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Two Chinas emerging as biopharmas shift toward innovative drugs

By Marie Powers, Staff Writer

Although innovation is the topic of much conversation in China's biopharma industry, Western companies are likely to observe that the notion of innovative drug development in China is more hype than reality. However, participants at the Chinabio Partnering Forum, held in Suzhou earlier this month, offered compelling evidence that Chinese biotechs are, indeed, transforming cutting-edge discoveries into drug pipelines, with partnering opportunities very much welcomed.

No one disputes that the opportunities for drugmakers in China are enormous. Characterizing the country as a BRIC nation, along with Brazil, Russia and India, is a misnomer at this point, according to Ray Stevens, co-founder of Ruiyi Inc. and professor in the departments of molecular biology and chemistry at the Scripps Research Institute. Stevens contended that China "is ahead of every other BRIC national by 10 years."

That assessment isn't far off. A recent Thomson Reuters white paper, "Overcoming Clinical Challenges in BRIC Markets," citing Feb. 18 data from Cortellis Clinical Trials Intelligence, indicated that more than 4,200 clinical trials were started in China over the past five years, compared to approximately 1,200 in Brazil, 2,600 in Russia and 2,300 in India over the same period. In fact, China is the only BRIC nation seeing an increase in the number of clinical trials initiated each year, according to the white paper. Forecasts suggest that China will become the second largest pharmaceutical market in the world in 2017, overtaking Japan.

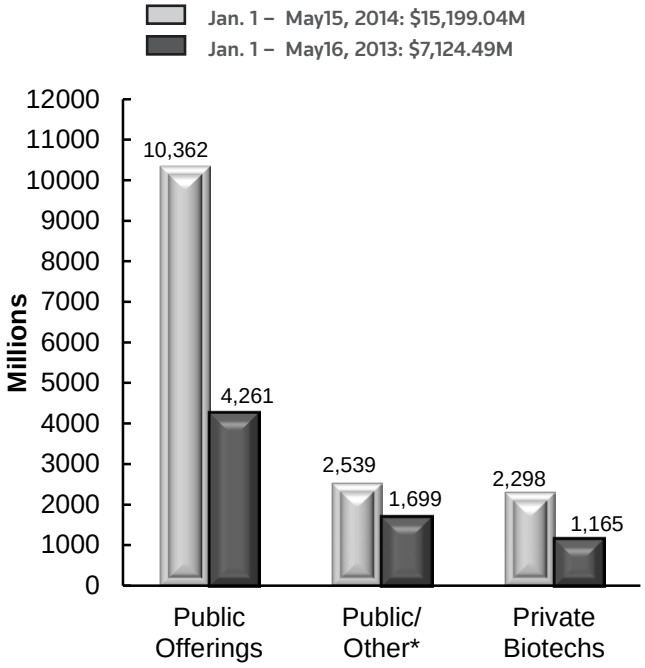
China's potential is attracting droves of returnees – Western-educated Chinese nationals – including tens of thousands of scientists with advanced degrees. Many also have work experience at big pharmas or biotechs and are eager to apply their knowhow to the Chinese market.

CHINESE BIOTECHS IN HUNT FOR PARTNERS

Consider Shenogen Pharma Group. After eight years of effort, the Beijing-based company advanced its lead compound, SNG-162 (Icaritin), into a phase II study in China. Shenogen was co-founded by two lab mates: Kun Meng, chairman and CEO, who completed his postdoctoral training at Harvard University and the Washington University School of Medicine, and Charlie Wang, chief science officer, who completed postdoctoral

See China, page 4

MONEY RAISED BY BIOTECH: 2014 VS. 2013



* Includes financings of public biotech firms with the exceptions of public offerings and certain investments from corporate partners.

INSIDE

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Companies jumping on potential sGC modulators payoff

By Michael Fitzhugh, Staff Writer

Merck & Co. Inc.'s new billion-dollar collaboration with Bayer AG threw a spotlight on drugs capable of amping up concentrations of the messenger molecule cyclic guanosine monophosphate (cGMP) to impact cardiovascular and gastrointestinal diseases. Now Bayer, Ironwood Pharmaceuticals Inc. and others are leveraging expertise built on the way to gaining FDA approval for drugs in the space to expand their use.

The only two FDA-approved drugs capable of modulating cGMP, Bayer's Adempas (riociguat) and Ironwood's Linzess (linaclotide), do so in different ways. Both impact targets in the guanylate cyclase family and both are showing potential for interesting new applications.

Adempas targets soluble guanylate cyclase (sGC), the only known nitric oxide receptor in the human body. Nitric oxide activates soluble guanylate cyclase, which in turn kicks off production of cGMP, relaxing arteries and thereby increasing blood flow and decreasing blood pressure. The FDA approved Adempas to treat two forms of pulmonary hypertension in October 2013, making it the first drug in its class to be approved to treat pulmonary arterial hypertension (PAH) and the first drug of any class to be shown to be effective for patients with chronic thromboembolic pulmonary hypertension.

Ironwood's Linzess (linaclotide), on the other hand, selectively activates guanylate cyclase C (GC-C), a receptor found almost exclusively in the membrane of cells lining the inner surface of intestinal tissue. In August 2012, it became the first and only GC-C activator approved by the FDA to treat chronic idiopathic constipation (CIC) and irritable bowel syndrome with constipation (IBS-C), easing abdominal pain in the latter, and netting 2013 sales of about $119 million. Now Ironwood is pursuing an expanded indication for Linzess in opioid-induced constipation (OIC).

The company is also developing another GC-C activator, IW-9179, to treat functional dyspepsia and gastroparesis. But there's much more opportunity to be realized in regulating cGMP, Ironwood's chief scientific officer, Mark Currie, told *BioWorld Insight*. "In our mind, there are very few regulators that have this kind of utility," he said.

Currie and his team now are advancing preclinical molecules that target sGC in a bid to address indications beyond the gastrointestinal space. They expect to initiate their first sGC clinical study in the first half of 2015, prioritizing evaluation of application in PAH and other cardiovascular indications.

By looking at ways to optimize the half-life and volume of distribution of molecules targeting sGC, Currie's team is on the path to finding ways to deliver a controlled day-long lowering of blood pressure and, in other cases, diminish fibroblast proliferation in the treatment of pulmonary, liver and kidney fibroses. They're also looking at ways to cross the blood-brain barrier to impact Alzheimer's disease and improve memory, he said.

SHARING RISKS AND REWARDS

Despite the promise of sGC modulators, major development efforts and clinical programs will be required to realize their potential. That's where the Bayer-Merck deal comes in. Both companies have been working on sGCs for years. Underscoring Bayer's desire to defray the major costs it will face developing Adempas and other sGCs for new indications, this month it struck a deal giving Merck full commercialization rights for Adempas outside the Americas, half the profits for its phase II sGC, vericiguat, and optionally half of any profit from several other sGCs the companies plan to co-develop. In exchange, Merck will shoulder half the development costs for those new drugs.

For the potential rewards Adempas and the other sGCs offer, Merck agreed to pay Bayer $1 billion up front and up to an additional $1.1 billion if Adempas and the other sGCs hit certain sales milestones. In addition to contributing its cash and one phase I compound, the Whitehouse Station, N.J.-based company also brings serious credibility in the cardiovascular market to the table. (See *BioWorld Today*, May 7, 2014.)

Importantly for both, Bayer's vericiguat, an investigational

See sGC, page 7

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Biotech sector continues to stall as general markets also tumble

By Peter Winter, Editor

With the general markets sliding last week over concerns about an uncertain economy, biotech companies followed suit and by close of market Thursday, the BioWorld Blue Chip Biotech Index had dropped almost 1 percent since the start of the month. This was, however, slightly better than the 1.5 percent decline in the Nasdaq Composite Index over the same period, while the Dow Jones Industrial average has so far held its own and remained unchanged.

Leading the decliners so far in May has been Seattle Genetics Inc., whose shares have fallen 8.7 percent despite reporting solid revenue growth in its first quarter results with net product sales of its Adcetris (brentuximab vedotin) antibody-drug conjugate for treating Hodgkin's lymphoma and anaplastic large-cell lymphoma coming in at $38.7 million compared to $33.9 million for the same period in 2013. Despite the fact that most analysts believe the company's prospects are very good going forward investors are still looking for more. It is a story that has been reflected across the whole spectrum of the sector – despite a generally positive first quarter for public biotech companies, the reports have failed to bring investors back to the fold. (See BioWorld Blue Chip Index, below.)

The BioWorld Growth Index, which includes companies with market caps in the range of $1 billion to $3 billion and a résumé that typically includes a strong drug pipeline and partnered products in late-stage clinical trials, has seen its value drop almost 8 percent since the end of the first quarter, but similar to the Blue Chip Index has only fallen a little over 0.5 percent in May. However, companies in the group have seen their share values go on a wild ride during this period of uncertainty for the sector.

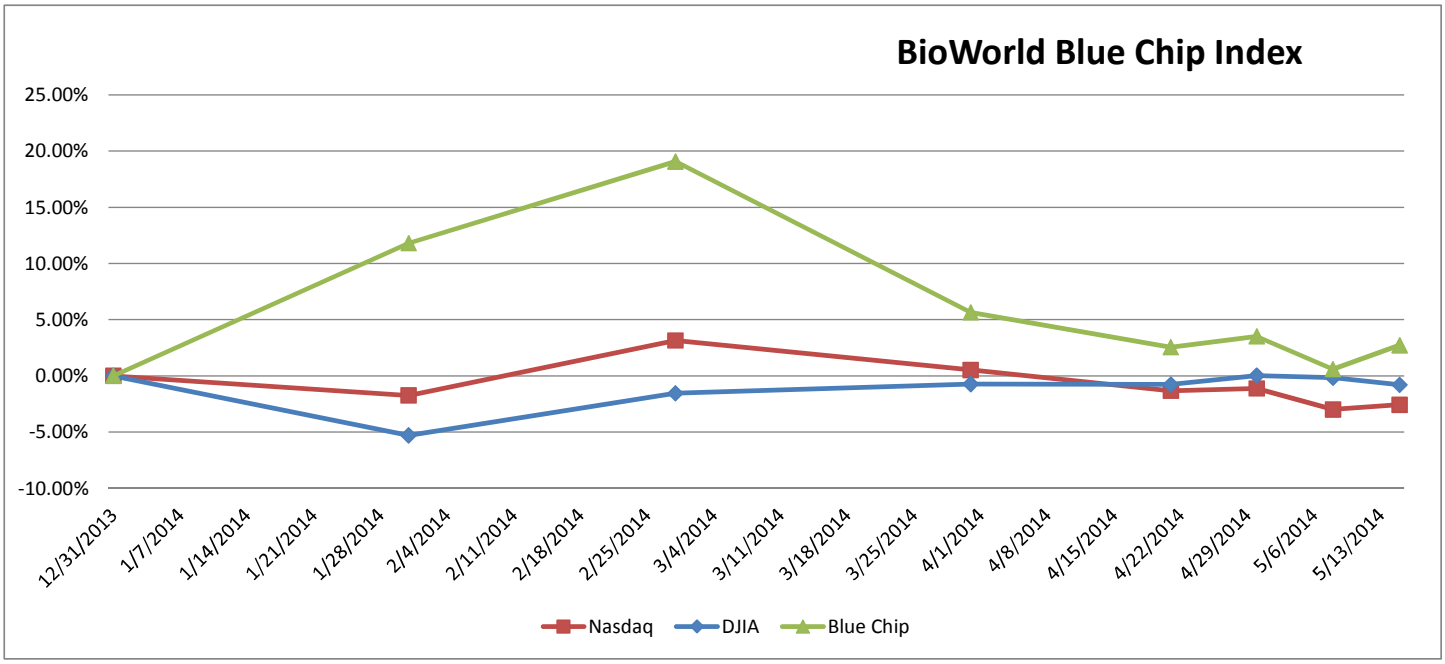
Leading RNAi therapeutics company Alnylam Pharmaceuticals Inc., for example, saw its share value drop 15 percent in April only to regain most of it back so far this month on the strength of reporting positive top-line results from its ongoing phase I trial of ALN-AT3, a subcutaneously administered RNAi therapeutic targeting antithrombin in development for the treatment of hemophilia and rare bleeding disorders.

Among the leading decliners in the group was Aegerion Pharmaceuticals Inc., of Cambridge, Mass., whose shares have tumbled 25 percent after posting a wider loss than analysts were expecting in its first quarter financials. The company did post $27 million in net product sales for its Juxtapid (lomitapide) capsules, approved to treat patients with homozygous familial hypercholesterolemia (HoFH), a genetic disorder that causes extremely high cholesterol levels. The amount represented a growth of 24 percent over net product sales from the U.S. business in the prior quarter.

News of Acorda Therapeutics Inc.'s complete response letter for the new drug application (NDA) related to Plumiaz (diazepam) for epileptic cluster seizures was enough to send its shares down about 14 percent so far this month. The company also said the nasal spray version of the benzodiazepine therapy is unlikely to win approval this year. (See *BioWorld Today*, May 5, 2014.)

The dive in share prices permeated across the board for all biotech companies. The BioWorld Emerging Biotech Index, which tracks selected small-cap companies that have

See Markets, page 7



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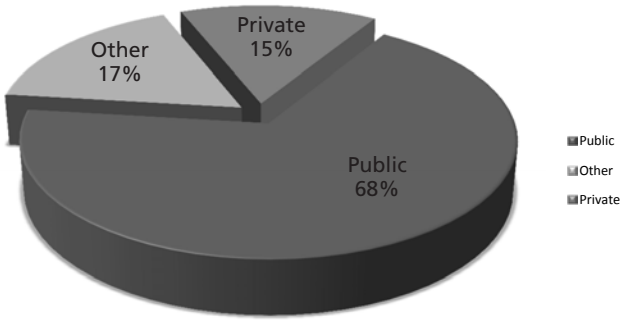

Public Offerings	68% ($10,362M)
Public/Other	17% ($2,539M)
Private Biotechs	15% ($2,298M)

China

Continued from page 1

training at Princeton University and served as an associate professor at Harvard Medical School. Wang also is a professor of pathology at Creighton University.

Jun Bao, the company's senior vice president, chief business officer and acting chief financial officer, holds a PhD from the University of Kansas. Bao's resume includes tenure at Glaxosmithkline plc in China and at U.S. firms Onyx Pharmaceuticals Inc., Icos Corp. – acquired in 2006 by Cialis (tadalafil) joint venture partner Eli Lilly and Co. – and Cell Therapeutics Inc. (See *BioWorld Today*, Oct. 18, 2006.)

Shenogen is targeting first-in-class drug development. Its SNG-162 is a naturally derived traditional Chinese medicine known as yingyang huo, a perennial grass whose name translates as "horny goat weed." The small molecule, which targets the estrogen receptor ER-alpha 36, is in a phase II study in China, with applications in liver and breast cancer as well as leukemia.

Shenogen was started with just $100,000 in seed money, according to Bao, and now has 80 employees. The company's business acumen and scientific prowess last year helped Shenogen attract a $20 million series C from a syndicate that included Qiming Venture Partners LLC, of Shanghai, and Legend Capital, of Beijing, an affiliate of tech company Lenovo, along with China Investment Wealth Venture Fund and Shenzhen Venture, a venture arm of the municipal government. Lead investors from Shenogen's series B, including IDG Venture and Lapham Group Inc., also joined. (See *BioWorld Asia*, Nov. 6, 2013.)

Bao attended Chinabio seeking partners.

"We'd like to find one or two partners for the market outside China," he told *BioWorld Insight*, "and in China we are also open for collaborations." The fact that SNG-162 has shown activity in solid tumors as well as leukemia is intriguing, Bao said, "and we've observed in the clinic, not just in animal studies, that every time we saw activity, we saw it first at metastasized sites. We're thinking that, if you combine our drug with some chemotherapy, it can wipe out metastasized sites as well as the primary tumor site, which would be a nice combination. But we can only do one or two trials at a time with our resources. A partner can do multiple trials."

