

# BIOWORLD™ TODAY

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## \$75M PRIVATE PLACEMENT

### Celsus Therapeutics, Volution attract A-listers to tie the knot as Akari

By Marie Powers, News Editor

On the cusp of their nuptials, [Celsus Therapeutics](#) plc and its betrothed, [Volution Immuno Pharmaceuticals](#) SA, attracted more guests to their party. The companies inked an agreement for a \$75 million private placement with a syndicate led by Deerfield that included Venrock, Vivo Capital, Foresite Capital, New Enterprise Associates, QVT Financial, RA Capital Management and additional undisclosed institutional investors.

Citigroup Global Markets Inc. and MTS Securities LLC acted as placement agents.

[See Celsus, page 3](#)

### Omeros readies rare clotting disease therapy for phase III

By Michael Fitzhugh, Staff Writer

[Omeros](#) Corp. (NASDAQ:OMER) shares rose to a record high Tuesday, climbing 72 percent overall to close at \$25.03 after the company said [OMS721](#), a therapy it's developing for the treatment of thrombotic microangiopathies (TMAs), continued to perform well in a small midstage trial.

[See Omeros, page 4](#)

## EUROPE

### ANTRUK screening for combos to break antibiotic resistance

By Nuala Moran, Staff Writer

LONDON – A charity formed by senior academics from across the UK to tackle the problem of antimicrobial resistance (AMR) is announcing its first research program Wednesday.

Antibiotic Research UK (ANTRUK) will begin its search for effective treatments

[See AMR, page 5](#)

## THE BIOWORLD BIOME

### HELPING THE BRAIN HELP ITSELF

### Unleashing immune system moves from cancer to neurodegeneration

By Anette Breindl, Senior Science Editor

Breaking the body's immune tolerance to tumors has been the biggest advance for cancer treatment in recent years. Now, scientists have reported that the same strategy might be successful against neurodegenerative diseases.

The notion that escape from immune surveillance could underlie both types of diseases is "counterintuitive on the surface, because one is a disease of proliferation and the other of neuronal death," Michal Schwartz told *BioWorld Today*.

But Schwartz and her colleagues contended that those differences mask an involvement of the immune system that is fundamentally similar in both disorders.

The team's approach, which they

[See Immune system, page 6](#)

## EUROPE

### Investors warm toward Norwegian biotech with cancer innovation park

By Cormac Sheridan, Staff Writer

DUBLIN – Norway's Prime Minister Erna Solberg helped focus a spotlight on Norway's emerging oncology cluster this week by performing the official opening of the Oslo Cancer Cluster Innovation Park, the first stage in an ambitious program to bring together in a single setting a critical mass in cancer research, education and cancer-related commercial biotechnology.

The first phase of the Oslo Cancer Cluster Innovation Park comprises a NOK1 billion (US\$121 million) investment in a 36,000-square-meter facility, which houses a biotech incubator for oncology start-ups, Norway's Cancer Registry, the Institute for Medical Informatics and Pathology at Oslo University Hospital and several other start-up and established biotech and biopharma firms.

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## REGULATORY FRONT

Mylan NV, of Hertfordshire, UK, received an **FDA** warning letter citing conditions at three of its drug manufacturing facilities in Bangalore, India, including two that were part of its 2013 acquisition of Agila Specialties. Inspections of the plants revealed contamination risks in aseptic areas stemming from torn gloves and gowns and workers' behavior, such as bumping into each other in fill areas or leaving barriers open while performing other duties. A number of other problems were cited, including the failure to determine the root cause of contaminated product or defects. Several of the violations were recurrent and long-standing, the FDA said. Thus, the problems noted by the inspectors "raise questions about the ability of [Mylan's] current corporate quality system to achieve overall compliance" with good manufacturing practices, according to the letter. Although acknowledging that Mylan acquired the Agila facilities recently, the FDA said a Sept. 9, 2013, warning letter should have put the drugmaker on notice about the problems at the plants. "Even without this warning letter, your corporate quality system should have detected and corrected the forgoing violations without FDA intervention," the agency concluded.

**Sen. Joe Manchin** (D-W.Va.) sent a letter Monday to acting **FDA** Commissioner Stephen Ostroff condemning the agency's decision last week to approve **Purdue Pharma LP's** Oxycontin (oxycodone hydrochloride) for use for children as young as 11. In the letter, the senator expressed his disgust with what he called the FDA's "foolish and deadly action." Noting that the agency is required to convene an advisory committee on matters that are of significant public interest, highly controversial or in need of a specific type of expertise, Manchin questioned why no advisory committee was consulted before the FDA approved the pediatric use. He informed Ostroff that he would be calling for a Senate investigation into the approval, as well as other FDA decisions to approve opioid drugs. "I cannot sit by while an agency of the federal government facilitates the poisoning of our children and their parents and grandparents," Manchin said.

## STOCK MOVERS 8/18/2015

Company	Stock in \$	Change in %
Nasdaq Biotechnology	-\$38.38	-0.99%
Aquinox Pharmaceuticals	-\$2.58	-12.46%
Esperion Therapeutics Inc.	-\$11.34	-15.02%
Omeros Corp.	+\$10.48	+72.03%
Vtv Therapeutics Inc.	+\$1.05	+11.71%

Biotechs showing significant stock changes Tuesday

## FINANCINGS

**Aclaris Therapeutics Inc.**, of Malvern, Pa., filed for a proposed \$86.25 million IPO as an emerging growth company. The number of shares and share price have not yet been disclosed. Proceeds will be used to complete three planned phase III trials and seek regulatory approval of A-101, a high-concentration hydrogen peroxide topical solution in development for seborrheic keratosis. Funds also will support continued R&D for A-101 in common warts and for other programs, including A-102, and for working capital and other general corporate purposes. Aclaris had about \$9.8 million in cash and equivalents as of June 30. Its principal stockholders include Vivo Ventures, Beacon Bioventures and Sofinnova Venture Partners, all of which participated in the company's series A and B rounds, each raising \$21 million. Jefferies, Citigroup and William Blair & Co. are acting as underwriters for the IPO. Upon pricing, Aclaris plans to list on Nasdaq under the ticker ACRS. (See *BioWorld Today*, Oct. 3, 2014.)

