Broad Institute in the driver’s seat following PTAB’s favorable ruling

By Marilyn Fenichel

The Patent Trial and Appeal Board (PTAB) has ruled in favor of the Broad Institute of MIT and Harvard in its dispute with the University of California (UC) Berkeley and the University of Vienna over intellectual property issues surrounding CRISPR/Cas9 technology. The powerful technology can be used to edit and modify the genome of organisms.

The saga began with the discovery of CRISPR/Cas9 in 2012. Dr. Jennifer Doudna, a structural biologist at UC Berkeley, in collaboration with Dr. Emmanuelle Charpentier, now director of the Max Planck Institute for Infection Biology, developed the technology for use with prokaryotes, or bacteria cells, and has a broad patent for their initial invention. Shortly thereafter, however, Broad’s Dr. Feng Zhang determined how to use the technology with eukaryotic cells, or plant and animal cells, and obtained several patents protecting this discovery. The UC Berkeley challenged these patents, claiming that Broad had simply built on its earlier discoveries.

The PTAB saw it differently. It sided with Broad, agreeing that the institute had indeed designed a new system. To date, 50 patents with claims to CRISPR/Cas9 have been issued, and 13 of those belong to the Broad Institute, MIT and affiliated groups.

With the interference removed, UC Berkeley is moving forward with its patent claim for CRISPR/Cas9 in 2012. Dr. Jennifer Doudna, a structural biologist at UC Berkeley, in collaboration with Dr. Emmanuelle Charpentier, now director of the Max Planck Institute for Infection Biology, developed the technology for use with prokaryotes, or bacteria cells, and has a broad patent for their initial invention. Shortly thereafter, however, Broad’s Dr. Feng Zhang determined how to use the technology with eukaryotic cells, or plant and animal cells, and obtained several patents protecting this discovery. The UC Berkeley challenged these patents, claiming that Broad had simply built on its earlier discoveries.

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Icon steps up for patient-reported outcome measure endpoint validation

By Jeremy Zucker

The FDA has chosen Icon to create industry-standard patient-reported outcome (PRO) measures and to validate those endpoints for antibacterial drug trials. Partnering on the initiative with Icon is the Biomarkers Consortium and the Foundation of the National Institutes of Health (FNHI). Also involved is the Infectious Diseases Society of America and the National Institute of Allergy and Infectious Diseases.

The academic research community and pharmaceutical and biotech companies are coming together to identify new methods for assessing the success factors for antibiotics. The research will be applied in clinical trials for antibacterial endpoints—hospital-acquired bacterial pneumonia (HABP), acute bacterial skin and skin structure infections (ABSSSI) and community-acquired bacterial pneumonia (CABP).

The FDA awarded Icon the contract following a period of review after the company responded to the FDA Broad Agency Announcement for solicitation of research to advance regulatory science.

The FDA is interested in these PRO instruments because they directly measure how a patient feels or functions in clinical trials of new drugs for CABP, HABP and ABSSSI. Studies conducted by Icon under the contract will address the refinement of clinical endpoints for three endpoints—hospital-acquired bacterial pneumonia (HABP), acute bacterial skin and skin structure infections (ABSSSI) and community-acquired bacterial pneumonia (CABP).

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Industry Briefs

Study Conduct

- President Lieutenant General of the Republic of Botswana, the Bristol-Myers Squibb Foundation, Texas Children’s Cancer and Hematology Centers (TXCH) and Baylor College of Medicine International Pediatric AIDS Initiative at Texas Children’s Hospital (BI-PAL), through public-private partnerships with the governments of Botswana, Uganda and Malawi, announced a $100 million initiative to create an innovative pediatric hematology-oncology treatment network in southern and eastern Africa. The comprehensive initiative called Global HOPE (Hematology-Oncology Pediatric Excellence) will build long-term capacity to treat and dramatically improve the prognosis of thousands of children with cancer and blood disorders in southern and eastern Africa. The initiative will also create significant clinical, educational and research capabilities. Doctors, nurses and ancillary professionals will be recruited from around the world to provide training to local healthcare professionals and to begin treating children with blood disorders and cancer immediately.

