

July 17, 2017

## Innovation Initiative to modernize the FDA

By Karyn Korieth

**N**ew FDA Commissioner Scott Gottlieb intends to release a far-reaching plan aimed at modernizing regulatory processes and speeding approvals for new drugs and devices, which could allow greater patient access to new medicines through unprecedented scientific and technological advancements.

Key elements of the plan, called the Innovation Initiative, include using computer models and virtual patients to develop and evaluate new drugs or devices, particularly those targeting personalized medicine and rare disease populations, and creating new regulatory standards to apply expedited pathways for regenerative medicine products. The FDA will also move forward with guidance in areas including novel clinical trial

design and use of real-world evidence in drug approvals, which has been made possible through recently developed digital technologies.

Gottlieb, who announced the new initiative in a blog post, said the plan builds on the 21st Century Cures Act, which made substantial changes to FDA regulations and gave the agency new powers to speed approval of drugs. The commissioner said goals of the Innovation Initiative include ensuring regulatory principles are “modern and efficient” and incorporate the “most up to date” scientific knowledge.

“We are at a point in science where new medical technologies hold out the promise of better treatments for a widening number of vexing conditions,” Gottlieb said. “We don’t want to

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## IndieBio, Science Exchange partnership aims to accelerate R&D science

By John W Mitchell

**I**n a move sure to make scientists happier at the prospect of less administrative burden, two companies—one that accelerates pharma and biotech research and another that figures out the research details—are joining forces.

**Science Exchange** and **IndieBio** last week announced a strategic partnership. The joint effort plans to bring new disease cures to market faster, cheaper and most certainly with less operating aggravation for pharma and biotech startups. IndieBio-backed startups plan to access Science Exchange’s network of 2,500 pre-contracted CROs to more quickly outsource much of the “nuts and bolts” research key to an innova-

tion launch.

“Most great scientific discoveries never leave the lab because they are not in the direct interest of pharma companies,” Jun Axup, Ph.D., science director of IndieBio, told **CenterWatch**. “But today, with the lowering cost of R&D and accessibility of laboratory resources, it’s becoming more and more possible to build biotech startups from the ground up.”

According to Dr. Axup, the greatest advantage startups have are focus and execution, but they are limited in funding, time, resources and sometimes specialized expertise. Cost-efficient, proven outsourcing, she said, can help alleviate such restraints. This

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## Industry Briefs

### CROs/Service Providers

► **Quanticate** has completed an investment initiative to upgrade its pharmacovigilance safety database and its in-house technology platform in preparation for the upcoming Article 2(3) of Directive 2010/84/EU that will impact the pharmaceutical industry later this year. The data-focused CRO has made the move following the **EMA's** planned launch of an improved version of EudraVigilance, the European information system for suspected adverse events to medicines in the European Economic Area, on November 22, 2017. The new system will simplify adverse event reporting in the EU and enhance data availability to stakeholders for improved signal detection activities. The capability expansion enables Quanticate to offer pharmaceutical clients direct and immediate access to a fully compliant safety database without them needing to invest in their own system or use an interim solution to ensure compliance.

► **Frenova Renal Research**, a drug and medical device contract clinical development services provider dedicated exclusively to renal research, has announced that its F1RST Up alliance of clinical research sites focusing on kidney disease has expanded to include 55 physician investigators representing 23 sites. The F1RST Up alliance membership includes nephrology practices caring for large numbers of chronic kidney disease (CKD) patients, including end stage renal disease (ESRD) patients. F1RST Up investigators are experienced clinical researchers in renal disease and its adjacencies, including vascular access and care, cardio-renal conditions, rare diseases, infectious diseases and transplant. The alliance leverages Frenova's access to proprietary clinical research assets to inform protocol design and to carry out highly targeted and efficient patient recruitment efforts.

► **INC Research Holdings**, a global phase I to IV CRO, has announced the launch of the com-

pany's Psychiatry Catalyst Site Network as part of its Catalyst program introduced in 2016 to strengthen collaborations with clinical research sites worldwide. The ultimate goal of the program is to enhance patient focus and optimize study delivery to drive improved predictability and increased efficiency for customers. INC selected 32 high-performing clinical research sites to participate in the initial launch of the Psychiatry Catalyst Site Network, which was formed to respond to the rapid growth and strong pipeline of psychiatric studies. The Psychiatry Catalyst Site Network joins INC's existing networks in place in support of oncology and vaccine clinical research. Sites selected for the Psychiatric Catalyst Site network will work with each other and with INC Research to deliver studies more quickly and more predictably, enabling the company to better deliver on the needs of its customers.

