

# Commercial insight: cell and gene therapy

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



**CELL THERAPY:** Two significant pipeline expansions occurred this past month, both including intellectual property licensed from the US National Institutes of Health (NIH). Lion Biotechnologies solidified its position as the leader in the development of tumor-infiltrating lymphocyte (TIL) therapies and gained exclusive, world-wide rights for the use of TILs in four additional tumor indications, including bladder, breast, lung, and HPV-associated cancers of the cervix and head and neck. Kite Pharma had a similarly fruitful month and was granted exclusive rights for the use of T-cell receptor (TCR) products targeting the MAGE A3 and A3/A6 antigens for several cancer types, including lung, pancreatic, gastric, and breast. Kite also announced a global licensing deal with Alpine Immune Sciences, a privately-held biotechnology company with a platform focused on the immune synapse. As part of the deal Kite secured two undisclosed transmembrane immunomodulatory protein (TIP) targets that it will leverage to produce its next generation of CAR products. Second- and third-generation CAR and TCR products will be designed not only to target cancer, but to simultaneously disrupt the tumor microenvironment for enhanced cytotoxicity and persistence.



**GENE THERAPY**  
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**GENE THERAPY:** The one story that caught my eye this month was the announcement that Vertex Pharma and CRISPR have entered into collaboration related to gene editing technology. This is a truly fascinating technology and presents a logical next step for gene-based therapies. Rather than simply replacing defective genes with corrected ones, the technology as applied, corrects the defective gene *in situ*, well at least that's the theory. This will hopefully remove the problem of delivering the new gene to the exact site where it is needed, which is one of the main barriers that simple gene replacement therapies face. However I am surprised that they are initially focusing efforts on correcting the genetic mutations that occur in cystic fibrosis (CF). One of the difficulties in developing a gene therapy for CF is that the specific defects caused by the mutation are spread across myriad cells in both the gastrointestinal and respiratory systems; therefore, to bring about any clinical improvement it is likely that you are going to have an effect in millions of cells across these two body systems. Some of the first developments with gene therapy in the early 1990's were targeted at CF and they encountered this very problem. So as a first step this looks like a hard ask to me and raises the question of why they are not targeting a disease where the mutation in cells is more localized such as the eye, the heart or in the CNS to prove this approach will work in humans. They must have their reasons...



### AMGEN WINS FDA APPROVAL FOR IMLYGIC™

Amgen have received approval from the US FDA for the use of Imlygic™ (talimogene laherparepvec, or T-vec) in melanoma patients.

Imlygic is a genetically modified herpes simplex virus type 1 designed to replicate within tumors and produce the immunostimulatory protein granulocyte-macrophage colony-stimulating factor (GM-CSF). The therapy concurrently attacks the tumor and mediates a heightened immune response – it causes cell lysis, which ruptures tumors, releasing tumor-derived antigens, which along with GM-CSF, may promote an anti-tumor immune response.

Amgen indicates the therapy will be available to patients in the USA within a week with a projected price point of \$65,000 per treatment round, although this will vary depending on the dose requirements of each patient.

Following this positive approval from the FDA, Amgen hopes to hear from the European regulators within the next couple of months.

“Not all melanoma patients currently benefit from available therapies, and Imlygic represents an important new option that can provide meaningful durable response for patients with this aggressive and complex disease. Immunotherapy is an exciting area for cancer research, and we are currently studying Imlygic in combination with other immunotherapies in advanced melanoma and other solid tumors,” commented Sean Harper, Executive Vice President of R&D, Amgen.

Unsurprisingly Amgen saw a boost to their stock value following the news rising from \$160.49 to \$162.87 on the 28 October.  
- Mudith Jayawardana



## OSIRIS ANNOUNCES PHASE 3 TRIAL FOR THE TREATMENT OF CHRONIC DIABETIC FOOT WOUNDS

Osiris Therapeutics, Inc. has initiated a Phase 3 clinical trial to assess the safety and efficacy of OTI-15-01 for the treatment of chronic diabetic foot ulcers.