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## Celsus

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New York-based Celsus disclosed last month that it would acquire Volution and combine the companies through a reverse merger into Akari Therapeutics plc, which will have offices in New York and London and trade on Nasdaq under the ticker AKTX. Closing of the oversubscribed private placement and the acquisition of Volution is subject to approval by shareholders of Celsus at a general meeting called for Sept. 16, plus satisfaction of customary conditions.

On a pro forma basis before the private placement becomes effective, Celsus said its current security holders will own approximately 8.32 percent of the combined company while Volution security holders will own the remainder, on a fully diluted basis.

Once the financing closes, Akari expects to receive net proceeds of approximately \$70 million from the sale of ordinary shares, represented by American depository shares (ADS). Based on Monday's closing price of 61 cents per ADS for Celsus (NASDAQ:CLTX), and assuming approximately 949.4 million fully diluted shares following the close of Volution's acquisition, Celsus said the purchase price of the financing would be equal to \$1.58 per currently trading ADS (or 15.8 cents per ordinary share), representing a 159 percent premium to the closing price of its shares on Monday.

Investors apparently liked that math, pushing Celsus to \$1.02 on Tuesday after financing plans were disclosed, for a gain of 41 cents, or 67 percent. Volume was nearly 3.7 million shares compared to a 90-day average of fewer than 60,000.

Investors must digest another change, however. Each ADS currently represents 10 ordinary shares, but that will change the day after Celsus shareholders meet. Pending approval of the Volution transaction, the ADS ratio will be adjusted so that each ADS – including those purchased by investors in the financing – will represent 100 ordinary shares.

Provided the wedding goes off without a hitch, the combined entity will be valued at \$150 million on a fully diluted basis, prior to completion of the private placement, which is expected to close by Sept. 18. Not a bad comeback for Celsus, which reported in February that its lead candidate, MRX-6 cream 2 percent, failed to demonstrate a difference in mean change from baseline to day 28 in children with mild to moderate atopic dermatitis in a phase II trial. That outcome sent shares of Celsus plummeting \$5.02, or 81.4 percent.

Failure of the phase II trial eliminated a short-term market entry for Celsus, according to CEO Gur Roshwalb, who joined the company in 2013 from Venrock, where he was a vice president on the health care team. Celsus also was low on cash, with just \$2.7 million in the bank as of June 30, according to a 10-K filing. Despite the clinical failure, the company was eager to keep its management team together, so Celsus started looking for a candidate to in-license or acquire.

"We knew we would have to go back out there and raise capital," Roshwalb told *BioWorld Today*, maintaining that the reverse merger with Volution had nothing to do with preserving or accessing cash.

#### **POTENTIALLY BEST IN CLASS SECOND-GENERATION C5 INHIBITOR\***

In the process of searching for an appropriate candidate to pad its pipeline, Celsus encountered privately held Volution, which had advanced its lead compound, Coversin (REV-576), a complement C5a receptor antagonist, into a phase I trial to treat thrombosis, where it showed 100 percent complement inhibition and a good safety profile, according to Celsus documents. The asset was gained from Evolutec Group plc, which halted clinical operations in 2009 following the phase III failure of its hay fever candidate, REV-131.

In addition to thrombosis, Coversin has potential applications in complement-related disorders in other therapeutic areas, according to Roshwalb, who also was impressed with Volution's scientific team. With RPC Pharma Ltd. as its only investor, Volution was considering whether to do a mezzanine round or IPO to raise additional cash to advance Coversin. Instead, a deal was struck with Celsus.

"It's really a marriage, if you will, not a merger," Roshwalb said. The combined company initially will focus on developing Coversin to treat rare and orphan autoimmune and inflammatory diseases caused by dysregulation of complement C5, including paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS).

The recombinant small protein is derived from a protein discovered in the saliva of the *Ornithodoros moubata* tick, where it helps to modulate the host immune system, allowing the parasite to feed without alerting the host to its presence or provoking an immune response. The new company's name, Akari, is a derivative of Acari, the taxon of arachnids that contains mites and ticks, although the word also translates from Japanese as "light," giving a larger meaning to the name, Roshwalb said.

The Volution team had synthesized the protein, which is now produced in *E. coli* by a contract manufacturer, with optimization and scale-up under way, according to Celsus documents.

Coversin, at just 17 kilodaltons, or kDa, is smaller than an antibody and can be self-administered by subcutaneous injection, "which we believe should provide considerable patient benefit over the current standard of care in several diseases," Roshwalb said.

Not surprisingly, Akari is targeting Coversin to grab a piece of the market now held by Soliris (eculizumab, Alexion Pharmaceuticals Inc.). Coversin has two U.S. and 18 foreign issued patents covering major global markets, with expected orphan drug and biologics license application exclusivity in the U.S. and EU for each approved indication.

[See Celsus, page 8](#)

## Omeros

### [Continued from page 1](#)

The positive results set the stage for a move to a fixed-dose stage of the trial, planning for a phase III study and a potential challenge to Alexion Pharmaceuticals Inc.'s top product, Soliris (eculizumab). Alexion shares (NASDAQ:ALXN) fell 2 percent, or \$3.80, to close at \$187.42 on Tuesday.

Seattle-based Omeros first shared data from the trial in February 2015, saying that investigators saw "meaningful" improvements in three markers of disease activity after three patients with a form of TMA called atypical hemolytic uremic syndrome (aHUS) were treated with the lowest dose of the candidate.

Additional data released Tuesday showed that the two aHUS patients in a mid-dose cohort of the study and the sole member of a high-dose cohort also saw improvements, with platelet counts – one of the disease activity measures – returning to normal in all three participants after the four-week treatment period, indicated by a statistically significant mean increase in platelet counts from baseline of about 68,000 platelets/mL ( $p = 0.0055$ ).