### R&D Trends

► Fueled by a desire for innovation and new medical breakthroughs, the biotech industry continues to enjoy substantial growth due to increased investment in R&D, according to the sixth annual study from **BDO USA**. The 2017 BDO Biotech Briefing, which examines the most recent 10-K SEC filings of publicly traded companies included in the NASDAQ Biotechnology Index, found that the average R&D spending across all mid-market biotech companies increased about 18% from 2015 to 2016, from an average of \$65.9 million to an average of \$80.6 million. The uptick in R&D spending is one factor that can explain the recent surge of innovation across all areas of the biotech industry, including

significant advances in biological sciences and pharmaceuticals, and the expansion of more effective drugs and curative and preventive treatments aimed at enhancing the quality of human life. In particular, a rising incidence of chronic illnesses due to an increasingly aging population continues to drive more advancements in drug products targeted at oncology and gene therapy to prevent and treat chronic illnesses. Recent legislative developments—such as the 21st Century Cures Act, aimed at streamlining **FDA** approval processes—will help speed up product development, and will potentially support novel innovation that can deliver efficacious drug therapies in the sector.

► **Daiichi Sankyo, Max Planck Innovation** and the **Lead Discovery Center** have signed an agreement providing Daiichi Sankyo with the option to receive the exclusive rights to a new lead compound for the treatment of cancer to be discovered and developed at the Lead Discovery Center. This new partnership builds on biology insights in the field of transcriptional regulation from the work of Matthias Geyer at the Max Planck Institute of Molecular Physiology in Dortmund, Germany, and the Research Center caesar (**Center of Advanced European Studies and Research**) in Bonn, Germany.

### Drug Sponsors

► **Enterome**, a pioneer of innovative therapies for microbiome-related diseases, and **Nestlé Health Science**, announced the joint creation of **Microbiome Diagnostics Partners** (MDP).

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## Industry Briefs (continued from page 2)

This company will bring together complementary platforms and capabilities enabling the discovery and development of innovative diagnostics through to commercialization in multiple disease areas, including inflammatory bowel diseases (IBD) and liver diseases. MDP will be a 50/50 joint venture between Enterome and Nestlé Health Science. Enterome will contribute its current microbiome diagnostic programs and intellectual property (IP) derived from its unique microbiome-based platform for the development of diagnostics in all therapeutic areas except immuno-oncology. **Prometheus Laboratories** (a Nestlé Health Science Company) will contribute its diagnostics development and commercialization expertise.

### Technology Solutions

▶ The **EQT VII** fund has agreed to acquire **Certara**, a provider of technology-driven decision support solutions for drug development, for an enterprise value of \$850 million. The company is being acquired from **Arsenal Capital Partners**. As part of the transaction, Arsenal Capital Partners will retain a minority ownership stake in Certara, with the company's current management team, led by Edmundo Muniz, M.D., Ph.D., continuing to lead the organization, building on a multi-year track record of both organic growth and strategic acquisitions. Certara is a provider of model-informed drug development technology and services, as well as a best-in-class provider of regulatory science, writing and submission management software and ser-

vices. Certara's solutions help inform the drug development and regulatory approval process and address the key efficacy, safety, productivity and commercial challenges facing the biopharma industry. The company serves 1,200 commercial companies, 250 academic institutions and numerous regulatory agencies, across 60 countries. Certara is headquartered in Princeton, New Jersey, with over 500 employees globally, including key operations and senior management in Northern Europe.

▶ **CRF Health**, a global provider of patient-centered eSource technology and service solutions for the life sciences industry, has collaborated with a research group led by Professor Ludwig Kappos at the **University Hospital Basel** (UHB) to develop an electronic implementation of the Neurostatus-Expanded Disability Status Scale (Neurostatus-EDSS), an established assessment and documentation tool for multiple sclerosis (MS) clinical trials. The newly developed electronic version of the Neurostatus-EDSS replaces the traditional paper forms that suffer from the typical associated issues, such as data inaccuracies and administration costs. Its ability to streamline data collection via an electronic form facilitates the capture of higher quality data and enables fast access to consistent data for further analysis. The new electronic version allows EDSS raters to review and track any inconsistencies in data entries and assessments at the click of a button.

▶ **Tempus**, a technology company focused on helping doctors personalize cancer care

by collecting, sorting and analyzing clinical and molecular data, will provide Moores Cancer Center at **UC San Diego Health** with molecular sequencing, analysis and decision support tools to enable further research on immune checkpoint inhibitors across cancer types. Moores Cancer Center is one of only 47 **NCI**-designated Comprehensive Cancer Centers in the U.S. The center is committed to innovation, and is developing and testing the effectiveness of new therapies and medical devices in fields including personalized therapy, immunotherapy, minimally invasive surgeries and more. Tempus provides molecular sequencing and clinical analytic solutions to top academic centers, hospital systems, associations and healthcare providers.

▶ **Exscientia**, an artificial intelligence (AI)-driven drug discovery company, has entered into a strategic drug discovery collaboration with **GlaxoSmithKline** (GSK). During this collaboration, Exscientia will apply its AI-enabled platform and combine it with the expertise of GSK, in order to discover novel and selective small molecules for up to 10 disease-related targets, nominated by GSK across multiple therapeutic areas. Exscientia will receive research payments from GSK to undertake new discovery programs with nominated targets with the goal of delivering preclinical candidates. In addition to research funding, Exscientia is eligible to receive near-term lead and preclinical candidate milestones. The total amount payable by GSK to Exscientia on achieving these milestones is £33 million, if all 10 projects are advanced. 



