TRENDS-in-MEDICINE

February 16, 2020

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 WORLD*Symposium* meeting on lysosomal diseases in Orlando.

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

✓ **Coronavirus** – The director of the CDC told Congress that "there is no evidence that this outbreak is at all under control." More than 68,000 people in China have contracted the virus, with >1,600 deaths. Cases outside China are still low, generally travelers who had been in China. The list of companies working on diagnostics, treatments, and vaccines continues to grow.

✓ EISAI's Belviq and Belviq XR (lorcaserin), a diet drug, is being voluntarily taken off the market because of an increased cancer risk.

✓ **The positive trial news:**

- BRAINSWAY's H6-coil – a dTMS system for adults with ADHD.
- MARKER THERAPEUTICS' MultiTAA – The FDA lifted the clinical hold on a trial of this T-cell therapy in AML.
- MERCK MSD's Keytruda (pembrolizumab) in a Phase III trial in PD-L1+ metastatic triple-negative breast cancer.
- PFIZER and ASTELLAS' Xtandi (enzalutamide) prolonged survival in a Phase III trial in non-metastatic castration-resistant prostate cancer.
- SEATTLE GENETICS and ASTELLAS' Padcev (enfortumab vedotin-ejfv) in a Phase Ib/II trial in previously untreated locally advanced/metastatic urothelial cancer.

✓ **The negative trial news:**

- BIOHAVEN PHARMACEUTICALS' troriluzole (BHV-4157) in a Phase III trial in generalized anxiety disorder.
- LOGICBIO THERAPEUTICS' LB-001 – A planned trial of this gene therapy for MMA was put on clinical hold by the FDA.
- OCULIS' OCS-01 (nanoparticle dexamethasone) in a Phase II trial in diabetic macular edema.
- ROCHE's gantenerumab, an IgG1 antibody, in autosomal dominant Alzheimer's disease.

SHORT TAKES

- AQUESTIVE THERAPEUTICS' Libervant (diazepam buccal film) – The FDA accepted for review a new drug application (NDA) for this oral benzodiazepine to help manage seizure clusters in refractory epilepsy patients. The PDUFA date is September 27, 2020.

- ARBUTUS BIOPHARMA's AB-452 – The company is giving up on this oral hepatitis B-specific RNA destabilizer and will focus instead on AB-729, an injectable next-generation RNAi.

- ARIA CV's Aria PH System, a device for treating pulmonary arterial hypertension, was granted breakthrough device designation by the FDA.

- ASTRAZENECA is giving up on two investigational cancer drugs – MEDI-7247, an anti-ASCT2 antibody-drug conjugate, and the combination of AZD-4635 (an anti-A2A) and oleclumab (MEDI-9447, an anti-CD73) for EGFR-mutated non-small cell lung cancer (NSCLC).

- BECTON DICKINSON is partnering with **Babson Diagnostics** on a long-term project to bring lab-quality diagnostic testing to retail pharmacies, combining Babson's automated sample handling and analytical technology with BD's capillary blood collection device.

- BIOHAVEN PHARMACEUTICALS' troriluzole (BHV-4157) missed the primary endpoint in a Phase III trial in generalized anxiety disorder (GAD), failing to show better efficacy than placebo, and development in GAD is being halted. However, the company is continuing to study this tripeptide glutamate modulator in obsessive compulsive disorder, Alzheimer's disease, and spinocerebellar ataxias (a genetic movement disorder).

- BRAINSWAY's H6-coil – In a 75-patient Israeli trial in adults with attention-deficit/hyperactivity disorder (ADHD), this deep transcranial magnetic stimulation (dTMS) system significantly improved patient-reported assessments of inattention. In addition, functional MRI showed a significant increase in activity in an area of the brain known to express reduced activity in adult ADHD.

- CT – According to a report by IMV Medical Information Division, >90 million CT scans were performed in the U.S. in 2019, up 3% from 2018, with about half of these performed on an emergency basis.

- DECIPHERA PHARMACEUTICALS' ripretinib (DCC-2618), a "switch pocket inhibitor," was granted priority review by the FDA as a treatment for advanced gastrointestinal stromal tumors (GIST). The PDUFA date is August 13, 2020.

- EPIZYME's Tazverik (tazemetostat) – The FDA accepted a supplemental new drug application (sNDA) for priority review for expanded approval of this EZH2 inhibitor as a treatment for relapsed/refractory follicular lymphoma. The PDUFA date is June 18, 2020.