OTI-15-01 is comprised of several components known to be critical for effective wound healing including a collagen-rich matrix, growth factors and neonatal fibroblasts, epithelial cells, endothelial cells, and mesenchymal stem cells (MSCs).

The trial will be conducted as a randomized, controlled, blinded study, expecting to enrol up to 224 patients from 20 sites. Patients will be randomized to receive OTI-15-01 plus standard of care versus standard of care alone, which includes debridement, a non-adherent dressing, and standardized off-loading.

The primary endpoint of this trial will be observing the proportion of patients that exhibit

complete wound healing by week 12. Secondary endpoints will include healing time, reduction of wound size by week 4, number of applications and overall safety which includes wound-specific adverse events. Patients that are part of the control arm that do not heal within 12 weeks will be able to receive the active drug in an open-label crossover arm of the trial for up to an additional 12 weeks. "This next-generation product meets the highest quality standards for a biologic, and contains consistent amounts of viable, potent cells. We believe that delivering these directly to the wound bed is the best approach for patients who are suffering from chronic non-healing diabetic foot ulcers." Commented Dr Lode Debrabandere, President and CEO, Osiris. - *Mudith Jayawardana.*



### EXPERT PICK

Wound healing remains a challenging space for cellular products given Medicare's decision, beginning in 2014, to bundle cellular and acellular products together, capping reimbursement well below \$1,500; a move that was detrimental to companies developing cellular wound healing products for diabetic foot ulcers and venus leg ulcers. Despite this, Osiris Therapeutics has continued development of its placental-based wound healing platform, and announced the launch of a Phase 3 study this past month investigating a patch composed of neonatal fibroblasts, epithelial cells, endothelial cells, and mesenchymal stem cells for treatment of chronic DFUs. Over the years, industry has found creative ways to utilize hospital waste products, such as neonatal skin and placenta, to engineer cellularized patches for topical wound healing applications. While this approach significantly reduces cost-of-goods, improving economics for industry, wound healing products approved through regulatory pathways will benefit greatly if they are reimbursed uniquely, aside from less expensive products that are simply cleared for marketing. - **Mark Curtis & Rahul Sarugaser**



## SPARK'S GENE THERAPY ACHIEVES PRIMARY ENDPOINT

A pivotal study of Spark Therapeutics' therapy for sight-blinded retinal dystrophies yielded promising results this month. The announcement came that their treatment, SPK-RPE65 reached the primary endpoint of their study and 2 out of 3 secondary endpoints, allowing for a biologics license application through the FDA next year.

A total of 31 patients took part in the small Phase III study, whereby improved vision was demonstrated after treatment with the gene therapy. The announcement saw Spark's pre-market trading shares soar by 60%.

"The majority of the subjects given SPK-RPE65 derived the maximum possible benefit that we could measure on the primary visual function test, and this impressive

effect was confirmed by a parallel improvement in retinal sensitivity," said Dr Albert Maguire, principal investigator in the trial and professor of ophthalmology at the Perelman School of Medicine of the University of Pennsylvania.

As a frontrunner in the field, following the successful trials, Spark will have to tackle the ongoing debate around pricing of potentially curative gene therapies. Currently expected to cost around \$1 million, payment plans are expected to be set up in order to spread the cost over a certain number of years. Additional work is still yet to be conducted to determine if these treatments provide a lifelong cure and what technologies are available that will allow for repeat treatments, if the treatments provide a limited effect. - *Mudith Jayawardana.*