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## Innovation Initiative (continued from page 1)

present regulatory barriers to the beneficial new medical innovations that add to the time, cost and uncertainty of bringing these technologies forward if they don't add to our understanding of the product's safety and benefits."

The Innovation Initiative was revealed as the FDA faces a central challenge of balancing regulations that promote speed and innovation of new drugs with the need for comprehensive benefit/risk data to ensure patient safety. Many industry groups express optimism about the agency's recent steps to accelerate drug development and approval processes, yet the industry doesn't support a less rigorous FDA review process that could put patients at risk and make it harder to convince physicians and insurance companies that new treatments delivered value.

"We are fully confident that the FDA's gold-standard commitment to safe and efficacious products will be maintained, even as the agency deploys new technologies, such as modeling and simulation, to advance products in development through the stages of clinical trials more efficiently," said Doug Peddicord, Ph.D., executive director of [Association of Clinical Research Organizations](#) (ACRO). "We look forward to new and updated guidance about the use of such 'in silico' tools across the spectrum of product development"

Industry groups and experts also praised the new commissioner's commitment to support and broaden the Cures Act, which was enacted in December 2016 during the Obama administration. The Cures Act includes requirements for the FDA to develop a new framework for incorporating patient perspectives into the drug development process and the use of real-world evidence in the approval of new uses for existing drugs. In his blog post, Gottlieb announced that the final work plan for steps to implement different aspects of the Cures Act has been posted.

"Since the 21st Century Cures Act was enacted in the final days of the Obama Administration, one might worry that the Trump Administration would not embrace it. But that is simply not the case. The FDA seems to be implementing it with

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**"The FDA is enthusiastically embracing the idea embedded in the legislation."**

—Bradley Merrill Thompson

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zeal. And indeed, that's the benefit of having produced the 21st Century Cures Act in a bipartisan manner," said Bradley Merrill Thompson, a lawyer with the firm [Epstein Becker & Green](#), who specializes in FDA and clinical trial issues. "The FDA is enthusiastically embracing the idea embedded in the legislation that the FDA has a responsibility to ensure that innovation flourishes. I really have to applaud the FDA for this initiative."

A key way that the Innovation Initiative proposes to modernize processes is through new FDA guidance regarding the use of computer modeling and simulation in clinical trials to improve the efficiency of both drug development and regulatory review. Computer models can help narrow possible drug designs, predict product safety, evaluate potential adverse event mechanisms, inform alternate study designs and help advance new therapies more quickly through the different clinical trial phases.

The FDA has begun to use computer modeling to build natural history databases, Gottlieb said, that could allow the FDA to better evaluate new therapies for Parkinson's disease, Huntington's disease, Alzheimer's disease and muscular dystrophy. The agency has also begun to develop a "family of virtual patients" for testing new devices and is investing in high-performance computing capabilities to allow review staff to manage the large data sets that have become a common element of drug applications.

"I am very glad to see that the FDA is investing in upgrades to its IT infrastructure that will allow it to make maximum use of some of the software analytics that will help the FDA spot potential safety risks, but also do so quickly and with the least burden on industry applicants," said Thompson. "We've made great strides in computer modeling and simulation the folks in industry

have been now using for a while. Putting the FDA in the position to likewise use those techniques is a real step forward that will both enhance safety and lessen the regulatory burden."

As part of the Innovation Initiative, in September the FDA will announce a comprehensive framework for development and oversight of regenerative medicine. The agency recently implemented the **Regenerative Medicine Advanced Therapy** (RMAT) designation, which provides an additional pathway to access expedited programs for certain cell and therapeutic tissue engineering therapies. The new effort will include a series of guidance documents that outline regulatory aspects governing regenerative medicine, including policies for demonstrating safety and effectiveness of these novel products.

The [Alliance for Regenerative Medicine](#) (ARM), the international advocacy organization representing the gene and cellular therapies and broader regenerative medicine sector, commended the FDA's new initiative. ARM had previously expressed concerns about proposals that sought to change approval pathways for regenerative medicine products by reducing or, in some cases, eliminating the FDA's regulatory authority, which could have allowed untested and unproven treatments to reach the market.

"We're very enthusiastic about the regenerative medicine-specific provisions in this announcement, especially the RMAT designation, as it's specifically designed, for the first time in the U.S., to apply expedited pathways to regenerative medicine products. Its implementation will ensure that safe and effective products reach patients in need as quickly as possible," said Michael Werner, ARM's executive director and head of legislative activities.

"We look forward to reviewing the details of Commissioner Gottlieb's Innovation Initiative once they emerge," said Sara Radcliffe, president and CEO of California **Life Sciences Association**. "The FDA's work heavily impacts the pace at which innovation is driven in California's life sciences industry, as well as the delivery of life-changing medicines and technologies far beyond the state of California." 

