Commercial insight: cell and gene therapy

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Providing a critical overview of the sector's commercial developments – M&As, licensing agreements & collaborations, financial results, IPOs and clinical/regulatory updates, with commentary from our Expert Contributors.

CELL THERAPY: After a prolonged process, Astellas was successful in bringing its takeover bid of Ocata to a close this past month. Shareholders pushed back on several occasions in attempt to thwart the bid, requiring Astellas to extend the period within which shareholders could tender shares on two occasions. Ultimately, a majority of shareholders accepted the $8.50 per share offered by Astellas, giving them a 79% premium to market as of the day the bid was announced. Shareholders argued the offer didn't reflect additional assets in the Ocata portfolio outside the core focus on ophthalmology. Ocata was one of the original pioneers in the RM space, having setup shop in the mid 90's. Despite a few bumps in the road the company endured more than 20 years. The acquisition by Astellas, while met with some resistance, will hopefully ensure Ocata's assets are developed swiftly and brought to market.

GENE THERAPY: Funding continues to be a hot-topic again this month and the recent IPOs in New York from both AveXis and Proteostasis have shown that the IPO window is still just about open. The other piece of financial news was that Renova

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Therapeutics has just received a substantial grant from the NIH to support its gene therapy development in heart failure. There is much debate about whether it is best to still try for an IPO at this time or wait till the markets improve, but I think this news from these three companies still shows that as a growing company you will take funding from wherever you can get it despite the difficulties. If the IPO window does firmly get closed off for the while, companies should not forget that grants are a good alternative source too.

BLUEBIRD BIO INITIATES FIRST CAR-T CLINICAL TRIAL

The first clinical study of Bluebird's chimeric antigen receptor T cell (CAR T) therapy, bb2121, was launched for patients with multiple myeloma. Following their option for an exclusive license, Celgene has opted in through a $10 million payment to secure the deal.

In what is perceived to be a sensible move on Celgene's part to secure their multiple myeloma position, they have honed their collaborative arrangement with Bluebird to concentrate on the CAR-T program.

bb2121 targets B cell maturation antigen (BCMA) and early clinical data from Dr James Kochenderfer of the National Cancer Institute, an investigator for the study, showed promise. A cancer patient with multiple myeloma experienced a complete remission two months following CAR-BCMA treatment. "BCMA is one of the most exciting targets in multiple myeloma, and we are eager to explore the potential of bb2121 to become an important new treatment option for patients living with multiple myeloma," explained Kochenderfer.

BCMA, a member of the tumor necrosis factor family, is a protein expressed by both normal and malignant plasma cells. "BCMA is uniformly expressed on 60% to 70% of cases of multiple myeloma. Because BCMA is only expressed by plasma cells and a small fraction of B cells, it is a promising target for treating multiple myeloma," said Kochenderfer.

Bluebird Bio made its oncology debut in the clinic and announced enrollment of the first patient in a multiple myeloma study investigating bb2121, a CAR product targeted to B cell maturation antigen (BCMA). While the hematological malignancy landscape is crowded, multiple myeloma has remained an indication that only a few companies have pursued. Neither Juno nor Kite have the indication in their pipelines, however Adaptimmune is partnered with GSK to try and tackle it with its TCR targeting NY-ESO. Celyad is also working on multiple myeloma with a CAR product targeting the NKG2D ligands. Early clinical evidence from the National Cancer Institute had positive signals. Bluebird has made itself a niche with a compelling target and could be the leader in this indication given its expertise and capitalization. - ***Mark Curtis & Rahul Sarugaser.***

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AVROBIO TO DEVELOP NOVEL, CLINICAL-STAGE CELL & GENE THERAPIES

Clinical-stage biotechnology company, AVROBIO Inc. have announced their launch plans. The company will accelerate the development of two novel cell and gene therapies established in the labs of Dr Christopher Paige and Dr Jeffrey Medin at the University Health Network (UHN) in Toronto.

The company will develop new therapies targeting cancer and other rare diseases. Phase 1 programs will hopefully be in the clinic by early to mid-2016 for acute myeloid leukemia and Fabry disease.

"AVROBIO's highly innovative therapies offer potentially life-altering impact for patients following a single infusion of genetically-modified cells," said Geoff MacKay, AVROBIO's President and CEO.

