

Commercial insight: cell and gene therapy

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: Universal Cells inked two collaborations this month, with Astellas and BlueRock, that will see its gene editing technology put to use for the generation of therapeutics based on gene-edited induced pluripotent stem cells. The Astellas Institute for Regenerative Medicine (AIRM) and Universal Cells entered a global license agreement to research and commercialize a novel cell therapy technology. Under the terms of the agreement Astellas will fully fund the research work, and while Astellas will gain global rights to the technology following development, Universal Cells has an ability to earn up to \$9M in upfront payments, \$115M in clinical and regulatory milestones, and a royalty. So the deal has the potential to be fruitful for both parties.



GENE THERAPY
Richard Philipson
Chief Medical Officer,
Trizell Ltd, UK



CELL THERAPY
Mark Curtis
Financial Portfolio
Manager,
Emerging Technologies
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Switzerland



GENE THERAPY: This month's news is dominated by Spark Therapeutics' success at a recent FDA Advisory Committee, which gave its unanimous recommendation to approve the company's treatment for RPE-65-mediated inherited retinal disease. Of course, a formal decision on Spark's Biologics License Application is still awaited from FDA, but the chances of this being negative now seem vanishingly small. The pricing of the product will be interesting, as the treatable population is limited (1000 – 3000 patients in the USA), but the company has plenty of other products in the pipeline with the potential to bring it further growth. uniQure has also had success with regulatory authorities this month, with the award of Orphan Drug Designation for its Huntington's disease treatment from FDA, and agreement from both FDA and EMA on its phase 3 plans using an improved transgene with higher factor IX protein expression for haemophilia B.



POSITIVE PHASE 1 RESULTS FROM ATARA'S MS CELL THERAPY TRIAL

California-based Atara Biotherapeutics have reported encouraging results from their ongoing Phase 1 trial of autologous T cell therapy ATA190 in progressive multiple sclerosis (MS) patients. The results show six of ten subjects experiencing improved clinical conditions between 2 and 14 weeks following the initial infusion of ATA190. The immunotherapy is specific to the Epstein-Barr Virus (EBV) and five of the six improved patients had greater reactivity against the target EBV antigens, suggesting a correlation between this and clinical improvements. The results were presented at the MSParis 2017 Congress, the 7th Joint Meeting of the European Committee for Treatment and Research in MS (ECTRIMS) and the Americas Committee for Treatment and Research in MS (ACTRIMS). The investigators on the study are from QIMR Berghofer Medical Research Institute and The University of Queensland who are working in collaboration with Atara.

Atara CSO Chris Haqq commented, 'We are encouraged by the clinical data reported by Prof. Pender, Prof. Khanna, and their colleagues from the first prospective trial of an EBV-specific T-cell

immunotherapy in progressive MS. The clinical improvements observed in six of ten progressive MS patients treated with autologous ATA190, including three patients who improved their EDSS score, highlights the potential that targeting EBV positive B-cells and plasma cells is a potential new treatment modality that could offer a novel alternative to available MS therapies. We look forward to continuing to develop both autologous ATA190 and allogeneic ATA188 in MS patients.'

Professors Michael Pender of The University of Queensland and Rajiv Khanna, Coordinator of QIMR Berghofer's Centre for Immunotherapy and Vaccine Development issued a joint statement regarding the update saying: 'This study adds to the mounting evidence for a pathogenic role of EBV infection in MS. Our work sets the stage for further clinical studies with autologous and allogeneic EBV-specific T-cell immunotherapy in MS and other autoimmune diseases. We are delighted Atara Biotherapeutics recently initiated a multicenter, multinational allogeneic ATA188 Phase 1 clinical study in patients with progressive or relapsing-remitting MS'.



