasing fatigue, dizziness, shortness of breath, swelling, atrial fibrillation, and chest pain.[3] Hereditary TTR-CM is caused by an abnormal gene. Wild-type TTR-CM is a slow progressive disease not caused by any known genetic mutation.[2] In the United States, hereditary amyloidosis occurs in approximately 1 in 100,000 Caucasians and more commonly in African-Americans. Wild-type TTR-CM is more common and found mostly in older men.[3,4]	None Onpattro (patisiran) and Tegsedi (inotersen) are FDA approved for the treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis.	Vyndaqel was granted Breakthrough Therapy designation and if approved will be the first drug for the treatment of transthyretin amyloid cardiomyopathy. Vyndaqel will be included in Specialty Guideline Management. *Anticipated impact: New spend*
Human Immuno-deficiency Virus (HIV)	Dovato (dolutegravir/ lamivudine) oral Pfizer/ GlaxoSmithKline/ ViiV Healthcare	The treatment of HIV type-1 infection in treatment-naive adults	Approved 04/08/2019	HIV is a virus spread through certain bodily fluids, which can result in weakening a person's immunity. The human body cannot remove HIV completely, even with treatment. HIV can lead to acquired immunodeficiency syndrome (AIDS), if not treated.[5] An estimated 1.1 million people in the United States, have HIV. Of those people, 15% (1 in 7) are unaware they are infected.[6]	**Complete Regimens:** Atripla (efavirenz/emtricitabine [FTC]/tenofovir disoproxil fumarate [TDF]), Biktarvy (bictegravir/FTC/ tenofovir alafenamide [TAF]), Complera (FTC/rilpivirine/ TDF), Genvoya (elvitegravir/cobicistat/ FTC/TAF), Juluca (dolutegravir/rilpivirine), Odefsey (FTC/rilpivirine/TAF), Stribild (FTC/ elvitegravir/ cobicistat/TDF), Symfi and Symfi Lo (efavirenz/ lamivudine/TDF), Triumeq (dolutegravir/abacavir/ lamivudine)	Dolutegravir/lamivudine is the first approved complete regimen with only two drugs for treatment of naïve HIV-1 infected patients. *Anticipated impact: Replacement spend*