## R&D (continued from page 1)

allows the startup to focus on what they do best—bringing their scientific innovations and breakthrough discoveries to market.

IndieBio bills itself as the world's largest life sciences accelerator, with nearly 70 funded startups in a little more than two years. Their platform offers companies around the world \$250,000 in seed money, a four-month acceleration program, dedicated mentorship and access to co-working and bio-safety level one and two labs.

These emerging companies are schooled in how to turn their science into products, learning skills like closing customers and raising follow-up investments. All the while, such startups are typically tightly staffed and stretched thin. This is where Science Exchange can step in and level the playing field.

"Now, an IndieBio company does not need to invest resources into sourcing, qualifying and contracting scientific service providers for their outsourced R&D, saving them significant time and cost," Elizabeth Iorns, Ph.D., founder and CEO of Science Exchange, said in an interview with CenterWatch. "In the last decade, outsourcing of R&D has become a core strategy for improving efficiency and providing access to innovation for top biopharmaceutical companies globally."

Dr. Iorns said that 40% of R&D spend is now outsourced and increasing. She noted that the shift in R&D spending to external providers has resulted in over 10,000 contracted CROs, as well as more than 1,000 contract manufacturers. It can be

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—Elizabeth Iorns, Ph.D., founder and CEO, Science Exchange

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an imperfect market for startups with no internal expertise or experience in contracting for services. She cited several points of "friction" that her company works to resolve, including:

- ▶ **Provider discovery:** Hard-to-identify providers that are qualified, available and cost-effective.
- ▶ **Price discovery:** Difficult to benchmark fair cost of projects.
- ▶ **Risk/trust:** Significant IP, confidentiality and compliance risk associated with outsourcing R&D.
- ▶ **Time delays:** Delays of two-plus months per provider due to qualification, contracting and setup for payment.
- ▶ **Supplier management:** Hard to track progress and managing ongoing, outsourced R&D projects and provider performance.

Also, Science Exchange, and companies similar to it, provides a platform to address many of the mundane tasks of running a business. This includes vendor contract-

ing, auditing, research ethics and accounts payable.

Dr. Axup noted that early startups are in a constant race against time. Most funding rounds last only 18 to 24 months and the money needs to be dedicated to pushing their product forward.

"Bringing everything in-house is often not as cost- and time-effective as outsourcing to specialized vendors," she said. "Furthermore, it allows the company to reduce overhead costs, parallelize R&D efforts and focus on scaling their product. Across pharma and biotech, we are seeing a trend toward outsourcing to increase productivity."

Dr. Axup added that Science Exchange helps nurture and manage R&D relationships by providing only verified vendors, as well as with communication transparency. This allows new biotech companies to compare the offerings of different vendors easily. Finally, confidential materials are protected, so the startup has more confidence about outsourcing their science.

"Science Exchange ... understands that the needs and timelines of an early startup are very different," said Dr. Axup. "While our partnership is non-exclusive, I believe our companies will find immense value in accessing Science Exchange's platform at their current stage and as they grow into larger companies."

With a focus on biology as a technology, IndieBio companies solve problems in a wide range of industries. This includes the future of food, biopharma and healthcare, agtech, regenerative medicine, neurotech, biomaterials and other fields. 

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# The Pulse on Site Success

By Dr. Christophe Berthoux



Increasing participation in clinical trials has long been a topic of discussion in the industry, and obstacles related to regulatory concerns, culture and communication are known to hinder outreach to the patient community. Fortunately, the focus on meaningful engagement has led to new ideas and opportunities to create dialogue between patients and professionals.

The use of technology has risen to the forefront as a direct method of interacting with patients. Implementing a patient portal that allows participants to log in and view a visit schedule, request appointment changes or access resources related to a study can support subject compliance by reminding patients of upcoming visits and providing a window of time to make changes. Automating this process, whether through a patient portal or a subscription service, will usually decrease some administrative burden for site staff, allowing more time to focus on providing care.

The process of engagement is not limited to interaction with study specific participants. Research sites can utilize their website as a tool to provide content aimed at the patient community. Providing valuable resources can help establish credibility and build trust with the patient. Implementing a call to action on the website can encourage patients to opt-in to receive additional communication. This provides an opportunity for sites to deliver custom content directly to their inbox related to clinical research education and potential study opportunities. Sites can also utilize their email database to occasionally survey potential patients about issues or to gain understanding of the community perception of clinical research.

