BioCentury
WEEK IN REVIEW | POWERED BY BCIQ

WEEK OF JUNE 5, 2017

COMPANY NEWS

DEALS

Advaxis Inc. (NASDAQ:ADXS), Princeton, N.J.
Bristol-Myers Squibb Co. (NYSE:BMY), New York, N.Y.
Business: Cancer

Advaxis Inc. (NASDAQ:ADXS) will sponsor a clinical trial evaluating its ADXS-DUAL in combination with PD-1 inhibitor Opdivo nivolumab to treat metastatic cervical cancer. The trial is slated to start this year.

ADXS-DUAL is the second-generation of axalimogene filolisbac (ADXS11-001), the company's live *Listeria monocytogenes*-based immunotherapy expressing E7 transforming protein (Human papillomavirus-16; HpV16gp2).

Array BioPharma Inc. (NASDAQ:ARRY), Boulder, Colo.
Bristol-Myers Squibb Co. (NYSE:BMY), New York, N.Y.
Business: Cancer

Array BioPharma Inc. (NASDAQ:ARRY) and Bristol-Myers Squibb Co. (NYSE:BMY) partnered to evaluate Array's binimetinib (MEK162) in combination with PD-1 inhibitor Opdivo nivolumab with or without Yervoy ipilimumab to treat metastatic colorectal cancer (mCRC) in patients with microsatellite stable tumors.

Next half, the partners plan to start a Phase I/II trial to determine the recommended dose regimens and evaluate safety, tolerability and preliminary antitumor activity of the combinations.

Array will sponsor the trial, which will be supported by both parties.

Binimetinib is a small molecule selective inhibitor of MAP kinase kinase 1 (MAP2K1; MEK1) and MEK2. Yervoy is a human mAb against cytotoxic T-lymphocyte associated protein 4 (CTLA-4; CTLA4; CD152).

AstraZeneca plc (LSE:AZN; NYSE:AZN), London, U.K.
Harvard Pilgrim Health Care Inc., Boston, Mass.
Business: Cardiovascular, Endocrine/Metabolic

Regional insurer Harvard Pilgrim Health Care Inc. (Boston, Mass.) signed two outcomes-based drug pricing contracts with AstraZeneca plc (LSE:AZN; NYSE:AZN).

The insurer signed a three-year contract for AZ's Brilinta ticagrelor, which is marketed in the U.S. to reduce the rate of cardiovascular death, myocardial infarction (MI) and stroke in patients with acute coronary syndrome (ACS) or a history of MI. Harvard Pilgrim will measure the reduction in hospitalizations for repeat acute coronary events for Brilinta patients compared to patients on Plavix clopidogrel. The reversible purinergic receptor P2Y G protein-coupled 12 (P2RY12; P2Y12) is approved in the EU as Brilique.

Harvard Pilgrim also signed a three-year contract for Type II diabetes treatment Bydureon, a once-weekly long-acting release (LAR) formulation of Byetta exenatide, a synthetic exendin-4. The insurer will pay for Bydureon based on its ability to achieve a predetermined HbA1c reduction goal in adhering diabetic patients.

In each case, the insurer will receive rebates if the drugs fail to meet the undisclosed outcomes criteria. Harvard Pilgrim said the deal does not feature price increases for drug over-performance. The insurer declined to disclose specific pricing terms. AZ deferred pricing questions to Harvard Pilgrim. AZ said it now has 13 "innovative agreements" which provide health plan cost predictability and flexibility.

Last month, Harvard Pilgrim and Amgen Inc. (NASDAQ:AMGN) signed an outcomes-based contract for cholesterol-lowering drug

Repatha evolocumab. Amgen agreed to pay rebates when patients experience an MI or stroke while taking the drug (see BioCentury, May 15).

Atreca Inc., Redwood City, Calif.
Dana-Farber Cancer Institute, Boston, Mass.

Business: Cancer

Atreca Inc. (Redwood City, Calif.) and Dana-Farber Cancer Institute (Boston, Mass.) partnered to study active immune response in cancer patients who respond to immunotherapy. The partners will use Atreca's Immune Repertoire Capture technology to study how immunotherapies generate active immune response; explore why only a subset of cancer patients respond to different therapies; and identify anti-tumor antibodies generated in the response. The technology identifies and generates sequences of antibodies and T cell receptors associated with immune response.

The study will focus on melanoma, non-small cell lung cancer (NSCLC) and renal cell cancer (RCC), with the potential to expand to other cancer types. Atreca declined to disclose details and Dana-Farber did not respond to inquiries.

Cirina Ltd., Hong Kong, China
Grail Inc., Menlo Park, Calif.

Business: Diagnostic

Circulating tumor DNA (ctDNA) testing company Grail Inc. (Menlo Park, Calif.) said it will merge with cancer diagnostics developer Cirina Ltd. (Hong Kong, China).

Grail spokesperson Charlotte Arnold told BioCentury that Cirina will become a subsidiary of Grail with a focus on the Southeast Asia region. In Hong Kong, Cirina will continue to operate under its existing name, while Cirina's U.S. workforce will join Grail. Cirina has an ongoing research collaboration with the Chinese University of Hong Kong.

Arnold declined to say whether specific IP held by Cirina drove the deal, but said the companies will combine their IP estates. Arnold also declined to disclose financial terms of the merger.

Cirina co-founder Dennis Lo will become a Grail scientific co-founder and join its scientific advisory board. According to the partners, Lo was the first scientist to discover circulating fetal DNA in a pregnant mother's blood. Cirina CEO Maneesh Jain will also join Grail's business development leadership team, Arnold said.

Decheng Capital's Min Cui will join Grail's board. Decheng's Victor Tong told BioCentury that the firm invested $12 million in Cirina's series A round, its only round of funding.

In March, Grail said it raised more than $900 million toward a planned $1 billion series B round. Arch Venture Partners was the lead investor (see BioCentury, March 6).

Grail began its first large-scale observational trial, the Circulating Cell-free Genome Atlas (CCGA) study, in December 2016. The company hopes to enroll hundreds of thousands of people in the study, with the goal of characterizing cell-free DNA profiles of both healthy subjects and cancer patients.

In April, Grail began the longitudinal, observational STRIVE study to evaluate a blood test for early breast cancer.

Corbin Therapeutics Inc., Montreal, Canada
Proteorex Therapeutics Inc., Toronto, Canada
University of Dundee, Dundee, U.K.

Business: Autoimmune

Corbin Therapeutics Inc. (Montreal, Canada) partnered with Proteorex Therapeutics Inc. (Toronto, Canada) and the University of Dundee (Dundee, U.K.) in separate deals to discover inhibitors of ubiquitin specific peptidase 15 (USP15). Corbin said USP15 may play a role in treating neuro-inflammatory diseases, including multiple sclerosis (MS).

Proteorex will use its chemistry platform to discover small molecule peptide conjugates that inhibit USP15. Corbin will have full rights to the compounds. Proteorex will receive an undisclosed upfront payment and is eligible for milestones.

Under the deal with the university, Dundee's Drug Discovery Unit will perform drug screening to identify small molecules and Corbin will carry out all *in vitro* and *in vivo* studies. Corbin said that each party will pay for its own work and that discovered products will be owned jointly.

In January, Corbin spun out of AmorChem L.P. (Montreal, Quebec) and received rights to USP15 technology.

Cystic Fibrosis Foundation, Bethesda, Md.
Arcturus Therapeutics Inc., San Diego, Calif.

Business: Pulmonary, Drug delivery

The Cystic Fibrosis Foundation Therapeutics Inc. affiliate of the Cystic Fibrosis Foundation (Bethesda, Md.) will provide Arcturus Therapeutics Inc. (San Diego, Calif.) up to $3 million in milestones under a two-year agreement to develop LUNAR-CF, the company's mRNA therapeutic targeting the cystic fibrosis transmembrane conductance regulatory (CFTR) gene. The preclinical therapeutic is formulated using the company's LUNAR nanoparticle delivery system.

The foundation said its development affiliate would also provide guidance through a project advisory group and non-exclusive access to non-commercial reagents and other resources should the LUNAR-CF program advance to clinical testing.

ImmunoGen Inc. (NASDAQ:IMGN), Waltham, Mass.
Sanofi (Euronext:SAN; NYSE:SNY), Paris, France

Business: Cancer

In exchange for $30 million, ImmunoGen Inc. (NASDAQ:IMGN) and Sanofi (Euronext:SAN; NYSE:SNY) eliminated potential milestone and royalties payments the biotech was eligible to

receive under two deals involving five compounds. The candidates are isatuximab, SAR408701, SAR566658, SAR428926 and an undisclosed antibody-drug conjugate (ADC).

Under the original deals, ImmunoGen and the pharma were co-developing ADCs developed using ImmunoGen's TAP and maytansinoid-based antibody-conjugate technologies.

Isatuximab, a humanized IgG1 mAb against CD38, is in a Phase III study to treat multiple myeloma (MM). SAR408701, a carcinoembryonic antigen (CEA)-related cell adhesion molecule 5 (CEACAM5; CD66e) ADC, is in a Phase I/II trial to treat solid tumors. SAR566658 is a humanized mAb against carbonic anhydrase VI (CAVI) linked to ImmunoGen's DM4 cytotoxic agent. The compound is in a Phase II trial to treat triple-negative breast cancer (TNBC). SAR428926 is an ADC targeting lysosomal-associated membrane protein 1 (LAMP1) in a Phase I trial to treat solid tumors.

ImmunoGen's lead program, mirvetuximab soravtansine, is in the Phase III FORWARD trial to treat platinum-resistant ovarian cancer. The compound is a folate receptor 1 (FOLR1; FR-alpha)-targeting mAb linked to ImmunoGen's DM4 cytotoxic agent.

Minerva Neurosciences Inc. (NASDAQ:NERV), Waltham, Mass.
Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.

Business: Neurology

Minerva Neurosciences Inc. (NASDAQ:NERV) and the Janssen Pharmaceutica N.V. subsidiary of Johnson & Johnson (NYSE:JNJ) amended a 2014 deal to co-develop and commercialize orexin 2 receptor (OX2R; HCRTR2) antagonist MIN-202. Minerva will gain global development rights to MIN-202 in insomnia and will repurchase Minerva shares owned by the pharma's Johnson & Johnson Innovation - JJDC Inc. unit for a nominal fee (see BioCentury, July 14, 2014).

Janssen is to pay Minerva $30 million up front and relinquish its rights to royalties on sales of MIN-202 in the EU and other territories. Minerva is eligible for two $20 million milestones tied to a Phase III trial of the candidate to treat insomnia. Janssen will also waive the $13 million in remaining payments Minerva owes in connection with MIN-202's Phase II program. Minerva will fund Phase III trials in insomnia on its own.

Janssen received $22 million up front in the original deal. In a private placement alongside Minerva's IPO, J&J purchased 3.3 million shares for $19.7 million, giving it an 18% stake in the company.

Minerva will retain its commercialization rights to MIN-202 in major depressive disorder (MDD) and pay Janssen royalties for that indication in its territories. Janssen will pay Minerva royalties on sales in the rest of the world.

The amended deal is contingent on J&J closing its acquisition of Actelion Ltd. (SIX:ATLN) (see BioCentury, Jan. 30).

Minerva expects the new funds will allow it to develop MIN-202 and another candidate, MIN-101, through 2019. By then, the company hopes to receive data from a Phase III trial of MIN-101 to treat schizophrenia and three Phase IIb trials of MIN-202 in insomnia and MDD.

MIN-101 is a serotonin (5-HT2A) and sigma-2 receptor antagonist. Its Phase III development is to begin next half. Minerva has its ex-Asian rights to MIN-101 from Mitsubishi Tanabe Pharma Corp. (Tokyo:4508).

Minerva declined to provide further details and J&J did not respond to an inquiry.

Protagonist Therapeutics Inc. (NASDAQ:PTGX), Newark, Calif.
Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.

Business: Autoimmune

Oral peptide therapeutic company Protagonist Therapeutics Inc. (NASDAQ:PTGX) granted the Janssen Biotech Inc. unit of Johnson & Johnson (NYSE:JNJ) exclusive, worldwide rights to co-develop and co-promote PTG-200, a preclinical candidate to treat inflammatory bowel disease (IBD).

Protagonist is to receive $50 million up front and is eligible for $325 million in clinical milestones and $615 million in regulatory and sales milestones, plus tiered double-digit royalties.