'THE QUALITY OF THE SCIENCE IS OUTSTANDING'

Suzhou Alphamab Co. Ltd. is another model of innovative drug discovery. Founded in 2008, the company has 90 employees, including more than 65 scientists in its R&D operation. Alphamab is advancing biosimilars as its bread and butter but also building a pipeline of novel oncology drugs. For example, KN-014 is a genetically engineered therapeutic protein that inhibits various isotopes of VEGF, PIGF and PDGF, with the goal of treating lung, bladder, colorectal and other metastatic cancers, as well as wet age-related macular degeneration. KN-010 is a bispecific antibody that binds different epitopes on cancer cells to prohibit cancer cell growth and proliferation through a variety of mechanisms. Designed for improved efficacy against tumors while preventing resistance, the drug is being developed to treat breast cancer and other solid tumors.

Founded last year, Mabspace Biosciences Co. also is focusing on antibodies for therapeutic and diagnostic applications. Using immune tolerance breaking technology, the company has identified three therapeutic antibodies, MSB-001, MSB-002 and MSB-003, and one diagnostic application, MSB-004, that it is seeking to partner. MSB-001 is designed as a best-in-class antibody that targets the tumor microenvironment for treating gastric and lung cancers. MSB-002 is a first-in-class antibody with the potential to treat tissue fibrosis and cancer, and MSB-003 is an antibody-targeted immunotherapy for lung cancer, renal cancer and melanoma.

And Ruiyi has a bi-coastal strategy in its effort to develop novel drugs for the Chinese market. The company is headquartered in La Jolla, Calif., but has its discovery efforts and research facility in the Zhangjiang Hi-Tech Park in Shanghai. Earlier this year, Ruiyi netted a $15 million series B round from its existing investors, which include 5AM Ventures, Versant Ventures, Apposite Capital, SR One, Merck Serono Ventures and Aravis SA. (See *BioWorld Asia*, April 2, 2014.)

Ruiyi is using its intermembranous Conformation Antigen Presenting System, or iCAPS, technology to leverage therapeutic specificity across a set of targets from the large G protein-coupled receptor (GPCR) family. Although nearly one-third of approved drugs modulate GPCRs, traditional drug discovery methods have been stymied in exploring many of the selective targeting GPCRs – especially those with small extracellular domains. Ruiyi's iCAPs changed that equation, enabling the purified, isolated, conformationally correct presentation of functional GPCRs, optimized to identify selective antibody inhibitors or activators with greater specificity, resulting in the

See China, page 6

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WEEK IN REVIEW

FINANCINGS

Aeterna Zentaris Inc. entered an at-market issuance sales agreement with MLV & Co. LLC.

Agile Therapeutics Inc. filed to raise up to $65 million in an IPO.

Celator Pharmaceuticals Inc. entered a $15 million loan agreement with Hercules Technology Growth Capital Inc.

Chase Pharmaceuticals Corp. raised $21 million in a series B financing round.

Imaginab Inc. completed a $21 million series B financing round.

Invivo Therapeutics Holdings Corp. closed a public offering for gross proceeds of approximately $16.1 million.

Gemmus Pharma Inc. said it closed a $3.3 million series B financing round.

Marinus Pharmaceuticals Inc. filed to raise up to $63 million in an IPO.

Radius Health Inc. postponed its IPO citing poor market conditions.

Sorrento Therapeutics Inc. expects to generate $25 million in gross proceeds from a public offering.

Sorbent Therapeutics Inc. said it closed a $6.5 million tranche of a $15 million series D financing.

ZS Pharma Inc. is seeking to raise up to $86 million from an IPO.

DEALS

Allergan Inc. has rejected the unsolicited April 22 acquisition proposal from **Valeant Pharmaceuticals International Inc.**

Aslan Pharmaceuticals Pte Ltd. signed an agreement with **CSL Ltd.** to develop an anti-IL-13 receptor monoclonal antibody, CSL334, for severe to moderate asthma.

Bristol-Myers Squibb Co. is testing PD-1 checkpoint inhibitor nivolumab in non-small-cell lung cancer with **Celldex Therapeutics Inc.**'s CD27-targeting varlilumab.

Jubilant Biosys Ltd. and **Orion Corp.** are collaborating to discover small-molecule inhibitors in the neuroscience therapeutic area.

Mabvax Therapeutics Inc., a privately held cancer immuno-therapy company, is merging with publicly traded **Telik Inc.**

Medimmune is testing its anti-PDL-1 immune checkpoint inhibitor, MEDI4736, in combination with **Incyte Inc.**'s oral indoleamine dioxygenase-1 inhibitor.

Merck & Co. Inc. signed a deal to sell nine of its ophthalmic products to Japan-based **Santen Pharmaceutical Co. Ltd.**

Prometic Life Sciences Inc. increased its ownership in **Nantpro LLC.**

Shire plc is acquiring **Lumena Pharmaceuticals Inc.** for $260 million up front plus undisclosed milestones.

Riemser Pharma GmbH acquired **Keocyt**, which markets pharmaceutical products to treat rare diseases.

Zalicus Inc. said **Horizon Discovery Group** will acquire its combination high-throughput screening platform and related assets for $8 million.

. . . AND MORE

Abeona Therapeutics has been granted FDA orphan drug designations for its lead investigational therapies for treatment of Sanfilippo syndromes A and B.

ALK-Abello A/S said partner **Merck & Co. Inc.** launched ragweed allergy immunotherapy tablet Ragwitek in Canada.

Beryllium has finalized its consolidation of assets from Emerald Bio and Decode Genetics and emerged as a drug discovery company.

Daiichi Sankyo Co. Ltd. discontinued a phase III trial for nimotuzumab after four treatment-related deaths were observed, all in the nimotuzumab arm.

Health Canada has suspended the establishment license of **Biolyse Pharma Corp.**'s facility in St. Catharines, Ontario, because of concerns with the manufacturing process.

Merck & Co. Inc. said the FDA approved Zontivity (vorapaxar) to reduce thrombotic cardiovascular events in patients with a history of heart attack or with peripheral arterial disease.

Provectus Biopharmaceuticals Inc. said its shares have been approved for listing on the NYSE MKT.

WORD ON THE STREET

"I think [cancer immunotherapy] is very exciting and ultimately will be beneficial to patients, but I do think it's blocked out a little bit of the sun at ASCO again."

Ron Squarer, CEO, Array Biopharma Inc.

"This study characterizes the extent to which our genetic makeup influences biomarkers in our blood, and it helps to define their general characteristics. With its help, we can begin to understand very comprehensively to what extent our physiological status is controlled by genetics and by nongenetic factors."

Nicole Soranzo, group leader at the Wellcome Trust Sanger Institute in Hinxton, Cambridgeshire, on a massive study exploring the ways in which common genetic variants influence the levels of various metabolites in human blood

"The decision to divest our ophthalmic business is part of our ongoing strategy to sharpen our commercial focus and improve our operational effectiveness."

Jay Galeota, president of hospital and specialty care at Merck & Co. Inc., on its decision to sell nine of its ophthalmic products to Japan-based Santen Pharmaceutical Co. Ltd.

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WEEK IN WASHINGTON

The EMA and the FDA released a draft joint proposal to facilitate multi-arm, multicompany clinical trials for pediatric treatments of Gaucher disease. The proposed approach would determine the safety and efficacy of several new medicines at the same time, reducing the total number of children enrolled since all the therapies would be evaluated against one control group. If successful, this concept could be used to develop multiple drugs for other rare diseases in a reduced time frame and with limited enrollment.

The FDA released the latest installment in its series of draft biosimilar guidances. It discusses the design and use of clinical pharmacology studies. Pharmacokinetic and pharmacodynamic data are required as part of the FDA's stepwise approach to demonstrating biosimilarity to the reference drug. The draft guidance identifies three key concepts – exposure and response assessment, evaluation of residual uncertainty and assumptions about analytical quality and similarity – as especially relevant to the development of biosimilars.

The Federal Trade Commission (FTC) is once again proposing a study of patent assertion entities, also known as patent trolls, and their impact on innovation and competition. The FTC first proposed the study last year. After reviewing 70 comments on the study plan, the commission revised the study to sharpen its focus and reduce the burden on participants.

The EMA issued an update on joint U.S.-European efforts to tackle the rising tide of antimicrobial resistance, detailing progress in converging the EMA and FDA regulatory requirements in the development of new antibacterial drugs. The move to merge regulation in that area sits under the umbrella of the Transatlantic Taskforce on Antimicrobial Resistance, established between the European Commission and the U.S. Department of Health and Human Services, to act together in fighting drug-resistant pathogens. //

China

Continued from page 4

potential development of more effective antibodies and other biologics.

The company's lead candidate, RYI-008, is an anti-IL-6 monoclonal antibody with potential uses in autoimmune diseases and cancer, with rheumatoid arthritis as a proposed initial indication. (See *BioWorld Today*, May 17, 2013.)

Ruiyi's Stevens was lured to China by the energy and entrepreneurship in the nation's science community. After teaching students from China for more than a decade and hearing the hype about their home country, he wanted to answer three questions: Is the science real? Is it high quality? And how would it lead to innovative drug development?

"I've been coming to China for 20 years, and I used to see a lot of empty buildings," Stevens told *BioWorld Insight*. After three years on the ground in Shanghai, he's convinced the science "is very real." His students at the iHuman Institute at ShanghaiTech University – a research university modeled after the Massachusetts Institute of Technology and developed from the ground up by the Shanghai Municipal Government and Chinese Academy of Sciences – already are publishing papers in *Nature* and *Science*.

"The quality of the science is outstanding," Stevens said, adding that the opportunity to conduct drug development is better in China than almost anywhere else in the world, including the U.S. "Putting Ruiyi in China was exactly the right thing to do," he said.

'YOU HAVE TO BE FULLY INVESTED'

That's not to say innovative drug development in China is a cakewalk. Locals cite challenges with importing cells, gaining access to radioactivity and interfacing with the China Food and Drug Administration (CFDA), along with mundane tasks such as making bank deposits, which can require a half-day of standing in line. The perception of drug development also is different in China, where biosimilars remain the standard and domestic investors view phase II as early stage.

For that reason, Ruiyi's lead compound uses well-established biology that is sufficiently innovative to help the CFDA gain experience working with the company's technology. "Once we have that one going, we can follow suit with our GPCR antibodies, which are innovative," Stevens said.

Pursuing innovative drug discovery and development in China requires commitment, he added.

"Everything is changing rapidly, so if you want to do drug development here, you can't do it part-time," he maintained. "You commit or you don't. If you care about quality, you have to be fully invested."

In some ways, however, China reminds Stevens of California, which was the "wild, wild West" in the early days of biotech.

"No one has really hit it big yet in China" in terms of innovative drug development, he said, "but our investors know this is where they need to be. We know a breakthrough drug is going to happen."

Until it does, patience is the key.

"Don't come to China because you think that's where the money is," Stevens advised. "Instead, come to China if you have a really good business model. There's double-digit growth potential and a large population of treatment-naïve patients with unmet medical needs. But you need long-term thinking, not just one or two years." //

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sGC

Continued from page 2

sGC it's developing in two phase IIb studies in an indication it's calling "worsening chronic heart failure," is included in the deal. Merck's lengthy experience selling cardiovascular treatments in the U.S. market could be a boon for Bayer, noted Bayer CEO Marijn Dekkers, during a conference call discussing the deal.

"The significant expertise that Merck has built as a top five global player in the cardiovascular area, and its strong presence in the large U.S. market, make Merck a partner of choice for our current and future sGC program," he said.

Without doubt, having a partner to foot the cost of what Dekkers called "a significant and comprehensive" phase III trial of vericiguat next year will help, too.

For vericiguat and other potential investigational sGC modulators, Bayer will lead the commercialization outside the Americas while Merck will lead commercialization in the Americas. Both companies will have the option to co-promote Adempas and the follow-on sGC modulators in each others' territories.

Meanwhile, Leverkusen, Germany-based Bayer already is looking for ways to expand riociguat's use. It's preparing to run phase II trials studying the drug's impact in pulmonary hypertension (PH) associated with interstitial idiopathic pneumonia, diffuse systemic sclerosis, cystic fibrosis and the rare Raynaud's disease.

The partners also are testing two more compounds in phase I, an unnamed Bayer sGC stimulator in development as a treatment for resistant hypertension and Merck's MK 8892 for PH and heart failure. A second Bayer compound is part of the deal, but is still preclinical.

MORE IN PLAY

Innovation alone won't make drugs targeting guanylate cyclases successful, of course. In PAH, for example, Adempas already has competition from Actelion Pharmaceuticals Ltd.'s Opsumit (macitentan), an oral version of Actelion's Tracleer (bosentan) that was approved in October 2013 for PAH. United Therapeutics Corp.'s Remodulin (treprostinil) is creating competition for Adempas, too. But clearly there's plenty of commercial and clinical interest carrying cGMP modulators ahead.

New York-based Synergy Pharmaceuticals Inc. is developing plecanatide, a drug targeting GC-C to treat the same indications as Linzess. The company intends to initiate pivotal phase III trials with IBS-C patients in the second half of 2014. It's additionally developing a GC-C agonist it calls SP-333 for the treatment of OIC and ulcerative colitis. (See *BioWorld Today*, Nov. 13, 2013.)

Tokyo-based Astellas Pharma Inc., Ironwood's development and marketing partner in Japan, also is working on sGCs and has built an extensive patent position in the space, but with very different chemistry than Ironwood's, Currie said.

"I think it's a natural progression as you see advances in these area that once you're able to bring clear benefit to patients," he said, "there will continue to be innovations." //

Markets

Continued from page 3

market caps averaging about $500 million, has seen its value drop about 1 percent in May.

Despite a rocky start to the month, the overall declines for biotech companies have not been as dramatic as predicted. There will still be some turbulence going forward but with investors squarely focused on the upcoming 50th meeting of the American Society of Clinical Oncology later this month this could be enough to smooth the ride for biotech stocks given the number of promising results from important cancer therapies that drug developers are scheduled to report. //

NEGOTIATE LUCRATIVE BIOPHARMA LICENSING DEALS

BIOPHARMACEUTICAL ROYALTY RATES ANALYSIS:

ESSENTIAL BENCHMARKS FOR DEALMAKING

New data from BioWorld reveal that biotechnology companies are now garnering higher royalty rates from pharmaceutical partners, higher than other biotech partners.

BioWorld Data's new resource, Biopharmaceutical Royalty Rates Analysis: Essential Benchmarks for Dealmaking, analyzed hundreds of licensing deals. According to the Royalty Rates Analysis, the average royalty rate for deals with a pharma licensee is 14.9% at the high end and 11.7% at the low end.