Social media offers a platform that allows for efficient, two-way engagement between sites and patients, and also serves as a means to share relevant content. According to the 2015 CISCRP Perceptions and Insights Study, 22% of respondents indicated social media and online resources as sources for information about clinical research. As more sites build their online presence, it is likely this number will increase. Most social platforms offer considerable opportunities

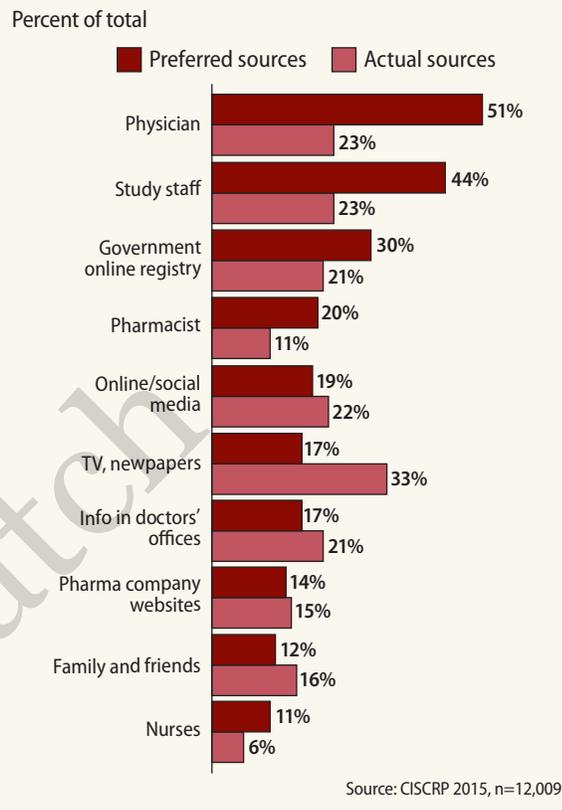
to grow an audience through paid or organic content. Facebook is known for advanced targeting, and can be particularly useful when looking to reach patients with specific interests or located in certain geographic areas.

Engaging with advocacy groups and online forums related to a range of therapeutic indications offers an opportunity for sites to connect with patients and gain valuable insights. Feedback provided by patients can help sites determine areas for improvement and ultimately enrich the patient experience for future studies. Online forums are an effective, low-cost option that can be utilized by multiple site members.

Patient engagement is not limited to technology, however, and the majority of patients prefer to receive information about clinical research from study staff or their primary care provider (See figure). Sites can explore within their town or city for opportunities to engage the local community. Many events will allow sites to set up a table or booth and interact with attendees. Sites can visit public events to share resources and educate the community, and may be able to encourage attendees to opt-in for further email communication for continued engagement. In addition to attending public events, a site could elect to organize a QA session or host a public forum, where people may come to learn more about clinical research.

Establishing a patient advisory panel that meets on a regular schedule will foster a collaborative environment among researchers, patients and caregivers. While online surveys are an effective tool to gauge the response of a large audience, smaller patient groups or councils can lead to more in-depth discussions about patient perception. This enables the site to focus on areas most relevant to their own patient community, and can also serve as a resource

## Preferred and actual sources for information about clinical research studies



for evaluating what content to share with the community at large.

The research community is aware that in order to advance new medicines, methods to bridge the gap between patients and the industry must be explored. With the most direct access to patients, research sites have the ability to come up with creative ideas and experiment with different approaches to establish meaningful dialogue with the patient community. It is no secret that putting the patient at the center will ultimately lead to more successful study results, and increased participation overall.

*Dr. Christophe Berthoux has been the chief executive officer at Synexus since September 2010. Synexus is the world's leading site management organization (SMO), dedicated to the recruitment and management of clinical trials across the globe for over 24 years. Synexus is proud to be the patient's choice for clinical research. Email comments and questions to sarah@vanepercy.com.*

# Drug & Device Pipeline News

 For news on trial results, FDA approvals and drugs in development, Join the LinkedIn Drug Research Updates group!