In the mid-dose cohort, the two patients with plasma therapy-resistant aHUS demonstrated an 86 percent decrease in mean fragmented red blood cell count, with such cells disappearing in one patient; a 71 percent increase in mean haptoglobin, with both patients reaching the normal range during treatment and one slipping slightly below normal a week after the last dose; and a 5 percent decrease in the mean levels of another marker of disease activity, lactate dehydrogenase, with levels in both patients remaining slightly elevated above normal range. Also of note, the patient in the high-dose cohort who, prior to OMS721 treatment, required repeated dialysis, was able to discontinue it during and following treatment with OMS721.

Analysts covering the company expressed cautious optimism about the trial's progress. Cowen and Co.'s Yatin Suneja noted that while his team was pleased with the data, especially if they could be replicated over longer treatment durations, "the efficacy bar set by Soliris in this indication is very high and designing/conducting a phase III trial in the presence of such an effective treatment could be challenging."

Roth Capital Partners analyst Elemer Piros pointed out that "to facilitate direct comparison with Soliris, we would have to examine longer-term (26 weeks) benefit. Having said that, the initial data appear to be robust, which might be indicative of sustainable benefit."

Soliris, the only FDA-approved therapy for aHUS, generated about \$2.2 billion in revenue for Alexion, making it a meaningful target. Its safety and effectiveness were established in two single-arm trials in 37 adult and adolescent patients with aHUS and one retrospective study in 19 pediatric and 11 adult patients with aHUS, according to the FDA.

Patients treated with Soliris during the studies supporting its approval experienced a favorable improvement in kidney function, including elimination of the requirement for dialysis in several patients with aHUS that did not respond to plasma therapy. They also exhibited improvement in platelet counts and other blood parameters that correlate with aHUS disease activity.

TMAs, including aHUS, are a family of rare, debilitating and life-threatening disorders characterized by excessive aggregations of platelets in the microcirculation of the body's organs, most commonly the kidney and brain. OMS721, Omeros' lead human monoclonal antibody in its mannan-binding lectin-associated serine protease-2, or MASP-2, program, is designed to treat the condition, which is thought to occur at a rate of about two cases per million people. On the whole, aHUS accounts for 5 percent to 10 percent of all cases of hemolytic uremic syndrome and disproportionately affects children.

Though the exact number of those affected by the diseases is unknown, more than 1,000 cases of aHUS have been reported to registries in the U.S. and Europe, according to Thomson Reuters Incidence and Prevalence Database.

OMS721 has received FDA orphan status for the inhibition of TMAs mediated by the complement system, a key part of the immune response, and has received fast track status for the treatment of patients with aHUS.

Based on results seen in patients participating in the phase II trial, investigator-requested extended access to OMS721 is now available for compassionate use to European patients with TMAs.

"Based on clinical data, we expect that we can deliver OMS721 either subcutaneously or intravenously at a frequency and dose that are both convenient and comfortable for patients while effectively eliminating lectin-pathway activity," said Omeros founder, CEO and Chairman Greg Demopoulos.

Omeros' first drug product, the FDA and European Commission-approved Omidria (phenylephrine and ketorolac), is used during cataract surgeries or intraocular lens replacements to maintain pupil size by preventing pupil constriction during operations and to reduce postoperative ocular pain. After initiating sales of the drug in February, Omeros started a broader U.S. launch effort in April.

In October, it decided to suspend a study of its phosphodiesterase 10 inhibitor, OMS824, in Huntington's disease over FDA concerns about higher-than-expected blood levels of the drug in a concurrent rat study. Since then, it has continued to develop a variety of additional preclinical programs as well as a phase II peroxisome proliferator-activated receptor gamma agonist candidate for treatment of addiction to opioids and to nicotine. (See *BioWorld Today*, Oct. 22, 2014.)

As of June 30, Omeros reported having \$51.4 million in cash, cash equivalents and short-term investments. //

## AMR

### [Continued from page 1](#)

against gram-negative superbugs by looking for existing drugs that, when used in combination with antibiotics that are losing their effectiveness, will override resistance mechanisms in some way.

The charity plans to screen some 2,000 marketed small-molecule drugs and 700-odd nutraceuticals in combination with four major classes of antibiotics, against resistant bacteria.

“What we will be looking for is any evidence of activity against antibiotic-resistant bacteria,” said Colin Garner, professor of pharmacology and CEO of ANTRUK. “These will be phenotypic screens looking for any evidence of inhibition of growth. We are not bothered about the mechanism, as long as we see a biological effect,” he told *BioWorld Today*.

Although there is some evidence in the literature that such combinations are effective, Garner said that will be the first time a systematic screen has been performed in a single laboratory.

At its simplest, any antibiotic resistance breakers discovered as a result of the screen could be taken in tablet form alongside an antibiotic, significantly lowering the requirement for safety testing. Alternatively, it may be necessary to combine two drugs in a new formulation.

The aim will be to get preclinical proof of concept, or possibly to do early clinical trials, before out-licensing. “As a charity we are not too bothered about making a profit down the line. There may be some intellectual property, but that’s not why we are doing it,” said Garner.

ANTRUK has chosen to attempt to extend the life of existing antibiotics as the short route to making a dent in AMR, providing a stop-gap until new antibiotics are developed. It believes it could get the first antibiotic/drug combination into use by 2020.

However, the much broader vision is to become a leading medical charity, kickstarting and driving forward novel antibiotic drug development, with Garner citing counterparts such as the Cystic Fibrosis Foundation as role models.

In pursuit of that, ANTRUK currently is drawing up a five-year research strategy, which will be finalized in the next couple of months. It is already decided that after the screening program to look for resistance breakers the next move will be to explore the use of combinations of antibiotics that could be effective against resistant infections. Garner said that unlike cancer, where there is considerable research into the use of combination therapies, there has been little effort to investigate the effects of combining antibiotics.

The strategy also will include de novo discovery programs where the founders of ANTRUK, who all have extensive hands-on experience in the field, have several approaches in mind.