- Clinerion and ASUS Life signed a strategic alliance to, together with the Show Chwan Healthcare System, deliver innovative services for the efficient running of clinical trials, clinical patient recruitment, market access and real-world evidence in Taiwan. ASUS Life is a unique joint venture that combines the power and reliability of ASUS’ information technology with the clinical research services of the Show Chwan Healthcare System, in Taiwan. The new alliance between Clinerion and ASUS Life helps shorten the time it takes to bring a new drug to market, benefitting patients who get earlier access to new medicines.

CROs/Service Providers

- PPD has entered into a collaboration with Frenova Renal Research, a drug and medical device contract clinical development services provider dedicated exclusively to renal research. Through this relationship, PPD will offer its clients the therapeutic expertise of Frenova’s global nephrology experts, as well as access to FIRST Up (Frenova Rapid Start Up), Frenova’s exclusive alliance of research sites designed to streamline, initiate and enroll clinical studies faster than industry norms with just-in-time patient recruitment.

- In order to support customers throughout their entire biological drug development process, GMP specialist Anacura and the R&D-focused RIC have initiated a collaboration under the moniker anaRIC biologics. anaRIC biologics is a CRO specialized in the analysis of complex, biological compounds, such as antibodies, proteins and peptides. These innovative products differ drastically from classical, small molecules and require a different set of skills to analyze. With 15 years of GMP and 30 years of R&D experience, anaRIC biologics is able to support this rapidly growing market segment throughout the entire drug development process.

- ProTrials Research announced the expansion of its suite of services. In response to the evolving needs of their clients, ProTrials has broadened their offering, adding both data management services and a variety of electronic data capture (EDC) platforms, which allow for a customized approach to meet individual client needs.

Technology Solutions

- iCardiac Technologies has launched QPoint, its next-generation electronic clinical outcome assessment (eCOA) platform, to address the growing demand for more efficient and innovative eCOA study services in drug development. In addition to a number of other benefits, QPoint delivers short and streamlined study configuration and startup to the industry. iCardiac set out to address key industry challenges such as long study startup times. The QPoint platform is engineered for rapid configuration to clinical trial protocols, saving multiple weeks—or more—in the study startup cycle. This is accomplished by study team members being able to customize study questionnaires and how they will appear on devices in real time through RapidStart, an interactive study setup and validation portal.

- NTT DATA announced the company’s existing relationship with Oracle is expanding to include end-to-end cloud capabilities for Oracle’s Healthcare Foundation, a unified healthcare analytics platform. The platform provides data integration and warehousing of clinical, financial, administrative and -omics modules in a cloud solution to customers worldwide. The healthcare cloud offering will provide organizations the ability to easily unify, grow or replace their existing healthcare data warehouse and data aggregation solutions. “This collaboration with Oracle will help healthcare organizations implement a cloud-based solution that will improve outcomes and deliver tangible business results,” said Dan Allison, president, global healthcare and life sciences, NTT DATA. “In addition, our work with Oracle reinforces NTT DATA’s commitment to innovation, as we bring data together to provide analytics for multiple stakeholders in the healthcare enterprise.”
Industry Briefs (continued from page 2)

- **BC Platforms**, a provider of genomic data management solutions, will join the Microsoft AI in Health Partner Alliance, an expanding group of partners focused on advancing health technology. This initiative includes investments in resources for Microsoft partners to capture new opportunities to apply artificial intelligence (AI) to healthcare. As part of its participation, BC Platforms will receive unique training and access to Microsoft technologies, engineering expertise and data sets.

- Three European technology SMEs, together with two research institutions, **Oxford University** and **Imperial College London**, have been awarded £2.5 million in funding from the European Commission under the H2020 Fast Track to Innovation (FTI) pilot to develop and launch a cell metabolism analysis platform that is predicted to be a part of routine in vitro cell biology and drug development. Led by **Luxcel Biosciences**, the consortium will demonstrate a Gold Standard platform, comprising cell assays, disease models and services, together with high performance metabolism tests designed for use on laboratory standard instrumentation—the multi-mode microtiter plate reader.

**Ethics/Regulatory**

- **Sarepta Therapeutics**, a commercial-stage developer of innovative RNA-targeted therapeutics, has entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV). Sarepta received the PRV when EXONDYS 51™ was approved by the **FDA** for the treatment of patients with Duchenne muscular dystrophy amenable to exon 51 skipping. The voucher was awarded by the FDA under a provision that encourages development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. With the passage of the 21st Century Cures Act, this PRV program has been extended through September 30, 2020.

