TRENDS-in-MEDICINE

**September 30, 2012**

*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 **European Society for Medical Oncology (ESMO)** meeting in Vienna, Austria.

## SHORT TAKES

- **ACHILLION PHARMACEUTICALS' ACH-3102 –** The company announced positive proof-of-concept Phase Ib results with this investigational second-generation pan-genotypic NS5A inhibitor in 12 patients with hepatitis C. A single dose of ACH-3102 in HCV-1a patients resulted in a mean maximum 3.74 $\log_{10}$ reduction in HCV RNA. Based on these data and the results of a Phase Ia trial in healthy subjects, Achillion initiated a 12-week Phase II trial of ACH-3102 (225 mg on Day 1 then 75 mg QD) in combination with ribavirin in patients with HCV-1b who are IL28B CC.

- **ASTELLAS PHARMA's tacrolimus extended-release** was submitted to the FDA to prevent rejection of transplanted livers in men and transplanted kidneys in adults.

- **ASTEX PHARMACEUTICALS' amuvatinib (MP-470) –** The company halted development of this investigational drug in platinum resistant small-cell lung cancer (SCLC) after it missed the primary endpoint (≥10% response rate) in a Phase II trial. Only 2 of 21 patients had a partial response (PR), and there were no complete responses (CRs). However, Astex said it would consider licensing the drug to another company if anyone is interested.

- **Benzodiazepines –** A 1,063-patient, French observational study published in the ***British Medical Journal*** found people age ≥65 who took benzodiazepines (anti-anxiety drugs) were at substantially higher risk of developing dementia than non-users. New-onset dementia occurred 60% more often in the benzodiazepine users vs. never-users. The researchers urged doctors and regulatory agencies to "consider the increasing evidence of the potential adverse effects of this drug class for the general population."

- **BETA PHARMA's Conmana (icotinib) –** A speaker at the Chinese Society of Clinical Oncology meeting said this Chinese tyrosine kinase inhibitor, which was launched in China in August 2011 to treat non-small cell lung cancer (NSCLC), will be submitted for approval in the rest of the world.

- **Bioabsorbable miniature medical devices –** A mouse study published in the journal ***Science*** reported that researchers at the University of Illinois at Urbana-Champaign have successfully created a physically transient form of silicon electronics – basically tiny medical devices sealed in silicone cocoons that start dissolving after a couple of weeks in the body (when they are no longer needed). These particular devices were designed to fight post-surgical infection by creating heat, but the concept has many other

potential applications. *The technology is probably 10 years away from widespread use, but it is very interesting.*

- **BIOGEN IDEC and ORPHAN BIOVITRUM's rFIXFc (recombinant Factor IX Fc fusion protein) –** The companies announced that this investigational treatment for hemophilia B met the primary endpoint (bleeding control) in a Phase III trial in 123 adult men and was well tolerated. In the study, >90% of bleeding episodes were controlled by one injection of rFIXFc. The companies plan to submit the drug to the FDA in 1H13, and a submission will be made to the European Medicines Agency (EMA) when a study in boys is completed.

- **BRISTOL-MYERS SQUIBB and PFIZER's Eliquis (apixaban) –** The companies resubmitted this oral anti-coagulant to the FDA to prevent stroke in atrial fibrillation patients, and they have a new FDA PDUFA date – March 17, 2013, almost exactly a year later than expected.

- **CARDIOME PHARMA's Brinavess (vernakalant) –** **Merck**, which discontinued development of this investigational drug (both IV and oral) to treat chronic atrial fibrillation six months ago, has decided to return the rights to Cardiome, which said it plans to continue development itself.

- **CELLCEUTIX's Kevetrin –** The company said the FDA has lifted the clinical hold that was put on this investigational cancer drug nine months ago because of problems at its manufacturing partner, **Formatech**, which has filed for Chapter 7 bankruptcy. The company plans to start its first human trial in advanced solid tumors.

