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.

GENE THERAPY
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GENE THERAPY: This month illustrates the challenges faced by companies developing and commercializing gene therapy products, with uniQure announcing workforce cuts and pipeline prioritization. Nevertheless, the field continues to march forwards with further exciting developments in CRISPR-Cas9, with the announcement of the first clinical trial using this technology in patients with metastatic non-small-cell lung cancer. There is also positive news from AveXis, who have announced that the US Food and Drug Administration (FDA) has accepted the use of natural history data for comparison in its pivotal single arm trial in SMA Type 1. The FDA is notoriously resistant to the use of natural history data, so this is a real achievement!

CELL THERAPY: Atlas Venture and Third Rock launched Magenta Therapeutics with a $50 million series A this past month, perhaps the culmination of a period of renewed interest and activity among industry in the area of stem cell transplant. The company's mission will be to bring transplant to the masses. While bone marrow transplant has been practiced for more

than 50 years, generally it has only been used in instances where the benefit of a cure outweighs the risk of GVHD, which is often fatal. It has predominantly been used as a cure for leukemias and lymphomas. However, transplant has great potential beyond oncology, and many multiples of patients with rare blood disorders and severe autoimmune disorders stand to benefit from a world of transplant without GVHD. Magenta will seek to develop technologies in three areas: patient conditioning, stem cell mobilization and stem cell expansion/modulation; all areas of critical significance to the advancement of transplant, and factors that can help reduce patient mortality. Removing GVHD from the picture with improved matching could make it possible to safely 'reset' a patient's immune system, which means the next generation of transplant technology could be a real threat to *in vivo* gene therapy in diseases such as Type 1 diabetes, multiple sclerosis, sickle cell anemia and Crohn's disease.

AGILIS' GENE THERAPY QUALIFIES FOR FDA'S PRIORITY REVIEW

The FDA has granted Rare Pediatric Disease designation to Agilis' gene therapy candidate, AGIL-AADC, for the treatment of aromatic L-amino acid decarboxylase (AADC) deficiency. The Rare Pediatric Disease (RPD) designation supplements the Orphan Drug Designation granted to AGIL-AADC by the FDA earlier this year. These designations will give Agilis access to the FDA's priority review pathway and the ability to qualify for a Priority Review Voucher (PRV) upon marketing approval of AGIL-AADC.

Agilis Biotherapeutics is a US-based biotechnology company specialized in the development of gene therapies for rare genetic diseases of the central nervous system. Its rare disease programs include gene therapy for AADC deficiency, Friedreich's ataxia and Angelman syndrome.

AADC deficiency is a rare genetic condition caused by mutations in the dopa decarboxylase gene, *AADC*, which catalyzes the final step in the synthesis of the neurotransmitters, dopamine and serotonin. AGIL-AADC is an adeno-associated virus vector containing the human gene for the AADC enzyme.

Mark Pykett, CEO of Agilis, commented: "We are pleased that FDA granted our request for rare pediatric designation for our gene therapy candidate for AADC deficiency. The Rare Pediatric Disease and Orphan Drug designations provide significant incentives in seeking marketing authorization in the USA that support our business strategies moving forward. RPD designation enables the potential award of a Priority Review Voucher, if the marketing application for AGIL-AADC is approved."

In additional news this month, the company has announced that the Taiwan Food and Drug Administration and the ethics committee at the National Taiwan University have authorized its Phase 2b clinical trial for the treatment of AADC deficiency.

The trial will be performed under the direction of Professor Paul

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Hwu at NTU Hospital. Dr Hwu and colleagues have treated 18 subjects to date in two prospective clinical studies using a single administration of the gene therapy. The present Phase 2b study will enroll a third cohort of patients into two parts, one evaluating the AGIL-AADC gene therapy dose used in prior studies and a second exploring single administration of an increased dose.