Company	Drug/Device	Medical Condition	Status	Sponsor Contact
ArQule	ARQ 531	B-cell malignancies	Phase I trials initiated enrolling 120 subjects	www.arqule.com
Prima BioMed	IMP321 (LAG-3lg fusion protein)	advanced stage solid tumor entities	Phase I trials planned enrolling 40 subjects	www.primabiomed.com.au
Valerion Therapeutics	VAL-1221	late-onset Pompe disease	Phase I/II trials initiated	www.valerion.com
BioLineRx	BL-8040, atezolizumab	metastatic pancreatic ductal adenocarcinoma	Phase Ib/II trials initiated enrolling 40 subjects	www.biolinerx.com
Prexton Therapeutics	Foliglurax	Parkinson's disease	Phase II trials initiated enrolling 165 subjects in the U.K., Germany, France, Austria, Spain and Italy	www.prextontherapeutics.com
Prothena	PRX002/RG7935	early Parkinson's disease	Phase II trials initiated enrolling 300 subjects	www.prothena.com
BeyondSpring	plinabulin	neutropenia	Phase II/III trials planned in China	www.beyondspringpharma.com
Alnylam Pharmaceuticals, Sanofi Genzyme	fitusiran	hemophilia A and B	Phase III trials initiated	www.alnylam.com www.sanofigenzyme.com
Exelixis, Bristol-Myers Squibb	Opdivo (nivolumab), Cabometyx (cabozantinib)	previously untreated, advanced or metastatic renal cell carcinoma	Phase III trials initiated	www.bms.com
Oramed Pharmaceuticals	ORMD-0801	type 2 diabetes	Phase III trials planned	www.ored.com
Resverlogix	apabetalone	cardiovascular disease, type 2 diabetes mellitus	Phase III trials initiated in Taiwan and China	www.resverlogix.com
Advicenne	ADV7103	distal Renal Tubular Acidosis	Orphan Drug designation granted by the EU	www.advicenne.com/en
Almac Discovery	ALM201	ovarian cancer	Orphan Drug designation granted by the FDA	www.almacgroup.com
Autifony Therapeutics	AUT00206	Fragile X Syndrome	Orphan Drug designation granted by the FDA	www.autifony.com
Biohaven Pharmaceutical	BHV-5000	Rett syndrome	Orphan Drug designation granted by the FDA	www.biohavenpharma.com
Boston Biomedical	DSP-7888	myelodysplastic syndrome	Orphan Drug designation granted by the FDA	www.bostonbiomedical.com
Horama	ORA-RLBP1	retinitis punctata albescens	Orphan Drug designation granted by the FDA	www.horama.fr
Ignyta	entrectinib	NTRK fusion-positive solid tumors	Orphan Drug designation granted by the FDA	www.ignyta.com
Reata Pharmaceuticals	bardoxolone methyl	Alport syndrome	Orphan Drug designation granted by the FDA	info@reatapharma.com www.reatapharma.com
Sobi	SOBI003	mucopolysaccharidosis type IIIA	Orphan Drug designation granted by the FDA	www.sobi.com
Principia Biopharma	PRN1008	pemphigus vulgaris	Orphan Drug designation granted by the FDA	info@principiabio.com www.principiabio.com
Eli Lilly	abemaciclib	hormone-receptor-positive, human epidermal growth factor receptor 2-negative advanced breast cancer	Priority Review granted by the FDA	www.lilly.com
Clinigen Group, Cumberland Pharmaceuticals	Totect (dexrazoxane hydrochloride)	reverse toxic effects of anthracycline chemotherapy in case of extravasation	FDA approved	www.clinigengroup.com www.cumberlandpharma.com
Emmaus Life Sciences	Endari (L-glutamine oral powder)	severe complications of sickle cell disease	FDA approved	info@emmausmedical.com www.emmauslifesciences.com

## Trial Results

### Gastroenterology

► **Allergan** reported results of two phase III studies (IBS-3001 and IBS-3002), which evaluated the efficacy and safety of **Viberzi** (eluxadoline) in nearly 2,500 irritable bowel syndrome with diarrhea (IBS-D) patients, of whom 36% reported use of loperamide in the 12 months prior to study randomization. Data demonstrate Viberzi safely and effectively treats the IBS-D symptoms of abdominal pain and diarrhea irrespective of prior use of loperamide. These analyses also demonstrated efficacy and safety in patients with loperamide rescue medication use compared to the overall population during the two studies. Among patients who reported prior loperamide use with inadequate symptom control, a significantly greater proportion of patients treated with eluxadoline were composite responders over weeks one to 12 compared with those treated with placebo (eluxadoline 100mg: 27% ( $P < 0.001$ ) and 75mg: 26.3% ( $p = 0.001$ ) versus placebo 12.7%). The most commonly reported adverse events (AEs) are consistent with the known safety profile of eluxadoline, which were nausea, abdominal pain, constipation and headache.

### Genetic diseases

► **Prometic Life Sciences** issued results of a phase II/III trial of **Ryplazym** (Plasminogen IV) in patients with congenital plasminogen deficiency. Prometic has previously reported data from this pivotal trial, which showed that Ryplazym treatment consistently replaced and maintained the plasminogen

concentration at an appropriate level and that it resolved all lesions in all 10 patients treated for 12 weeks. These data fulfilled the clinical information required for the Biologics License Application (BLA) filing with the FDA for the Accelerated Regulatory Pathway Approval. Under the same pivotal phase II/III protocol, these 10 patients have been treated for an additional 36 weeks, for a total drug exposure period of 48 weeks. In addition to the dossier filed with the FDA, the 48-week clinical efficacy data will form the basis for the upcoming regulatory filing with Health Canada in the fourth quarter of 2017. The Ryplazym trial is still underway, and the dataset for all 15 patients that will have been treated over a period of 48 weeks is expected to be an acceptable demonstration of the efficacy of plasminogen therapy in patients with plasminogen congenital deficiency for full licensure, without the need for any additional clinical trials. The data demonstrates that in 10 patients treated with Ryplazym for a total of 48 weeks, there was no recurrence of lesions and no safety or tolerability issues observed related to this longer-term dosing.