This critical negotiation and analysis tool contains:

- More than 320 licensing deals with all deal terms and key background information on the drug or drug technology
- Median royalty rates and up-front licensing fees, details that can be used as benchmarks and comparators
- Covers hot disease topic areas and more: Alzheimer's, cancer, cardiovascular, diabetes, hepatitis and pain management

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FDA approvals in April

Company	Drug	Indication
Alk-Abello A/S (Copenhagen) and Merck & Co. Inc. (Whitehouse Station, N.J.)	Ragwitek	Ragweed allergic rhinitis
Biogen Idec Inc. (Cambridge, Mass.)	Alprolix	Hemophilia B
Dyax Corp. (Cambridge, Mass.)	Kalbitor	Acute hereditary angioedema
Genmab A/S (Copenhagen) and Glaxosmithkline plc (London)	Arzerra	Chronic lymphocytic leukemia
Greer Laboratories Inc. (Lenoir, N.C.)	Oralair	Allergic rhinitis
Kaleo Inc. (Richmond, Va.)	Evzio	Opioid overdose

Phase I clinical trials update

April 2014

Company (location)	Product	Description	Indication	Status	Date
CANCER					
Access Pharmaceuticals Inc. (New York)	Mugard	Muco-adhesive polymer treatment	Biopsy-proven oral lichen planus	Results from a pilot study of 20 patients showed statistically significant improvement in ulcerations and pain	4/16/14
Affimed Therapeutics AG (Heidelberg, Germany)	AFM13	Monotherapy	Advanced relapsing/ refractory Hodgkin lymphoma	Phase I data confirmed safety in 28 patients	4/11/14
Aprea AB (Stockholm)	APR-246	A compound aimed at restoring normal function to tumor suppressor protein p53	Relapsed platinum-sensitive high-grade serous ovarian cancer	Dosing started in its phase I/II trial of APR-246; it will enroll about 180 patients in a two-part design	4/17/14
Arno Therapeutics Inc. (Flemington, N.J.)	Onapristone	An oral, anti-progestin hormone blocker	Advanced castration-resistant prostate cancer	Enrolled the first patient in a phase I/II trial	4/8/14
Celldex Therapeutics Inc. (Hampton, N.J.)	CDX-1401	Vaccine consisting of a fully human monoclonal antibody with specificity for the dendritic cell receptor DEC-205 linked to the NY-ESO-1 tumor antigen	Solid tumors	Final phase I data demonstrated robust antibody and T-cell responses and evidence of clinical benefit in patients with very advanced cancers and suggest that CDX-1401 may predispose patients to better outcomes on subsequent therapy with checkpoint inhibitors	4/17/14

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Cleveland Biolabs Inc. (Buffalo, N.Y.)	CBL0102	Quinacrine; an orally administered small molecule	Advanced cancers	Phase I trial characterized the drug's safety, profiled its pharmacokinetics, and assessed it for preliminary evidence of antitumor activity in the liver	4/17/14
Deciphera Pharmaceuticals LLC (Lawrence, Kan.)	LY3009120	Pan-RAF inhibitor	Cancer	Started a phase I trial	4/8/14
Development Center for Biotechnology (Taiwan)	DCBCI0901	mTOR inhibitor	Cancer	Received approval from the FDA and the TFDA to begin phase I trials	4/25/14
Dicerna Pharmaceuticals Inc. (Watertown, Mass.)	DCR-MYC	A synthetic double-stranded RNA in a stable lipid particle	Solid tumors, multiple myeloma or lymphoma	Started a phase I trial	4/18/14
Helix Biopharma Corp. (Aurora, Ontario)	L-DOS47	A targeted antibody derived from naturally occurring plant enzyme urease	Stage IV recurrent or metastatic nonsquamous non-small-cell lung cancer	Received FDA approval to start a phase I trial	4/23/14
Immatics Biotechnologies GmbH (Tuebingen, Germany)	IMA950	Cancer vaccine	Adult brain tumor	A phase I trial met its primary endpoints of safety and immunogenicity	4/24/14
Innate Pharma SA (Marseille, France)	lirilumab	Anti-KIR; checkpoint inhibitor	Solid tumors	Began the cohort expansion portion of an open label phase I trial	4/1/14
Karyopharm Therapeutics Inc. (Natick, Mass.)	KPT-330	Selinexor	Leukemia	Started a phase I trial of selinexor in children with high-risk malignancies resistant to standard chemotherapy	4/22/14
Peregrine Pharmaceuticals Inc. (Tustin, Calif.)	Bavituximab	Immunotherapy	Advanced melanoma	Opened enrollment of a trial of bavituximab in combination with Yervoy (ipilimumab, Bristol-Myers Squibb Co.)	4/24/14
Precision Biologics Inc. (Dallas)	NEO-102	A reformulation of the monoclonal antibody NPC-1C	Refractory metastatic pancreatic and colorectal cancer	Phase I/IIa data showed preliminary signs of activity based on stabilization of disease	4/11/14
Sorrento Therapeutics Inc. (San Diego)	RTX	Resiniferatoxin	Intractable cancer pain	Phase I/II data from six patients showed clinically meaningful improvement in quality of life, achieving, on average, a 20% reduction in their pain intensity	4/7/14

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Tocagen Inc. (San Diego)	Toca 511 and Toca FC	Vocimagene amiretrorepvec and flucytosine; gene therapy candidate	Recurrent high-grade glioma	Interim data of patients treated with the combination showed it to be safe and well tolerated with minimal treatment-related toxicity	4/11/14
Xbiotech Inc. (Austin, Texas)	Xilonix	The firm's noncytotoxic antitumor therapy	Advanced cancer	Results from a phase I/II study showed an excellent safety profile, while overall, patients' constitutional symptoms improved	4/18/14
CARDIOVASCULAR					
Capricor Therapeutics Inc. (Los Angeles)	CAP-1002	An allogeneic cardiospere-derived stem cell	Myocardial infarction	Phase I/II data showed it met its primary endpoint of safety	4/1/14
Capstone Therapeutics Inc. (Tempe, Ariz.)	AEM-28	An ApoE mimetic peptide	Low-density lipoprotein/non-high-density lipoprotein cholesterol reduction	Dosing was started in the phase Ia trial	4/9/14
Cytori Therapeutics Inc. (San Diego)	Cytori Cell Therapy	Employs a mixed population of adipose derived regenerative cells extracted from a patient's own fat tissue using Cytori's Celution system	Chronic ischemic heart failure	Data from the PRECISE trial phase I/IIa feasibility trial demonstrated statistically significant differences in cardiac functional capacity between treated and placebo groups	4/15/14
CENTRAL NERVOUS SYSTEM					
Cynapsus Therapeutics Inc. (Toronto)	APL-130277	A single 25-mg sublingual strip dose of apomorphine	Parkinson's disease	Interim data from its pilot study indicated that a higher load of drug on the strip does result in a higher amount of drug entering the bloodstream	4/3/14
Glenmark Pharmaceuticals SA (Mumbai, India; subsidiary of Glenmark Pharmaceuticals Ltd.)	GBR 900	Monoclonal antibody that targets TrkA	Chronic pain	Is entering human trials	4/30/14
Prothena Corp. plc (Dublin)	PRX002	Monoclonal antibody targeting alpha-synuclein	Parkinson's disease	Started a phase I trial	4/10/14
Vernalis plc (Winnersh, UK)	V81444	An A2A antagonist	Parkinson's disease, attention deficit hyperactivity disorder and other disorders	Results from a phase Ib/II proof-of-concept study showed it achieved a statistically significant improvement in the number of correct scores in PERMP-P measure ($p = 0.019$) compared to placebo	4/25/14

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DIABETES					
Aspireo Pharmaceuticals Ltd. (Tel Aviv, Israel)	DG3173	Somatroprim; a somatostatin analogue	Diabetes	Final phase Ib data demonstrated that while octreotide significantly inhibited the secretion of insulin and glucagon in humans and showed a significant and sustained increase of plasma glucose levels, the company's DG3173 had much less of an effect on insulin and glucagon release	4/23/14
Halozyme Therapeutics Inc. (San Diego)	Hylenex	Recombinant and a new formulation	Type 1 diabetes	The primary endpoint of non-inferiority of A1C levels at six months was met for the CONSISTENT 1 trial	4/1/14
Sernova Corp. (London, Ontario)	Cell Pouch	Contains insulin-producing islets	Unstable type 1 diabetes	Interim results from a phase I/II study demonstrated that in the first group of patients the implanted Cell Pouch is showing longer-term safety and biocompatibility	4/23/14
INFECTION					
Galectin Therapeutics Inc. (Norcross, Ga.)	GR-MD-02	Targets galectin-3	Non-alcoholic steatohepatitis with advanced fibrosis	The first patient in cohort 2 of its phase I trial was successfully dosed with 4 mg/kg, which is double the dose given in cohort 1; all eight patients have received their first infusion in cohort 2	4/18/14; 4/24/14
Hepatera Ltd. (Moscow)	Myrcludex B	Inhibits the entry mechanism of HBV and HDV	Chronic hepatitis B virus and hepatitis delta virus	Initiated a trial investigating it for the effects of Myrcludex in combination with pegylated interferon and the use of the entry inhibitor as pre-treatment before interferon therapy is initiated	4/3/14
INFLAMMATORY					
Ablynx NV (Ghent, Belgium)	ALX-0061	Anti-IL-6R nanobody	Inflammatory diseases	Started dosing in healthy volunteers in a phase I trial evaluating a subcutaneous formulation	4/23/14
MISCELLANEOUS					
Agios Pharmaceuticals Inc. (Cambridge, Mass.)	AG-348	A small molecule activator of PKR	Pyruvate kinase deficiency	Started a phase I trial	4/18/14
Allena Pharmaceuticals Inc. (Newton, Mass.)	ALLN-177	An orally administered recombinant oxalate degrading enzyme	Hyperoxaluria and kidney stones	Phase I data demonstrated a statistically significant difference in the reduction of urinary oxalate levels in 30 healthy subjects when the treatment was compared with placebo (p = 0.0002)	4/30/14

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Critical Pharmaceuticals Ltd. (Nottingham, UK)	Teriparatide	Nano-enabled intranasal teriparatide product	Osteoporosis	Started a phase I trial in healthy postmenopausal women	4/16/14
Galectin Therapeutics Inc. (Norcross, Ga.)	GR-MD-02	Galectin inhibitor	Advanced fibrosis	The first phase I cohort showed a therapeutic effect on fibrosis, inflammation and cellular injury	4/2/14
Kamada Ltd. (Ness Ziona, Israel)	Glassia	Liquefied human alpha-1 antitrypsin	Graft-vs.-host disease	Company is starting a small phase I/II proof-of-concept study	4/4/14
Lpath Inc. (San Diego)	Lpathomab	An antibody to lysophosphatidic acid	Brain injury	Started a study of brain injury induced by blast overpressure	4/24/14
Nitto Denko Corp. (Osaka, Japan)	RNAi-based drug	Consists of an siRNA that inhibits the cause of fibrosis, as well as a targeted lipid nanoparticle delivery system	Fibrosis in the liver and other organs	Dosing of healthy volunteers in a phase I study has completed	4/23/14
Oxford Biomedica plc (Oxford, UK)	Retinostat	A lentiviral vector-based treatment	Neovascular wet age-related macular degeneration	Completed the planned recruitment of 21 patients into its phase I trial	4/8/14
Prothena Corp. plc (Dublin)	NEOD001	A monoclonal antibody	AL amyloidosis and persistent organ dysfunction	Interim data from an ongoing phase I study showed that NEOD001 administered intravenously once per month was generally safe and well tolerated at the doses studied	4/28/14
Questcor Pharmaceuticals Inc. (Anaheim Hills, Calif.)	H.P. Acthar Gel	Repository corticotropin	Nephrotic syndrome	Clinical results showed it could be a potentially useful therapy for inducing remission of proteinuria	4/22/14
Revance Therapeutics Inc. (Newark, Calif.)	RT002	Injectable botulinum toxin type A	Moderate to severe glabellar (frown) lines	Phase I/II data showed that the trial met its primary efficacy and safety endpoints	4/23/14
Stemcells Inc. (Newark, Calif.)	HuCNS-SC	Human neural stem cells	Spinal cord injury	Completed enrollment of the phase I/II trial	4/18/14
RESPIRATORY					
Xenetic Biosciences Inc. (Lexington, Mass.)	Pulmoxen	A modified form of recombinant human Dnase I	Cystic fibrosis	Positive results from its phase I trial in Russia showed that two doses of 2,500 IU and 5,000 IU were found to be safe and well tolerated	4/8/14

NOTES

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Phase II clinical trials update
April 2014

Company (location)	Product	Description	Indication	Status	Date
AUTOIMMUNE					
Cytos Biotechnology AG (Schlieren, Switzerland)	CYT003	Therapeutic vaccine	Allergic asthma	Failed in a phase IIb trial	4/15/14
Galapagos NV (Mechelen, Belgium) and Glaxosmithkline plc (London)	GSK2586184	A selective JAK1 inhibitor	Psoriasis	GSK2586184 met the primary endpoint in phase IIa psoriasis study, with patients given the drug achieving a 75% or greater improvement from baseline in their Psoriasis Area Severity Index scores at week 12	4/18/14
Gene Signal International SA (Lausanne, Switzerland)	GS-101	Aganirsen; an antisense oligonucloetide	Psoriasis	A phase IIa study demonstrated that topical application of GS-101 reduced the size of psoriatic lesions and inflammation compared to placebo	4/11/14
Geneuro SA (Geneva)	GNbAC1	Monoclonal antibody that targets the MSRV-Env protein	Multiple sclerosis	The one-year open-label extension of its phase IIa study in 10 patients was completed, with results showing that the treatment was well tolerated over the long term and that pharmacodynamic responses could be observed	4/18/14
Phosphagenics Ltd. (Melbourne, Australia)	Tretinoin	Formulated with Phosphagenics' transdermal delivery technology TPM	Acne	Completed enrollment in a phase II trial	4/24/14
Synthetic Biologics Inc. (Rockville, Md.)	Trimesta	Oral estriol; oral, once-daily treatment	Relapsing-remitting multiple sclerosis	Topline data from the phase II trial demonstrated that Trimesta given with first-line RRMS therapy Copaxone (glatiramer acetate injection) resulted in reduced annualized relapse rate and improved cognitive function at 12 months of treatment as compared to placebo plus Copaxone	4/30/14
CANCER					
Ambit Biosciences Corp. (San Diego)	Quizartinib	Selective FLT3 inhibitor	FLT3-ITD positive acute myeloid leukemia	Started a phase II cohort of a phase I/II study of quizartinib in combination with either 5-azacitidine or low-dose cytarabine	4/8/14

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Dendreon Corp. (Seattle)	Provenge	Sipuleucel-T	Biochemically recurrent prostate cancer	Phase II STAND data suggested tumor-specific T-cell responses appear to be enhanced and sustained when Provenge is given following androgen deprivation therapy	4/14/14
Halozyme Therapeutics Inc. (San Diego)	PEGPH20	Pegylated recombinant human hyaluronidase	Pancreatic cancer	Halted its phase II trial following an unexpected imbalance in thromboembolic event rate between treatment and control groups; FDA placed a clinical hold on patient enrollment and dosing	4/7/14; 4/10/14
Oncosec Medical Inc. (San Diego)	Immunopulse	DNA IL-12 DNA-based immunotherapy	Cutaneous T-cell lymphoma	Company is relaunching a phase II trial	4/8/14
Progenics Pharmaceuticals Inc. (Tarrytown, N.Y.)	PSMA ADC	Comprises a fully human monoclonal antibody selectively targeting prostate-specific membrane antigen linked to a chemotherapeutic drug	Prostate cancer	Completed enrollment in the chemotherapy-naïve cohort in its phase II trial of PSMA ADC; that cohort of 36 patients, all of whom progressed on hormonal therapies, was added to the phase II study following positive response to PSMA ADC in patients in the chemotherapy-experienced setting	4/25/14
Puma Biotechnology Inc. (Los Angeles)	PB272	Neratinib; a pan-HER tyrosine kinase inhibitor	Metastatic breast cancer	Phase II data showed that the neratinib-containing regimen graduated, based on a high probability of success in phase III with a signature of HER2-positive/HR-negative; neratinib achieved an estimated pCR rate of 55.6% compared to the control arm, which had an estimated pCR rate of 32.6%	4/9/14
Spectrum Pharmaceuticals Inc. (Henderson, Nev.)	Melphalan	Captisol-enabled propylene glycol-free	Multiple myeloma	Met the primary endpoints in a pivotal phase II trial	4/24/14
Ventirx Pharmaceuticals Inc. (Seattle)	VTX-2337	A small molecule that targets Toll-like receptor 8	Recurrent or persistent epithelial ovarian, fallopian tube or primary peritoneal cancer	Completed enrollment in GOG-3003 (NCT01666444), a randomized, placebo-controlled phase II trial of VTX-2337 in combination with pegylated liposomal doxorubicin	4/18/14

