Life Science Leader

Magazine Article | March 1, 2019

Companies-To-Watch 2018 Annual Roundup

Source: Life Science Leader

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At the end of each year, I go back to the companies we featured for that year in the “Companies To Watch” section and ask them to update me on their progress. This year, rather than try to cover all bases, I will highlight a few specific points.

An overriding event has informed my choice of highlights. It is happening right now, as I write this. The stock market has just gone crazy, through the weeks of Christmas, New Year's, and the JP Morgan Healthcare Conference. Though I always say CtW is not about stocks, the current market has invoked some thoughts I believe are relevant as a tool for understanding this industry.

In 2018, it turned out that most of the companies covered in this column were public; only three were private. All but three of the public companies are on NASDAQ, one is on the New York exchange, one on Euronext, and one is a "penny stock" on the OTC market. When selecting companies to cover, I do not look at stock status or performance, so the patterns last year and in previous years are purely accidental. In some years, private companies or those on other exchanges may have had the majority. This year's pattern may reflect the higher- than-normal likelihood that companies at early stages took part in the surge of IPOs over the past few years.

So, peeking at the data, how did our 2018 CtW public companies fare on the market during the month from early December to mid-January? Actually, not too bad. Though all of them lost some value in that period, and a few had particularly bumpy rides, they all appeared to be recovering and headed back to their starting prices by the end. Keep in mind, these are not Apple stocks; not one of the group ended the period above $2.00 a share.

What conclusions can we draw from those few data points? (Well, inferences might be a more practical goal.) One, at such tiny values, even a movement of a few cents could be challenging in certain cases, as when a company faces a severe cash shortage. But also, in general, we can say the big movements in the overall stock market at the end of the year did not obviously rock the worlds of our CtW. They all weathered the changes, and some made significant progress all through the crisis. Big disclaimer: No one I know seems to have missed the main point the market drove home — anything can change from one pole to the other, any time.

I'm not suggesting stock performance doesn't matter. It is a fundamental component of market cap and therefore key to leverage in partnering, licensing, or mergers. I am suggesting that, if you are watching a company over time, there is a more important set of data to observe, the R&D kind. Although companies may fail because of poor business management, or maybe cash out early in an ultimately ill-fated deal, most live or die by the human clinical data they generate. Don't just look at proof of concept or "response." Even Phase 2 trials often mislead. Many programs look great until they reach Phase 3. With all of that in mind, we present the following updates from our 2018 Companies to Watch.

JANUARY

Daré Biosciences

Expanding a female-products pipeline beyond birth control.

"At the start of 2018 we were a one-product company focused entirely on advancing our first-in-class, nonhormonal, monthly birth control ring Ovaprene. We are now developing a diverse women's health portfolio focused on several indications with persistent unmet needs including contraception, vaginal health, sexual health, and fertility. We've now acquired global rights to five subsequent novel innovations: 1) a topical cream formulation of sildenafil, the active ingredient of Viagra, which has potential to be the first FDA-approved product for female sexual arousal disorder, a condition much like erectile dysfunction in men; 2) an intravaginal ring platform, which allows for more convenient administration of a wide range of drugs for women; 3) a tamoxifen insert for the treatment of vulvar and vaginal atrophy, a condition caused by reduced estrogen levels that leads to chronic vaginal dryness, irritation, and painful sex; 4) a novel target that has potential in both male and female contraception; and 5) a solution-to-gel formulation of Clindamycin for the treatment of bacterial vaginosis (BV). Headed into 2019 we are prepared to announce a number of important milestones including results from our Ovaprene prepivotal study, initiation of our sildenafil cream Phase 2b at-home study, and the initiation of the single Phase 3 registrational trial for our BV program. We will continue to push forward to bring to market novel innovations in women's reproductive and sexual health that improve outcomes and facilitate convenience for women."

SABRINA MARTUCCI JOHNSON

Founder, President, & CEO

FEBRUARY

Zavante, now Nabriva

The company was acquired, renamed, and augmented.