Next half, Protagonist plans to start a Phase I trial of the oral antagonist of IL-23 receptor in healthy volunteers. The company will also fund 20% of Janssen's planned Phase II trial to treat Crohn's disease (CD). The deal includes a $125 million milestone if Janssen elects to continue PTG-200's development beyond the latter trial's Phase IIa stage, and a $200 million milestone if it does so following its Phase IIb portion. The companies also plan to develop PTG-200 in ulcerative colitis (UC).

Janssen said PTG-200 will add to its immunology portfolio targeting the IL-23 pathway. The company markets IL-12 and IL-23 inhibitor Stelara ustekinumab to treat psoriasis, psoriatic arthritis and CD. Next quarter, it anticipates launching Tremfya guselkumab, a mAb targeting the p19 subunit of IL-23. Tremfya is under FDA review to treat psoriasis. Spokesperson Brian Kenney told BioCentury J&J is planning Phase III trials of Tremfya to treat CD.

J&J's venture arm, Johnson & Johnson Innovation -- JJDC Inc., invested in Protagonist prior to its IPO.

Vaximm AG, Basel, Switzerland
Merck KGaA (Xetra:MRK), Darmstadt, Germany

Business: Cancer

Vaximm AG (Basel, Switzerland) will conduct two open-label trials evaluating its VXM01 in combination with PD-L1 inhibitor Bavencio avelumab from Merck KGaA (Xetra:MRK). Vaximm declined to provide a start date for the trials, which will enroll patients with glioblastoma or metastatic colorectal cancer (mCRC).

VXM01 is an oral T cell immunotherapy based on the recombinant, live, attenuated *Salmonella typhi* vaccine strain Ty21a that carries the

VEGF receptor 2 (VEGFR-2; KDR/Flk-1) gene. The candidate has completed multiple Phase I trials in cancer. Bavencio has accelerated approval in the U.S. to treat Merkel cell and urothelial carcinomas.

Pfizer Inc. (NYSE:PFE) and Merck share rights to Bavencio under a 2014 deal (see BioCentury, Nov. 24, 2014).

X-Chem Inc., Waltham, Mass.
Vertex Pharmaceuticals Inc. (NASDAQ:VRTX), Boston, Mass.

Business: Pharmaceuticals

X-Chem Inc. (Waltham, Mass.) will use its X-Chem's DEX library to discover small molecules against undisclosed targets implicated in severe genetic diseases. Vertex Pharmaceuticals Inc. (NASDAQ:VRTX) has exclusive options to license and develop compounds discovered under the deal, which may be expanded to include additional targets.

X-Chem will receive an upfront payment and is eligible for development, regulatory and sales milestones, as well as royalties. The companies did not respond to inquires.

COMPANY NEWS

SALES & MARKETING

Exact Sciences Corp. (NASDAQ:EXAS), Madison, Wis.
UnitedHealth Group Inc. (NYSE:UNH), Minneapolis, Minn.

Business: Diagnostic

In a coverage determination guideline, insurer UnitedHealth Group Inc. (NYSE:UNH) said it will cover colorectal cancer screening assay Cologuard from Exact Sciences Corp. (NASDAQ:EXAS) beginning July 1.

Commercial payers have increasingly extended coverage to Cologuard since the U.S. Preventive Services Task Force began including the test among its guidelines in June 2016, putting Cologuard on equal footing with colonoscopy and fecal immunohistochemical test (FIT). In April, the company raised its 2017 guidance after reporting stronger-than-expected first quarter sales of Cologuard.

Cologuard is a non-invasive stool DNA test that utilizes a multiplexed quantitative Invader assay for the simultaneous detection of methylated and unmethylated sequences in the promoter region of the vimentin (VIM) gene.

Merck & Co. Inc. (NYSE:MRK), Kenilworth, N.J.

Business: Cancer

The U.K.'s NICE issued a final appraisal determination (FAD) recommending that Keytruda pembrolizumab from Merck & Co. Inc. (NYSE:MRK) be added to the Cancer Drugs Fund for PD-L1 positive metastatic non-small cell lung cancer (NSCLC) in a first-line setting. Specifically, NICE recommend Keytruda to treat metastatic NSCLC in adults whose tumors express PD-L1 with at least a 50% tumor proportion score and have no EGFR or anaplastic lymphoma kinase (ALK)-positive mutations. Keytruda is a humanized IgG4 mAb against PD-1.

Merck & Co. Inc. (NYSE:MRK), Kenilworth, N.J.

Business: Cancer

Germany's Institute for Quality and Efficiency in Healthcare (IQWiG) said in an early benefit assessment that Keytruda pembrolizumab from Merck & Co. Inc. (NYSE:MRK) offers a "hint of considerable added benefit" in overall survival (OS) for a population of patients with locally advanced or metastatic non-small cell lung cancer (NSCLC). Specifically, the assessment compares the drug to cisplatin- or carboplatin-based chemotherapy in chemotherapy-naïve patients whose tumors express PD-L1 at least 50% of the time and contain no EGFR or anaplastic lymphoma kinase (ALK) activating mutations. The European Commission expanded Keytruda's label to include the indication in late January (see BioCentury, Feb. 6).

The agency said Merck submitted data from its Phase III KEYNOTE-024 trial. IQWiG said there were also hints of added benefit for Keytruda in morbidity and health-related quality of life (QOL) measures, and a hint of lesser harm vs. comparators in terms of severe side effects.

Keytruda is a humanized IgG4 mAb against PD-1.

Shionogi & Co. Ltd. (Tokyo:4507), Osaka, Japan

Business: Neurology

Shionogi & Co. Ltd. (Tokyo:4507) launched Intuniv guanfacine in Japan to treat pediatric ADHD. The Japanese National Health Insurance (NHI) list price of the once-daily adrenergic receptor alpha 2a (ADRA2A) agonist is ¥412.2 ($3.71) for a 1 mg tablet and ¥544.3 ($4.90) for a 3 mg tablet.

Takeda Pharmaceutical Co. Ltd. (Tokyo:4502), Osaka, Japan

Business: Cancer

In late April, the U.K.'s NICE issued draft guidance recommending against the use of Ninlaro ixazomib from Takeda Pharmaceutical Co. Ltd. (Tokyo:4502) to treat relapsed or refractory multiple myeloma (MM). The agency is considering Ninlaro in combination with lenalidomide and dexamethasone to treat MM patients who have had at least one prior therapy.

The agency said although Ninlaro is approved as a second-line therapy, it is only likely to be used in patients who have had two or three previous therapies. For patients who have had two or three prior therapies, NICE said the minimum estimate of cost effectiveness varied between £138,000 ($177,082) and £176,000 ($225,843) per quality-adjusted life year (QALY) gained compared with the current treatment.

NICE said that for MM patients who had one prior therapy, Ninlaro was "less effective" and "cost more" than the current treatment of Velcade bortezomib plus dexamethasone. Takeda also markets

Velcade, a small molecule dipeptide boronic acid proteasome inhibitor, to treat MM.

NICE did say that Ninlaro "may be more effective" after three previous therapies than after two previous therapies.

The agency noted that Ninlaro is "not suitable" for use in the Cancer Drugs Fund because it is "unlikely to be cost effective at its current price even if the uncertainty about its effectiveness is reduced." Ninlaro is an oral proteasome inhibitor.

Veracyte Inc. (NASDAQ:VCYT), South San Francisco, Calif.
Anthem Inc. (NYSE:ANTM), Indianapolis, Ind.

Business: Diagnostic

Veracyte Inc. (NASDAQ:VCYT) said Anthem Inc. (NYSE:ANTM) issued a positive coverage decision for Afirma Gene Expression Classifier for thyroid cancer diagnosis. Afirma is a preoperative molecular diagnostic classifier based on genomic measurements from fine needle aspirate (FNA) samples.

COMPANY NEWS

OTHER NEWS

Amgen Inc. (NASDAQ:AMGN), Thousand Oaks, Calif.

Business: Endocrine/Metabolic

In a newly filed lawsuit, Amgen Inc. (NASDAQ:AMGN) is seeking to compel FDA to accept pediatric study reports related to hyperparathyroidism drug Sensipar cinacalcet and extend by six months Amgen's patent exclusivity for the drug.

The complaint, filed in the U.S. District Court for the District of Columbia, requests injunctive relief by June 6 requiring FDA to accept the study reports and recognize the pediatric exclusivity, vacating its decision last week to deny the exclusivity. Amgen said a key Sensipar patent, U.S. Patent No. 6,011,068, is due to expire March 8, 2018.

Sponsors are eligible for additional exclusivity if they conduct pediatric studies of drugs upon FDA request. In its complaint, Amgen said FDA asked the company in May 2010 to conduct studies of Sensipar in dialysis-dependent pediatric patients with chronic kidney disease (CKD) and secondary hyperparathyroidism. Amgen said it was able to complete three of four required studies to the agency's satisfaction.

The incomplete trial on which FDA based its denial, Study 3, was to include at least 15 patients aged 28 days to six years with CKD and secondary hyperparathyroidism. According to Amgen, there are about 300 secondary hyperparathyroidism patients under age six on dialysis in the U.S. at any particular time.

The company had enrolled eight patients by February 2013, when a death in Study 2 prompted FDA to place a partial clinical hold on trials of Sensipar in pediatric secondary hyperparathyroidism. Amgen said from then until FDA lifted the suspension of enrollment and dosing in April 2014, six patients left Study 3. The company said only four patients ultimately met the trial's completion criteria.

Despite Study 3, Amgen claims it "fairly responded" to FDA's requests as required by statute to gain pediatric exclusivity. The company said it provided data from two juvenile toxicity studies, a bioavailability and bioequivalence study, the four clinical study reports, a pediatric dialysis registry, and several other analyses. In its denial letter, FDA claimed that if Amgen had submitted an "appropriate safety assessment in younger children," the data could have been considered a fair response, even if an insufficient number of patients had completed Study 3.

Sensipar is a second-generation calcimimetic.

Emmes Corp., Rockville, Md.
National Institutes of Health, Bethesda, Md.

Business: Infectious

In early May, Emmes Corp. (Rockville, Md.) received a four-year contract for up to $27.6 million from NIH's National Institute of Allergy and Infectious Diseases (NIAID) to conduct a Phase II/IIb trial of Zika vaccine ZIKVwt (VRC-ZKADNA090-00-VP). The product is DNA plasmid Zika vaccine.

NIAID began the trial in March and will enroll about 2,500 healthy volunteers (see BioCentury, April 10).

Helsinn Healthcare S.A., Lugano, Switzerland
Eisai Co. Ltd. (Tokyo:4523), Tokyo, Japan

Business: Gastrointestinal

In early May, a federal appeals court invalidated U.S. Patent Nos. 7,947,724; 7,947,725; 7,960,424; and 8,598,219 from Helsinn Healthcare S.A. (Lugano, Switzerland) covering Aloxi palonosetron. The decision allows competition from a generic palonosetron product from Teva Pharmaceutical Industries Ltd. (NYSE:TEVA; Tel Aviv:TEVA). Aloxi is a selective serotonin (5-HT3) receptor antagonist indicated in the U.S. to prevent chemotherapy-induced nausea and vomiting (CINV) and post-operative nausea and vomiting (PONV). Helsinn's partner Eisai Co. Ltd. (Tokyo:4523) markets Aloxi in the U.S.

The decision reversed a November 2015 decision from the U.S. District Court for the District of New Jersey in a case brought by Helsinn and partner Roche (SIX:ROG; OTCQX:RHHBY) which accused Teva, Dr. Reddy's Laboratories Ltd. (NYSE:RDY) and the Sandoz unit of Novartis AG (NYSE:NVS; SIX:NOVN) of patent infringement. Roche licensed Aloxi to Helsinn in 1998.

In 2015, Helsinn said it had settled with Sandoz and Dr. Reddy's and that neither would be allowed to launch a generic palonosetron in the U.S. prior to September 2018 except under undisclosed circumstances (see BioCentury, Oct. 26, 2015).

According to Orange Book, the four patents include pediatric exclusivity extensions and expire in July 30, 2024.

CLINICAL NEWS

REGULATORY

Alkermes plc (NASDAQ:ALKS), Dublin, Ireland
Acorda Therapeutics Inc. (NASDAQ:ACOR), Ardsley, N.Y.
Biogen Inc. (NASDAQ:BIIB), Cambridge, Mass.

Product: Fampyra (Ampyra dalfampridine - U.S.) fampridine (BIIB041)
Business: Autoimmune

The European Commission granted standard marketing approval to Fampyra fampridine from Biogen Inc. (NASDAQ:BIIB) to improve walking in multiple sclerosis patients. Fampyra previously had conditional approval in the EU for the indication.