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CARDIOVASCULAR					
Arca Biopharma Inc. (Westminster, Colo.)	Gencaro	Bucindolol hydrochloride	To prevent atrial fibrillation in patients with heart failure or left ventricular dysfunction	Genetically screened the first patient in its phase IIb/III trial	4/18/14
Glycomimetics Inc. (Gaithersburg, Md.)	GMI-1070	Rivipansel	Vaso-occlusive crisis in people with sickle cell disease	Phase II data showed it improved efficacy outcomes independent of hydroxyurea	4/15/14
Isis Pharmaceuticals Inc. (Carlsbad, Calif.)	ISIS-APOCIIIRx	An antisense drug	High triglycerides	Phase II data showed patients treated achieved average reductions of as much as 71% in apolipoprotein C-III a component of very low density lipoprotein, up to 64% in triglycerides, and average increases of up to 52% in high-density lipoprotein cholesterol	4/1/14
CENTRAL NERVOUS SYSTEM					
Alder Biopharmaceuticals Inc. (Bothell, Wash.)	ALD403	Genetically engineered monoclonal antibody that targets calcitonin gene-related peptide	To prevent migraine	A randomized, double-blind, placebo-controlled proof-of-concept trial showed it met the primary endpoint of the study by significantly reducing mean migraine days per month vs. placebo during weeks five to eight	4/24/14
Ampio Pharmaceuticals Inc. (Greenwood Village, Colo.)	Ampion	Single intra-articular injection	Inflammation-associated pain in symptomatic osteoarthritis of the knee	In a subgroup of patients with moderate to severe OAK, there were statistically significant improvements in WOMAC pain ($p = 0.005$) and function scores ($p = 0.04$) over 20 weeks	4/10/14
Brainstorm Cell Therapeutics Inc. (New York and Petach Tikvah, Israel)	Nurown	Autologous mesenchymal stem cells secreting neurotrophic factors	Amyotrophic lateral sclerosis	FDA approved commencement of its phase II trial	4/29/14
Civitas Therapeutics Inc. (Chelsea, Mass.)	CVT-301	An inhaled formulation of levodopa	Parkinson's disease	Reported positive results from a phase IIb trial of CVT-301 showing the study met its primary endpoint	4/29/14
Cytokinetics Inc. (South San Francisco)	Tirasemtiv	Fast skeletal muscle troponin activator	Amyotrophic lateral sclerosis	Failed in a phase IIb trial	4/28/14

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Genervon Biopharmaceuticals LLC (Pasadena, Calif.)	GM604	Experimental therapy	Amyotrophic lateral sclerosis	Phase IIa data showed that at 10 weeks following completion of dosing clinical measurements of ALS progression remained the same in two of eight patients, while the rates of degradation of those clinical measurements had slowed in five of the remaining six treated patients	4/29/14
Intra-Cellular Therapies Inc. (New York)	ITI-007	5-HT2A receptor antagonist	Schizophrenia	Early data from a phase II study reveal that low doses of the drug show it acts as a potent 5-HT2A receptor antagonist, and that, as the dose is increased, it gradually engages other key brain receptors with regional selectivity	4/8/14
Prana Biotechnology Ltd. (Melbourne, Australia)	PBT2	Imaging agent	Alzheimer's disease	The phase II Imaging trial, IMAGINE, did not meet its primary endpoint of a statistically significant reduction in the levels of beta-amyloid plaques	4/1/14
DIABETES					
Adocia SAS (Lyon, France)	Biochaperone Lispro	Ultra-fast formulation of insulin	Diabetes	Phase IIa data showed that in comparison to Humalog (Eli Lilly and Co.) commercial insulin, it met the primary endpoint and showed a significant increase in Biochaperone Lispro bioavailability in the first half-hour compared to Humalog	4/10/14
Concert Pharmaceuticals Inc. (Lexington, Mass.)	CTP-499	A multi-subtype selective inhibitor of phosphodiesterases	Diabetic kidney disease	Missed the primary endpoint in a phase II trial of CTP-499 in patients with diabetic kidney disease, but the 48-week results showed some promise in a longer treatment duration	4/28/14
Islet Sciences Inc. (Raleigh, N.C.) and **BHV Pharma Inc.** (Research Triangle Park, N.C.)	Remogliflozin etabonate	A selective SGLT2 inhibitor	Type 2 diabetes and nonalcoholic steatohepatitis	Two 12-week phase IIb studies showed that at week 12, twice-daily dosing of remogliflozin etabonate produced a statistically significant trend in dose response for change from baseline with changes in HbA1c ranging from -1.0 to -1.4%; once-daily dosing demonstrated a trend in dose response for change from baseline in HbA1c above the lowest dose with changes in HbA1c ranging from -0.5 to -0.8%	4/17/14

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Lexicon Pharmaceuticals Inc. (The Woodlands, Texas)	LX4211	Sodium glucose transporter inhibitor	Type 1 diabetes	Phase II data showed it met the primary endpoint of reducing mealtime insulin use as well as several secondary endpoints	4/15/14
Noxxon Pharma AG (Berlin)	NOX-E36	Emapticap pegol	Diabetic nephropathy	Phase IIa proof-of-concept data showed statistically significant reductions in urinary albumin excretion and improved glycemic control	4/7/14
Oramed Pharmaceuticals Inc. (Jerusalem)	OMRD-0801	An orally ingestible insulin capsule	Type 2 diabetes	Phase IIa data showed that the oral insulin appeared to be safe and well tolerated for the dosing regimen tested	4/25/14
Xeris Pharmaceuticals Inc. (Austin, Texas)	G-Pump	Stable liquid glucagon in an Insulet Corp. Omnipod infusion pump	Type 1 diabetes	Dosed the first subject in a phase II study designed to treat hypoglycemia	4/28/14
GASTROINTESTINAL					
Athersys Inc. (Cleveland)	Multistem	Stem cell product	Treatment-refractory ulcerative colitis	Product failed to moderate the severity of disease in a phase II study of 128 patients; it did not show statistically significant improvement compared to placebo	4/29/14
INFECTION					
Arrowhead Research Corp. (Pasadena, Calif.)	ARC-520	RNAi therapeutic	Chronic hepatitis B infection	The first cohort of eight patients was fully enrolled and dosed in a phase IIa trial	4/2/14
Gilead Sciences Inc. (Foster City, Calif.)	Sovaldi	Sofosbuvir; nucleotide analogue polymerase inhibitor	Hepatitis C virus	Phase II data of 400 mg of Sovaldi with the NS5A inhibitor ledipasvir 90 mg, with and without ribavirin twice-daily, showed 100% of treatment-naïve genotype 3 patients who got 12 weeks of the combo plus ribavirin, and 64% of the same patients without ribavirin achieved SVR 12 weeks after treatment	4/11/14
Medivir AB (Stockholm)	Simeprevir	Protease inhibitor	Genotype 1 hepatitis C virus infection	Phase II data from cohort 2 in the interferon-free COSMOS study showed that 93% of prior null responder and treatment-naïve patients achieved sustained virologic responses 12 weeks after the end of treatment with simeprevir and Sovaldi (sofosbuvir, Gilead Sciences Inc.)	4/15/14

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MISCELLANEOUS					
Akebia Therapeutics Inc. (Cambridge, Mass.)	AKB-6548	A hypoxia-inducible factor prolyl hydroxylase inhibitor	Anemia associated with chronic kidney disease	Completed enrollment in its ongoing 200-patient phase IIb study	4/16/14
Auris Medical AG (Basel, Switzerland)	AM-101	An N-methyl-d-aspartate (NMDA) receptor antagonist	Acute inner ear tinnitus	Phase IIb data demonstrated it was well tolerated and safe and established proof of concept	4/1/14
Celgene Corp. and **Acceleron Pharma Inc.** (Cambridge, Mass.)	Sotatercept	Formerly known as ACE-011; targets TGF-beta molecules	Anemia in end-stage renal disease	Interim phase IIa data showed dose-dependent increases in hemoglobin in patients on hemodialysis	4/25/14
Isis Pharmaceuticals Inc. (Carlsbad, Calif.)	ISIS-SMNRx	An antisense drug	Spinal muscular atrophy	Phase II data showed that of seven infants, five are alive without the need for permanent ventilation; in another phase II study in children with SMA encouraging preliminary results were also observed in two additional functional tests: the six-minute walk test and the upper limb module test	4/30/14
Ohr Pharmaceutical Inc. (New York)	Squalamine	An anti-angiogenic small molecule that counteracts multiple growth factors	Wet form age-related macular degeneration	Completed enrollment of its phase II trial with 142 patients	4/30/14
Rxi Pharmaceuticals Corp. (Marlborough, Mass.)	RXI-109	An sd-rxRNA compound that targets connective tissue growth factor	Abnormal dermal scars	The first patient has enrolled in a phase IIa study	4/30/14
Scioderm Inc. (Durham, N.C.)	SD-101	A topical therapy	Blisters and lesions in patients with epidermolysis bullosa	Completed enrollment in a phase IIb study (SD-003) to evaluate the safety and efficacy of SD-101; the study enrolled a total of 48 subjects, ages 6 months and older	4/2/14
RESPIRATORY					
Anergis SA (Epalinges, Switzerland)	Allert	Birch pollen allergy vaccine	Birch pollen allergy	The long-term efficacy trial is fully enrolled, including 196 patients enrolled that participated in the 2013 field-based phase IIb trial of Allert	4/24/14
DBV Technologies SA (Bagneux, France)	Viaskin	Epicutaneous immunotherapy	Peanut allergy	A patient enrolled in the phase II ARACHILD study who received 18-month epicutaneous immunotherapy with Viaskin remained desensitized to peanut allergy after one year off-treatment, with a strict peanut diet	4/17/14

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Company (location)	Product	Description	Indication	Status	Date
Novavax Inc. (Gaithersburg, Md.)	RSV-F	Protein nanoparticle vaccine candidate	Respiratory syncytial virus	Phase II data in 720 healthy women of childbearing age showed it was well tolerated with no serious adverse events; it also demonstrated significant increases in RSV-F antibody levels across all doses and formulations, as well as clear increases in RSV neutralizing antibodies	4/30/14
Theravance Inc. (South San Francisco)	TD-4208	A nebulized aqueous solution	Moderate-to-severe chronic obstructive pulmonary disease	Started a dose-ranging phase IIb study	4/14/14

NOTES

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Phase III clinical trials update
April 2014

Company (location)	Product	Description	Indication	Status	Date
AUTOIMMUNE					
Anacor Pharmaceuticals Inc. (Palo Alto, Calif.)	AN2728	Boron-based phospho-diesterase-4 inhibitor	Atopic dermatitis	Enrolled the first patient in a pair of pivotal phase III trials	4/1/14
Biogen Idec Inc. (Cambridge, Mass.)	Plegridy	Peginterferon beta-1a	Relapsing multiple sclerosis	Two-year phase III data from the ADVANCE trial demonstrated favorable results on relapse rates, magnetic resonance imaging findings and disease progression when dosed every two weeks	4/30/14
Catalyst Pharmaceutical Partners Inc. (Coral Gables, Fla.)	Firdapse	Amifampridine phosphate	Lambert-Eaton myasthenic syndrome	Reached patient enrollment target for its pivotal phase III	4/2/14
CANCER					
Agenus Inc. (Lexington, Mass.) and **Glaxosmithkline plc** (London)	MAGE-A3ii	Cancer immuno-therapeutic that contains QS-21 Stimulon adjuvant	Non-small-cell lung cancer	Companies are terminating the phase III MAGRITi study after it missed both the first and second co-primary endpoints	4/3/14
Ambit Biosciences Inc. (San Diego)	Quizartinib	An oral, once-daily selective inhibitor of FLT3	Relapsed/ refractory acute myeloid leukemia	Initiated the phase III QUANTUM-R study comparing quizartinib as a monotherapy to chemotherapy regimens in patients with the FMS-like tyrosine kinase-3 (FLIT3)-ITD mutation	4/9/14
Amgen Inc. (Thousand Oaks, Calif.)	T-vec	Cancer immunotherapy talimogene laherparepvec	Melanoma	Phase III secondary endpoint data on overall survival showed a "strong trend" despite a statistical miss; the trend in favor of T-vec achieved a "p" value of 0.051	4/7/14
Celsion Corp. (Lawrenceville, N.J.)	Thermodox	Heat-sensitive liposome anticancer platform	Cancer	Final trial results from a 701-patient trial suggest that it may significantly improve overall survival, compared to a control, in patients whose lesions undergo the radiofrequency ablation treatment for 45 minutes or more	4/11/14
Heron Therapeutics Inc. (Redwood City, Calif.)	Sustol	Formerly APF530; 5-HT3 receptor antagonist	To prevent chemotherapy-induced nausea and vomiting	Started a phase III label expansion study	4/1/14

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Oncogenex Pharmaceuticals Inc. (Bothell, Wash.) and **Teva Pharmaceutical Industries Ltd.** (Jerusalem)	Custirsen	A drug designed to block production of clusterin	Metastatic castrate-resistant prostate cancer	Failed to perform better than standard chemotherapy in the pivotal Synergy trial; adding custirsen to docetaxel and prednisone did not significantly improve overall survival for the 1,022 men	4/29/14
Oncolytics Biotech Inc. (Calgary, Alberta)	Reolysin	Oncolytic virus candidate	Second-line, platinum-refractory, taxane-naïve head and neck cancers	The phase III REO 018 study testing Reolysin in combination with carboplatin and paclitaxel showed a statistically significant improvement (p = 0.0072); an analysis of overall survival also demonstrated a statistically significant improvement in the Reolysin arm vs. control arm (p = 0.0146)	4/9/14
Oncothyreon Inc. (Seattle) and **Merck KGaA** (Darmstadt, Germany)	Tecemotide	Cancer vaccine	Unresectable, locally advanced stage III non-small-cell lung cancer	Started a phase III trial	4/8/14
Onyx Pharmaceuticals Inc. (South San Francisco) and **Bayer Healthcare Pharmaceuticals Inc.** (Whippany, N.J.)	Nexavar	Sorafenib tablets	Locally recurrent or metastatic, progressive, differentiated thyroid carcinoma	Results from the phase III DECISION trial demonstrated that Nexavar tablets significantly extended the time patients lived without their disease worsening	4/25/14
Sorrento Therapeutics Inc. (San Diego)	Cynviloq	Paclitaxel polymeric micelle	Metastatic breast cancer and non-small-cell lung cancer	The first patient was dosed in the pivotal trial, TRIBECA (TRIal designed to evaluate bioequivalence between Cynviloq and Abraxane)	4/2/14
CARDIOVASCULAR					
Amgen Inc. (Thousand Oaks, Calif.)	Evolocumab	Protein convertase subtilisin/kexin type 9 inhibitor	Cholesterol	Phase III data confirmed phase II results showing evolocumab brought down low-density lipoprotein cholesterol by 50-60%	4/1/14
Armetheon Inc. (Sunnyvale, Calif.)	Tecarfarin	Oral anticoagulant therapy	Prosthetic heart valves	Reached an agreement with the FDA on an SPA for the final pivotal trial of tecarfarin	4/30/14
Biogen Idec Inc. (Cambridge, Mass.) and **Swedish Orphan Biovitrum AB** (Stockholm)	Eloctate	Recombinant factor VIII Fc fusion protein	Hemophilia A	Positive top-line results of the Kids A-LONG phase III trial showed it was generally well tolerated and no neutralizing antibodies that might interfere with its activity were detected	4/11/14

