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4TH QUARTER 2019
PIPELINE REPORT

Be "in the know". Confidio is committed to helping clients anticipate pharmaceutical trends and potential shifts in medication cost management. Confidio remains informed of the latest FDA drug approvals, medications in development, and upcoming specialty/biosimilar pipeline therapies.

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Look for these Drug Pipeline Reports each quarter.

PIPELINE HIGHLIGHTS:

Recent Approvals

- **Rinvoq (upadacitinib)** – Moderate to severe rheumatoid arthritis
- **Polivy (polatuzumab vedotin)** – Cancer: Diffuse large B cell lymphoma (DLBCL); Follicular lymphoma; B-cell lymphoma
- **Xpovio (selinexor)** – Cancer: Refractory multiple myeloma
- **Piqray (alpelisib)** – Cancer: Hormone receptor positive breast cancer
- **Vyleesi (bremelanotide)** – Female hypoactive sexual desire disorder

Anticipated FDA Approvals

- **Istradefylline** – Parkinson's disease 8/27/2019
- **AR101** – Peanut allergy 1/2020
- **Crizanlizumab** – Sickle cell disease 1/2020
- **Elexacaftor (VX-445) + tezacaftor + ivacaftor** – Cystic fibrosis 3/2020
- **Vumerity (diroximel fumarate)** – Relapsing forms of multiple sclerosis (MS). Expected FDA decision: 4Q2019
- **RVT-802** – Treatment of primary immune deficiency resulting from congenital athymia. Expected FDA decision: 12/2019

Market Launched Biosimilar Approvals

- **Mvasi (bevacizumab-awwb)** – Biosimilar for reference product Avastin (15% savings) – FDA Approved 9/2017; Launched 7/2019
- **Kanjinti (trastuzumab-anns)** – Biosimilar for reference product Herceptin (15% savings) – FDA Approved 6/2019; Launched 7/2019

Anticipated Biosimilars FDA Launch Dates

- **Ogivri (trastuzumab-dkst)** – Biosimilar for reference product Herceptin – FDA Approved 12/2017; Anticipated Launch Date 11/2019
- **Zirabev (bevacizumab-bvzr)** – Biosimilar for reference product Avastin -- FDA Approved 6/2019; Anticipated Launch Date Q4 2019
- **Truxima (rituximab-abbs)** – Biosimilar for reference product Rituxan – FDA Approved 11/2018; Anticipated Launch Date Q3-4 2019
- **Ruxience (rituximab-pvvr)** – Biosimilar for reference product Rituxan – FDA Approved 7/2019; Anticipated Launch Date Q3-4 2019

Recent FDA Approvals

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
8/27/2019	**Price TBD** **Pharmacy Benefit**	**Brand name** **Nourianz** istradefylline (aka KW-6002) Kyowa Hakko Kirin	**Parkinson's Disease (PD)**	Oral Used together with levodopa/carbidopa for "off" episodes (where levodopa/carbidopa effect is decreased). Once-daily dosing	Istradefylline is the first drug of its type (adenosine A 2A receptor antagonist) for PD Current treatments fall into three categories; each drug in these categories has significant clinical limitations; istradefylline has fewer side effects.	**Therapy Options:** All available as generics with annual WAC cost from $131-$2902 › Dopamine agonists (pramipexole; ropinirole; apomorphine; rotigotine): › COMT inhibitors (entacapone; tolcapone) › MAO B inhibitors (rasagiline; safinamide; selegiline)
8/16/2019	**$59,000** **Pharmacy Benefit**	**Brand name** **Rinvoq** upadacitinib (aka ABT-494) Abbvie	**Inflammatory conditions:** Moderate to severe rheumatoid arthritis	Oral	Janus kinase (JAK) inhibitor. FDA Priority Review status. Also being studied for atopic dermatitis (has FDA Breakthrough Therapy status for this), ulcerative colitis, Crohn's disease.	**Therapy Options:** › Humira (adalimumab) $67,263 › Xeljanz (tofacitinib) $109,025/year
8/16/2019	**$63,875** **Pharmacy Benefit**	**Brand name** **Inrebic** fedratinib Impact Biomedicines Celgene	**Bone Marrow Dysfunction:** Intermediate-2 or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis	Oral	Second generation Janus kinase (JAK) inhibitor, granted priority review by FDA Available through Expanded Access, which allows patients with immediately life-threatening conditions or serious diseases or conditions to receive an investigational drug outside of clinical trials when no alternatives are available. Black box warning flags concerns such as serious and fatal brain damage.	**Therapy Options:** › Preferred: bone marrow replacement with hematopoietic cell transplant $350,000 to $800,000 › Jakafi $159,519