The approval is based on data from the Phase III ENHANCE trial in which Fampyra led to clinically meaningful improvements in significantly more patients' walking ability (43.2% vs. 33.6%, p=0.006) and mobility (43.4% vs. 34.7%, p=0.03) vs. placebo. Walking ability, the primary endpoint, was measured by the self-reported 12-item MS Walking Scale (MSWS-12). Mobility was measured by clinician-reported timed up-and-go speed.

Biogen has ex-U.S. rights to the sustained-release formulation of 4-aminopyridine (4-AP) from Acorda Therapeutics Inc. (NASDAQ:ACOR). Acorda has worldwide rights to the product from Alkermes plc (NASDAQ:ALKS). Acorda markets the product in the U.S. as Ampyra to improve walking ability in MS patients.

Almirall S.A. (Madrid:ALM), Barcelona, Spain
Sun Pharmaceutical Industries Ltd. (BSE:524715; NSE:SUNPHARMA)
Merck & Co. Inc. (NYSE:MRK), Kenilworth, N.J.

Product: Tildrakizumab (MK-3222) (formerly SCH 900222)
Business: Autoimmune

Sun Pharmaceutical Industries Ltd. (BSE:524715; NSE:SUNPHARMA) said FDA accepted for review a BLA for tildrakizumab (MK-3222) to treat moderate to severe plaque psoriasis. The company declined to disclose the PDUFA date.

In March, EMA validated an MAA for the product in the indication (see BioCentury, April 3).

Sun has exclusive, worldwide rights to the humanized mAb against IL-23 subunit alpha (IL23P19; IL-23A) from Merck & Co. Inc. (NYSE:MRK), and Almirall S.A. (Madrid:ALM) has rights from Sun to develop and commercialize the product for psoriasis in Europe (see BioCentury, Sept. 22, 2014 & Aug. 8, 2016).

Biogen Inc. (NASDAQ:BIIB), Cambridge, Mass.
Ionis Pharmaceuticals Inc. (NASDAQ:IONS), Carlsbad, Calif.

Product: Spinraza nusinersen (IONIS-SMNRx) (formerly ISIS-SMNRx)
Business: Neurology

The European Commission approved Spinraza nusinersen (IONIS-SMNRx) from Biogen Inc. (NASDAQ:BIIB) to treat spinal muscular atrophy (SMA). Biogen has rights to Spinraza from Ionis Pharmaceuticals Inc. (NASDAQ:IONS). The approval triggered a $50 million milestone payment to Ionis, which is also eligible for tiered royalties (see BioCentury, Aug. 8, 2016).

Spinraza is the first SMA drug approved in the EU. FDA approved Spinraza in December. The product is an antisense oligonucleotide that modulates splicing of survival of motor neuron 2 centromeric (SMN2) mRNA (see BioCentury, Jan. 2).

BioMarin Pharmaceutical Inc. (NASDAQ:BMRN), San Rafael, Calif.

Product: Brineura cerliponase alfa (BMN 190) (formerly BMN-190)
Business: Neurology

The European Commission approved Brineura cerliponase alfa (BMN 190) from BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) to treat neuronal ceroid lipofuscinosis type 2 (CLN2), a form of Batten disease.

In April, FDA approved Brineura, making it the first drug approved to treat CLN2. It is a recombinant human tripeptidyl peptidase-1 (TPP1) enzyme replacement therapy (see BioCentury, May 1).

Hansa Medical AB (SSE:HMED), Lund, Sweden

Product: IdeS
Business: Transplant

EMA granted PRIority MEdicine (PRIME) designation to IdeS from Hansa Medical AB (SSE:HMED) to prevent renal transplant rejection in human leukocyte antigen (HLA)-sensitized patients. The product is a *Streptococcus pyogenes* enzyme that cleaves IgG antibodies. Hansa plans to submit a BLA to FDA for the product in 2018.

Jazz Pharmaceuticals plc (NASDAQ:JAZZ), Dublin, Ireland

Product: Vyxeos (CPX-531)
Business: Cancer

Jazz Pharmaceuticals plc (NASDAQ:JAZZ) said FDA accepted and granted Priority Review to an NDA for Vyxeos (CPX-351) to treat acute myelogenous leukemia (AML). Its PDUFA date is Sept. 30.

Jazz completed its rolling NDA submission for Vyxeos on March 31 (see BioCentury, April 17).

The product has breakthrough therapy and Fast Track designations from FDA, as well as Orphan Drug designations from FDA and EMA, to treat AML. Jazz gained Vyxeos, a liposomal formulation of cytarabine and daunorubicin encapsulated at a 5:1 molar ratio using CombiPlex technology, through its acquisition of Celator Pharmaceuticals Inc. (see BioCentury, June 6, 2016).

Mitsubishi Tanabe Pharma Corp. (Tokyo:4508), Osaka, Japan
Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.

Product: Invokana (Canaglu - Japan) canagliflozin (TA-7284, JNJ-

28431754); Invokamet canagliflozin/metformin IR (Vokanamet - EU); Invokamet XR canagliflozin/metformin XR
Business: Endocrine/Metabolic

FDA will require a new boxed warning label for Type II diabetes drug canagliflozin from Mitsubishi Tanabe Pharma Corp. (Tokyo:4508) and Johnson & Johnson (NYSE:JNJ), noting increased risk of leg and foot amputations. The warning was prompted by final results from the Phase III CANVAS and Phase IV CANVAS-R trials which showed that amputations occurred about twice as often in patients treated with canagliflozin compared to patients who received placebo.

J&J markets Invokana, a sodium-glucose cotransporter 2 (SGLT2) inhibitor, in the U.S. and EU.

Invokamet canagliflozin/metformin IR is a fixed-dose combination of canagliflozin and immediate-release metformin. J&J markets the product as Invokamet in the U.S. and as Vokanamet in the EU.

Invokamet XR canagliflozin/metformin is a fixed-dose combination of canagliflozin and extended-release metformin.

J&J has worldwide rights to canagliflozin, except in some parts of Asia, from Mitsubishi.

Nicox S.A. (Euronext:COX), Sophia Antipolis, France

Product: Zerviate cetirizine (AC-170)
Business: Inflammation

Nicox S.A. (Euronext:COX) said FDA approved an NDA for Zerviate cetirizine (AC-170) to treat ocular itching associated with allergic conjunctivitis. The approval is Nicox's first in the U.S.

Last October, FDA issued a complete response letter for Zerviate, citing a GMP inspection at a third party facility. The company resubmitted an NDA on March 8 (see BioCentury, Oct. 17, 2016 & March 13, 2017).

Nicox is seeking a U.S. partner for Zerviate, and anticipates a launch in 2018. It has not priced the drug.

Zerviate is a topical formulation of cetirizine, a histamine H1 receptor (HRH1) antagonist. Nicox submitted the NDA under section 505(b)(2) of the Food, Drug and Cosmetic Act, which allows sponsors to reference data on safety and efficacy from the scientific literature or previously approved products. Cetirizine is the active ingredient in the antihistamine Zyrtec.

Novartis AG (NYSE:NVS; SIX:NOVN), Basel, Switzerland

Product: Zykadia ceritinib (LDK378)
Business: Cancer

FDA granted regular approval to Zykadia ceritinib (LDK378) from Novartis AG (NYSE:NVS; SIX:NOVN) for the first-line treatment of metastatic non-small cell lung cancer (NSCLC) in anaplastic lymphoma kinase (ALK)-positive patients.

The small molecule ALK inhibitor had accelerated approval since 2014 to treat ALK-positive, metastatic NSCLC in patients who progressed on or are intolerant to Xalkori crizotinib (see BioCentury, May 5, 2014).

The agency based its approval on the Phase III ASCEND-4 trial in which Zykadia significantly improved median progression-free survival (PFS) vs. chemotherapy (16.6 vs. 8.1 months, HR=0.55, 95% CI: 0.42, 0.73, one-sided p<0.0001). Zykadia also led to a confirmed objective response rate (ORR) of 73% vs. 27% for chemotherapy.

Xalkori is a dual inhibitor of c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene), ALK and their oncogenic variants.

Novo Nordisk A/S (CSE:NOVOB; NYSE:NVO), Bagsvaerd, Denmark

Product: Rebinyn (Refixia - EU) nonacog beta pegol (N9-GP, pegylated recombinant human Factor IX, PEG-rFIX)
Business: Hematology

Novo Nordisk A/S (CSE:NOVOB; NYSE:NVO) said FDA approved a BLA for Rebinyn nonacog beta pegol (N9-GP, pegylated recombinant human Factor IX, PEG-rFIX) to treat and control bleeding in patients with hemophilia B. Specifically, the therapy is approved for on-demand treatment and control of bleeding episodes and the perioperative management of bleeding around the time of surgery in adults and children. Novo plans to launch the product in 1H18.

In March, EMA's CHMP recommended approval of an MAA for the product to treat and prevent bleeding in patients ages ≥12 with hemophilia B. The product has Orphan Drug designation in the EU, where it is called Refixia (see BioCentury, April 3).

Rebinyn is a glyco-pegylated derivative of recombinant human Factor IX.

Roche (SIX:ROG; OTCQX:RHHBY), Basel, Switzerland

Product: Ventana PD-L1 (SP263) Assay
Business: Diagnostic

Roche (SIX:ROG; OTCQX:RHHBY) received CE Mark approval for its Ventana PD-L1 (SP263) Assay to identify metastatic non-small cell lung cancer (NSCLC) patients who are eligible for treatment with Keytruda pembrolizumab. The assay already has CE Mark approval as a complementary diagnostic for Opdivo nivolumab to treat NSCLC.

Merck & Co. Inc. (NYSE:MRK) markets Keytruda. Ono Pharmaceutical Co. Ltd. (Tokyo:4528) and Bristol-Myers Squibb Co. (NYSE:BMY) share rights to Opdivo. Ventana PD-L1 (SP263) Assay is an *in vitro* diagnostic which uses immunohistochemistry (IHC) technology to visually enhance and score PD-L1 protein on tumor-infiltrating immune cells.

Sumitomo Dainippon Pharma Co. Ltd. (Tokyo:4506), Osaka, Japan

Product: Glycopyrrolate (SUN-101) (formerly EP-101)
Business: Pulmonary

The Sunovion Pharmaceuticals Inc. unit of Sumitomo Dainippon Pharma Co. Ltd. (Tokyo:4506) said FDA issued a complete response letter for SUN-101 as a long-term maintenance treatment for airflow obstruction in patients with chronic obstructive pulmonary disease (COPD). SUN-101 is a formulation of glycopyrrolate, a long-acting muscarinic antagonist (LAMA), delivered via the eFlow nebulizer system.

Sunovion did not disclose a specific reason for the CRL and did not respond to inquiries. The company said it will work with FDA to determine a path forward for the therapy and added it would not need to conduct additional studies.

Pari GmbH (Starnberg, Germany) developed the eFlow system. Sumitomo gained SUN-101 through its 2012 acquisition of Elevation Pharmaceuticals Inc. (see BioCentury, Sept. 10, 2012).

Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), Novato, Calif.

Product: rhGUS (UX003)
Business: Endocrine/Metabolic

Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE) said U.S. and EU regulators are each reviewing rhGUS (UX003), its therapy to treat mucopolysaccharidosis VII (MPS VII; Sly syndrome).

The company said FDA granted Priority Review to its BLA for rhGUS. Its PDUFA date is Nov. 16. Ultragenyx expects an opinion in 1H18 from EMA's CHMP on its MAA, which the agency has accepted for review. rhGUS has Orphan Drug designation in the U.S. and EU for the indication.

rhGUS is an enzyme replacement therapy consisting of recombinant human glucuronidase beta. Data from a 12-patient Phase III trial announced in July 2016 showed that rhGUS met the primary endpoint of reducing urinary glycosaminoglycan (GAG) excretion after 24 weeks of treatment compared to baseline (see BioCentury, July 18, 2016).

Ultragenyx has said that FDA advised against using a primary clinical endpoint due to the disease's rarity and heterogeneity, and that the agency plans to evaluate the study "based on the totality of the data on a patient-by-patient basis." The company also said EMA "has agreed that approval under exceptional circumstances could be possible" based on the Phase III trial, with urinary GAG levels serving as a surrogate primary endpoint.

There are no approved therapies for MPS VII.

CLINICAL NEWS

CLINICAL RESULTS

Aerie Pharmaceuticals Inc. (NASDAQ:AERI), Irvine, Calif.