- **Certara**, a provider of decision support technology and consulting services for optimizing drug development and improving health outcomes, has announced that the Standards Council of Canada (SCC) has awarded Good Laboratory Practices (GLP) certification to its Certara Strategic Consulting (CSC) Montreal facility. CSC Montreal has passed the requisite inspection and study audits and is now recognized as a GLP compliant Toxicokinetic Test Site by SCC.

**R&D Trends**

- **Becton, Dickinson and Company** (BD), a global medical technology company, has announced a new alliance dedicated to help address the rising opioid crisis in acute care settings. The alliance is a new initiative stemming from the recently launched BD Institute for Medication Management Excellence. The industry alliance will expand on the key initiatives established by the BD Institute: Opioid Control and Management, End-to-End IV Safety and Medication Availability concentrating on identifying effective ways of tracking controlled substances across the entire end-to-end medication management process with the ability to detect, monitor and ultimately reduce drug diversion.

**Drug Sponsors**

- **viihealth**, a next-generation customer engagement company dedicated to helping global life science clients improve business results, has acquired **Hubdata**, a French-based provider of decision analytics SaaS solutions. The Hubdata team has utilized machine learning-based advanced analytics to drive business outcomes, such as customer loyalty, yield management, repeat revenue and profitability for international clients. The Hubdata platform provides viihealth the foundational core to effectively add advanced data analytics that provide prediction and prescription guidance in its solution suite to address the needs of life science marketers.

- **Integra LifeSciences**, a global provider of medical technology, has made a binding offer to acquire the **Johnson & Johnson** Codman Neurosurgery business for $1 billion in cash. Codman Neurosurgery offers a portfolio of devices focused on advanced hydrocephalus, neuro-critical care and operative neurosurgery. If the binding offer is accepted, upon closing, Integra will be a global provider of neurosurgical products. “This proposed transformational acquisition of Codman Neurosurgery creates compelling value for our shareholders, employees and patients,” said Peter Arduini, Integra’s president and chief executive officer. “Its innovative portfolio and global reach will enable us to enhance our position in the neurosurgery market, while also building a global infrastructure that will benefit Integra as a whole. We look forward to welcoming the more than 600 Codman Neurosurgery employees to the Integra team.”

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Patent struggle  (continued from page 1)

use of the technology with eukaryotic cells. But for now, Broad is in the driver’s seat.

“This decision is a clear victory for Broad,” said Jacob Sherkow, an intellectual property attorney at the New York Law School in New York City. “My guess is that UC Berkeley will appeal it to the U.S. Court of Appeals for the Federal Circuit, with the hope of winning outright. If I was their lawyer, that’s what I would recommend.”

For academic institutions, which conduct research for noncommercial purposes, this decision may have little impact. Both institutions have been generous in licensing the technology to academic institutions both in the U.S. and abroad.

In the commercial world, however, the situation is different, in part because of the approach UC Berkeley and Broad have taken in licensing their intellectual property. They have chosen to license them to surrogate companies, which take on the patent, the profits and much of the risk. UC Berkeley has granted an exclusive license to conduct research on the whole genome to a company called Caribou Biosciences, which in turn has a sublicense agreement with Intellia Therapeutics. This company is working on in vivo indications for liver disease and ex vivo systems with stem cells. The Broad Institute has a exclusive licensing agreement with Editas Medicine, which is also working on human applications.

For now, companies like Intellia, which has its license from UC Berkeley, are putting a positive spin on the ruling. Though admitting to being disappointed in PTAB’s decision, Jose Rivera, executive vice president, General Counsel & Operations of Intellia, said, “It is important to note what the ruling did and did not do. It did not make a determination about who invented the technology first, and it acknowledged the overlap between the two sets of claims. With the interference removed, UC Berkeley’s patent can move forward, and we believe they will prevail.”

But Sherkow has a different perspective. “It is unclear how generous Broad is going to be in giving licenses to technology companies,” he said. “What’s more, it is possible that if UC Berkeley receives its patent, companies may have to pay fees to both institutions to use the CRISPR/Cas9 technology. Either way, it is likely that they will be paying fees to Broad.”

If that’s the case, the results of a survey of CRISPR trends may be providing even more good news. Conducted by Synthego, a company that provides synthetic RNA and other genome-editing tools, the survey was designed to find out directly from the research community how scientists are using CRISPR technology and what their plans are for the future.

