TRENDS-in-MEDICINE

September 6, 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…

Trends-in-Medicine
Stephen Snyder, *Publisher*
2731 N.E. Pinecrest Lakes Blvd.
Jensen Beach, FL 34957
772-285-0801
Fax 772-334-0856
www.trends-in-medicine.com
TrendsInMedicine@aol.com

NOTE: The weekly Covid-19 section is on Page 4, but there was also a special **Covid-19 Update: The Industry Perspective** on September 5. If you haven't seen that yet, let us know.

Be careful, be safe, and be well.

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

✓ GLAXOSMITHKLINE and INNOVIVA's Trelegy Ellipta (fluticasone furoate + umeclidinium + vilanterol) – An FDA advisory committee voted 14-1 against an expanded label for reduction of all-cause mortality in COPD.

✓ **Positive trial results:**

- AMYLYX PHARMACEUTICALS' AMX-0035 (sodium phenylbutyrate + taurourso-deoxycholic acid) – in ALS.
- AVENU MEDICAL's Ellipsys Vascular Access System – in endovascular-AVFs in dialysis patients.
- BLUEBIRD BIO's elivaldogene autotemcel (eli-cel, Lenti-D) – in a Phase II/III trial in cerebral adrenoleukodystrophy (CALD).
- IONIS PHARMACEUTICALS' PKKRx and PKK-LRx – in severe bradykinin-mediated angioedema.
- NOVARTIS
  - LNP-023 – in paroxysmal nocturnal hemoglobinuria (PNH) when added to eculizumab.
  - and INCYTE's Tabrecta (capmatinib) – in metastatic MET exon 14+ NSCLC.

✓ **Mixed trial results:** AKEBIA THERAPEUTICS' vadadustat failed on safety in two Phase III trials in CKD patients not on dialysis but met the efficacy endpoint.

✓ **Negative trial results:**

- ACASTI PHARMA's CaPre (EPA + DHA) – in hypertriglyceridemia.
- JOHNSON & JOHNSON/JANSSEN's pimodivir – in hospitalized patients with influenza A. Development was stopped.
- SANOFI and REGENERON PHARMACEUTICALS' Kevzara (sarilumab) – in Covid-19.

SHORT TAKES

■ ABBVIE licensed **I-Mab's lemzoparlimab**, an anti-CD47 for treating cancer.

- ACASTI PHARMA's CaPre (EPA + DHA), a krill-oil derived omega-3 drug, missed the primary endpoint in a Phase III trial, TRILOGY-2, failing to significantly reduce triglycerides vs. placebo (30.4% vs. 17.9%) in patients with hypertriglyceridemia. That makes two Phase III failures.

- AKEBIA THERAPEUTICS' vadadustat, an oral HIF-PHI, missed the primary *safety* endpoints in the two Phase III PRO2TECT trials in chronic kidney disease (CKD) patients not on dialysis, though it met the primary *efficacy* endpoint, showing non-inferiority to Amgen's Aranesp (darbepoetin alfa) on hemoglobin change from Weeks 24-36. The company said it still plans to submit vadadustat to the FDA.

- ANGIOSOMA/SOMACEUTICALS bought the exclusive global rights to a patented formula for an investigational treatment for multiple sclerosis lesions.

- Anticholinergic drugs – A 688-patient study by University of California San Diego School of Medicine researchers, published in the journal ***Neurology***, found that anticholinergic drugs may accelerate cognitive decline, especially in older people with biological or genetic risk factors.

- AVENU MEDICAL's Ellipsys Vascular Access System – A 123-patient study at Richmond Vascular Center, published in the ***Journal of Vascular and Interventional Radiology***, found that this minimally-invasive device (an endovascular-AVF or endo-AVF) required fewer secondary procedures in dialysis patients than an arteriovenous fistula (AVF). In addition, the average from fistula creation to dialysis was 66 days with Ellipsys vs. an historic rate of 135 days with surgical fistulas.

- BLUEBIRD BIO's elivaldogene autotemcel (eli-cel, Lenti-D) – Long-term results for the 32-patient Phase II/III STARBEAM trial of this gene therapy in cerebral adrenoleukodystrophy (CALD) showed that 87% of patients with 24 month follow-up achieved the primary endpoint – survival without any of six major functional disabilities – and 97% had stable neurologic function score (NFS), including 75% with an NFS score of 0.