The granting of Rare Pediatric Disease designation to Agilis' gene therapy treatment for AADC deficiency could prove valuable for the company, if it is later successful in an application for a Priority Review Voucher (PRV). While such a designation is not required to receive a voucher, requesting this in advance will, according to FDA, expedite a sponsor's future request for a PRV. These vouchers, once granted, can be bought and sold and have traded for large sums of money. In August 2015, AbbVie paid a record-breaking $350 million for a voucher originally awarded to United Therapeutics, although at present the voucher remains unused. – *Richard Philipson*

PROMETHERA'S HEPASTEM SHOWS BENEFICIAL EFFECT IN MICE WITH FATTY LIVER DISEASE

Promethera Biosciences, a Belgium-based clinical-stage biotechnology company specialized in the development of cell therapies for liver diseases, has announced preclinical data of its proprietary HepaStem program for the treatment of nonalcoholic steatohepatitis (NASH).

Data presented by Promethera at the *67th Annual Meeting of the American Association for the Study of Liver Disease* in Boston showed the beneficial effect of HepaStem in reducing liver inflammation and improving fibrosis symptoms in a NASH mouse model.

Promethera's HepaStem consists of human adult liver-derived mesenchymal stem cells. A Phase 1/2 study conducted in 20 pediatric patients suffering from inherited metabolic disorders showed that the treatment was safe and well tolerated.

NASH is a type of nonalcoholic fatty liver disease (NAFLD) and is a manifestation of the metabolic syndrome and hepatic disorders with the presence of steatosis, hepatocyte injury and inflammation. In this preclinical study, HepaStem was administered with or without immunosuppression to a NASH mouse model. HepaStem significantly reduced inflammation, NAFLD activity score and fibrotic collagen deposition in liver sections at 9 weeks of age. These cells were also shown to secrete high levels of hepatocyte growth factor, which is important for halting liver fibrosis development. Co-culturing of HepaStem cells with T-lymphocytes or dendritic cells decreased the activation of both these cell types *in vitro*, proving the immunomodulatory effect of HepaStem cells.

CHINESE SCIENTISTS BECOME FIRST TO TEST CRISPR IN HUMANS, AS 'SPUTNIK 2.0' BEGINS

Chinese scientists, led by oncologist Lu You at Sichuan University in Chengdu, have injected modified cells using CRISPR into a patient with a form of lung cancer at the West China Hospital. This follows on from China being the first in using CRISPR-edited human embryos, as well as CRISPR-edited monkeys. The team from China has reached this important clinical milestone ahead of T-cell biology specialist Dr Carl June and a series of major US medical academic centers funded by the billionaire co-founder of Napster Sean Parker.

Speaking to *Nature*, Dr June said: "I think this is going to trigger 'Sputnik 2.0', a biomedical duel on progress between China and the USA, which is important since competition usually improves the end product." He now eyes his own team's clinical trial to start early next year, he told the journal.

The Chinese team plans to treat ten people in all with a series of injections, and the primary focus is on safety, given how early-stage the research is. There are long-running concerns about whether this gene-editing tech could produce some severe side effects (notably, an unwanted autoimmune response), so showing safety will be paramount in these early days.

Each group is using CRISPR in slightly different ways, but the fundamental aspect sees the tech used to edit genes by leveraging an RNA guide molecule to enter in specific cells. A protein called Cas9 then attaches to the DNA and essentially cuts it, all of which either gets rid of, completely removes or replaces a gene with a better strand of DNA. It could be used in a host of diseases, but oncology appears to be the favored early target.

According to the report in *Nature*, the team removed immune cells from the recipient's blood and then disabled a gene in them using CRISPR-Cas9. Lu's team then cultured the edited cells, increasing their number, and injected them back into the patient, who has metastatic non-small-cell lung cancer. The hope is that, without PD-1, the edited cells will attack and kill the cancer.

ONES TO WATCH

The announcement of the start of the first clinical trial using CRISPR-Cas9 technology is an important milestone for this approach, and marks a victory for Chinese scientists, led by oncologist Lu You at Sichuan University in Chengdu, over their US competitors. There have been long-running concerns about whether this gene-editing technology could produce some severe side effects (notably, an unwanted autoimmune response), so showing safety will be paramount in these first patients treated. Furthermore, there are lingering questions over who owns the patents to these technologies, likely to be the subject of further court battles. -Richard Philipson

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JUNO HALTS ITS PHASE 2 CAR-T TRIAL A SECOND TIME

Juno Therapeutics, a Seattle-based biopharmaceutical company specialized in the development of cell-based cancer immunotherapies, has announced that it has voluntarily halted its Phase 2 CAR-T trial following two patient deaths. Both of these deaths were due to cerebral edema.