- ETERNYGEN – A preclinical study, presented at the Global NASH Congress in London, U.K., suggests that inhibiting the plasma membrane tricarboxylate transporter INDY may be a new approach to treating non-alcoholic steatohepatitis (NASH).

- FIBROGEN and ASTRAZENECA's roxadustat – The FDA accepted an NDA for this orally administered, small molecule hypoxia-inducible factor (HIF) prolyl hydroxylase inhibitor to treat anemia in chronic kidney disease (CKD) in dialysis and non-dialysis patients. The PDUFA date is December 20, 2020.

- GILEAD SCIENCES/KITE PHARMA's KTE-X19 – The FDA granted priority review to the BLA for this CAR T therapy in relapsed/refractory mantle cell lymphoma. The PDUFA date is August 10, 2020.

- GLYCOMIMETICS' rivipansel – **Pfizer** ended it licensing agreement over this failed treatment for vaso-occlusive crisis in sickle cell disease patients, returning all rights to GlycoMimetics.

- INTUITIVE SURGICAL bought **Orpheus Medical**, a health information technology company in Israel.

- KALVISTA PHARMACEUTICALS' KVD-001 – **Merck MSD** abandoned its option on this kallikrein inhibitor, which failed in a Phase II trial in diabetic macular edema (DME).

- KLEO PHARMACEUTICALS' KP-1237 – The FDA approved an investigational new drug (IND) application for this CD38-targeting antibody recruiting molecule (ARM), clearing the way for a trial, in combination with autologous natural killer cells, to treat multiple myeloma in patients who have already had a hematopoietic stem cell transplant.

- LIGAND PHARMACEUTICALS is buying the core assets of **Icagen**, including programs Icagen partnered with Roche in neurological diseases and with the Cystic Fibrosis Foundation in cystic fibrosis. Ligand is also getting six preclinical programs in diabetes, Parkinson's disease, pain, and other disorders.

- LOGICBIO THERAPEUTICS' LB-001 – The FDA put a planned I/II trial of this gene therapy – a recombinant adeno-associated viral vector with human methylmalonyl-COA mutase (MMUT) – for treating methylmalonic acidemia (MMA) on hold. The Agency wants answers to some undisclosed clinical and non-clinical questions, and the company said it hopes to provide those answers within 30 days.

- MARKER THERAPEUTICS' MultiTAA (multi-tumor associated antigen) – The FDA lifted the clinical hold on a trial of this T-cell therapy in post-transplant acute myeloid leukemia (AML). The hold was placed in November 2019 when the FDA asked for more technical and manufacturing information, and the company resolved the issue by finding another supplier.
- MEDTRONIC bought Digital Surgery, a surgical artificial intelligence company, to bolster its surgical robotics work.
- NOVARTIS' capmatinib, a MET inhibitor, was granted priority review by the FDA as a treatment for METex14 mutated advanced NSCLC.
- NQ MEDICAL's neuroQWERTY, software that tracks the progression of Parkinson's disease, was granted breakthrough device designation by the FDA.
- OCULIS' OCS-01 (nanoparticle dexamethasone) – This topical eye drop missed the primary endpoint in the 144-patient, 12-week Phase II DX-211 trial in diabetic macular edema (DME), failing to significantly improve best corrected visual acuity (BCVA) – +2.62 letters vs. +1.04 letters with placebo (p=0.125). However, it did significantly decrease central macular thickness (CMT).
- PREVAIL THERAPEUTICS' PR-001, an AAV-based gene therapy for neuronopathic Gaucher disease, was granted rare pediatric disease designation and orphan drug status by the FDA.
- ROCHE's gantenerumab, an IgG1 antibody, missed the primary endpoint in an investigator-initiated Phase II/III trial in autosomal dominant Alzheimer's disease, failing to significantly slow the rate of cognitive decline (using the DIAN multivariate cognitive endpoint) vs. placebo. Details will be presented at the Advances in Alzheimer's and Parkinson's Therapies (AAT-AD/PD) Focus Meeting in Vienna, Austria, in April 2020. However, Roche is not giving up on gantenerumab; the two ongoing Phase III trials in the common form of Alzheimer's disease are continuing.
- SEATTLE GENETICS and ASTELLAS' Padcev (enfortumab vedotin-ejfv) – The updated results of the Phase Ib/II EV-103 trial in previously untreated locally advanced/metastatic urothelial cancer patients ineligible for treatment with cisplatin-based chemotherapy, presented at the ASCO Genitourinary Cancers Symposium (ASCO-GU) in San Francisco, showed that adding this Nectin-4-directed antibody and microtubule inhibitor conjugate to Merck MSD's Keytruda (pembrolizumab), a PD-1 inhibitor, resulted in a confirmed tumor response in 73% of patients with no new safety signals. At a median follow-up of 11.5 months, the study results continued to meet outcome measures for safety and demonstrated encouraging clinical activity.
- SOLITON's Generation II Rapid Acoustic Pulse (RAP), a tattoo removal device licensed from MD Anderson Cancer Center, was submitted to the FDA through the 510(k) pathway as an accessory to the 1064 nm Q-Switched laser.
- ZEALAND PHARMA is buying the assets of Valeritas, which gives it the V-Go Wearable Insulin Delivery device.

