Commercial insight: cell and gene therapy

JAN 2016

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: The cell therapy industry got off to quick start in January. Intrexon's expertise in synthetic DNA continues to make the company a popular partner in the cell and gene therapy space. In 2015, multiple companies partnered with Intrexon to incorporate the RheoSwitch technology into their respective platforms, which allows for temporal control over expression of cytokines and other protein elements. This trend should continue into 2016 as developers pursue cell therapies that are safer and more tightly regulated. To this end, Cellectis announced an article in *Scientific Reports* that describes the generation of a "switch-on" architecture, which allows for inducible expression of chimeric antigen receptors (CARs). This approach is an alternative, and potentially a replacement, for safeguard systems that utilize suicide genes to eradicate therapeutic cells in the case of cytokine storms. One key benefit of inducible CARs is that expression of a construct can be temporarily turned off by withdrawing an oral activator, whereas the use of suicide genes requires therapeutic cells to be wiped out in their entirety.

GENE THERAPY
Alan Boyd
CEO, Boyds, UK

CELL THERAPY
Mark Curtis
Bus. Dev. Analyst, CCRM, Canada

Rahul Sarugaser
VP, Corp. Dev., Hemostemix, Canada

GENE THERAPY: At the JP Morgan Healthcare Conference in early January, Jeff Marrazzo the CEO and Co-Founder of Spark Therapeutics, outlined his vision for the company over the next 3 years. The presentation was very detailed and laid out the plans for advancing 10 total programmes across three therapeutic areas by 2018. The plans are ambitious but the good thing is that the company has the funds to do this. This is probably the most detailed display of a company's gene therapy portfolio that most of us familiar within the space have seen from any company. It shows how far this treatment modality has now advanced and heralds the way for more regulatory filings and hopefully approvals. I think Spark has now shown us what the future may look like and that gene-based medicines are here to stay.

ADURO ANNOUNCES START OF PHASE I STUDY FOR TREATING LUNG CANCER

Aduro Biotech, Inc. has started a Phase I study of their LADD immune-oncology therapy, ADU-214 for the treatment of lung cancer. The study will assess the intravenous administration of ADU-214 in patients with advanced or metastatic non-small cell lung cancer. Up to 40 patients are expected to be enrolled, with around 12 participating in the dose-escalation portion of the trial. An additional 30 patients will be treated to further expand the trial to characterize safety and preliminary immunological and clinical activity.

"We are extremely pleased to see the first immuno-oncology therapy resulting from our license agreement with Janssen enter the clinic," said **Stephen T Isaacs**, Chairman, President and Chief Executive Officer of Aduro.

"With more than 200,000 new diagnoses this year and over 400,000 people living with lung cancer in the United States alone, new therapeutics are desperately needed. We believe ADU-214 may offer new hope to patients suffering from this aggressive disease."

PROMISING RESULTS FROM UNIQURE'S GENE THERAPY TRIAL

uniQure have presented new promising results from their gene therapy clinical trial for hemophilia B.

Encouraging data was presented from uniQure's low-dose cohort of an ongoing Phase I/II clinical trial in adult hemophilia B patients. As sufferers of hemophilia B do not produce sufficient amounts of factor IX, which is required for blood clotting, excessive bleeding often ensues. Current treatment involves repeated infusions of recombinant factor IX.

uniQure's AAV5/FIX gene therapy, AMT-060 consists of a codon-optimised wild type *FIX* gene

DOI: 10.18609/cgti.2016.001

and the LP1 liver promoter together with the AAV5 viral vector. The gene therapy is administered without immunosuppressant therapy.

Results using AMT-060 displayed results where four of the five patients were able to halt prophylactic recombinant factor IX. Of these, two patients' factor IX levels rose from 2%, before treatment, to 5.5% and 4.5% of normal levels.

"Thus far, the overall tolerability and FIX expression profile in the low-dose cohort is encouraging for patients with hemophilia B and support the continuation of the study." – **Professor Frank WG Leebeek**, an investigator in the study at the Erasmus Medical Center, Netherlands

"Today, we are the only AAV gene therapy company in the world with both proprietary, commercial-scale manufacturing capabilities and encouraging clinical data across multiple diseases," highlighted uniQure CEO Dan Soland.

