TRENDS-in-MEDICINE

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by Lynne Peterson

Quick Takes

…Highlights from this week's news relating to drugs and devices in development that are not covered in other *Trends-in-Medicine* reports…

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NOTE: Subscribe to ***Trends-in-Medicine*** for coverage of the American Heart Association's **International Stroke Conference** in Los Angeles and the **Advances in Genome Biology and Technology** (AGBT) meeting on Marco Island, FL.

Top news of the week (*read details in other sections of Quick Takes*)

✓ Coronavirus – The outbreak continues to spread inside and outside China, and more companies are working on development of diagnostics, treatments, and vaccines.

✓ ESPERION THERAPEUTICS' Nexletol (bempedoic acid) was approved to treat hypercholesterolemia.

✓ INSULET's Omnipod Horizon insulin pump will be integrated with three CGMs: Abbott's FreeStyle Libre and Dexcom's G6 and G7.

✓ **Positive trial news:**

- INCYTE's ruxolitinib (topical formulation) in a second Phase III trial in atopic dermatitis.
- VTV THERAPEUTICS' TTP-399 in Type 1 diabetes as an add-on to insulin.

✓ **The negative news:**

- FIVE PRIME THERAPEUTICS' cabiralizumab + Bristol-Myers Squibb's Opdivo (nivolumab) failed in a Phase II trial in second-line advanced pancreatic cancer.
- MERCK MSD's Keytruda (pembrolizumab) – The FDA rejected expanded use of a 30-minute 400 mg Q6W infusion of this PD-1 inhibitor in 6 cancers.
- TEVA's Austedo (deutetrabenazine), a VMAT2 inhibitor, missed the primary endpoint in two pediatric trials in Tourette syndrome.

SHORT TAKES

■ ABLATIVE SOLUTIONS' Peregrine System – A European postmarketing study of this infusion catheter for renal denervation, published in the ***Journal of the American College of Cardiology: Cardiovascular Interventions***, met the primary endpoint – no major periprocedural vascular complications, major bleeding, acute kidney injury, or death through Day 30.

■ ACTINIUM PHARMACEUTICALS' Iomab-B (iodine-131 apamistamab) – Additional data from the Phase III SIERRA trial in elderly patients with relapsed/refractory acute myeloid leukemia (AML), presented at the Transplantation and Cellular Therapy (TCT) Meetings in Orlando showed that patients who got this anti-CD45 therapy had significantly lower rates of key side effects – febrile neutropenia, sepsis, and mucositis

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vs. control (salvage chemotherapy). The trial had a protocol amendment recently, with the ~30% of patients who are expected to fail induction therapy with venetoclax + hypomethylating agents (current guideline therapy) now eligible for enrollment in SIERRA.

- ALX ONCOLOGY's ALX-148 was granted fast track status by the FDA for two indications: as a first-line treatment for head and neck squamous cell carcinoma and as a second-line treatment of HER2+ gastric or gastroesophageal junction carcinoma.

- BIOCRYST PHARMACEUTICALS' berotralstat (BCX-7353) – The FDA accepted for review the new drug application (NDA) for this oral, once-daily treatment for hereditary angioedema (HAE) attacks. The PDUFA date is December 3, 2020, and the company said the FDA does not plan to have an advisory committee review the drug.

- CARDIOVALVE's transcatheter tricuspid valve replacement (TTVR) system was granted breakthrough device status by the FDA, which also cleared the way for an early feasibility trial to begin.

- CELLECTIS and SERVIER expanded their collaboration on UCART19 products, giving Servier additional rights to develop and commercialize all next-generation gene-edited allogeneic CAR T cell products targeting CD19, including ALLO-501A. Cellectis also regained exclusive control of five undisclosed allogeneic CAR T cell targets covered by the initial agreement.

- DUPONT NUTRITION & BIOSCIENCES is collaborating with MRM Health on new treatments for metabolic diseases, inflammatory bowel diseases, and maybe spondyloarthritis.

- GENFIT's elafibranor (GFT-505) – The interim top-line results of the Phase III RESOLVE-IT trial of this PPARα/δ agonist in non-alcoholic steatohepatitis (NASH), which were expected in March 2020, have been delayed to 2Q20 in order to incorporate the latest FDA guidance. The company insisted the delay is not due to concerns about efficacy, safety, or tolerability.