### EXPERT PICK

The big news this past month in the gene therapy space has come from both Spark and Bluebird. Spark announced the results of their phase III pivotal study for the treatment of the inherited retinal disease, Leber's Congenital Amaurosis (LCA) with SPK-RPE65. Not only did they reach the primary

endpoint of the study but also two of the three main secondary endpoints. Working in rare and orphan diseases such as LCA is not easy – with no current treatments available for this disease, the difficulty in developing such products is determining what endpoint you use to demonstrate efficacy. Initially there was no obvious answer to this question and there was clearly much debate between the company and the regulators about what it should be, which eventually resulted in a public advisory meeting being held by the FDA to discuss the issue. The endpoint finally chosen was that of a visual functional test which involved patients navigating a maze – this was an untried methodology as an endpoint for product approval, but it has clearly paid off and will hopefully pave the way not only for the approval of the product but it will also help in the development of additional therapies to treat other retinal diseases. Following the BLA filing the only issue that Spark will need to resolve is that of the pricing of their product – but as I have said before, this is a nice problem to have and how many other gene therapy companies would like to have this problem too? - *Alan Boyd*



## SUCCESSFUL GMP PRODUCTION PROCESS FOR ALLOGENEIC UCART19

Cellectis, a biopharmaceutical company focused on developing immunotherapies based on gene edited CAR-T cells, announced that a series of production runs were successfully performed on their lead TALEN gene editing product candidate, UCART19 under GMP conditions.

This demonstrates that UCARTs can be manufactured in GMP conditions as well as highlighting the capacity of Cellectis' pipeline of UCART product candidates to be manufactured for clinical investigation.

UCART19 is an allogeneic engineered T-cell product for the treatment of CD19-expressing

hematologic malignancies, initially developed in chronic lymphocytic leukemia and acute lymphoblastic leukemia. The manufacturing process of Cellectis' CAR T- cells (UCART) produces frozen, off-the-shelf, allogeneic, engineered CAR T-cells. The TALEN-based gene editing is designed to suppress T-cell alloreactivity and confer resistance to alemtuzumab to the T-cells.

"Cellectis has reached a critical milestone both for the Company and our industry, creating new opportunities for patients." Commented David JD Sourdive, Executive Vice President, Corporate Development. - *Mudith Jayawardana.*



## UNIVERSAL CAR T

Cellectis stands apart from other CAR T developers with its allogeneic platform for oncology. While an off-the-shelf, frozen, bulk-manufactured product offers advantages in terms of the cost of materials, manufacturing, and logistics, non-patient-specific cells elicit immune responses that can not only lead to destruction of therapeutic cells, but cause serious adverse events, including cytokine storms and graft-versus-host disease (GvHD).

Cellectis is attempting to circumvent these issues with its universal CAR T (UCART) products by engineering T cells with a TALEN gene-editing platform that knocks out *TCR-α* and *CD52* genes. Now, with completion of three GMP manufacturing runs of UCART19, a CAR T product that will be targeted to CD19 expressing hematological cancers, the company is poised to enter the clinic to achieve proof-of-principle in humans. - *Mark Curtis & Rahul Sarugaser*



## ADAPTIMMUNE GROWTH LEADS TO LONG-TERM EXPANSION PLANS IN PHILADELPHIA

Adaptimmune, a clinical stage biopharmaceutical company focused on the use of T-cell therapy to treat cancer, has announced plans to establish their US headquarters and clinical operations in Philadelphia. The newly developed 47,400 square foot facility, to be constructed at The Navy Yard in Philadelphia, will house a cGMP manufacturing facility designed to support the clinical development and initial commercialization of Adaptimmune's engineered immunotherapies.

*"Adaptimmune is in a period of rapid growth on both sides of the Atlantic, and we are*

*putting in place the facilities to enable us to deliver our promising pipeline into the clinic and beyond,"*

*commented James Noble, CEO of Adaptimmune.*

This news follows Adaptimmune's previous announcement of the construction of a new laboratory and office building in Milton Park, Oxfordshire, UK. This new facility, which will provide ~67,000 square feet of rentable area, is scheduled for completion late 2016, which will coincide with the establishment of their US headquarters. - *Mudith Jayawardana.*



## HIGHS & LOWS FOR BLUEBIRD BIO

Bluebird Bio's shares recently took a hit, dropping by 15%, when a patient in their gene therapy study for  $\beta$ -thalassemia had to be given two blood transfusions after developing symptoms of anemia having gone 7 years transfusion free prior to this.