Product: Roclatan netarsudil/latanoprost (PG324)
Business: Ophthalmic
Molecular target: Rho kinase; Norepinephrine transporter
Description: Fixed-dose combination of Rhopressa netarsudil 0.02%, a dual inhibitor of Rho kinase and the norepinephrine transporter, and latanoprost 0.005%, a prostaglandin analog
Indication: Treat intraocular pressure (IOP) in glaucoma and ocular hypertension patients
Endpoint: Change from baseline in IOP at various time points; safety and change from baseline in visual acuity, visual field and pupil size
Status: Phase III data
Milestone: Start Phase III (mid-2017); submit NDA (1H18); submit MAA (2H19)

Aerie Pharmaceuticals Inc. (NASDAQ:AERI) reported top-line data from the Phase III Mercury 2 trial in 750 patients with open-angle glaucoma or ocular hypertension with a baseline intraocular pressure (IOP) of >20 to <36 mmHg showing that once-daily Roclatan netarsudil/latanoprost (PG324) eye drops given in the evening met the primary endpoint of a lower mean IOP at 9 measured time points vs. each of Roclatan's components -- Aerie's Rhopressa netarsudil 0.02% and latanoprost 0.005% eye drops. Specifically, Roclatan lowered mean IOP by 2.2-3.3 mmHg over Rhopressa ($p<0.0001$ for all) and by 1.5-2.4 mmHg over latanoprost ($p<0.0001$ for all). IOP was measured at 8 a.m., 10 a.m. and 4 p.m. at week 2, week 6 and day 90.

Additionally, 27% of patients receiving Roclatan achieved a ≥40% reduction in IOP at day 90 vs. 5% for Rhopressa and 8% for latanoprost ($p<0.0001$ for both). Furthermore, 56% of patients receiving Roclatan achieved a mean diurnal IOP of ≤16 mmHg at day 90 vs. 25% for Rhopressa and 36% for latanoprost ($p<0.0001$ for both). The most common adverse event reported was hyperemia. No treatment-related serious adverse events were reported.

Secondary endpoints of the double-blind, U.S. trial include safety and change from baseline in visual acuity, visual field and pupil size.

Aerie plans to submit an NDA to FDA for Roclatan in 1H18. The company also plans to start the European Phase III Mercury 3 trial of the product mid-year, and hopes to submit a regulatory application in Europe in 2H19.

Roclatan is a fixed-dose combination of Rhopressa, a dual inhibitor of Rho kinase and the norepinephrine transporter, and latanoprost, a prostaglandin analog. Rhopressa is under FDA review to treat IOP in patients with glaucoma and ocular hypertension; the PDUFA date is Feb. 28, 2018.

Array BioPharma Inc. (NASDAQ:ARRY), Boulder, Colo.
Aslan Pharmaceuticals Ltd. (TPEx:6497), Singapore

Product: Varlitinib (ARRY-543, ASLAN001)
Business: Cancer
Molecular target: EGFR; HER2
Description: Oral small molecule inhibitor of EGFR, HER2 and HER4
Indication: Treat metastatic solid tumors

Endpoint: Safety, dose limiting toxicities (DLTs) and maximum tolerated dose (MTD); overall response rate (ORR) and pharmacokinetics
Status: Phase Ib data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Aslan Pharmaceuticals Ltd. (TPEx:6497) reported data from 15 evaluable patients with metastatic solid tumors in an open-label Phase Ib trial showing that varlitinib (ARRY-543, ASLAN001) plus cisplatin-based doublet chemotherapy led to 3 partial responses and 10 cases of stable disease. In 1 cholangiocarcinoma patient, the combination reduced liver tumor size by 87% after 2 cycles and led to complete resolution in 1 liver lesion after 4 cycles. In another cholangiocarcinoma patient without measurable lesions in the liver or biliary tract, the combination led to disease control for 15.2 months. The combination was well tolerated. Patients received twice-daily 300-500 mg varlitinib plus cisplatin and 5-fluorouracil (5-FU) or cisplatin and capecitabine for 6 cycles before switching to varlitinib monotherapy.

Varlitinib is an oral small molecule inhibitor of EGFR, HER2 and HER4. The product is in Phase II testing to treat biliary tract cancer and gastric cancer. Aslan has exclusive, worldwide rights to varlitinib from Array BioPharma Inc. (NASDAQ:ARRY) (see BioCentury, July 18, 2011).

Blueprint Medicines Corp. (NASDAQ:BPMC), Cambridge, Mass.

Product: BLU-285
Business: Cancer
Molecular target: Stem cell factor (SCF) receptor tyrosine kinase (c-Kit; KIT; CD117); Platelet derived growth factor receptor A (PDGFRA; PDGFR2; CD140A)
Description: Inhibitor of c-Kit exon 17 mutations and PDGFRA with the D824V amino acid substitution
Indication: Treat unresectable gastrointestinal tumors (GIST) and other relapsed or refractory solid tumors
Endpoint: Maximum tolerated dose (MTD) and recommended Phase II dose (Part 1), safety (Parts 1 and 2) and overall response rate (ORR) (Part 2); pharmacokinetics, duration of response, progression-free survival (PFS) and clinical benefit rate (CBR)
Status: Additional Phase I data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Blueprint Medicines Corp. (NASDAQ:BPMC) reported additional data from an open-label, international Phase I trial evaluating once-daily 30-600 mg oral BLU-285 to treat unresectable gastrointestinal tumors (GIST) and other relapsed or refractory solid tumors. In 17 evaluable patients with platelet derived growth factor receptor A (PDGFRA; PDGFR2; CD140A)-driven GIST, BLU-285 led to an overall response rate (ORR) of 41%, including 7 confirmed partial responses, plus 10 cases of stable disease. In 11 evaluable patients with stem cell factor (SCF) receptor tyrosine kinase (c-Kit; KIT; CD117)-driven GIST, BLU-285 led to an ORR of 18%, including 2 partial responses, plus 5 cases of stable disease.

The trial is evaluating primary endpoints of maximum tolerated dose (MTD) and recommended Phase II dose (Part 1), safety (Parts 1 and 2) and ORR (ORR) (Part 2). Secondary endpoints include pharmacokinetics, duration of response, progression-free survival (PFS) and clinical benefit rate (CBR).

Last December, Blueprint reported preliminary data from the trial (see BioCentury, Dec. 12, 2016). BLU-285 is an inhibitor of c-Kit exon 17 mutations and PDGFRA with the D824V amino acid substitution.

Bristol-Myers Squibb Co. (NYSE:BMY), New York, N.Y.
Ono Pharmaceutical Co. Ltd. (Tokyo:4528), Osaka, Japan

Product: BMS-986016
Business: Cancer
Molecular target: Lymphocyte-activation gene 3 (LAG3; CD223)
Description: IgG4 mAb targeting LAG3
Indication: Treat advanced solid tumors
Endpoint: Safety, objective response rate (ORR), disease control rate (DCR) and duration of response
Status: Phase I/IIa data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Bristol-Myers Squibb Co. (NYSE:BMY) reported data from a cohort of 31 evaluable melanoma patients who progressed on or after anti-PD-1 or anti-PD-L1 therapy in a Phase I/IIa trial showing that 80 mg BMS-986016 plus Opdivo nivolumab every 2 weeks led to an objective response rate (ORR) of 16% and disease control rate (DCR) of 45%. The open-label, dose-escalation, international trial is evaluating BMS-986016 with and without Opdivo in about 360 patients with advanced solid tumors.

BMS-986016 is an IgG4 mAb targeting lymphocyte-activation gene 3 (LAG3; CD223).

BMS has worldwide rights to Opdivo from Ono Pharmaceutical Co. Ltd. (Tokyo:4528), except in Japan, South Korea and Taiwan, where Ono and BMS are partnered for the product. The companies are also partnered to jointly develop and commercialize BMS-986016 in Japan, South Korea and Taiwan (see BioCentury, July 28, 2014).

CASI Pharmaceuticals Inc. (NASDAQ:CASI), Rockville, Md.

Product: ENMD-2076
Business: Cancer
Molecular target: Aurora kinase A (AURKA; Aurora-A); VEGF receptor 2 (VEGFR-2; KDR/Flk-1)
Description: Inhibitor of AURKA and multiple tyrosine kinases
Indication: Treat recurrent clear cell ovarian cancer
Endpoint: 6-month progression-free survival (PFS) rate and overall response rate (ORR); duration of response, biomarkers and safety
Status: Phase II data

Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, CASI Pharmaceuticals Inc. (NASDAQ:CASI) reported data from 37 evaluable patients with recurrent clear cell ovarian cancer in a Phase II trial showing that once-daily 275 mg oral ENMD-2076 led to 2 partial responses, plus 25 cases of stable disease. Median progression-free survival (PFS) was 3.7 months and the estimated 6-month PFS rate was 20%. In patients with AT rich interactive domain 1A (ARID1A) loss (n=19), median PFS was 4.1 months and the estimated 6-month PFS rate was 31%. In ARID1A-positive patients (n=17), median PFS was 3.6 months and the estimated 6-month PFS rate was 12%. ENMD-2076 was well tolerated.

The open-label, Canadian trial is evaluating 6-month PFS rate and overall response rate (ORR) as its co-primary endpoints. Secondary endpoints include duration of response, biomarkers and safety.

The trial was conducted by the Princess Margaret Phase 2 Consortium. ENMD-2076 is an inhibitor of aurora kinase A (AURKA; Aurora-A) and multiple tyrosine kinases.

Epizyme Inc. (NASDAQ:EPZM), Cambridge, Mass.
Eisai Co. Ltd. (Tokyo:4523), Tokyo, Japan

Product: Tazemetostat (E7438, EPZ-6438)
Business: Cancer
Molecular target: Enhancer of zeste homolog 2 (EZH2)
Description: Selective inhibitor of EZH2
Indication: Treat SWI/SNF related matrix associated actin dependent regulator of chromatin subfamily b member 1 (SMARCB1; SNF5; INI1)-negative tumors or relapsed or refractory synovial sarcoma
Endpoint: Progression-free survival (PFS) (relapsed or refractory synovial sarcoma cohort) and disease control rate (DCR) (epithelioid sarcoma cohort); objective response rate (ORR), PFS in other cohorts, overall survival (OS), duration of response, safety and pharmacokinetics
Status: Interim Phase II data
Milestone: Additional Phase II data (2017); Submit NDA (2018)

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Epizyme Inc. (NASDAQ:EPZM) reported interim data from a cohort of 31 evaluable patients with SWI/SNF related matrix associated actin dependent regulator of chromatin subfamily b member 1 (SMARCB1; SNF5; INI1)-negative relapsed or refractory epithelioid sarcoma in a Phase II trial showing that twice-daily 800 mg oral tazemetostat (E7438, EPZ-6438) led to a disease control rate (DCR), the cohort's primary endpoint, of 32%, including 4 confirmed partial responses and 6 cases of stable disease for ≥32 weeks. Tazemetostat led to median progression-free survival (PFS) of 5.7 months and median duration of response of 7 months in the epithelioid sarcoma cohort. Next year, Epizyme plans submit an NDA to FDA seeking accelerated approval for tazemetostat to treat epithelioid sarcoma based on data from the cohort.

Additionally, Epizyme said it will not advance tazemetostat as a monotherapy to treat synovial sarcoma after the product led to 10 cases of stable disease in a cohort of 33 evaluable synovial sarcoma patients. No objective responses were observed in the cohort.

The open-label, international trial is slated to enroll about 180 patients with INI1-negative tumors or relapsed or refractory synovial sarcoma. Secondary endpoints include objective response rate (ORR), PFS in other cohorts, overall survival (OS), duration of response and pharmacokinetics. Epizyme plans to report data from the rest of the trial's cohorts this year.

Tazemetostat is a selective inhibitor of enhancer of zeste homolog 2 (EZH2). Eisai Co. Ltd. (Tokyo:4523) has Japanese rights to tazemetostat from Epizyme (see BioCentury, March 16, 2015).

Genentech Inc., South San Francisco, Calif.
NewLink Genetics Corp. (NASDAQ:NLNK), Ames, Iowa
Roche (SIX:ROG; OTCQX:RHHBY), Basel, Switzerland

Product: Navoximod (GDC-0919, NLG919, RG6078)
Business: Cancer
Molecular target: Indoleamine 2,3-dioxygenase (INDO; IDO)
Description: Oral small molecule inhibitor of INDO
Indication: Treat locally advanced or metastatic solid tumors
Endpoint: Dose-limiting toxicities (DLTs) and safety; maximum tolerated dose (MTD), recommended Phase II dose, pharmacokinetics, objective response rate (ORR) and duration of response
Status: Preliminary Phase Ib data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, the Genentech Inc. unit of Roche (SIX:ROG; OTCQX:RHHBY) reported preliminary data from 45 evaluable patients with locally advanced or metastatic solid tumors in the dose-escalation portion of a Phase Ib trial showing that navoximod (GDC-0919, NLG919, RG6078) plus PD-L1 mAb Tecentriq atezolizumab led to 4 partial responses and 11 cases of stable disease. The combination was generally well tolerated. The maximum tolerated dose (MTD) has not been identified. Patients received twice-daily 50-1,000 mg oral navoximod for 21 days plus Tecentriq every 3 weeks. The open-label, international trial is evaluating dose-limiting toxicities (DLTs) and safety as the primary endpoints. Secondary endpoints include MTD, recommended Phase II dose, pharmacokinetics, objective response rate (ORR) and duration of response. The trial is enrolling about 305 patients in expansion cohorts.