- HERON THERAPEUTICS' HTX-011 (bupivacaine + meloxicam) – The FDA extended the PDUFA date for this non-opioid postoperative painkiller by three months because, in part, additional data was submitted by the company. The new PDUFA date is June 26, 2020. On a more positive note, the FDA re-inspected the contract manufacturing site and it passed.

- Hospital ratings – A study by Cornell University researchers found that how patients rate hospitals is based more on things like amenities and hospitality services (e.g., quiet rooms, better food, friendly nurses) than on actual care/procedure outcomes.

- IMMATICS BIOTECHNOLOGIES is collaborating with GlaxoSmithKline on development of novel adoptive cell therapies for cancer – T-cell receptor (TCR) therapeutics, starting with autologous T cell therapies with the option to add allogeneic cell therapies using Immatics' ACTallo approach.

- INCYTE's ruxolitinib (topical formulation) – This JAK1/2 inhibitor met the primary endpoint in a second Phase III trial (TRuE-AD1) in atopic dermatitis, with significantly more ruxolitinib patients achieving IGA-TS vs. inactive control (50% with 0.75% and 54% with 1.5% vs. 15% for control). Ruxolitinib also met the secondary endpoints.

- INSULET's Omnipod Horizon Automated Insulin Delivery System – The company announced at the International Conference on Advanced Technologies and Treatments for Diabetes in Madrid that this wearable insulin pump will be integrated with three continuous glucose monitors (CGMs): Abbott's FreeStyle Libre and Dexcom's G6 and G7 (when it is released).

- INTEGER HOLDINGS bought Inomec, an Israeli medical device manufacturer.

- KADMON's KD-025 – Expanded results from the previously reported interim analysis of the ROCKstar trial of this oral ROCK2 inhibitor, presented at the TCT Meetings, showed the objective response rate in graft-versus-host disease was consistent with earlier data across key subgroups. Three patients had a complete response.

- LAKEPHARMA is partnering with NJ Biopharmaceuticals to create a "seamless workflow" on antibody drug conjugates.

- MAGENTA THERAPEUTICS' MGTA-456 – In updated data, presented at the TCT Meetings, from a Phase II trial in patients with severe inherited metabolic disorders – including cerebral adrenoleukodystrophy (cALD), mucopolysaccharidosis type IH (MPS I), and metachromatic leukodystrophy – this hematopoietic stem cell therapy showed clinically meaningful and durable effects. Treated patients experienced early robust engraftment and immune reconstitution.

- MERCK MSD's Keytruda (pembrolizumab) – The FDA rejected six supplemental biologics license applications (BLAs) for a 30-minute 400 mg Q6W infusion to treat melanoma, Hodgkin's lymphoma, primary mediastinal large B-cell lymphoma, gastric cancer, hepatocellular carcinoma, and Merkel cell carcinoma, issuing a complete response letter (CLR). Merck did not say what the issue is.

- PCI PHARMA SERVICES, a supply chain services provider, bought **Bellwyck Pharma Services**, a pharmaceutical packaging and labeling company.
- PFIZER is collaborating with **Saama Technologies** on development and deployment of artificial-intelligence-powered analytics to reduce challenges that clinical trial managers and monitors face in running trials.
- ROCHE's Tecentriq (atezolizumab) – A supplemental BLA for this PD-L1 inhibitor was granted priority review by the FDA as a first-line treatment for advanced non-small cell lung cancer (NSCLC) – squamous and non-squamous – in patients with no EGFR or ALK mutation and with high PD-L1 expression. The PDUFA date is June 19, 2020.
- SEATTLE GENETICS' tucatinib – An NDA for this oral TKI was granted priority review by the FDA for use in combination with Roche's Herceptin (trastuzumab) and Xeloda (capecitabine) to treat breast cancer. The PDUFA date is August 20, 2020.
- TEVA's Austedo (deutetrabenazine) – This VMAT2 inhibitor missed the primary endpoint in two pediatric trials – the Phase II/III ARTISTS-1 and the Phase III ARTISTS-2 – in moderate-to-severe Tourette syndrome, failing to significantly reduce motor and phonic tics on the YGTSS-TTS vs. placebo.
- TOCAGEN is doing a reverse merger with **Forte Biosciences**.
- VIFOR PHARMA and AKEBIA THERAPEUTICS' vadadustat – Vifor bought a priority review voucher for this drug to treat chronic kidney disease, but didn't say from whom or at what price.
- VTV THERAPEUTICS' TTP-399 – In Part 2 of the Phase II Simplici-T1 trial in Type 1 diabetes, this liver-selective glucokinase activator (on top of insulin) met the primary endpoint, significantly reducing HbA_{1c} vs. placebo at Week 12.
- WAT MEDICAL's HeadaTerm TENS – The company reported that a trial done in Turkey found that this non-invasive neuronal electrical stimulation device for treating acute migraines, used for 20 minutes, decreased migraine pain intensity significantly better than control for both 0-120 minutes and at 120 minutes.