The survey found that 87% of users are new to gene editing, and 55% of that group have plans to conduct experiments within six months. The survey also showed that 35% of new researchers plan to conduct experiments directly related to therapeutic applications, with stem cell research representing 18%, drug discovery 11% and CAR-T therapies 6%.

“It was a big surprise to us that the large majority of new CRISPR users are also new to gene editing. This tells us there’s a tremendous hands-on interest in CRISPR as a genome editing technique.” —Mike Dabrowski, president & co-founder of Synthego

The Synthego survey points to the potential for new customers, who may find that Broad is a force to contend with. If Sherkow’s predictions prove to be correct, these customers may be paying licensing fees to Broad at some point down the road.

To get around Broad’s potential influence, academic institutions may want to reconsider exclusive licenses, which may not be necessary. “Instead of giving companies licenses to work on the entire DNA of the human genome, they may want to consider only part of it or for specific indications,” Sherkow noted. “This also may be a good time to look for new nuclei and recombinant nuclei that can be used in the system.”

Sherkow then offered one more suggestion. “Before the appeal process begins, companies may get a better deal from Broad. Perhaps they should start thinking about making nice with the institute.”

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endpoints for trials in patients with these serious infections. HABP is a leading cause of death in intensive care units and one of the most common hospital-acquired infections. CABP and ABSSSI also result in high rates of morbidity worldwide. PROs are important because they are a measurement based on a report that comes from the patient about the status of the patient’s health condition without amendment or interpretation of the patient’s report by a clinician or any other healthcare representative.

“Despite the high mortality and morbidity of these conditions, sponsors engaged in this clinical research do not currently have a consistent methodology to assess primary endpoints, which can slow development and ultimately delay the delivery of medicines to patients,” said Brittany Erana, senior director, Clinical & Scientific Research, Icon Commercialization and Outcomes. “The PROs currently in development could be used to define endpoints that can provide direct evidence of treatment benefit on how patients feel or function. When developed appropriately, they can increase the efficiency and clinical relevance of clinical trials.”

The PRO project is a critical component of the work that Icon, together with the FNIH and the Biomarkers Consortium, has been carrying out for the FDA to support the development of safe and effective antibacterial treatments in areas of unmet need in infectious disease. The relationship dates back to 2012, with the FNIH-funded development of PRO measures for use with patients diagnosed with CABP and ABSSSI. In 2014, the FDA funded the development of the third PRO measure for patients diagnosed with HABP. All three instruments have been designed to accurately and comprehensively assess the symptoms of each disease area at various time points over the course of the infection. Icon’s Clinical Outcomes Assessment (COA) scientific research team has shown content validity for all three instruments through the analysis of qualitative data collected through interviews with patients and clinical experts.

Icon’s COA team will design and execute a psychometric validation study for the three PRO instruments in accordance with the FDA guidance for PRO measures used to support labeling claims. The project will be performed in line with the Drug Development Tool (DDT) Qualification Program.

As part of the project scope, these measures will be adapted for administration via electronic Clinical Outcomes Assessment technology (eCOA). This technology will allow patients to answer the questionnaire on a handheld device at specified time points over the course of the infection in order to measure the effects of antibacterial drugs.

Once the measures are psychometrically validated, it is intended that each measure will be used as a standard tool to gather key endpoint data on future clinical research in each of the respective indications. Icon will also be conducting a formal translatability assessment of the measures to minimize risk in the future translation and linguistic validation process. The process will help to ensure the content is written in such a way that it can be easily translated and linguistically validated in additional languages.

“As the PROs are available on the eCOA system where data is transmitting instantly upon patient completion, the data will be available for assessment in real time,” said Erana.

The PRO instruments are in the final stages of development. Icon’s eCOA system is being developed in parallel. “We are actively recruiting sites and patients to participate in the psychometric validation research stage,” said Erana. “Once the eCOA system has gone through all phases of the software development lifecycle, including design, testing and user acceptance testing, the handheld device and web portal will be available for use in the research study.”

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**PRO endpoints** (continued from page 1)
The Pulse on Technology

By Elisa Cascade

If you build it, they will come.” While this was clearly the case in the baseball film “Field of Dreams,” the same does not hold true for all site-facing clinical trial technology. As the last Pulse on Technology column highlighted, several technologies are still sitting on the bench when it comes to use across all trials. This column takes a closer look at the technology with the lowest awareness and adoption: electronic informed consent (eConsent). To continue the baseball analogy, picture eConsent as a little boy sitting on the bench waiting to get into the game.