UniQure has released the initial results from its hemophilia B gene therapy clinical study with AMT-060. Although the results are from the first 'low-dose' cohort of the study, they have shown that four out of the five patients treated have been able to discontinue prophylactic recombinant factor IX administration. The product itself has a codon-optimized wild-type *FIX* gene with an LP1 liver promotor in an AAV 5 vector. It is of interest that the product has been given without concomitant immunosuppression. It was also reported that AMT-060 was well tolerated by the patients. These results build on the results that were reported previously in 2011 and 2014 by Amit Nathwani, from Royal Free, London, in his publications in the *New England Journal* using the same *FIX* gene cassette, which UniQure licenced. The results from the rest of the study will be awaited with interest and in particular the durability of the response – but so far it's a good start. - ***Alan Boyd.***

ORPHAN DRUG DESIGNATION RECEIVED BY ADURO'S CRS-207 & GVAX PANCREAS IN THE EU

The European Medicines Agency has granted orphan drug designation for Aduro's CRS-207 and GVAX Pancreas for the treatment of pancreatic cancer.

Enrolment has been completed for the Phase 2b ECLIPSE trial for Aduro's novel LADD and GVAX immunotherapies, being developed for the treatment of metastatic pancreatic cancer. A total of 303 patients have been enrolled in the USA and Canada for the randomized, controlled three-arm trial.

ECLIPSE is designed to evaluate the safety, immune response and efficacy of the combination immunotherapy of CRS-207 and GVAX Pancreas compared to other chemotherapies. A treatment arm to evaluate CRS-207 as a monotherapy is also included as part of the trial. The primary endpoint of the trial is overall survival in the primary

cohort of patients who have received two or more prior therapies for metastatic disease. A second cohort of patients who received one prior therapy for metastatic disease is also being assessed.

"We are extremely pleased to receive Orphan Drug Designation in the EU for CRS-207 and GVAX pancreas, which, taken together with our Breakthrough Designation granted by the US Food and Drug Administration in the USA, represent important regulatory milestones in our global strategy to develop new immunotherapies for this underserved population," said **Stephen T Isaacs**, Chairman, President and Chief Executive Officer of Aduro.

SPARK UNVEILS PLANS TO HAVE 10 CLINICAL-STAGE GENE THERAPY PROGRAMS BY 2018

Spark Therapeutics Inc., outlined their vision, as part of the JP Morgan 34th Annual Healthcare Conference in San Francisco, USA, including the expansion of their portfolio through 2018 as well as the launch of their first commercial product in 2017.

A total of 10 programs will be advanced across three therapeutic franchises by 2018. This will include one commercially approved therapy, two product candidates in pivotal-stage trials and at least seven additional programs in clinical proof-of-concept trials. Treatments for two new indications were unveiled as part of the programs. These include Leber hereditary optic neuropathy and a program targeting Huntington's disease.

"The overwhelmingly positive data from our pivotal Phase 3 program for RPE65-mediated blindness, together with the multi-year durability data presented from the same program, provide strong validation of the Spark platform for AAV-based gene therapy that we are deploying across a large and growing pipeline of product candidates," said Spark Co-founder and Chief Executive Officer, **Jeffrey D Marrazzo.**

"Our results reflect the power of a true platform that combines proven capabilities across vector selection, design and manufacture, a history of collaborating with regulators to optimize clinical development and develop novel clinical endpoints, and our position at the forefront of shaping a patient-centric, commercial model for gene therapies. We are now leveraging this platform through internal innovation and commercialization, partnering and external collaboration to transform the treatment of a wide range of severe genetic diseases in three target tissues – the eye, the liver and the central nervous system."

DOI: 10.18609/cgti.2016.001

NEWLINK GENETICS CORPORATION COMPLETES PHASE 3 ENROLMENT

NewLink Genetics Corporation has completed enrolment for the Phase 3 PILLAR (Pancreatic Immunotherapy with algenpantucel-L for Locally Advanced non-Resectable cancer) clinical trial of algenpantucel-L for patients with borderline resectable or locally advanced unresectable pancreatic cancer.