Animal health news

- ELANCO ANIMAL HEALTH plans to sell the European and U.K. rights to **Bayer's Drontal and Profender** de-wormer product lines to Vetoquinol, a French pharmaceutical company, as part of its effort to obtain European Commission clearance for its acquisition of Bayer's global animal health business.

Very early research news

- CAR T – University of Utah researchers have created a CAR T that targets both cancer cells and cancer *stem* cells using an antibody against CD229. The results in mice and cells taken from multiple myeloma patients, published in ***Nature Communications***, have been promising.
- Cognition – Researchers at Albany Medical College reported in the ***Journal of Experimental Medicine*** that IL-33 and IL-5, which cause inflammatory responses, might be useful in treating age-related cognitive decline and neurodegenerative disease by activating a type of immune cell in the brain.
- Zika – A study by researchers at Walter Reed Army Institute of Research, published in ***Nature Medicine***, showed that the MZ4 antibody has a positive effect on both the dengue and Zika viruses and could lead to an effective vaccine against both diseases.

NEWS IN BRIEF

BAYER

- Is transferring some of its small molecule research and development to Nuvisan.
- **and ORION's Nubeqa (darolutamide).** A study in healthy volunteers, presented at ASCO-GU, found no decrease in cerebral blood flow (CBF) vs. placebo with this oral antiandrogen, but CBF was significantly decreased with Pfizer and Astellas' Xtandi (enzalutamide).
- **Xofigo (radium Ra-223 dichloride).** A study, presented at ASCO-GU found that combining Xofigo with Nanjing Xinbai's Provenge (sipuleucel-T, SipT) prolonged progression-free survival (PFS) vs. either agent alone in prostate cancer, suggesting a synergistic effect of the combination.

BRISTOL-MYERS SQUIBB

- **CELGENE/JUNO THERAPEUTICS' lisocabtagene maraleucel (JCAR-017, liso-cel).** The FDA accepted a biologics license application (BLA) for this CAR T therapy in diffuse large B-cell lymphoma and granted it priority review. The PDUFA date is August 17, 2020.
- **Opdivo (nivolumab) + Yervoy (ipilimumab).** Updated results from the CheckMate-214 trial, presented at ASCO-GU showed that at 42 months untreated advanced/metastatic renal cell carcinoma (RCC) patients continued to show superior results with this combination vs. Pfizer's Sutent (sunitinib) – better overall survival (52% vs. 39%), objective response rate (42% vs. 26%), duration of response, and complete response.
- **Opdivo (nivolumab) monotherapy.** Five-year follow-up of the CheckMate-025 trial, presented at ASCO-GU, showed superior overall survival and objective response rate vs. everolimus in previously treated advanced/metastatic RCC.

MERCK MSD

- **Keytruda (pembrolizumab).** This PD-1 inhib-itor, met the primary endpoint in the 847-patient Phase III KEYNOTE-355 trial in PD-L1+ metastatic triple-negative breast cancer, significantly improving progression-free survival when added first-line to nab-paclitaxel, paclitaxel, or gemcitabine/carboplatin vs. chemotherapy alone.
- **MK-6482.** The results of a Phase I/II trial of this oral HIF inhibitor in advanced clear cell RCC, presented at ASCO-GU, showed that it was well tolerated, with a favorable safety profile, and at 13.0 months the objective response rate was 24% and the median PFS was 11 months.