Juno's Phase 2 ROCKET trial was designed to evaluate the safety and efficacy of JCAR015 for the treatment of relapsed or refractory B-cell Acute Lymphoblastic Leukemia (B-ALL). JCAR015, the investigational product candidate, uses genetically modified autologous T cells to eliminate leukemia cells. The infused T cells express a chimeric antigen receptor (CAR) that binds leukemia cells, which express the CD19 protein on the cell surface and initiate a cell-killing response against the cancer cell. Patients receiving CAR-T therapies received doses of chemotherapy beforehand to make the tumor more vulnerable to the CAR-T cells.

Earlier this year, we had reported the news about FDA's decision to halt Juno's trial after three patient deaths. Juno had strongly suspected that these deaths occurred due to the chemotherapy agent fludarabine. This clinical hold was later removed by FDA after Juno amended the protocol and decided to use cyclophosphamide as the pre-conditioning agent instead of fludarabine. They believed that this amendment would stop future deaths. However, today's news has proved that fludarabine is not the only culprit.

Juno share fell by nearly 30% in after-hours trading, while rivals Kite Pharma and bluebird bio dropped by 4 and 5%, respectively. Juno has notified the FDA of the voluntary hold and is working with the agency and the Data and Safety Monitoring Board to assess data and determine next steps.

AVEXIS ANNOUNCES SINGLE-ARM DESIGN FOR US PIVOTAL STUDY OF AVXS-101 IN SMA PATIENTS

AveXis, a clinical-stage gene therapy company specialized in the development of treatments for orphan and life-threatening neurological diseases, has announced a single-arm design for its US pivotal study of AVXS-101, for the treatment of spinal muscular atrophy (SMA).

AVXS-101 is a gene therapy candidate developed for the one-time treatment of SMA type 1 and is the only gene therapy in development for SMA. The trial will use natural history of the disease as a comparator and will enroll approximately 20 patients. This update follows AveXis' meeting with the FDA in September.

In addition to evaluating safety, the planned program is expected to evaluate achievement of motor milestones, specifically patients'

ability to sit unassisted, as well as an efficacy measure defined by the time from birth to an 'event', defined as death or requiring at least 16 hours per day of ventilation support for breathing for greater than 2 weeks in the absence of an acute reversible illness.

Sean Nolan, President and CEO of AveXis, commented: "We believe the Type B meeting had a positive tone, with the FDA offering a number of constructive suggestions, which we believe will better enable implementation of a pivotal study design that is most appropriate for the patients suffering from this devastating disease. With the feedback needed from the FDA to move forward with our pivotal trial, we plan to proceed as expeditiously as possible to begin the study in the first half of 2017."

The company's strategy with the SMA type 1 program is to complete the ongoing Phase 1 trial and, in parallel, execute on the single-arm pivotal trial, while continuing collaborative discussions with the FDA regarding the most expeditious pathways for FDA approval of AVXS-101.

OPEXA'S T-CELL THERAPY FAILS TO MEET EFFICACY END POINTS

Opexa Therapeutics, a Texas-based biopharmaceutical company specialized in the development of personalized immunotherapies for autoimmune disorders, has announced that its Phase 2b Abili-T trial of Tcelna® has not met the primary and secondary end points in patients with secondary progressive multiple sclerosis (SPMS).

Tcelna® consisted of an autologous pool of myelin reactive T cells expanded *ex vivo* with immunodominant epitopes selected from three myelin antigens. The cells were attenuated by irradiation to prevent further proliferation before releasing product for administration. Approximately 5 weeks after receipt of the subject's whole blood, the subjects received either Tcelna (30-45 x 106 cells in 2 ml) or placebo (Tcelna excipients) and received two annual courses of five subcutaneous doses each year (at 0, 4, 8, 12 and 24 weeks).

The Abili-T clinical trial was designed as a randomized, double-blind, placebo-controlled study to evaluate the safety and efficacy of Tcelna® in SPMS patients. The study included 183 patients and was conducted at 35 clinical trial sites in the US and Canada. The end points of the study included a reduction in brain volume change (primary) and a reduction in the rate of sustained disease progression (secondary). Although Tcelna showed a favorable safety and tolerability profile, it did not meet both the primary and secondary end points.