- **COSMO PHARMACEUTICALS' Methylene Blue MMx –** The company is looking to sell itself and hopes an investigational dye it developed (and which has finished Phase II development) to help detect pre-cancerous polyps will attract a buyer.

- **DEPOMED's Gralise (gabapentin) –** The company sued the FDA, requesting market exclusivity for this shingles pain treatment, which was approved in January 2011 and received orphan drug status but didn't get marketing exclusivity.

- **GALAXY BIOTECH's fibroblast growth factor 2 (FGF2) antibody – Roche** licensed exclusive worldwide development and commercialization rights to this investigational cancer treatment, which is still in the preclinical stage.

- **GLAXOSMITHKLINE and THERAVANCE's Breo/Relvar (fluticasone furoate + vilanterol)** was submitted to the FDA to treat chronic obstructive pulmonary disease (COPD). The PDUFA date is May 12, 2013.

- **HAEMONETICS'** purchase of **Hemerus Medical** is delayed because the FDA postponed approval of Hemerus' SOLX system, and the deal was contingent on SOLX getting FDA approval. The FDA wants additional information on SOLX before making an approval decision, which now is expected by mid-2013.

- **Insomnia drugs –** The U.K.'s Royal Pharmaceutical Society warned that its survey of 2,077 people found that too many people are self-diagnosing themselves as insomniacs and self-medicating with sleeping pills without seeking medical advice. In the survey, 30% of people said they had taken sleeping pills for >1 month without getting advice and 14% had gone six months.

- **Medical tourism –** Premera Blue Cross Blue Shield, an Alaska insurance company, reportedly will pay for airfare and hotel if its members ***want*** to travel to Washington state to have hip or knee replacement surgery (and some other procedures) because the surgery is less expensive there. Other groups, such as the National Education Association's Alaska Health Plan, are also planning to organize out-of-Alaska medical trips.

- **MEDTRONIC** is buying a Chinese orthopedic implant manufacturer, **Kanghui Holdings**, which specializes in trauma and spine products.

- **MERCK's lonafarnib –** A 2.5-year study published in the ***Proceedings of the National Academy of Sciences*** suggested that this cancer drug may be able to treat progeria, a fatal premature aging disease, by slowing or even reversing some negative cardiac effects and boosting weight. The study could not determine whether the drug affects survival in these patients.

- **MORPHOSYS' MOR-103 –** The company is optimistic that it can find a partner after positive Phase II results were reported in rheumatoid arthritis.

- **Multiple sclerosis –** A 315-patient study published in ***Science Translational Medicine*** found that gene transcription patterns separate relapsing-remitting multiple sclerosis (RRMS) patients into two groups – those who respond to current therapies (type B) and those who don't (type A). The risk of a disease event (e.g., relapse) was 40% less for type B patients. By year 5, >80% of type A patients had experienced an event vs. slightly more than 60% of type B patients.

- **Obesity vaccine? –** A mouse study presented at the Obesity Society annual meeting suggested that an obesity vaccine targeting the hormone somatostatin may eventually be developed to help humans lose weight. The researchers gave a vaccine containing purified chimeric somatostatin protein to obese mice on a high-fat diet and found the vaccinated mice lost up to 20% of their body weight in the first week and maintained the weight loss over the next two weeks while the unvaccinated mice gained weight.

- **Pedicle screws –** The FDA's Orthopaedic and Rehabilitation Devices Advisory Committee recommended that posterior cervical pedicle and lateral mass screws be designated as Class 2 devices. The panel also addressed proposed indications for use, including use for C1-C2 fixation, use in skeletally immature patients, and use in the absence of fusion.