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Oral Oncology	Balversa (erdafitinib) oral Janssen Pharmaceuticals	The treatment of locally advanced or metastatic urothelial cancer and certain fibroblast growth factor receptor (FGFR) genetic alterations whose tumors have progressed after prior chemotherapy	Approved 04/12/2019	Urothelial cancer is the most common type of bladder cancer that starts in the interior lining of the bladder. It is the sixth most common type of cancer in the United States with an estimated 81,190 new cases diagnosed in the United States in 2018.[7] FGFR alterations occur in 10-20% of cases of metastatic urothelial cancer, and are associated with poor outcomes.[8]	None IV therapies FDA approved for urothelial cancer, but not specifically for patients with FGFR alterations, include: Bavencio (avelumab), Imfinzi (durvalumab), Keytruda (pembrolizumab), Opdivo (nivolumab), Tecentriq (atezolizumab)	Erdafitinib has been granted Breakthrough Therapy designation. It is the first oral, targeted agent for urothelial cancer in patients with FGFR alterations who have failed other therapies. Erdafitinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend (shift from medical to pharmacy benefit)*
	entrectinib oral Roche/ Genentech/Ignyta	The treatment of advanced ROS1 fusion-positive non-small cell lung cancer (NSCLC), including in patients with brain metastases, and the treatment of NTRK fusion-positive advanced cancer, regardless of tissue origin	Pending FDA approval 08/18/2019	Lung cancer is the second most common cancer. NSCLC is the most common type of lung cancer. Close to 84% of all lung cancer diagnoses are NSCLC.[9] ROS1 alterations occur in approximately 1% of NSCLC patients.[10] NTRK gene fusion positive cancer is rare. The prevalence is higher in rare pediatric and adult carcinomas; meanwhile, detection is lower in common tumors such as NSCLC, breast, colorectal, renal cell carcinoma, melanoma, and head and neck squamous cell.[11,12,13]	**ROS1 fusion positive NSCLC:** Xalkori (crizotinib) Lorbrena (lorlatinib) − off-label use Zykadia (ceritinib) − off-label use **NTRK fusion positive cancer:** Vitrakvi (larotrectinib)	Entrectinib has been granted Breakthrough Therapy designation. If approved, entrectinib would provide another oral option for ROS1 fusion positive NSCLC in patients with brain metastases and for the treatment of NTRK fusion positive advanced cancer. Entrectinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Oral Oncology (continued)	fedratinib oral Celgene/Impact Biomedicines	The treatment of myelofibrosis (MF)	Pending FDA approval 09/03/2019	MF is a chronic blood cancer. MF occurs when there is excessive scar tissue formation in the bone marrow which results in abnormal production of normal blood cells. Symptoms of MF include tiredness, weakness, or shortness of breath, fullness or discomfort or pain in the left upper area of the abdomen, fever, night sweats, weight loss, bone pain, easy bleeding, and susceptibility to infection.[14] MF affects fewer than 200,000 people in the United States.[15] The annual incidence ranges from 0.3 to 1.5 cases per 100,000 persons.[16]	Jakafi (ruxolitinib)	If approved, fedratinib will provide a once daily option for patients with MF who have insufficient response or cannot tolerate Jakafi. Fedratinib will be included in Specialty Guideline Management. *Anticipated impact: Incremental spend*
	quizartinib oral Daiichi Sankyo	The treatment of relapsed or refractory acute myeloid leukemia (AML) in patients with FLT3-ITD mutation	Pending FDA approval 08/25/2019	AML is a type of blood cancer that starts in certain immature blood cells and progresses quickly.[17] The average lifetime risk for AML is less than 0.5%. It occurs most commonly in individuals 45 years of age and older. Approximately 30% of patients diagnosed with AML have a FLT3-ITD mutation, which is associated with poor outcomes.[18]	Xospata (gilteritinib) (Traditional IV chemotherapy used off-label)	Quizartinib has been granted Breakthrough Therapy designation. Upon approval, it would provide another oral, targeted treatment option for FLT3-ITD mutated AML. Quizartinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Oral Oncology (continued)	selinexor oral Karyopharm Therapeutics	The treatment of penta-refractory multiple myeloma (MM), in combination with dexamethasone	Pending FDA approval 07/06/2019	MM is a cancer of plasma cells. The overgrowth of plasma cells in the bone marrow can lead to low blood counts (red blood cells, white blood cells and platelets). Impaired ability to fight infection as well as bone fractures are other common features of MM.[19] The average lifetime risk for MM is approximately 0.8% with diagnosis most commonly in individuals 55 years of age and older.[20]	**Oral Agents**: Farydak (panobinostat), Ninlaro (ixazomib), Pomalyst (pomalidomide), Revlimid (lenalidomide), Thalomid (thalidomide) **Infused:** Darzalex (daratumumab), Empliciti (elotuzumab), Kyprolis (carfilzomib), Velcade (bortezomib)	If approved, selinexor would represent a new mechanism of action and therapy option for patients that have not responded to multiple prior MM treatments. Selinexor will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*
Psoriasis	risankizumab subcutaneous injection AbbVie/ Boehringer Ingelheim	The treatment of moderate-to-severe chronic plaque psoriasis in adults	Pending FDA approval 04/25/2019	Psoriasis is an chronic autoimmune disease primarily affecting the skin and joints. The most common form, plaque psoriasis, causes thick, scaly patches on the skin that often can itch, cause pain, crack and bleed.[21] Psoriasis is estimated to affect 8 million Americans, or about 2.2% of the population[22], with the plaque psoriasis subtype accounting for 80-90% of cases.[23]	**Topical Agents:** Various creams and ointments used for mild-to-moderate psoriasis **Oral Agents:** Otezla (apremilast) **SC injectable biologic agents:** Cimzia (certolizumab pegol), Cosentyx (secukinumab), Enbrel (etanercept), Humira (adalimumab), Ilumya (tildrakizumab), Siliq (brodalumab), Stelara (ustekinumab), Taltz (ixekizumab), Tremfya (guselkumab)	Risankizumab, if approved, would provide another subcutaneously administered option for treatment of plaque psoriasis. Risankizumab will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

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Rheumatoid Arthritis (RA)	upadacitinib oral AbbVie	The treatment of moderate-to-severe RA in adults	Pending FDA approval 08/20/2019	RA is chronic autoimmune and inflammatory disease which mainly attacks the joints in the hands, wrists and knees.[24] RA affects more than 1.3 million Americans. RA typically begins between the ages of 30 and 50. About 75% of RA patients are women.[25]	**Oral JAK inhibitors:** Olumiant (baricitinib), Xeljanz (tofacitinib), Xeljanz XR (tofacitinib) **Other disease-modifying antirheumatic drugs:** Multiple oral and injectable products are approved for moderate-to-severe RA	Upadacitinib, if approved, would provide another oral, once daily administered option for treatment of moderate-to-severe RA in adults. Upadacitinib will be included in Specialty Guideline Management. *Anticipated impact: Replacement spend*