Neil K Warma, President and CEO of Opexa, commented: "We are disappointed that Tcelna did not meet the predefined endpoints in the Abili-T trial. We will evaluate the full data set over the coming weeks and review cash preservation options while we consider the best path forward for the company."

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CELYAD POSTS INITIAL DATA OF ITS NKG2D CAR T TRIAL

Celyad, a Belgium-based pharmaceutical company specialized in the development of engineered cell therapies, has posted preliminary results of its Phase 1 trial designed to evaluate chimeric-antigen receptor (CAR) T cells (CM-CS1 T cells) that recognize NKG2D-ligands on the surface of cancer cells.

The current Phase 1 dose-escalation study is designed to investigate the safety and feasibility of administering a single intravenous dose of CM-CS1 CAR T-cells in patients with acute myeloid leukemia (AML), advanced myelodysplastic syndrome (MDS-RAEB) and multiple myeloma. The trial also evaluates the persistence and function of the CM-CS1 T cells in the body.

In the trial, patients' T cells are collected and engineered such that these T cells can recognize NKG2D-ligands present on the surface of cancer cells. The modified CM-CS1 T cells are administered back to the patient by a single intravenous infusion. No chemotherapy was used prior to infusion of the CM-CS1 T cells. Data will be collected up to 24 months after infusion.

Preliminary data released by Celyad after 28 days of assessment shows that ten participants have completed this period without dose-limiting toxicities. During this period, no subjects experienced cytokine release syndrome, cell-related neurotoxicity, autoimmunity or CAR-T-related death. Two-fifths of participants suffered a toxicity of grade three or greater. Grade four adverse events included intracochlear bleed, neutropenia and thrombocytopenia. The company thinks these toxicities were related to disease progression and not its therapy. CAR T cells did not persist beyond 1 week and that is consistent with pre-clinical models.

Celyad is now aiming to get approval from the FDA and EMA to initiate its Phase 1/2a trial to evaluate the efficacy of these CAR T cells in seven types of solid and hematological cancers. Results from this study will indicate whether Celyad's CAR-T cells work.

UNIQURE CUTS WORKFORCE AND PRIORITIZE PIPELINE PROJECTS

uniQure, a leading biopharmaceutical company specialized in the development of gene therapies for severe genetic diseases, has announced the completion of a strategic review intended to refocus its pipeline while consolidating its manufacturing and reconstructing its R&D operations.

The Amsterdam-based company announced that it intends to cut 20–25% of its workforce – around 50–60 positions by the end of 2017. Through this, uniQure aims to generate €5–6 million in annual cost savings.

Through this initiative, the company aims to prioritize programs in

hemophilia B, Huntington's disease and cardiovascular disease. It will potentially discontinue licensing agreements with its collaborator for the treatment of Sanfilippo B, and will pursue partnering opportunities for its Parkinson's disease program. This pipeline refocusing is expected to reduce an additional expense of €11–15 million over the next 2 years.

uniQure also aims to restructure its R&D operation in Amsterdam while consolidating GMP manufacturing at its Lexington facility in the USA. Currently, the Lexington facility has the capability of scaling up to 2000L capacity – a capability that positions it for late-stage development and commercialization of its gene therapies. uniQure expects to reduce operating expenses and create a more efficient company focused on the successful and timely development of gene therapies for patients with serious unmet medical needs.

uniQure's share price had been volatile the past 12 months after it dropped plans to file FDA approval for Glybera (alipogene tiparvovec), a gene therapy for lipoprotein lipase deficiency that was launched onto the market in Europe in 2012.

Matthew Kapusta, interim CEO of uniQure, commented: "The strategic restructuring brings enhanced focus to our pipeline, streamlines operations and improves our financial strength. We are committed to the timely development of our programs in hemophilia B and Huntington's disease, as well as the support of our collaboration with BMS. Along with our investigators and collaboration partner, we are enthusiastic about the interim data from our ongoing Phase 1/2 study of AMT-060 in hemophilia B and will allocate necessary resources to expedite bringing AMT-060 to market, with commercial manufacturing capabilities in our state-of-the-art US facility, which is already in place."