When it comes to “readiness to play,” eConsent has many features that could positively impact the efficiency of clinical trial conduct. It improves the consent process for the patient through the use of interactive elements including combinations of video and audio narration, illustrated glossary terms and enabling patients to highlight areas they have questions about for discussions with sites. Research has shown that patients who are presented with eConsent better understand the purpose and process of the trial, which, in turn, correlates with a higher patient retention rate.

eConsent can also benefit sites in a number of ways, such as having the consent written in patient-friendly language, always surfacing the most up-to-date version of the informed consent form, electronically recording the date/time/version of consent and by helping sites use their time with the patient more productively. In addition to ensuring that the patient is presented with a comprehensive description of the informed consent, sites are able to identify where patients are having trouble with the consent language and can focus their discussion more specifically on those areas. Indeed, in the 2016 DrugDev Annual Investigator Survey, the majority of global investigators agreed that eConsent would decrease site burden and be easier to use than paper consent.

Despite these positive attitudes, however, eConsent remains on the bench; only 28% of global sites have used eConsent on at least one study, with 2% of sites reporting use across all studies. And despite the baseball analogy, the low level of experience with eConsent is true across all countries (similar to being on the “subs bench” in a football/soccer match): Argentina, Australia, Brazil, Germany, India, South Africa, the U.S. and the U.K. all reported use by between 20% and 30% of sites. India and South Africa had the lowest levels of experience with eConsent (19% and 5%, respectively).

So why is eConsent still stuck on the bench given its potential to positively improve patient retention rates? Anecdotally, concerns have been raised regarding ethics, site burden and patient acceptability, but when directly asked, “Why have you not used eConsent on all clinical trials,” 77% of global sites responding to the 2016 Annual Investigator Survey reported that they had not been asked by the study sponsor. Less than 15% of sites actually expressed material concerns; 12% said that their local IRB or ethics board did not approve the use, 7% thought that the technology would be unacceptable to patients and 6% thought it would be difficult to incorporate into their site workflow.

It is clear from the research that sites are open to getting eConsent onto the field of play and using the technology. They just need to be asked! Providing education and support to sites should speed the progression from awareness to trial to adoption where the patients, sites and sponsors can all benefit. So the message on eConsent could well be summed up as, “Put me in coach. I’m ready to play today!”

Elisa Cascade is an expert in clinical trial innovation and technology with over 25 years of industry experience. She leads DrugDev’s Data Solutions business working with sponsors, CROs, and sites to improve clinical trial operations through standardization, industry-wide collaboration (including TransCelerate’s Investigator Registry and Investigator Databank), and a beautiful technology experience. Email elisa.cascade@drugdev.com or tweet DrugDev at @drugdevinc.
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<td>FDA expanded approval granted</td>
<td><a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a></td>
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<tr>
<td>Chugai Pharmaceutical</td>
<td>(Alecensa) alectinib hydrochloride</td>
<td>anaplastic lymphoma kinase positive, metastatic non-small cell lung cancer</td>
<td>EU approved</td>
<td><a href="http://www.chugai-pharm.co.jp/english">www.chugai-pharm.co.jp/english</a></td>
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<td>Perrigo</td>
<td>hydrocodone bitartrate and homatropine methylbromide</td>
<td>n/a</td>
<td>FDA approved</td>
<td><a href="http://www.perrigo.com">www.perrigo.com</a></td>
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<td>Otsuka Pharmaceutical, Lundbeck Canada</td>
<td>Rexulti (brexpiprazole)</td>
<td>schizophrenia</td>
<td>Health Canada approved</td>
<td><a href="http://www.otsukacanada.com">www.otsukacanada.com</a></td>
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<td>Royal Philips</td>
<td>IntelliVue Guardian</td>
<td>inpatient continuous monitoring system</td>
<td>FDA approved</td>
<td><a href="http://www.usa.philips.com">www.usa.philips.com</a></td>
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For news on trial results, FDA approvals and drugs in development, Join the LinkedIn Drug Research Updates group!
Gastroenterology

- **Pfizer** and **Celltrion Healthcare** released results of **Inflectra** (infliximab CT-P13) for moderate to severe Crohn's disease (CD). The randomized, double-blind, parallel-group, phase III study compared overall safety and efficacy between Inflectra and Remicade in terms of Crohn's Disease Activity Index (CDAI)-70 response rates. The primary endpoint of the 54-week study was collected at week six to demonstrate that Inflectra is similar to Remicade in the treatment of CD. The clinical trial enrolled 214 patients and met its primary endpoint demonstrating that, at six weeks, Inflectra was similar to Remicade in the treatment of CD, thereby meeting the criterion for non-inferiority. Crohn's Disease Activity Index (CDAI-70), a well-established assessment of treatment response in CD. The response rates, 71.4% for Inflectra and 75.2% for Remicade, were not statistically significantly different. Inflectra is marketed as Inflectra (infliximab-dyyb) in the U.S. and under other brand names in some countries.