AVR-01 is being developed as an anti-cancer immunotherapy which triggers the immune system to first detect, and then eradicate, tumor cells. A patient's cancer cells are genetically modified to express one of the most powerful immune signalling agents, the cytokine IL-12. The modified cells are then infused back into the patient where they quickly activate cytotoxic $CD4^+$ T cells which specifically target tumor cells and thus eliminate the cancer. A long-lasting anti-cancer immune response is maintained via both $CD4^+$ and $CD8^+$ cytotoxic T cells.

AVR-02 is designed to deliver lasting benefits for patients with Fabry disease. The company's approach is to genetically modify a patient's own cells by adding a functional copy of the faulty gene. $CD34^+$ hematopoetic stem cells are genetically modified to express an enzyme and then infused back into the patient. The objective is to deliver long-lasting or permanent, continuous elevation of endogenous enzyme thereby significantly improving patient outcomes.

"We are very proud to carry forward the ground-breaking work of our founding scientists with investment from Atlas Venture and partnership with the Center for Commercialization of Regenerative Medicine (CCRM). We have built a team capable of accelerating the development of truly important therapies for patients in immense need." - Geoff MacKay.

This month I have selected as my choice both AVROBIO and Juventas as the 'Ones to Watch'. The reason for this is that AVROBIO have announced their launch plans detailing the development of two novel gene therapy programmes to treat acute myeloid leukemia and Fabry disease using the combination of autologous cell transplantations that have been modified by gene therapy. As a procedure this is not particularly new, but it is interesting to see that many groups are now adopting this approach and building on the success that other groups have already had. This must de-risk the programmes to some extent and clearly investment groups such as Atlas Ventures have recognized this and are willing to invest - *Alan Boyd.*

FDA GRANTS BREAKTHROUGH THERAPY DESIGNATION FOR ADAPTIMMUNE'S T-CELL THERAPY

The company's affinity enhanced T-cell therapy targeting NY-ESO in synovial sarcoma has been granted Breakthrough Therapy designation by the FDA. The treatment will target patients who test positive for the HLA-A*201, HLA-A*205 or HLA-A*206 alleles with tumors expressing the NY-ESO-1 antigen.

The decision to grant Breakthrough Designation was based on data presented at the 2015 Annual Meeting of the Society of Immunotherapy for Cancer (SITC) from a phase I/II trial in patients with unresectable, metastatic or recurrent synovial sarcoma who have previously received chemotherapy.

Patients were treated with lymphodepleting chemotherapy followed by immunotherapy with T-cells engineered to recognize an HLA-A2 restricted NY-ESO-1 peptide. The data demonstrated 60% of patients responded when receiving the target dose of cells, with 90% of those patients still alive at long-term follow up.

Adaptimmune is currently aiming to initiate pivotal studies with their affinity enhanced T-cell therapy targeting NY-ESO in synovial sarcoma towards the end of the year.

"We are committed to investigating the potential of our NY-ESO-1-T cell therapy across a variety of cancers. We are pleased that the FDA has granted Breakthrough Therapy designation for our T-cell therapy in synovial sarcoma, recognizing both the unmet need for patients suffering from this disease as well as the promise of these early data. We look forward to working closely with the FDA to expedite the clinical development of this therapeutic candidate." - Dr Rafael Amado, Adaptimmune's CMO.

CYTORI CELL THERAPY ELICITS SUSTAINED IMPROVEMENT IN PATIENTS WITH SCLERODERMA

Cytori Therapeutics, Inc. presented data demonstratng that a single dose of Cytori Cell Therapy™ (ECCS-50) resulted in a sustained improvement in hand dysfunction for two years in patients with scleroderma, a rare autoimmune disease that causes tissue hardening due to the accumulation of fibrotic tissue.

"Hand dysfunction in scleroderma is both disabling and hard to treat. The standard treatments we rely on for these patients are often not effective or are poorly tolerated because of side effects. The broad efficacy signals along with a very favorable safety profile makes ECCS-50 one of the more promising new therapeutic options for these difficult to treat patients." said Dr Guy Magalon, the trial co-principal investigator at Assistance Publique-Hôpitaux de Marseille, France, who presented the data on behalf of the team.

The results from the 12 month follow up for this trial were published

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in August 2015 in Rheumatology. The data suggests that a single dose of ECCS-50 was safe and that the treated patients were able to display significant improvements in hand symptoms.

"The scientific findings of sustained symptomatic and functional improvement coupled with the direct patient feedback we have received from other scleroderma patients treated apart from this trial leads us to be optimistic about the potential for ECCS-50 to help patients worldwide," highlighted Dr Marc Hedrick, President and CEO of Cytori Therapeutics Inc.