ASTERIAS DOSES THIRD COHORT IN SCISTAR TRIAL

Asterias Biotherapeutics, a US-based clinical-stage biotechnology company, has dosed the first patient in the third cohort of its ongoing Phase 1/2a SCiSTAR trial, designed to evaluate the activity of escalating doses of AST-OPC1 (oligodendrocyte progenitor cells) in complete cervical spinal cord injury patients.

The trial included three cohorts: an initial cohort of three patients who received 2 million AST-OPC1 cells and a second cohort of five patients who received 10 million AST-OPC1 cells. The third set of patients will receive the highest dose of 20 million cells.

Dose escalation procedure to 20 million cells was initiated after the company received safety clearance from its Data Monitoring Committee. This approval was based on AST-OPC1's continued favorable safety profile in its first and second cohort of patients.

The SCiSTAR study is partly funded by a $14.3 million grant from the California Institute of Regenerative Medicine. AST-OPC1 is derived from human embryonic

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stem cells and *in vitro* and preclinical studies have shown its efficacy in improving the pathologies associated with spinal cord injury.

Dr Edward Wirth, CMO of Asterias, commented: "We have been very encouraged by the early clinical efficacy and safety data for AST-OPC1, and we now look forward to evaluating the 20 million cell dose in complete cervical spinal cord injury patients. Based on extensive pre-clinical research, this is in the dosing range where we would expect to see optimal clinical improvement in these patients."

SANOFI GENZYME OPTS IN CO-DEVELOPMENT AND CO-COMMERCIALIZATION OF ALNYLAM'S FITUSIRAN

Alnylam, a Cambridge, MA-based biopharmaceutical company specialized in the development of RNA interference (RNAi)-based therapeutics, has announced that Sanofi Genzyme, its collaborator, has elected to opt in to co-develop and co-commercialize fitusiran in the USA, Canada and Western Europe. The opt-in decision was based on recent promising interim clinical results from a Phase 1 study of fitusiran. Alnylam intends to initiate the fitusiran Phase 3 program in early 2017.

Fitusiran is an investigational RNAi therapeutic developed by Alnylam for the treatment of hemophilia and other rare bleeding disorders. This ongoing Phase 1 trial is a multi-center, dose-escalation study to evaluate the safety and tolerability of multiple doses of subcutaneously administered fitusiran in patients with hemophilia, with and without inhibitors.

Alnylam and Sanofi Genzyme, the specialty care global business unit of Sanofi entered an agreement in 2014 to accelerate and expand the development and commercialization of RNAi therapeutics across the world. Under the terms of the initial agreement, Alnylam retained product rights in the US, Canada and Western Europe, while Sanofi Genzyme obtained the right to access certain programs in Alnylam's current and future Genetic Medicines pipeline in the rest of the world through the end of 2019. The expanded right will give Sanofi the right to co-develop and co-commercialize fitusiran in the USA, Canada and Western Europe along with Alnylam.

Sanofi Genzyme will be required to make payments totaling up to $75 million upon the achievement of development and regulatory milestones for fitusiran. Upon the initiation of the first global Phase 3 clinical trial for fitusiran, Alnylam will earn a milestone payment of $25 million.

Sanofi Genzyme has elected not to opt in for ALN-AS1, an investigational RNAi therapeutic for acute hepatic porphyrias.

Dr David Meeker, Executive VP and Head of Sanofi Genzyme, commented: "We are pleased to collaborate with Alnylam to develop this important and innovative RNAi therapeutic option for people living with hemophilia across the world. This expanded collaboration with Alnylam supports our

deep and lasting commitment to patients with rare diseases. We look forward to sharing our operational, regulatory and commercial experience with Alnylam as we advance this investigational product."

AVACTA COLLABORATES WITH MSKCC TO ADVANCE CAR-T CELL THERAPY

Avacta Life Sciences, a UK-based biotechnology company developing Affimer® biotherapeutics and research reagents, has announced the initiation of a research collaboration with Memorial Sloan Kettering Cancer Center (MSKCC) to investigate the use of its Affimer technology in novel CAR-T cell-based immunotherapy.

Avacta's Affimer is an engineered alternative to antibodies. The simple structure and biophysical properties of these non-antibody binding proteins potentially provide significant advantages over antibody fragment technology currently used in CAR-T cell modification.