ZIOPHARM DOSES FIRST PATIENT IN PEDIATRIC BRAIN TUMOR GENE THERAPY TRIAL

Boston based biotech firm Ziopharm oncology has dosed the first patient in its Phase 1 pediatric brain tumor trial. The study is

investigating the use of the gene therapy Ad-RTS-hIL-12 with veladimex for the treatment of patients with recurrent or progressive brain

tumors in the cortex, and patients with diffuse intrinsic pontine glioma (DIPG).

Ad-RTS-hIL-12 is an adenoviral vector designed to trigger an anti-tumor immune response by expressing the cytokine hIL-12. Ziopharm's approach is combinatorial; patients are also given the activator ligand veledimex which employs the RheoSwitch Therapeutic System® (RTS®) in order to control the expression of hIL-12. Patients from the two tumor groups will be given a single intertumoral injection and then monitored to assess the safety and tolerability of the therapy.

Previous studies of Ad-RTS-hIL-12 in adult patients have yielded positive results which include the proliferation of pseudo-progression instead of tumor progression due to the infiltration of immune cells into the area. Ziopharm is therefore hopeful that this latest study will be similarly encouraging. CMO Francois Lebel commented: 'Studies in

adults with recurrent glioblastoma have shown that Ad-RTS-hIL-12 with veledimex is not only well tolerated, but also have shown growing evidence that this treatment elicits a targeted immune response against brain tumor cells that gives rise to improvement in overall survival. We look forward to advancing our studies in pediatric patients with brain tumors as these patients have limited-to-no therapeutic options.'

Stewart Goldman, M.D., Division Head Hematology-Oncology at Lurie Children's hospital where the first patient was treated further added, 'Pediatric gliomas are a devastating diagnosis for children and families, and DIPG, specifically, while rare, is extremely aggressive and always a fatal disease with no viable treatment options. We look forward to evaluating the potential of Ad-RTS-hIL-12 plus veledimex as a treatment option for children with brain tumors.'



APPROVAL FOR KITE'S CAR-T THERAPY, YESCARTA

Kite Pharma's CAR-T therapy for Non Hodgkins lymphoma, Yescarta, has been approved by the FDA.

The therapy is the second to gain approval in the novel class of drugs that engineer a patient's own immune cells to target cancer. Trials of Yescarta led to instances of long remissions in patients who had undergone two cycles of unsuccessful chemotherapy. This gives the drug a potential market of around 3,500 patients a year in the USA.

Due to concerns about the serious side effects of the therapy experienced by trial subjects, the rollout

of Yescarta will be staggered with 10 to 15 institutions using the therapy initially.

This is hoped to rise to 70 or more centers. Kite also hopes to expand the remit of the \$373,000 treatment for earlier stage lymphomas as well as solid tumor cancers.

Caron Jacobson, one of the doctors who helped to carry out the trialing commented regarding some of the patients that had been treated, 'Many patients were seriously contemplating their own mortality. We would be talking to them about other clinical trials, but also about hospice care and quality of life and

EXPERT
PICKKITE PHARMA EARNS
SECOND T CELL
APPROVAL

Gilead went from a company with no cell therapies in its pipeline to having an approved CAR-T therapy, almost overnight. The FDA granted approval to Kite Pharma's KTE-C19 (Yescarta) for relapsed or refractory diffuse large B cell lymphoma in patients that have previously failed two or more systemic therapies. Gilead's recent acquisition of Kite Pharma positions it to be a force in the development of CAR-T therapies. - Mark Curtis

comfort. You're really seeing people get their life back. After a couple weeks in the hospital and a couple

weeks at home, they go back to work. On its face, it's quite remarkable and revolutionary.'

UNANIMOUS BACKING FROM FDA
ADVISORY PANEL FOR SPARK'S
LUXTURNA

The FDA's Cellular, Tissue and Gene Therapies Advisory Committee have unanimously recommended approval for Spark Therapeutic's gene therapy for vision loss due to confirmed biallelic RPE65-mediated inherited retinal disease (IRD). Phase 1 and 3 data for the therapy, LUXTURNA, prompted the results of the non-binding vote that will nonetheless be considered by the FDA. Phase 3 trialing of LUXTURNA marked the first ever completed randomized controlled trialling of a gene therapy. Results were based on bilateral multi-luminance mobility test (MLMT) scores and full-field light sensitivity threshold (FST) testing. These showed functional vision maintained in a significant majority of subjects following a single dose. Additionally, there were

no adverse events observed, giving the therapy a strong safety profile alongside its efficacy.