ZIOPHARM ANNOUNCES FIRST PATIENT ENROLLED IN PHASE I STUDY

Biopharmaceutical company, ZIOPHARM Oncology, Inc., announced the enrolment of their first patients in a Phase I clinical study of their second-generation non-viral CD19-specific CAR modified T-cell therapy for patients with advance lymphoid malignancies.

The Sleeping Beauty system, a transposon-transposase non-viral system used to deliver genes that encode CARs and T-cell receptors into lymphocytes, was used to modify the CD19-specific T cells to achieve stable CAR expression in T cells. This system is exclusively licensed by Intrexon Corporation through the University of Texas MD Anderson Cancer Center, USA. This system is hoped to have a number of advantages over traditional viral delivery, including lower costs for generating modified T cells, minimal *ex vivo* processing requirement and the potential to target solid tumor antigens.

"Sleeping Beauty offers the potential to significantly reduce the expense and simplify the implementation of genetically modified T cells, both of which are critical to the personalization and broad application of immunotherapies based on CARs and TCRs," explained Laurence Cooper, CEO of ZIOPHARM.

A trial, currently being conducted at MD Anderson, will use second-generation Sleeping Beauty CAR+ T cells with an altered CAR construct designed to improve persistence and anti-tumor response.

ZIOPHARM'S COMPETITIVE EDGE?

Ziopharm separates itself from the pack with the use of the Sleeping Beauty transposon-transposase system in its second generation CD19 CAR product, which is able to stably express CARs in cells without the need for viruses. The company is accessing the technology via its collaboration with Intrexon, which has a license to the system from MD Anderson Cancer Centre. Implementing a non-viral approach to CAR production will introduce process efficiencies and eliminate manufacturing costs that will give Ziopharm a competitive edge. The company's second generation CD19 product, using Sleeping Beauty, also comes with the added benefit of a revised CAR construct to improve persistence and potency. Overall, Ziopharm has a unique platform combining a number of interesting synthetic DNA technologies that position it well for the development of a new wave of cell and gene products - *Mark Curtis & Rahul Sarugaser.*

JUVENTAS TESTS GENE THERAPY ON WOUNDS IN PHASE II TRIAL

Juventas has initiated a new clinical trial to assess whether the company's gene therapy could be used to treat patients with advanced peripheral artery disease

The company has enrolled the first patient in a Phase II trial, which will treat people who suffer from wounds caused by advanced peripheral artery disease, a disease that restricts blood flow to the limbs, which causes pain and can sometimes lead to amputation.

Previous human trials have shown that the drug, JVS-100, has "the ability to improve micro-vascular blood flow and accelerate dermal wound closure," according to the lead investigator for the new trial, Mehdi Shishehbor.

Thus, the therapy might be able to help patients enrolling in the new trial. They've already undergone another procedure called below-the-knew revascularization, but many of them "continue to suffer with non-healing wounds on their feet that interfere with their mobility and day-to-day living," Shishehbor said in a news release.

In relation to Juventas, they are developing a treatment for wounds, such as leg ulcers, that have resulted from patients having the concomitant disease of peripheral arterial disease. Much work has been done in the past in relation to treating peripheral vascular disease with gene based therapies and they did not work. However there is a clear unmet need in this area so it is probably worth looking at it again - *Alan Boyd*.

AVEXIS SURVIVES THE MARKET STORM

AveXis has attracted a solid set of investors interested in their gene therapy for rare cases of spinal muscular atrophy. They hit the midpoint in their price range, pricing its shares at $20 with the help of investors who had signalled their willingness to snap up about a third of the 4.75 million shares on offer.

Like many of the other biotechs in the gene therapy field, the company relies on an AAV vector for delivery, in this case a working copy of a survival motor neuron gene needed to spur production of missing protein. Although they only have a handful of patients in Phase I, AveXis now has $95 million to fund their work.

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EDITAS MEDICINE ANNOUNCES PRICING OF IPO

Editas Medicine, Inc., announced the pricing of its initial public offering of 5,900,000 shares of common stock at a public offering price of $16.00 per share. The company has granted the underwriters a 30-day option to purchase up to an additional 885,000 shares of common stock from Editas Medicine at the public offering price. The company's common stock has been approved for listing on the NASDAQ Global Select Market and is expected to begin trading under the ticker symbol "EDIT."