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Portola Pharmaceuticals Inc. (South San Francisco)	Betrixaban	Once-daily Factor Xa anticocagulant	Critical cardiovascular conditions	Launched a global, pivotal phase III trial to determine whether extended preventive treatment can prevent blood clots in the leg and lung that can occur in hospitalized cardiovascular disease patients	4/15/14
Regado Biosciences Inc. (Basking Ridge, N.J.)	REG1	Antithrombotic drug	For patients undergoing percutaneous coronary intervention, or angioplasty	Company is expanding enrollment in the phase III trial comparing REG1 to Angiomax (bivalirudin, the Medicines Co.) after achieving its 1,000-patient enrollment milestone	4/4/14
CENTRAL NERVOUS SYSTEM					
Alkermes plc (Dublin)	Aripiprazole lauroxil	Long-acting injectable antipsychotic	Schizophrenia	Favorable phase III data demonstrated statistically significant reductions from baseline in Positive and Negative Syndrome Scale total scores at week 12, compared to placebo	4/9/14
Avanir Pharmaceuticals Inc. (Aliso Viejo, Calif.)	Nuedexta	Dextromethorphan HBr and quinidine sulfate	Pseudobulbar affect	Phase III data showed that 19% of treated patients achieved remission from symptoms after one week; 50% of patients achieved remission by the end of the 12-week trial	4/29/14
Avanir Pharmaceuticals Inc. (Aliso Viejo, Calif.)	AVP-825	A breath-powered powder sumatriptan intranasal treatment	Severe migraine pain	Migraine relief for some patients began as early as 15 minutes following treatment, and a statistically significant greater number of treated patients experienced headache relief compared to placebo at all time points from 30 minutes through two hours	4/29/14
Cytokinetics Inc. (South San Francisco)	Tirasemtiv	A novel skeletal muscle activator	Amyotrophic lateral sclerosis	Results from BENEFIT-ALS showed that of the 711 patients enrolled in the trial, 605 were randomized to double-blind treatment with either tirasemtiv or placebo for 12 weeks; the trial did not achieve its primary efficacy endpoint,, but did result in a statistically significant and potentially clinically meaningful reduction in the decline of slow vital capacity	4/30/14

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Ironshore Pharmaceuticals & Development Inc. (subsidiary of Highland Therapeutics Inc.; Toronto)	HLD-200	Formulation of the stimulant methylphenidate	Attention-deficit hyperactivity disorder	Began enrollment in a phase III study of HLD-200	4/29/14
Navidea Biopharmaceuticals Inc. (Dublin, Ohio)	Lymphoseek	Technetium Tc 99m tilmanocept	Pain	Navidea is testing Lymphoseek injection in a head-to-head study with radiolabeled sulfur colloid to see which drug causes patients less pain	4/21/14
DIABETES					
Biodelivery Sciences International Inc. (Raleigh, N.C.)	Clonidine	Topical gel	Pain associated with diabetic neuropathy	Enrolled the first patient in a phase III trial	4/4/14
Evoke Pharma Inc. (Solana Beach, Calif.)	EVK-001	An intranasal formulation of metoclopramide	Acute and recurrent diabetic gastroparesis	Enrolled the first patient in the phase III trial	4/23/14
INFECTION					
Abbvie Inc. (Chicago)	ABT-450	Ritonavir; fixed-dose combination of ABT-450 (150/100 mg) co-formulated with ombitasvir (ABT-267) 25 mg, dosed once daily, and dasabuvir (ABT-333) 250 mg with or without RBV, dosed twice daily	Hepatitis C virus infection	Phase III data showed patients achieved sustained virologic response rates of 91.8% and 95.9% in the 12-week and 24-week treatment arms, respectively	4/15/14
Durata Therapeutics Inc. (Chicago)	Dalvance	Dalbavancin	Acute bacterial skin and skin structure infections caused by susceptible gram-positive bacteria	Durata began enrollment of a phase IIIb trial to evaluate the efficacy and safety of Dalvance for injection, in a single 1,500-mg dose infused over 30 minutes	4/25/14
Dynavax Technologies Corp. (Berkeley, Calif.)	Helisav-B	Vaccine that combines hepatitis B surface antigen with a Toll-like receptor 9 agonist	Hepatitis B virus	Started a phase III trial	4/16/14

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Emergent Biosolutions Inc. (Rockville, Md.)	Biothrax	Anthrax vaccine adsorbed post-exposure prophylaxis	Anthrax	Completed the last licensure-enabling study in its Biothrax post-exposure prophylaxis program; it met both its primary and secondary endpoints	4/17/14
Enanta Pharmaceuticals Inc. (Watertown, Mass.) and **Abbvie Inc.** (Chicago)	ABT-450, ABT-267 and ABT-333	Three direct-acting antiviral regimen, boosted protease inhibitor ABT-450/ritonavir, NS5A inhibitor ABT-267 and non-nucleoside polymerase inhibitor ABT-333	Chronic genotype 1 hepatitis C virus infection	Pivotal phase III SAPPHIRE-I and II study data showed patients achieved sustained virologic response rates 12 weeks post-treatment of 96.2% (n = 455/473) and 96.3% (n = 286/297), respectively	4/14/14
Gilead Sciences Inc. (Foster City, Calif.)	Sovaldi	Sofosbuvir	Genotype 2 chronic hepatitis C virus infection	Positive top-line results from a phase III trial of Sovaldi with ribavirin showed it met its primary efficacy endpoint of superiority compared to a predefined historical control sustained virologic response rate	4/4/14
Medivir AB (Stockholm)	Simeprevir	NS3/4A protease inhibitor	Chronic genotype 1 hepatitis C virus	Two phase III trials are recruiting patients to study simeprevir in combination with sofosbuvir in treatment-naïve and treatment-experienced patients with and without cirrhosis	4/3/14
Alexion Pharmaceuticals Inc. (Cheshire, Conn.)	Soliris	Eculizumab	Relapsing neuromyelitis optica and refractory generalized myasthenia gravis	Initiated separate multinational, placebo-controlled trials to evaluate the safety and efficacy of eculizumab in patients	4/25/14
MISCELLANEOUS					
Amicus Therapeutics Inc. (Cranbury, N.J.)	Migalastat	The oral, small-molecule chaperone for alpha-galactosidase A	Fabry disease	Phase III data showed that subjects who switched from placebo to migalastat after month six turned up a statistically significant reduction in kidney interstitial capillary GL-3 at month 12 (p = 0.013), and those who stayed on migalastat for 12 months showed a durable reduction	4/30/14

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Auxilium Pharmaceuticals Inc. (Chesterbrook, Pa.) and **Swedish Orphan Biovitrum AB** (Stockholm)	Xiaflex	Collagenase clostridium histolyticum (CCH)	Peyronie's disease	Additional phase III data showed improved clinical outcomes; adverse events were mostly localized to the penis, non-serious and resolved without intervention before the next injection	4/15/14
Biomarin Pharmaceutical Inc. (San Rafael, Calif.) and **Merck KGaA** (Darmstadt, Germany)	Kuvan	Sapropterin dihydrochloride	Phenylketonuria	Phase IIIb SPARK data showed it met its primary endpoint	4/25/14
Intercept Pharmaceuticals Inc. (New York)	OCA	Obeticholic acid	Primary biliary cirrhosis	Phase III POISE trial data showed it met its primary endpoint ($p < 0.0001$)	4/15/14
Lipocine Inc. (Salt Lake City)	LPCN 1021	Oral testosterone undecanoate	Hypogonadal men with low testosterone	Completed enrollment of its SOAR (Study of Oral Androgen Replacement) pivotal phase III study	4/30/14
Pacira Pharmaceuticals Inc. (Parsippany, N.J.)	Exparel	Bupivacaine liposome injectable suspension	Total knee arthroplasty	Phase III data supported the safety and efficacy to achieve a femoral nerve block in patients; there was a 24% reduction in total opioid use	4/8/14
Repros Therapeutics Inc. (The Woodlands, Texas)	Androxal	Enclomiphene	Testosterone deficiency	Reached its enrollment goal of 120 subjects in the first of two head-to-head studies comparing Androxal to the leading U.S. testosterone replacement therapy	4/15/14
Revo Biologics Inc. (Framingham, Mass.)	Atryn	Antithrombin, recombinant	Preeclampsia	Started a phase III program in pregnant women during the 24th to 28th week of pregnancy	4/1/14
Xoma Corp. (Berkeley, Calif.)	XOMA 052	Gevokizumab	Pyoderma gangrenosum	Based on its meeting with the FDA, plans are being finalized for a gevokizumab phase III program expected to include two double-blind, placebo-controlled clinical studies	4/29/14
RESPIRATORY					
Theravance Inc. (South San Francisco) and **Glaxosmithkline plc** (London)	Fluticasone furoate and vilanterol	Combination of inhaled corticosteroid fluticasone furoate and long-acting beta2 agonist vilanterol	Chronic obstructive pulmonary disease	Started a phase III study testing the combination treatment, FF/VI 100/25 mcg once daily, compared to VI 25 mcg once daily, administered via the Ellipta inhaler	4/23/14

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SPA = Special protocol assessment.

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American Association for Cancer Research
April 2014

Company (location)	Product	Description	Indication	Status	Date
Agios Pharmaceuticals Inc. (Cambridge, Mass.)	AG-221	Isocitrate dehydrogenase-2-targeting candidate	Relapsed or refractory	Phase I data showed complete remissions in three of seven evaluable patients	4/8/14
Bergenbio A/S (Bergen, Norway)	BGB324	A small-molecule inhibitor of the Axl receptor tyrosine kinase	Cancer	Phase Ia data showed the drug to be safe and well tolerated in doses up to 1.5 g daily with a predictable pharmacokinetics profile and long plasma half-life, allowing for different dosing options	4/10/14
Bind Therapeutics Inc. (Cambridge, Mass.)	BIND-014	A targeted polymeric nanoparticle containing cytotoxic agent docetaxel	Prostate cancer	Phase I data demonstrated a greater dose intensity by about 50% at once weekly for three weeks dosing (Q1W), as compared to once every week dosing described in a previous study	4/9/14
Calithera Biosciences Inc. (South San Francisco)	CB-839	Selective, oral glutaminase inhibitor	Cancer	Phase I data showed that a 100-mg dose resulted in peak plasma levels of drug at or above 300 nm and generated a greater than 80% inhibition of glutaminase in platelets four hours after dosing	4/9/14
Deciphera Pharmaceuticals LLC (Lawrence, Kan.) and **Eli Lilly and Co.** (Indianapolis)	LY3009120	Pan-RAF inhibitor; a small-molecule kinase inhibitor	Cancer	Started a phase I trial	4/8/14
Delmar Pharmaceuticals Inc. (Vancouver, British Columbia)	VAL-083	A bifunctional alkylating agent	Refractory glioblastoma multiforme	Data from its ongoing phase I/II trial showed that the drug is well tolerated, with no drug-related serious adverse events detected in 26 patients treated to date	4/10/14
Immunogen Inc. (Waltham, Mass.)	IMGN853	A targeted antibody payload compound	Cancer	Clinical activity was seen starting at doses of 3.3 mg/kg, and its dose-limiting toxicity was the reversible ocular side effects (blurred vision and keratitis) reported with antibody-drug conjugates (ADCs)	4/10/14
Immunomedics Inc. (San Diego)	IMMU-130	Anti-CEACAM5 antibody conjugated to the irinotecan-metabolite, SN-38	Metastatic colorectal cancer	Phase I data showed it was therapeutically active in all three trials, but a more frequent dosing schedule was more active in patients	4/8/14

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ISA Pharmaceuticals BV (Leiden, the Netherlands)	ISA101	Cancer vaccine	Advanced cervical carcinoma	Phase I data showed that ISA101 produced strong immune responses in almost all of the advanced cervical carcinoma patients treated	4/11/14
Lion Biotechnologies Inc. (Los Angeles)	TILs	Tumor infiltrating lymphocytes	Stage 4 metastatic melanoma	Data showed evidence of clinical efficacy from a phase II trial	4/8/14
Merck & Co. Inc. (Whitehouse Station, N.J.)	MK-3475	Anti-PD-1 immunotherapy	Advanced melanoma and advanced non-small-cell lung cancer	Responses were observed in patients with PD-L1 negative tumors; although the preliminary findings for both tumor types suggest that higher PD-L1 expression was associated with higher overall response rates	4/8/14
Merrimack Pharmaceuticals Inc. (Cambridge, Mass.)	MM-398	A nanoliposomal version of irinotecan	Cancer	Phase I data demonstrated that Feraheme was well tolerated	4/9/14
Mirna Therapeutics Inc. (Austin, Texas)	MRX34	Double-stranded microRNA mimic of naturally occurring tumor suppressor miR-34	Unresectable primary liver cancer	Interim safety data from an open-label phase I study showed that the drug has a manageable safety profile	4/9/14
Noxxon Pharma AG (Berlin)	NOX-H94	Lexaptepid pegol; a Spiegelmer designed to bind and neutralize hepcidin	Anemic cancer	Data from a pilot phase IIa study showed significant increases in hemoglobin levels (greater than 1 g/dL) in five of 12 patients (42%) in response to monotherapy	4/9/14
Oncoethix SA (Lausanne, Switzerland)	OTX015	Synthetic small-molecule inhibitor of BET bromodomain proteins 2/3/4	Hematologic malignancies	Interim results from an ongoing phase I trial showed linear pharmacokinetics and excellent tolerance	4/9/14
Pfizer Inc. (New York)	Palbociclib	CDK inhibitor	Metastatic estrogen receptor-positive breast cancer	Phase II PALOMA-1 data showed it doubled progression-free survival in an open-label phase II trial	4/8/14
Provectus Biopharmaceuticals Inc. (Knoxville, Tenn.)	PV-10	A 10% solution of Rose Bengal	Melanoma	Data showed significant decrease in melanoma cells in patients' injected tumors 7-14 days after intralesional PV-10 treatment that was accompanied by similar decrease in uninjected bystander tumors	4/8/14
Puma Biotechnology Inc. (Los Angeles)	PB272	Neratinib	Breast cancer	Phase II data showed an estimated pCR rate of 55.6% compared to the control arm, which had an estimated pCR rate of 32.6%	4/8/14

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Company (location)	Product	Description	Indication	Status	Date
Sunesis Pharmaceuticals Inc. (South San Francisco)	Vosaroxin	Quinolone derivative	Acute myeloid leukemia and high-risk myelodysplastic syndrome	An ongoing phase Ib/II University of Texas MD Anderson Cancer Center-sponsored trial of vosaroxin in combination with decitabine in older patients showed that the combination has been found to be effective and well tolerated	4/9/14
Tolero Pharmaceuticals Inc. (Salt Lake City)	Alvocidib	A cyclin-dependent kinase	Chronic lymphocytic leukemia	In 62 patients, patient response was correlated with mitochondrial response from pre-treatment specimens to peptides known to interact with proteins that promote cell survival: Bim, Noxa, Bad, Bmf and Hrk	4/8/14
Viralytics Ltd. (Sydney)	Cavatak	Oncolytic immunotherapy	Advanced melanoma	Interim results from the ongoing phase II CALM trial showed antitumor activity in non-injected metastatic tumors in patients participating in the trial, and investigators reported partial or complete reduction of non-injected tumors in multiple patients who had been on treatment at least eight weeks	4/9/14

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Pharma clinical and FDA action update
April 2014