"I am delighted to be part of this important study, with the potential for helping patients with pancreatic cancer in clear need of pioneering immunotherapies like algenpantucel-L," – **Harish Lavu**, Associate Professor of Surgery at Thomas Jefferson University, USA and a lead investigator for PILLAR.

"With the addition of our trial of indoximod, one of our IDO pathway inhibitors, in combination with chemotherapy for patients with metastatic pancreatic cancer, we now have clinical trials in all stages of this disease for which patients have very limited treatment options." – **Charles Link Jr,** Chairman and CEO of NewLink Genetics.

ANNAPURNA ADDS NEW GENE THERAPY PROGRAMS

After rounding up the $12 million Series A, adenovirus vector pioneer, gene therapy company Annapurna will look to deliver three new gene therapy programs that will hopefully move into the clinic through a partnership with Weill Cornell Medicine, USA. Under a series of licensing agreements, Annapurna will advance several gene-therapy programs already initiated at Weill's Department of Genetic Medicine.

Targets include an IND-level program for Alpha1-antitrypsin (A1AT) deficiency, a genetic condition that destroys lung tissue. Hereditary angioedema is next in line, with interesting animal data supporting efforts to develop one-time cures for severe allergies.

The collaboration is headed up by **Dr Ron Crystal,** Chairman of Genetic Medicine at Weill, who has extensive experience of the highs and lows endured by the field.

"With Annapurna, we intend to advance promising discoveries made in our laboratories at Weill Cornell Medicine into clinical studies," said Crystal. "Both sides understand the urgent need to make the promise of gene therapy a reality for patients needing better treatment options. It's not dissimilar from monoclonal antibodies – gene therapy needed a big learning curve."

"It's not dissimilar from monoclonal antibodies – gene therapy needed a big learning curve." Ron Crystal, Chairman of Genetic Medicine, Weill Cornell, USA.

ADAPTIMMUNE EVALUATE AFFINITY ENHANCED T-CELL THERAPY IN LUNG CANCER

Adaptimmune has initiated a Phase I/II study of its affinity enhanced T-cell therapy targeting MAGE-A10 cancer antigen in patients with locally advanced or metastatic (Stage IIIb or IV) non-small cell lung cancer. The MAGE-A10 antigen is expressed in a number of solid tumor cell types, with the immunogenicity of the antigen having been established.

"The initiation of this study is an important step in our goal to identify and develop new T-cell-based immunotherapeutics to combat non-small cell lung cancer and other cancers, and we are excited to initiate clinical development of another of our promising affinity enhanced TCR therapeutic candidates," said **Dr Rafael Amado**, Adaptimmune's Chief Medical Officer.

This is an open label, 3+3 dose escalation study of autologous T-cells genetically engineered with an affinity optimized MAGE-A10 TCR in patients with stage IIIb or stage IV NSCLC expressing the MAGE-A10 antigen. The study will enrol up to 32 patients in leading clinical centres located in the United States and Europe and will assess the safety and tolerability of Adaptimmune's affinity enhanced T-cell therapy targeting MAGE-A10.

PLURISTEM REACHES AGREEMENT WITH JAPAN'S PMDA

Pluristem Therapeutics Inc. has received approval from Japan's Pharmaceuticals and Medical Devices Agency (PMDA) for the design of the final trial needed to apply for conditional approval of PLX-PAD cells in the treatment of critical limb ischemia (CLI).

"With this achievement we have advanced our strategy to expedite commercialization of our cell products. Pluristem is now positioned favorably to accelerate negotiations with those Japanese pharma companies interested in becoming dominant players in the expanding regenerative medicine market in Japan," stated Pluristem chairman and CEO Zami Aberman.

Data will be collected from 75 patients suffering with CLI, with the study having a primary endpoint of a patient being CLI free for 60 days. The patients will be sorted into 3 random groups of 25. Group one will receive an initial 150 million PLX-PAD cell dose with a second 150 million cell dose eight weeks later. Group two will be treated with an initial 300 million PLX-PAD cells, followed eight weeks later by a second dose of 300 million cells. Group three will receive two placebo doses.