RENOVA THERAPEUTICS RECEIVES NIH GRANT TO CONTINUE GENE THERAPY RESEARCH

The National Institutes of Health (NIH) has awarded Renova Therapeutics a grant to support the company's preclinical gene therapy research for congestive heart failure. This is the first portion of a second NIH grant, totalling $1.6 million that the San Diego-based biopharmaceutical company is receiving. Their work focuses on increasing heart function by intravenous delivery of the *urocortin-2* gene via an adeno-associated viral vector.

The grant will help fund Renova's research and its scientific team headed by co-founder Dr H Kirk Hammond, whose paracrine gene therapy approach has been shown to dramatically improve animal models of congestive heart failure and type 2 diabetes.

This approach involves sustained activity following systemic delivery of a gene therapy to the blood, and distribution to other organs where it can exert beneficial effects on target organs from a distance. This single-injection approach is a foundation for Renova Therapeutics products that have the potential to bring about permanent improvements in cardiovascular and metabolic diseases.

"What we're working on is a true innovation for a large patient population that has seen only incremental improvements in medicines, resulting in small changes in outcomes. With paracrine gene therapy, we're talking about the potential to attenuate the debilitating effects of heart failure with a simple IV injection, a common procedure that can be administered in any doctor's office." - Jack Reich, CEO and co-founder of Renova Therapeutics.

ASTELLAS TO ACQUIRE ALL OUTSTANDING SHARES OF OCATA THERAPEUTICS

Astellas Pharma Inc has successfully completed a tender offer to purchase all issued and outstanding shares of common stock of Ocata Therapeutics, Inc. for $8.50 per share.

Astellas commenced the tender offer on November 19, 2015, and the tender offer, previously extended, expired February 9, 2016, and was not extended. As a result of the merger, Ocata's stock will no longer be traded on the NASDAQ Global Market and will not be listed.

ADURO BIOTECH RECEIVES MILESTONE PAYMENTS FROM JANSSEN

Aduro Biotech, Inc. has received $22.4 million in clinical development milestone payments from license partner Janssen Biotech, Inc. The companies have been working in collaboration on the development of ADU-214 for the treatment of lung cancer, and ADU-741 for the treatment of prostate cancer, using Aduro's LADD technology platform.

LADD is Aduro's proprietary platform of live-attenuated double-deleted Listeria monocytogenes strains that have been engineered to induce a potent innate immune response and to express tumor-associated antigens to induce tumor-specific T cell-mediated immunity.

"Our relationship with Janssen has been exceptionally productive, with ADU-214 for the treatment of lung cancer and ADU-741 for the treatment of prostate cancer advancing in clinical studies. We believe these therapeutics may offer important alternatives for patients suffering from these aggressive cancers." said Stephen T Isaacs, Chairman, President and CEO of Aduro.

COBRA BIOLOGICS & CENTRE FOR PROCESS INNOVATION ANNOUNCE COLLABORATION

Cobra Biologics Ltd, international CDMO of biologics and pharmaceuticals, and the Centre for Process Innovation, a UK-based technology innovation centre, are collaborating on a project which will focus on the development of an Industrial Manufacturing Platform for Adeno-Associated Virus (AAV) production to support gene therapy and regenerative medicine. The £1.8M project, which is being led by Cobra, is being funded to a value of £1.4M by Innovate UK via their

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2015 competition for the development of regenerative medicines and cell therapies.

The project is one of five which together comprise an £8 million investment into collaborative R&D projects that address critical challenges faced by UK companies developing regenerative medicines and cell therapies as clinical treatments and commercial products.

The collaboration between Cobra and CPI will focus on AAV vectors which are safe and effective and are currently the delivery vehicle of choice for gene therapy treatments. However the advancement of these therapies into clinical trials is currently hampered by the lack of robust scalability needed to manufacture AAV vectors.

The proposed collaboration between Cobra and CPI will develop in depth scientific and technical understanding to allow a scalable and flexible manufacturing process to be developed to produce, purify and characterise a range of AAV vectors. This will enable the acceleration of more potential products into clinical testing and ultimately new medicines. This in turn will increase the chances of treatments being developed for a whole range of currently intractable diseases.