- BOSTON SCIENTIFIC's Eluvia, a paclitaxel-eluting vascular stent, was granted a new technology add-on payment by the Centers for Medicare and Medicaid Services (CMS), starting October 2020. This means in-hospital use (which is ~40% of use) will see an increased reimbursement of 65%. Notably, the add-on payment only applies to Eluvia, not Cook's Zilver, another FDA-cleared paclitaxel-eluting peripheral stent that is priced at about half the price of Eluvia.

- CHANGE HEALTHCARE bought **Prometheus Analytics** from Altarum.

- CRYOLIFE bought **Ascyrus Medical**, which has an aortic repair device.

- DAIICHI SANKYO's miladametan (DS-3032) – The worldwide rights to this MDM2 inhibitor for solid tumors and hematologic malignancies were licensed to **Rain Therapeutics**, and it will be renamed RAIN-32.

- Gene editing – A National Academy of Sciences panel issued new guidelines which concluded that gene editing of embryos is not yet safe and could only be ethically applied in a narrow set of circumstances (e.g., muscular dystrophy, beta-thalassemia, cystic fibrosis, and Tay-Sachs disease, etc.). The panel also called for the creation of international bodies to counsel scientists and government and to track reports of germline editing occurring.

- GLAXOSMITHKLINE and INNOVIVA's Trelegy Ellipta (fluticasone furoate + umeclidinium + vilanterol) – The FDA's Pulmonary-Allergy Drugs Advisory Committee voted 14-1 *against* a proposed labeling claim for reduction in all-cause mortality in patients with chronic obstructive pulmonary disease (COPD), generally agreeing that the IMPACT trial did not provide substantial evidence to support that claim.

- JOHNSON & JOHNSON/JANSSEN's pimodivir – After an interim analysis found this non-nucleoside was unlikely to be successful in two Phase III trials in hospitalized patients with influenza A, J&J ended the trial and scrapped development of the drug, which it bought in 2014 from Vertex Pharmaceuticals.

- NESTLÉ is buying **Aimmune Therapeutics**, which will give it Palforzia (AR-101), an FDA-approved oral immunotherapy for peanut allergy.

- Non-alcoholic steatohepatitis (NASH) – A study, published in the ***Journal of Hepatology***, raises questions about the reliability of liver biopsies for advanced fibrosis, though they remain the gold standard for trial efficacy in NASH. The researchers examined the biopsies in the failed trial of Cirius Therapeutics' MSDC-0602K, an oral insulin sensitizer, in the Phase IIb EMMINENCE trial in NASH, which had an "unexpectedly" high placebo response rate. They found the reason for that was a "lack of reliability" of liver biopsy interpretation by pathologists, leading them to conclude that liver biopsy interpretation in NASH is unreliable.

- ORCA BIO is partnering with **Lyell Immunopharma** on identification of next-generation T cell therapies, combining precision purification T cell technologies from Orca with Lyell's expertise in T cell biology.

- PFIZER signed a new master service agreement with **Parexel**.

- PQ BYPASS' Detour, a system for percutaneous femoro-popliteal bypass in treating complex and extremely long blockages in the superficial femoral artery (SFA), was granted breakthrough device designation by the FDA.

- RADIOISOTOPE LIFE SCIENCES (RLS) bought **GE Healthcare**'s 31 radiopharmacies in the U.S.

- REGENT PACIFIC bought **Deep Longevity**, an aging and longevity research firm.

- SANOFI and REGENERON PHARMACEUTICALS' Kevzara (sarilumab), an anti-IL-6, missed the primary endpoint and key secondary endpoints in a 420-patient Phase III non-U.S. trial in Covid-19, with both doses (200 mg and 400 mg) failing to significantly improve clinical symptoms. Kevzara also failed to significantly reduce duration of hospital stay, reduce mortality, or improve the time to clinical improvement, though there were numerical trends in some of these measures. Sanofi said no further studies are planned for this in Covid-19.

- SANTHERA PHARMACEUTICALS got the license and worldwide rights to vamorolone, a treatment for Duchenne muscular dystrophy, from **Idorsia**, which got it from ReveraGen BioPharma.