DOI: 10.18609/cgti.2016.001

FDA CLEARS FATE THERAPEUTICS' NEW DRUG APPLICATION FOR PROTMUNE

The US Food and Drug Administration has approved Fate Therapeutics' investigational new drug (IND) application for ProTmune as a means to prevent acute graft-versus-host disease (GvHD) and cytomegalovirus (CMV) infection. These are the leading causes of morbidity and mortality in patients undergoing a hematopoietic stem cell transplantation (HSCT). ProTmune is a programmed cellular immunotherapy consisting of donor-sourced peripheral blood cells which have been functionally modulated using two small molecules.

Fate Therapeutics plans to initiate a multi-center, randomized, controlled Phase 1/2 clinical trial in adult patients with hematologic malignancies undergoing mobilized peripheral blood HCT. The primary endpoint of the trial will be to ascertain the safety and tolerability, and to assess the potential of ProTmune to prevent acute GvHD and CMV infection.

"We believe the use of ProTmune as the donor cell source for HCT can meaningfully improve patient outcomes, decrease hospital length of stay by mitigating use of in-hospital drug treatments, and substantially reduce the overall cost of care." – Scott Wolchko, President and Chief Executive Officer of Fate Therapeutics.

EXPERT PICK

Fate Therapeutics continues to be a leader in the identificationofmoleculesfor programming cell behavior and function. The company recently brought ProTmune into the clinic, a programmed, mobilized peripheral blood product used to reconstitute the bone marrow compartment following ablation with chemotherapy. Mobilizing a donor to collect stem cells from the peripheral blood is less invasive than aspirating stem cells from the bone marrow, so peripheral blood is becoming a preferred source, however it is associated with increased graft-versus-host disease. ProTmune circumvents this barrier by programming T cells in a donor sample to promote immune tolerance. - ***Mark Curtis & Rahul Sarugaser***

CELLECTIS FILES FIRST CLINICAL TRIAL APPLICATION FOR UCART19

Cellectis has submitted a clinical trial application to the Medicines & Healthcare products Regulatory Agency (MHRA) to initiate a first-in-human clinical investigation for UCART19 in leukemia in the UK. The study will aim to include CD19-positive Acute Lymphoblastic Leukemia patients.

"It has been a privilege preparing this application with our team, partners, investigators and subcontractors, in close interaction with MHRA, rewarding many years of intense work to overcome the challenges that are inherent to advanced therapy medicinal products. This achievement marks an important step toward making UCART19 available to patients," – **Stephan Reynier**, Chief Regulatory and Compliance Officer, Cellectis.

FDA GRANTS ORPHAN DRUG DESIGNATION TO PLURISTEM'S PLX-PAD CELLS

Pluristem Therapeutics, Inc. have had their PLX-PAD cells granted Orphan Drug Designation by the FDA for treating severe preeclampsia. Occurring in approximately 1% of Western pregnancies, preeclampsia is one of the most common medical complications of pregnancies and is a leading cause of premature births, stillbirths, neonatal and maternal deaths.

PLX-PAD cells were able to improve several parameters of preeclampsia in animal models. Additional studies demonstrate that PLX-PAD cells are safe for both the mother and the fetus.

"Attainment of Orphan Drug Designation for our cells in severe preeclampsia exemplifies our global strategy of bringing cell therapies to patients through accelerated approval pathways. We are encouraged by the US FDA designation that demonstrates Pluristem's commitment to the program and the potential promise it holds to address a serious, unmet medical need faced by pregnant women every year." – **Zami Aberman**, chairman and CEO of Pluristem.

COLLABORATION BETWEEN TAKEDA AND ENGENE

A new collaboration between Japan's Takeda and Montreal's enGene will make use of enGene's Gene Pill delivery platform to develop gene therapies for gastrointestinal diseases. The technology has to ability to deliver either DNA or RNAi to mucosal tissues to induce or suppress protein expression, for a given disease, whilst avoiding the efficiency issues and side effects brought about when administered through the bloodstream.

DOI: 10.18609/cgti.2016.001

Under the terms of the agreement with Takeda, enGene will work on two undisclosed targets where Takeda will ultimately decide to license the candidates or not. EnGene will receive an upfront payment and reimbursement of all R&D costs. In addition, enGene will be eligible to earn milestone payments for the product candidates based on specific research, clinical, regulatory and commercial milestones. The two companies will also be working on using the Gene Pill technology to delivery antibodies orally where Takeda will have the exclusive option to obtain a right of first negotiation for up to three antibody targets.