Navoximod is an oral small molecule inhibitor of indoleamine 2,3-dioxygenase (INDO; IDO). In 2014, NewLink Genetics Corp. (NASDAQ:NLNK) granted Genentech exclusive, worldwide rights to develop and commercialize navoximod (see BioCentury, Oct. 27, 2014).

Gilead Sciences Inc. (NASDAQ:GILD), Foster City, Calif.

Product: Bictegravir/emtricitabine/tenofovir alafenamide
Business: Infectious

Molecular target: HIV reverse transcriptase
Description: Fixed-dose combination of bictegravir (GS-9883), an integrase strand transfer inhibitor (INSTI), emtricitabine, a nucleoside reverse transcriptase inhibitor (NRTI), and tenofovir alafenamide, a prodrug of the NRTI tenofovir
Indication: Treat HIV-1 infection
Endpoint: Proportion of patients with virologic failure defined as HIV-1 RNA levels of ≥50 copies/mL at week 48; proportion of patients with HIV-1 RNA levels of <50 copies/mL at week 48 and change from baseline in CD4+ T cell count
Status: Phase III data
Milestone: Submit NDA (2Q17); submit MAA (3Q17)

Gilead Sciences Inc. (NASDAQ:GILD) reported data from the Phase III GS-US-380-1844 and GS-US-380-1878 trials in virologically suppressed patients with HIV-1 infection showing that once-daily 50/200/25 mg bictegravir/emtricitabine/tenofovir alafenamide met the primary endpoint in both trials of non-inferiority in the proportion of patients with virologic failure defined as HIV-1 RNA levels of ≥50 copies/mL at week 48. Gilead plans to submit an NDA to FDA this month and an MAA to EMA in 3Q17 for the combination.

In the double-blind, international GS-US-380-1844 trial, patients were randomized to switch to bictegravir/emtricitabine/tenofovir alafenamide or stay on their existing regimen of either Tivicay dolutegravir plus abacavir/lamivudine or Triumeq dolutegravir/abacavir/lamivudine. In the open-label, international GS-US-380-1878 trial, patients were randomized to switch to bictegravir/emtricitabine/tenofovir alafenamide or stay on a suppressive regimen that included 2 nucleoside/nucleotide reverse transcriptase inhibitors and a boosted protease inhibitor. Regimens included Prezista darunavir from Johnson & Johnson (NYSE:JNJ) or Reyataz atazanavir from Bristol-Myers Squibb (NYSE:BMY), plus abacavir/lamivudine or Truvada emtricitabine/tenofovir disoproxil fumarate. Each trial enrolled 520 patients.

The product is a fixed-dose combination of bictegravir (GS-9883), an integrase strand transfer inhibitor (INSTI); emtricitabine, a nucleoside reverse transcriptase inhibitor (NRTI); and tenofovir alafenamide, a prodrug of the NRTI tenofovir. Gilead markets Descovy emtricitabine/tenofovir alafenamide.

Gilead Sciences Inc. (NASDAQ:GILD), Foster City, Calif.

Product: Bictegravir/emtricitabine/tenofovir alafenamide
Business: Infectious
Molecular target: HIV reverse transcriptase
Description: Fixed-dose combination of bictegravir (GS-9883), an integrase strand transfer inhibitor (INSTI), emtricitabine, a nucleoside reverse transcriptase inhibitor (NRTI), and tenofovir alafenamide, a prodrug of the NRTI tenofovir
Indication: Treat HIV-1 infection
Endpoint: Proportion of patients with HIV-1 RNA levels of <50 copies/mL at week 48; proportion of patients with HIV-1 RNA levels of <50 copies/mL at week 96 and 144 and change from baseline in CD4+ T cell count
Status: Phase III data
Milestone: Submit NDA (2Q17); submit MAA (3Q17)

Gilead Sciences Inc. (NASDAQ:GILD) reported data from the Phase III GS-US-380-1489 and GS-US-380-1490 trials in treatment-naïve patients with HIV-1 infection showing that once-daily 50/200/25 mg bictegravir/emtricitabine/tenofovir alafenamide met the primary endpoint in both trials of non-inferiority to a dolutegravir-containing regimen in the proportion of patients who achieved HIV-1 RNA levels of <50 copies/mL at week 48. Gilead plans to submit an NDA to FDA this month and an MAA to EMA in 3Q17 for the combination.

In GS-US-380-1489, bictegravir/emtricitabine/tenofovir alafenamide was non-inferior to Triumeq dolutegravir/abacavir/lamivudine on the primary endpoint. In GS-US-380-1490, bictegravir/emtricitabine/tenofovir alafenamide was non-inferior to Tivicay dolutegravir plus Gilead's Descovy emtricitabine/tenofovir alafenamide on the primary endpoint. The double-blind, international trials each enrolled 600 patients.

The product is a fixed-dose combination of bictegravir (GS-9883), an integrase strand transfer inhibitor (INSTI); emtricitabine, a nucleoside reverse transcriptase inhibitor (NRTI); and tenofovir alafenamide, a prodrug of the NRTI tenofovir.

Hutchison China MediTech Ltd. (LSE:HCM; NASDAQ:HCM), Hong Kong, China

Product: Sulfatinib (HMPL-012)
Business: Cancer
Molecular target: VEGF receptor; Fibroblast growth factor (FGF) receptor (FGFR)
Description: Selective inhibitor of VEGF receptor, FGFR and colony-stimulating factor 1 receptor (CSF1R; C-FMS; CD115)
Indication: Treat advanced medullary thyroid cancer (MTC) or radioiodine-refractory differentiated thyroid cancer (DTC)
Endpoint: Objective response rate (ORR); safety, disease control rate (DCR), progression-free survival (PFS) and time to response
Status: Phase II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Hutchison China MediTech Ltd. (LSE:HCM; NASDAQ:HCM) reported data from 17 evaluable patients with advanced medullary thyroid cancer (MTC) or radioiodine-refractory differentiated thyroid cancer (DTC) in a Phase II trial showing that once-daily 300 mg oral sulfatinib (HMPL-012) in 28-day cycles led to 4 confirmed partial responses and 13 cases of stable disease. There was 1 partial response in the MTC cohort and 3 in the DTC cohort. Sulfatinib was well tolerated with proteinuria, hypertriglyceridemia, hypertension, elevated

blood bilirubin levels and diarrhea reported as the most common adverse events.

The primary endpoint of the open-label, Chinese trial is objective response rate (ORR). Secondary endpoints include safety, disease control rate (DCR), progression-free survival (PFS) and time to response.

Sulfatinib is a selective inhibitor of VEGF receptor, fibroblast growth factor (FGF) receptor (FGFR) and colony-stimulating factor 1 receptor (CSF1R; C-FMS; CD115).

Lion Biotechnologies Inc. (NASDAQ:LBIO), San Carlos, Calif.

Product: LN-144 (formerly Contego)
Business: Cancer
Molecular target: NA
Description: Autologous T cell therapy that utilizes tumor infiltrating lymphocytes (TILs) derived from the patient's tumor
Indication: Treat advanced metastatic melanoma
Endpoint: Objective response rate (ORR); complete response rate, duration of response, disease control rate (DCR), progression-free survival, overall survival (OS) and safety
Status: Interim Phase II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Lion Biotechnologies Inc. (NASDAQ:LBIO) reported interim data from 9 evaluable patients with advanced metastatic melanoma who failed ≥1 prior systemic therapy in a Phase II trial showing that LN-144 led to an objective response rate (ORR), the primary endpoint, of 33%, including 1 complete response and 2 partial responses, plus 2 cases of stable disease. Median time to best response was 3 months. The most common treatment-emergent adverse event reported was hypophosphatemia. Patients received LN-144 followed by IL-2 after a non-myeloablative chemotherapy preparative regimen.

The open-label, U.S. trial is enrolling about 60 patients. Secondary endpoints include complete response rate, duration of response, disease control rate (DCR), progression-free survival, overall survival (OS) and safety.

The product is an autologous T cell therapy that utilizes tumor infiltrating lymphocytes (TILs) derived from the patient's tumor.

Merck KGaA (Xetra:MRK), Darmstadt, Germany

Product: Tepotinib (MSC2156119J)
Business: Cancer
Molecular target: c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)
Description: Small molecule c-MET inhibitor
Indication: Second-line treatment of c-MET-positive advanced hepatocellular carcinoma (HCC)
Endpoint: Dose-limiting toxicities (DLTs); time to progression, progression-free survival (PFS), time to symptomatic progression, overall survival (OS), overall response rate (ORR), disease control rate (DCR) and biological response rate
Status: Phase Ib/II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Merck KGaA (Xetra:MRK) reported data from 17 patients with hepatocellular carcinoma (HCC) who failed prior treatment with Nexavar sorafenib in the Phase Ib portion of a Phase Ib/II trial showing that second-line treatment with once-daily 300 or 500 mg oral tepotinib (MSC2156119J) led to 2 partial responses and 4 cases of stable disease. In the 2 patients with a partial response, tepotinib led to reductions in tumor size from baseline of 55% and >60%. Median overall survival (OS) was 7.2 months. The recommended Phase II dose of tepotinib is 500 mg daily. Tepotinib was well tolerated with no dose-limiting toxicities (DLTs) reported. The most common treatment-related adverse events were peripheral edema, lipase increase, acute kidney injury, renal impairment, fatigue, nausea and asthenia.

The Phase Ib portion of the open-label trial enrolled patients with c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)-positive, advanced HCC. The Phase II portion of the trial is ongoing. The trial's primary endpoint is DLTs. Secondary endpoints include time to progression, progression-free survival (PFS), time to symptomatic progression, overall survival (OS), overall response rate (ORR), disease control rate (DCR) and biological response rate.

Tepotinib is a small molecule c-MET inhibitor.

Merck KGaA (Xetra:MRK), Darmstadt, Germany

Product: Tepotinib (MSC2156119J)
Business: Cancer
Molecular target: c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)
Description: Small molecule c-MET inhibitor
Indication: Treat advanced lung adenocarcinoma harboring the c-MET exon 14 skipping mutation
Endpoint: Objective response rate (ORR); duration of response, disease control rate (DCR), progression-free survival (PFS), overall survival (OS), safety, quality of life (QOL) and pharmacokinetics
Status: Phase II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Merck KGaA (Xetra:MRK) reported data from 3 evaluable patients with advanced lung adenocarcinoma in a Phase II trial showing that once-daily 500 mg oral tepotinib (MSC2156119J) led to 2 unconfirmed partial responses and 1 case of stable disease. Tepotinib was well tolerated.

The open-label, international trial is enrolling about 60 lung adenocarcinoma patients who previously failed 1-2 prior therapies,

including a platinum-doublet-containing regimen, and whose tumors harbor a skipping mutation in exon 14 of c-MET receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene).

The trial's primary endpoint is objective response rate (ORR). Secondary endpoints include duration of response, disease control rate (DCR), progression-free survival (PFS), overall survival (OS), safety, quality of life (QOL) and pharmacokinetics.

Tepotinib is a small molecule c-MET inhibitor.

Merck KGaA (Xetra:MRK), Darmstadt, Germany

Product: Tepotinib (MSC2156119J)
Business: Cancer
Molecular target: c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)
Description: Small molecule c-MET inhibitor
Indication: First-line treatment of c-MET-positive advanced hepatocellular carcinoma (HCC)
Endpoint: Dose-limiting toxicities (DLTs), safety and time to progression; progression-free survival (PFS), overall survival (OS), pharmacokinetics, objective response rate (ORR) and disease control rate (DCR)
Status: Phase Ib/II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Merck KGaA (Xetra:MRK) reported data from 27 Asian patients with hepatocellular carcinoma (HCC) in the Phase Ib portion of an open-label Phase Ib/II trial showing that first-line treatment with once-daily oral tepotinib (MSC2156119J) led to 2 partial responses, 8 cases of stable disease and 1 case of non-complete response/non-progressive disease. Five patients were progression free for >8 months. Tepotinib was well tolerated with no dose-limiting toxicities (DLTs) reported. The maximum tolerated dose (MTD) was not reached.