Spark's head of R&D commented: 'Today's unanimous advisory committee vote recommending the approval of LUXTURNA moves us closer to bringing this investigational adeno-associated viral (AAV) vector gene therapy to patients with vision loss due to confirmed biallelic RPE65-mediated IRD. The clinical program for LUXTURNA includes patient data that show efficacy for up to four years on endpoints including bilateral multi-luminance mobility test (MLMT) score change and full-field light sensitivity threshold (FST) testing, with observation ongoing. We look forward to continuing to work with FDA as it completes its review of LUXTURNA'

ORPHAN DESIGNATION FOR
PLURISTEM'S ARS PLACENTA CELL
THERAPY

Placenta based cell therapy product PLX-R18 has been granted orphan drug designation by the FDA. The drug's developers, Pluristem, have a potentially expedited pathway to approval, as well as extended exclusivity and tax credits as a result of the decision.

PLX-R18 secretes proteins which trigger the regeneration of

hematopoietic cells in bone marrow, and hence the production of red blood cells. This has been designed to treat patients with certain cancers or cancer treatments, immune-mediated bone marrow failure, or acute radiation syndrome (ARS)- the latter of which is the subject of the orphan designation.

In vivo testing of PLX-R18 to date has demonstrated improvement in survival rates and improved cell recovery across all three major blood lineages. Pluristem is preparing for a human trial in ARS, and is currently enrolling patients in a Phase I trial in incomplete bone marrow recovery following hematopoietic cell transplantation (HCT). U.S. Department of Defense's (DOD) Armed Forces Radiobiology Research Institute (AFRRI) and Fukushima Medical University in

Japan are also conducting investigations into the therapeutic cells.

'Pluristem has a vast and dynamic program developing our PLX-R18 therapy as a treatment for ARS, which can potentially save many lives. Receiving Orphan Drug Designation brings us one step closer to providing a next-generation medical countermeasure against ARS, which is especially important given today's volatile political climate', commented CEO Zami Aberman.



ORPHAN DESIGNATION FOR uniQure's HUNTINGTON'S DISEASE GENE THERAPY

The FDA has granted uniQure orphan drug designation for their gene therapy targeting the root cause of inherited condition Huntington's disease. There are no current approaches which attempt to treat Huntington's from its underlying cause. This, and the rarity of the disease coupled with the innovation of the therapy – AMT-130 prompted the FDA's decision.

The mechanism by which AMT-130 targets Huntington's is to silence the *huntingtin* gene using an artificial micro-RNA which is delivered directly to the brain using an AAV5 vector. Orphan designation will aid uniQure's road to market with tax and financial incentives as well as exclusivity following approval.

In additional news this month, uniQure has announced that following multi-disciplinary meetings with the FDA and EMA, the company plans to expeditiously advance AMT-061, which combines

an AAV5 vector with the FIX-Padua mutant, into a pivotal study in 2018 for patients with severe and moderately severe hemophilia B.

AMT-061 and AMT-060, the latter of which has been tested in 10 patients in an ongoing Phase 1/2 clinical trial, are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. The gene variant, referred to as FIX-Padua, expresses a protein with a single amino acid substitution that has been reported in multiple preclinical and nonclinical studies to provide an approximate 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein. All other critical quality attributes of AMT-061 are expected to be comparable to those of AMT-060, as AMT-061 utilizes the same AAV5 capsid and proprietary insect cell-based manufacturing platform.