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
6/28/2019	**$102,966.50** **Pharmacy Benefit**	**Brand name** **Thiola EC** tiopronin delayed release Retrophin	**Kidney stones:** Cystinuria, a rare inherited disease leading to recurrent kidney stones.	Oral Product is available as 100mg and 300mg tablets. Its formulation allows dosing with or without food. The original formulation must be taken 1 hour before or 2 hours after meals. Available as 100mg and 300mg tablets.	Some people can manage the disease through increased fluid intake, decreased salt and potassium citrate supplements.	**Therapy Options:** Thiola (tiopronin) (available only as 100mg tablets, same price as Thiola EC) Procedures › Lithotripsy › Ureteroscopy › Nephrolithotomy › Surgery
8/2/2019	**$240,900** **Pharmacy Benefit**	**Brand name** **Turalio** pexidartinib (aka PLX3397) Plexxikon Daiichi Sankyo	**Rare, painful, joint condition:** Pigmented villonodular synovitis (PVNS), also known as TGCT (orphan disease)	Oral PVNS is an uncommon, non-cancerous condition where cells within a joint multiply and cause pain, movement limitations and joint damage. Cause is unknown.	Tyrosine kinase inhibitor FDA has granted priority review status and Breakthrough Therapy designation. The American Society of Clinical Oncology (ASCO) selected pexidartinib as one of five significant advancements of the year in rare disease treatment.	**Therapy Options:** › Surgery
7/3/2019	**$264,000** **Pharmacy Benefit**	**Brand name** **Xpovio** selinexor Karyopharm	**Bone Marrow Cancer:** Penta- Refractory Multiple Myeloma	Oral (80mg twice weekly) FDA application for a topical product was withdrawn.	First in class product (selective inhibitor of nuclear export)	**Therapy Options:** › Bortezomib (Velcade) $68,828 › Carflizomib (Kyprolis) $138,518 › Ixazomib (Ninlaro) $132,834
05/24/2019	**$202,053** **Pharmacy Benefit**	**Brand name** **Piqray** alpelisib Novartis	**Breast Cancer:** Hormone receptor positive (R-positive, HER2-negative, PIK3CA-mutated)	Oral Administered as 300mg with food daily until disease progression or unacceptable toxicity. Used in combination with fulvestrant.	First-in-class Phosphoinositide 3-kinase (PI3K) inhibitor	**Therapy Options:** › Abemaciclib (Verzenio): $152,935 › Palbociclib (Ibrance) $154,559 › Ribociclob (Kisqali): $163,634
06/21/2019	**$899** (First pack of 4 injectors will be free and subsequent refills will cost no more than $99). **Pharmacy Benefit**	**Brand name** **Vyleesi** bremelanotide AMAG Pharmaceuticals Palatin	**Low sexual desire** in females (hypoactive sexual desire disorder, HSDD)	SQ Given 45 minutes before sexual activity, no more than 8X/month. Discontinue after 8 weeks if no response. Indicated only for treatment of premenopausal women with HSDD that causes marked distress or interpersonal difficulty.	Anticipated to launch in September Not for HSDD due to a medical or psychiatric condition, problems in a relationship or medications or drugs. Not for postmenopausal women or in men or to enhance sexual performance.	Addyi: $4800

Recent FDA Approvals *(continued)*

Approval Date	Estimated WAC / Year & Benefit coverage	Drug Name / Manufacturer	Treatment Indication	Disease or Administration Comments	Clinical Comments	Therapy Options Approximate WAC/Year
6/10/2019	**$154,544 for 6 cycles** **Medical Benefit**	**Brand name** **Polivy** polatuzumab vedotin (aka DCDS4501A) Genentech Roche Seattle Genetics	**Lymphoma (cancer):** Diffuse large B cell lymphoma (DLBCL); Follicular lymphoma; B-cell lymphoma	IV Given every 21 days for 6 cycles. Many patients with these cancers have limited treatment options, poor prognosis	First-in-class product, granted priority review status by FDA. Drug is attached to antibody that targets the cancer cells. Used in combination with bendamustine and rituximab.	**Therapy Options:** Various combination drug regimens are used in these cancers, e.g. R-CHOP @ $12,554 for 3 cycles
8/15/2019	**$204,560** **Pharmacy Benefit**	**Brand name** **Rozlytrek** entrectinib (aka RG6268) Roche Genentech	**Cancer:** Solid tumors, including non-small cell lung cancer, that have specific genetic features	Oral	Tyrosine kinase inhibitor FDA has granted Breakthrough Therapy Designation (BTD) status for the treatment of NTRK fusion-positive cancers in some patients. Therapy is personalized.	**Therapy Options** › Vitrakvi $393,600