The Phase Ib portion of the open-label trial enrolled treatment-naïve patients with c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)-positive advanced HCC to receive once-daily 300, 500 or 1,000 mg tepotinib in 21-day cycles. The Phase II portion of the trial is evaluating first-line treatment with tepotinib compared to Nexavar sorafenib.

The trial's primary endpoints are DLTs, safety and time to progression. Secondary endpoints include progression-free survival (PFS) , overall survival (OS), pharmacokinetics, objective response rate (ORR) and disease control rate (DCR).
Tepotinib is a small molecule c-MET inhibitor.

Merck KGaA (Xetra:MRK), Darmstadt, Germany

Product: Tepotinib (MSC2156119J)
Business: Cancer
Molecular target: c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)
Description: Small molecule c-MET inhibitor
Indication: Treat c-MET-positive, locally advanced or metastatic non-small cell lung cancer (NSCLC) harboring an EGFR mutation
Endpoint: Recommended Phase II dose; pharmacokinetics, safety and antitumor activity
Status: Phase Ib data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Merck KGaA (Xetra:MRK) reported data from 18 patients with non-small cell lung cancer (NSCLC) in an Asian Phase Ib trial showing that once-daily 300 and 500 mg oral tepotinib (MSC2156119J) plus Iressa gefitinib led to 6 partial response and 4 cases of stable disease. The recommended Phase II dose of tepotinib in combination with Iressa is 500 mg daily. The combination was well tolerated with no dose-limiting toxicities (DLTs) reported. The most common treatment-related adverse events were diarrhea, rash and amylase increase.

The trial enrolled patients with c-Met receptor tyrosine kinase (c-MET; MET; HGFR; c-Met proto-oncogene)-positive, locally advanced or metastatic NSCLC harboring an EGFR mutation. The trial's primary endpoint is recommended Phase II dose. Secondary endpoints include pharmacokinetics, safety and antitumor activity.

A Phase II trial is evaluating tepotinib plus Iressa as second-line treatment in about 156 patients with c-MET-positive NSCLC.

Tepotinib is a small molecule c-MET inhibitor.

NewLink Genetics Corp. (NASDAQ:NLNK), Ames, Iowa

Product: Indoximod (1-MT, NLG8189, 1-methyl-D-tryptophan)
Business: Cancer
Molecular target: Indoleamine 2,3-dioxygenase (INDO; IDO)
Description: INDO pathway inhibitor
Indication: Treat metastatic castration-resistant prostate cancer (CRPC)
Endpoint: Immune response to PA2024 at 14 weeks; safety, overall survival (OS), progression-free survival (PFS), objective response rate (ORR) and quality of life (QOL)
Status: Phase II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, NewLink Genetics Corp. (NASDAQ:NLNK) reported data from 35 evaluable patients with metastatic castration-resistant prostate cancer (CRPC) in a Phase II trial showing that indoximod (1-MT, NLG8189, 1-methyl-D-tryptophan) following completion of treatment with Provenge sipuleucel-T led to no significant difference in augmenting immune response to PA2024 at week 14, the primary endpoint, vs. placebo following Provenge. PA2024 is the fusion protein component of Provenge consisting of recombinant prostatic acid phosphatase and GM-CSF. Additionally, there was difference in prostate-specific antigen (PSA; KLK3) progression between treatment arms.

Indoximod following Provenge did significantly improve median radiographic progression-free survival (PFS) vs. placebo following Provenge (10.3 vs. 4.1 months, p=0.011). Median overall survival (OS), a secondary endpoint, has not yet been reached. The double-blind, U.S. trial enrolled 46 patients to receive twice-daily 600 mg oral indoximod or placebo for 6 months following Provenge. Other secondary endpoints in the trial include safety, PFS, objective response rate (ORR) and quality of life (QOL). The trial was sponsored by the Masonic Cancer Center.

Indoximod is an indoleamine 2,3-dioxygenase (INDO; IDO) pathway inhibitor.

Puma Biotechnology Inc. (NASDAQ:PBYI), Los Angeles, Calif.
Pfizer Inc. (NYSE:PFE), New York, N.Y.

Product: Oral neratinib (HKI-272, PB272)
Business: Cancer
Molecular target: EGFR1 (HER1; ErbB1); HER2
Description: Oral inhibitor of HER1, HER2 and HER4 kinases
Indication: Treat HER2-positive breast cancer with brain metastases
Endpoint: Composite CNS objective response rate (ORR) requiring a ≥50% reduction in volumetric sum of target CNS lesions (volumetric ORR), no progression of non-target or non-CNS lesions, no new lesions, no escalating steroid use and no progressive neurologic symptoms; progression-free survival (PFS), overall survival (OS), CNS response by Macdonald criteria, first site of disease progression, safety, and association of circulating tumor cell (CTC) count and OS
Status: Additional Phase II data
Milestone: PDUFA date (7/21/2017)

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Puma Biotechnology Inc. (NASDAQ:PBYI) reported data from 37 evaluable HER2-positive breast cancer patients with progressive brain metastases in the third cohort of the Phase II TBCRC 022 trial showing that once-daily 240 mg oral neratinib (HKI-272, PB272) plus capecitabine led to a ≥50% reduction in volumetric sum of target CNS lesions in 49% of patients. The 12-month overall survival (OS) rate was 63%.

The open-label, U.S. trial is evaluating the primary endpoint of a composite CNS objective response rate (ORR) requiring a ≥50% reduction in volumetric sum of target CNS lesions (volumetric ORR), no progression of non-target or non-CNS lesions, no new lesions, no escalating steroid use and no progressive neurologic symptoms. Secondary endpoints include progression-free survival (PFS), OS, CNS response by Macdonald criteria, first site of disease progression, safety, and association of circulating tumor cell (CTC) count and OS. The trial was sponsored by the Dana-Farber Cancer Institute. Puma previously reported data from the trial (see BioCentury, June 9, 2014).

Last month, FDA's Oncologic Drugs Advisory Committee voted 12-4 that the overall benefit-risk profile for neratinib supports approval to treat HER2-positive breast cancer in the extended adjuvant setting. Puma has proposed the brand name Nerlynx for neratinib, which has a July 21 PDUFA date. An MAA for neratinib is under review in the EU (see BioCentury, May 29).

Neratinib is an inhibitor of HER1, HER2 and HER4 kinases. Puma has exclusive, worldwide rights to develop and commercialize neratinib from Pfizer Inc. (NYSE:PFE) (see BioCentury, Oct. 10, 2011).

Regeneron Pharmaceuticals Inc. (NASDAQ:REGN), Tarrytown, N.Y.
Sanofi (Euronext:SAN; NYSE:SNY), Paris, France

Product: REGN2810, SAR439684
Business: Cancer
Molecular target: PD-1
Description: Human mAb against PD-1
Indication: Treat advanced solid tumors
Endpoint: Safety and dose-limiting toxicities (DLTs); overall response rate (ORR) and immune-related response
Status: Additional Phase I data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Regeneron Pharmaceuticals Inc. (NASDAQ:REGN) reported data from an expansion cohort of 23 evaluable patients with unresectable locally advanced or metastatic cutaneous squamous cell carcinoma (CSCC) in a Phase I trial showing that 3 mg/kg IV REGN2810 (SAR439684) every 2 weeks for up to 48 weeks led to an overall response rate (ORR) of 52%, including 3 complete responses and 9 partial responses, plus 4 cases of stable disease. Median progression-free survival (PFS) and overall survival (OS) have not been reached.

The open-label, international trial is evaluating REGN2810 to treat advanced solid tumors as monotherapy and in combination with hypofractionated radiotherapy and other antitumor regimens. The trial's primary endpoints are safety and dose-limiting toxicities (DLTs). Secondary endpoints include ORR and immune-related response. The trial includes multiple expansion cohorts evaluating REGN2810 at the recommended dose of 3 mg/kg every 2 weeks.

Regeneron previously reported preliminary data from the trial (see BioCentury, June 6, 2016). The human mAb against PD-1 is in a Phase II trial to treat advanced CSCC.

Regeneron and Sanofi (Euronext:SAN; NYSE:SNY) are partnered to jointly develop and commercialize REGN2810 (see BioCentury, Aug. 3, 2015).

Targovax A/S (OSE:TRVX), Oslo, Norway

Product: TG01
Business: Cancer
Molecular target: NA
Description: Peptide vaccine targeting Ras mutations associated with cancer
Indication: Treat pancreatic cancer

Endpoint: Safety and immune response; disease-free survival (DFS), overall survival (OS) and relationship between K-Ras (KRAS) status and recurrence
Status: Additional Phase I/II data
Milestone: NA

In an abstract released ahead of the American Society of Clinical Oncology meeting in Chicago, Targovax A/S (OSE:TRVX) reported data from 19 evaluable patients with surgically resected pancreatic cancer in the open-label, European Phase I/II TG01-01 trial showing that 0.7 mg intradermal TG01 plus GM-CSF and gemcitabine led to a median overall survival (OS) of 33.1 months.

The trial's primary endpoints are safety and immune response. Secondary endpoints include disease-free survival (DFS), OS and the relationship between K-Ras (KRAS) status and recurrence. Targovax has previously reported data from the trial (see BioCentury, Feb. 17, 2014; July 20, 2015 & March 14, 2016).

TG01 is a peptide vaccine targeting Ras mutations associated with cancer.

Teva Pharmaceutical Industries Ltd. (NYSE:TEVA; Tel Aviv:TEVA), Petah Tikva, Israel
Otsuka Pharmaceutical Co. Ltd., Tokyo, Japan
Pfizer Inc. (NYSE:PFE), New York, N.Y.

Product: Fremanezumab (TEV-48125) (formerly RN-307, LBR-101)
Business: Neurology
Molecular target: Calcitonin gene-related peptide (CGRP)
Description: Humanized mAb targeting the CGRP ligand
Indication: Prevent chronic migraine
Endpoint: Mean change from baseline to 12 weeks in the number of monthly headache days of at least moderate severity; response rate, defined as achieving a >50% reduction in the number of monthly headache days, onset of efficacy, efficacy as monotherapy and disability
Status: Phase III data
Milestone: Phase III data (mid-2017); submit BLA (2017)

Teva Pharmaceutical Industries Ltd. (NYSE:TEVA; Tel Aviv:TEVA) reported top-line data from the Phase III HALO CM trial in 1,130 patients with chronic migraine showing that both dosing regimens of subcutaneous fremanezumab (TEV-48125) met the primary and all secondary endpoints vs. placebo. Teva plans to submit a BLA to FDA for fremanezumab this year.

On the primary endpoint, monthly and quarterly dosing regimens of fremanezumab reduced the number of monthly headache days of at least moderate severity during the 12-week period after the first dose by 4.6 and 4.3 days, respectively, vs. 2.5 days for placebo ($p<0.0001$ for both). Both regimens of fremanezumab also met the secondary endpoints of improving response rate, onset of efficacy, efficacy as monotherapy and disability vs. placebo.

Patients received placebo; a single dose of 675 mg fremanezumab (the quarterly regimen); or a single dose of 675 mg fremanezumab followed by once-monthly 225 mg fremanezumab for 2 months (the monthly regimen).

In the coming weeks, Teva plans to report top-line data from the Phase III HALO EM trial of fremanezumab to treat episodic migraine.

Fremanezumab is a humanized mAb targeting the calcitonin gene-related peptide (CGRP) ligand. Teva has exclusive, worldwide rights to the product from Pfizer Inc. (NYSE:PFE) (see BioCentury, June 9, 2014). Otsuka Pharmaceutical Co. Ltd. (Tokyo, Japan) has exclusive, Japanese rights to the product from Teva (see BioCentury, May 29).

United Biomedical Inc., Hauppauge, N.Y.
United Neuroscience Inc., Hauppauge, N.Y.

Product: UB-311
Business: Neurology
Molecular target: Beta amyloid
Description: Endobody vaccine targeting beta amyloid designed to induce high B cell specific responses while avoiding T cell inflammation
Indication: Treat Alzheimer's disease
Endpoint: Safety and immunogenicity
Status: Phase I data
Milestone: Phase II data (mid-2018)

United Neuroscience Inc. (Hauppauge, N.Y.) said UB-311 led to a 100% responder rate in a Phase I trial in 19 patients ages 50-80 with mild to moderate Alzheimer's disease. A responder was defined as achieving any increase from baseline in anti-beta amyloid antibody titers. UB-311 was well tolerated with injection-site swelling and agitation reported as the most common adverse events. Data were published in *Alzheimer's & Dementia: Translational Research & Clinical Interventions*. The 48-week, open-label, Taiwanese trial evaluated the safety and immunogenicity of a 300 µg dose of intramuscular UB-311 given at weeks 0, 4 and 12.