Most Expensive Pharmacy Drugs in the U.S. (according to GoodRx)

Company	Brand	Generic	Monthly Cost
Amryt Pharma	Myalept	metreleptin	$71,306
Horizon Therapeutics	Ravicti	glycerol phenylbutyrate	$55,341
Merck KGaA	Mavenclad	cladribine	$53,730
Horizon Therapeutics	Actimmune	interferon gamma-1b	$52,777
Dompé	Oxervate	cenegermin-bkbj	$48,498
Takeda	Takhzyro	lanadelumab-flyo	$45,464
Vyera Pharmaceuticals	Daraprim	pyrimethamine	$45,000
Amryt Pharma	Juxtapid	lomitapide	$44,714
Takeda	Cinryze	C1 esterase inhibitor	$44,141
Retrophin	Chenodal	chenodiol	$42,570

PFIZER

- **and ASTELLAS' Xtandi (enzalutamide).** The results of the Phase III PROSPER trial showed that adding Xtandi to androgen deprivation therapy (ADT) significantly improved overall survival (a key secondary endpoint in the trial) vs. ADT alone in non-metastatic castration-resistant prostate cancer (nmCRPC). The companies previously announced that the primary endpoint (metastasis-free survival) was met.
- **Talzenna (talazoparib).** Interim results of the Phase II TALAPRO-1 trial in metastatic castration-resistant prostate cancer, presented at ASCO-GU, showed "encouraging" activity and good tolerability.

CORONAVIRUS (2019-NCOV) UPDATE

- **The name.** The Wuhan coronavirus disease now has an official name – **COVID-19** – replacing the temporary name NCP (novel coronavirus pneumonia).
- **Extent of the outbreak.** There are no signs the outbreak is slowing.
  - In China, >68,000 cases have been confirmed, with >1,600 deaths. More than 1,700 healthcare workers in China have contracted the virus, and 6 healthcare workers died.
  - Outside mainland China, there have been ~700 cases and 4 deaths.
  - The director of the Centers for Disease Control and Prevention, Robert Redfield, Jr., told Congress, "Right now, there is no evidence that this outbreak is at all under control…It is definitely not controlled."
  - The first European death was an 80-year-old Chinese man in France who had been quarantined in a Paris hospital. His daughter also has the virus and is being treated at the same hospital.
  - There are now 15 coronavirus cases in the U.S., the two latest a person in Texas who had traveled to China and is in quarantine at a Texas military base and another California case.
- **Symptoms.** According to the World Health Organization (WHO), 80% of people infected with coronavirus only experience mild cold symptoms, with 15% getting pneumonia, and 5% needing intensive care.
- **Cruise ships.** Three cruise ships in Asia have been affected.
  - The Diamond Princess, which is quarantined in Yokohama harbor, Japan, now has >350 cases, including ≥40

Americans, though a few very old patients were allowed to leave the ship. The U.S. government sent a plane to bring *uninfected* Americans on the ship back for quarantine in the U.S. Several other countries are also retrieving their citizens.

- Holland America's Westerdam, which had not been able to find a port that would allow it to dock, was finally allowed into a port in Cambodia. No one on the ship tested positive for the virus, but an 83-year-old American woman who left the ship and flew to Malaysia has tested positive and is in quarantine in Malaysia.
- Passengers on the ship off Hong Kong were allowed into port and allowed to disembark.

■ Diagnostics. The issues with the accuracy of the CDC test for COVID-19, which has way too many false negatives, has created opportunities for other tests to be developed, and several are being developed. The CDC has defended its test, blaming false negatives on tests given too early for the virus to be detected, but that doesn't explain situations where a single patient gets a negative test, then a positive test, then a negative test all within a couple of days.

There also was a reagent issue with the tests the CDC sent to state/local labs, and the CDC said it is reformulating the reagents and will send them out quickly (but without specifying when that would be).

- **Cepheid** is developing an automated molecular diagnostic test using its Xpert Xpress Flu/RSV cartridge technology.
- **Co-Diagnostics**' Logix Smart 2019-nCoV, a research use only test for COVID-19, was introduced by Co-Diagnostics for use in hospitals, labs, and other institutions.
- **HiberGene Diagnostics**, an Irish company with a distribution partner in Shenzhen, China, is developing a rapid diagnostic test, using its templates for flu and respiratory diseases.
- **Kogene Biotech's 2019 Novel Coronavirus RT-PCR** kit was given emergency use approval by the Korea Food and Drug Administration and the Korea Centers for Disease Control and Prevention. The company will distribute the test to 50 local hospitals with testing laboratories and to the Korea CDC.
- **Qiagen** is working on a standard quantitative RT-PCR assay and an expansion to its QIAstat-Dx Respiratory Panel for detecting COVID-19 using ***nasal swabs***. The PCR assay is expected to operate on any real-time PCR platform.
- Researchers at the National University of Singapore's Institute for Health Innovation & Technology are developing a rapid (30-minute) detection test kit based on enzyme-assisted nanocomplexes for visual identification of nucleic acids. It would need approval from Singapore's Health Sciences Authority before it can be marketed.