"We are very excited to be collaborating with the team at Takeda to investigate new medicines for the specialty GI market using enGene's innovative gene delivery platform for the gut," said enGene CEO **Anthony Cheung**. "...this alliance provides our company an excellent opportunity to grow our drug development capabilities through working with a leading pharmaceutical company in the gastroenterology space."

GENE DELIVERY VECTOR MAKER RECEIVES INVESTMENT FROM PFIZER

4D Molecular Therapeutics, an emerging biopharmaceutical company, is one of four companies receiving part of a $46 million investment from Pfizer as part of their drug discovery efforts. Specializing in the development of gene delivery vectors, 4D Therapeutics will pass on the rights to the Big Pharma to exclusively licence at least one adeno-associated virus for cardiac disease targets.

Through the use of their Directed Vector Evolution platform, 4D Therapeutics claims to be able to create a library of 100 million gene vectors and, over the course of a year, find the most promising variant of a particular payload's delivery. These gene vectors are optimized for efficient gene delivery and tissue specificity such that gene uptake can be facilitated in a variety of organs including the eye, lung and brain. Pfizer claims that these vectors also have the ability to evade antibodies that may potentially neutralize the payload.

"There is exciting scientific discovery happening both within Pfizer and beyond our walls, and we look forward to...investigating areas where we feel we could make a difference for patients," said **Dr Mikael Dolsten,** President of Pfizer Worldwide R&D.

BIG PHARMA TAKING NOTICE

For many years in the gene therapy space 'Big Pharma' have stayed clear of getting involved but now that significant results are coming through and products are starting to achieve regulatory approval, the interest of Big Pharma is growing. This is shown by two developments this month namely the agreements between Takeda and enGene and Pfizer and 4D Molecular Therapeutics. Both these announcements relate to early stage developments but I think they show a step in the right direction. With the fall in the Stock Market value of many of the gene therapy companies listed in the USA over recent weeks, Big Pharma may now be considering that it is a good time to take the next step and acquire some companies – only time will tell. ***Alan Boyd***

ADURO RECEIVES MILESTONE PAYMENT FROM JANSSEN

Payment to Aduro from Janssen Biotech, Inc. comes from the acceptance of an IND application by the FDA for ADU-741, a LADD immunotherapy for the treatment of prostate cancer.

Aduro entered into the agreement with Janssen in May 2014, granting Janssen an exclusive, worldwide license to certain product candidates specifically engineered for the treatment of prostate cancer. Aduro received an upfront payment and milestone payment associated with both the submission and the acceptance of the IND. Aduro is also eligible to receive future development, regulatory and commercialization milestone payments up to a potential total of $345.5 million.

"We are extremely pleased with the progress Janssen has made with the development of ADU-741," said Stephen T Isaacs, Chairman, President and CEO of Aduro.

Janssen plans to initiate a multi-center Phase 1 clinical trial to determine the safety and immunogenicity of ADU-741.

CLINICAL & PRECLINICAL RESEARCH COLLABORATION FOR LION BIOTECH & MEDIMMUNE

Cancer immunotherapy specialists, Lion Biotechnologies have entered into a collaboration with MedImmune to conduct clinical and preclinical research in immuno-oncology.

Lion will fund and conduct two Phase 2a clinical trials that will combine MedImmune's investigational PD-L1 inhibitor durvalumab with tumor-infiltrating lymphocytes (TIL) for the treatment of patients with metastatic melanoma, and head and neck cancer.

The preclinical research, funded by MedImmune and conducted by Lion, will focus on identifying and evaluating effective therapeutic combinations of MedImmune's checkpoint antibodies, using TIL as an *in vitro* model of the tumor microenvironment.

"In preclinical studies, TIL and anti-PD-1/PD-L1 combination regimens have demonstrated significant anti-tumor activity, suggesting a synergistic effect. We look forward to working with MedImmune to further develop this approach and other combination therapies that we believe have the potential to meaningfully improve outcomes for patients." – Elma Hawkins, PhD, Lion's president and CEO.