Very early research news

- Antibiotics – MIT researchers reported in the journal ***Cell*** that, using artificial intelligence, they found an agent, which they called halicin, that actually could be a powerful new treatment for antibiotic-resistant bacteria. The researchers also found several other promising antibiotic candidates, and they plan to test those further.
- Cystic fibrosis – Dutch researchers reported in the journal ***Cell Stem Cell*** that they were able to correct the CFTR mutation that causes cystic fibrosis using CRISPR with base editing instead of Cas9. However, the study was in organoids, not patients.
- Ovarian cancer – Researchers at Fox Chase Cancer Center have identified a new target for treating high-grade serous ovarian cancer – MRCKA kinase – that could lead to new drugs, MRCKA inhibitors. In a study, published in ***Science Signaling***, they showed that a small molecule MRCKA inhibitor blocked formation of spheroids, suggesting that the drug could reduce resistance to chemotherapy.

NEWS IN BRIEF

FIVE PRIME THERAPEUTICS

- Cabiralizumab. The combination of this CSF-1 receptor inhibitor with Bristol-Myers Squibb's Opdivo (nivolumab), a PD-1 inhibitor, ± chemotherapy missed the primary endpoint in a 160-patient Phase II trial in second-line advanced pancreatic cancer, failing to prolong progression-free survival.
- Licensed several preclinical antibodies to **Seattle Genetics** for use in development of antibody drug conjugate therapies.

CORONAVIRUS (2019-NCOV) UPDATE

- Extent of the problem. More than 78,000 people in mainland China have been infected, and deaths total >2,500, so the fatality rate is back up to 3% (which is 40-50 times the rate for influenza). And Covid-19 appears to be just as fatal outside China as inside.

  The number of cases outside China, though still small in comparison to mainland China, continue to grow, with ≥29 countries affected. South Korea and Italy have initiated regional lockdowns. The situation in northern Italy is so concerning that Austria halted train traffic between the two countries.
- Fatality rate. A new study by Chinese researchers, published in the ***Chinese Journal of Epidemiology***, looked at records for 72,314 patients and found that men are more likely to die from Covid-19 than women. The fatality rate for men was 2.8% vs. 1.7% for women. Men caught the virus just as much as women (51% of confirmed cases were men).

  However, the researchers stopped short of saying men have a biologically worse outlook. Rather, they suggested it may

be other social factors. The unexpected findings of the study: the elderly and people with pre-existing health conditions were most at risk of a fatal case of Covid-19. The fatality rate was 14.8% in people age ≥80, 8% for age 70-79, and 3.6% for age 60-69.

The Chinese researchers put the overall fatality rate for Covid-19 at 2.3%. The pre-existing conditions with the worst outcomes with Covid-19 were: cardiovascular disease (10.5%), diabetes (7.3%). For non-elderly patients with no underlying conditions, the fatality rate was 0.9%.

- **Getting the name right.** The Wuhan coronavirus' correct name is **SARS-CoV-2**. **Covid-19** is the disease that the virus causes.

- **Transmission.** It has been thought that symptoms of infection with the virus take 2-14 days to appear, so that anyone who is exposed but doesn't develop symptoms within 14 days is negative. However, Chinese officials have reported a case where the incubation period was 27 days, suggesting that a 14-day quarantine may not be sufficient in all cases.

  The FDA said there have been no reported cases of transmission of Covid-19 through human cell, tissue, or cellular- or tissue-based products in the U.S. But the Agency said that sites may want to screen to be sure the donors have not traveled to an active Covid-19 area, do not live with someone diagnosed with Covid-19, or themselves have the diagnosis/suspicion of Covid-19.