Anticipated FDA Approvals

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
12/2019	**Brand name** **generic name TBD** RVT-802 Enzyvant Roivant	**Primary immunodeficiency** (complete DiGeorge Syndrome, cDGS)	Comprised of specially-treated cells that are implanted into muscle; the result is production of functioning immune cells. cDGS is a rare pediatric condition, uniformly fatal if untreated (usually within 2 years due to infection)	Tissue-based regenerative therapy Granted by FDA: › Breakthrough therapy designation › Regenerative Medicine Advanced Therapy (RMAT) designation, a component of the 21st Century Cures Act, reserved for cell therapies, therapeutic tissue engineering products, and human cell and tissue products intended to mitigate serious or life-threatening conditions	**Therapy Options:** › Thymic or hematopoietic cell transplantation (HCT)
4Q2019	**Brand name** **Vumerity** diroximel fumarate Biogen Alkermes	**Multiple Sclerosis,** relapsing forms	Oral	May cause fewer gastrointestinal problems compared to Tecfidera, its chemical cousin.	**Therapy Options:** › Tecfidera › Copaxone › Aubagio › Gilenya › Avonex › Rebif › Betaseron › Ocrevus

Anticipated FDA Approvals *(continued)*

Expected FDA Review Date	Drug Name / Manufacturer	Indication	Disease or Administration Comments	Clinical Comments	Therapy Options
1/2020	**Brand name** **generic name TBD** AR101 Aimmune	**Peanut allergy**	Oral powder Peanut allergy is common in children, with an incidence of approximately 1.4-4.5%	Uses gradually increasing doses of peanut protein to desensitize patients. ICER evaluation predicted cost-effectiveness if priced as expected, and concluded that evidence for improved outcomes with AR101 compared to peanut avoidance alone was promising but inconclusive.	**Therapy Options:** › Peanut avoidance › Viaskin (transdermal patch currently in development)
1/2020	**Brand name** **TBD** crizanlizumab Novartis	**Blood condition** (sickle cell disease)	IV In sickle cell disease, deformed red blood cells clog blood vessels, causing severe pain and life-threatening complications.	Blocks adhesion of sickled cells. Granted breakthrough therapy designation by FDA on 1/8/2019.	**Therapy Options:** › Hydroxyurea › Stem cell transplant
3/19/2020	**Brand name** **TBD** elexacaftor/ ivacaftor/ tezacaftor (aka VX-445/ TEZ/IVA) Vertex	**Cystic fibrosis (CF)**	Oral CF is a rare, life-shortening disease caused by one or more of 2000 *CFTR* gene mutations. The mutated genes produce defective proteins, which lead to a buildup of mucus in the lungs, causing chronic infections and progressive damage.	FMechanism of action: Corrects the genetic defect resulting in the defective protein, and helps the healthy proteins to function. Treats the largest group of CF patients who remain without a treatment for the underlying cause of their disease. Breakthrough therapy designation	**Therapy Options:** › Symptomatic treatment
CRL* issued on 8/19/2019	**Brand name** **TBD** golodirsen (aka SRP-4053) Sarepta Therapeutics	**Duchenne Muscular Dystrophy (DMD)**	IV DMD is a fatal genetic neuromuscular disorder affecting one in 3,500 - 5,000 males born worldwide.	Golodirsen targets DMD with a specific genetic mutation that affects about 8% of DMD patients. It is not curative but may delay worsening of symptoms. Cost-effectiveness has been questioned. Was granted accelerated approval and priority review status by FDA.	**Therapy Options:** › Exondys (eteplirsen) › Emflaza (deflazacort) › Prednisone
CRL* issued on 3/22/2019	**Brand name** **Zynquista** sotagliflozin Lexicon Phamaceuticals; Sanofi	**Diabetes:** For patients with type 1 diabetes who lack adequate blood sugar control on insulin therapy.	Oral Used along with insulin	Diabetic ketoacidosis, a serious diabetes complication, was more frequent in those given sotagliflozin compared to insulin alone. In January 2019, the FDA Advisory Committee were evenly divided on whether benefits of Zynquista outweighed risks. Received approval in Europe 8/9/2019.	**Therapy Options:** › Insulin

*CRL (Complete Response Letter) is a communication to a drug's manufacturer from the FDA indicating that the application for the drug cannot be approved in its present form.