'Our mission in hemophilia B has always been to develop the safest and most effective gene therapy

with the broadest application to patients. We believe AMT-061 moves us closer to this goal, as it has the potential to provide optimized clinical and tolerability benefits to nearly all severe and moderately severe patients with hemophilia B,” stated Matthew Kapusta, chief executive officer of uniQure. “We are delighted to have received constructive guidance from both the FDA and EMA, which we believe allows us to expeditiously advance AMT-061 into a pivotal study next year, as previously planned. In anticipation of this, we have begun GMP production of AMT-061 in our Lexington facility and preparations for the pivotal study are underway.”

“I believe AMT-061 has the potential to be an important gene therapy for patients suffering with hemophilia B,” stated Steven Pipe, M.D., professor of pediatrics and pathology and pediatric medical director of the hemophilia and coagulation disorders program at the University of Michigan. “Based on the data generated to date, AMT-061 may be the first gene therapy to provide durable, curative benefits to nearly all patients with hemophilia B, without the complications associated with capsid-related immune responses. I very much look forward to serving as an investigator in this exciting Phase III program.”



NIKON CELL INNOVATION TO MANUFACTURE MULTISTEM® FOR ATHERSYS IN JAPAN

Nikon CeLL innovation's (NCLi) Japan facility will be used by Athersys to manufacture the company's patented stem cell therapy MultiStem® under a new manufacturing services agreement.

An allogeneic product, MultiStem® will be produced for the treatment of ischemic stroke via tissue repair and healing. This is hoped to increase the tight treatment window for strokes to 36 hours. The therapy will be developed and



Fascinating news from uniQure, which has reached agreement with FDA and EMA regarding a change in its treatment for haemophilia B, with a switch in transgene from wild-type factor IX (FIX) to the allelic variant FIX-Padua (AMT-060), which gives rise to much higher levels of expression of factor

IX protein. The agreements with regulators pave the way to a pivotal open-label, single dose study of AMT-061 in patients with moderate or severe haemophilia B, although a concurrent dose-confirmation study with AMT-061 is also required. AMT-060 (wild-type FIX) has already been evaluated in 10 patients in a phase 1/2 study, but the switch will make the new uniQure product more competitive with Spark Therapeutics' rival product SPK-9001, which produces improvements in FIX activity of more than 60%, versus approximately 10% increases seen after treatment with AMT-060. Whether it's right to fixate on protein increases, rather than clinical outcomes, has become a moot point – both companies are now targeting high levels of expression of FIX.- Richard Philipson

marketed in Japan by HEALIOS K.K., Athersys' collaborator under a licensing deal.

'We are excited about our new alliance with Nikon CELL innovation, focused on establishing the manufacturing infrastructure that will enable us and Healios to achieve our goals in Japan. Nikon brings important capabilities to the partnership, including an extensive investment in facilities and technology. We and Healios agree that this puts us both in a strong position to

achieve our goals. With a successful study outcome and subsequent product registration, Athersys' MultiStem cell therapy would represent the first major advancement in more than twenty years for the treatment of acute stroke. We intend to be ready for success, and this alliance represents an important step toward establishing the capabilities that will enable us to take full advantage of this significant opportunity,' commented Gil Van Bokkelen, Athersys CEO.



AVROBIO LICENSES CYSTINOSIS PROGRAM FROM GENSTEM THERAPEUTICS

Avrobio has expanded its lysosomal storage diseases portfolio with the licensing of Genstem Therapeutic's cystinosis program. Avrobio is developing gene therapies for this class of diseases and expect to file and IND filing for this latest addition in time for 2018 clinical trialing.

The gene therapy approach being taken for cystinosis employs the use of peripheral blood stem cells from a patient which are then genetically modified to carry a functional copy of the faulty *CTNS* gene. The engineered cells are then delivered back to the patient in a one-time

infusion. It is hoped that this will alleviate adverse symptoms of the condition which can include renal failure, blindness and severe muscle weakness among others.