“The people who are on our steering committee have been

involved in many unsuccessful projects. They know the pitfalls and what to look out for. No single pharma or biotech has that breadth of experience in-house,” said Garner.

### AN EXPERIENCED TEAM

While ANTRUK looks to medical charities that have directly funded development of new treatments as role models, it differs from most of those in being formed not by patients or patients’ relatives, but by researchers who are concerned about the problem of AMR.

The founders were first brought together in 2012 by Anthony Coates, a world-leading expert in AMR and professor of medical microbiology at St George’s University Hospital, London, who for a number of years was a lone voice in pointing to the growing threat of resistance.

Coates is the founder and chief scientific officer of Helperby Therapeutics Ltd., which is developing a platform technology for killing the dormant, nondividing bacteria within an infection, rather than the rapidly multiplying bacteria that are targeted with conventional antibiotics. The company likens that to killing the roots, rather than the branches of an infection.

Others involved in ANTRUK include David Brown, a former pharma executive who was co-inventor of Pfizer Inc.’s Viagra (sildenafil), Chris Brown, a professor at the School of Life Sciences at Warwick University, and Laura Piddock, professor of microbiology at Birmingham University.

While Coates’ idea was to discuss possible research programs and look for traditional government funding, the decision in 2014 to form a charity opened the way to tapping other funding sources.

“This is a neglected area and there aren’t lots of funding streams. The Medical Research Council is the UK’s leading funder, with £35 million [US\$54.9 million] to be spread over five years, which is nothing,” said Garner. “The field requires considerable amounts of new money.”

Initially, ANTRUK is aiming to raise £120,000 to carry out the antibiotic resistance breaker screen. However, the larger ambition is to attract funding of up to £30 million in the next five to seven years, through a combination of traditional fundraising, corporate sponsorship and donations from trusts and foundations.

“In this area there hasn’t been enough focus on the charity sector as being a possible solution. Everyone is talking about pharma, but charities can cover the ground from patients to research labs and that’s something other organizations can’t do in the same way,” Garner said.

There is still some way to go in giving ANTRUK a profile and appeal to match that of charities raising money for cancer, arthritis or Alzheimer’s disease. That will depend on building greater awareness of AMR and the threat it poses, Garner said. “The issue is slowly coming into focus, but more effort is needed to highlight seriousness of the problem.” //

## Immune system

[Continued from page 1](#)

published in the Aug. 18, 2015, online issue of *Nature Communications*, is basically the opposite of previous attempts to get at Alzheimer's disease by targeting inflammation.

The team was able to both reduce amyloid-beta plaques and reverse cognitive deficits in mice with Alzheimer's disease by transiently depleting regulatory T cells, essentially "unleashing the power of the immune system to repair the brain," in the words of first author Kutli Baruch.

"In all neurodegenerative diseases, there is a local inflammation associated with the pathology," senior author Schwartz explained. And so "in the past, it was very easy to believe that you can resolve [them] with systemic anti-inflammatory or immune-suppressive drugs."

Logical or not, though, that approach has been a bust in practice. "Anti-inflammatories have failed in the clinic," she said. And her own team's research is leading her to the conclusion that trying to treat neurodegenerative disorders with immunosuppressive drugs may be akin to "treating frostbite with ice."

Schwartz's lab, which is located at the Israeli Weizmann Institute of Science, was a pioneer in demonstrating that the immune system plays a protective role in healthy brain functioning and brain aging, but also that its involvement "needs to be well orchestrated" in order to be helpful.

In 2014, Schwartz, Baruch and their colleagues showed that the aging brain became less able to recruit immune cells, and that in animals, this was associated with cognitive deficits.

In the work now published in *Nature Communications*, first authors Baruch and Neta Rosenzweig looked specifically at the role of regulatory T cells – a subset of T cells that inhibit immune responses, protecting the body from autoimmune diseases and immune responses that go on past the time they are necessary to fight infections.

That protective effect is "great in immune homeostasis," Schwartz said. But her team's work suggests that "perhaps it is not so great in neurodegenerative disease."

Specifically, the team found that in a transgenic mouse model of Alzheimer's disease, those regulatory T cells hindered the ability of the body's immune system to fight brain pathology.

When the researchers depleted regulatory T cells from the circulation in the mice, the animals mounted an immune response that helped in removing the plaques. In those animals, T-cell depletion reversed cognitive deficits.

Notably, that reversal could occur even in animals with relatively late-stage disease. Many people believe that one reason Alzheimer's disease clinical trials have racked up failure after failure is that they intervene too late in the course of the disease, when irreversible damage has already been done.

Treating earlier-stage patients, however, which is often the suggested solution, has its own set of issues, from identifying patients early enough to the costs of a decades-long trial that

could be necessary to prove that such a trial would be effective. (See *BioWorld Today*, Jan. 2, 2013.)

The fact that there have been more than a dozen failures of experimental Alzheimer's drugs in late-stage trials also illustrates the fact that removing plaques is not necessarily enough to make a clinical difference in the disease.

Schwartz noted, though, that her team's approach is broader than typical plaque-busting.

"What we are doing is to recruit the immune system to fight brain degeneration," she said. "This is more comprehensive than treating a specific disease-related mechanism."

The work suggests that "treatment with available drugs that can reduce immune suppression, or more specifically break immune tolerance," could be useful to treat Alzheimer's disease and perhaps, neurodegeneration more broadly.

One possibility for unleashing the immune system is to use the same drugs that have been developed for breaking tolerance to tumors.

Once the leash is off, the details differ. In the case of cancer immunotherapy, the benefits stem from the activation of killer T cells. In the work now published by the Weizmann team, depleting regulatory T cells affected T cells that facilitate recruitment of macrophages, which played a role in plaque removal either directly or via their effects on microglia – the brain resident immune cells. The team is currently trying to understand the exact cellular underpinnings of the effects they have described.