Biosimilar Pipeline

Approval Date	Launch Date	Biosimilar Name	Biosimilar WAC / Year Cost	Reference Product	Reference Product WAC / Year Cost	Disease Category*
May 2018	June 2018	Retacrit™ ** (epoetin alfa-epbx)	$154	Epogen Procrit (Epoetin Alfa)	Epogen - $232 Procrit - $360	**Hematopoietic:** Treatment of anemia due to chronic kidney disease, Zidovudine in HIV-infected patients, chemotherapy in cancer patients, and reduction of allogeneic red blood cell transfusion in patients' elective surgery
December 2017	Anticipated November 2019	Ogivri (trastuzumab-dkst)	TBD Upon Launch	Herceptin (trastuzumab)	$$226,895.76 (dosing at 6mg/kg/wk)	**Cancer:** Treatment of Human Epidermal growth factor Receptor 2 (HER2) adjuvant breast cancer, metastatic breast cancer, and metastatic gastric cancer
December 2018	Anticipated December 2019	Herzuma (trastuzumab-pkrb)	TBD Upon Launch			
January 2019	Anticipated December 2019	Ontruzant (trastuzumab-dttb)	TBD Upon Launch			
March 2019	Anticipated December 2019	Trazimera [Pfizer] (trastuzumab-qyyp)	TBD Upon Launch			
June 2019	July 19	Kanjinti ** (trastuzumab-anns)	$192,257.52			
Expected FDA Review TBD	To Be Determined	Grastofil (filgrastim)	TBD Upon Launch	Neupogen (filgrastim)	$5,314	**Hematopoietic:** To reduce the incidence of infection in patents receiving chemotherapy, reduce the duration and time to recovery from neutropenia caused by chemotherapy, mobilization of progenitor blood cells for collection by leukapheresis, and reducing the incidence and duration of complications due to severe neutropenia.
Expected FDA Review TBD	To Be Determined	Filgrastim Kashiv (filgrastim)	TBD Upon Launch			
Expected FDA Review September 2019	Anticipated 4Q 2019	TX01 (filgrastim)	TBD Upon Launch			
March 2015	September 2015	Zarxio® ** (filgrastim-sndz)	$4,390			
August 2012	2015	Granix® ** (tbo-filgrastim)	$3,999			
July 2018	October 2018	Nivestym™ ** (filgrastim-aafi)	$3,504			
September 2017	July 2019	Mvasi** (bevacizumab-awwb)	$123,286.80	Avastin (bevacizumab)	$144,872 (dosing at 10mg/kg q2wks)	**Cancer:** Treatment of Metastatic colorectal cancer, Non-squamous non-small cell lung cancer, Glioblastoma, Metastatic renal cell carcinoma, Persistent, recurrent, or metastatic carcinoma of the cervix
June 2019	Anticipated 4Q 2019	Zirabev (bevacizumab-bvzr)	TBD Upon Launch			