- SAVARA PHARMACEUTICALS' Molgradex (molgramostim nebulizer solution) – The company discontinued the open-label Phase IIa ENCORE trial of this inhaled GM-CSF for treating non-tuberculous mycobaterial (NTM) lung infections in cystic fibrosis patients, in part because of Covid-19 factors. Among the 12 patients with ≥20 weeks of therapy, 5 had a good response vs. none of the placebo patients.

- SYNCHRON's Stentrode, a brain-computer implant that works from inside a blood vessel, was granted breakthrough device status by the FDA.

- TANDEM DIABETES CARE – The results of the Protocol-5 trial of the t:slim X2 insulin pump with Control-IQ closed-loop technology (an artificial pancreas) were published in the ***New England Journal of Medicine***, showing that this closed-loop system had positive outcomes in children age 6-13.

- UNUM THERAPEUTICS' BOXR (bolt-on chimeric receptor) CAR-T platform and its BOXR1030 program were sold to **Sotio**.

Very early research news

- Amyotrophic lateral sclerosis (ALS) – A mouse study by U.K. and Japanese researchers, published in ***EBioMedicine***, found that ebselen and several other ebselen-based compounds developed at the University of Liverpool can change the toxic characteristics of SOD1, which causes some cases of ALS. The study also showed delay of disease onset with ebselen in mice.

- Oncology – Preclincial research by investigators at City of Hope, published in ***Science Translational Medicine***, found that combining anti-CD19 CAR T therapy with an oncolytic virus (OV19t) killed solid tumors in mice.

NEWS IN BRIEF

AMYLYX PHARMACEUTICALS' AMX-0035 (sodium phenylbutyrate + tauroursodeoxycholic acid)

In the pivotal 137-patient, 24-week CENTAUR trial in ALS, published in the ***New England Journal of Medicine***, this neuroprotective therapy:

- Met the primary endpoint, significantly improving the ALSFRS-R score. On average, AMX-0035 patients scored 2.32 points higher than placebo patients, which doesn't sound like much, but a 1-2 point drop can mean the difference between an individual able to self-feed vs. the need for a feeding tube or walking with assistance vs. not walking at all.
- Met a number of secondary endpoints, though it wasn't powered for those endpoints, including slower reduction in lung function, less frequent hospitalizations, and less overall decline in muscle strength.
- Was well tolerated.

GILEAD SCIENCES

- Exclusively licensed **Jounce Therapeutics**' tumor-infiltrating regulatory T cells program, including JTX-1811, a CCR8 inhibitor, for which Gilead will run all clinical trials.

- KITE's Yescarta (axicabtagene ciloleucel). A supplemental biologics license application (sBLA) for this CAR T therapy was submitted to the FDA for expanded approval as a ≥3-line treatment for relapsed/refractory follicular lymphoma and marginal zone lymphoma.

IONIS PHARMACEUTICALS

- Is buying back **Akcea Therapeutics**, which gives it full control of Tegsedi (inotersen) for peripheral nerve damage caused by hereditary transthyretin amyloidosis and Waylivra (volanesorsen) for familial chylomicronemia syndrome.

- PKKRx and PKK-LRx. The results of a compassionate-use trial in patients with severe bradykinin-mediated angioedema, published in the ***New England Journal of Medicine***,

showed that these antisense drugs significantly reduced the production of prekallikrein (PKK), which play a key role in activating acute attacks of hereditary angioedema (HAE). And the drugs reduced the number of breakthrough attacks per month, including one patient with complete resolution.

NOVARTIS

- Licensed an FGF21 agonist for NASH to **Boston Pharmaceuticals**, which named it BOS-580 and will handle the clinical trials.
- LNP-023. The results of an open-label Phase II trial, presented at the European Society for Blood and Marrow Transplantation (EBMT) virtual meeting, showed that this oral Complement Factor B inhibitor, improved hematological response and biomarkers of disease activity in paroxysmal nocturnal hemoglobinuria (PNH) patients when added to eculizumab (Alexion Pharmaceuticals' Soliris), with 80% of patients achieving hemoglobin >12g/dL without transfusion.
- and INCYTE's Tabrecta (capmatinib). In the Phase II GEOMETRY mono-1 trial in metastatic non-small cell lung cancer (NSCLC) with a MET exon 14 (METex14) skipping mutation, published in the ***New England Journal of Medicine***, this MET inhibitor showed overall response rate of 68% in treatment-naïve patients and 41% in previously treated patients. And METex14 was shown to be a good biomarker for identifying responders.