The collaboration will be led by Dr Renier Brentjens of MSKCC, and is intended to develop a new class of CAR-T cell therapy that incorporate Affimer molecules. Under the terms of the agreement, Avacta will develop Affimer molecules that bind different regions of CD19, a surface protein specific to B cells involved in lymphomas. These Affimer molecules will be incorporated in CAR-T cells and their anti-tumor function will be tested *in vitro* and in *in vivo* animal models. The ownership of the results generated directly as part of this collaboration will be shared between Avacta and MSKCC.

Alastair Smith, Avacta's CEO, commented: "We are delighted to be working with a world-leading team in the field to demonstrate the benefits that Affimer technology could bring to CAR-T therapy. The generation of positive data in these validated models of disease has the potential to open up highly valuable licensing and partnering opportunities for Avacta in this therapy area, which has attracted so much attention in the past couple of years."

CELL MEDICA EXPANDS PARTNERSHIP WITH BAYLOR TO DEVELOP CAR-NKT CELLS

Cell Medica and Baylor College of Medicine announces the expansion of their co-development partnership to create off-the-shelf allogeneic cell therapy targeting natural killer T (NKT) cells.

Cell Medica, a London-based pharmaceutical company specialized in the development of cellular therapeutics for the treatment of cancer and infectious diseases, have announced a co-development partnership with Baylor College of Medicine, Houston to develop engineered immune cell-based technologies for the treatment of solid tumors. The collaboration will provide Cell Medica with an exclusive license over

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Baylor's several cell and gene technologies and an option to license new products introduced into the co-development partnership by Baylor's leading research teams in the field of genetically engineered immune cells.

The collaboration will apply chimeric antigen receptor (CAR) technology to NKT cells as a novel immune cell type with biological properties that may be particularly effective for targeting solid tumors. The two companies will also seek to develop genetically engineered T-cell receptor (TCR) for use in NKT cells and T cells. Cell Medica expects the collaboration to generate a significant number of new products for its cellular immunotherapy pipeline. Baylor will conduct the preclinical and Phase 1 clinical research under the guidance of the Joint Steering Committee including members from both organizations.

Under the terms of its co-development agreement, Baylor will perform the research required to develop off-the-shelf NKT cell therapies and Cell Medica will fund research and development aimed for both oncology and non-oncology applications and will have an exclusive right to license future products within the co-development plan. Cell Medica has paid an up-front fee for the exclusive licensing arrangements and will make additional payments to exercise its exclusive option to license future products. As part of its upfront payment, Cell Medica has agreed to pay Baylor in preference shares that are convertible into common shares.

Gregg Sando, CEO of Cell Medica, commented: "Creating a safe and effective off-the-shelf product that can be used in multiple patients would unlock the full potential of cellular immunotherapy for the treatment of cancer. The addition of this exciting new project to our co-development program shows how we can continue to leverage the strong research capability and industry know-how captured within this collaboration."

When it became apparent that standard CAR T-cell technologies weren't going to have the same impact on solid tumor cells as they do on liquid tumors, immunotherapy developers and academia alike began exercising their creative powers to identify new approaches to tackling the solid tumor dilemma. We've seen many deals between Pharma and Biotech combining checkpoint inhibitors with CAR technologies, we've seen an interest in small molecule programming in culture, and of course layered modifications of CAR T cells to secrete cytokines or express surface proteins that increase cytotoxicity and persistence. We have also seen a renewed interest in NK cells, initially as cell lines leveraging their wild-type killing function, but then as modified cell lines, either to enhance antibody-dependent killing or to convert NK cells to targeted killing agents using CAR technology. The Cell Medica–Baylor deal this past month is a great example of the technology mash-up happening in industry. Under a co-development agreement, Cell Medica and Baylor will develop NKT cells, or NK cells modified to express a CAR or a TCR, in addition to other modifications that are designed to thwart inhibitory signaling from solid tumors. As part of the deal, Cell Medica will license/option a platform for cell engineering, three cancer targets for NKT products and a TCR technology. – ***Mark Curtis***

KOLON SIGNS LICENSING DEAL WITH JAPANESE PHARMA COMPANY

Kolon Life Science, a Korean biopharmaceutical company has signed a 500 billion Won ($438 million) out-licensing deal with Japan's Mitsubishi Tanabe Pharma Corporation over Kolon's Invossa, an allogeneic cell therapy to treat degenerative arthritis.

