Pipeline Drugs to Watch Report

Q2 2024

Notable Upcoming Launches

CVS Health

Specialty

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER[1]	PROPOSED INDICATION[1]	PHASE OF STUDY[1]	SELECT AVAILABLE FDA-APPROVED THERAPIES*	COMMENTS
Gastrointestinal Disorders	**elafibranor** oral Genfit/Ipsen	The treatment of primary biliary cholangitis (PBC) in adults with inadequate response to ursodiol	Pending FDA approval 06/10/2024	Ocaliva (obeticholic acid) oral **Off-label:** oral fibrates (e.g., fenofibrate) **Pipeline agent:** seladelpar oral (pending FDA approval 08/14/2024)	Elafibranor was granted Breakthrough Therapy designation. If approved, it would provide an alternative second-line treatment option for PBC, with a favorable adverse effect profile. **Anticipated impact:** Incremental spend, pharmacy benefit
Hemophilia, Von Willebrand Disease & Related Bleeding Disorders	**fidanacogene elaparvovec** IV Pfizer	The treatment of severe hemophilia B in adults without inhibitors	Pending FDA approval 04/27/2024	Factor IX products **Gene Therapy:** Hemgenix (etranacogene dezaparvovec-drlb) IV	Fidanacogene elaparvovec was granted Breakthrough Therapy designation. If approved, it would provide an additional gene therapy option for patients with hemophilia B without inhibitors. **Anticipated impact:** Replacement spend, medical benefit
Hormonal Therapies	**TransCon PTH** (palopegteriparatide) SC Acendis Pharma	The treatment of hypoparathyroidism in adults	Pending FDA approval 05/14/2024	Oral calcium + vitamin D Natpara SC (parathyroid hormone): Only available through a special use program that will be permanently discontinued by the end 2024 due to ongoing manufacturing issues.	If approved, TransCon PTH would be the only exogenous parathyroid hormone product, and would provide a treatment option for patients who fail to achieve an adequate response to calcium and vitamin D supplementation. **Anticipated impact:** Incremental spend, pharmacy benefit

Specialty (continued)

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER[1]	PROPOSED INDICATION[1]	PHASE OF STUDY[1]	SELECT AVAILABLE FDA-APPROVED THERAPIES*	COMMENTS
Inflammatory Bowel Disease	**Skyrizi** (risankizumab-rzaa) IV & SC AbbVie/Boehringer Ingelheim	The treatment of moderate-to-severe ulcerative colitis in adults (supplemental indication)	Pending FDA approval 06/28/2024	**IV/SC:** Entyvio (vedolizumab), adalimumab (e.g., Humira and biosimilars), Omvoh (mirikizumab-mrkz), infliximab (Remicade, Zymfentra and biosimilars), Simponi (golimumab), Stelara (ustekinumab) **Oral:** Rinvoq (upadacitinib), Velsipity (etrasimod), Xeljanz/XR (tofacitinib), Zeposia (ozanimod)	Skyrizi would be the third interleukin-23 antagonist approved for ulcerative colitis and would provide an additional therapy option for patients with moderate-to-severe ulcerative colitis. **Anticipated impact:** Replacement spend, pharmacy benefit
Muscular Dystrophy	**Elevidys** (delandistrogene moxeparvovec-rokl) IV Sarepta Therapeutics	The treatment of Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene (supplemental indication)	Pending FDA approval 06/22/2024	**Exon skipping therapies IV:** Amondys 45 (casimersen), Exondys 51 (eteplirsen), Viltepso (viltolarsen), Vyondys (golodirsen) **Pipeline agent:** givinostat oral (pending FDA approval 03/21/2024)	If approved, this expansion would remove the age restriction and ambulatory status requirement from the current label. Elevidys would be added to corticosteroid therapy. **Anticipated impact:** Incremental spend, medical benefit

Specialty (continued)