■ Supply chain. FDA Commissioner Stephen Hahn, MD, said there have been no drug or medical device shortages reported in the U.S. so far due to the coronavirus outbreak.

■ Clinical trials. Nearly 500 clinical trials have a site in Wuhan, China, and ~20% of global trials are now conducted in China or have sites there. There is no word yet on how COVID-19 and the quarantines are affecting these trials.

■ Treatment

- **BrightGene Bio-Medical Technology**, a Chinese company, is mass producing **Gilead Sciences' remdesivir**, an antiviral originally developed to treat Ebola and Marburg virus. BrightGene said it will have to license the drug from Gilead to sell it commercially.
- **Vir Biotechnology** said it has identified two antibodies and hopes to test them against live virus.
- **WuXi Biologics**, a Chinese pharma, has 240 scientists working on finding an antibody.

■ Vaccines

- **Johnson & Johnson** is collaborating with the Biomedical Advanced Research and Development Authority (BARDA) to expedite development of a vaccine.
- **Moderna** and the National Institutes of Health (NIH) are looking for a manufacturer to make the potential vaccine they are working on when and if it is ready for commercialization.

REGULATORY NEWS

Regulatory tidbits

■ FDA budget. President Trump's proposed FY2021 budget would increase overall funding for the FDA to $6.2 billion – $2.9 billion in user fees and $3.3 billion in discretionary budget authority. The administration is also proposing an $18 million funding increase for continued development of a portal and knowledge management system for medical devices.

■ Insulin. States are starting to set price limits on insulin. A Minnesota Senate committee passed a bill that would require pharmaceutical firms to provide free insulin both on an emergency basis and in an ongoing basis to income-eligible patients in the state. Under the legislation, eligible patients would be required to pay $75 to the pharmacy for a 30-day supply. And a bill was introduced in Connecticut

that would limit the monthly price of insulin to $50 and insulin-related supplies to $100. Connecticut pharmacists also would be permitted to dispense insulin without a prescription in emergency situations.

- Peripheral artery disease (PAD). The FDA issued final guidance on 510(k) clearance for peripheral vascular atherectomy devices.
- Sterilization. The attorneys general of 11 states asked the Environmental Protection Agency (EPA) to set tighter regulations for companies/plants that emit ethylene oxide – and to work with the FDA to encourage research on alternatives to EtO sterilization.
- Supply chain. WHO issued draft guidance on the design of pharmaceutical and vaccine tracking and tracing systems. Comments will be accepted through February 28, 2020.
- Tobacco. The Trump administration wants to remove the oversight of tobacco and e-cigarette products from the FDA and give it to a new agency under the Department of Health and Human Services whose only mission would be tobacco.

FDA approvals/clearances

- AGILE THERAPEUTICS' Twirla (AG200-15, 120 µg levonorgestrel + 30 µg ethinyl estradiol), a weekly contraceptive patch, was approved for use in women with a BMI <30.
- BAXTER and COSMED's Q-NRG+, a portable metabolism monitor, was granted 510(k) clearance for use in measuring a patient's resting energy expenditure and creating a prescription and nutrition therapy plan.
- BRAINTREE LABORATORIES' Pizensy (lactitol), an osmotic laxative, was approved to treat chronic idiopathic constipation in adults.
- CAPTION HEALTH's Caption Guidance, artificial intelligence-powered software designed to assist and guide medical professionals without specialized training in doing a cardiac ultrasound exam, was granted de novo clearance.
- HYPERFINE's small, portable, bedside MRI system was granted 510(k) clearance.
- MEDICREA's UNiD IB3D, a patient-matched interbody cage, was granted 510(k) clearance for use in spine surgery.
- ORTHOFIX MEDICAL's STIM onTrack, a mobile app version 2.1, was approved for use with the company's CervicalStim, PhysioStim, and SpinalStim bone growth stimulators. The app helps patients adhere to their bone growth treatment plan, with healthcare providers able to remotely monitor the patient's status.
- Prescription-to-OTC (over the counter) switch approvals:
  - **GlaxoSmithKline's Voltaren Arthritis Pain** (diclofenac sodium topical gel 1%) for arthritis pain.
  - **Alcon's Pataday** Twice Daily Relief (olopatadine HCl 0.1% eye drops) for itchy and red eyes due to pollen, ragweed, grass, animal hair, or dander.
  - **Alcon's Pataday** Once Daily Relief (olopatadine HCl 0.2% eye drops) for itchy and red eyes due to pollen, ragweed, grass, animal hair, or dander.
- VARIAN MEDICAL SYSTEMS' Ethos, an artificial intelligence-based device that provides healthcare providers with a view of a patient's anatomy using multimodality images.