"In July 2018, Zavante was acquired by Nabriva Therapeutics, a clinical-stage biopharmaceutical company engaged in the research and development of novel anti-infective agents to treat serious infections. In October 2018, Nabriva Therapeutics submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) to seek marketing approval for Contepo (fosfomycin for injection) to treat complicated urinary tract infections (cUTIs), including acute pyelonephritis. Nabriva also planned to submit an NDA for lefamulin, a first-in-class pleuromutilin antibiotic available for IV and oral administration in humans to treat community-acquired bacterial pneumonia (CABP), in the fourth quarter of 2018. The NDA will be supported by the company's Phase 3 program, which includes two pivotal Phase 3 trials

evaluating the safety and efficacy of both intravenous and oral lefamulin in the treatment of adults with CABP compared to current gold-standard therapies. In both studies, lefamulin met all FDA and European Medicines Agency (EMA) primary and secondary endpoints, and was shown to be generally well tolerated. Both Contepo and lefamulin have been granted Qualified Infectious Disease Product (QIDP) and Fast Track designations by the FDA, which enables priority review of the NDAs, following acceptance. Nabriva is positioned to potentially launch two first-in-class antibiotics in 2019. Both products address significant unmet medical needs where patients and their physicians oftentimes struggle with the increasing challenges of bacterial resistance to conventional antibiotic therapies."

TED SCHROEDER

President & CEO

MARCH

AmpliPhi Biosciences

Advancing to late-stage development of bacteriophages to overcome antibiotic resistance.

"AmpliPhi has made significant progress with an updated set of positive clinical outcomes from our ongoing expanded access program for seriously ill patients who suffer from antibiotic-resistant infections. Eighty-four percent of patients achieved treatment success at the end of therapy as determined by physicians' assessment. Twenty-one patients at seven hospitals, with serious or life-threatening infections not responding to antibiotics, were treated with AB-SA01 or AB-PA01. More than 1,000 doses of AmpliPhi's bacteriophage product candidates have now been administered as part of the program since mid-2017. Treatment was generally well tolerated, with no treatment-related serious adverse events. Additionally, AmpliPhi received positive FDA feedback for AB-SA01 and AB-PA01 following two Type B meetings. AmpliPhi announced that the FDA is in general agreement with the design of four proposed randomized clinical trials. Two of the trials are intended to evaluate AB-SA01 for the treatment of S. aureus bacteremia and prosthetic joint infections. The other two trials are intended to evaluate AB-PA01 for the treatment of P. aeruginosa hospital-acquired and ventilator-associated pneumonia (HAP/VAP) and bacteremia. Importantly, no additional preclinical or clinical data is required to proceed with any of the four trials. We look forward to building on the successes achieved in 2018 by initiating at least one clinical trial in 2019 and helping treat additional patients under AmpliPhi's ongoing expanded access program."

DR. PAUL GRINT

CEO

APRIL

CytoDyn

Acquisition widens therapeutic focus from HIV to cancer and inflammation.

"On July 12, 2018, the company announced certain leadership changes in connection with the strategic expansion and entry into certain cancer and immunologic indications. Denis Burger, Ph.D., resigned as chief science officer and from the company's board. Nader Pourhassan now serves as president and CEO. The company also acquired Prosta- Gene to help drive its strategic expansion of lead product PRO 140 development into the cancer and inflammatory space. Richard Pestell, CEO of ProstaGene, is CytoDyn's new CSO. "Research has shown CCR5 to be an important receptor involved in tumor cell metastasis and immune cell trafficking, thus making it an interesting therapeutic target for a variety of important disease indications," said Dr. Pestell. "Preclinical research with PRO 140, which blocks the CCR5 receptor, has shown its ability to effectively inhibit cancer cell invasiveness, and CytoDyn's clinical studies of PRO 140 in HIV have shown the antibody to have an excellent safety and tolerability profile in humans."

INFORMATION COMPILED FROM COMPANY PRESS RELEASES

MAY

Landos Biopharma

Moving on to Phase 2 development with its lead product for IBD.

"Landos has continued to advance its pipeline of new compounds for the treatment of autoimmune diseases, including inflammatory bowel disease (IBD), Crohn's disease, ulcerative colitis, Type 1 diabetes, and lupus. Landos' lead clinical asset, BT-11, is a novel, oral, gut-restricted small molecule therapeutic targeting the lanthionine synthetase C-Like 2 (LANCL2) pathway in the gastrointestinal tract for treatment of IBD. Landos' Phase 1 SAD and MAD studies of BT-11 completed enrollment with topline results showing no dose-limiting toxicities and a wide safety margin for BT-11. Topline results were released early January 2019. Based

on successful Phase 1 testing following FDA clearance of two open INDs for BT-11 for the treatment of ulcerative colitis and Crohn's disease, Landos is planning to initiate Phase 2 studies in Crohn's and ulcerative colitis patients in early 2019. The company is raising a $60 million Series B round to fund the Phase 2 development of BT-11 and to advance several additional clinical candidates in its pipeline to INDs and Phase 1 studies in 2019."

JOSEP BASSAGANYA-RIERA

Chairman & CEO

JUNE

Sensorion

Starting with narrow ear disease indications; working toward the large market of vertigo.