But though the downstream effects may be different, Schwartz said she thinks that some of the same medications that break immune tolerance in cancer could be useful in Alzheimer's disease. She said her team is currently testing that hypothesis in animal studies, and that so far, the data are "promising." //

### FINANCINGS

**Benitec Biopharma Ltd.**, of Sydney, priced its U.S. IPO of 1.5 million American depositary shares (ADSs), representing 30 million fully paid ordinary shares of Benitec and warrants to purchase another 500,000 ADSs, or 10 million fully paid ordinary shares, at \$9.21 apiece per ADS and 1 cent per warrant, for expected gross proceeds of \$13.8 million. Each ADS represents 20 ordinary shares of Benitec. The warrants, which have an exercise price of \$5.50 apiece, may be exercised immediately and expire on Aug. 21, 2020. Benitec granted the underwriters a 45-day option to purchase up to 225,000 additional ADSs and/or 75,000 warrants to purchase ADSs to cover overallotments. The company said proceeds from the offering will advance its therapeutic programs, which are based on gene silencing programs using its DNA-directed RNA interference, or ddRNAi, technology. The ADSs and warrants began trading Tuesday on Nasdaq as BNTC and BNTCW, respectively. Shares slumped to \$7.97 for a loss of \$1.24, or 13.5 percent, on the day. The IPO is expected to close by Aug. 21. Maxim Group LLC is sole book-running manager.

## Norway

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Unusually, a high school is also located at the site, and its students will have the opportunity to interact with scientists working in the park. A second stage, comprising 8,000 square meters is in development, Jónas Einarsson, the founder and driving force behind the initiative, told *BioWorld Today*. Eventually, the plan is also to rehouse the adjacent Norwegian Radium Hospital (originally built in 1932) in a new facility on the site as well.

Despite Norway's considerable wealth – the country's oil-fueled sovereign wealth fund was valued at \$800 billion at the end of 2014 – it has always adopted a cautious approach to investing in its science and technology infrastructure. "Everything is supposed to be sector-neutral," Einarsson said.



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Unlike certain wealthy countries in the Middle East or in Asia, Norway is not attempting to buy success by importing elite research institutions or Nobel-prize-winning rock stars – and it has produced two home-grown laureates, neuroscientists May-Britt Moser and Edvard Moser, who shared the 2014 prize in medicine or physiology with John O'Keefe.

"Basic research is not bad at all," Einarsson said. Persuading government to go further than that has been the problem. "What we've been struggling with is to get them to understand that to get anything beyond publishing papers we have to invest in the next stage." Even so, there is evidence to suggest that the biotech climate in Europe's far north is starting to warm up.

Einarsson is CEO of the Norwegian Radium Hospital Research Foundation, which operates an evergreen seed fund, valued at NOK300 million to NOK400 million, in terms of cash on hand and investments made. It has long been an important source of seed funding for early stage biotech firms, but the success of Algeta ASA, which Bayer AG acquired for \$2.9 billion in 2013, and of Nordic Nanovector ASA, which upsized its IPO to NOK500 million in March, has raised the visibility of the sector substantially. (See *BioWorld Today*, Dec. 20, 2013, and March 23, 2015.)

"It's been almost a paradigm shift," Einarsson said. The sector has attracted some NOK1.2 billion (US\$145 million) during the last 15 to 18 months. "We've attracted Norwegian investors who have not looked at this sector before," Einarsson said.

Co-investors include Gjelsten Holding AS, an industrial holding company; Inven2, the research commercialization arm of the University of Oslo and Oslo University Hospital; Birk Venture, a young Norwegian venture capital firm; and Healthcap, the Swedish-based VC firm that was the largest shareholder in Algeta.

Immuno-oncology is the main focus of the first wave of tenants in the new incubator. They include Ultimovacs AS, which is conducting phase I/IIa trials of a therapeutic vaccine directed against human telomerase enzyme, UV1, in patients with prostate cancer, non-small-cell lung cancer and melanoma. The vaccine is based on synthetic peptides derived from tumor epitopes identified in long-term cancer survivors who had been treated with various telomerase vaccines or with telomerase-transfected dendritic cells.

Lifandis AS, formerly Hunt Biosciences AS, is a publicly owned company that offers industry partners an entry point to Norway's longitudinal health study Hunt (Nord-Trøndelag Health Study). That has amassed biological samples from individual and family health histories on about 120,000 people over several decades, which are linked to national and regional patient registries.

Another tenant, Normetrix AS, is developing novel cancer biomarkers, based on academic discoveries. There's also Lytix Biopharma AS, which is developing an oncolytic peptide, LTX-315, currently in a phase I/IIa trial in patients with solid tumors. //

## Celsus

### [Continued from page 3](#)

Once the engagement between the two companies was set, they targeted a \$45 million raise to take the combined entity through the second quarter of 2017, allowing Akari to read out phase II data for Coversin in PNH and, potentially, also in aHUS. The additional funds will provide the company with extra bandwidth to look at other indications and formulations, such as a subcutaneous version with a longer half-life that would not require daily dosing. Akari also will continue to advance preclinical assets gained from the Celsus pipeline.

The merged entity will include eight to 10 people, mostly from the Celsus management and Volution scientific teams, although the number is expected to increase modestly over the

next 12 months, Roshwalb said.

As to a long-term business strategy, “all avenues are available,” he maintained, adding that decisions will be driven by the data.

“We do believe we have potentially the best in class second-generation C5 inhibitor,” Roshwalb said. “The data will give us a lot of guidance as to whether we’ll take it forward ourselves or partner it out.”

Celsus began life a decade ago as Morria Biopharmaceuticals plc, which was investigating a preclinical candidate, MRX-3, to treat asthma and initiated clinical studies of the multi-functional anti-inflammatory drug, MRX-6, in contact dermatitis and the phospholipase A2 inhibitor, MRX-4, in allergic rhinitis. Morria began trading Over-the-Counter in 2013 and, when Roshwalb joined the company, changed its name to Celsus. //

## Wondering what you missed in *BioWorld Insight*?

### MARKET IMPACT OF FIRST BIOSIMILAR MAB CONTAINED – SO FAR

Celltrion Inc. just racked up its 62nd approval for Remsima, adding Australia to the growing list of countries where the infliximab biosimilar can be marketed. But the South Korean company is finding that it’s going to take more than approvals for biosimilars to crack the blockbuster biologics market. Remsima, the first biosimilar monoclonal antibody (MAb) to be approved in highly regulated countries, is making a small dent in the market share of its reference product, Janssen Biotech Inc.’s Remicade. But outside of Norway, the impact has been relatively negligible. That could change quickly. All it would take is a pricing war, favorable results from a switching trial and, perhaps, U.S. approval.