- **PEREGRINE PHARMACEUTICALS' bavituximab –** The company said the Phase II data it released earlier in September on this investigational treatment for NSCLC – which showed a doubling in survival – was not reliable because of mistakes made by a contract research organization (CRO) it had hired to deliver the treatment to study patients. Peregrine said it found "major discrepancies" between patient test results and treatment code assignments by the CRO. *"Censoring" half the patients in the study would be at least a major discrepancy*. Apparently, this is not the first time Peregrine has had data "discrepancies" in a clinical trial.

- **QUESTCOR PHARMACEUTICALS' Acthar (adrenocorticotropic hormone, ACTH) –** The company's marketing practices for this gel treatment for nephrotic syndrome, multiple sclerosis, and infantile seizures are under federal investigation. Questcor disclosed the investigation in an SEC filing but did not say which federal agency is heading the investigation. Aetna earlier this month said it is dropping all reimbursement for Acthar except for infantile spasms, though treatment of patients currently receiving Acthar gel will be covered until they complete treatment, and it appears that other insurers may follow suit.

- **RECKITT BENCKISER's Suboxone (buprenorphine + naloxone) –** The company is withdrawing the tablet form of this opioid dependency treatment, which went off patent in 2009, and will focus on development of a new formulation with patent protection. The company still has a patent-protected film formulation on the market.

- **Second Sight Medical Products' Argus II Retinal Prosthesis System –** The FDA's Ophthalmic Devices Panel of the Medical Devices Advisory Committee voted unanimously (19-0) to recommend approval of this device, which is intended to help patients with retinitis pigmentosa who are nearly blind to regain at least some vision. The device includes a video camera attached to a pair of eyeglasses, a processing unit, and an implanted retinal prosthesis. The eyeglasses capture images, which are sent to a processing unit worn on a belt. The processor transforms the images into an electrical stimulation pattern that is transmitted to the implanted retinal prosthesis.

- **Silvestrol –** Ohio State University signed an agreement with the Sarawak Biodiversity Centre in Malaysia to collaborate on the further development and commercialization of this investigational cancer drug (for leukemia, lymphoma, and other malignancies) derived from a tropical tree that grows on the island of Borneo. Clinical trials are not expected to start until 2015-2016.

- **SPECTRAL DIAGNOSTICS' Toraymyxin (polymyxin B fiber column) –** The FDA approved the company's proposed revision to the ongoing Phase III EUPHRATES trial of this investigational treatment for septic shock to allow two rather than one interim analysis. *Of course, there is a statistical penalty when this happens.*

- **SUCAMPO PHARMACEUTICALS and TAKEDA PHARMACEUTICALS' Amitiza (lubiprostone)** was granted priority review by the FDA for an expanded indication – treatment of opioid-induced constipation in patients with non-cancer chronic pain. This means there could be a decision by January 2013.

- **VALEANT PHARMACEUTICALS** bought the rights to **Visudyne** (verteporfin injection), a treatment for wet age-related macular degeneration (AMD), from **QLT**.

- **Wireless ECG monitoring systems –** A science advisory – published in ***Circulation: Journal of the American Heart Association*** and endorsed by the American College of Cardiology and the Heart Rhythm Society – warns that wireless ECG monitoring systems may have clinically significant delays between when a patient's heart rhythm is measured and when it shows up on a bedside monitor. Doctors are advised to use hard-wired telemetry systems when instantaneous assessment is needed for clinical decision-making (e.g., cardiac resuscitation, hospital "codes," and defibrillation).

## NEWS IN BRIEF

### Acne – viruses open new treatment option

Scientists reported in ***mBio***, a journal of the American Society for Microbiology, that viruses (phages) might be useful in treating acne, either as a direct therapy or as a component. The researchers isolated phages and *P. acnes* bacteria from human volunteers with and without acne, then sequenced the phages' genomes.

They found the phages were remarkably similar, sharing >85% of their DNA. The lack of genetic diversity suggests that resistance to phage-based antimicrobial therapy is less likely to develop. All of the phages carry a gene that makes the protein endolysin, an enzyme that is thought to break down bacterial cell walls and kill the bacteria, suggesting that endolysin from these phages might also be useful as a topical anti-acne therapeutic.