Company (location)	Product	Description	Indication	Status	Date
Abbvie Inc. (North Chicago, Ill.)	ABT-888	Veliparib	Locally advanced or metastatic squamous non-small-cell lung cancer	Started a global phase III trial	4/16/14
Astrazeneca plc (London)	Byetta	Glucagon-like peptide 1 receptor agonist exenatide	Parkinson's disease	New results showed that those who previously had been exposed to exenatide had an advantage of 5.6 points over their controlled-tested counterparts when using the blinding MDS-UPDRS motor subscale	4/16/14
Astrazeneca plc (London) and Pfizer Inc. (New York)	12 drugs	12 drugs targeted to specific mutations	Advanced lung cancer	Opened a phase II trial testing 12 drugs in a single study	4/22/14
Bayer AG (Leverkusen, Germany)	Ciprofloxacin	Dry powder for inhalation	Non-cystic fibrosis bronchiectasis	FDA granted orphan drug designation	4/24/14
Bayer Healthcare (Whippany, N.J.)	Xofigo	Radium Ra 223 dichloride	Bone predominant metastatic castration-resistant prostate cancer	Began enrollment in a phase III trial studying Xofigo injection in combination with abiraterone acetate and prednisone/ prednisolone	4/3/14
Boehringer Ingelheim GmbH (Ingelheim, Germany)	Volasertib	A Polo-like kinase 1 inhibitor	Acute myeloid leukemia	FDA and European Commission granted orphan designation	4/18/14
Boehringer Ingelheim GmbH (Ingelheim, Germany)	Pradaxa Â	Dabigatran etexilate	Deep vein thrombosis and pulmonary embolism	The CHMP of the EMA issued a positive opinion recommending approval of Pradaxa Â; FDA approved it in patients who have been treated with a parenteral anticoagulant for five to 10 days, and to reduce the risk of recurrent DVT and PE in patients who have been previously treated	4/29/14
Boehringer Ingelheim GmbH (Ingelheim, Germany) and Eli Lilly and Co. (Indianapolis)	Epagliflozi-nand linagliptin	Combination tablet; sodium glucose co-transporter-2 inhibitor and a dipeptidyl peptidase-4 inhibitor	Type 2 diabetes	FDA accepted for filing an NDA	4/15/14

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Bristol-Myers Squibb Co. (New York)	Daclatasvir and asunaprevir	An NS5A replication complex inhibitor and a NS3 protease inhibitor	Genotype 1b hepatitis C virus	Submitted an NDA; phase III data showed that DCV plus ASV given in a 24-week regimen gained SVR12 among treatment-naïve patients (90%), peg-interferon/ribavirin non-responders (82%) and peg-interferon/ribavirin ineligible or intolerant (82%)	4/8/14; 4/11/14
Bristol-Myers Squibb Co. (New York) and Gilead Sciences Inc. (Foster City, Calif.)	Atazanavir sulfate and cobicistat	A protease inhibitor and pharmacokinetic enhancer	HIV	Submitted an NDA	4/15/14
Daiichi Sankyo Co. Ltd. (Tokyo)	Edoxaban	Oral, once-daily direct factor Xa inhibitor	To prevent stroke and blood clot complications	Began enrolling patients into the phase III ENSURE-AF study	4/1/14
Dompe Group (Milan)	rhNGF	Recombinant human nerve growth factor eye drops	Retinitis pigmentosa	Started a phase Ib/II study to assess the safety and potential efficacy of the eye drops in two doses vs. placebo	4/4/14
Eisai Co. Ltd. (Tokyo)	Zonegran	Zonisamide	Partial epilepsy	Phase III data showed it was well tolerated and efficacious	4/23/14
Eli Lilly and Co. (Indianapolis)	Cyramza	Ramucirumab; a vascular endothelial growth factor receptor 2 antagonist	Advanced or metastatic gastric cancer or gastroesophageal junction adenocarcinoma	FDA approved Cyramza as a single-agent treatment	4/23/14
Forest Laboratories Inc. (New York) and Gedeon Richter plc (Budapest, Hungary)	Cariprazine	An atypical antipsychotic	Depressive episodes of bipolar I disorder	Phase IIb data showed statistically significant improvements were observed in the 1.5 mg/day group relative to placebo at six weeks for the primary endpoint, the Montgomery-Asberg Depression Rating Scale total score, and the key secondary endpoint, the Clinical Global Impressions – Severity score	4/1/14
Glaxosmithkline plc (London)	Tanzeum	Albiglutide subcutaneous injection	Type 2 diabetes	FDA approved it	4/16/14
Glaxosmithkline plc (London)	Ellipta	Umeclidinium	Chronic obstructive pulmonary disease, including chronic bronchitis and emphysema	Received marketing authorization in Canada	4/18/14

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Company (location)	Product	Description	Indication	Status	Date
Ipsen SA (Paris)	Dysport	An injectable form of botulinum toxin type A	Upper limb spasticity	Phase III data demonstrated a statistically significantly higher proportion of responders in muscle tone improvement vs. placebo	4/15/14
Janssen Biotech Inc. (Horsham, Pa.)	Sylvant	Siltuximab; an interleukin-6 antagonist	Castleman's disease	FDA approved it	4/25/14
Janssen Research & Development LLC (Raritan, N.J.; part of Johnson & Johnson)	Prezista combination	Once-daily, fixed-dose antiretroviral combination tablet containing protease inhibitor Prezista (darunavir) and cobicistat	HIV	Submitted an NDA	4/2/14
Janssen-Cilag International (Beerse, Belgium)	Vokanamet	A fixed-dose therapy combining canagliflozin and immediate-release metformin hydrochloride in a single tablet	Type 2 diabetes mellitus	European Commission approved Vokanamet	4/28/14
Lipella Pharmaceuticals Inc. (Pittsburgh, Pa.)	LP-09	Topical botulinum toxin type A formulation	Overactive bladder	Clinical data showed significant reductions in both urinary urgency and urinary frequency	4/9/14
Merck & Co. Inc. (Whitehouse Station, N.J.)	MK-5172 and MK-8742	The NS3/4A protease inhibitor and an HCV NS5A replication complex inhibitor	Hepatitis C virus	Phase II data showed sustained viral responses with 12-week dosing in genotype 1 treatment-naïve patients reached 94% to 98%, even without ribavirin	4/11/14
Merck & Co. Inc. (Whitehouse Station, N.J.)	Grastek	Immunotherapy tablet	Grass pollen allergy	FDA approved it	4/16/14
Novartis AG (Basel, Switzerland)	LCZ696	Angiotensin receptor neprilysin inhibitor	Chronic heart failure with reduced ejection fraction	The data monitoring committee unanimously recommended early closure of the PARADIGM-HF phase III study, indicating patients with chronic heart failure with reduced ejection fraction (HF-REF) who received LCZ696 lived longer without hospitalization than those who received standard care	4/1/14
Novartis AG (Basel, Switzerland)	Zykadia	Ceritinib	Anaplastic lymphoma kinase-positive metastatic non-small-cell lung cancer	FDA approved it	4/30/14

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Company (location)	Product	Description	Indication	Status	Date
Pfizer Inc. (New York)	Tofacitinib	JAK inhibitor	Moderate to severe plaque psoriasis	Top-line phase III data showed that tofacitinb, as a 5-mg or 10-mg dose taken as a pill twice daily, met the primary endpoints of statistically significant superiority over placebo	4/23/14
Roche AG (Basel, Switzerland)	Roactemra	Tocilizumab; subcutaneous formulation	Moderate to severe active rheumatoid arthritis	Received a positive opinion from the CHMP	4/29/14
Sanofi Pasteur (vaccines division of Sanofi SA; Paris)	Vaccine	Dengue vaccine candidate	Dengue disease	Phase III data showed a reduction of 56% of dengue disease cases	4/29/14
Sanofi Pasteur MSD (Lyon, France)	Gardasil	Quadrivalent human papillomavirus vaccine	Papillomavirus	European Commission granted marketing authorization for a 2-dose schedule at 0 and six months in children ages 9 to 13	4/8/14
Sanofi SA (Paris)	Adacel	Tetanus toxoid, reduced diphtheria toxoid and acellular pertussis vaccine adsorbed	To prevent tetanus, diphtheria and pertussis	FDA expanded the approved age indication of Adacel for active booster immunization for the prevention of tetanus, diphtheria and pertussis as a single dose in subjects, ages 10 through 64	4/2/14
Teva Pharmaceutical Industries Ltd. (Jerusalem)	Generic Lovaza	Omega-3-acid ethyl esters capsules	Hypertri-glyceridemia	Approved in the U.S.	4/9/14
Teva Pharmaceutical Industries Ltd. (Jerusalem)	Pridopidine	An oral, small molecule	Huntington's disease	Enrolled the first patient in the Pride-HD study, a phase II trial	4/25/14
Teva Pharmaceutical Industries Ltd. (Jerusalem)	Duoresp Spiromax	A new dry-powder inhaler containing a budesonide and formoterol fumarate dehydrate	Asthma and chronic obstructive pulmonary disease	The European Commission has granted marketing authorization	4/30/14
Twi Pharmaceuticals Inc. (Taipei)	Generic Procardia XL	Nifedipine extended-release tablets	Chronic cardiovascular disease	FDA approved it	4/8/14
Upsher-Smith Laboratories Inc. (Maple Grove, Minn.)	USL261	Intranasal midazolam	Epilepsy	Phase I data demonstrated it was rapidly absorbed and exhibited a short half-life	4/30/14

NOTES

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Non-U.S. clinical trials and regulatory actions
April 2014

Company (location)	Product	Description	Indication	Status	Date
CANCER					
Bioalliance Pharma SA (Paris)	Livatag	Doxorubicin Transdrug	Advanced hepatocellular carcinoma	European independent board of experts in charge of the safety profile of the Relive phase III trial unanimously recommended continuing the study without modification	4/15/14
Cytrx Corp. (Los Angeles)	Aldoxorubicin	Combines doxorubicin with a linker-molecule that binds specifically to albumin	Advanced soft tissue sarcomas	Received orphan medicinal product designation from the European Commission	4/1/14
Erytech Pharma SA (Lyon, France)	Graspa/Ery-asp	An enzyme formulation of L-asparaginase	Acute myeloid leukemia	Was granted orphan drug designation by the FDA	4/3/14
E-Therapeutics plc (Oxford, UK)	ETS2101	Dexanabinol	Solid tumors	The UK phase Ia trial showed it was well tolerated at all six dose escalations with no serious diverse events	4/1/14
CARDIOVASCULAR					
Aegerion Pharmaceuticals Inc. (Cambridge, Mass.)	Lomitapide	Adjunct treatment	Homozygous familial hyperchol-erolemia	Patient enrollment was initiated in Japan in a phase III trial	4/10/14
Regeneron Pharmaceuticals Inc. (Tarrytown, N.Y.) and **Sanofi SA** (Paris)	Alirocumab	A monoclonal antibody targeting PCSK9	High cholesterol	The first phase II study in Japanese patients met its primary endpoint	4/2/14
CENTRAL NERVOUS SYSTEM					
Coronado Biosciences Inc. (Burlington, Mass.)	CNDO-201	Trichuris suis ova	Autism spectrum disorder	Launched in Jerusalem a randomized, double-blind, placebo-controlled, study of 60 patients ages 6 to 17	4/11/14
Intelgenx Corp. (St. Laurent, Quebec) and **Redhill Biopharma Ltd.** (Tel-Aviv, Israel)	Versafilm	Oral thin film formulation of rizatriptan	Migraine	Initiated a comparative bioavailability clinical study comparing Versafilm to the European reference drug	4/29/14

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Company (location)	Product	Description	Indication	Status	Date
Salix Pharmaceuticals Ltd. (Raleigh, N.C.) and **Progenics Pharmaceuticals Inc.** (Tarrytown, N.Y.)	Relistor	Methylnaltrexone bromide subcutaneous injection	Opioid-induced constipation in chronic noncancer pain	Submission to the EMA has been accepted for review	4/23/14
INFECTION					
Chongqing Zhifei Biological Products Co. Ltd. (Hong Kong)	AC-Hib vaccine	Vaccine against group A and C meningococcus and haemophilus influenzae type B	Infectious diseases	The CFDA gave the green light to Chongqing to launch its joint vaccine against group A and C meningococcus and haemophilus influenzae type B	4/17/14
INFLAMMATORY					
Glenmark Pharmaceuticals Ltd. (Mumbai, India)	GRC 27864	Targets microsomal prostaglandin E synthase-1	Chronic inflammatory diseases and pain management	Plans to begin phase I testing in the UK	4/4/14
MISCELLANEOUS					
Alexion Pharmaceuticals Inc. (Cheshire, Conn.)	Soliris	Eculizumab; a terminal complement inhibitor	To prevent graft rejection following solid organ transplantation	European Commission has granted an orphan drug designation	4/24/14
Alnylam Pharmaceuticals Inc. (Cambridge, Mass.)	ALN-TTRsc	RNAi drug	Transthyretin-mediated amyloidosis	EMA's Committee for Orphan Medicinal Products adopted a positive opinion	4/3/14
Apricus Biosciences Inc. (San Diego)	Vitaros	Topical version of vasodilator alprostadil	Erectile dysfunction	Approved in Luxembourg; approved in Spain	4/4/14; 4/25/14
Hospira Inc. (Lake Forest, Ill.)	Retacrit/ Silapo	Epoetin zeta; biosimilar	Renal anemia	European data showed it appeared to be as safe as other epoetin alfa products	4/29/14
Keryx Biopharmaceuticals Inc. (New York)	Zerenex	Ferric citrate coordination complex	Hyperphos-phatemia in patients with chronic kidney disease	EMA determined that the firm's MAA is valid	4/3/14
Thrombogenics NV (Leuven, Belgium)	Jetrea	Ocriplasmin	Vitreomacular traction	Was approved in Malaysia	4/18/14

NOTES

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FDA submissions, approvals & other actions

April 2014

Company (location)	Product	Description	Indication	Status	Date
CANCER					
Cubist Pharmaceutical Inc. (Lexington, Mass.)	CUDC-427	An oral, small-molecule SMAC mimetic	Solid tumors or lymphoma	FDA said its complete response submission to the November 2013 partial clinical hold showed it is safe to proceed; it was placed on partial clinical hold following the death of a patient who progressed to liver failure approximately one month following discontinuation of CUDC-427 dosing	4/1/14
Genmab A/S (Copenhagen) and **Glaxosmithkline plc** (London)	Arzerra	Ofatumumab; a CD20-directed monoclonal antibody	Chronic lymphocytic leukemia	FDA approved a supplemental BLA for the use of Arzerra in combination with chlorambucil for the treatment of previously untreated patients with CLL for whom fludarabine-based therapy is considered inappropriate	4/18/14
Kite Pharma Inc. (Santa Monica, Calif.)	N/A	An autologous engineered T-cell product designed to target CD19 expression	B-cell malignancies	FDA granted orphan drug designation	4/1/14
Pharmacyclics Inc. (Sunnyvale, Calif.)	Imbruvica	Ibrutinib	Chronic lymphocytic leukemia or small lymphocytic lymphoma	Submitted a supplemental NDA, based on data from the phase III RESONATE study, PYC-1112, a head-to-head comparison of Imbruvica vs. Arzerra (ofatumumab, Genmab A/S and Glaxosmithkline plc) in 391 patients	4/9/14
Tolero Pharmaceuticals Inc. (Salt Lake City)	Alvocidib	Flavopiridol	Acute myeloid leukemia	FDA granted orphan drug designation	4/24/14
CARDIOVASCULAR					
Biogen Idec Inc. (Cambridge, Mass.)	Alprolix	Coagulation Factor IX, Fc fusion protein	Hemophilia B	FDA approved it for the control and prevention of bleeding episodes, perioperative (surgical) management and routine prophylaxis in adults and children with hemophilia B	4/1/14
Dyax Corp. (Cambridge, Mass.)	Kalbitor	Ecallantide; peptide inhibitor of plasma kallikrein	Acute hereditary angioedema	Won FDA approval to treat patients older than 12 years	4/7/14