Peter Coleman, CEO Cobra Biologics commented: "Cobra has been a leading organisation in the development of the gene therapy sector for over 15 years now through its plasmid DNA and viral service offerings. We have seen through our customers an extraordinary growth in new potential medicines being put forward that utilise viral vectors as part of their production, such as CAR T-Cell therapies. The collaboration between Cobra and CPI will help to address the need for a scalable AAV production process to meet the current and future needs of the gene therapy and regenerative medicine community in clinical trials and commercial supply."

Dr Fergal O'Brien, Director of Biologics at CPI said: "CPI is delighted to be collaborating with Cobra Biologics Ltd and applying our expertise in developing scalable and industrial manufacturing platforms to AAV production. We see this project as a key enabler in meeting the current and future needs of the biologics industry and are delighted to be supporting a leading UK company in this sector."

ADAPTIMMUNE AND GSK EXPAND THEIR STRATEGIC COLLABORATION

Adaptimmune Therapeutics and GlaxoSmithKline have expanded the terms of their strategic collaboration in hopes of accelerating Adaptimmune's lead clinical cancer program. The affinity enhanced T cell immunotherapy will target the NY-ESO-1 antigen, with trials in synovial sarcoma.

The companies will accelerate the development of Adaptimmune's NY-ESO therapy into pivotal studies for patients with synovial sarcoma and will explore development in myxoid round cell liposarcoma.

"We are delighted to broaden our collaboration with GSK, which is also fully committed to the development of this revolutionary T-cell therapy. We believe that our affinity enhanced T-cell programs have the potential

to deliver important clinical benefit to cancer patients, and it is therefore essential that we accelerate our efforts to meet their needs. We are working closely with GSK to expedite development of our affinity enhanced T-cell therapy targeting NY-ESO, and if we succeed in generating pivotal data consistent with that of our ongoing studies, we believe it has the potential to be the first engineered T-cell therapy to reach the market," said James Noble, Adaptimmune's CEO.

The studies will be conducted by Adaptimmune, with funding provided by GSK for the pivotal studies and sharing of the costs of the combination studies via a success-based milestone structure.

"With this expanded collaboration, we have the opportunity to accelerate the lead program in synovial sarcoma toward pivotal trials and also to investigate several other tumor types and combine the T-cell therapy with immune-modulating therapies such as checkpoint inhibitors" said Dr Axel Hoos, Senior Vice President, Oncology R&D, GSK.

SCCA, FRED HUTCHINSON, & JUNO ANNOUNCE NEW IMMUNO-ONCOLOGY CLINICAL TRIALS UNIT

The Seattle Cancer Care Alliance (SCCA), Fred Hutchinson Cancer Research Center and Juno Therapeutics have announced a new clinical trials unit (CTU) specializing in immuno-oncology. All 3 will be investing in the new CTU with Juno providing additional research funds to Fred Hutch to support their pre-clinical and clinical research on cancer immunotherapies.

The CTU will have a dedicated space for research teams to collect trial data and specimens from patients, in addition to having trial patients in one location and is expected to be operational from the middle of 2016.

"Clinical trials are crucial steps in bringing safer, more effective cancer therapies to the patients who need them. The CTU will enable patients participating in clinical trials to get all of their care at a single site, with a team of dedicated surgeons, radiologists, pathologists and medical oncologists." - Fred Appelbaum, MD, SCCA Executive Director and President.

BUSY MONTH FOR CALADRIUS BIOSCIENCES

Caladrius Biosciences has entered into an agreement with SPS Cardio to out-license the patent and commercialization rights for the company's CD34 ischemic repair technology in select territories outside of the USA

SPS will fund the further development of this technology and will conduct a phase 2 proof-of-concept clinical trial in India to evaluate CD34 cell therapy for the prevention of adverse events in patients with heart failure. SPS will receive

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an exclusive license to the CD34 cell therapy technology for use in acute myocardial infarction and chronic heart failure in India and in other territories outside the UA.

"We are pleased to out-license our CD34 technology in AMI and CHF to SPS as it is in keeping with our strategic focus to build on our core competency in cell therapy process development and manufacturing. This agreement allows us to preserve capital, while retaining our ability to enter into separate development and commercialization partnerships for other indications and in other territories, such as for critical limb ischemia in Japan," - David J Mazzo, CEO of Caladrius.

Caladrius have also licensed exclusive global rights to their cell-derived dermatological technology for skin applications to the biotechnology company, AiVita Biomedical, Inc.

AiVita was founded in 2016 by Caladrius' former Chief Science Officer, Dr Hans Keirstead, with a goal of advancing commercial and clinical stage programs that employ the use of regenerative medicines to manufacture and distribute a cosmetic skincare products.