### INCREASE IN FDA APPROVALS TIED TO FAIL FAST AND CHEAPLY

With 41 FDA approvals last year and 107 approvals over the last three calendar years – compared to 77 in the prior three years and 64 in the 2006-2008 time frame – there’s no doubt that companies have been more effective in moving drugs through the approval goal line. According to data compiled in the new CMR International 2015 Pharmaceutical R&D Factbook, the higher productivity hasn’t come from a higher throughput but from a higher success rate. With smaller pipeline volumes in the early phases and a declining number of projects terminated in phase III development, it appears companies are following the mantra of failing fast and cheaply.

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## FINANCINGS

**Kuros Biosurgery AG**, of Zurich, Switzerland, closed a financing round of CHF15 million (US\$15.35 million) and said additional investment is expected in a second closing this year. The round was led by new investors Lifecare Partners, of Switzerland, and LSP Life Sciences Partners, of the Netherlands, with participation by unnamed family offices from Germany and Switzerland. Kuros said Omega Funds, of Boston, also joined as a shareholder during the round, which also was supported by existing investors VI Partners (via Venture Incubator) and the Swiss Helvetia Fund Inc. Kuros said proceeds will bring lead sealant candidate, KUR-023, to market in Europe and the U.S. and will enable the company to advance lead orthobiologics product, KUR-111, into phase III studies. In conjunction with the financing, Gerhard Ries, managing partner of Lifecare, Jörg Neermann, partner at LSP, and Dominik Ellenrieder joined the Kuros board.

**VBI Vaccines Inc.**, of Cambridge, Mass., closed a private placement led by new investor RTW Investments, together with existing investors Arch Ventures Partners and Perceptive Advisors. VBI sold 3 million common shares at an average price of \$2.0952 apiece for gross proceeds of approximately \$6.29 million.

**Wave Life Sciences**, of Boston, completed an oversubscribed \$66 million series B preferred stock financing led by new investor Foresite Capital. Additional new investors included Fidelity Management and Research Co., New Leaf Venture Partners, Redmile Group, Jennison Associates (on behalf of undisclosed clients), Cormorant Asset Management and private funds advised by Clough Capital Partners LP. RA Capital Management, a lead investor in Wave’s \$18 million series A in February, also participated in the B round, joined by series A investor Kagoshima Shinsangyo Sosei Investment. Leerink Partners LLC acted as the exclusive placement agent. Wave is developing stereopure nucleic acid therapeutics, including candidates for Huntington’s disease and Duchenne muscular dystrophy, across oligonucleotide modalities that include RNAi, antisense and exon-skipping, among others. The company said it plans to file its first investigational new drug applications by the end of next year. (See *BioWorld Today*, Feb. 3, 2015.)

**OTHER NEWS TO NOTE**

**Aeolus Pharmaceuticals Inc.**, of Mission Viejo, Calif., highlighted the July publication of a study in mice demonstrating that treatment with AEOL-10150, the company's broad-spectrum catalytic antioxidant, significantly improved memory and decreased nerve death after exposure to pilocarpine, an organophosphate used to simulate epilepsy and nerve agent exposure. The paper, "Reactive oxygen species mediate cognitive deficits in experimental temporal lobe epilepsy," was published in the July 2015 issue of *Neurobiology of Disease*.

**Biogen Inc.**, of Cambridge, Mass., the ALS Association and Columbia University Medical Center will collaborate to advance the understanding of similarities and differences in the amyotrophic lateral sclerosis (ALS) disease process and the influence of genes on clinical features of the disease. The project, Genomic Translation for ALS Clinical Care, will encompass next-generation genetic sequencing and detailed clinical phenotyping in 1,500 people with ALS. The goal is to provide a basis to develop precision medicines or individually tailored therapies for ALS. The collaboration also will set the stage for a nationwide effort to ensure the genomic characterization of patients with ALS. Current participating sites include the Cedars-Sinai Board of Governors Regenerative Medicine Institute, Columbia University Medical Center, Duke Medical Center, Houston Methodist, the Scotland ALS clinic network, University of Minnesota and Hennepin County Medical Center, University of Utah, University of Washington and Washington University in St. Louis. The project is being funded through Biogen's \$30 million strategic alliance with Columbia and \$3.5 million from the ALS Association through funds raised in the Ice Bucket Challenge. (See *BioWorld Insight*, Oct. 20, 2014.)

**Biospecifics Technologies Corp.**, of Lynbrook, N.Y., said its board authorized an increase in the repurchase amount in its previously approved stock repurchase program for up to \$2.5 million of its outstanding common shares. The company plans to repurchase stock through a broker in the open market and to hold any reacquired stock in treasury. Biospecifics, which is partnered on Xiaflex (collagenase clostridium histolyticum) with **Endo International plc**, of Dublin, through its 2014 acquisition of Auxilium Pharmaceuticals Inc. and is advancing candidates internally, reported approximately 6.85 million common shares as of June 30. On Tuesday, shares (NASDAQ:BSTC) gained \$1.43 to close at \$53.05.