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Company (location)	Product	Description	Indication	Status	Date
The Medicines Co. (Parsippany, N.J.)	Fibrocabs	A dry powder formulation of fibrinogen and thrombin	To control mild or moderate bleeding	FDA accepted the filing of a BLA for hemostatic agent Fibrocabs	4/4/14
CENTRAL NERVOUS SYSTEM					
Impax Pharmaceuticals (division of Impax Laboratories Inc.; Hayward, Calif.)	IPX066	An extended-release capsule formulation of carbidopa and levodopa	Parkinson's disease	Resubmitted an NDA with updated safety and stability information	4/14/14
Intelgenx Corp. (Saint Laurent, Quebec) and **Redhill Biopharma Ltd.** (Tel Aviv, Israel)	Versafilm	Oral thin-film formulation of rizatriptan	Acute migraines	FDA acknowledged receipt of the companies' response to the complete response letter and has requested further clarifications	4/25/14
Kaleo Inc. (Richmond, Va.)	Evzio	Naloxone autoinjector	Opioid overdose	FDA approved it	4/4/14
Pozen Inc. (Chapel Hill, N.C.)	PA8140/ PA32540	Gastrointestinal-friendly aspirin candidates	Pain	FDA issued a complete response letter	4/29/14
Qrxpharma Ltd. (Sydney)	Moxduo	An immediate-release dual opioid (oxycodone and morphine)	Moderate to severe acute pain	FDA's Anesthetic and Analgesic Drug Products Advisory Committee voted to recommend against approval	4/24/14
DIABETES					
Mannkind Corp. (Valencia, Calif.)	Afrezza	Insulin human [rDNA origin]) powder	Type 1 and 2 diabetes	FDA's Endocrinologic and Metabolic Drugs Advisory Committee recommended approval	4/2/14
Psivida Corp. (Watertown, Mass.) and **Alimera Sciences Inc.** (Atlanta)	Iluvien	Fluocinolone acetonide intravitreal insert	Diabetic macular edema	The FDA set a PDUFA date of Sept. 26 for Iluvien following resubmission of the NDA	4/15/14
INFECTION					
Cubist Pharmaceutical Inc. (Lexington, Mass.)	Sivextro	Tedizolid	Acute bacterial skin and skin structure infections	FDA committee recommended approval, and a study specifically in adolescents and teens before the label is extended for pediatric use	4/1/14
Cubist Pharmaceuticals Inc. (Lexington, Mass.)	Ceftolozane/ tazobactam	Antibiotic	Complicated urinary tract infections and complicated intra-abdominal infections	Submitted an NDA	4/22/14

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Company (location)	Product	Description	Indication	Status	Date
Durata Therapeutics Inc. (Chicago)	Dalvance	Dalbavancin; a second-generation, semisynthetic lipoglycopeptide	Acute bacterial skin and skin structure infections	FDA committee recommended approval	4/1/14
Edmond Pharma Srl (Milan)	Erdosteine	A small-molecule thiol product	Bronchiectasis	FDA granted orphan drug designation	4/29/14
Emergent Biosolutions Inc. (Rockville, Md.)	Biothrax	Anthrax vaccine adsorbed	Anthrax disease	FDA has granted orphan drug designation	4/22/14
Enanta Pharmaceuticals Inc. (Watertown, Mass.) and **Abbvie Inc.** (Chicago)	ABT-450	Oral, interferon-free regimen	Chronic genotype 1 HCV infection	Submitted an NDA	4/23/14
Gilead Sciences Inc. (Foster City, Calif.)	Cobicistat	A pharmaco-enhancing, or boosting, agent that increases blood levels of the protease inhibitors atazanavir and darunavir, and the integrase inhibitor elvitegravir	HIV	FDA accepted the company's refiling of two NDAs	4/23/14
MISCELLANEOUS					
Sarepta Therapeutics Inc. (Cambridge, Mass.)	Eteplirsen	Exon-skipping candidate	Duchenne muscular dystrophy	FDA said the NDA "should be fileable" with existing data	4/22/14
RESPIRATORY					
Alk-Abello A/S (Copenhagen) and **Merck & Co. Inc.** (Whitehouse Station, N.J.)	Ragwitek	An allergen extract	Ragweed allergic rhinitis	FDA approved the BLA for Ragwitek as an immunotherapy for short ragweed pollen-induced allergic rhinitis, with or without conjunctivitis, confirmed by positive skin test or in vitro testing for pollen-specific IgE antibodies for short ragweed pollen	4/18/14
Greer Laboratories Inc. (Lenoir, N.C.)	Oralair	Under-the-tongue allergen extract	Allergic rhinitis	FDA approved Oralair to treat allergic rhinitis with or without conjunctivitis	4/3/14

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10 BIGGEST U.S. WINNERS FOR THE WEEK			
By Percent		By Dollars	
Bluebird Bio	38.05	Intercept Pharma	44.89
Poniard Pharma	37.50	Regeneron Pharma	16.02
Rexahn Pharma	22.78	Clovis Oncology	8.13
Avax Tech	20.00	Bluebird Bio	6.94
Intercept Pharma	19.07	Alexion Pharma	5.06
Stemcells	17.80	Biogen Idec	4.67
Clovis Oncology	17.00	Salix Pharma	3.95
Neurogesx	16.25	Forest Labs	3.78
Savient Pharma	16.13	Relypsa	3.10
Relypsa	15.82	Alnylam Pharma	2.97

10 BIGGEST U.S. LOSERS FOR THE WEEK			
By Percent		By Dollars	
Unigene	-37.50	Insys	-8.83
Sorrento Thera	-37.42	United Therap	-5.86
Kamada	-32.34	Puma Biotech	-5.10
Glycomimetics	-29.03	Receptos	-4.94
Insys	-27.02	Kamada	-4.34
Alseres	-25.00	Portola Pharma	-3.71
Dara Biosciences	-24.46	Ani Pharma	-3.53
Accentia Biopharma	-22.45	Agios Pharma	-3.35
Receptos	-16.02	Sorrento Thera	-2.99
NovaBay	-15.69	Newlink Genetics	-2.91

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BIOWORLD STOCK REPORT FOR PUBLIC BIOTECHNOLOGY COMPANIES

COMPANY	SYMBOL	CLOSE 5/9	CLOSE 5/16	%CHANGE WK	%CHANGE YTD	VOL (000)
Aastrom Biosci	ASTM	3.8	3.97	4.47	22.91	807
Acadia Pharma	ACAD	18.52	19.05	2.86	-23.77	7448
Acceleron Pharma	XLRN	31.87	31.98	0.35	-19.24	1565
Accentia Biopharma	ABPI	0.0049	0.0038	-22.45	26.67	93
Access Pharma	ACCP	0.44	0.44	0.00	76.00	358
Acelrx Pharma	ACRX	9.76	8.88	-9.02	-21.49	2717
Achaogen	AKAO	12.53	13.3	6.15	-6.99	451
Achillion	ACHN	2.79	2.67	-4.30	-19.58	5446
Acorda Therap	ACOR	30.17	30.97	2.65	6.06	2888
Adamas Pharma	ADMS	19.23	18.76	-2.44	33.90	155
Adamis	ADMP	6.19	6.79	9.69	6.26	1644
Adma Biologics	ADMA	7.35	7.5	2.04	-3.85	29
Advaxis	ADXS	2.65	2.65	0.00	-37.50	373
Aegerion Pharma	AEGR	32.62	31.76	-2.64	-55.24	4859
Aeolus	AOLS	0.25	0.25	0.00	8.70	126
Aerie Pharma	AERI	14.85	15.33	3.23	-14.64	290
Aeterna Zentaris	AEZS	1.05	1.08	2.86	-21.74	1854
Affymax	AFFY	0.6	0.67	11.67	-14.10	292
Affymetrix	AFFX	7.61	7.83	2.89	-8.63	5028
Agenus	AGEN	2.57	2.67	3.89	1.14	1674
Agios Pharma	AGIO	36.58	33.23	-9.16	38.75	1848
Akebia Thera	AKBA	22.21	21.53	-3.06	-19.36	536
Albany Molecular	AMRI	14.63	15.72	7.45	55.95	1662
Alcobra	ADHD	15.85	16.82	6.12	-6.56	263
Alexion Pharma	ALXN	152.87	157.93	3.31	18.85	5741
Alexza Pharma	ALXA	4.18	4.23	1.20	-10.57	329
Alimera Sciences	ALIM	5.17	5.14	-0.58	9.36	542
Alkermes	ALKS	44.13	43.59	-1.22	7.21	3852
Alnylam Pharma	ALNY	54.33	57.3	5.47	-10.89	6094
Alseres	ALSE	0.04	0.03	-25.00	50.00	25
Amag Pharma	AMAG	17.29	17.93	3.70	-26.15	2446
Amarin	AMRN	1.51	1.37	-9.27	-30.46	13082
Ambit Biosciences	AMBI	6.18	6.42	3.88	-33.40	134
Amgen	AMGN	111.48	111.94	0.41	-1.88	16948
Amicus	FOLD	2.13	2.17	1.88	-7.66	1244
Ampio Pharma	AMPE	7.04	7.24	2.84	1.54	3888
Ampliphi Biosci	APHB	0.56	0.51	-8.93	2.00	60
Anacor Pharma	ANAC	14.59	13.97	-4.25	-16.75	1340
Ani Pharma	ANIP	30.74	27.21	-11.48	35.51	797
Anthera Pharma	ANTH	2.73	2.99	9.52	-2.61	1475
Applied Genetic Tech	AGTC	12.1	13.1	8.26	-11.25	58
Aquinox Pharma	AQXP	9.97	10.23	2.61	-14.39	29
Aradigm	ARDM	0.2	0.2	0.00	17.65	236
Arena Pharma	ARNA	7.19	6.43	-10.57	9.91	29329
Argos Thera	ARGS	8.56	8.49	-0.82	6.13	65
Ariad Pharma	ARIA	6.67	6.36	-4.65	-6.74	33303
Arqule	ARQL	1.4	1.46	4.29	-32.09	2399
Array Biopharma	ARRY	3.94	4.02	2.03	-19.76	8266
Aryx	ARYX	0.0105	0.01	-4.76	0.00	24
Auspex Pharma	ASPX	16.45	16.53	0.49	5.56	520
Auxilium Pharma	AUXL	20.78	19.47	-6.30	-6.08	6330
Avanir	AVNR	4.87	4.92	1.03	46.43	18104
Avax Tech	AVXT	0.0125	0.015	20.00	200.00	474
Aveo Pharma	AVEO	1.18	1.13	-4.24	-38.25	3085
Bellus Health	BLUSF	0.81	0.69	-14.81	76.92	18
Bind Thera	BIND	9.13	8.91	-2.41	-40.95	403
Biocryst Pharma	BCRX	7.7	8.31	7.92	9.34	3294
Biodel	BIOD	2.14	2.11	-1.40	-7.46	776
Biodelivery Sci	BDSI	8.36	8.69	3.95	47.54	2288
Biogen Idec	BIIB	286.65	291.32	1.63	4.20	6133
Biolinerx	BLRX	1.96	1.99	1.53	-29.18	176
Biomarin Pharma	BMRN	58.54	57.59	-1.62	-18.14	5245
Bio-path Holdings	BPTH	2.65	2.7	1.89	-32.50	584
Biota Pharma	BOTA	3.01	2.86	-4.98	-31.74	846
Bluebird Bio	BLUE	18.24	25.18	38.05	20.02	2013
Cara Thera	CARA	13.88	14.03	1.08	8.68	423
Cardiome	CRME	8	7.66	-4.25	22.95	784
Cardium Thera	CRXM	0.6	0.55	-8.33	-33.73	46
Catalyst Pharma	CPRX	1.73	1.83	5.78	-6.15	1350
Celator	CPXX	2.65	2.47	-6.79	-18.21	47
Celgene	CELG	146.67	147.32	0.44	-12.81	14063
Cell Therapeutics	CTIC	2.71	2.59	-4.43	35.60	8850
Celladon	CLDN	10.3	9.55	-7.28	16.75	428
Celldex Therap	CLDX	12.79	13.33	4.22	-44.94	22001
Cel-Sci	CVM	1.11	1.13	1.80	91.53	2433
Celsion	CLSN	3.08	3.08	0.00	-20.82	324
Cempra	CEMP	8.78	8.65	-1.48	-30.19	600
Cerulean Pharma	CERU	6.5	6.46	-0.62	-5.69	73
Cerus	CERS	3.89	4.03	3.60	-37.52	3133
Chelsea Therap	CHTP	6.57	6.55	-0.30	47.52	32350
Chemocentryx	CCXI	5.11	4.94	-3.33	-14.68	616
Chimerix	CMRX	15.4	16.38	6.36	8.41	1132
Cleveland Biolabs	CBLI	0.56	0.56	0.00	-52.14	1370
Clovis Oncology	CLVS	47.83	55.96	17.00	-7.15	4961
Codexis	CDXS	1.55	1.43	-7.74	2.14	217