The patient in question, subject 1003, is from the LG001 study which was a proof-of-concept trial of Bluebird's gene therapy technology using their previous-generation HPV569 vector to insert a corrective gene. As stated by Bluebird, they have since developed a new and hopefully more effective vector for gene delivery.

After going 7 years without a transfusion, the patient recently required 2 blood transfusions after experiencing symptoms of anemia. Expression of HbAT87Q and the vector copy number in peripheral blood leukocytes had remained unchanged with no serious adverse effects, indicating persistence in the gene therapy.

Despite the evidence that points towards gene therapies providing lasting benefits, concerns still remain as to whether they can provide lifelong cures. If evidence suggests that the benefits are limited to a certain period of time, pricing will become subject to alteration.

In some positive news from Bluebird Bio, Prof. Marina Cavazzana,

Professor of Hematology and Director of the Department of Biotherapy at Hospital Necker, University Paris Descartes presented a review of their clinical study experience with lentiviral-based gene therapies for  $\beta$ -thalassemia at the *10th Annual Cooley's Anemia Foundation Symposium* in Chicago.

As lead investigator for the HGB205 clinical study, Professor Cavazzana summarized the results from an ongoing study using LentiGlobin® BB305 for the treatment of  $\beta$ -thalassemia major and severe sickle cell disease.

Professor Cavazzana also discussed the advances in lentiviral vector design, specifically the evolution from the first-generation HPV569 lentiviral vector to bluebird bio's current lentiviral vector, BB305, which results in a substantially improved vector copy number and HbAT87Q expression.

"The data from the LG001 study were invaluable to our efforts over the last 5 years to optimize our gene therapy approach with improvements to the potency, robustness and manufacturing for the next-generation lentiviral vector, BB305."

Bluebird's chief medical officer David Davidson remains hopeful moving forward: "The data from the LG001 study were invaluable to our efforts over the last 5 years to optimize our gene therapy approach with improvements to the potency, robustness and manufacturing for the next-generation lentiviral vector, BB305." - *Mudith Jayawardana*



## EXPERT PICK

Turning now to Bluebird and their news that a patient treated years ago with a gene therapy for  $\beta$ -thalassemia, recently required two blood transfusions for his anemia having been transfusion-free for 7 years. Following this news their share price dropped by about 15%. So is this a real issue for Bluebird? Personally I do not think so. If you step back and look at the background to this story: the gene therapy that was used in this patient was developed more than 10 years ago and it has clearly worked in this patient and a couple of others. No doubt from the patient's perspective they are very pleased to have gone such a long time without needing a transfusion, as it needs to be remembered that these patients typically need a transfusion every 4–6 weeks. When Bluebird took on this project, they knew that the product would need further development to improve both the vector copy number and HbA expression and that's just what they have spent the last 3–4 years doing. The results of their endeavors are now evident given the results of recent studies that have also just been released. The history of product development in pharmaceuticals has many examples of the second product in a new treatment modality doing much better than the first, as the second product builds on the experience of the first pathfinder – think Tagamet and Zantac, Captopril and Enalapril and Accolate and Singulair, to name but a few – it will be no different in gene therapy - **Alan Boyd**





## NEW RESEARCH PARTNERSHIP BETWEEN UCL & TAKEDA

A new research collaboration was announced between University College London (UCL), a leading university in CNS research with a class reputation in the field of neurodegeneration and rare neurological disorders, and Takeda Pharmaceutical Company Limited, the largest pharmaceutical company in Japan and one of the global leaders of the industry.

The collaboration will focus on the identification of genes or signalling pathways that modify neurodegenerative disease processes affecting neuronal health such as Motor Neurone Disease, Amyotrophic lateral sclerosis, Huntington's and Parkinson's disease.