Rheumatology

A national patient survey by the American College of Rheumatology (ACR) found that from 2019 to 2020:

- There was a 52% decline in patients currently seeing a rheumatologist.
- Out-of-pocket treatment costs more than doubled, from $475 to $1,000.
- ~66% of patients had a telehealth appointment with their rheumatologist within the past year, most often because of Covid-19.
- Nearly 50% of the patients were subjected to step therapy or prior authorization requirements.

COVID-19 WEEKLY HIGHLIGHTS

- **Antibodies.** A study in Iceland of 1,797 patients who recovered from Covid-19, published in the ***New England Journal of Medicine***, found that the antiviral antibodies for SARS-CoV-2 in those patients did not decrease for at least 4 months from the time of diagnosis.
- **Convalescent plasma.** The National Institutes of Health's Covid-19 Treatment Guidelines Panel said it doesn't agree with the FDA's decision to grant emergency use authorization to convalescent plasma, saying there are not sufficient data to recommend either for or against its use.
- **U.S. projections.** The University of Washington's Institute for Health Metrics and Evaluation (IHME) is warning that U.S. Covid-19 deaths could rise from the current toll of ~188,000 to >400,000, with the worst case U.S. scenario >620,000, and a best case U.S. scenario of 257,286 deaths.
- **Vaccines**
  - **Distribution.** The Center for Disease Control and Prevention (CDC) advised states to be ready for distribution of a Covid-19 vaccine by November 2020. While there may not be a vaccine then, if there is one, states need to be prepared, otherwise a last-minute scramble likely would create havoc.
  - **Moderna Therapeutics' mRNA-1273** – CEO Stéphane Bancel said enrollment was "slowed" in its 30,000-patient U.S trial of its vaccine in order to ensure sufficient minorities are enrolled, "I would rather we have higher diverse participants and take one extra week. [Diversity] matters more to us than speed." As of August 28, >17,000 people were enrolled – 20% Hispanic and 7% Black, and the company goal is 13.4% Black.
  - **Novavax's NVX-CoV2373** – The Phase I results from a Phase I/II trial evaluating two doses of this recombinant vaccine, adjuvanted with Matrix-M, were published in the ***New England Journal of Medicine***, showing good tolerability and neutralizing titers in all participants.
  - **Russian vaccine.** The results of Phase I and II trials of 76 adults (age 18-60) for this adenovirus-based vaccine – which were the basis for the Russian government's approval – were published in ***The Lancet***, showing that all participants produced an antibody response, with no serious adverse events.
  - **U.S. attitudes.** A survey of 2,067 adults by ***Stat*** and Harris Poll found that
    - ✓ 78% of respondents – 72% of Republicans and 82% of Democrats – are worried the Covid-19 vaccine approval process "is being driven more by politics than science."

- ✓ 83% – 80% of Republicans and 85% of Democrats – said they would worry about a Covid-19 vaccine that is approved quickly.
- ✓ 68% still have faith that the FDA will only approve a safe vaccine.
- ✓ 72% doubt a vaccine will be available before 2021.

- **WHO.** The Trump administration said it is not going to join the international Covax coalition for Covid-19 vaccine access, which is being led by the World Health Organization (WHO), Gavi, and the Coalition for Epidemic Preparedness Innovations. Germany and the U.K. did join Covax. The U.S. plans to work instead with international partners.

REGULATORY NEWS

Regulatory tidbits

- AbbVie. The House Oversight and Reform Committee is set to subpoena AbbVie over the pricing of Humira (adalimumab), a TNF inhibitor for rheumatoid arthritis, and Imbruvica (ibrutinib), a BTK inhibitor for chronic lymphocytic leukemia (CLL), alleging that the company has failed to comply with Committee requests.
- CMS. The Centers for Medicare and Medicaid Services proposed a rule – Medicare Coverage of Innovative Technology – that would expedite Medicare coverage approval for medical devices that were granted breakthrough device status by the FDA. The coverage would be for four years, starting the day they are cleared for use by the FDA. Devices approved over the last year would also be eligible.
- FDA
  - **Combination products.** An independent report on the FDA's review of combination products found the Agency generally did well but still has room for improvement in both communication and technology.
  - **Generic drugs.** The FDA issued a new manual of policies and procedures for transferring drug application ownership for generic drugs (e.g., those due to mergers and acquisitions).
  - **Nitrosamine.** The FDA issued new guidance, effective immediately, on detection and prevention of "objectionable levels" of nitrosamine (e.g., NDMA) contamination of active pharmaceutical ingredients and finished drug products.
  - **PRO.** The FDA issued draft guidance on how medical device manufacturers can collect and use patient-reported outcomes during surveillance and evaluation of their devices. The Agency is accepting comments through October 30, 2020.
- Generic drugs. Legislation was passed in California that, if signed by the governor, will require the state's Health and Human Services Agency to develop partnerships to boost competition, reduce prices, and mitigate shortages of generic prescription drugs.