'We are pleased to partner with Dr Stephanie Cherqui and her team at the University of San Diego (UCSD), as well as with GenStem Therapeutics, Inc. as we share their vision of developing a novel and potentially transformative gene therapy for the treatment of patients with cystinosis,' commented Geoff MacKay, Avrobio's CEO.



RARE PEDATRIC DISEASE DESIGNATION FOR ATARA'S T CELL THERAPY, ATA230

The FDA has granted Atara Biotherapeutics Rare Pediatric Disease Designation for their T cell immunotherapy ATA230. The allogeneic product is designed to treat congenital cytomegalovirus (CMV)

infection by targeting antigens expressed by CMV. The designation will allow Atara to advance to priority review for a biologics license or new drug application for future

products under the FDA's voucher system.

CMV can be life threatening in patients with compromised immune systems, and can also result in blindness, deafness, or brain damage. Current approaches are comprised of small molecule antivirals, leaving a significant unmet need that Atara hopes will be addressed by ATA230. The therapy has undergone Phase 1 and two Phase 2 clinical studies in immunocompromised patients with CMV viremia or disease who are refractory or resistant to antiviral drug treatment in the post-transplant setting thus

far. Plans are in place to expand the clinical development of the product to Phase 3.

CEO Isaac Ciechanover commented, 'FDA's Rare Pediatric Disease Designation, following the recent orphan drug designation for ATA230, further underscores the high unmet medical need in treating congenital CMV infection. FDA's Rare Pediatric Disease Designation, following the recent orphan drug designation for ATA230, further underscores the high unmet medical need in treating congenital CMV infection.'



DOSING COMPLETE IN AGILIS'S AFIL-AADC TRIAL

Agilis Biotherapeutics has completed dosing of the final patients in likely the largest trial (by cohort size) of a gene therapy for the central nervous system (CNS). The therapy, named AGIL-AADC is intended for the treatment of

Aromatic L-amino acid decarboxylase (AADC) deficiency.

AADC arises as a result of a mutation in the *DCC* gene which codes the dopamine and serotonin producing enzyme, AADC. This deficiency results in severe neurological impairment, initially characterized by a lack of motor milestones being reached.

Agilis is preparing a Biologics License Application (BLA) for AGIL-AADC which they hope to submit by the end of 2018. The application is backed up by solid safety and efficacy data from trialing of AGIL-AADC. These results tracked improvements in motor skills such as grasping, sitting unassisted, and standing, in patients over a five year period post-treatment.

CMO Kirsten Gruis, said,

'The emerging clinical data indicating sustained motor function improvements following a single



ONES TO WATCH

ATARA CONTINUES TO DELIVER

Atara continues to deliver intriguing results with its

Epstein Barr Virus (EBV)-targeted T cell therapies. Most recently the company announced that six of ten patients in a Phase 1 study investigating ATA190 in patients with progressive multiple sclerosis (MS) showed clinical improvement following administration of the therapy. Importantly, clinical improvement was associated with T cell reactivity to EBV, an agent known to have pathogenic role in MS. The company also recently received a Rare Pediatric Disease Designation from the FDA for ATA230 in treating congenital cytomegalovirus infection. - Mark Curtis

administration of AGIL-AADC gene therapy are encouraging, with patients achieving and maintaining motor milestones that would not otherwise be seen in children with severe AADC deficiency. Over the period of observation, important functional gains have been observed

to date in the context of a good safety and tolerability profile. These data support the premise that gene therapy may be able to provide durable benefits to patients with debilitating disorders affecting the central nervous system.'



BLUEBIRD BIO'S STEM CELL-GENE THERAPY OFFERS HOPE FOR CALD PATIENTS

Interim results from bluebird bio's Phase 2/3 study demonstrate the safety and efficacy hematopoietic stem cells transduced with Lenti-D lentiviral vector for treating early-stage cerebral adrenoleukodystrophy (CALD).