**Biothera Inc.**, of Eagan, Minn., said Cancer Research UK (CRUK), will fund preclinical research evaluating the efficacy of the company's investigational cancer immunotherapy, Imprime PGG, in combination with monoclonal antibody therapies as a precursor to a phase I/II study in Rituxan (rituximab, Biogen Inc. and Roche AG)-refractory follicular lymphoma. The research will be conducted at the Manchester

Academic Health Sciences Center, which forms part of Cancer Research UK's Combinations Alliance, a joint initiative between the Cancer Research UK Center for Drug Development, the UK's Experimental Cancer Medicine Center Network and commercial partners to boost research combining different drugs with conventional chemotherapy, radiotherapy and new targeted treatments. The research will utilize preclinical models to determine whether Imprime PGG, through innate immune modulation, enhances the efficacy of two anti-CD20 MAbs, Arzerra (ofatumumab, Genmab A/S) and Gazyva (obinutuzumab, Roche AG), that the FDA has approved as treatments for chronic lymphocytic leukemia and which have shown potential to treat follicular non-Hodgkin's lymphoma. The two MAbs have slightly different mechanisms of action and the study will be designed to determine which MAb will be most effective to develop in the clinic with Imprime PGG.

**Chrysalis Biotherapeutics Inc.**, of Galveston, Texas, said data demonstrating that TP508, its lead candidate, activates stem cells in the gastrointestinal system to prevent breakdown of the intestinal mucosa and decrease ionizing radiation-induced mortality were published online at *Nature Journals*. Data showed a single injection of TP508 stimulated proliferation of intestinal stem cells and reversed effects of nuclear radiation exposure.

**Deciphera Pharmaceuticals LLC**, of Waltham, Mass., highlighted the publication of study results describing the preclinical profile of its phase I cancer candidate, altiratinib, a spectrum-selective inhibitor of MET, TIE2 and VEGFR2 kinases. The article describes how altiratinib's balanced inhibition of the three key kinases was achieved using Deciphera's switch control kinase inhibitor platform and how altiratinib was shown to inhibit not only wild-type MET, but also oncogenic mutant forms of MET not readily inhibited by other MET inhibitors in development, according to the company. The article, which will appear in the September 2015 issue of *Molecular Cancer Therapeutics*, was pre-published online on Tuesday.

**Evoke Pharma Inc.**, of Solana Beach, Calif., said it received an FDA letter indicating the agency's concurrence with its proposed pediatric study plan for EVK-001. That includes a full waiver of the requirement to conduct pediatric studies on the basis that diabetic gastroparesis is an adult disease. The company expects that the pediatric study plan will be included in its anticipated new drug application filing. Shares of Evoke (NASDAQ:EVOK) gained 48 cents, or 10.4 percent, to close Tuesday at \$5.11.

**Insys Therapeutics Inc.**, of Phoenix, said it filed a citizen petition with the Drug Enforcement Agency to request the agency reschedule its synthetic pharmaceutical cannabidiol (CBD) from schedule I to schedule IV. The company said it believes the current classification of synthetic CBD as a schedule I compound is a significant barrier to the progress of research studies that explore its value in the treatment of several serious medical conditions.

**OTHER NEWS TO NOTE**

**Kythera Biopharmaceuticals Inc.**, of Westlake Village, Calif., which is in the process of being acquired by Dublin-based **Allergan plc**, said it submitted a marketing authorization application in the European Union seeking approval of ATX-101 (deoxycholic acid) injection as a treatment for the reduction of submental fat when the presence of submental fat has a psychological impact for the patient. The application was submitted via the decentralized procedure with Sweden as the reference member state and is supported by results from four pivotal phase III trials conducted in Europe and North America. ATX-101 was approved earlier this year in the U.S., where it is marketed as Kybella. (See *BioWorld Today*, April 30, 2015, and June 18, 2015.)

**La Jolla Pharmaceutical Co.**, of San Diego, said the FDA granted orphan drug designation for two small-molecule kinase inhibitors designed to selectively block a specific member of the bone morphogenetic protein (BMP) type-I receptor family, ALK2, for fibrodysplasia ossificans progressive (FOP). La Jolla said it entered a worldwide, exclusive license agreement with Vanderbilt University covering that technology. The seven members of the BMP type-I receptor family, activin receptor-like kinase (ALK) 1-7, play critical roles in human development and physiology. In turn, the improper activation of those receptor pathways is responsible for a wide range of disease conditions. For example, FOP, a rare genetic disorder where the body turns muscle into bone, is caused by a genetic mutation in ALK2 that results in excessive signaling of that pathway. The company will fund further research under the program at Vanderbilt in return for rights to acquire compounds emerging from the program.

**Kadimastem Ltd.**, of Ness Ziona, Israel, said it intends to file with the FDA a pre-investigational new drug application for its cell-based treatment for amyotrophic lateral sclerosis (ALS) in the upcoming weeks after what is said was the successful conclusion of its proof-of-efficacy testing with a preclinical trial. The trial tested the efficacy of injecting astrocytes produced through the company's technology from pluripotent stem cells into the spinal fluid of an ALS rat model, demonstrating what the company said was a significant improvement in the rats' life expectancy.

**Merck & Co. Inc.**, of Kenilworth, N.J., is recalling about 276,000 units of oral chemotherapy drug Temodar and its generic version, temozolomide, due to cracked caps, rendering the child-resistant closure ineffective. No injuries have been reported.

**Mylan NV**, of Potters Bar, UK, said it has been sued by London-based **BTG International Ltd.** and units of New Brunswick, N.J.-based **Johnson & Johnson** – Janssen Biotech Inc., Janssen Oncology Inc., and Janssen Research & Development LLC – in the U.S. District Court for the District of New Jersey, in connection with the filing of an abbreviated new drug application (ANDA) with the FDA for abiraterone acetate tablets, 250 mg. That product is the generic version of Zytiga,

which is indicated in combination with prednisone for the treatment of patients with metastatic castration-resistant prostate cancer. Mylan said it believes it is one of the first companies to have filed a substantially complete ANDA containing a paragraph IV certification for Zytiga and expects to be eligible for 180 days of marketing exclusivity upon final FDA approval.