UB-311 is an endobody vaccine targeting beta amyloid designed to induce high B cell specific responses while avoiding T cell inflammation. Data from a Phase II trial of UB-311 to treat early to mild AD are expected in mid-2018.

In 2014, United Neuroscience spun out of United Biomedical Inc. (Hauppauge, N.Y.) and received exclusive, worldwide rights to UB-311.

CLINICAL NEWS

CLINICAL STATUS

Alnylam Pharmaceuticals Inc. (NASDAQ:ALNY), Cambridge, Mass.
The Medicines Co. (NASDAQ:MDCO), Parsippany, N.J.

Product: Inclisiran (formerly ALN-PCSsc, PCSK9si)
Business: Endocrine/Metabolic
Molecular target: PCSK9

Description: Subcutaneous formulation of small interfering RNA (siRNA) against PCSK9
Indication: Treat familial hypercholesterolemia and atherosclerotic cardiovascular disease
Endpoint: Change from baseline in LDL-C
Status: Phase III start
Milestone: Phase III start (2H17); submit NDA (year end 2019)

Next half, The Medicines Co. (NASDAQ:MDCO) and Alnylam Pharmaceuticals Inc. (NASDAQ:ALNY) plan to begin placebo-controlled Phase III trials to evaluate 300 mg subcutaneous inclisiran on day 1, 90 and every 6 months thereafter for a total of 4 doses over 18 months in about 3,000 patients with familial hypercholesterolemia and atherosclerotic cardiovascular disease. The primary endpoint will be the change from baseline in LDL-C. The partners plan to submit an NDA to FDA for inclisiran by year end 2019.

The Medicines Co. also plans to begin a cardiovascular outcomes trial (CVOT) in about 14,000 patients with atherosclerotic cardiovascular disease and/or risk equivalents to evaluate LDL-C reduction with inclisiran. The trial's primary endpoint will be a composite of coronary heart disease (CHD) death, non-fatal myocardial infarction (MI) and fatal and non-fatal ischemic stroke.

The product is a subcutaneous formulation of small interfering RNA (siRNA) against PCSK9. The Medicines Co. has exclusive, worldwide rights to develop and commercialize the ALN-PCS RNAi program from Alnylam to treat hypercholesterolemia under a 2013 deal (see BioCentury, Feb. 11, 2013).

Array BioPharma Inc. (NASDAQ:ARRY), Boulder, Colo.

Aslan Pharmaceuticals Ltd. (TPEx:6497), Singapore

Product: Varlitinib (ARRY-543, ASLAN001)
Business: Cancer
Molecular target: EGFR; HER2
Description: Oral small molecule inhibitor of EGFR, HER2 and HER4
Indication: Treat advanced or metastatic biliary tract cancer
Endpoint: Objective response rate (ORR); progression-free survival (PFS), overall survival (OS), safety, pharmacokinetics and duration of response
Status: Phase II/III started
Milestone: NA

Aslan Pharmaceuticals Ltd. (TPEx:6497) began the Phase II/III TreeTopp trial to evaluate varlitinib (ARRY-543, ASLAN001) as second-line therapy to treat 120 patients with advanced or metastatic biliary tract cancer. The double-blind, international trial will evaluate 300 mg oral varlitinib twice daily for 2 weeks of each 3-week cycle plus capecitabine vs. placebo plus capecitabine. The primary endpoint is objective response rate (ORR). The trial will also evaluate safety, progression-free survival (PFS), overall survival (OS), pharmacokinetics and duration of response.

The company said varlitinib has Orphan Drug designation from Korea FDA to treat biliary tract cancer.

Varlitinib is an oral small molecule inhibitor of EGFR, HER2 and HER4. Aslan has exclusive, worldwide rights to the product from Array BioPharma Inc. (NASDAQ:ARRY) (see BioCentury, July 18, 2011).

Autifony Therapeutics Ltd., Stevenage, U.K.

Product: AUT00206
Business: Neurology
Molecular target: Potassium channel Kv3
Description: Potassium channel Kv3 modulator
Indication: Treat schizophrenia
Endpoint: Safety and pharmacokinetics
Status: Phase Ib started
Milestone: NA

Autifony Therapeutics Ltd. (Stevenage, U.K.) began a double-blind, placebo-controlled, U.K. Phase Ib trial of twice-daily 800 mg oral AUT00206 for 4 weeks as adjunctive therapy in about 24 recently diagnosed schizophrenic patients. The trial is evaluating safety and pharmacokinetics. AUT00206 is a potassium channel Kv3 modulator.

Biohaven Pharmaceutical Holding Co. Ltd. (NYSE:BHVN), New Haven, Conn.

Product: Trigriluzole (BHV-4157)
Business: Neurology
Molecular target: NA
Description: Prodrug formulation of riluzole, a glutamate modulating agent
Indication: Treat hereditary spinocerebellar ataxia
Endpoint: Change in total score on the Scale for the Assessment and Rating of Ataxia (SARA); total time of the 8-meter walk, Sheehan Disability Scale, Patient and Clinician Global Impression of Change scales, safety and pharmacokinetics
Status: Completed Phase IIb/III enrollment
Milestone: Submit NDA (early 2018)

Biohaven Pharmaceutical Holding Co. Ltd. (NYSE:BHVN) completed enrollment of about 180 patients with hereditary spinocerebellar ataxia in a Phase IIb/III trial evaluating once-daily 140 mg oral trigriluzole (BHV-4157) for 8 weeks. The company plans to submit an NDA to FDA for the product in early 2018.

The double-blind, placebo-controlled, U.S. trial's primary endpoint is the change in total score on the Scale for the Assessment and Rating of Ataxia (SARA). Secondary endpoints include total time of the 8-meter walk, Sheehan Disability Scale, Patient and Clinician Global Impression of Change scales, safety and pharmacokinetics.

Trigriluzole is a prodrug formulation of riluzole, a glutamate modulating agent. It has Fast Track and Orphan Drug designations in the U.S. for spinocerebellar ataxia.

CASI Pharmaceuticals Inc. (NASDAQ:CASI), Rockville, Md.

Product: ENMD-2076
Business: Cancer
Molecular target: Aurora kinase A (AURKA; Aurora-A); VEGF receptor 2 (VEGFR-2; KDR/Flk-1)
Description: Inhibitor of AURKA and multiple tyrosine kinases
Indication: Treat locally advanced or metastatic triple-negative breast cancer (TNBC)
Endpoint: Clinical benefit rate (CBR)
Status: Completed Phase II enrollment
Milestone: NA

In April, CASI Pharmaceuticals Inc. (NASDAQ:CASI) completed enrollment of 41 patients in a Phase II trial of ENMD-2076 in previously treated patients with locally advanced or metastatic triple-negative breast cancer (TNBC). The open-label, U.S. trial is evaluating 250 mg oral ENMD-2076 daily in 28-day cycles. The primary endpoint is clinical benefit rate (CBR).

ENMD-2076 is an inhibitor of aurora kinase A (AURKA; Aurora-A) and multiple tyrosine kinases.

Celltrion Inc. (KOSDAQ:068270), Incheon, South Korea
Pfizer Inc. (NYSE:PFE), New York, N.Y.

Product: Remsima biosimilar infliximab (Inflectra, CT-P13)
Business: Autoimmune
Molecular target: Tumor necrosis factor (TNF) alpha
Description: Biosimilar of infliximab, a humanized mAb against TNF alpha
Indication: Treat autoimmune diseases including rheumatoid arthritis (RA), psoriasis and Crohn's disease (CD)
Endpoint: NA
Status: Clinical trial start
Milestone: NA

Celltrion Inc. (KOSDAQ:068270) said it has received clearance from China FDA to begin clinical trials of Remsima biosimilar infliximab. The company said it will become the first non-Chinese company to begin clinical studies of a biosimilar mAb in China.

Celltrion declined to say when the first study will begin, but noted that the company submitted an IND to CFDA for Remsima in January 2014. The mAb against tumor necrosis factor (TNF) alpha is a biosimilar of Remicade from Johnson & Johnson (NYSE:JNJ), which is approved to treat autoimmune diseases including rheumatoid arthritis (RA), psoriasis and Crohn's disease (CD).

Remsima is approved in the U.S. and EU. Celltrion shares its rights with Pfizer Inc. (NYSE:PFE) in Europe, while Pfizer holds rights in the U.S. and markets it as Inflectra.

GeNeuro S.A. (Euronext:GNRO), Geneva, Switzerland

Product: GNbAC1
Business: Endocrine/Metabolic
Molecular target: Endogenous retrovirus group W member 1 (HERVW; ERVW-1)
Description: Humanized mAb targeting the envelope protein of human HERVW
Indication: Treat Type I diabetes
Endpoint: Safety; biomarker analyses, insulin production, glycemia and antibody production
Status: Phase IIa started
Milestone: Complete Phase IIa enrollment (year end 2017); Phase IIa data (3Q18)

In April, GeNeuro S.A. (Euronext:GNRO) began a Phase IIa trial to evaluate GNbAC1 in about 60 recently diagnosed adults with Type I diabetes.

The placebo-controlled, Australian trial will evaluate safety as the primary endpoint. Secondary endpoints include biomarker analyses, insulin production, glycemia and antibody production. The company expects to complete enrollment by year end and to report data in 3Q18.

In early 4Q17, GeNeuro expects to report data from the Phase IIb CHANGE-MS trial of GNbAC1 in relapsing-remitting multiple sclerosis (RRMS). Servier (Neuilly-sur-Seine, France) has an option to license exclusive rights to develop and commercialize GNbAC1 worldwide, excluding the U.S. and Japan, after CHANGE-MS is completed (see BioCentury, Dec. 8, 2014 & Jan. 11, 2016).

The product is a humanized mAb targeting the envelope protein of human endogenous retrovirus group W member 1 (HERVW; ERVW-1).

Immunovaccine Inc. (TSX:IMV; OTCQX:IMMVF), Halifax, Nova Scotia

Product: DPX-E7
Business: Cancer
Molecular target: E7 transforming protein (Human papillomavirus-16; HpV16gp2)
Description: Cancer vaccine targeting the *Human papillomavirus*-16 formulated with DepoVax platform technology
Indication: Treat incurable HPV 16-related oropharyngeal, cervical and anal cancers
Endpoint: Safety and CD8+ T cells in peripheral blood and tumor tissue; overall response rate (ORR), overall survival (OS) and progression-free survival (PFS)
Status: Phase Ib/II started
Milestone: NA

In April, Immunovaccine Inc. (TSX:IMV; OTCQX:IMMVF) said that the Dana-Farber Cancer Institute (Boston, Mass.) began a Phase Ib/II trial of the biotech's DPX-E7 plus low-dose cyclophosphamide in 44 patients who are HLA-A*02-positive with incurable HPV 16-related oropharyngeal, cervical and anal cancers.

The open-label, U.S. trial will evaluate 2 priming doses of DPX-E7 given 3 weeks apart followed by a booster dose every 8 weeks until clinical progression. The primary endpoints are safety and changes

in CD8+ T cells in peripheral blood and tumor tissue. Secondary endpoints include overall response rate (ORR), overall survival (OS) and progression-free survival (PFS).

DPX-E7 is a cancer vaccine targeting the E7 transforming protein (*Human papillomavirus*-16; HpV16gp2) formulated with DepoVax platform technology.

Intensity Therapeutics Inc., Westport, Conn.

Product: INT230-6
Business: Cancer
Molecular target: Dendritic cells
Description: Intratumoral aqueous solution comprising cisplatin and vinblastine
Indication: Treat advanced solid tumors
Endpoint: Safety; tumor response and pharmacokinetics
Status: Phase I/II started
Milestone: NA

Intensity Therapeutics Inc. (Westport, Conn.) started the Phase I/II IT-01 trial to evaluate intratumoral INT230-6 with or without anti-PD-1 antibodies in about 60 patients with advanced solid tumors. Patients will receive injections every 14 or 28 days.

The open-label, U.S. and Canadian trial will evaluate safety as its primary endpoint and tumor response and pharmacokinetics as secondary endpoints.

INT230-6 is an intratumoral aqueous solution comprising cisplatin and vinblastine.