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER[1]	PROPOSED INDICATION[1]	PHASE OF STUDY[1]	SELECT AVAILABLE FDA-APPROVED THERAPIES*	COMMENTS
Oncology – Intravenous	**Abecma** (idecabtagene vicleucel) IV Celgene/ Bristol-Myers Squibb/ Bluebird Bio	The treatment of adults with relapsed or refractory multiple myeloma who have received 2 to 3 prior lines of therapy (supplemental indication)	Pending FDA approval 03/29/2024	Combination therapy from multiple classes for relapsed or refractory disease: alkylating agents, glucocorticoids, immunomodulatory agents, proteosome inhibitors and anti-CD38 antibodies (e.g., Darzalex [daratumumab] IV/SC, Sarclisa [isatuximab-irfc]) IV, Tecvayli (teclistamab-cqyv) SC, Xpovio (selinexor) oral	If approved, Abecma would move from 5th-line or later to 3rd-line or later, significantly expanding the eligible patient population. The FDA held an Oncologic Drugs Advisory Committee meeting on March 15, 2024, to review data supporting the application for Abecma in earlier lines of therapy. **Anticipated impact:** Replacement spend, medical benefit
	Carvykti (ciltacabtagene autoleucel) IV Johnson & Johnson	The treatment of relapsed or refractory multiple myeloma in patients who have received 1 to 3 prior lines of therapy (supplemental indication)	Pending FDA approval 04/05/2024		If approved, Carvykti would move from 5th-line or later to 2nd-line or later, significantly expanding the eligible patient population. The FDA held an Oncologic Drugs Advisory Committee meeting on March 15, 2024, to review data supporting the application for Carvykti in earlier lines of therapy. **Anticipated impact:** Replacement spend, medical benefit
	imetelstat IV Geron Corporation	The treatment of transfusion-dependent anemia in adults with low- to intermediate-1 risk myelodysplastic syndromes who have failed to respond to, have lost response to, or are ineligible for, erythropoiesis-stimulating agents (ESAs)	Pending FDA approval 06/16/2024	2nd-line therapies: Reblozyl (luspatercept-aamt) SC, lenalidomide oral (e.g., Revlimid)	Imetelstat would provide an additional 2nd-line therapy option in patients who have failed to respond to, have lost response to, or are ineligible for, ESAs. The FDA held an Oncologic Drugs Advisory Committee meeting on March 14, 2024, to review data supporting the application for imetelstat. **Anticipated impact:** Replacement spend, medical benefit

Specialty (continued)

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER[1]	PROPOSED INDICATION[1]	PHASE OF STUDY[1]	SELECT AVAILABLE FDA-APPROVED THERAPIES*	COMMENTS
Oncology – Intravenous (continued)	**patritumab deruxtecan** IV Daiichi Sankyo	The third-line treatment of locally advanced or metastatic non-small cell lung cancer in adults whose tumors have EGFR exon 19 deletions or exon 21 (L858R) substitution mutations who have received prior treatment with a tyrosine kinase inhibitor (TKI) and platinum-based chemotherapy	Pending FDA approval 06/26/2024	3rd-line and later therapies include successive rounds of EGFR inhibitors (e.g. Tagrisso [osimertinib] oral), immunotherapy (e.g., Opdivo [nivolumab] IV, Keytruda [pembrolizumab]) IV, and platinum-based chemotherapy	Patritumab deruxtecan was granted Breakthrough Therapy designation. If approved, it would provide a 3rd-line or later therapy option. There is no standardized treatment for EGFR-mutated non-small cell lung cancer with progression on EGFR-directed TKIs. **Anticipated impact:** Replacement spend, medical benefit
	tarlatamab IV Amgen	The treatment of advanced small cell lung cancer (SCLC) with disease progression on or after platinum-based chemotherapy and at least 1 other prior line of therapy	Pending FDA approval 06/12/2024	2nd-line and later therapies: chemotherapy, Opdivo (nivolumab) IV, Keytruda (pembrolizumab) IV, Zepzelca (lurbinectedin) IV	Tarlatamab was granted Breakthrough Therapy designation. If approved, it would provide a 3rd-line therapy option for SCLC. **Anticipated impact:** Replacement spend, medical benefit
Paroxysmal Nocturnal Hemoglobinuria	**crovalimab** IV & SC Roche/ Genentech/Chugai Pharmaceutical	The treatment of paroxysmal nocturnal hemoglobinuria (PNH) in patients ages 12 years and older	Pending FDA approval 06/22/2024	**IV:** Soliris (eculizumab), Ultomiris (ravulizumab-cwvz) **SC:** Empaveli (pegcetacoplan) **Oral:** Fabhalta (iptacopan)	If approved, this would provide a once monthly, self-administered treatment option for PNH. **Anticipated impact:** Replacement spend, potential shift from medical to pharmacy benefit

Specialty (continued)