"Licensing this dermatological technology to AiVita is yet another step forward in streamlining our strategic focus while monetizing non-core assets through royalty- and/or other milestone-driven transactions. In this case, the technology fits neither the type nor the scope of manufacturing," explained David J Mazzo.

NEW GENE THERAPY FOR CYSTIC FIBROSIS TO BE DEVELOPED BY PFIZER & UNIVERSITY OF IOWA

The University of Iowa (UI) Research foundation has entered into a license and sponsored research agreement with Pfizer to support the development of potential gene therapies for cystic fibrosis (CF).

The research will be carried out in the laboratories of Prof. John Engelhardt and Prof. Ziying Yan, experts in gene therapy research with 25 years of experience in the field. The groups will collaborate with Pfizer's Genetic Medicines Institute, led by Michael Linden, to develop the CF gene therapy. CF is caused by a mutation in a single gene – the hope therefore is that by delivering a functional copy of the defective gene through gene therapydisease can be corrected.

"Efficiency of delivery is key to any gene therapy approach. The transport vehicle we have developed is an engineered virus that is highly efficient for entering human airway cells. Additionally, the 'truck' we use for transportation can carry a larger payload, which is especially important for cystic fibrosis since the diseased gene is very large," explains Prof. Engelhardt.

Researchers will use a delivery vehicle that is a hybrid of the AAV and the human bocavirus. This new viral vector was developed by Profs Engelhardt, Yan and bocavirus expert Prof. Jianming Qiu, from the University of Kansas. This engineered virus has the additional potential of having applications as a

vaccine against viral infections of the lung.

The teams at UI will focus their efforts on testing the efficacy of the vector in CF model systems, whilst Pfizer will develop packaging systems for the vector and will work to optimize the manufacturing process.

"This collaboration with Pfizer brings unique strengths that could enable the translation of this new vector system to clinical trials for CF lung disease and the discovery of potential new gene therapy applications for the vector system" - Prof. Engelhardt.

The announcement that Pfizer and the University of Iowa are intending to collaborate on a new gene therapy for the treatment of cystic fibrosis (CF) is interesting given the problems that many groups have had in the past in trying to develop a gene based treatment for this disease. However, the viral delivery system that is being developed, using a combination of an AAV vector and the human Bocavirus as a hybrid vector, theoretically does make sense. The Bocavirus has not really been utilised for gene therapy delivery being a parvovirus that is a probable cause of respiratory and gastrointestinal infections in humans. However, considering that the gene defect in CF causes both problems in both the lung and the gut in patients, the utilisation of a viral vector that normally attacks these bodily systems could be used to good effect. It will be interesting to see what the anticipated route of administration turns out to be used – so will it be systemic or inhalational and which would you choose? - *Alan Boyd*

SPARK THERAPEUTICS ACQUIRES GENABLE TECHNOLOGIES

Spark Therapeutics has acquired Ireland-based Genable Technologies, a private gene therapy innovator with which Spark has collaborated since 2014 in the development of Genable's therapeutic program targeting one of the most prevalent forms of inherited retinal disease.

The acquisition brings RhoNova™ into Spark's portfolio – a potential treatment targeting rhodopsin-linked autosomal dominant retinitis pigmentosa (RHO-adRP), an disease that routinely leads to visual impairment and in the most severe cases to blindness. Using an AAV vector developed and manufactured at Spark, RhoNova™ is designed to both suppress the expression of a faulty gene and deliver normal copies of the *RHO* gene to restore normal expression. RhoNova™ has been granted Orphan Drug Designation in both the US and Europe in addition to the Advanced Therapy Medicinal Product designation from the European Medicines Agency. There is currently no approved pharmacologic treatment for RHO-adRP, which affects an estimated 12,000 patients in the USA and the five major European markets.

"This transaction advances our strategy of leveraging Spark's best-in-class gene therapy platform through a combination of internal innovation, acquisition and collaboration," said

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Jeffrey D Marrazzo, Spark co-founder and chief executive officer. "Genable's technology and promising RhoNova™ development program further strengthens our portfolio of treatments for IRDs, which is led by our Phase 3 program for RPE65-mediated blindness which recently reported overwhelmingly positive pivotal stage data." outside of this initial indication." The consideration paid to Genable shareholders consisted of $6 million in cash and 265,000 shares of Spark common stock. Additional financial terms were not disclosed.