COMPANY	SYMBOL	CLOSE 5/9	CLOSE 5/16	%CHANGE WK	%CHANGE YTD	VOL (000)
Columbia Labs	CBRX	6.58	6.45	-1.98	-2.42	93
Compugen	CGEN	8.38	7.95	-5.13	-11.17	1408
Conatus Pharma	CNAT	5.46	5.67	3.85	-12.09	1312
Concert Pharma	CNCE	9.58	8.63	-9.92	-39.14	597
Corcept	CORT	1.84	2	8.70	-37.69	3273
CorMedix	CRMD	1.74	1.55	-10.92	25.00	612
Coronado Bio	CNDO	1.55	1.77	14.19	-32.70	1501
Cortex Pharma	CORX	0.03	0.03	0.00	0.00	183
Cubist Pharma	CBST	67.47	66	-2.18	-4.17	3169
Curis	CRIS	2.01	1.88	-6.47	-33.33	2128
Cyclacel Pharma	CYCC	3.27	3.09	-5.50	-23.13	529
Cytokinetics	CYTK	4.28	4.69	9.58	-27.85	4803
Cytrx	CYTR	3.18	3.38	6.29	-46.09	4991
Dara Biosciences	DARA	1.39	1.05	-24.46	-61.11	1437
Dendreon	DNDN	1.94	2.12	9.28	-29.10	12694
Depomed	DEPO	10.57	10.93	3.41	3.31	5511
Diadexus	DDXS	0.84	0.77	-8.33	-14.44	1119
Dicerna Pharma	DRNA	17.08	16.17	-5.33	-64.85	615
Dipexium Pharma	DPRX	9.92	9.25	-6.75	-34.12	108
Discovery Labs	DSCO	1.53	1.68	9.80	-25.33	1972
Durata Thera	DRTX	14.4	15.93	10.63	24.55	2443
Durect	DRRX	1.39	1.35	-2.88	-21.97	1482
Dyax	DYAX	6.38	6.2	-2.82	-17.77	4835
Dynavax	DVAX	1.38	1.31	-5.07	-33.16	8282
Egalet	EGLT	11.75	11.35	-3.40	-5.42	172
Eleven Biothera	EBIO	11.29	10.78	-4.52	-0.65	131
Emergent Biosol	EBS	22.73	20.77	-8.62	-9.66	3371
Emisphere Tech	EMIS	0.22	0.22	0.00	22.22	324
Enanta Pharma	ENTA	34.7	36.45	5.04	33.61	709
Endocyte	ECYT	6.73	6.52	-3.12	-38.95	6223
Entremed	ENMD	1.75	1.83	4.57	9.58	89
Enzo Biochem	ENZ	3.54	3.71	4.80	27.05	1027
Enzon Pharma	ENZN	0.89	0.94	5.62	-18.97	1387
Epizyme	EPZM	21.68	22.75	4.94	9.38	1015
Esperion Thera	ESPR	13.68	14.59	6.65	6.19	176
Evoke Pharma	EVOK	8.01	6.81	-14.98	-8.59	95
Exelixis	EXEL	3.45	3.33	-3.48	-45.68	9240
Fate Thera	FATE	6.85	6.79	-0.88	8.12	54
Five Prime Thera	FPRX	13.64	12.7	-6.89	-24.36	521
Flamel Tech	FLML	11.36	10.41	-8.36	29.32	784
Flexion Thera	FLXN	12.24	12.6	2.94	-13.99	192
Fluidigm	FLDM	29.9	27.08	-9.43	-29.26	2470
Forest Labs	FRX	89.5	93.28	4.22	55.39	8046
Furiex Pharma	FURX	103.64	103.84	0.19	147.18	989
Galena Biopharma	GALE	2.33	2.17	-6.87	-56.25	9028
Galmed Pharma	GLMD	7.01	6.56	-6.42	-54.16	116
Generex Biotech	GNBT	0.03	0.03	0.00	0.00	21582
Genocea Biosci	GNCA	18.28	18.06	-1.20	64.18	296
Genomic Health	GHDX	26.2	25.35	-3.24	-13.39	608
Genvec	GNVC	2.35	2.21	-5.96	-4.74	107
Geron	GERN	1.8	1.87	3.89	-60.55	13626
Gilead Sciences	GILD	79.76	80.8	1.30	7.59	53403
Glycomimetics	GLYC	9.13	6.48	-29.03	-28.08	549
Gtx	GTXI	1.38	1.4	1.45	-15.15	1576
GW Pharma	GWPH	68.62	68.31	-0.45	64.44	3703
Halozyme	HALO	7.37	7.67	4.07	-48.83	22688
Harvard Bio	HBIO	4.16	4.08	-1.92	-13.19	549
Heat Biologics	HTBX	4.12	3.96	-3.88	-44.23	111
Horzon Pharma	HZNP	13.25	12.88	-2.79	69.03	8440
Hyperion Thera	HPTX	24.76	25.43	2.71	25.77	550
Idenix Pharma	IDIX	5.12	5.69	11.13	-4.85	5576
Idera Pharma	IDRA	2.47	2.59	4.86	-44.06	10214
Ignyta	RXDX	7.32	7.15	-2.32	-22.28	189
Immune Pharma	IMNP	2.65	2.72	2.64	18.26	22
Immunocellular	IMUC	1.16	1.17	0.86	27.17	1823
Immunogen	IMGN	11.86	11.3	-4.72	-22.97	3488
Immunomedics	IMMU	3.21	3.23	0.62	-29.78	3875
Incyte	INCY	52.08	49.59	-4.78	-2.05	14614
Infinity Pharma	INFI	9.21	9.24	0.33	-33.09	2608
Insmed	INSM	14.46	13.85	-4.22	-18.53	2900
Insys	INSY	32.68	23.85	-27.02	-38.39	11880
Intercept Pharma	ICPT	235.37	280.26	19.07	310.46	1935
Intermune	ITMN	36.07	34.31	-4.88	132.93	10167
Ironwood Pharma	IRWD	12.65	13.62	7.67	17.31	8510
Isis Pharma	ISIS	23.42	24.7	5.47	-38.00	17653
Jazz Pharma	JAZZ	129.45	126.54	-2.25	-0.02	3914
Kalobios Pharma	KBIO	1.89	1.88	-0.53	-57.47	972
Kamada	KMDA	13.42	9.08	-32.34	-38.94	1930
Karyopharm Thera	KPTI	26.59	26.13	-1.73	14.01	789
Keryx Biopharma	KERX	12.11	12.79	5.62	-1.24	9536
Kythera Biopharma	KYTH	31.37	33.22	5.90	-11.06	995
La Jolla Pharma	LJPC	8.77	8.71	-0.68	8.88	371
Lexicon	LXRX	1.42	1.41	-0.70	-21.67	5463
Ligand Pharma	LGND	62.83	64.77	3.09	23.14	868
Macrogenics	MGNX	19.01	19.09	0.42	-30.40	751

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BIOWORLD STOCK REPORT FOR PUBLIC BIOTECHNOLOGY COMPANIES

COMPANY	SYMBOL	CLOSE 5/9	CLOSE 5/16	%CHANGE WK	%CHANGE YTD	VOL (000)
Mannkind	MNKD	6.22	7.02	12.86	35.00	42994
Marina Biotech	MRNA	0.75	0.75	0.00	87.50	141
Mast Thera	MSTX	0.56	0.61	8.93	32.61	5849
Medgenics	MDGN	6.39	7	9.55	16.86	251
Medicinova	MNOV	1.91	1.84	-3.66	-14.02	66
Merrimack Pharma	MACK	6.68	6.8	1.80	27.58	8538
Metabolix	MBLX	1.06	1.01	-4.72	-19.84	460
Momenta Pharma	MNTA	10.69	11.95	11.79	-32.41	4514
Myrexis	MYRX	0.18	0.17	-5.56	-5.56	99
Nektar Therap	NKTR	10.93	10.95	0.18	-3.52	4076
Nephrogenex	NRX	5.41	5.65	4.44	-52.44	55
Neurocrine Biosci	NBIX	13.84	13.17	-4.84	41.01	2554
Neurogesx	NGSX	0.008	0.0093	16.25	132.50	30
Newlink Genetics	NLNK	23.91	21	-12.17	-4.59	2616
Northwest Bio	NWBO	5.2	5.83	12.12	54.64	5627
NovaBay	NBY	1.02	0.86	-15.69	-30.08	1417
Novavax	NVAX	3.97	4.15	4.53	-18.95	16048
NPS Pharma	NPSP	27.52	26.04	-5.38	-14.23	8724
Ocera Thera	OCRX	7.89	7.74	-1.90	-40.42	152
Omeros	OMER	11.88	10.15	-14.56	-10.10	2354
Oncogenex Pharma	OGXI	3.78	3.84	1.59	-53.96	1141
Oncomed Pharma	OMED	23.22	22.96	-1.12	-22.22	942
Onconova Thera	ONTX	5.28	5	-5.30	-56.45	1090
Oncothyreon	ONTY	2.71	2.69	-0.74	52.84	1920
Ophthotech	OPHT	30.79	31.08	0.94	-3.93	1930
Opko Health	OPK	8.24	8.17	-0.85	-3.20	8298
Orasure Tech	OSUR	6.02	6.01	-0.17	-4.45	2684
Orexigen	OREX	5.29	5.36	1.32	-4.80	12953
Osiris Thera	OSIR	15.71	15.76	0.32	-1.99	2282
Oxigene	OXGN	2.85	3.07	7.72	21.83	1529
Oxis International	OXIS	0.0076	0.0074	-2.63	105.56	23
Oxygen BioThera	OXBT	4.74	4.92	3.80	9.33	372
Pacific Biosciences	PACB	4.23	4.45	5.20	-14.91	2615
Pacira Pharma	PCRX	73.5	75.11	2.19	30.65	1761
Pain Therapeutics	PTIE	5.07	4.59	-9.47	-5.56	1750
Palatin Tech	PTN	1.1	1.07	-2.73	46.58	567
Peregrine Pharma	PPHM	1.73	1.75	1.16	25.90	4912
Pharmacyclics	PCYC	95.08	98.02	3.09	-7.34	7059
Pharmathene	PIP	1.49	1.45	-2.68	-22.04	553
Pharmos	PARS	0.08	0.08	0.00	100.00	88
Poniard Pharma	PARD	0.32	0.44	37.50	51.72	2
Portola Pharma	PTLA	23.8	20.09	-15.59	-21.98	1070
Pozen	POZN	8.39	8.27	-1.43	2.86	1975
Progenics Pharma	PGNX	3.52	3.61	2.56	-32.27	3142
Prosensa	RNA	7.01	6.1	-12.98	30.62	970
Protalix Biothera	PLX	3.85	3.82	-0.78	-1.80	1316
Protein Design	PDLI	8.44	8.84	4.74	4.74	12682
PTC Thera	PTCT	16.96	15.07	-11.14	-11.20	793
Puma Biotech	PBYI	69.4	64.3	-7.35	-37.89	1789
Qiagen	QGEN	22.33	22.59	1.16	-5.12	3556
QLT Inc.	QLTI	5.42	4.99	-7.93	-10.41	417
Raptor Pharma	RPTP	7.83	8.45	7.92	-35.10	3381
Receptos	RCPT	30.84	25.9	-16.02	-10.66	1227
Recro Pharma	REPH	6.07	6.49	6.92	-21.81	67
Regado Biosciences	RGDO	5.64	5.33	-5.50	11.74	1296
Regeneron Pharma	REGN	279.41	295.43	5.73	7.34	3901
Regulus Thera	RGLS	6.47	6.41	-0.93	-13.26	534
Relypsa	RLYP	19.6	22.7	15.82	-9.20	1120
Repligen	RGEN	17.83	17.72	-0.62	29.91	3262
Repros	RPRX	15.18	15.7	3.43	-14.21	1079
Revance Thera	RVNC	30.28	31.8	5.02	18.44	676
Rexahn Pharma	RNN	0.79	0.97	22.78	90.20	26523
Rigel Pharma	RIGL	2.86	3.12	9.09	9.47	5110
Ruthigen	RTGN	6.3	6.7	6.35	-7.59	121
Rxi Pharma	RXII	3.16	2.99	-5.38	3.10	423
Sagent Pharma	SGNT	21.23	21.01	-1.04	-17.22	716
Salix Pharma	SLXP	103.56	107.51	3.81	19.54	4311
Sangamo Biosci	SGMO	12.31	13	5.61	-6.41	5427
Sarepta Thera	SRPT	32.07	31.42	-2.03	54.25	5910
Savient Pharma	SVNTQ	0.0062	0.0072	16.13	-64.00	266
Sciclone Pharma	SCLN	4.78	4.82	0.84	-4.37	1513
Seattle Genetics	SGEN	35.35	34.25	-3.11	-14.14	5060
Siga Tech	SIGA	2.8	2.68	-4.29	-18.04	774
Sinovac Biotech	SVA	6.26	5.89	-5.91	-3.76	810
Soligenix	SNGX	1.83	2.03	10.93	12.78	129
Sophiris Bio	SPHS	3.16	3.11	-1.58	-15.95	43
Sorrento Thera	SRNE	7.99	5	-37.42	-38.27	3103
Spectrum Pharma	SPPI	7.3	7.51	2.88	-15.14	5067
Stemcells	STEM	1.18	1.39	17.80	13.01	3813
Stemline Thera	STML	14.24	12.89	-9.48	-34.23	673
Sucampo Pharma	SCMP	6.8	6.86	0.88	-27.02	601
Sunesis	SNSS	5.01	4.67	-6.79	-1.48	1464
Supernus Pharma	SUPN	7.73	8.23	6.47	9.15	2379
Synageva Bio	GEVA	83.8	82.3	-1.79	27.16	1056
Synergy Pharma	SGYP	3.78	3.71	-1.85	-34.10	5183
Synta	SNTA	4.13	4.18	1.21	-20.23	5981
Targacept	TRGT	3.87	3.73	-3.62	-10.12	839
Telik	TELK	1.31	1.28	-2.29	7.56	3406
Tengion	TNGN	0.18	0.17	-5.56	-32.00	355
Tesaro	TSRO	24.01	25.96	8.12	-8.07	3019
Tetralogic Pharma	TLOG	5.13	5.15	0.39	-45.90	445
Tetraphase Pharma	TTPH	9.27	8.83	-4.75	-34.69	1058
The Medicines Co.	MDCO	25.49	25.52	0.12	-33.92	2917
Theravance	THRX	26.86	27.37	1.90	-23.23	4845
Threshold	THLD	3.75	3.62	-3.47	-22.48	2110
Titan Pharma	TTNP	0.58	0.64	10.34	6.67	560
Transcept Pharma	TSPT	2.93	3.09	5.46	-8.04	1067
Transgenomic	TBIO	4.25	4	-5.88	-27.54	279
Trevena	TRVN	4.72	4.46	-5.51	-31.38	251
Trinity Biotech	TRIB	23.44	23.96	2.22	-4.69	281
Ultragenyx	RARE	36.55	34.48	-5.66	-18.39	822
Unigene	UGNEQ	0.0008	0.0005	-37.50	-16.67	43
Uniqure	QURE	9.27	9.61	3.67	-34.22	289
United Therap	UTHR	106.5	100.64	-5.50	-11.00	2926
Vanda Pharma	VNDA	10.17	9.77	-3.93	-21.27	4522
Ventrus Bio	VTUS	0.96	1.06	10.42	-72.25	1497
Verastem	VSTM	7.45	7.67	2.95	-32.72	766
Vermillion	VRML	3.04	2.95	-2.96	25.00	103
Versartis	VSAR	28.02	27.53	-1.75	-12.24	345
Vertex Pharma	VRTX	65.05	65.03	-0.03	-12.48	6831
Vical	VICL	1.19	1.23	3.36	4.24	3729
Vital Therapies	VTL	12.5	12.38	-0.96	3.17	109
Vivus	VVUS	5.23	4.83	-7.65	-46.81	9351
Xencor	XNCR	8.35	8.07	-3.35	-11.71	273
Xenoport	XNPT	3.28	3.74	14.02	-34.96	1903
Xoma	XOMA	3.64	3.63	-0.27	-46.06	8256
Zalicus	ZLCS	0.9	1.02	13.33	-8.11	983
Zogenix	ZGNX	2.29	2.12	-7.42	-38.37	5220

LONDON STOCK EXCHANGE

Company	Symbol	5/9	5/16	%WK	%YTD	Volume
Circassia	CIR	305	289.25	-5.16	-6.69	184
Horizon Discovery	HZD	179.925	169.975	-5.53	-20.76	990
Oxford Biomedica	OXB	1.95	2.07	6.15	-10.00	981
Puricore	PURI	42.2485	33.37	-21.01	-28.85	4109
Renovo Group	RNVO	15.75	16.1	2.22	-15.26	195
Skyepharma	SKP	240	237	-1.25	106.90	2696
Vernalis	VER	30.9625	31.5	1.74	-14.86	442

Note: Prices are denoted in pence.

TORONTO STOCK EXCHANGE

Company	Symbol	5/9	5/16	%WK	%YTD	Volume
Adherex Technol	AHX	1.16	1.25	7.76	247.22	234
Antibe Thera	ATE	0.54	0.52	-3.70	-1.89	47
Bellus Health	BLU	0.85	0.75	-11.76	87.50	272
Bioniche Life Sci	BNC	0.2	0.18	-10.00	-43.75	488
Cynapsus Thera	CTH	0.57	0.57	0.00	-25.00	909
Lorus Therap	LOR	0.45	0.46	2.22	-19.30	1712
Oncolytics Biotech	ONC	1.35	1.39	2.96	-16.27	358
Resverlogix	RVX	0.66	0.66	0.00	37.50	140
Tekmira Pharma	TKM	12.92	12.18	-5.73	44.14	498
Theratechnologies	TH	0.52	0.49	-5.77	0.00	191

Note: Prices are denoted in Canadian dollars.

NOTES

Trading volumes for Nasdaq, Amex and NYSE are recorded as the total number of shares traded (in thousands) on a weekly basis (cumulative Monday through Friday); the weekly and YTD % changes are from IPO completion, where applicable.

Average Percent Change Week: -0.27%

Range: -37.50% to +38.05%; Number Of Companies: 288
(does not include LSE or TSX; not market weighted)

Average Percent Change YTD: -2.34%

Range: -72.25% to +310.46%; Number Of Companies: 288
(does not include LSE or TSX; not market weighted)

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