FDA recalls/warnings

- EISAI's Belviq and Belviq XR (lorcaserin) – The FDA asked the company to voluntarily take this diet drug off the market because a safety trial found an increased rate of cancer, and Eisai agreed. The FDA is not recommending patients who took lorcaserin undergo any special screening, but patients are advised to immediately cease taking any pills they already have.
- MEDTRONIC's MiniMed 600-series insulin pumps – A Class I recall was initiated due to a missing/broken retainer ring that can cause the delivery of an incorrect insulin dose. There has been at least one death and >2,000 injuries.

European Regulatory News

- BIONEER's AccuPower, a hepatitis B virus quantitative PCR kit, was granted a CE-IVD Mark.
- Cyproterone (e.g., Bayer's Androcur or Cyprostat) – The European Medicines Agency's Pharmacovigilance Risk Assessment Committee (PRAC) recommended medicines containing this anti-androgen (≥10 mg) should only be used for hirsutism (excessive hair growth), androgenic alopecia, acne, and seborrhea – and for the reduction of sex drive in sexual deviations in men only when other options are not suitable – after a review found an increased risk of meningioma.
- ENDOMAG's Magseed, a soft tissue magnetic marker, was granted expanded approval for use before surgery and chemotherapy to help detect suspicious lymph nodes and identify tumor areas.
- GREENBONE ORTHO's GreenBone Bone Substitute was granted a CE Mark.

U.K.'s National Institute for Health and Care Excellence (NICE) News

- AXONICS' sacral neuromodulation (SNM) – NICE recommended use of this rechargeable wireless neuromodulation device to treat patients with overactive bladder.
- MERCK MSD's Keytruda (pembrolizumab) – NICE rejected use of this PD-1 inhibitor in combination with Pfizer's Inlyta (axitinib) in untreated advanced renal cell carcinoma (RCC), saying the long-term benefits are unknown and it is not cost-effective.

Regulatory news from other countries

- *China.* ROCHE's Tecentriq (atezolizumab) – The National Medical Products Administration approved use of this PD-L1 inhibitor in combination with chemotherapy (carboplatin + etoposide) for first-line treatment of extensive-stage small cell lung cancer.

2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
February 18	**Merck MSD's Keytruda** (pembrolizumab) – 6 sBLAs for a 30-minute Q6W infusion to treat melanoma, Hodgkin's lymphoma, primary mediastinal large B-cell lymphoma, gastric cancer, hepatocellular carcinoma, and Merkel cell carcinoma	PDUFA date
February 19	**Adverse event reporting** using ICH standards	FDA public meeting
February 21	**Lundbeck/Alder BioPharmaceuticals' eptinezumab** (ALD-403), a CGRP inhibitor for migraine	PDUFA date
February 21	**Esperion Therapeutics' bempedoic acid** monotherapy to treat hypercholesterolemia	PDUFA date
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
March 26	**Intercept Pharmaceuticals' obeticholic acid** to treat liver fibrosis due to non-alcoholic steatohepatitis (NASH)	PDUFA date *postponed until June 26*
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 tba	**AstraZeneca's Imfinzi** (durvalumab) + tremelimumab for small cell lung cancer	PDUFA date (*estimated late March*)

more - 2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
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
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 22 *tentative*	**Intercept Pharmaceuticals' obeticholic acid** to treat liver fibrosis due to non-alcoholic steatohepatitis (NASH)	FDA's Gastrointestinal 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 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 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
August 5	**DBV Technologies' Viaskin Peanut** for treating children with peanut allergy	FDA target action 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 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 20	**FibroGen and AstraZeneca's roxadustat** for anemia of chronic kidney disease	PDUFA date