Infectious diseases

- **Gilead Sciences** reported 144-week data from two phase III studies (Studies 104 and 111) evaluating the safety and efficacy of **Genvoya** (elvitegravir 150mg, cobicistat 150mg, emtricitabine 200mg and tenofovir alafenamide 10mg) for the treatment of HIV-1 infection in treatment-naive adults. In the combined analysis, a total of 1,733 treatment-naive adults with HIV were randomized to receive either Genvoya or Stribild. At week 144, 84.2% (n=729/866) of patients taking Genvoya and 80% (n=694/867; 95% CI: 0.6% to 7.8%, p=0.021) of patients taking Stribild achieved HIV-1 RNA levels less than 50 copies/mL. Additionally, at week 144, 81.1% (n=702/866) of patients taking Genvoya and 75.8% (n=657/867; 95% CI: 1.5 to 9.2%, p=0.006) of patients taking Stribild achieved HIV-1 RNA levels less than 20 copies/mL, a secondary endpoint. At week 144, virologic failure was similar between groups (Genvoya, 4.6%; Stribild, 3.9%); the difference in overall results was driven by fewer discontinuations on Genvoya due to adverse events or other reasons not related to efficacy (Genvoya, 11.2%; Stribild, 16%). There were statistically significant fewer adverse events leading to discontinuation in the Genvoya arm compared to the Stribild arm (Genvoya, 1.3%; Stribild, 3.3%, p=0.01). The most common drug-related adverse events in both groups were nausea, diarrhea and headache.

Oncology

- **OncoGenex Pharmaceuticals** reported results of **apatorsen** in two randomized phase II clinical trials for bladder and prostate cancer. The Borealis-2 trial evaluated apatorsen in combination with docetaxel treatment in 200 patients with metastatic bladder cancer whose disease had progressed following first-line platinum-based chemotherapy. Patients who received apatorsen treatment experienced a 20% reduction in risk of death, compared to patients receiving docetaxel alone (overall survival hazard ratio (HR)=0.80; 80% CI: 0.65-0.98; p=0.078). Partial or complete responses occurred in 16.2% patients receiving apatorsen plus docetaxel compared to 10.9% patients receiving docetaxel alone with median response durations of 6.2 months versus 4.4 months, respectively. Higher baseline serum heat shock protein 27 (Hsp27) levels were significantly prognostic for indicating an almost two-fold higher risk of death (HR=1.96; p=0.0001). In an exploratory analysis on a subset of patients (20% of total) who completed at least two treatment cycles and had either a decrease in serum Hsp27 levels from baseline or had only a 20.5% increase in serum Hsp27 levels from baseline, the reduction in risk of death with apatorsen treatment was 71% (HR=0.29: 80% CI: 0.18-0.48; interaction p=0.0727). The Pacific trial evaluated the ability of apatorsen, when added to Zytiga (abiraterone acetate), to reverse or delay treatment resistance in 72 men who were experiencing a rising PSA on Zytiga alone. The primary endpoint evaluated the proportion of patients who were progression free (clinical and radiologic) at study day 60 with apatorsen added to Zytiga, compared to continuing Zytiga alone. In men receiving apatorsen, 33% were progression free at study day 60 compared to 17% for those men receiving Zytiga alone. For patients with five circulating tumor cells (CTCs) at baseline, 22% vs 11% of patients had a CTC reduction to less than five CTCs when apatorsen was added to Zytiga vs Zytiga alone, respectively. Clinical data from trials in bladder and prostate cancers demonstrated apatorsen was well-tolerated and improved patient outcomes when administered in combination with standard-of-care treatments.
Drug reimportation into the U.S. is gathering steam in Congress. But an act of Congress isn’t needed to make it happen. A trio of senators have reminded Tom Price, the new secretary of the HHS, that he has only to say the word and then the FDA can exercise the authority it already has to allow pharmacists and wholesale retailers to import cheaper prescription drugs from Canada. The agency could also grant waivers permitting individual patients to import drugs for personal use. Price should immediately consider making such a certification, the senators said, when a drug: is off patent or no longer marketed in the U.S. by the company that developed it; has significant and unexplained price increases; has no direct competition in the market; and is produced in another country by the brand manufacturer that developed it or by a well-known generic manufacturer that commonly sells prescription drugs in the U.S. The senators noted that in many instances, cheaper Canadian drugs are made by a reputable generic company or by the same manufacturer as the pricier one sold in the U.S. market. The senators also asked Price to consider a fast track approval process for imported drugs. “Fast track approval is key,” the lawmakers said in a letter to the HHS secretary, “because regulatory costs can deter market competition.”