Biosimilar Pipeline *(continued)*

Approval Date	Launch Date	Biosimilar Name	Biosimilar WAC / Year Cost	Reference Product	Reference Product WAC / Year Cost	Disease Category*
December 2017	No US Launch	Ixifi™ (infliximab-qbtx)	Not anticipated to be launched due to acquisition	Remicade (infliximab)	$49,340	**Immunological Agent:** Ankylosing spondylitis, Crohn's disease (fistulizing), adult, Crohn's disease, adult and pediatric (6 years or older), Plaque psoriasis, Psoriatic arthritis, Rheumatoid arthritis in combination with methotrexate, Ulcerative colitis, adult
Expected FDA Review December 2019	Anticipated December 2019	ABP 710 (infliximab)	TBD Upon Launch			
April 2017	July 2017	Renflexis® ** (infliximab-abda)	$31,831			
April 2016	November 2016	Inflectra® ** (infliximab-dyyb)	$39,980			
August 2017	Anticipated 2023	Cyltezo™ (adalimumab-adbm)	TBD Upon Launch	Humira (adalimumab)	$67,263	**Immunosuppressant:** Treatment of ankylosing spondylitis, juvenile idiopathic arthritis, rheumatoid arthritis, psoriatic arthritis, plaque psoriasis, Crohn's disease, and/or ulcerative colitis
Expected FDA Review November 2019	Anticipated 2023	PF-06410293 (adalimumab)	TBD Upon Launch			
July 2019	Anticipated 2023	Hadlima (adalimumab)	TBD Upon Launch			
September 2016	Anticipated 2023	Amjevita™ (adalimumab-atto)	TBD Upon Launch			
October 2018	Anticipated 2022-2023	Hyrimoz (adalimumab-adaz)	TBD Upon Launch			
August 2016	Anticipated 2019-2029	Erelzi® (etanercept-szzs)	TBD Upon Launch	Enbrel (etanercept)	$64,629	**Immunosuppressant:** Ankylosing spondylitis, Juvenile idiopathic arthritis (2 years or older), Plaque psoriasis adult, Psoriatic arthritis, Rheumatoid arthritis
April 2019	Anticipated 2020-2029	Eticovo (etanercept-ykro)	TBD Upon Launch			
November 2018	Anticipated Q 3-4 2019	Truxima™ (rituximab-abbs)	TBD Upon Launch	Rituxan (rituximab)	$40,712	**Cancer:** TTreatment of patients with non-Hodgkin's lymphoma, chronic lymphocytic leukemia, rheumatoid arthritis, granulomatosis with polyangiitis and microscopic polyangiitis.
July 2019	Anticipated Q 3-4 2019	Ruxience (rituximab-pvvr)	TBD Upon Launch			

Biosimilar Pipeline *(continued)*

Approval Date	Launch Date	Biosimilar Name	Biosimilar WAC / Year Cost	Reference Product	Reference Product WAC / Year Cost	Disease Category*
June 2018	July 2018	Fulphila™ ** (pegfilgrastim-jmdb)	$4,175	Neulasta (pegfilgrastim)	$6,231	**Hematopoietic:** Reduce incidence of infection, as manifested by febrile neutropenia, in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with a clinically significant incidence of febrile neutropenia.
November 2018	January 2019	Udenyca** (pegfilgrastim-cbqv)	$4,175			
Expected FDA Review October 2019	Anticipated October 2019	LA-EP2006 (pegfilgrastim)	TBD Upon Launch			
Expected FDA Review TBD	To Be Determined	Lapelga™ CHS-1701 (pegfilgrastim)	TBD Upon Launch			

**Green asterisks above indicate launched products.

*Indications for the biosimilars may vary from the originator and from each other, and are continually evolving. Indications listed here are for the originator product.

The above information was assembled from government and clinical resources for knowledge purposes only. Information and drugs were selected by clinicians based on therapy and potential clinical impact without any manufacture affiliations or conflicts of interest. Approval status, dates, and WAC price are subject to variation. This document should not be exclusively used for decision-making purposes. WAC pricing data should be used for benchmarking purposes only. Prices listed above should not be used alone to set or adjudicate any prices for reimbursement or purchasing functions or considered to be an exact price for a single product and/or manufacturer.

Bibliography

B, A. (2019, August 16). *FDA approves Celgene's bone marrow cancer treatment*. Retrieved from Reuters: https://www.reuters.com/article/us-celgene-fda/fda-approves-celgenes-bone-marrow-cancer-treatment-idUSKCN1V61BS

Biogen. (2019, February 25). *Alkermes and Biogen Announce U.S. Food and Drug Administration Acceptance of Diroximel Fumarate New Drug Application for Multiple Sclerosis*. Retrieved from Globenewswire.com: https://www.globenewswire.com/news-release/2019/02/25/1741439/0/en/Alkermes-and-Biogen-Announce-U-S-Food-and-Drug-Administration-Acceptance-of-Diroximel-Fumarate-New-Drug-Application-for-Multiple-Sclerosis.html

CF News Today. (2019, July 23). *Elexacaftor (VX-445)*. Retrieved from cysticfibrosisnewstoday.com: https://cysticfibrosisnewstoday.com/elexacaftor-vx-445/

Enzyvant. (2017, April 17). *Enzyvant Receives FDA Breakthrough Therapy Designation and Regenerative Medicine Advanced Therapy Designation for Investigational Therapy RVT-802*. Retrieved from roivant.com: https://roivant.com/enzyvant-receives-fda-breakthrough-therapy-designation-and-regenerative-medicine-advanced-therapy-designation-for-investigational-therapy-rvt-802/