- **Clinical trials.** Last week, we noted that ~500 clinical trials have a site in Wuhan, China, and ~20% of global trials are now conducted in China or have sites there, but there hadn't been any reports of trial disruption because of the coronavirus. That changed this week. The CEO of IQVIA (formerly Quintiles and IMS Health) said, "Patients are simply not going [to clinical trial sites]. The patients who are enrolled in a trial are simply not going to visit the hospitals where all the sites are in China because that's kind of the more dangerous spot right now."

- **Drug prices.** Sun Pharma said ingredient and finished product prices are increasing because of "speculative buying" due to fear of coronavirus-related price increases.

- **Insurance.** Some Canadian life insurance companies are declining to cover patients who have or have had Covid-19, and some life insurance carriers there are requiring people to be certified disease-free for at least three months to buy a policy. And insurance policies everywhere may have an exclusion for epidemics and pandemics.

- **Diagnostics.**
  - Researchers at Hong Kong Polytechnic University have developed an automated multiplex diagnostic system that detects as many as 40 infectious respiratory disease pathogens, including Covid-19 in ~1 hour.
  - **NOVACYT/PRIMERDESIGN's Covid-19 test** was granted a CE Mark for use by hospitals or laboratories. The company also submitted an emergency use authorization (EUA) application to the FDA.
  - **SEEGENE's Allplex 2019-nCoV Assay** – Korea's Ministry of Food and Drug Safety has approved this second test, a single-tube assay for Covid-19, for emergency use. It also has a CE-IVD Mark in Europe.

- **Treatment.**
  - **ABCELLERA** is working on identifying neutralizing antibodies in people who recovered from Covid-19 in the hopes of finding a treatment.
  - **BIOCRYST PHARMACEUTICALS' galidesivir** – The company is working with the U.S. Biomedical Advanced Research and Development Authority (BARDA) on repurposing this antiviral to treat Covid-19.
  - **Chloroquine phosphate** – China's Ministry of Science and Technology suggested that this old, generic anti-malarial drug should be included in the standard regimen for treating Covid-19.
  - **Gilead Sciences' remdesivir** – The 700-patient clinical trial in China of this antiviral reportedly is having recruitment problems because to enroll a patient must not have used any other treatments in the previous 30 days.
  - **RUXIN MEDICAL SYSTEMS' CoughSync** – This Israeli-developed non-invasive device is under review by Chinese regulators as a way to clear secretions from Covid-19 patient airways. It is already approved in Europe.
  - **SIRNAOMICS** is exploring gene-silencing techniques to see if it can find a way to turn off key genes in the virus.
  - **ZHEJIANG HISUN PHARMA's Favilavir (favipiravir)** – This flu drug, which is approved in Japan, is under review by China's Ministry of Science and Technology as a potential treatment for Covid-19.

- **Vaccines.**
  - **SANOFI** is collaborating with BARDA on development of a recombinant DNA vaccine, based on research conducted by a flu vaccine company Sanofi bought, Protein Sciences.
  - Researchers at the University of Texas at Austin and the National Institutes of Health, working together, reported in the journal *Science* that they have developed a 3D atomic-scale map of the part of coronavirus that attaches itself to human cells and causes infection – what is referred to as the "spike protein." This is considered a

key step toward development of vaccines and antiviral drugs.

- **Unanswered questions.** Some of the major unanswered questions include:
  - If influenza antivirals – e.g., Roche's Tamiflu (oseltamivir) – don't work, *why would an **antiretroviral** drug used to treat HIV work*? Is Covid-19 a retrovirus? Something in between a flu virus and a retrovirus?
  - If Covid-19 requires an antiretroviral-type drug to knock it out, *could it have reservoirs* where it persists in people who get the virus and then seemingly recover – at least test negative? Could it reactivate in the future? ***Trends-in-Medicine*** asked Francis Collins, MD, PhD, director of the National Institutes of Health, this question, and his answer was: "**We don't know yet**."
  - *Is the transmission rate affected by the level of infection?* That is, can mildly infected people transmit the virus as easily or as much as heavily infected people? Can you get the virus as easily from an asymptomatic infected person as from a symptomatic infected person?
  - *Are the tests being used by other countries more accurate than the CDC's test* which has been plagued with false negatives? Why is the CDC's seemingly imperfect test the only one being used in the U.S.? Is it simply because CDC developed it when other tests may be better?
  - Exactly *how is the U.S. quarantine being conducted* of the American cruise ship passengers flown back from Japan who are being held at U.S. military bases in Texas and California? What exactly is the housing? Just how much contact do the they have with each other? If interaction is allowed, what happens if one or more of the quarantined people becomes positive for Covid-19? Would the 14-day quarantine period have to re-start for anyone who had been in any contact (or just close contact) with that infected person? If so, some people could be in quarantine a very long time.

