Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q3 2018-Q4 2018

CVSHealth

Therapeutic Category	Product Name, Route of Administration and Manufacturer[1]	Proposed Indication[1]	Phase of Study[1]	Disease Prevalence and Background	Select Available FDA-Approved Therapies	Comments
Amyloidosis	Tegsedi (inotersen) subcutaneous (SC) injection Akcea Therapeutics/Ionis Pharmaceuticals	The treatment of hereditary transthyretin amyloidosis (hATTR) with polyneuropathy, also known as familial amyloid polyneuropathy	Pending U.S. Food and Drug Administration (FDA) approval 10/06/2018	Amyloidosis is characterized by abnormal protein (amyloid) deposition in various organs and tissues and results in progressive organ dysfunction.[2] hATTR is a rare type of amyloidosis caused by a genetic mutation. Patients with hATTR may have symptoms primarily involving the peripheral nerves (polyneuropathy), though the heart is frequently affected as well. It is estimated that hATTR affects 1 in 100,000 Caucasians in the United States but is more common in African-Americans.	None	Patisiran has been granted Breakthrough Therapy designation. Inotersen and patisiran would be the first FDA-approved therapies for hATTR. Both agents will be included in Specialty Guideline Management. *Anticipated impact: new spend*
	patisiran intravenous (IV) infusion Alnylam Pharmaceuticals		Pending FDA approval 08/11/2018			
Hereditary Angioedema	lanadelumab SC injection Shire	The prevention of acute angioedema attacks in patients 12 years and older with type I and type II hereditary angioedema (HAE)	Pending FDA approval 08/26/2018	HAE is a rare, genetic disorder characterized by episodic attacks of edema (swelling) that can be life-threatening.[3] The edema can affect the face, throat, abdomen and other areas. It is estimated to occur in 1 per 50,000-150,000 Americans. Treatment modalities include routine prophylaxis to prevent attacks, management of acute attacks and limited prophylactic therapy in situations where attacks may occur (e.g., dental surgery, endoscopy).	Prophylaxis: Cinryze (C1 esterase inhibitor, human) IV, danazol oral, Haegarda (C1 esterase inhibitor [human]) SC Additional agents are approved for acute treatment of HAE attacks.	The FDA has granted Breakthrough Therapy designation to lanadelumab. Upon approval, it will provide an additional subcutaneous, self-administered product for the prophylaxis of HAE attacks. Lanadelumab will be included in Specialty Guideline Management. *Anticipated impact: replacement spend*

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Lipid Disorders	Waylivra (volanesorsen) SC injection Akcea Therapeutics/Ionis Pharmaceuticals	The treatment of familial chylomicronemia syndrome (FCS; also referred to as familial lipoprotein lipase deficiency)	Pending FDA approval 08/30/2018	FCS is a genetic disorder characterized by the inability to metabolize certain fats in the blood.[4] As a result, FCS patients have severely elevated triglyceride levels, which can lead to recurrent and potentially fatal pancreatitis. FCS affects approximately 1 per 250,000 people.	None	If approved, volanesorsen would be the first treatment option for FCS. It will be included in Specialty Guideline Management. *Anticipated impact: new spend*
Lysosomal Storage Disorders	Amigal (migalastat) oral Amicus Therapeutics	The treatment of Fabry disease in patients 16 years and older with amenable mutations	Pending FDA approval 08/13/2018	Fabry disease is an inherited enzyme deficiency disorder that results in toxic accumulations of fatty sugar molecules.[5] Complications of Fabry disease include renal failure, heart failure, and stroke. Death often occurs as a result of these complications by the fourth or fifth decade of life. Fabry disease occurs in approximately 1 per 40,000 people. It is estimated that 30 to 50% of this population have mutations amenable to treatment with migalastat.[6]	Fabrazyme (agalsidase beta) IV	Migalastat would offer the first oral treatment option for Fabry disease, and will be included in Specialty Guideline Management. *Anticipated impact: replacement spend (shift from medical to pharmacy benefit)*

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Oral Oncology	Tibsovo (ivosidenib) oral Agios Pharmaceuticals	The treatment of relapsed or refractory acute myeloid leukemia (AML) in patients with isocitrate dehydrogenase-1 (IDH1) mutations	Pending FDA approval 08/21/2018	AML is a type of blood cancer that starts in certain immature blood cells and progresses quickly.[7] The average lifetime risk for AML is less than 0.5%. It occurs most commonly in individuals 45 years of age and older. Approximately 6.4% of AML patients have an IDH1 mutation.[8] Nearly one-third of patients diagnosed with AML have the FLT3 mutation, which is associated with poor outcomes.[9]	None (traditional IV chemotherapy used)	Ivosidenib would offer the first targeted treatment option for IDH1 mutations and will be included in Specialty Guideline Management. *Anticipated impact: replacement spend (shift from medical to pharmacy benefit)*
	gilteritinib oral Astellas	The treatment of relapsed or refractory AML in patients with FLT3 mutations	Pending FDA approval 11/29/2018		None (traditional IV chemotherapy used)	If approved, gilteritinib would be the first targeted agent for FLT3-mutant AML in patients who have failed other therapies. It will be included in Specialty Guideline Management. *Anticipated impact: replacement spend (shift from medical to pharmacy benefit)*
	glasdegib oral Pfizer	The first-line treatment of AML, in combination with low-dose chemotherapy	Pending FDA approval 12/27/2018		Traditional IV chemotherapy	Glasdegib would be an oral alternative for patients who are not candidates for intensive IV chemotherapy. This agent will be included in Specialty Guideline Management. *Anticipated impact: replacement spend (shift from medical to pharmacy benefit)*