### Oncology

► **Amgen** reported positive results from the final analysis of the phase III ASPIRE trial. The international, randomized ASPIRE (CArifilzomib, Lenalidomide, and Dexamethasone versus Lenalidomide and Dexamethasone for the treatment of Patients with Relapsed Multiple Myeloma) trial evaluated **KYPROLIS** in combination with lenalidomide and dexamethasone

versus lenalidomide and dexamethasone alone in patients with relapsed multiple myeloma, following treatment with one to three prior regimens. The primary endpoint of the trial was PFS, defined as the time from treatment initiation to disease progression or death. Secondary endpoints included OS, overall response rate (ORR), duration of response (DOR), disease control rate, health-related quality of life (HR-QoL) and safety. Patients were randomized to receive KYPROLIS (20mg/m<sup>2</sup> on days one and two of cycle one, escalating to 27mg/m<sup>2</sup> on days eight, nine, 15 and 16 of cycle one and continuing on days one, two, eight, nine, 15 and 16 of subsequent cycles), in addition to a standard dosing schedule of lenalidomide (25mg per day for 21 days on, seven days off) and low-dose dexamethasone (40mg per week in four-week cycles), versus lenalidomide and low-dose dexamethasone alone. The study randomized 792 patients at sites in North America, Europe and Israel. The study met the key secondary endpoint of overall survival (OS), demonstrating that KYPROLIS (carfilzomib), lenalidomide and dexamethasone (KRd) reduced the risk of death by 21% over lenalidomide and dexamethasone alone (Rd) (median OS 48.3 months for KRd versus 40.4 months for Rd, HR=0.79, 95% CI, 0.67-0.95). Per protocol, patients received 18 cycles of KYPROLIS with Rd before continuing treatment with Rd alone to progression. This KRd regimen of twice-weekly KYPROLIS administered at 27mg/m<sup>2</sup> is currently approved in the U.S., European Union and other countries based on the primary analysis of progression-free survival (PFS) in the ASPIRE study. 

# 2018 Top Innovators

Innovation is crucial in the long, complicated drug development process. Nominate or submit an innovation you or your organization is working or collaborating on that will drive (or change) the industry into the next decade and beyond.

The January 2018 issue of *The CenterWatch Monthly* will showcase up to 20 new innovations and the people involved.

Submit an Innovation Entry by October 15, 2017

Visit [centerwatch.com/news-resources/top-innovators.aspx](http://centerwatch.com/news-resources/top-innovators.aspx) for further details and online submission form.

The CenterWatch Monthly

April 2018 A CenterWatch Publication Volume 25, Issue 04



20 innovators  
changing the face of the clinical trials industry

Profile by Kerry Austin  
The CenterWatch Monthly makes it with you: the drug development and companion for their forward thinking  
Other innovators work to improve patient care in the system and success about the

## Biotech Review

▶ The pharma industry in the U.K. is turning to the courts to prevent a new “budget impact” test from being used to bring down drug prices. The test, introduced on April 1, means that any drug determined by the health technology assessment body **NICE** as being cost-effective will nonetheless automatically be referred for “commercial discussions” to reduce the price if it will cost more than £20 million (\$25.7 million) per annum in any of the first three years. The **Association of the British Pharmaceutical Industry** (ABPI) and the **Bioindustry Association** objected when the test was announced. Now, after prolonged backroom exchanges, ABPI said it has “reluctantly” applied for a judicial review on the grounds that the budget impact test breaches the **National Health Service** (NHS) constitution. “After many months of raising concerns with NICE, NHS England and the **Department of Health**, and offering to work constructively on alternative proposals, we have applied to formally challenge these proposals in court,” said Mike Thompson, ABPI chief executive.

▶ India’s **Central Drugs Standard Control Organization** (CDSCO) has been streamlining regulations to standardize its rules on the market for pharmaceuticals. “There have been many changes done not just to the Drugs and Cosmetics Rules, but a lot of other related regulations over the past few years,” Abhishek Chawla, an officer at CDSCO, told *BioWorld*. “The government wants to make it easier for both domestic firms and multinational companies to operate in the country. “A new version of the Drug and Cosmetics Rule is currently being laid out by lawmakers and will be presented to parliament sometime in the future,” he added. No timeline has been given for the presentation of the new rules. But there have already been some regulatory changes, with attention paid to general improvements and efficiency of the system. For example, as noted in the G.S.R. 224(E), a timeline of 21 working days is now in place to issue or deny the permission of narcotic drugs and psycho-

tropic substances (NDPS) for export or import. The new rules also call for a reduction in the timeline of reviews of applications from nine months to four months, while the timeline to issue licenses has gone from 45 days to 10 days.

▶ G20 leaders meeting in Hamburg, Germany, announced the formation of a global body to oversee the discovery and development of new antimicrobial drugs. The **Global Antimicrobial Resistance Collaboration Hub** (GAMRCH) will act to coordinate various other international efforts, to invigorate research and to promote investment across all stages of antimicrobial development. That work will include examining “practical market incentive options” that avoid reliance on high prices or high volume sales, to encourage pharma companies to invest in developing new antibiotics. The hub will be open to all G20 countries, guest countries and non-governmental donors. Members will be expected to put in additional funds over and above what they are currently investing in antimicrobial resistance (AMR) research, but there will be no required minimum investment. The U.K. medical charity **Wellcome Trust** and the **Bill and Melinda Gates Foundation** were the first nongovernmental bodies to pledge support for GAMRCH, which will be under German leadership.