Imminent changes to Taiwan’s drug approval process could dramatically improve the country’s pharmaceutical sector, potentially shortening approval times and boosting growth. Drug developers operating in the country are paying close attention to upcoming reforms, which should be implemented soon. In keeping with President Tsai Ing-wen’s five-plus-two innovative industries initiative, which favors the biomedical sector, the government in Taiwan recently posted a number of policies, such as the inclusion of regenerative medicine and precision medicine in the Act for the Development of Biotech and New Pharmaceuticals Industry. This month, Executive Yuan also passed draft regulations governing the establishment of the National Centre for Drug Evaluation, designed to improve the drug review process without reliance on government funding. The new national agency, which would provide guidance to drug manufacturers and expedite the launch of new drugs by reducing the drug approval time frame by one to two months, is a key component of the government’s plans to strengthen the attractiveness of Taiwan’s patented medicine market.

Science may be in the business of answering questions, but medicine, agriculture and policy based on scientific findings remain uncertain business. Those uncertainties do not always show up as spectacularly as they did in the 2016 presidential election. But in decision-making based on scientific facts, those facts go through the filter of human cognition—which tends to think about risk in ways that are not just about statistics, and can, in fact, be at odds with them. In a wide-ranging talk about the promise and peril of biotechnology, 2009 Nobel Prize winner and Royal Society President Venkatraman Ramakrishnan told the audience at the American Association for the Advancement of Science meeting that, in general, humans are “particularly sensitive to catastrophic risk,” which is one reason that many people are more frightened of terrorism than car accidents. “We are less worried about risk associated with gradual change,” which is rational under some conditions and not under others.

The nomination of Seema Verma to the administrator of the Centers for Medicare & Medicaid Services (CMS) came with many of the typical tough questions and a concomitant reluctance to answer directly at times, but Verma’s answers often reverted to an emphasis on choice and competition, including on the question of negotiated drug prices. Sen. Orrin Hatch (R-Utah), chairman of the Senate Finance Committee, commenced the hearing with the remark that “the failings of Obamacare are urgent and must be addressed in short order,” noting that another major carrier, Humana, has indicated it will leave the health insurance exchanges next year. Ranking member Ron Wyden (D-Ore.) made a different case, stating that a rule proposed on February 15 by CMS—which would expand the period for pre-enrollment verification and allow insurers to collect back premiums—sends a message. “The message is insurance companies are back in charge,” and patients “will take a back seat,” Wyden said. Wyden inquired as to what sort of administrative changes Verma would consider in getting a handle on costs associated with Medicare Part D, the prescription drug plan, and Verma responded that she would favor policies “that continue to put senior citizens in charge of their healthcare.”

It is a tumultuous year for Korean conglomerate Samsung, whose group chief Jay Y. Lee was arrested Friday in connection to a corruption scandal, and Samsung Biologics, the company’s contract manufacturer arm of biologic drugs, which come under scrutiny with allegations of being involved in unlawful accounting practices. The issue with Samsung Biologics was put under the spotlight when Sim Sang-jeung, chairwoman of the minor Justice Party in South Korea, started questioning accounting methods that seemed to boost Samsung Biologics’ net profits. That practice, in turn, boosted market valuations when the firm was seeking a public listing in Seoul. Samsung Biologics has been unprofitable since it was founded in 2011, but in 2015 the firm suddenly reported a net profit of KRW900 billion ($1.66 billion). The sudden profit was created by a revaluation of its stake in its subsidiary, Samsung Bioepis.

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