Genetic and Rare Diseases Information Center. (2015, May 12). *Cystinuria*. Retrieved from rarediseases.info.nih.gov: https://rarediseases.info.nih.gov/diseases/6237/cystinuria

Institute for Clinical and Economic Review. (2019, June). *A LOOK AT ORAL IMMUNOTHERAPY AND VIASKIN PEANUT FOR PEANUT ALLERGY*. Retrieved from ICER-review.org: https://icer-review.org/wp-content/uploads/2019/07/ICER_PeanutAllergy_RAAG_071019.pdf

IPD Analytics, LLC. (2019, August). IPD Analytics. Retrieved from https://secure.ipdanalytics.com/User/Pharma/RxStrategy.

Joseph, S. (2019, August 6). *UPDATE 1-Amag's femaile libido injectable therapy to sell at $899*. Retrieved from msn.com: https://www.msn.com/en-us/news/world/update-1-amags-female-libido-injectable-therapy-to-sell-at-899/ar-AAFqAFN

Khor S, Beca J, Krahn M, et. Al. (2014, August 14). *Real world costs and cost-effectiveness of rituximab for diffuse large B-cell Lymphoma patients: A population-based analysis*. Retrieved from biomedcentral.com: http://www.biomedcentral.com/1471-2704/14/586.

Liu, A. (2019, August 5). *Daiichi Sankyo wades deeper into oncology with FDA go-ahead in rare joint cancer*. Retrieved from fiercepharma.com: https://www.fiercepharma.com/pharma/daiichi-sankyo-making-oncology-a-key-focus-nabs-turalio-fda-go-ahead-for-rare-joint-cancer

Liu, A. (2019, July 8). *Karyopharm's Xpovio nabs myeloma nod despite checkered past. But 5th line?* Retrieved from fiercepharma.com: https://www.fiercepharma.com/marketing/karyopharm-s-xpovio-nabs-multiple-myeloma-nod-despite-checkered-past-but-5th-line

Mathias, T., & Singh, S. (2019, August 16). *AbbVie prices new rheumatoid arthritis drug at $59,000 a year*. Retrieved from Reuters: https://www.reuters.com/article/us-abbvie-fda/abbvie-prices-new-rheumatoid-arthritis-drug-at-59000-a-year-idUSKCN1V61MJ

Mission Pharmacal Company. Thiola EC Prescribing information (2019, June).

Retrophin. (2019, June 28). *Retrophin Announces FDA Approval of THIOLA ES (tiopronin) 100mg and 300mg Tablets for the Treatment of Cystinuria*. Retrieved from Globenewswire.com: https://www.globenewswire.com/news-release/2019/06/28/1876255/0/en/Retrophin-Announces-FDA-Approval-of-THIOLA-EC-tiopronin-100mg-and-300mg-Tablets-for-the-Treatment-of-Cystinuria.html

Reuters (2019, August 16). *UPDATE 2-Roche cancer treatment priced at $17,050 a month, lower than rival Vitrakvi*. Retrieved from MSN.com: https://www.msn.com/en-us/news/world/update-1-roche-personalized-cancer-treatment-to-cost-about-204560-a-year/ar-AAFU18f

Sarepta Therapeutics Inc. (2019, August 19). *BRIEF-Sarepta Therapeutics Receives Complete Response Letter From The U.S. Food And Drug Administration For Golodirsen New Drug Application*. Retrieved from MSN.com: https://www.msn.com/en-us/news/world/brief-sarepta-therapeutics-receives-complete-response-letter-from-the-us-food-and-drug-administration-for-golodirsen-new-drug-application/ar-AAG277u

Tefferi, A. (2019, February 14). *Management of primary myelofibrosis*. Retrieved from UpToDate.com: https://www.uptodate.com/contents/management-of-primary-myelofibrosis?search=treatment%20of%20myelofibrosis&source=search_result&selectedTitle=1~134&usage_type=default&display_rank=1

Vertex. (2019, May 30). *Vertex Selects Triple Combination Regimen of VX-445, Tezacaftor and Ivacaftor to Submit for Global Regulatory Approvals in Cystic Fibrosis*. Retrieved from Marketwatch.com: https://www.marketwatch.com/press-release/vertex-selects-triple-combination-regimen-of-vx-445-tezacaftor-and-ivacaftor-to-submit-for-global-regulatory-approvals-in-cystic-fibrosis-2019-05-30