**Portage Biotech Inc.**, of Toronto, said subsidiary Biohaven Pharmaceutical Holding Co. Ltd. acquired worldwide intellectual property rights to a portfolio of more than 300 glutamate-modulating prodrugs owned by **ALS Biopharma LLC**, of Doylestown, Pa., for an undisclosed amount, expanding the company's plans to develop a portfolio of compounds for diseases where aberrations in glutamate function have been implicated in the pathophysiology of the underlying illness. Potential target indications thought to involve glutamate neurotransmission include amyotrophic lateral sclerosis, Alzheimer's disease, Rett syndrome, dementia, dystonia, tinnitus, anxiety disorders, affective disorders and a variety of cancers, the company said. The prodrugs covered by the agreement were designed and prepared by Fox Chase Chemical Diversity Center Inc. through a research program funded, in part, by the NIH. (See *BioWorld Today*, Jan. 3, 2014.)

**Tapimmune Inc.**, of Seattle, said the Mayo Clinic's investigational new drug application for a folate receptor alpha vaccine has been fully transferred to the company as part of the recently completed licensing agreement between the parties. Tapimmune said the transfer marks a critical step in reaching the company's milestone of initiating phase II trials in patients with triple-negative breast cancer or ovarian cancer.

**Visterra Inc.**, of Cambridge, Mass., reported that new preclinical results for VIS410, a monoclonal antibody in development for the treatment of seasonal and pandemic influenza, were published online in the *Proceedings of the National Academy of Sciences*. In the paper, "A Broadly Neutralizing Human Monoclonal Antibody is Effective Against H7N9," Visterra scientists and scientists at the Massachusetts Institute of Technology, University of Hong Kong and Utah State University describe preclinical data demonstrating that VIS410 binds to a wide range of representative group 1 and group 2 influenza viruses, and the antibody protected mice challenged with the H3N2 and H7N9 virus. Additionally, the paper presents preclinical data that VIS410 demonstrated a synergistic effect with Tamiflu (oseltamivir, Roche AG), an existing small-molecule antiviral drug. Visterra said the data support the continued development of the candidate as a single-administration treatment for seasonal and pandemic influenza A. (See *BioWorld Today*, Oct. 2, 2014.)

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## IN THE CLINIC

**Arno Therapeutics Inc.**, of Flemington, N.J., said it received authorization from the UK's Medicines and Healthcare products Regulatory Authority to begin enrolling patients in the second stage of an open-label phase I/II trial evaluating its lead compound, onapristone, in men with advanced castration-resistant prostate cancer (CRPC). The second-stage protocol has been modified to evaluate onapristone in combination with Zytiga (abiraterone, Johnson & Johnson) in patients with CRPC that expresses the progesterone receptor (PR) or the T878A androgen receptor mutation, a mutation that has been demonstrated to bind progesterone. The study also includes a phase II cohort of patients with PR-positive CRPC who will receive single agent onapristone. (See *BioWorld Today*, Oct. 31, 2013.)

**Bavarian Nordic A/S**, of Copenhagen, said it started a phase I study of its MVA-BN RSV vaccine candidate against respiratory syncytial virus (RSV). The trial, being conducted in the U.S., will evaluate the safety, tolerability and immunogenicity of a recombinant MVA-BN-based RSV vaccine in 63 healthy adults, ages 18 to 65. Subjects will be enrolled into three groups to receive different doses of MVA-BN RSV. One group will enroll subjects of 50 to 65 years of age who will receive a higher dose of MVA-BN RSV in order to evaluate the immune responses in an elderly population, which is a key target for the vaccine.

**Boehringer Ingelheim GmbH**, of Ingelheim, Germany, said phase III data published in *Respiratory Medicine* from the OTEMTO 1 and 2 trials showed that Spiolto Respimat provided clinically meaningful improvements in SGRQ total score, compared to placebo, in patients with chronic obstructive pulmonary disease. SGRQ is a disease-specific patient-reported instrument that evaluates symptoms, activity limitation and the social and emotional impact of the disease. A reduction in SGRQ score represents an improvement, while a reduction of 4 points or more compared to placebo is considered clinically meaningful. In the OTEMTO trials, patients taking Spiolto Respimat reported a reduction in SGRQ total score of 4.67 points compared to placebo.

**Oncosec Medical Inc.**, of San Diego, said it enrolled the first patient in a phase II, investigator-sponsored trial led by the University of California, San Francisco, to assess the antitumor activity, safety and tolerability of the combination of Oncosec's investigational therapy, Immunopulse IL-12, and Kenilworth, N.J.-based **Merck & Co. Inc.**'s approved anti-PD-1 agent, Keytruda (pembrolizumab), in patients with unresectable metastatic melanoma. The primary endpoint is the best overall response rate of the combination regimen in patients whose tumors are characterized by low numbers of tumor-infiltrating lymphocytes. The single-arm study will enroll about 42 patients. Immunopulse is designed to enhance the local delivery and uptake of DNA-based immune-targeting agents.

**Remedy Pharmaceuticals Inc.**, of New York, said it launched an open-label pilot study testing Cirara, a high-affinity inhibitor

of Sur1-Trpm4 channels, in patients with acute traumatic cervical spinal cord injuries (SCI). The study will enroll and treat up to a maximum of 10 patients who will be matched in a 1-to-3 ratio with historical controls. Patients with cervical (C4-C8) level complete or incomplete injuries, ages 18 to 70, will be eligible for inclusion in the study, and data obtained from the pilot program should inform the design of further multicenter phase II/III studies evaluating the efficacy of Cirara in improving functional outcomes following SCI. Remedy said Cirara is being developed for intravenous delivery at the bedside or in an ambulance.

**Tonix Pharmaceuticals Holding Corp.**, of New York, said it is conducting the Atease study, a randomized, double-blind, placebo-controlled clinical trial to evaluate the efficacy and safety of TNX-102 SL for the treatment of patients with military-related post traumatic stress disorder (PTSD). The 12-week study is measuring performance of TNX-102 SL 2.8 mg on the total Clinician-Administered PTSD Scale for DSM-5 score as the primary endpoint. Enrollment in the 220-patient trial is about 50 percent complete, and the company expects top-line data in the first half of next year. TNX-102 SL is designed with antagonist activities at the serotonin-2A, alpha-1 adrenergic and histamine H1 receptors to provide broad-spectrum symptom improvement in PTSD by targeting sleep and the stress response.

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