REGULATORY NEWS

Regulatory tidbits

- FDA guidances. The FDA's Center for Biologics Evaluation and Research (CBER) issued a list of the guidance documents it expects to publish this year, and that list includes: consideration for the development of CAR T therapies, human gene therapies for neurodegenerative conditions, and therapies incorporating genome editing.
- Gene-drug interactions. The FDA released a table listing >50 gene-drug interactions, but the FDA said the table is not yet complete and will not affect *current* regulatory requirements/policies, including the policy on companion diagnostics.
- Illegal drugs. The FDA and the government of India discovered 500 shipments of unapproved cancer and HIV drugs as well as opioids at an international mail facility.
- Immunotoxicity. The FDA issued new draft guidance on non-clinical safety evaluations for immunotoxicity, replacing the prior 2002 guidance.
- Orthopedics. The Centers for Medicare and Medicaid Services (CMS) proposed extending its hip and knee replacement payment model – the Comprehensive Care for Joint Replacement Model – for another 3 years. And CMS wants to add outpatient replacements to the model, which currently only covers inpatient procedures.
- Pharmacogenetic testing. The FDA remains concerned about companies promoting pharmacogenetic tests to help manage medication treatment. As a result, the Agency's Center for Drug Evaluation and Research (CDER) and its Center for Devices and Radiological Health (CDRH) collaborated on a table, posted on the FDA website, with the FDA view of these tests. The table is considered a work-in-progress.
- Sterilization. The director of the FDA's CDRH, Jeffrey Shuren, MD, added his name to the list of people/organizations urging the Environmental Protection Agency (EPA) to set emissions limits on ethylene oxide (EtO).
- Vaping. The FDA wants input from the public on what the Agency can do to help prevent future cases of e-cigarette and vaping-associated lung injury (EVALI).

FDA approvals/clearances

- ACUTUS MEDICAL's SuperMap, a real-time heart mapping algorithm for use in atrial fibrillation ablation, was cleared for use.
- ASURAGEN's AmplideX Fragile X Dx and Carrier Screen Kit was approved to help diagnose Fragile X syndrome.
- COMPUMEDICS' Orion LifeSpan Magnetoencephalography, a brain activity-mapping device, was granted 510(k) clearance for use on both adult and pediatric patients.
- ESPERION THERAPEUTICS' Nexletol (bempedoic acid), an oral, once daily adenosine triphosphate (ATP) citrate lyase inhibitor, was approved to treat hypercholesterolemia as an adjunct to maximum tolerated statin therapy in patients with heterozygous familial hypercholesterolemia (HeFH) or established atherosclerotic cardiovascular disease.

- FLOWONIX MEDICAL's Prometra II Pump System was approved for intrathecal baclofen delivery for treating spasticity in several conditions, including multiple sclerosis.
- HERMES MEDICAL SOLUTIONS' Affinity, viewing and analysis software for nuclear medicine and molecular imaging exams, was granted 510(k) clearance.
- KINETICOR's Motion Correction System, an app for helping to reduce the need for repeat MRI scans due to unintentional patient movement, was granted 510(k) clearance for use with Siemens Healthineers' Magnetom Skyra 3T MRI scanner.
- LUNDBECK/ALDER BIOPHARMACEUTICALS' Vyepti (eptinezumab, ALD-403), a CGRP inhibitor, was approved to treat migraine.
- NORTHSTAR MEDICAL RADIOISOTOPES – Two additional molybdenum-99 filling lines were approved at the company's facility in Columbia MO, which the company said will allow it to immediately increase Mo-99 production.
- RECRO PHARMA/BAUDAX BIO's Anjeso (IV meloxicam) – A third try at approval of this long-acting NSAID was successful; it was approved for the management of moderate-to-severe pain.