### Anticoagulants – benefit may not outweigh the risk

A meta-analysis published in the ***Archives of Internal Medicine*** found that benefits (reduction in ischemic events) of the new oral anticoagulants – e.g., **Boehringer Ingelheim's Pradaxa** (dabigatran) and **Johnson & Johnson's Xarelto** (rivaroxaban) – are offset by the risks (increased bleeding) in patients after an acute coronary syndrome (ACS). The researchers examined seven trials and found a statistically significant but "modest" reduction in ischemic events and a non-significant decrease in overall deaths, for a net clinical benefit odds ratio of 0.98 (Nss, p=0.57). And this "modest" benefit came at a cost of a three-fold increase in the risk of major bleeding.

### AstraZeneca's Brilinta (ticagrelor) – safe and effective in elderly

A new subanalysis of the PLATO trial, published in ***Circulation: Cardiovascular Quality and Outcomes***, found that age had no impact on the efficacy of this antiplatelet drug vs. clopidogrel in ACS patients, suggesting the benefits of the drug are consistent regardless of age. The subanalysis found:

- In PLATO, elderly patients (age ≥75) had a higher rate of thrombotic cardiovascular (CV) events and a higher rate of major bleeding.
- The reduction in thrombotic CV events vs. clopidogrel was similar in patients age ≥75 and patients <age 75.
- There was no significant interaction between age and the drug on major bleeding or non-CABG major bleeding.
- Dyspnea rates and ventricular pauses were higher in elderly vs. younger patients, but that was true with both Brilinta and clopidogrel, but there was no evidence of an age-related treatment interaction.

### Breast cancer – four distinct types

A comprehensive genetic analysis of breast cancer, published in the journal ***Nature***, may fundamentally change the way scientists think about breast cancer. The researchers identified four genetically distinct types of the cancer. Within each type, they found genetic changes driving many cancers. The discoveries are expected to lead to new treatments using drugs already approved for other cancers and to new treatment approaches. However, it probably will still take years to translate into transformative new treatments.

Even within these four types of breast cancer, individual tumors are driven by their own sets of genetic changes. So, just one treatment per type will probably not be enough. The investigators identified at least 40 genetic alterations that might be drug targets. Many of them are already being developed for other cancers with the same mutations.

Perhaps the biggest surprise in the study was the discovery that triple negative breast cancer is completely different from the other types of breast cancer and more closely resembles ovarian cancer and a type of lung cancer, suggesting there may be a common cause. The findings also suggest that some triple negative breast cancer patients could be treated with ovarian cancer drugs that are less toxic than the anthracyclines often prescribed to triple negative breast cancer patients.

### Gout – new guidelines

New clinical guidelines from the American College of Rheumatology for managing gout were published in ***Arthritis Care & Research***. The recommendations include:

- Treating with xanthine oxidase inhibitors (XOIs) to lower urate levels.
- Treating with allopurinol or febuxostat (**Takeda's Uloric**) as a first-line treatment.
- Screening patients for allopurinol hypersensitivity before they start taking the drug.
- Lowering urate levels to <6 mg/dL.
- If the target urate level is not reached with one XOI, then add a uricosuric agent.
- Acute gout attacks require medication within 24 hours of onset and for the duration of the attack.

- Pegloticase (**Savient Pharmaceuticals' Krystexxa**) is appropriate for patients with severe disease who can't tolerate or don't respond to urate-lowering drugs.
- Non-steroidal anti-inflammatory drugs (NSAIDs), corticosteroids, and oral colchicine are appropriate first-line options to treat acute attacks.
- All patients should get prophylaxis with anti-inflammatory medication when urate-lowering treatment begins.
- Oral colchicine or a low-dose NSAID is appropriate first-line therapy to prevent acute gout attacks.