"In August 2018, Sensorion received the European authorization to initiate a SENS-401 Phase 2 trial in sudden sensorineural hearing loss (SSNHL), making this Sensorion's second European Voluntary Harmonization Procedure Application approval. Interim topline data for this international multicenter Phase 2 trial with SENS-401 for treating SSNHL in adults is expected in Q4 2019. Sensorion is also preparing a Phase 2 study of SENS-401 for the prevention of ototoxicity induced by cisplatin in a pediatric population to be launched in 2019. The company is also currently conducting two Phase 2 trials of their other candidate for inner ear disease, SENS-111, now known as Seliforant. For their study on acute unilateral vestibulopathy (AUV), results are expected in H2 2019. Commercially, the value of Seliforant goes far beyond this AUV indication and resides in the opportunity to get the full label for vertigo. Preclinical and Phase 1 clinical data already confirmed that Seliforant modulates the peripheral vestibular apparatus, reduces symptoms associated with vestibular dysfunction, and is safe and well tolerated. Sensorion has initiated exclusive negotiations with the Pasteur Institute for hearing loss gene therapy programs with the goal of negotiating a framework agreement to obtain the exclusive licenses to develop and commercialize gene therapy product candidates for restoration, treatment, and prevention of hearing loss disorders."

NAWAL OUZREN

CEO

AUGUST

Acceleron

Positive Phase 3 results pave way for possible regulatory approvals in myelodysplastic syndromes and beta-thalassemia.

"Acceleron recently reported positive results with our lead product candidate, luspatercept, in two Phase 3 trials — one in myelodysplastic syndromes (MDS) and one in beta-thalassemia — with our partner, Celgene. Luspatercept, an investigational first-in-class erythroid maturation agent believed to work by regulating latestage red blood cell maturation, met all primary and key secondary endpoints in patients with transfusion-dependent beta-thalassemia and patients with lower-risk MDS. Along with Celgene, we plan to file for approval in the U.S. and EU in the first half of 2019. In addition to the recent initiation of our Phase 3 trial in treatment-naïve MDS patients, we have ongoing luspatercept clinical trials in nontransfusion-dependent beta-thalassemia and myelofibrosis. We continue to advance our wholly owned programs in neuromuscular and pulmonary diseases. We are expecting preliminary results from our ongoing Phase 2 trials of ACE-083 in the second half of 2019 for facioscapulohumeral muscular dystrophy (FSHD) and by year-end 2019 for Charcot-Marie-Tooth disease (CMT). We are also advancing a Phase 2 trial and an exploratory study in pulmonary arterial hypertension, a rare, progressive, and life-threatening blood vessel disorder for which currently there is no cure."

HABIB DABLE

President & CEO

SEPTEMBER

United Neuroscience

Adding Parkinson's to the target list for its therapeutic vaccines.

"In the fourth quarter, United Neuroscience (UNS) strengthened its vaccine pipeline with the presentation of preclinical evidence for UB-312, a Parkinson's disease vaccine candidate, at the Parkinson's U.K. Research Conference. Looking forward to 2019, UNS will commence a Phase 1 study with UB-312. UNS also recently announced new executive appointments, including vice president of preclinical development, head of research and development operations and project management, and head of clinical operations. These highly accomplished executives will enable UNS to continue advancing the clinical and preclinical vaccine programs to address a multitude of brain health issues."

MEI MEI HU

CEO

OCTOBER

Symbiotix Biotherapies

Closer to clinical trials for TREG modulators derived from the human microbiome.

"Symbiotix Biotherapies is a pioneering, venture-backed microbiome company founded on the basis of over two decades of research at Brigham & Women's Hospital, Harvard Medical School, California Institute of Technology, and Dartmouth College. Symbiotix is developing a novel class of TREG-modulating therapeutics based on molecules derived from the human microbiome and is focused on treating patients with inflammatory bowel disease (IBD), multiple sclerosis (MS), and other serious immune-mediated diseases. Symbiotix has continued to leverage its technology platform, product pipeline, and broad IP portfolio to advance discussions that will facilitate the commencement of human clinical trials in the next 12 to 18 months."

NADER YAGHOUBI

President & CEO

NOVEMBER

Athersys

Phase 3 progress for MultiStem ischemic stroke treatment and a China deal.

"A Phase 3 clinical trial of MultiStem regenerative-medicine product for ischemic stroke is moving ahead at a good pace. [In December, Athersys announced extension of an option that would allow Healios to obtain a license for the development and commercialization of MultiStem therapy for certain indications in China.] China now represents the second-largest healthcare market in the world, surpassing Japan, and we and Healios both view it as being strategically important to our long-term goals and objectives."

GIL VAN BOKKELEN

Chairman & CEO