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER[1]	PROPOSED INDICATION[1]	PHASE OF STUDY[1]	SELECT AVAILABLE FDA-APPROVED THERAPIES*	COMMENTS
Pulmonary Arterial Hypertension	**sotatercept** SC Merck	The treatment of pulmonary arterial hypertension (PAH) in adults, as an add-on to stable background therapy	Pending FDA approval 03/26/2024	**Inhaled:** Ventavis (iloprost), Tyvaso/ Tyvaso DPI (treprostinil) **Injectable:** epoprostenol (e.g., Flolan, Veletri), Remodulin (treprostinil) **Oral:** Adempas (riociguat), Letairis (ambrisentan), Opsumit (macitentan), Orenitram (treprostinil), Tracleer (bosentan), sildenafil (e.g., Liqrev, Revatio), tadalafil (e.g., Adcirca, Tadliq), Uptravi (selexipag)	Sotatercept was granted Breakthrough Therapy designation and would provide the first potentially disease-modifying therapy in PAH; it would be used in addition to standard background therapy. **Anticipated impact:** Incremental spend, pharmacy benefit
Pulmonary Disorders	**Dupixent** (dupilumab) SC Regeneron Pharmaceuticals/ Sanofi	The treatment of moderate-to-severe chronic obstructive pulmonary (COPD) disease with type 2 inflammation (supplemental indication)	Pending FDA approval 06/27/2024	Daliresp (roflumilast) oral, azithromycin (e.g., Zithromax) oral	Dupixent received Breakthrough Therapy designation for COPD. It would be the first biologic approved for COPD and will be used as add on to triple (or max-tolerated) controller therapy. The manufacturer estimates ~300,000 individuals in the US have uncontrolled COPD with type 2 inflammation. **Anticipated impact:** Incremental spend, pharmacy benefit

The global market for asthma and COPD drugs is growing at an annual rate of more than 5 percent, according to some estimates. Read our Insights post, "**Drugs in the pipeline for COPD**," to learn more.

First-time Biosimilars

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER(S)	REFERENCE PRODUCT AND MANUFACTURER	REFERENCE BRAND INDICATIONS	PROPOSED BIOSIMILAR INDICATION(S)	ANTICIPATED BIOSIMILAR LAUNCH	COMMENTS
Ocular Disorders	**aflibercept** intraocular injection Johnson & Johnson/ Biocon	**Eylea** (aflibercept) Regeneron	• Neovascular (wet) age-related macular degeneration (AMD) • Macular edema following retinal vein occlusion • Diabetic macular edema • Diabetic retinopathy	• Neovascular (wet) AMD • Macular edema following retinal vein occlusion • Diabetic macular edema • Diabetic retinopathy	2Q 2024 (Pending FDA approval 02/25/2024)	Biosimilars for Eylea could launch as early as 2Q 2024. Timing of launch may be subject to patent litigation. Specialty product **Anticipated impact:** Replacement spend (potential for decreased spend), primarily medical benefit
	aflibercept intraocular injection Amgen			Indications TBD	2Q 2024 (Pending FDA approval 06/01/2024)	
	aflibercept intraocular injection Celltrion			Indications TBD	2Q 2024 (Pending FDA approval 06/29/2024)	
	aflibercept intraocular injection Coherus BioSciences/ Formycon/Klinge Biopharma			Indications TBD	2Q 2024 (Pending FDA approval 06/29/2024)	

First-time Biosimilars (continued)

THERAPEUTIC CATEGORY	PRODUCT NAME, ROUTE OF ADMINISTRATION AND MANUFACTURER(S)	REFERENCE PRODUCT AND MANUFACTURER	REFERENCE BRAND INDICATIONS	PROPOSED BIOSIMILAR INDICATION(S)	ANTICIPATED BIOSIMILAR LAUNCH	COMMENTS
Rheumatologic Disorders	**Tofidence** (tocilizumab-bavi) IV Bio-Thera Solutions/ Biogen	**Actemra** (tocilizumab) Genentech/ Roche	• Rheumatoid arthritis • Giant cell arteritis • Systemic sclerosis-associated interstitial lung disease • Polyarticular juvenile idiopathic arthritis (JIA) • Systemic JIA • Cytokine release syndrome	• Rheumatoid arthritis • Polyarticular JIA • Systemic JIA	2Q 2024 (Approved 09/29/2023)	Tofidence may be the first biosimilar of Actemra to launch in the US. At least two other Actemra biosimilars are awaiting FDA approval. Specialty product **Anticipated impact:** Replacement spend (potential for decreased spend), primarily medical benefit

1. RxPipeline, February 2024.

*U.S. Food and Drug Administration