X-linked adrenoleukodystrophy, also known as Lorenzo's Oil disease, is caused by mutations in the *ABCD1* gene that encodes a protein of the peroxisomal membrane named ALDP. It affects one in every 21,000 male births worldwide. The cerebral form of the disease, CALD is characterized by demyelination and neurodegeneration and is fatal. Allogeneic hematopoietic stem cell transplantation is the only effective treatment option available currently, but it carries significant risk.

In the study published in *New England Journal of Medicine*, Eichler *et al.* report interim results of bluebird bio's Phase 2/3 STARBEAM trial. The study evaluated the safety and efficacy of transplantation with autologous CD34⁺ hematopoietic stem cells transduced with Lenti-D (lentiviral vector that encoded *ABCD1* gene) for the treatment of cerebral adrenoleukodystrophy in children. The primary end point of the study was being alive and

having no major functional disability at 24 months after infusion. The study assessed the occurrence of graft-versus-host disease, death and major functional disabilities, as well as changes in neurologic function.

17 boys were included in the study and received the Lenti-D Drug Product intravenously following myeloablative conditioning. The interim analysis was performed at the median follow-up was 29.4 months. 15 out of 17 patients were alive at the end of 24 months, with no graft failure and no major functional disabilities. Out of the remaining 2, one died from disease progression and the other one was withdrawn from the study and died from complications of a subsequent allogeneic transplantation.

Positive results obtained from the 15 patients suggest that Lenti-D gene therapy may be a safe and effective alternative to allogeneic stem-cell transplantation in boys with early-stage cerebral adrenoleukodystrophy. However, additional follow-up is needed to fully assess the duration of response and long-term safety of the treatment.

Prof. Adrian Thrasher at UCL's Great Ormond Street Institute of Child Health and co-investigator of

the study commented: 'This data we are seeing from this study of gene therapy is a really exciting development for boys with CALD. Not only does the data show that Lenti-D has the potential to minimise further progression of this devastating disease, but the technique also avoids

the need for a matched bone marrow donor, which can be difficult to find and can delay vital treatment. The results from this trial thus far are very encouraging and will pave the way for further development of state-of-the-art treatments for children with life-limiting conditions.'



EXPERT PICK

Bluebird Bio's announcement of positive results from its phase 2/3 trial in cerebral adrenoleukodystrophy (CALD) offers hope to families living with this devastating condition. Boys are typically diagnosed with this X-linked condition in early childhood and most die within a decade if not treated

with allogeneic haematopoietic stem-cell transplantation. An interim analysis of outcomes in 17 patients treated using Bluebird's Lenti-D product suggests that the treatment was effective in 15/17 patients, with treated boys generally showing stable disease or limited progression of disease on MRI of the head, as compared with the known rates of lesion progression among untreated boys. This and other studies indicate that some disease progression on MRI during the first year after transplantation is common and therefore reinforce the urgency in identifying cerebral disease early and treating it swiftly. A longer follow-up and a larger sample size are needed to confirm the low potential for genotoxic effects and the clinical efficacy and safety of gene therapy in this condition, but these interim data are definitely encouraging. – Richard Philipson

LICENSING AGREEMENTS & COLLABORATIONS



JUNO LICENSES TRIANNI MOUSE MODEL FOR CAR T DEVELOPMENTS

Juno Therapeutics has signed a licensing agreement that will grant it use of the Trianni Mouse Model. The monoclonal antibody discovery platform will be used to identify fully human binders that can be engineered into CAR-T cell therapies. The technology is high throughput, direct to CAR, and single sequencing.

David Meininger, TRIANNI's Chief Business Officer commented, 'TRIANNI is pleased to bolster Juno's lead generation capabilities

through a license to The Trianni Mouse. We are confident that Juno's deep expertise in developing engineered T cells will potently synergize with the industry-leading human antibody repertoires provided by The Trianni Mouse.'

Juno's CSO Hy Levitsky commented, 'The Trianni Mouse offers an in vivo platform for generating a diverse human antibody response to tumor targets of interest.'