Under the terms of the contract, Kolon Life Science will receive an upfront payment of 27.3 billion Won and a milestone payment of 471.6 billion Won from Mitsubishi Tanabe Pharma. Kolon will produce and provide Invossa to Japan, while Mitsubishi Tanabe Pharma will have exclusive development and marketing rights for Invossa in Japan. Mitsubishi Tanabe Pharma is the world's 50th pharmaceutical exporter and the largest distributor of rheumatoid arthritis drugs in Japan.

Kolon's Invossa is a cell therapy drug containing genetically engineered chondrocytes designed to deliver growth factors to the damaged cartilage via an intra-articular injection, to treat degenerative arthritis. The company has completed a Phase 3 study of the drug and is now under a regulatory review in Korea. Study results have shown that a single Invossa injection in degenerative arthritis patients resulted in pain reduction for more than a year and increased physical activities.

MAGENTA COMPLETES SERIES A FINANCING

Magenta Therapeutics, a new Cambridge, MA-based biotechnology company focusing on the development of stem cell therapeutics for patients with immune- and blood-based diseases, has announced the successful completion of a $48.5 million Series A financing. The round was led by Third Rock Ventures and Atlas Venture, two firms that have built the company together over the past year with the vision to apply new stem cell science to treat patients with autoimmune diseases, genetic blood disorders and cancer.

Jason Gardner, CEO and co-founder of Magenta, commented: "There has been terrific innovation in stem cell science recently, and it is time to bring this forward to patients. During the past year, we have incubated Magenta with Third Rock and Atlas, and conducted extensive diligence on the key science in collaboration with many leading experts in the field. Our ultimate goal is to reboot the blood and immune systems safely to make a significant impact on the overall quality of life for a much broader group of patients that can benefit from transplant."

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FATE ANNOUNCES PRIVATE FINANCING

Fate Therapeutics, a San Diego-based biopharmaceutical company specialized in the development of cellular immunotherapies for cancer and immune disorders, has announced that it has entered a definitive securities purchase agreement with certain institutional and other accredited investors.

The private placement is being led by Redmile Group with participation from BVF Partners L.P., EcoR1 Capital LLC and Franklin Advisers and individual investors including members of the Company's Board of Directors. Gross proceeds from the private placement are expected to be approximately $57 million. Leerink Partners acted as the exclusive placement agent to the company in connection with the private financing.

The company will use the funds to advance its pipeline of programmed cellular immunotherapies and for general corporate purposes.

KITE PHARMA APPOINTS JIAN IRISH AS SENIOR VP OF SUPPLY CHAIN

Kite Pharma, a California-based biopharmaceutical company specialized in the development of cancer immunotherapy products, has announced the appointment of Dr Jian Irish as the Senior Vice President of Supply Chain. Dr Irish will be responsible for establishing and securing a reliable supply chain, including sourcing, logistics, inventory management, procurement, supply and operations planning process at Kite.

Dr Irish joins Kite from Sanofi where she was the Vice President of Biologics Strategic Supply, Sourcing and Partnerships. Prior to that, she served as Vice President of Biologics Product Development Technology Transfer at Sanofi and was the Executive Director, General Manager of Japan Asia Pacific Supply Chain at Amgen.

SALIM YAZJI JOINS CALIMMUNE AS EXECUTIVE VP AND CMO

Calimmune, a US-based clinical-stage gene therapy company specialized in genome editing, has appointed Dr Salim Yazji as its executive Vice President and CMO. Dr Yazji has more than 20 years of experience working with clinical development teams. Most recently, he served as the Vice President and Global Therapeutics Head of Oncology for Baxalta, Inc. Prior to Baxter International's spinoff of its bioscience business unit to create Baxalta, he served as a Vice President and Therapeutic Area Head of Oncology at Baxter. Prior to that, Dr Bartlett held various leadership and scientific positions at Novartis Pharmaceutical, Exelixis, Inc., PDL BioPharma, Inc. and Johnson & Johnson.