Support will be provided from the National Institute for Health

Research University College London Hospitals Biomedical Research Centre, for an initial period of 3 years. The work will be carried out at Takeda's research unit based in Cambridge, Takeda Cambridge Limited.

"We are looking forward to collaborating with UCL's world-class researchers. This cooperation will help us to identify and validate novel therapeutic pathways in central nervous system diseases, which is one of Takeda's core therapeutic areas – ultimately leading to new treatments for patients suffering from neurodegenerative disorders." commented Dr. Tetsuyuki Maruyama, General Manager of Takeda's Pharmaceutical Research Division. - *Mudith Jayawardana.*



## KITE PHARMA SIGNIFICANTLY EXPANDS PIPELINE TO TREAT SOLID TUMOR INDICATIONS

Kite Pharma, Inc. has entered into an exclusive, worldwide licence with the US NIH for T-cell receptor (TCR)-based products directed against melanoma-associated antigens (MAGE) A3 and A3/A6 for treating tumors expressing MAGE.

"We believe that our broad portfolio of TCR product candidates, including those targeting MAGE antigens, holds great promise in addressing the significant unmet needs of patients"

commented Dr Arie Belldegrun, Kite's President and CEO.

MAGE-expressing tumors include a number of common solid cancers such as lung, breast, gastric and pancreatic.

The National Cancer Institute's (NCI) Chief of Surgery, Dr Steven A Rosenberg, and special advisor to Kite, is currently conducting two Phase 1–2a clinical trials of TCR-based products that target MAGE antigens under a Cooperative Research and Development Agreement (CRADA) between Kite and the NCI. - *Mudith Jayawardana.*





## LION BIOTECHNOLOGIES OBTAINS EXCLUSIVE LICENSE TO DEVELOP TIL CANCER TREATMENTS

Lion Biotechnologies have been given an exclusive, worldwide license from the US National Institutes of Health to develop and commercialize their tumor-infiltrating lymphocytes (TIL) immunotherapy approach in four additional cancer types. This agreement, which builds upon an already existing licensing arrangement for metastatic melanoma, provides Lion with exclusive rights to certain patents to develop TIL to treat bladder, lung, breast and HPV-associated cancers.

“In addition to the efficacy previously reported in melanoma, we believe that TIL therapy has the potential to demonstrate significant

clinical benefit in the treatment of many solid tumors,” said Dr Elma Hawkins, Lion’s President and CEO.

Lion will make an upfront payment to the NIH, with half payable within 60 days of closing and the remaining balance a year later. Additional milestone payments, which will vary according to indication, will be based on completion of specific clinical, regulatory and commercial achievements. The agreement also calls for royalties to be payable to the NIH based on revenues, and certain additional payments under different sublicense scenarios. - *Mudith Jayawardana*.



## ADURO BIOTECH RECEIVES MILESTONE PAYMENT FOR SUBMISSION OF IND APPLICATION

Aduro Biotech, Inc. announced the receipt of a milestone payment from Janssen Biotech, Inc. for the submission of an Investigational New Drug (IND) Application to the US FDA for Aduro’s LADD immunotherapy, ADU-214. The LADD immunotherapy is being developed for the treatment of non-small cell lung cancer.

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

“We believe there is tremendous potential with our LADD immunotherapy platform and our partnerships, like this one with Janssen, supplement our own efforts and provide additional resources to evaluate the clinical value of our technology in multiple tumor types.” - Stephen T. Isaacs, chairman, president and chief executive officer of Aduro.

The IND will enable Janssen, Aduro’s license partner for ADU-214, to initiate a multi-center Phase 1 trial to evaluate the safety and immunogenicity of intravenous administration of ADU-214, which they hope to initiate by the end of 2015. - *Mudith Jayawardana*.