NEW COMPANY VIRALGEN TO PRODUCE AAV VECTORS IN SPAIN.

A new company for the manufacture of double strand AAV in Spain has been established by AskBio and Columbus Venture Partners. Named Viralgen Asklepios Bio-Pharmaceutical, the new company starts life with financial backing from its founders as well as the Basque government.

Viralgen is expected to leverage the manufacturing experience of AskBio to produce GMP compliant batches of AAV vectors for use globally. An additional effect of the new company is the creation of around 50 jobs for locals.

‘This is a great opportunity to establish unique gene manufacturing

capabilities in Europe,’ said Sheila Mikhail, President and CEO of AskBio. ‘Our partnership with Columbus and the support of the Basque government will hopefully attract significant talent to the region and have immediate impact on the gene therapy market in Europe and the rest of the world.’

Javier García Cogorro of Columbus Venture Partners added: ‘We are excited about the exclusive viral vector capabilities this partnership will create. Viralgen success will be based on three areas: outstanding technology, quality of the location to attract talent, and commitment from the Basque Country Government.’



AIRM AND UNIVERSAL CELLS TO DEVELOP NOVEL CELL THERAPY

Universal Cells have closed an exclusive worldwide license agreement with Astellas Institute for Regenerative Medicine (AIRM) for the development of a novel cell therapy. Universal Cell’s proprietary technology will allow the companies to create products that do not require Human Leukocyte Antigen (HLA) matching, making them widely accessible.

Under the agreement the research stage of development will be conducted jointly by the two companies whilst the program will be fully funded by AIRM. Worldwide commercialization rights will be held by AIRM with Universal Cells being eligible for up to \$9 million in upfront payments and research

milestones. A further \$115 million is potentially available to Universal Cells subjects to clinical and regulatory milestones being reached.

AIRM President Yoshitsugu Shitaka commented, ‘I am pleased to enter into this collaboration with Universal Cells for a novel cell therapy using their proprietary Universal Donor Cell technology based on gene editing by rAAV (recombinant Adeno-Associated Virus) and cell engineering of pluripotent stem cells. This is definitely an important step forward in developing a potential new option as an innovative cell therapy to treat devastating diseases with high unmet medical needs.’

Claudia Mitchell, CEO of Universal Cells praised the vision of

AIRM and their commitment to the regenerative medicine field stating: ‘This research collaboration will leverage Universal Cells unique solution to major challenge of cell

therapies – the immune rejection of allogeneic cells. We look forward to bringing real world Regenerative Medicine treatments to the market with Astellas.’



PRELIMINARY PROXY STATEMENT FILED FOR INOTEK AND ROCKET MERGER

Inotek Pharmaceuticals Corporation has filed the Company’s preliminary proxy statement with the US Securities and Exchange Commission (SEC) for the proposed merger of Inotek Pharmaceuticals and Rocket Pharmaceutical.

From the terms of the merger, it is expected that shareholders of Rocket will receive shares of newly issued Inotek common shares in a private placement. Rocket shareholders will own approximately 81% of the combined Company, and current Inotek shareholders approximately 19% of the combined Company.

Rocket is developing gene therapies based on lentiviral vector (LVV) delivery methods. The company currently has an ongoing trial for the treatment of Fanconi Anemia, and has an additional three therapies advancing towards the clinic.

Rocket CEO Gaurav Shah commented, ‘We have built our pipeline

applying stringent criteria. We invest in validated assets with strong mechanistic rationale and established preclinical proof of concept. We focus on rare, untreated diseases where there is a high unmet need, with clear clinical endpoints, and a well-defined regulatory pathway. With the funding provided by the proposed merger with Inotek, we believe we are well-positioned to achieve early clinical success and maintain first-mover advantage in each of our exciting programs.’