▶ The BioWorld Biopharmaceutical index grew a healthy 6.6% in the second quarter, well ahead of the general markets, thanks to a late June spurt in valuations, causing the index to grow 5.8% last month. In contrast, the Nasdaq Composite index lost almost 1% in value in June and closed the quarter up 3.9%, and the Dow Jones Industrial Average eked out a 1.6% jump in value for the month and was up 3.3% for the quarter. Year to date, the Biopharmaceutical index is up almost 15%. There appeared to be no single event that helped cause a positive spike in valuations last month. The **Cowen and Co.** biotechnology analyst team in its monthly biotech

thermometer report to clients said multiple reasons have been put forth to explain the sector’s performance, but the “most frequently cited is the talk of a favorable executive order from the Trump administration on drug pricing.”

▶ China’s healthcare reforms have made great strides toward developing the country’s pharmaceutical industry, but the current ecosystem may not be enough for China to compete on the world stage of drug development, said industry experts and regulators at last week’s GF Securities Life Sciences Investment Conference. As the 13th Five-Year Plan has made clear, progress in life sciences is a national priority for China. Joining the **International Council for Harmonization** (ICH) as a regulatory member, which the **CFDA** announced on June 20, is one recent milestone the country has achieved in that area. But speakers also said that in order for China’s pharmaceutical industry to rise to the global stage, the whole life sciences ecosystem needs to mature, not just individual elements like regulation and clinical research. Developing a strong patent system is an important step that China has yet to take, said David Beier, a recognized leader in healthcare policy and regulatory affairs. China’s patent applications have increased dramatically in recent years. Beier said he urges China researchers to recognize that their own work is producing many inventions of global value that need IP protection. In addition, the **CFDA** needs to further speed up the approval of IND applications and clear up the backlog. With a shorter approval time, financing should become easier for drugmakers, with more investors willing to invest. 

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## Upcoming Event Highlights

### Conferences

SEPTEMBER 6, 2017  
**Mobile In Clinical Trials**  
 Boston, MA

SEPTEMBER 7-8, 2017  
**Disruptive Innovations US**  
 Boston, MA

NOVEMBER 12-15, 2017  
**MAGI WEST 2017**  
 San Francisco, CA

[ [VIEW ALL CONFERENCES](#) ]

### Training Programs

JULY 25-26, 2017  
**Auditing Techniques for Clinical Research Professionals**  
 San Diego, CA

AUGUST 1-3, 2017  
**Monitoring Clinical Drug Studies: Beginner**  
 Philadelphia, PA

AUGUST 16-17, 2017  
**Working with CROs: Building a Partnership for Project Success**  
 San Francisco, CA

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### Webinars

JULY 20, 2017  
**Introduction to Clinical Research**

JULY 24, 2017  
**CMS-Medicare Coverage Analysis, Budgeting and Billing Compliance**

JULY 26, 2017  
**30-Hour Clinical Data Management On-Boarding Program**

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## Featured Jobs

### Clinical Research Consultative Sales

GuideStar  
 Deerfield, IL

### Case Manager

Direction Home, Akron Canton Area  
 Agency on Aging  
 Uniontown, OH

### Research Study Coordinator - Psychiatric Mood Disorders

UT Southwestern Medical Center  
 Dallas, TX

### Senior Facilities Technician

Vertex Pharmaceuticals  
 San Diego, CA

### Field Clinical Affairs Analyst

Abbot Laboratories  
 Plymouth, MN

### Speech and Language Pathologist

Morgan Memorial Hospital  
 Athens, GA

### Research Study Coordinator

Siyon Clinical Research  
 Santa Rosa, CA

### Manager, Research Data Management Unit

Penn State University  
 Hershey, PA

### Project Coordinator

ERT  
 Raleigh, NC

### Project Manager

Bracket  
 Wayne, PA

### Human Resources Manager

Mark Twain Medical Center  
 San Andreas, CA

### Clinical Research Associate I

Romark, L.C.  
 Tampa, FL

### Dermatology Medical Assistant

Dermatology Inc. of Virginia Beach  
 Virginia Beach, VA

### Research Assistant

ProSciento, Inc.  
 San Diego, CA

### Neurology Faculty Opportunities

UCSF Fresno  
 Fresno, CA

### Clinical Trial Specialist

Express Scripts  
 Kansas City, MO

### Total Joint Arthroplasty as Chief of the Service

Brigham and Women's Hospital  
 Boston, MA

### Clinical Research Coordinator

Medix  
 Phoenix, AZ

### Associate Director Recovery Services

Mental Health & Recovery Board of  
 Wayne-Holmes Co.  
 Wooster, OH

### Institute Research Investigator - Neurodegeneration Consort

MD Anderson Cancer Center  
 Houston, TX

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