FDA approvals

- B. BRAUN's SpaceStation MRI, which enables MRI-safe infusion pumps, was granted 510(k) clearance.
- BAXTER's Theranova, a kidney dialyzer for use in hemodialysis, was approved through the de novo pathway.
- BRISTOL-MYERS SQUIBB/CELGENE's Onureg (oral azacitidine) was approved to treat adults with acute myeloid leukemia (AML) in first remission but unable to continue intensive therapy.
- CTL AMEDICA's Mondrian, a lumbar intervertebral fusion cage for use in spinal surgery, was cleared for use.
- FUJIFILM/SONOSITE's point-of-care ultrasound system (POCUS) was granted 510(k) clearance for lung and cardiac imaging in Covid-19 patients.
- GETINGE's Flow-e and Flow-c, customizable and portable anesthesia workstations, were granted 510(k) clearance.
- LILLY's Trulicity (dulaglutide) – Two additional once-weekly doses of this GLP-1 agonist were approved to treat Type 2 diabetes – 3.0 mg and 4.5 mg.
- MASIMO's O3 Regional Oximetry was granted expanded 510(k) clearance for use in monitoring somatic tissue oxygenation saturation.
- MEDTRONIC's MiniMed 770G System, a closed loop diabetes management device that automatically monitors glucose and provides basal insulin as needed, along with smartphone connectivity, was approved for use by Type 1 diabetics age ≥2.
- ROCHE
  - **cobas HIV-1/HIV-2 Qualitative test** was approved for use on the cobas 6800/8800 systems in the U.S.
  - **cobas SARS-CoV-2 & Influenza A/B Test**, the first commercial test for fully automated high throughput systems, to detect and differentiate SARS-CoV-2, influenza A, and/or influenza B with a single sample, was granted emergency use authorization (EUA) on the cobas 6800/8800 systems.

- TISSUE REGENERATION TECHNOLOGIES' Orthogold 100, an extracorporeal shock treatment device for use in healing superficial burns, was granted 510(k) clearance.
- TRANSMED7's Concorde ST and Concorde US, soft tissue biopsy devices, were granted 510(k) clearance.

FDA recalls/warnings

- ACELLA PHARMACEUTICALS received a warning letter about violations of current good manufacturing practices (cGMP) by its contract manufacturing organizations (CMOs) that makes its thyroid medication – the one recalled in May 2020.
- BECTON DICKINSON's Alaris – The recall of these infusion pumps, due to hardware issues that may cause them not to operate as expected, was upgraded to Class I after three serious issues were reported.
- Covid-19
  - The FDA and the Federal Trade Commission jointly sent a warning letter to **1 Party At A Time** for selling an unapproved product with fraudulent Covid-19 claims.
  - **Lattice Biologics** got a warning letter for marketing an unapproved amniotic fluid product, AmnioBoost, to treat Covid-19 related conditions.
- MYLAN's amiodarone and tranexamic acid – Four lots were recalled due to a labeling mix up.

European Regulatory News

- U.K. – The Medicines and Healthcare products Regulatory Agency (MHRA) issued new guidances on drug, device, and clinical trial regulations that will go into effect at the end of the Brexit transition period in January 2021.
- GEDEON RICHTER's Esmya (ulipristal acetate) and generics – The Pharmacovigilance Risk Assessment Committee (PRAC) of the European Medicines Agency (EMA) recommended revoking marketing authorization for use of these progesterone receptor modulators to treat the symptoms of uterine fibroids, saying their review confirmed a risk of liver injury, including the need for liver transplantation. However, PRAC noted that this does not apply to single-dose ulipristal acetate emergency contraceptives (e.g., HRA Pharma's ellaOne).
- TAW PHARMA's dexamethasone – The EMA has begun consideration of a marketing application for this steroid to treat hospitalized adults with Covid-19, and the Committee for Medicinal Products for Human Use (CHMP) will conduct a review.