### ROCHE/GENENTECH

- **Avastin (bevacizumab).** A 60-patient, sham-controlled trial presented at the EURETINA meeting in Milan, Italy, found that visual acuity significantly improved in patients with central retinal vein occlusion (CRVO) and macular edema who were treated with Avastin. At 12 months, 60% of Avastin patients gained ≥15 letters vs. 33% in the sham group (which got Avastin but later). The authors concluded that delaying Avastin treatment reduced its efficacy.
- **Lucentis (ranibizumab).** A study presented at EURETINA found that giving Lucentis injections to wet AMD patients on a regular monthly schedule did not reduce vision loss vs. treating them on an individual per-patient schedule. Another study, with 48 patients, presented at the meeting found that low-dose transpupillary thermotherapy (TTT) significantly reduced the number of Lucentis injections needed to reduce vision loss. Over 2 years, patients in the TTT group got 6.3 Lucentis injections vs. 8.0 injections for the no-TTT patients.

### VERTEX PHARMACEUTICALS

- **ALS-2158.** Development of this investigational hepatitis C drug, an adenosine nucleotide analog prodrug, was stopped due to lack of efficacy in a 7-day viral kinetic study.
- **ALS-2200 (VX-135).** This hepatitis C drug, a uridine nucleotide analog prodrug (an NS5B polymerase inhibitor) licensed from **Alios BioPharma**, is moving into Phase II after a 7-day Phase I viral kinetic study found that a 200 mg dose of ALS-2200 + ribavirin led to a 4.18 $\log_{10}$ reduction in HCV RNA with 5 of 8 naïve HCV-1 patients below the limit of quantification (≤25 IU/mL). No patients discontinued due to adverse events, and there were no serious adverse events. Data from this study will be presented at the American Association for the Study of Liver Diseases (AASLD) meeting in Boston in November. Pending FDA agreement, the company plans to initiate a Phase II trial of ALS-2200 + ribavirin and another Phase II trial of ALS-2200 + **Incivek** (telaprevir).

## REGULATORY NEWS

### FDA gets authority to oversee clinical trial data

The Department of Health and Human Services (HHS) gave the FDA authority to investigate potentially false, misleading, or missing data from clinical trials submitted to the **ClinicalTrials.gov** database.

### FDA tidbits

- The **Office of Regulatory Affairs** is being reorganized. The FDA is combining the functions of its Office of Regional Operations and three other units as part of an effort to improve the Office of Regulatory Affairs.
- The FDA hired a **contract research organization**, Vince & Associates Clinical Research, to study the bioequivalence of branded and generic drugs.
- Following a Government Accountability Office report that **wireless medical technologies** are potentially vulnerable to intentional data security threats, FDA has said it plans to add a review of "information security risks" to its evaluations of medical device software.
- The FDA's **Drug Shortages Staff** (DSS) are being moved from the Office of New Drugs and elevated to the Office of the Center Director under the leadership of Douglas Throckmorton, MD, deputy director for regulatory programs.

### FDA and Kidney Society form partnership

The American Society of Nephrology (ASN) and the FDA announced a new partnership, the Kidney Health Initiative (KHI), to improve patient safety and promote development of new therapies of patients with kidney disease. KHI is a collaborative effort intended to be a forum for scientific collaboration and dialogue with patient groups and others concerned about kidney health.

### FDA urged to speed up some drug approvals

The President's Council of Advisors on Science and Technology proposed a two-fold increase in the number of new prescription drugs approved annually. In its report, the council suggested that the FDA begin by accelerating the approval process on treatments developed for patients

considered high-risk, such as the morbidly obese. However, the council did not outline how the FDA could limit drugs approved for special medical use to high-risk patients only.