## KITE PHARMA & ALPINE IMMUNE SCIENCES TO COLLABORATE IN TARGETING IMMUNE SYNAPSE

Kite Pharma, Inc. have entered into a worldwide research and license agreement with Alpine Immune Sciences, Inc. (AIS) to discover and develop protein-based immunotherapies targeting the immune synapse to treat cancer. The partnership will aim to accelerate Kite's efforts to establish the next generation of T cell therapies specifically aimed at tackling the immune-inhibitory mechanisms found in the tumor microenvironment.

AIS will grant Kite an exclusive license to two programs from its transmembrane immunomodulatory protein (TIP™) technology, which Kite will use to further engineer into CAR and TCR candidates.

"We believe the ability of AIS' TIP™ technology to modulate the immune synapse can be incorporated into engineered T-cell therapies to advance CAR and TCR product candidates into multiple tumor types. This collaboration is another example of Kite's continuing commitment to advancing our pipeline through transformative technologies grounded in innovative science." Stated Arie Beldegrun, Chairman, President and CEO of Kite.

Kite will make an upfront payment of \$5 million to AIS, with additional payments to support their ongoing research. AIS will be eligible to receive milestone payments based on the successful achievement of pre-specified research, clinical, and regulatory milestones. - *Mudith Jayawardana.*



## J&J FURTHER SETTLES IN CANADIAN BIOTECH SCENE WITH \$690M IN R&D DEALS

With their new R&D outpost scheduled for opening in spring 2016, Johnson & Johnson have further strengthened their Canadian ties through a number of collaborative deals worth up to \$690 million with Canadian startups, Novera Therapeutics and enGene. The partnerships with Novera and enGene will focus on projects in oncology and inflammatory bowel disease (IBD) respectively.

enGene Inc., based in Montréal, has developed a flexible nucleotide

(DNA and RNAi) delivery technology targeting mucosal tissues to treat numerous prevalent, chronic diseases via the induction or suppression of protein expression levels. J&J put up an undisclosed starter payment and equity investment to collaborate with enGene on EG-12, a therapy to treat IBD by targeting cells lining the intestine. Up to \$341 million will be paid out by J&J throughout the collaboration, with the option to take full ownership of EG-12 after the proof-of-concept stage. J&J

have also been given rights to use en-  
Gene's gene-delivery technology on a  
second, undisclosed target.

As mentioned, this news is hot  
on the heels of J&J's announcement  
last month that they plan to devel-  
op a 40,000 square foot biotech

incubator in Toronto with room  
for up to 50 startups, as part of the  
company's growing JLABS pro-  
gram. This development will pro-  
vide tenants the resources for their  
work on early-stage drugs and tech-  
nologies. - *Mudith Jayawardana*.



## VOYAGER GOES PUBLIC WITH \$86M GENE THERAPY IPO

Despite fears that the window for  
IPOs is closing for biotech compa-  
nies, Cambridge, MA-based Voy-  
ager Therapeutics has filed its IPO  
with hopes of raising \$86 million  
to fund its early-stage work on gene  
therapy for CNS diseases.

Voyager's frontrunner gene therapy  
is a Phase I treatment approach that  
uses an AAV vector to deliver a thera-  
py for Parkinson's disease. Delivery of  
the enzyme, aromatic L-amino acid  
decarboxylase (AADC) to the brain  
can promote the conversion of L-do-  
pa into dopamine to help alleviate  
the symptoms of the disease. Other

products in development at Voyager  
include preclinical treatments for oth-  
er conditions of the CNS, including  
Amyotrophic Lateral Sclerosis and  
Huntington's disease.

Earlier this year, Big Pharma  
Company, Sanofi collaborated with  
Voyager, providing a \$100 million in  
cash and offering up to \$745 million  
in milestones for successful pipeline  
work, with Voyager keeping the US  
commercialization rights.

All eyes will be on Voyager's IPO  
which will either calm or fuel the  
jittery biotech market. - *Mudith  
Jayawardana*.