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

- JOHNSON & JOHNSON's Spravato (esketamine) – For the second time, NICE rejected use of this nasal spray S-enantiomer of ketamine in combination with an SSRI or SNRI for treatment-resistant major depressive disorder (MDD) over continued concern about efficacy.
- PFIZER and MERCK KGAA's Bavencio (avelumab) – NICE issued final guidelines recommending the use of this PD-L1 inhibitor in combination with Inlyta (axitinib) to treat advanced renal cell carcinoma.

Regulatory news from other countries

- *Canada.* KNIGHT THERAPEUTICS and PUMA BIOTECHNOLOGY's Nerlynx (neratinib) – A supplemental new drug application was submitted to Health Canada for expanded approval as a treatment for HER2+ metastatic breast cancer.
- *China.* SIRTEX MEDICAL and CHINA GRAND PHARMACEUTICAL's SIR-Spheres Y-90 microspheres – Permission for filing of a new drug application was granted by the National Medical Products Administration for this treatment for liver cancer.

2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
August 28	**Lipocine's Tlando** (oral testosterone) for hypogonadism (resubmission)	PDUFA date **Postponed indefinitely**
September 8	Classification of **facet screw systems** from unclassified to Class II *and* classification of non-invasive bone growth stimulators from Class III to Class II	FDA's Orthopaedic and Rehabilitation Devices Advisory Committee virtual meeting
September 9	**Classification of 3 devices** from unclassified to Class II: semi-constrained toe joint prostheses, intracompartmental pressure monitors, and intra-abdominal pressure monitoring devices	FDA's Orthopaedic and Rehabilitation Devices Advisory Committee virtual meeting
September 9	Immediately-in-effect guidance on **coronavirus diagnostic tests**	FDA virtual Town Hall
September 10	**Multiple function device products**: policy and considerations	FDA webinar
September 10	Advancing the science of real-world data to address the **Covid-19 pandemic**	FDA webcast
Sept. 10-11	Review of postmarketing studies of **Purdue Pharma's reformulated OxyContin ER** (oxycodone hydrochloride extended-release)	Virtual meeting of the FDA's Anesthetic and Analgesic Drug Products Advisory Committee jointly with the Drug Safety and Risk Management Advisory Committee
Sept. 14-16	Quality management and robust **manufacturing**	FDA and Parenteral Drug Association joint virtual conference
September 15	**Bausch Health/Eton Pharmaceuticals' EM-100**, an eyedrop for treating ocular itchiness (resubmission)	PDUFA date *Postponed from August 10*
September 15	**Safety review** of 6 drugs, devices, and biologics – and acute dystonia associated with ADHD	FDA's Pediatric Advisory Committee virtual meeting
Sept. 17-18	Use of real-world evidence to assess the effectiveness of **preventive vaccines**	FDA virtual public workshop
September 24	Safety and performance-based **pathway** criteria for devices (final guidance)	FDA webinar
September 24	The Mayo Clinic experience with **point-of-care manufacturing**	FDA webcast
September 25	Challenges in **inhaled antifungal** drug development	FDA virtual public workshop
September 27	**Aquestive Therapeutics' Libervant** (diazepam buccal film) for seizure clusters in epileptics	PDUFA date
Sept. 28-30	Global summit on regulatory science: **Emerging Technologies**	FDA virtual summit **https://aralliance.org/gsrs/**
September 29	**Patient preference information use** in medical device regulatory decisions	FDA public meeting webcast
Sept. 29-30	Annual Scientific Computing Days: **Real-world evidence**	FDA and National Center for Toxicological Research (NCTR) *May be virtual*
September 30	**Patient-reported outcomes** (PROs) and medical device investigations	FDA virtual public meeting
September 30	**Mesoblast's Kyoncil** (remestemcel-L) for steroid-refractory acute graft-versus-host disease in pediatric patients	PDUFA date
October 2	Discussion of strains to be included in **flu virus vaccine** for 2021 southern hemisphere flu season	FDA's Vaccines and Related Biological Products Advisory Committee virtual meeting
October 6	Patient-focused drug development for **stimulant use disorder**	FDA virtual public meeting
October 7	**TransMedics' TransMedics Organ Care System** (OCS) – normothermic heart transplant transport	FDA's Circulatory System Devices Advisory Committee virtual meeting
October 8	Assessing changes in the PK of drugs in **liver disease**	FDA virtual public workshop in collaboration with the University of Maryland Center of Excellence in Regulatory Science & Innovation
October 9	**Alkermes' ALKS-3831** (olanzapine/samidorphan) for schizophrenia and bipolar disorder	FDA's Psychopharmacologic Drugs Advisory Committee and the Drug Safety and Risk Management Advisory Committee joint virtual meeting
October 13	Patient-focused drug development for **systemic sclerosis**	FDA virtual public meeting
October 13-14	Update and **strategic plan** for 2021-2025 for National Antimicrobial Resistance Monitoring System (NARMS)	FDA's Center for Veterinary Medicine webcast
October 14	Enhancing development of **radiopharmaceuticals and radiological devices**	FDA-NRC virtual workshop
October 18	**Roche's Herceptin** (trastuzumab) **+ Perjeta** (pertuzumab) – a fixed dose combination to treat HER2+ breast cancer	PDUFA date
October 19	Complex generic **drug-device combination products**	FDA/DIA virtual conference
October 22	**Covid-19 vaccines** general discussion	FDA's Vaccines and Related Biological Products Advisory Committee virtual meeting
October 22	**Artificial intelligence** and machine learning in medical devices	FDA's Patient Engagement Advisory Committee virtual meeting
October 22-23	Pediatric dose selection	FDA virtual public workshop