## FDA approvals/clearances

- **ABBOTT's Humira (adalimumab)** received expanded approval to treat moderate-to-severe ulcerative colitis in adults. It was already approved to treat rheumatoid arthritis, ankylosing spondylitis, Crohn's disease, plaque psoriasis, and juvenile idiopathic arthritis.
- **BAYER and ONYX PHARMACEUTICALS' Stivarga (regorafenib)**, a multikinase inhibitor, was approved to treat metastatic colorectal cancer a month ahead of its PDUFA date. In its pivotal trial, Stivarga improved overall survival by 1.4 months and progression-free survival (PFS) by 9 days. The drug will carry a boxed warning about possible fatal liver toxicity.
- **BOSTON SCIENTIFIC/CAMERON HEALTH's S-ICD**, a subcutaneous (and lead-less) implantable cardioverter defibrillator, was approved. The company is required to conduct a 1,616-patient, five-year postmarketing study to assess the long-term safety and performance of the device and to assess differences in effectiveness across genders.
- **INTELOMED's CVInsight** system, which measures and records patients' pulse rates, SpO2, and other vital signs, received 510(k) clearance.
- **MINDCHILD MEDICAL's Meridian**, a device for monitoring fetal heart rates noninvasively, received 510(k) clearance.
- **REGENERON PHARMACEUTICALS' Eylea (aflibercept)** was granted expanded approval to treat macular edema following central retinal vein occlusion.
- **SMITHS MEDICAL's Portex CorrectInject**, which delivers epidural and spinal anesthesia drugs, was given 510(k) clearance.
- **TELEFLEX's Arrow FlexBlock** system, which helps doctors during ultrasound-guided catheter placement procedures, was cleared for use.

## FDA recalls/warning letters

- **CARDIOMEMS' HF Pressure Measurement System –** The company received a warning letter after a pre-PMA inspection found irregularities related to the CHAMPION trial and the company's premarket application. Specifically, the FDA said the company:
  - Failed to submit an accurate investigational plan, including failure to tell the Agency that the company would be making recommendations to the study investigators for medical management of the patients.
  - Doctored records, changing emails and Device Return Forms.
  - Failed to give investigators sufficient information to conduct the trial properly.
  - Allowed inadequate informed patient consent.
- **STRYKER's Neptune Rover Waste Management System –** The Class 1 recall of these products – which are used to collect fluid waste during surgery – was expanded to include three versions of the product after two injuries and one fatality occurred with the devices, which were being sold without FDA clearance.

## European regulatory news

- **ALMIRALL's Constella (linaclotide) –** The Committee for Medicinal Products for Human Use (CHMP) recommended the EMA approve this irritable bowel syndrome (IBS) drug for adults with moderate-to-severe IBS with constipation (IBS-C). If approved, this would be the first IBS drug approved in the European Union.
- **BOSTON SCIENTIFIC's Reliance 4-Front** line of defibrillator leads received a CE Mark.
- **BRISTOL-MYERS SQUIBB and PFIZER's Eliquis (apixaban) –** CHMP recommended the EMA approve this oral anticoagulant to prevent stroke in atrial fibrillation patients.
- **MEDTRONIC's CoreValve Evolut –** A 23 mm transcatheter aortic valve was granted a CE Mark.
- **New medical device oversight –** The European Union's Health Commissioner is proposing new safety and monitoring rules for medical devices, including formation of an investigative panel that would "have the possibility to pick out medical devices on certain risk-based criteria to decide whether to go into an in-depth analysis of the processes."

■ **NOVARTIS**

- **Eucreas (vildagliption + metformin)** – CHMP recommended approval for Type 2 diabetics not controlled with diet, exercise, and a combined metformin/sulphonylurea treatment.
- **Galvus (vildagliptin)** – CHMP recommended approval in combination with insulin for Type 2 diabetics not controlled with diet, exercise, and regular insulin doses.
- **Votubia (everolimus)** – CHMP recommended approval to treat benign kidney tumors (tuberous sclerosis complex, TSC-related angiomyolipomas).

■ **REGENERON PHARMACEUTICALS' Eylea (aflibercept)** – CHMP recommended the EMA approve Eylea to treat patients with wet AMD. A final EMA decision is expected in 4Q12.