LION BIOTECHNOLOGIES APPOINTS NEW CHIEF MEDICAL OFFICER

Lion Biotechnologies has announced Steven A Fischkoff, MD, as CMO Dr Fischkoff brings over 25 years of biopharmaceutical experience, previously serving as Vice President of clinical and medical affairs at Celgene Cellular Therapeutics where he was responsible for overseeing the development of cell-based products as treatments for malignant and non-malignant diseases.

"Steven brings to Lion a deep knowledge of the immuno-oncology space, combined with a broad range of clinical, management and regulatory experience. We are very pleased to welcome him to our management team and look forward to his medical leadership as we continue to advance and expand our clinical pipeline." – Elma Hawkins, Lion's President and CEO.

KITE PHARMA EXPANDS COMMERCIAL LEADERSHIP TEAM

Kite Pharma has appointed three industry executives to be part of the company's integrated commercial leadership team. The new members of the team include: Diane Parks as Senior Vice President of Marketing, Sales & Market Research; Elizabeth Faust as Vice President of Medical Affairs; and Kimberly Metcalf, Vice President of Customer Engagement, Training & Development.

The team will be responsible for the company's commercial and medical affairs strategy when planning the potential launch of Kite's investigational T cell therapy, KTE-C19, for patients with relapsed and/or refractory B cell malignancies.

"As a group, they bring the range of critical skills and proven capabilities needed to prepare for commercialization. They have been closely involved in the launch of several major oncology therapies, and they understand how markets are reshaped by therapeutic innovations that improve patient outcomes. Our team is already fully engaged in market analysis and strategy development activities." - Shawn Tomasello, CCO at Kite.

ONCOSEC APPOINTS NEW HEAD OF CLINICAL DEVELOPMENT AND OPERATIONS

OncoSec Medical Inc. has appointed Sharron Gargosky, PhD, as Head of Clinical Development and Operations. Dr Gargosky brings over 20 years of experience in the field, having managed research programs from the early phases right through to the approval process by the FDA.

"Dr. Gargosky's deep expertise in clinical operations and development will significantly contribute to the strength of OncoSec's management team to move our

clinical programs forward, prioritize our product development strategy, and identify opportunities to leverage ImmunoPulse to address a significant unmet medical need in oncology."

- Punit Dhillon, President and CEO of OncoSec.

STEPHEN CARTT APPOINTED AS PRESIDENT AND CEO OF ASTERIAS BIOTHERAPEUTICS

Industry veteran, **Stephen L Cartt** has been announced as President and Chief Executive Officer of Asterias Biotherapeutics, Inc. In addition, Mr Cartt will serve as a member of the board of directors. Mr Cartt succeeds Pedro Lichtinger, who served as President, CEO and a member of the Board of Directors since June 2014.

Don Bailey was also announced as a new member of the board of directors. Mr Bailey, who previously served as President and Chief Executive Officer of Questcor Pharmaceuticals, will succeed Alfred Kingsley as Chairman.

"I look forward to working with the Asterias team, Don and the Board to further the company's clinical development of these important treatments. We believe that over time, these efforts have the potential to build significant returns for our shareholders," said Mr Cartt

TOCAGEN INC. APPOINTS NEW VICE PRESIDENT, REGULATORY AFFAIRS AND QUALITY ASSURANCE

Tocagen Inc., a clinical-stage, cancer-selective gene therapy company, have announced John Wood as Vice President of Regulatory Affairs and Quality Assurance. Mr Wood holds more than 20 years of regulatory affairs experience focusing on first-in-class drugs for diseases with high unmet need.

"John holds extensive experience in regulatory affairs activities, including overseeing drug approvals by the FDA and international regulatory agencies," – Harry Gruber, CEO of Tocagen.

VOYAGER THERAPEUTICS PROMOTES JEFF GOATER

Voyager Therapeutics, Inc., a clinical-stage gene therapy company developing treatments for diseases of the CNS, announced it has promoted **Jeff Goater**, former senior vice president of finance and business development, to chief financial officer. In this new role, Mr Goater will be responsible for overseeing the company's financial strategy and planning, investor and public relations, corporate operations and business development.

"Since the company's inception in 2014, Jeff has been a highly regarded member of the leadership team and this promotion is a reflection of his significant contributions to the company's growth, including Voyager's successful initial public offering in November 2015." – Steven Paul, President and CEO of Voyager.

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