more - 2020 FDA Advisory Committees and Other Regulatory Dates of Interest (*items in RED are new since last week*)		
Date	**Topic**	**Committee/Event**
October 24	**Spectrum Pharmaceuticals' Rolontis** (eflapegrastim) for treating chemotherapy-induced neutropenia	PDUFA date
October 25	**Regeneron Pharmaceuticals' REGN-EB3** for Ebola	PDUFA date
October 27	Antimicrobial drugs to treat **gonorrhea**	FDA virtual public workshop *Postponed from August 21*
October 30	Integrated assessment of **marketing applications**	FDA virtual public workshop
November 13	**Orthopedic device-related infections**	FDA virtual public workshop
November 15	**Alkermes' ALKS-3831** (olanzapine + samidorphan) for bipolar I disorder and schizophrenia	PDUFA date
November 16	**Bristol-Myers Squibb/Celgene/Juno Therapeutics' lisocabtagene maraleucel** (JCAR-017, liso-cel) for DLBCL	PDUFA date *Postponed from August 17*
November 19	**CBD and other cannabinoids** – sex and gender differences in use and response	FDA's Office of Women's Health virtual Scientific Conference
November 20	**Eiger BioPharmaceuticals' Zokinvy** (lonafarnib) for progeria and progeroid laminopathies	PDUFA date
November 25	**Revance Therapeutics' daxibotulinumtoxinA** for moderate-to-severe glabellar lines	PDUFA date
November 27	**Rhythm Pharmaceuticals' setmelanotide** for pro-opiomelanocortin deficiency obesity and leptin receptor deficiency obesity	PDUFA date
December tba	**Pfizer and Lilly's tanezumab** to treat moderate-to-severe osteoarthritis pain	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
December 20	**Myovant Sciences' Relumina** (relugolix) for advanced prostate cancer	PDUFA date
December 26	**Sumitomo Dainippon Pharma/Urovant Sciences' vibegron** for overactive bladder	PDUFA date
December 30	**Almirall and Athenex's tirbanibulin** (KX2-391, KX-01) for actinic keratosis	PDUFA date
2021		
January 20	**Merck MSD and Bayer's vericiguat** in HFrEF	PDUFA date
January 26	Non-clinical immunogenicity assessment of **generic peptide products**	FDA virtual public workshop
January 27	**Protalix BioTherapeutics and Chiesi's pegunigalsidase alfa** (PRX-102) for Fabry disease	PDUFA date
February 11	**Regeneron Pharmaceuticals' evinacumab** in severe homozygous familial hypercholesterolemia (HoFH)	PDUFA date
February 15	**G1 Therapeutics' trilaciclib** for small cell lung cancer	PDUFA date
March 7	**Biogen and Eisai's aducanumab** for Alzheimer's disease	PDUFA date