[1] RxPipeline, March 2019.
[2] Amyloidosis Foundation Facts. Available at http://amyloidosis.org/. Assessed on March 20, 2019.
[3] National Organization of Rare Disorders. Available at https://rarediseases.org/rare-diseases/amyloidosis/. Accessed on March 28, 2019.
[4] Pfizer Pharmaceutical. https://www.pfizer.com/health-wellness/disease-conditions/rare-diseases/transthyretin-amyloidosis. Accessed on March 28, 2019.
[5] Center for Disease Control and Prevention. Available at https://www.cdc.gov/hiv/basics/whatishiv.html. Accessed on March 21, 2019.
[6] Centers for Disease Control and Prevention. HIV in the United States. https://www.cdc.gov/hiv/statistics/overview/ataglance.html. Accessed March 20, 2019.
[7] National Cancer Institute. Cancer Stat Facts: Bladder Cancer. Available at: https://seer.cancer.gov/statfacts/html/urinb.html. Accessed March 25, 2019.
[8] Siefker-Radtke, A, et al. First results from the primary analysis population of the phase 2 study of erdafitinib (ERDA; JNJ-42756493) in patients (pts) with metastatic or unresectable urothelial carcinoma (mUC) and FGFR alterations (FGFRalt). Journal of Clinical Oncology. Abstract #4503.
[9] American Society of Clinical Oncology. https://www.cancer.net/cancer-types/lung-cancer-non-small-cell/statistics. Accessed March 22, 2019.
[10] Gainor J, et al. Novel Targets in Non-Small Cell Lung Cancer: ROS and RET fusions. http://theoncologist.alphamedpress.org/content/18/7/865.full.pdf+html. Oncologist 2013;18:865-875. Accessed on March 22, 2019.
[11] Okamura R, et al. Analysis of *NTRK* Alterations in Pan-Cancer Adult and Pediatric Malignancies: Implications for NTRK-Targeted Therapeutics. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6329466/pdf/nihms-999212.pdf. Accessed on March 27, 2019.
[12] Cocco E, et al. NTRK fusion-positive cancers and TRK inhibitor therapy. Nature Reviews Clinical Oncology. 2018; 15: (12): 731-47. https://www.nature.com/articles/s41571-018-0113-0. Accessed on March 27, 2019.
[13] Chen Y, Chi P. Basket trial of TRK inhibitors demonstrates efficacy in TRK fusion-positive cancers. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5992878/pdf/13045_2018_Article_622.pdf. Accessed on March 27, 2019.
[14] Myeloproliferative Neoplasm Research Foundation. http://www.mpnresearchfoundation.org/Primary-Myelofibrosis. Accessed on March 22, 2019.
[15] Leukemia and Lymphoma Society. https://www.lls.org/myeloproliferative-neoplasms/myelofibrosis. Accessed on March 22, 2019.
[16] National Institute of Health. https://rarediseases.info.nih.gov/diseases/8618/myelofibrosis. Accessed on March 22, 2019.
[17] American Cancer Society. About acute myeloid leukemia (AML). Available at https://www.cancer.org/cancer/acute-myeloid-leukemia/about.html. Accessed March 25, 2019.
[18] Bienz, et al. Risk Assessment in Patients with acute myeloid leukemia and a normal karyotype. Available at https://www.ncbi.nlm.nih.gov/pubmed/15746041. Accessed March 25, 2019.

[19] American Cancer Society. About Multiple Myeloma. Available at https://www.cancer.org/cancer/multiple-myeloma/about/what-is-multiple-myeloma.html. Accessed March 25, 2019.
[20] National Cancer Institute. Cancer Stat Facts: Myeloma. Available at: https://seer.cancer.gov/statfacts/html/mulmy.html. Accessed March 25, 2019.
[21] National Psoriasis Foundation. About Psoriasis. Available at https://www.psoriasis.org/about-psoriasis. Accessed March 25, 2019.
[22] National Psoriasis Foundation. Statistics. Available at https://www.psoriasis.org/content/statistics. Accessed March 25, 2019.
[23] American Academy of Dermatology. What is psoriasis? Available at https://www.aad.org/public/diseases/scaly-skin/psoriasis/what-is-psoriasis. Accessed March 25, 2019.
[24] Centers for Disease Control and Prevention. Available at: https://www.cdc.gov/arthritis/basics/rheumatoid-arthritis.html. Accessed March 22, 2019.
[25] The American College of Rheumatology. https://www.rheumatology.org/I-Am-A/Patient-Caregiver/Diseases-Conditions/Rheumatoid-Arthritis. Accessed March 22, 2019.

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