Specialty Pharmacy Pipeline

Drugs to Watch

Anticipated Launches | Q2 2018-Q3 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 — CVS Health Initial Recommendations
Amyloidosis	inotersen subcutaneous 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	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	The FDA is expected to review the applications for inotersen and patisiran by July 6, 2018 and August 11, 2018, respectively. Patisiran has been granted Breakthrough Therapy Designation. If approved, inotersen and patisiran would be the first FDA-approved therapies for hATTR. Both agents will be included in Specialty Guideline Management subsequent to approval.
	patisiran intravenous infusion Alnylam Pharmaceuticals		Pending FDA approval			
Hereditary Angioedema	lanadelumab subcutaneous 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	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) intravenous, danazol oral, Haegarda (C1 esterase inhibitor [human]) subcutaneous *Additional agents are approved for acute treatment of HAE attacks.*	The FDA has granted Breakthrough Therapy Designation to lanadelumab and is expected to review the application by August 26, 2018. If approved, lanadelumab will provide an additional subcutaneous, self-administered product for the prophylaxis of HAE attacks. Lanadelumab will be included in Specialty Guideline Management subsequent to approval.

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Immune (Idiopathic) Thrombocyto-penic Purpura (ITP)	Tavalisse (fostamatinib) oral Rigel Pharmaceuticals	The treatment of chronic immune thrombocytopenia, also known as ITP	Pending FDA approval	ITP is a rare bleeding disorder in which the blood does not clot normally due to thrombocytopenia (i.e., low platelet level) leading to easy bruising and bleeding.[4] ITP is considered chronic when it lasts for longer than one year. It is estimated that ITP occurs in 9-10 per 100,000 adults in the United States. The disease is three times more common in women than in men.	Corticosteroids (intravenous and oral), injectable immune globulins, Nplate (romiplostim) subcutaneous, Promacta (eltrombopag) oral	The FDA is expected to review the application by April 17, 2018. If approved, fostamatinib will offer an additional oral option for ITP. Fostamatinib will be included in Specialty Guideline Management subsequent to approval.
Lipid Disorders	volanesorsen subcutaneous injection Akcea Therapeutics/Ionis Pharmaceuticals	The treatment of familial chylomicronemia syndrome (FCS; also referred to as familial lipoprotein lipase deficiency)	Pending FDA approval	FCS is a genetic disorder characterized by the inability to metabolize certain fats in the blood.[5] 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	The FDA is expected to review the application for volanesorsen by August 30, 2018. If approved, volanesorsen would be the first treatment option for FCS. Volanesorsen will be included in Specialty Guideline Management subsequent to approval.
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	Fabry disease is an inherited enzyme deficiency disorder that results in toxic accumulations of fatty sugar molecules.[6] 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 amenable mutations.[7]	Fabrazyme (agalsidase beta) intravenous	The FDA is expected to review the application by August 13, 2018. If approved, migalastat would be the first oral treatment option for Fabry disease. Migalastat will be included in Specialty Guideline Management subsequent to approval.

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Oral Oncology	binimetinib oral Array BioPharma encorafenib oral Array BioPharma	Combination use for the treatment of advanced malignant melanoma in patients with BRAF V600 mutations	Pending FDA approval	It is estimated that 2.2% of Americans will be diagnosed with melanoma during their lifetime.[8] Although melanoma accounts for only 2% of skin cancer cancers, it is the most common cause of skin cancer death. BRAF mutations are present in 37 to 50% of all malignant melanomas.[9]	**BRAF-targeted, oral combination regimens:** Zelboraf (vemurafenib) + Cotellic (cobimetinib), Tafinlar (dabrafenib) + Mekinist (trametinib)	The FDA is expected to review the applications for binimetinib and encorafenib by June 30, 2018. If approved, the agents will offer another oral combination regimen for BRAF V600 mutation-positive advanced melanoma. Binimetinib and encorafenib will be included in Specialty Guideline Management subsequent to approval.
	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	AML is a type of blood cancer that starts in certain immature blood cells and progresses quickly.[10] 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.[11]	None (Idhifa [enasidenib] is approved for AML with IDH2 mutations)	The FDA is expected to review the application by August 21, 2018. If approved, ivosidenib would offer the first targeted treatment option for AML patients with IDH1 mutations. Ivosidenib will be included in Specialty Guideline Management subsequent to approval.
	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	Lung cancer is the second most common cause of cancer and is the leading cause of cancer death in the United States.[12] NSCLC is responsible for 80% to 85% of all lung cancer cases while the ALK mutation is present in approximately 5% of these cases. Nearly all patients treated with ALK inhibitors will eventually develop resistance.[13]	Alecensa (alectinib), Alunbrig (brigatinib), Zykadia (ceritinib)	The FDA has granted Breakthrough Therapy Designation to lorlatinib and is expected to review the application by August 12, 2018. If approved, lorlatinib would provide an additional treatment for relapsed or refractory ALK-positive NSCLC. Lorlatinib will be included in Specialty Guideline Management subsequent to approval.