FDA recalls/warnings

- ABBOTT's NC Trek RX and NC Traveler RX, coronary dilatation catheters, were recalled (Class I) because balloons from affected lots may not deflate as intended.
- BECTON DICKINSON's Alaris extension sets – The company issued a field safety notice about some lots, urging users to inspect and quarantine affected devices, and destroy unused units because of reports that the devices' male luer becoming disconnected from the female luer, possibly resulting in medication leakage and therapy disruptions.
- RESMED's Stellar 100 and 150, non-invasive and invasive ventilators, were recalled (Class I) due to sound alarm failures.
- TARO PHARMACEUTICALS' phenytoin (oral suspension) – Two lots of this seizure medicine were voluntarily recalled because of possible under/over dosing due to the product not re-suspending when shaken.
- TELEFLEX MEDICAL's Comfort Flo Humidification Systems manufactured before June 2019 were recalled (Class I) worldwide due to the potential for water to flood the column and enter the circuit which could be aspirated by the patient.

European Regulatory News

- ABBOTT's Gallant, implantable cardioverter defibrillators (ICDs) and cardiac resynchronization therapy defibrillators (CRT-Ds), were granted a CE Mark.
- CERUS ENDOVASCULAR's Contour Neurovascular System for treating bifurcated saccular intracranial aneurysms, was granted a CE Mark.
- MERIT MEDICAL SYSTEMS' Cianna Scout, a wire-free surgical guidance system, was granted a CE Mark.
- QUOTIENT LIMITED's Initial Serological Disease Screening Microarray was granted a CE Mark for use with the company's MosaiQ diagnostic platform in detecting syphilis and cytomegalovirus.

Regulatory news from other countries

- *Africa.* MERCK MSD's Ervebo, an Ebola vaccine, was approved by the national health authorities of four countries: Burundi, the Democratic Republic of the Congo, Ghana, and Zambia.
- *Canada.* Health Canada said it will begin accepting some clinical trial submissions in electronic common technical document format.
- *China.*
  - ROCHE/CHUGAI's Rozlytrek (entrectinib) was granted expanded approval for the treatment of ROS1+ unresectable/advanced/metastatic NSCLC.
  - VIFOR PHARMA formed a joint venture with **Fresenius Kabi** in China that will focus on patient blood management.
- *Japan.* BRISTOL-MYERS SQUIBB's Opdivo (nivolumab) – The Ministry of Health, Labour, and Welfare (MHLW) approved this PD-1 inhibitor to treat unresectable/recurrent esophageal cancer in patients whose cancer progressed after chemotherapy, without regard to PD-L1 status.