Molecular Partners AG (SIX:MOLN), Schlieren, Switzerland

Product: MP0250
Business: Cancer
Molecular target: VEGF; Hepatocyte growth factor/scatter factor (HGF/SF)
Description: Designed ankyrin repeat protein (DARPin) that inhibits VEGF and HGF/SF
Indication: Treat refractory and relapsed multiple myeloma (MM)
Endpoint: Overall response rate (ORR); safety, immunogenicity, progression-free survival (PFS), duration of response
Status: Phase II started
Milestone: Phase II data (2017); additional Phase II data (2018)

Molecular Partners AG (SIX:MOLN) began an open-label, European Phase II trial to evaluate MP0250 in combination with Velcade bortezomib plus dexamethasone in about 40 patients with refractory and relapsed multiple myeloma (MM). Patients will receive 6, 8 or 12 mg/kg IV MP0250 every 3 weeks until progression or toxicity. The company expects data from the trial this year and additional data in 2018.

The trial's primary endpoint is overall response rate (ORR). Secondary endpoints include safety, immunogenicity, progression-free survival (PFS) and duration of response.

The company also said it plans to submit an IND to FDA in 2H17 for a Phase Ib/II trial to evaluate MP0250 in combination with Tagrisso osimertinib to treat EGFR-mutated T790M-positive non-small cell lung cancer (NSCLC).

MP0250 is a designed ankyrin repeat protein (DARPin) that inhibits VEGF and hepatocyte growth factor/scatter factor (HGF/SF).

Pfizer Inc. (NYSE:PFE), New York, N.Y.

Product: Lorlatinib (PF-06463922)
Business: Cancer
Molecular target: Anaplastic lymphoma kinase (ALK); c-ros proto-oncogene 1 receptor tyrosine kinase (ROS1)
Description: Inhibitor of ALK and ROS1
Indication: First-line treatment of advanced ALK-positive non-small cell lung cancer (NSCLC)
Endpoint: Progression-free survival (PFS); overall survival (OS), objective response rate (ORR), intracranial ORR, intracranial time to progression, duration of response, time to tumor response, clinical benefit response (CBR) and safety
Status: Phase III started
Milestone: NA

Pfizer Inc. (NYSE:PFE) said it "recently" began the Phase III CROWN trial of lorlatinib (PF-06463922) as first-line treatment of locally advanced or metastatic, anaplastic lymphoma kinase (ALK)-positive non-small cell lung cancer (NSCLC).

The open-label, U.S. and Japanese trial is comparing once-daily 100 mg oral lorlatinib vs. twice-daily 250 mg oral Xalkori crizotinib in about 280 patients. The primary endpoint is progression-free survival (PFS). Secondary endpoints include overall survival (OS), objective response rate (ORR), intracranial ORR, intracranial time to progression, duration of response, time to tumor response, clinical benefit response (CBR) and safety.

Pfizer concurrently said that FDA granted lorlatinib breakthrough therapy designation to treat ALK-positive metastatic NSCLC in patients previously treated with ≥1 ALK inhibitor. Lorlatinib also has Orphan Drug designation from FDA to treat advanced ALK-positive NSCLC.

Lorlatinib is an inhibitor of ALK and c-ros proto-oncogene 1 receptor tyrosine kinase (ROS1).

Vical Inc. (NASDAQ:VICL), San Diego, Calif.

Product: Herpes simplex virus type 2 (HSV-2) vaccine, Vaxfectin-formulated plasmid DNA HSV-2 vaccine (VCL-HB01)
Business: Infectious
Molecular target: Not applicable
Description: Therapeutic plasmid DNA vaccine encoding the HSV-2 glycoprotein D and tegument protein VP11/12 (UL46) formulated with Vaxfectin lipid adjuvant
Indication: Vaccinate against HSV-2 infection
Endpoint: Lesion recurrences; safety, time to first recurrence and proportion of patients who are recurrence free

Status: Completed Phase II enrollment
Milestone: Phase II data (2Q18)

In April, Vical Inc. (NASDAQ:VICL) completed enrollment of 225 adults with symptomatic genital herpes simplex virus type 2 (HSV-2) infection in a Phase II trial evaluating 1 mL intramuscular VCL-HB01 (HSV-2 vaccine, Vaxfectin-formulated plasmid DNA HSV-2 vaccine) every 28 days for 4 doses. The double-blind, placebo-controlled, U.S. trial's primary endpoint is lesion recurrences. Secondary endpoints include safety, time to first recurrence and proportion of patients who are recurrence free.

Vical expects to report top-line data in 2Q18.

VCL-HB01 is a therapeutic plasmid DNA vaccine encoding the HSV-2 glycoprotein D and tegument protein VP11/12 (UL46) formulated with Vaxfectin lipid adjuvant.

Yisheng Biopharma Co. Ltd., Beijing, China

Product: YS-ON-001
Business: Cancer
Molecular target: NA
Description: Multi-component cancer vaccine with broad immunomodulating effects
Indication: Treat advanced solid tumors
Endpoint: Safety and dose-limiting toxicities (DLTs); tumor response rate and immunogenicity
Status: Phase I started
Milestone: NA

Yisheng Biopharma Co. Ltd. (Beijing, China) began an open-label, Singaporean Phase I trial to evaluate YS-ON-001 in about 41 patients with advanced solid tumors. The trial will evaluate thrice-weekly intramuscular YS-ON-001 in 21-day cycles for 12 cycles in the dose-escalation part. After the recommended Phase II dose is determined, the trial will enroll patients in the Phase Ib part with specific tumor types including breast and liver cancer.

The trial is evaluating safety and dose-limiting toxicities (DLTs) as the primary endpoints and tumor response rate and immunogenicity as secondary endpoints.

YS-ON-001 is a multi-component cancer vaccine with broad immunomodulating effects. The product has Orphan Drug designation in the U.S. to treat hepatocellular carcinoma (HCC).

FINANCIAL NEWS

COMPLETED OFFERINGS

Bicycle Therapeutics Ltd., Cambridge, U.K.

On June 1, Bicycle Therapeutics Ltd. (Cambridge, U.K.) raised £40 million ($51.3 million) in a series B round led by new investor Vertex Ventures HC. New investors Cambridge Innovation Capital and Longwood Fund also participated, as did existing investors Novartis Venture Fund, SR One, SV Life Sciences and Atlas Venture.

The company is developing short peptide sequences constrained by a chemical scaffold to create a structure with two peptide loops. The bicyclic peptides are designed to have the affinity and specificity of antibodies.

Check-Cap Ltd. (NASDAQ:CHEK), Isfiya, Israel

On May 30, Check-Cap Ltd. (NASDAQ:CHEK) raised $2.7 million through the sale of 1.3 million shares at $2 in a private placement with a sole institutional investor. The investor also received one-year warrants to purchase up to 1.3 million shares at $2.125.

The company is developing an ingestible capsule device utilizing X-ray and wireless technology for preparation-free colorectal cancer screening.

Deciphera Pharmaceuticals LLC, Waltham, Mass.

Cancer play Deciphera Pharmaceuticals LLC (Waltham, Mass.) raised $52 million on June 1 in a series C round led by Viking Global Investors, Redmile Group and Sphera Global Healthcare Fund. Existing investor New Leaf Venture Partners also participated.

Immunovaccine Inc. (TSX:IMV; OTCQX:IMMVF), Halifax, Nova Scotia

On May 31, immunotherapy company Immunovaccine Inc. (TSX:IMV; OTCQX:IMMVF) raised C$10 million ($7.4 million) through the sale of 7.7 million shares at C$1.30 in a bought deal underwritten by Echelon Wealth Partners, National Bank Financial and Mackie Research Capital.

InMed Pharmaceuticals Inc. (CNSX:IN; OTCQB:IMLFF), Vancouver, B.C.

Cannabinoid-based therapeutics developer InMed Pharmaceuticals Inc. (CNSX:IN; OTCQB:IMLFF) raised C$5.8 million ($4.3 million) through the sale of 12.8 million units at C$0.45 in a follow-on underwritten by Canaccord Genuity, Eight Capital and Roth Capital Partners.

Each unit comprises a share and a two-year warrant to purchase 0.5 shares, with each whole warrant exercisable at C$0.65. The figures include a 1.7 million unit overallotment sold on May 31.

Inthera Bioscience AG, Zurich, Switzerland

On May 31, oncology company Inthera Bioscience AG (Zurich, Switzerland) raised CHF10.5 million ($10.8 million) in a series A round led by Merck Ventures. Also participating were Aglaia BioMedical Ventures and Novo Seeds, as well as an undisclosed private investor.

InventisBio Inc., Shanghai, China

On May 30, cancer and gout company InventisBio Inc. (Shanghai, China) raised $19 million in a series B round led by OrbiMed Asia Partners. Existing investor Lilly Asia Ventures also participated.

Oncolytics Biotech Inc. (TSX:ONC; OTCQX:ONCYF), Calgary, Alberta

On May 24, cancer company Oncolytics Biotech Inc. (TSX:ONC; OTCQX:ONCYF) raised C$10 million ($7.4 million) through the sale of 14.3 million units at C$0.70 in a follow-on underwritten by Canaccord Genuity and Paradigm Capital. Each unit comprises a share and a five-year warrant to purchase a share at C$0.95.

Pharming Group N.V. (Euronext:PHARM), Leiden, the Netherlands

On May 16, specialty pharma Pharming Group N.V. (Euronext:PHARM) raised $100 million in a debt facility from OrbiMed Advisors. The four-year, senior secured loan bears interest at 12%.

Strekin AG, Basel, Switzerland

On May 31, Strekin AG (Basel, Switzerland) raised CHF10 million ($10.3 million) in its third seed round from undisclosed private investors. The company is developing STR001, a PPAR gamma agonist, to prevent hearing loss.

TearClear Corp., Copley, Ohio

On May 30, ophthalmic company TearClear Corp. (Copley, Ohio) said it completed a $4.5 million series A round led by Visionary Venture Fund and Bluestem Capital.

Xbrane Biopharma AB (SSE:XBRANE), Stockholm, Sweden

On May 24, biosimilar and long-acting injectables company Xbrane Biopharma AB (SSE:XBRANE) raised SEK20 million ($2.3 million) through the sale of 655,738 shares at SEK30.50 to Active Invest-Sweden, Thomas Eklund, Zirkona and Quantify.

FINANCIAL NEWS

OTHER FINANCIAL NEWS

Bain Capital LLC, Boston, Mass.

Bain Capital LLC (Boston, Mass.) closed its first life sciences fund at $720 million, the firm told BioCentury. The firm plans to invest the fund globally in biopharmaceuticals, medical devices, diagnostics and other technologies associated with life sciences.

The Bain Capital Life Sciences Fund includes $600 million raised from external investors, and $120 million from Bain Partners.

Bain Capital launched the fund last year. Its life sciences team includes Managing Director Adam Koppel, who rejoined the firm last year after serving as EVP of corporate development and chief strategy officer at Biogen Inc. (NASDAQ:BIIB); Managing Director Jeffrey Schwartz; and partner Jeffrey Green.

Global Health Innovative Technology (GHIT) Fund, Tokyo, Japan

The Global Health Innovative Technology (GHIT) Fund (Tokyo, Japan), a public-private partnership spearheaded by the Japanese government to fight neglected infectious diseases, said it has secured $200 million to fund its next five years of operation. The funding includes $130 million pledged by the Japanese government last year.

The Bill & Melinda Gates Foundation, the Wellcome Trust and undisclosed private companies have also backed GHIT. The fund received $100 million during its first phase, beginning in April 2013.

During its next phase of operation, GHIT plans to expand its role in R&D and collaborate further with global health entities, including the Global Alliance for Vaccine Immunization (GAVI); the Global Fund to Fight AIDS, Tuberculosis and Malaria; the United Nations Development Program (UNDP); the United Nations Children's Fund (UNICEF); and the World Health Organization (WHO).

Lyfe Capital, Shanghai, China

Lyfe Capital (Shanghai, China) said it closed its second fund with about $420 million, including $288.8 million and RMB900 million ($131.3 million). The firm expects to invest Lyfe Capital Fund II in biopharma, medical device, diagnostics, animal health, healthcare services and digital health companies.

Lyfe, which has typically invested in Greater China, has also opened an office in Palo Alto, Calif., and expects to make new investments in the U.S.

The firm said the new fund surpassed its targets of $230 million and RMB800 million ($116.7 million).

LPs in the fund included pension funds, endowments, institutional investors, family offices and funds-of-funds based in the U.S., Europe, Australia and Asia.

The firm now has $500 million and about RMB1.45 billion ($212 million) under management.

BIOCENTURY WEEK IN REVIEW

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