# Specialty Pharmacy Pipeline

## Drugs to Watch

Anticipated Launches | Q1 2018-Q2 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 |
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| Human Immuno-deficiency Virus (HIV) Medications | ibalizumab intravenous injection<br><br>Theratechnologies/ TaiMed Biologics | The treatment of multi-drug resistant HIV, in combination with other antivirals | Pending U.S. Food and Drug Administration (FDA) approval | HIV is a lifelong viral infection that affects CD4 T cells, a type of white blood cell. As the infection progresses, the immune system becomes compromised and is no longer able to effectively fight off infection and disease. There are currently ~1.1 million Americans living with HIV. Though there is no cure for HIV, antiretroviral therapy can effectively control the disease for many years. Today, patients with HIV can have near-normal life spans.[2] | **Agents for Multi-Drug Resistant HIV:** Aptivus (tipranavir) oral, Fuzeon (enfuvirtide) SC injection, Intelence (etravirine) oral<br><br>*Other antiretroviral agents may be considered depending on the results of patient-specific drug resistance testing.* | The FDA is expected to review the application for ibalizumab by April 3, 2018. Ibalizumab has been granted Breakthrough Therapy Designation. If approved, ibalizumab will offer another salvage therapy option for patients with multi-drug resistant disease.<br><br>The FDA is expected to review the application for bictegravir/ emtricitabine/TAF by February 12, 2018. If approved, the product will offer an additional oral complete regimen option.<br><br>Both agents will be evaluated for inclusion in utilization management strategies subsequent to approval. |
| | bictegravir/ emtricitabine/ tenofovir alafenamide (TAF) oral<br><br>Gilead | The treatment of HIV in adults | Pending FDA approval | | **Oral complete regimen fixed-dose combination agents:** Atripla (efavirenz/ emtricitabine/tenofovir disoproxil fumarate [TDF]), Complera (rilpivirine/ emtricitabine/TDF), Genvoya (elvitegravir/ cobicistat/ emtricitabine/TAF), Juluca (dolutegravir/ rilpivirine), Odefsey (rilpivirine/ emtricitabine/TAF), Stribild (elvitegravir/ cobicistat/ emtricitabine/TDF), Triumeq (dolutegravir/ abacavir/lamivudine) | |




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| Immune (Idiopathic) Thrombocyto-penic Purpura (ITP) | Tavalisse (fostamatinib) oral<br><br>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.[3] 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 (IV and oral), IV immune globulins, Nplate (romiplostim) SC injection, 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. |
| Oral Oncology | binimetinib oral<br><br>Array BioPharma<br><br>encorafenib oral<br><br>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.[4] Although melanoma accounts for only 2% of skin cancer cancers, it is the most common case of skin cancer death. BRAF mutations are present in 37 to 50% of all malignant melanomas.[5] | **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. |
| Phenyl-ketonuria (PKU) | pegvaliase subcutaneous injection<br><br>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.[6] 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) – limited to BH4-responsive PKU; most patients with mild PKU and approximately 10% of patients with classic PKU respond to Kuvan.[7] | 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. |




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| Psoriasis | tildrakizumab subcutaneous injection<br><br>Sun Pharma/ Merck | The treatment of moderate-to-severe chronic plaque psoriasis | Pending FDA approval | Psoriasis is an immune disease primarily affecting the skin and joints.[8] The most common form, plaque psoriasis, causes irritated, scale-covered patches on the skin. Psoriasis is estimated to affect 7.5 million Americans, or about 2.2% of the population, with the plaque psoriasis subtype accounting for 80-90% of cases. | **Oral Agent:**<br>Otezla (apremilast)<br><br>**SC injectable biologic agents:**<br>Cosentyx (secukinumab), Enbrel (etanercept), Humira (adalimumab), Siliq (brodalumab), Stelara (ustekinumab), Taltz (ixekizumab), Tremfya (guselkumab) | The FDA is expected to review the application by March 24, 2018. If approved, tildrakizumab will offer another SC option for plaque psoriasis. Tildrakizumab will be included in Specialty Guideline Management subsequent to approval. |
| Rare Disorders - Other | burosumab subcutaneous injection<br><br>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.[9] 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.[10] | None | The FDA is expected to review the application by April 17, 2018, and was granted Breakthrough Therapy Designation (pediatric patients). If approved, burosumab would be the first disease modifying treatment option for X-linked hypophosphatemia. Burosumab will be included in Specialty Guideline Management subsequent to approval. |




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| Rare Disorders - Other | Ryplazim (plasminogen) intravenous infusion<br><br>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.[11] 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 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. |
| Cystic Fibrosis (CF) | tezacaftor/ivacaftor oral<br><br>Vertex | The treatment of CF in patients 12 years of age and older with two copies (homozygous) of the F508del mutation<br><br>The treatment of CF in patients 12 years and older with one copy of F508del-CFTR mutation and a second mutation that results in residual CFTR function. | Pending FDA approval | CF is a genetically inherited disease, there are ~30,000 Americans with CF.[12]<br><br>CF is caused by a mutation of the cystic fibrosis transmembrane conductance regulator (CFTR) gene, which is involved in the transportation of electrolytes and water.[4] The most common CFTR mutation is F508del, with 86.5% of CF patients having at least one copy of this mutation.[13] | **Homozygous F580del mutation:**<br>Orkambi (ivacaftor/ lumacaftor)<br><br>**F508 del/residual function mutations:**<br>Kalydeco (ivacaftor) | The FDA is expected to review the application by February 28, 2018. If approved, tezacaftor/ivacaftor will offer an additional therapy option for CF patients with targeted mutations; it has been granted a Breakthrough Therapy Designation for the homozygous population. Tezacaftor/ivacaftor will be included in Specialty Guideline Management subsequent to approval. |




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