2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
February 24	**FDA Rare Disease Day:** Supporting the future of rare disease product development	FDA public meeting
Feb. 25-26	Discussion of the evolving role of **artificial intelligence in radiological imaging**	FDA public workshop
February 26	AM: **STEBA Biotech's padeliporfin di-potassium** powder for solution for injection for treating localized prostate cancer PM: **Lilly's Cyramza** (ramucirumab) – expanded approval for use with erlotinib as a first-line treatment of metastatic NSCLC with EGFR exon 19 deletions or exon 21 substitution mutations	FDA's Oncologic Drugs Advisory Committee
February 26	**Acacia Pharma's Barhemsys** (IV amisulpride) for post-operative nausea and vomiting (PONV)	PDUFA date
February 26	**Esperion Therapeutics' bempedoic acid in combination with ezetimibe** to treat hypercholesterolemia	PDUFA date
February 27	**BeiGene's zanubrutinib**, a BTK inhibitor for mantle cell lymphoma	PDUFA date
February 28	Criticality of **tobacco use assessment** in oncology therapeutic trials	FDA-AACR-IASLD workshop
March 3	Development of **individualized therapeutics**	FDA workshop
March 4	Selection of strains for the 2020/2021 flu vaccine	FDA's Vaccines and Related Biological Products Advisory Committee
March 5	Advancing animal models for **antibacterial drug development**	FDA public workshop
March 5	Medical **extended reality**: evaluation practices for virtual and augmented reality	FDA public workshop
March 9	**Intarcia Therapeutics' ITCA-650** (exenatide implant) for Type 2 diabetes	PDUFA date
March 9	Competitive marketplace for **biosimilars**	FDA/FTC public workshop
March 9	Detection of **circulating tumor DNA** for cancer screening	FDA public workshop
March 10	**Bristol-Myers Squibb's Opdivo** (nivolumab) **+ Yervoy** (ipilimumab) for advanced hepatocellular carcinoma	PDUFA date
March 10	Patient-focused drug development for **stimulant use disorder**	FDA public meeting
March 11	Use of **over-the-counter antiseptics** in the food handler setting	FDA's Non-Prescription Drugs Advisory Committee
March 12	U.S.-Japan **cellular and gene therapy conference**: Exosomes in cancer treatments and other diseases	FDA conference in conjunction with Japan's Ministry of Education, Culture, Sports, Science, and Technology
March 15	**Astellas and Seattle Genetics' enfortumab vendotin**, an antibody-drug conjugate for treating metastatic/locally-advanced urothelial cancer	PDUFA date
March 17	**Eton Pharmaceuticals' ET-105** (reformulated lamotrigine) as adjunctive therapy for partial seizures in Lennox-Gastaut syndrome patients	PDUFA date
March 23	Identification of concepts and terminology for **multi-component biomarkers**	FDA workshop
March 25	**Bristol-Myers Squibb/Celgene's ozanimod** (RPC-1063) for relapsing MS	PDUFA date
March 25	**Zogenix's Fintepla** (fenfluramine, ZX-008) for Dravet syndrome	PDUFA date
March 26	**Heron Therapeutics' HTX-011** (bupivacaine + meloxicam) for postoperative pain	PDUFA date **Extended by the FDA to June 26, 2020**
March 26	**IntelGenx Technologies' Rizaport** (RHB-103) for migraine	PDUFA date
March 27	Modernizing **FDA's data strategy**	FDA public meeting
March 28	**Rockwell Medical's Triferic** (ferric pyrophosphate) for anemia	PDUFA date
March 30	Patient-focused drug development for **vitiligo**	FDA public meeting
March 31	Use of patient preference information in medical device regulatory decisions – risk:benefit and beyond	FDA public workshop
March tba	**AstraZeneca's Imfinzi** (durvalumab) + tremelimumab for small cell lung cancer	PDUFA date (*estimated late March*)
April tba	**Lilly's empagliflozin + linagliptin + metformin extended-release**, a triplet for Type 2 diabetes	PDUFA date
April tba	**Puma Biotechnology's Nerlynx** (neratinib) – expanded approval as a ≥3-line treatment for HER2+ metastatic breast cancer	PDUFA date
April 1	FDA communications about the **safety of medical devices**	FDA public meeting
April 2	Reducing the risk of **Zika virus transmission** in blood and blood components	FDA's Blood Products Advisory Committee
April 3	Testing for **hepatitis B** surface antigen (HBsAg) in blood donations	FDA's Blood Products Advisory Committee
April 3	Consultation on **International Council for Harmonisation** (ICH)	FDA and Health Canada joint meeting