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Oral Oncology (continued)	lorlatinib oral Pfizer	The treatment of relapsed or refractory advanced non-small cell lung cancer (NSCLC) in patients with anaplastic lymphoma kinase (ALK) mutations	Pending FDA approval 08/12/2018	Lung cancer is the second most common cause of cancer and is the leading cause of cancer death in the United States.[10] NSCLC is responsible for 80 to 85% of all lung cancer cases.[10] The ALK mutation is present in approximately 5% of NSCLC patients. EGFR mutations occur in ~10% of NSCLC cases in the United States.[11]	Alecensa (alectinib), Alunbrig (brigatinib), Xalkori (crizotinib), Zykadia (ceritinib)	The FDA has granted Breakthrough Therapy designation to lorlatinib. Upon approval, it would provide an additional treatment for relapsed or refractory ALK-positive NSCLC. Lorlatinib will be included in Specialty Guideline Management. *Anticipated impact: replacement spend*
	dacomitinib oral 09/30/2018	The first-line treatment of NSCLC in patients with epidermal growth factor receptor (EGFR) mutations	Pending FDA approval 09/30/2018		Gilotrif (afatinib), Iressa (gefitinib), Tarceva (erlotinib), Tagrisso (osimertinib)	If approved, dacomitinib would offer another therapeutic option for the initial treatment of EGFR-mutant NSCLC. Dacomitinib will be included in Specialty Guideline Management. *Anticipated impact: replacement spend*
	talazoparib oral Pfizer	The treatment of HER2-negative advanced or metastatic BRCA-mutated breast cancer	Pending FDA approval 12/07/2018	BRCA mutations increase the risk of developing breast cancer. About 12 percent of women will have breast cancer in her lifetime; however, up to 65 percent of women with BRCA mutations develop breast cancer. Breast cancer is characterized by the presence or absence of certain receptors, markers, and mutations. BRCA-mutated breast cancer tends to be aggressive and occur in younger women.[12]	Lynparza (olaparib)	Talazoparib would provide another oral, targeted treatment option for BRCA-mutant breast cancer. Talazoparib will be included in Specialty Guideline Management. *Anticipated impact: replacement spend*

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Oral Oncology (continued)	larotrectinib oral Loxo Oncology/ Array Biopharma/ Bayer	The treatment of relapsed or refractory unresectable or metastatic solid tumors with neurotropic tropomyosin receptor kinase (NTRK)-fusion proteins	Pending FDA approval 11/26/2018	NTRK mutations are relatively rare, but occur across many tumor types, including some forms of breast, lung, and colorectal cancers. It is estimated there are fewer than 5,000 patients in the United States with NTRK fusion proteins.[13] Larotrectinib is believed to be tissue agnostic (i.e., effective for tumors with the NTRK biomarker, regardless of tissue origin).	None (traditional IV chemotherapy used)	Larotrectinib has been granted Breakthrough Therapy designation. If approved, it would be the first targeted drug for NTRK fusion protein mutations and would be included in Specialty Guideline Management. *Anticipated impact: replacement spend (shift from medical to pharmacy benefit)*

[1] RxPipeline, July 2018.
[2] National Organization for Rare Disorders. Available at https://rarediseases.org/rare-diseases/amyloidosis/. Accessed March 27, 2018.
[3] Medscape. Hereditary Angioedema. Available at http://emedicine.medscape.com/article/135604-overview#a4. Accessed March 26, 2018.
[4] National Organization for Rare Disorders. Available at: https://rarediseases.org/rare-diseases/familial-lipoprotein-lipase-deficiency/. Accessed March 28, 2018.
[5] Medscape. Available at https://emedicine.medscape.com/article/1952086-overview#a1. Accessed March 27, 2018.
[6] Amicus Therapeutics. Available at http://www.amicusrx.com/fabry_disease.php. Accessed March 27, 2018.
[7] American Cancer Society. About acute myeloid leukemia (AML). Available at https://www.cancer.org/cancer/acute-myeloid-leukemia/about.html. Accessed March 26, 2018.
[8] My Cancer Genome. IDH1 in acute myeloid leukemia. Available at https://www.mycancergenome.org/content/disease/acute-myeloid-leukemia/idh1/. Accessed March 26, 2018.
[9] 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 July 2, 2018.
[10] American Cancer Society. Non-small cell lung cancer. Available at https://www.cancer.org/cancer/non-small-cell-lung-cancer.html. Accessed March 26, 2018.
[11] My Cancer Genome. Lung Cancer. Available at https://www.mycancergenome.org/content/disease/lung-cancer. Accessed June 25, 2018.
[12] National Cancer Institute. BRCA Fact Sheet. Available at https://www.cancer.gov/about-cancer/causes-prevention/genetics/brca-fact-sheet#q1. Accessed June 25, 2018.
[13] Loxo Oncology Pipeline presentation. Available at https://ir.loxooncology.com/docs/events/LOXO_19-Dec-2016_Call_Slides.pdf. Accessed June 25, 2018.