DOI: 10.18609/cgti.2016.001

COLLABORATION BETWEEN FIBROCELL &INTREXON

Cell and gene therapy company, Fibrocell Science, Inc. has entered into an Exclusive Channel Collaboration with Intrexon Corporation, a synthetic biology company. This will lead to the development of genetically modified fibroblasts as a means of treating chronic inflammatory and degenerative diseases of the joints.

Intrexon's cellular engineering capabilities will be combined with Fibrocell's proprietary fibroblast platform to produce cell-based therapeutics that have been altered such that they express one or more proteins at the sites of joint inflammation. In turn, this should help overcome current treatment approaches for chronic inflammatory and degenerative diseases of the joint.

Under the terms of the agreement, Intrexon will receive a technology access fee of $10 million and up to $30 million and $22.5 million will be provided as regulatory and commercial milestone payments.

KITE PHARMA EXPANDS CLINICAL & RESEARCH PARTNERSHIP WITH THE INTL CANCER INSTITUTE

Kite Pharma has entered into a Cooperative Research and Development Agreement (CRADA) with the National Cancer Institute for the research and development of fully human anti-CD19 CAR products against B-cell lymphomas and leukemias. The agreement will also focus on the development of next-generation CAR programs directed against other novel antigens for the treatment of B-cell lymphomas and leukemias.

"The new CRADA is a natural step in our life cycle management strategy to further improve the success of CAR therapy for patients with advanced B-cell malignancies," said Arie Belldegrun, Chairman, President, and CEO of Kite Pharma.

EXCLUSIVE LICENSE AGREEMENT BETWEEN MEMORIAL SLOAN KETTERING & JUNO

Memorial Sloan Kettering (MSK) Cancer Center and Juno Therapeutics, Inc. have entered in an exclusive license agreement. The agreement will make use of a novel, fully-human MUC16 binder for the development of CAR cell therapies for patients with cancer that are MUC16 positive. The binder was developed by Eureka Therapeutics and MSK Cancer Center, under a collaborative agreement.

"CAR cell therapy has shown promising potential for treating hematologic malignancies, and Eureka is working to accelerate the potential

of the technology in a broader array of patients by developing antibodies that can recognize solid tumor antigens with the stringency required for CAR immunotherapy" – Dr. Cheng Liu, President and CEO off Eureka Therapeutics.

MSK granted Juno Therapeutics an exclusive, worldwide license to develop and commercialize the MUC16 binding domains as part of CAR cell therapies. They will receive an upfront payment and potential future payments upon achievement of certain development, regulatory, and sales milestones, and annual net sales royalty payments.

SPONSORED RESEARCH AGREEMENT: IMMUNOCELLULAR THERAPEUTICS & UNIVERSITY OF MARYLAND

ImmunoCellular Therapeutics has entered into a sponsored research agreement with Eduardo Davila, associate professor of microbiology and immunology at the University Of Maryland School Of Medicine, and the University of Maryland, Baltimore. The agreement will cover three projects.

The first project will evaluate immune modulators that could potentially enhance T-cell killing of tumor cells. These small molecule modulators have been shown increase tumor antigen expression while simultaneously decreasing expression of ligands, such as PD-L1 and PDL2, which decrease T-cell activity.

A second project will explore the combination of engineered killer T-cells and dendritic cell immunotherapies on tumor killing. The combination of these engineered T-cells with ImmunoCellular's dendritic cell immunotherapies has the potential to enhance tumor cell killing.

UNIVERSITY COLLABORATIONS CONTINUE TO DRIVE INNOVATION

The cell therapy industry is growing ever more intertwined with the academic community as access to discovery platforms and niche scientific expertise have become absolutely essential to advancing targets and therapeutics into the clinic (and to remain in the race in what is a very aggressive competitive landscape). This past month was a testament to this with a multitude of research collaborations announced between immunotherapy companies and universities in both the United States and Europe. ImmunoCellular Therapeutics, in particular, has leveraged early-stage partnerships to invigorate its pipeline, including the addition of a Stem-to-T-Cell program in collaboration with the MD Anderson Cancer Center, last year, and the recent announcement of a collaboration with the University of Maryland to pursue several enhancements of its immunotherapy platforms, including molecules for increasing T cell cytotoxicity via programming, and novel peptide configurations for improving induction of T cell response from dendritic cell vaccines. - ***Mark Curtis & Rahul Sarugaser***