more - 2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
April 4	**Bristol-Myers Squibb/Celgene and Acceleron Pharma's Reblozyl** (luspatercept-aamt) – expanded approval to include very low-to-intermediate risk myelodysplastic syndrome-associated anemia in patients with ring sideroblasts who require red blood cell transfusions	PDUFA date
April 13-15	Good **simulation practices** in health technologies	FDA public workshop
April 20-21	Annual **Sentinel** review	FDA public workshop
April 21	**GlaxoSmithKline's Trelegy Ellipta** (fluticasone furoate + umeclidinium + vilanterol inhalation powder) – claim for reduction in all-cause mortality in COPD	FDA's Pulmonary-Allergy Drugs Advisory Committee
April 22 *tentative*	**Intercept Pharmaceuticals' obeticholic acid** to treat liver fibrosis due to non-alcoholic steatohepatitis (NASH)	FDA's Gastrointestinal Drugs Advisory Committee
April 23	**Lilly/Avid Radiopharmaceuticals' flortaucipir F18**, an IV radioactive diagnostic agent for PET imaging of the brain	FDA's Medical Imaging Drugs Advisory Committee
April 25	**Sanofi's MenQuadfi**, a meningococcal vaccine	PDUFA date
April 26	**Neurocrine Biosciences' opicapone** to treat Parkinson's disease	PDUFA date
April 27	**United Therapeutics' Trevyent** (treprostinil) to treat pulmonary arterial hypertension (PAH)	PDUFA date
April 29	Topic not announced yet	FDA's Pediatric Advisory Committee
April 30	**Sanofi's isatuximab** for relapsed/refractory multiple myeloma	PDUFA date
May 4	**2020 generic drug** regulatory science initiatives	FDA public workshop
May 12	**Johnson & Johnson and Halozyme's Darzalex** (daratumumab) subcutaneous delivery for multiple myeloma	PDUFA date (*estimated*)
May 12-13	**Regulatory education for industry**	FDA conference
May 14	**Sunovion Pharmaceuticals' dasotraline** for moderate-to-severe binge eating disorders	PDUFA date
May 14	**Blueprint Medicines' Ayvakit** (avapritinib, BLU-285) for *fourth-line* GIST	PDUFA date *extended by FDA from February 14*
May 15	**Allergan's bimatoprost sustained-release** for treating glaucoma	PDUFA date (*estimated*)
May 15	**Clovis Oncology's Rubraca** (rucaparib) – expanded approval to treat advanced prostate cancer	PDUFA date
May 24	**Roche's risdiplam** (RG-7916) to treat spinal muscular atrophy	PDUFA date
May 25	**Evofem Biosciences' Amphora** (L-lactic acid + citric acid + potassium bitartrate), a hormone-free contraceptive (resubmission)	PDUFA date
May 26	**Regeneron Pharmaceuticals and Sanofi's Dupixent** (dupilumab) – expanded approval to include treatment of children age 6-11 with atopic dermatitis	PDUFA date
May 30	**Incyte's pemigatinib** for previously treated, locally-advanced/metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements	PDUFA date
June 2	**Immunomedics' sacituzumab govitecan** (IMMU-132) as a ≥3-line treatment for metastatic triple-negative breast cancer	PDUFA date
June 2	**Foamix Pharmaceuticals' FMX-103** (minocycline foam) to treat moderate-to-severe papulopustular rosacea	PDUFA date
June 4	**Merck MSD's Recarbrio** (imipenem + cilastatin + relebactam) – expanded approval to treat hospital-acquired Gram-negative bacterial pneumonia and ventilator-associated bacterial pneumonia (HABP/VABP)	PDUFA date
June 11	**Viela Bio's inebilizumab** for first-line monotherapy of neuromyelitis optica spectrum disorder	PDUFA date
June 18	**Epizyme's Tazverik** (tazemetostat) – expanded approval to treat relapsed/refractory follicular lymphoma	PDUFA date
June 19	**Roche's Tecentriq** (atezolizumab) – expanded use as first-line treatment for advanced NSCLC with high PD-L1 expression and no ALK or EGFR mutations	PDUFA date
June 26	**Heron Therapeutics' HTX-011** (bupivacaine + meloxicam) for postoperative pain	PDUFA date **Extended by the FDA from March 26**
June 26	**Intercept Pharmaceuticals' obeticholic acid** to treat liver fibrosis due to non-alcoholic steatohepatitis (NASH)	PDUFA date *extended from March 26*
July 13	**Averitas Pharma's Qutenza** (capsaicin patch) – expanded approval to treat neuropathic pain from diabetic peripheral neuropathy	PDUFA date

more - 2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
August 5	**DBV Technologies' Viaskin Peanut** for treating children with peanut allergy	FDA target action date
August 10	**Gilead Sciences/Kite Pharma's KTE-X19** for relapsed/refractory mantle cell lymphoma	PDUFA date
August 13	**Deciphera Pharmaceuticals' ripretinib** (DCC-2618) for GIST	PDUFA date
August 17	**Bristol-Myers Squibb/Celgene/Juno Therapeutics' lisocabtagene maraleucel** (JCAR-017, liso-cel) for DLBCL	PDUFA date
August 20	**Seattle Genetics' tucatinib** – in combination with Roche's Herceptin + Xeloda – to treat breast cancer	PDUFA date
August 27	**Cassiopea's clascoterone cream 1%**, a topical androgen receptor inhibitor for acne	PDUFA date
September 27	**Aquestive Therapeutics' Libervant** (diazepam buccal film) for seizure clusters in epileptics	PDUFA date
Sept. 28-30	Global summit on regulatory science: **Emerging Techologies**	FDA summit **https://aralliance.org/gsrs/**
October 24	**Spectrum Pharmaceuticals' Rolontis** (eflapegrastim) for treating chemotherapy-induced neutropenia	PDUFA date
November 15	**Alkermes' ALKS-3831** (olanzapine + samidorphan) for bipolar I disorder and schizophrenia	PDUFA date
November 25	**Revance Therapeutics' daxibotulinumtoxinA** for moderate-to-severe glabellar lines	PDUFA date
December 3	**BioCryst Pharmaceuticals' berotralstat** (BCX-7353) for hereditary angioedema attacks	PDUFA date
December 20	**FibroGen and AstraZeneca's roxadustat** for anemia of chronic kidney disease	PDUFA date