## IPO ACTIVITY CONTINUES

This past month more  
gene therapy and bio-  
tech companies IPO'd. In  
the gene therapy space it  
was Dimension who made  
their debut and although  
their share price at IPO  
was below their initial  
estimates they still man-  
aged to raise a significant  
amount of cash for the

business. In the after-mar-  
ket, like many other bio-  
technology companies  
that have floated recently,  
their share price fell back  
a bit. So the question that  
the City and Wall Street  
are now asking is 'is the  
IPO window in the USA  
about to close shut?'. Only  
time will tell, but it will  
be interesting to watch  
what happens to Voyager,  
a gene therapy company

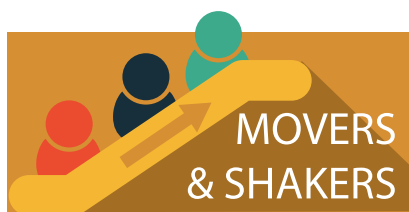
developing treatments for  
CNS diseases who have re-  
cently filed for their IPO.  
Watch this space as they  
say – the IPO window has  
been open for a long time  
now in the USA and inevi-  
tably it will close – but let's  
hope it's not just yet as it is  
clear that companies that  
have raised funds this way  
have definitely prospered  
and moved their products  
along. - *Alan Boyd*



## NORTHWEST BIO ENTERS INTO \$30 MILLION AGREEMENT

Northwest Biotherapeutics, a US company developing personalized dendritic-cell-based vaccine, DCVax®, for the treatment of solid tumors, announced that they have entered into a funding agreement worth \$30 million with UK-based Woodford Investment Management. The current purchase of 5,454,545 shares (at \$5.50 a piece) brings Mountford's total ownership to 28.1%.

Linda Powers, CEO of NW Bio commented on this new agreement, "NW Bio has reported encouraging interim clinical data from both its DCVax-L and DCVax-Direct clinical programs, both last spring and recently, with patient survival exceeding expectations. With this new funding from Woodford we look forward to moving these clinical programs ahead vigorously while continuing to build our organization." - *Mudith Jayawardana*.



## MOVERS & SHAKERS



## MINA THERAPEUTICS APPOINTS DAVID BLAKELY AS CSO

MiNA Therapeutics has appointed **Dr David Blakely** as their CSO, leading the company's R&D activities.

"I am thrilled to join the excellent team at MiNA Therapeutics and am looking forward to supporting the Company, building on their successful discovery and

development of RNA activation therapies."

Dr Blakely previously worked for AstraZeneca, holding numerous roles within research and development for over 28 years. Serving as Chief Scientist in Oncology, Dr Blakely developed AstraZeneca's strategy in therapeutic oligonucleotides, involving collaborations with Isis Pharmaceuticals, Regulus

Therapeutics and Moderna Therapeutics. Dr Blakey led AstraZeneca's first major collaboration in therapeutic antibodies with Abgenix, leading a team that identified 36 therapeutic targets in the space of 3 years and progressed a pipeline of fully human antibodies including the MEDI4736 PDL-1 antibody, currently in Phase III clinical development.



## NANTKWEST APPOINTS NEW SENIOR VICE PRESIDENT OF GLOBAL MANUFACTURING

NantKwest, Inc. has appointed **Dr Stephen Farrand** as SVP of Global Manufacturing. NantKwest is a clinical stage immunotherapy company that specializes on harnessing the power of the innate immune system through the use of natural killer cells as a

treatment for cancer, infectious diseases and inflammatory disease.

Previously, Dr Farrand acted as Vice President Bioprocess Development for Merck Research Laboratories. His leadership played a pivotal role in the successful development and commercialization

of Merck's PD-1 checkpoint inhibitor, Keytruda.

"Dr. Farrand is an experienced executive, with an impressive track record of achievement in chemistry, manufacturing and control (CMC) development," said Dr Patrick Soon-Shiong, CEO, NantKwest.



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