DOI: 10.18609/cgti.2016.001

Finally, the third project will test novel peptide configurations for use with dendritic cell immunotherapies to potentially induce enhanced T-cell responses. "ImmunoCellular's research and early development strategy is to complement and enhance our dendritic cell and Stem-to-T-cell technology platforms and create potent cell-based cancer immunotherapeutic clinical candidates and combinations," – Steven Swanson, ImmunoCellular Senior Vice President, Research.

EXCLUSIVE PARTNERSHIP BETWEEN SORRENTO & KAROLINSKA INSTITUTE

Sorrento Therapeutics, Inc. has formed an exclusive partnership with the Karolinska Institutet (KI), Stockholm, Sweden, to carry out immuno-oncology research and to develop new natural killer cell-based therapies.

"Through this partnership, Sorrento further establishes its subsidiary, TNK Therapeutics, as one of the premier companies in the cellular therapy space. Building upon the academic and clinical excellence at KI and Sorrento's expertise in antibody research and development, our partnership will stimulate innovation and may ultimately lead to new ground breaking therapies to improve the lives of cancer patients and their caretakers." – Dr Henry Ji, President and CEO of Sorrento

Sorrento will sponsor preclinical and clinical research and development programs focused on natural killer cell biology and adoptive cell therapies. Sorrento will obtain full rights for the resulting developments. The research will be carried out at the Karolinska Institute.

NEW ADDITIONS TO ADAPTIMMUNE'S MANUFACTURING SENIOR MANAGEMENT

In preparation for their clinical and commercial scale up, Adaptimmune has appointed **William Buecheler** as Vice President, Manufacturing Operations, and **Phil Bassett** as Head of Process Development. Mr Buecheler will be responsible for Adaptimmune's global manufacturing operations and will lead the implementation of scale up plans. Mr Bassett will be in charge of process development activities.

Mr Buecheler brings over 25 years of industry experience and Mr Bassett will be bringing over 15 years of process manufacturing experience in both small biotech and large biopharma environments.

"Adaptimmune is in the midst of scaling up its manufacturing expertise, which significantly enhances our ability to run multiple clinical studies of our TCR therapies and, eventually, commercialize our products. Manufacturing excellence is key to our long term success. Hiring the best in the field of manufacturing is critical to us, and Bill and Phil are already making significant contributions." said James Noble, CEO at Adaptimmune.

NEW CHIEF COMMERCIAL OFFICER APPOINTED AT KITE

Shawn Cline Tomasello has been appointed as Chief Commercial Officer at Kite Pharma. Ms. Tomasello has over 30 years of experience within the life sciences industry, having led commercial and medical affairs efforts at Pharmacyclics and commercial efforts at Celgene and Genentech.

"Shawn's tremendous record of accomplishment, including her leadership of three of the most successful programs in hematology-oncology, will be invaluable as we advance our lead product candidate, KTE-C19, in the clinic and prepare for commercialization. We are thrilled that she is joining our team at this important time for Kite." – Arie Belldegrun, Chairman, President, and CEO at Kite Pharma.

CELLECTIS ANNOUNCES NEW CHIEF MEDICAL OFFICER

Dr Loan Hoang-Sayag has been appointed the role of Chief Medical Officer at Cellectis. Dr Hoang-Sayag is a physician in hematology and medical oncology with 23 years of experience, previously serving as Senior Director of Medical Science at Quintiles. She will be responsible for bringing the company's product candidates into clinical stage development in addition to strategic and operational management of all therapeutic activities for Cellectis.

"Dr Hoang-Sayag's experience in oncology clinical development – specifically in strategy and delivery in all phases in the pharmaceutical, biotechnology and clinical research spaces – enables her to lead Cellectis' strategy and generate awareness around the breakthrough work that we are doing as a leader and innovator in the field," – Dr André Choulika, Chairman and CEO of Cellectis.

Written by Mudith Jayawardena, Cell and Gene Therapy Insights

DOI: 10.18609/cgti.2016.001