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Phenyl-ketonuria (PKU)	pegvaliase subcutaneous injection BioMarin Pharmaceutical/ Merck Serono	The treatment of severe PKU	Pending FDA approval	PKU is an inherited disorder characterized by the lack of an enzyme called phenylalanine hydroxylase which is used by the body to process phenylalanine, an essential building block of proteins.[14] PKU causes toxic buildup of phenylalanine and can lead to complications including seizures, delayed development, behavioral problems, and intellectual disability. PKU occurs in approximately 1 in 10,000 to 15,000 infants born in the United States.	Kuvan (sapropterin) oral – limited to BH4-responsive PKU; most patients with mild PKU and approximately 10% of patients with classic PKU respond to Kuvan.[15]	The FDA is expected to review the application by May 28, 2018. If approved, pegvaliase would be the first enzyme replacement therapy available for PKU. Pegvaliase will be included in Specialty Guideline Management subsequent to approval.
Rare Disorders - Other	burosumab subcutaneous injection Kyowa Hakko Kirin/ Ultragenyx Pharmaceutical	The treatment of X-linked hypophosphatemia (XLH) in pediatric patients one year and older and in adults	Pending FDA approval	XLH is an inherited disorder in which excessive amounts of phosphorus, a mineral needed for proper bone and tooth formation, are eliminated through the urine.[16] Complications of XLH may include bowed legs, impaired growth and short stature, bone pain, and other skeletal and dental abnormalities. XLH occurs in approximately 1 in 20,000 newborns.[17]	None	The FDA is expected to review the application for burosumab by April 17, 2018, and has granted Breakthrough Therapy Designation (pediatric patients). If approved, burosumab would be the first disease-modifying treatment option for XLH. Burosumab will be included in Specialty Guideline Management subsequent to approval.

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Rare Disorders - Other	Ryplazim (plasminogen) intravenous infusion ProMetic BioSciences	The treatment of congenital (type I) plasminogen deficiency	Pending FDA approval	Plasminogen is a protein that is involved in breaking down fibrin, a protein needed in the production of blood clots, to allow for growth of normal tissue. [18] Patients with type 1 plasminogen deficiency, an inherited condition, develop fibrin deposits resulting in inflamed growths on various tissues. Complications vary based on the site of the growths and can include eye damage, vision loss, ulcers, airway obstruction, etc. Congenital plasminogen deficiency occurs in 1 to 2 per 1 million people.	None	The FDA is expected to review the application for plasminogen by April 14, 2018. If approved, the product would be the first treatment option for congenital plasminogen deficiency. Plasminogen will be included in Specialty Guideline Management subsequent to approval.
Rheumatoid Arthritis	Olumiant (baricitinib) oral Eli Lilly/Incyte	The treatment of moderate-to-severe rheumatoid arthritis (RA) in adults who have had an inadequate response or intolerance to methotrexate	Pending FDA approval	RA is an autoimmune disorder in which the immune system attacks healthy tissues.[19] RA can cause pain, stiffness, and decreased function of the hands and feet as well as other joints. Other organs such as the lungs and eyes may be affected as well. RA is estimated to affect more than 1.3 million Americans; approximately 75% of those affected are women.	**Selected biologic injectable agents:** Actemra (tocilizumab), Cimzia (certolizumab pegol), Enbrel (etanercept), Humira (adalimumab), Kevzara (sarilumab), Kineret (anakinra), Orencia (abatacept), Simponi (golimumab) **Selected oral agents:** methotrexate (e.g., Trexall), Xeljanz (tofacitinib), Xeljanz XR (tofacitinib extended release)	The FDA is expected to review the application by June 15, 2018. If approved, baricitinib will offer an additional oral therapy option. Baricitinib will be included in Specialty Guideline Management subsequent to approval.

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