■ **VIVUS' Qsiva (phentermine/topiramate ER)** – The company said that "preliminary feedback" from CHMP suggests the advisory panel will ***not*** recommend approval of this diet drug.

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## U.K.'s National Institute for Health and Clinical Excellence (NICE) news

**BOEHRINGER INGELHEIM's Actilyse (alteplase)** – NICE changed its usage recommendation for this treatment for acute ischemic stroke, saying it can now be used up to 4.5 hours after the onset of the stroke vs. 3 hours previously.

| Upcoming FDA Advisory Committees and Other Regulatory Meetings of Interest (*items in RED are new since last week*) | | |
|---|---|---|
| **Date** | **Topic** | **Committee/Event** |
| **2012** | | |
| October 3 | Discussion of establishment of a new subcommittee to evaluate the FDA's efforts to address the **challenges in the Science Board's 2007 report** | FDA's Science Board |
| October 5 | Validity, reliability, and usability of **glaucoma imaging devices** | FDA and American Glaucoma Society public workshop |
| October 12 | **Celgene's Abraxane** (nab-paclitaxel) to treat NSCLC | PDUFA date |
| October 16 | **NPS Pharmaceuticals' Gattex** (teduglutide) for short bowel syndrome | FDA's Gastrointestinal Drugs Advisory Committee |
| October 17 | **Aegerion Pharmaceuticals' lomitapide** to treat homozygous familial hypercholesterolemia | FDA's Endocrinologic and Metabolic Drugs Advisory Committee |
| October 17 | **ThromboGenics' ocriplasmin** to treat vitreomacular adhesions | PDUFA date |
| October 18 | **Sanofi/Genzyme** and **Isis Pharmaceuticals' Kynamro** (mipomersen) to reduce cholesterol in patients with homozygous familial hypercholesterolemia | FDA's Endocrinologic and Metabolic Drugs Advisory Committee |
| October 21 | **Impax Laboratories' IPX-066** for Parkinson's disease | PDUFA date |
| **October 23-24** | **Update on scientific initiatives and accomplishments** over the past year, including an update from the NanoCore Subcommittee and from the Office of Science Coordination. In addition, CBER, CDER, and other FDA centers will discuss their center-specific research strategic needs | Science Advisory Board to the National Center for Toxicological Research meeting |
| October 24 | **Hologic's Selenia Dimensions 3D System –** expanded indication to combine digital breast tomosynthesis with synthetic 2D images for cancer screening | FDA's Radiological Devices Advisory Committee |
| October 29 | **Cornerstone Therapeutics/Cardiokine Biopharma's Lixar** (lixivaptan, CRTX-080) to treat hyponatremia | PDUFA date |
| October 29-30 | Discussion of benefits, risks, and abuse of drugs containing **hydrocodone** | FDA's Drug Safety and Risk Management Advisory Committee |
| November 8 | **Novo Nordisk's Tresiba** (degludec) **and Ryzodeg** (degludecPlus) | FDA's Endocrinologic and Metabolic Drugs Advisory Committee |
| November 9 | **MSD Consumer Care's "Oxytrol for Women,"** an over-the-counter transdermal oxybutynin to treat overactive bladder in women | FDA's Non-prescription Drugs Advisory Committee |
| November 14 | Optimization of outcomes with **ventricular assist devices** (VADs) for patients with heart failure | CMS' MEDCAC |
| November 21 | **Pfizer's tofacitinib**, an oral JAK inhibitor for rheumatoid arthritis | PDUFA date (extended from August 21) |
| **November 28** | **Johnson & Johnson's bedaquiline** to treat patients with multi-drug resistant pulmonary tuberculosis | FDA's Anti-infective Drugs Advisory Committee |
| November 28 | Discussion of the use of **absorbable material** in a variety of medical devices | FDA Workshop on Absorbable Medical Devices: Lessons Learned From Correlations of Bench Testing and Clinical Performance |
| November 29 | **Exelixis' cabozantinib** to treat medullary thyroid cancer | PDUFA date |
| December 4 | Discussion (no votes) of pediatric development plans for **GlaxoSmithKline's trametinib, Threshold Pharmaceuticals' TH-302, Boehringer Ingelheim's volasertib** (BI-6727)**,** and **Amgen's blinatumomab** (MT-103) | FDA's Pediatric Oncology subcommittee of the Oncologic Drugs Advisory Committee (ODAC) |
| December 15 | **Human Genome Sciences' raxibacumab** to treat anthrax | PDUFA date |
| **December 20** ***(tentative)*** | **Hemispherx Biopharma's Ampligen** (poly I: poly C12U) to treat chronic fatigue syndrome (CFS) | FDA's Pulmonary-Allergy Drugs Advisory Committee (not confirmed). Remember the FDA reorganized the Office of New Drugs and put CFS in the Division of Pulmonary, Allergy, and Rheumatology Products (DPARP) |
| December 21 | **Alexza Pharmaceuticals' Adasuve** (loxapine) for agitation associated with schizophrenia or bipolar disorder | PDUFA date |
| December 28 | **Biogen Idec's BG-12** for multiple sclerosis | PDUFA date |
| December 29 | **Aegerion Pharmaceuticals' lomitapide** to treat homozygous familial hypercholesterolemia | PDUFA date |
| December 29 | **Johnson & Johnson's bedaquiline** to treat multidrug-resistant tuberculosis | PDUFA date |
| December 30 | **NPS Pharmaceuticals' Gattex** (teduglutide) for short bowel disease | PDUFA date |