‘We believe the proposed merger with Rocket provides an attractive opportunity for our shareholders,’ said David P. Southwell, President and CEO of Inotek. ‘Rocket has several near-term clinical and proof of concept catalysts, a well-funded operation and a management team with rare disease and gene therapy leadership expertise.’



BLUEROCK AND UNIVERSAL CELLS TO COLLABORATE ON IPS CELL DEVELOPMENT

Cambridge-based BlueRock Therapeutics has entered into a licensing and collaboration agreement with the Seattle-based company

Universal Cells. The agreement will see the two companies work on engineering induced pluripotent stem (iPS) cell lines for use by BlueRock

in allogeneic, off the shelf cell therapies. The collaboration will leverage Universal Cell's proprietary genome editing technology to create the iPS lines.

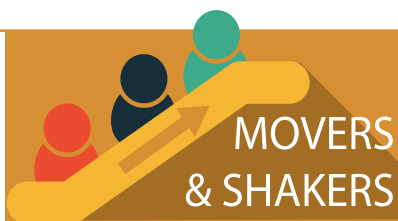
'BlueRock is at the cutting edge of the cell therapy field and our collaboration with the company represents an important step in their efforts to develop off-the-shelf cell therapies for degenerative diseases. We are honored that our technology will support the advancement of BlueRock's cell therapies' commented Universal Cell CEO, Claudia Mitchell.

BlueRock's CEO Emile Nuwaysir added, 'This partnership with Universal Cells provides us with an important technology useful to advance universally-compatible, allogeneic cell therapies. There are hundreds of millions of patients suffering from degenerative diseases where the root cause is the loss of healthy cells. BlueRock is developing breakthrough cell therapies to treat these diseases, and Universal

GENSIGHT BIOLOGICS APPOINTS BARRETT KATZ AS CMO

Gensight Biologics has appointed a new CMO, Barrett Katz who joins the company on November 1st from Montefiore Medical Center and the Albert Einstein College of Medicine in New York, where he was a professor.

Dr Katz's responsibilities at Gensight will encompass clinical development and medical affairs where he will draw on extensive industry and academic experience. Some of his accomplishments in industry include holding the post of Medical affairs and strategy VP at Eyetech,



the company which brought the first medical therapy for macular degeneration to market; being the CMO at Fovea Pharmaceuticals; and CEO of Danube Pharmaceuticals.

Having received his medical degree from Case-Western Reserve University School of Medicine, and MBA from University of Rochester's Simon School of Business, Dr Katz's subsequent academic career has included fellowships at Harvard and the University of California, as well as faculty positions at various universities.

Cells is an excellent technology partner in that endeavor.'



SANFILIPPO FOUNDATIONS COLLABORATE ON \$13.85 MILLION GRANT TO ABEONA

In order to further advance their gene therapy candidates for Sanfilippo Syndrome Type A (MPS IIIA) and Sanfilippo Syndrome Type B (MPS IIIB), Abeona Therapeutics has been granted \$13.5 million from a collaborative agreement between nine Sanfilippo foundations. Additionally, Abeona received \$5.0 million through the cash exercise of 625,000 common stock purchase warrants which were created

in 2015 and had a November 2017 excisable deadline.

Timothy J. Miller, Abeona's CEO commented, 'Abeona is pleased to continue our global collaboration with the Sanfilippo foundations to help further advance our gene therapy programs for MPS III disease. The effort and expertise that we continue to commit to the ABO-102 and ABO-101 programs puts us in a strong position to further extend the important progress



reported to date. We are grateful to the foundations for their ongoing commitment to identifying and facilitating the development of clinical innovation to treat patients with MPS III disease.'

'The importance of reducing the heparan sulfate as a cause of disease

burden cannot be understated, and the clinical data demonstrated by Abeona enabled us to provide additional support in the pursuit of finding new paradigms to treat all children with Sanfilippo syndrome,' stated Board Member of Team Sanfilippo, Carl Kapes.

Written by Haaniah Hamid and Applonia Rose, Cell and Gene Therapy Insights



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