| Upcoming FDA Advisory Committees and Other Regulatory Meetings of Interest (*items in RED are new since last week*) | | |
|---|---|---|
| **Date** | **Topic** | **Committee/Event** |
| | **2013** | |
| January 16 | **Santarus' Uceris** (budesonide) for ulcerative colitis | PDUFA date (extended from October 16, 2012) |
| January 17 | **NuPathe's Zelrix** (transdermal sumatriptan), a migraine patch | PDUFA date |
| January 29 | **Sanofi/Genzyme** and **Isis Pharmaceuticals' Kynamro** (mipomersen) for homozygous familial hypercholesterolemia | PDUFA date |
| January 30 | **Raptor Pharmaceutical's cysteamine bitartrate** delayed-release (RP-103) to treat nephropathic cystinosis | PDUFA date |
| **February 2** | **Hemispherx Biopharma's Ampligen** (poly I: poly C12U) to treat chronic fatigue syndrome | PDUFA date |
| February 10 | **Celgene's pomalidomide** for relapsed/refractory multiple myeloma | PDUFA date |
| February 24 | **Dynavax's Heplisav** hepatitis B vaccine | PDUFA date |
| February 28 | **Lundbeck** and **Otsuka's aripiprazole depot** to treat schizophrenia | PDUFA date |
| March 1 | **Zogenix's Zohydro** (extended-release hydrocodone) for chronic pain | PDUFA date |
| **March 17** | **Bristol-Myers Squibb and Pfizer's Eliquis** (apixaban,) an oral anticoagulant to prevent stroke in atrial fibrillation patients | ***New*** PDUFA date |
| April 11 | **Sanofi/Genzyme** and **Bayer's Lemtrada** (alemtuzumab) for multiple sclerosis | PDUFA date canceled because the FDA refused to accept the filing |
| April 29 | **Shire's Vyvanse** (lisdexamfetamine dimesylate), sNDA for ADHD treatment in children | PDUFA date |
| **May 12** | **GlaxoSmithKline and Theravance's Breo/Relvar** (fluticasone furoate + vilanterol) to treat chronic obstructive pulmonary disease (COPD) | PDUFA date |