Commercial insight: cell and gene therapy

SEPT 2015

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: Competition in the development of chimeric antigen receptor (CAR) and T-cell receptor (TCR) products is aggressive, and has been a driver in M&A transactions in the space for the last 12 months as cell-based immunotherapy companies vie for auxiliary assets to enhance core platforms and capabilities. This past month, Aduro Biotech joined the ranks of Juno Therapeutics and Kite Pharma by acquiring Netherlands-based BioNovion for €29 million, a private company with a portfolio of antibody candidates and a discovery platform for development of novel CARs. In March of this year Kite Pharma purchased T Cell Factory, also located in the Netherlands, which gave the company a platform for discovery of novel TCRs. A few months later Juno followed suit with the purchase of Boston-based X-BODY, a company with a platform for the discovery of fully human antibodies. Whereas, historically, companies may have struck up research collaborations to access these platforms, large IPOs and private placements have endowed immunotherapy companies with the cash to bring expertise in-house. This is a trend we are also seeing on the manufacturing front, where Juno, Kite, and Adaptimmune are in the process of building-out GMP cell manufacturing capabilities

GENE THERAPY
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GENE THERAPY: This month's news stories have been all about fund raising and IPOs but it is noticeable that this is all focused on the USA, with even companies based outside of the USA turning to the USA for investment. The USA has, for a long time, been the place to go to raise money for life science companies but why are we not seeing more activity on the other side of the Atlantic? There have been a few IPOs over the past year in Europe but nothing like the numbers we have seen in the USA nor raising the same amounts of monies that have been raised there. It is difficult to know why - is it that Europeans are more risk averse or is it related to overall European economy? A question I think that should be addressed.

PHASE 2 STUDY OF METASTATIC MELANOMA THERAPY YIELDS PROMISING RESULTS

New data has been released from an ongoing Phase 2 clinical trial conducted at the National Cancer Institute (NCI) using cancer immunotherapies developed by Lion Biotechnologies, Inc. for metastatic melanoma. The randomized trial involves the use of tumor-infiltrating lymphocytes (TILs) in patients with advanced metastatic melanoma, some of whom were refractory to checkpoint inhibitors.

Patients undergoing TIL treatment were found to exhibit positive long-term, durable objective response rates (ORR). Data presented from the study at the recent AACR Cancer Immunotherapy Conference demonstrated that patients could maintain a positive response ranging from 30 to 47 months after receiving TIL treatment.

The 101 recruited patients were assigned to one of two treatment protocols: i) TIL treatment using chemoablation or ii) TIL treatment followed by total body irradiation. There was an overall response rate of 54% in both treatment arms combined, with no major differences between the two groups. Further to this, 35% of patients demonstrated no disease progression at 4 years.

"We hope to continue along this trajectory in our recently initiated Phase 2 study in the treatment of refractory metastatic melanoma." Elma Hawkins, Lion's President and CEO. - *M Jayawardana*

BENITEC EXPANDS HEPATITIS C TRIALS TO FOURTH SITE

Benitec Biopharma's ongoing Phase 1/2a trial of TT-034, a treatment targeting hepatitis C, has added a fourth clinical site in Texas, USA. The new site, at the Methodist Health System Clinical Research Institute in Dallas, led by principal investigator Parvez Mantry, has prescreeing already established, and joins the other trial sites, including Duke Clinical Research Institute, University of California in San Diego, and the Texas Liver Institute.

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The Sydney-based gene therapy company uses its technology of DNA-directed RNAi to develop gene therapies around RNA interference. Its lead candidate, TT-034, is intended as a treatment for hepatitis C, by reducing its viral load, through targeting liver cells with short hairpin RNAs against three specific areas of the hepatitis C genome.

The addition of the new site to the trial comes as Benitec starts to utilize funds obtained in August, when Benitec listed on the Nasdaq, and raised $14 million through an offering of 1.5 million shares, at $9.21 a piece. - *D Babra*

TREATMENT FOR AML RECEIVES EU ORPHAN DRUG DESIGNATION

The announcement came from Stemline Therapeutics, Inc. that their treatment for acute myeloid leukemia (AML), SL-401, was granted Orphan Drug Designation by the European Medicines Agency. SL-401 currently holds the same designation in the USA for the treatment of AML and Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN).

"SL-401's orphan designation from regulators in both the USA and Europe provides Stemline with several potential avenues to accelerate clinical development in AML and other orphan indications." Eric K Rowinsky, Stemline's CMO and Head of R&D.

SL-401 directly targets specific receptors found on cancer stem cells and tumor bulk of hematologic cancers, including AML and BPDCN. Currently, SL-401 is being evaluated in 3 trials across 7 indications with clinical programs including trials in BPDCN, early and late stage AML as well several high risk myeloproliferative neoplasms. - *D Babra*

BLUEBIRD ON THE MOVE

Massachusetts-based Bluebird Bio will relocate to a new 253,000-square-foot headquarters in Kendall Square, in the latter part of 2016.

The gene therapy company, who went public in 2013, with 184 people on staff at its Second Street lab and office, has signed a 10-year lease for a new space at 60 Binney Street, a building that is presently under construction, through a partnership with Alexandria Real Estate Equities Inc.

Bluebird Bio remains one of Massachusetts' most highly valued, with a current value of $3.3billion, and share prices that have more than tripled since going public.

Alexandria is currently building the new Bluebird headquarters as part of a two-building development, set to expand Kendall Square by 907,000-square-feet at 50 and 60 Binney Street. The new site at 60 Binney Street will place Bluebird within walking distance from the renowned Massachusetts Institute of Technology. - *D Babra*

FIBROCELL & INTREXON RECEIVE FDA FEEDBACK ON NOVEL THERAPY FOR RARE FORM OF EPIDERMOLYSIS

Fibrocell Science, Inc., a cell and gene therapy company focused on developing first-in-class therapies for rare connective tissue and dermatological diseases, and Intrexon Corporation, a leader in synthetic biology, have reported an update on the position of an Investigational New Drug (IND) application for FCX-007, Fibrocell's novel gene therapy targeted at the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a genetic condition which affects the production of collagen type VII, and often results in babies being born missing swathes of skin.

The update included feedback from the FDA on the IND and related largely to a potential Phase I/II clinical trial protocol, as well as toxicology and safety of the therapy. The initial IND included data from a pharmacology/toxicology-based study, based on the injection of FCX-007 into RDEB human skin xeongrafted onto severe combined immunodeficient (SCID) mice, and showed no toxicity. However, as part of their feedback, the FDA have asked for a toxicology-based study involving the injection of FCX-007 into non-grafted SCID mice, which is set to begin in the final quarter of this year.

"Our goal remains the successful development of FCX-007 for the patients and families suffering from this devastating disease, and we expect to initiate the Phase I/II clinical trial in the second quarter of 2016," commented David Pernock, Chairman and CEO of Fibrocell.

Fibrocell intends to amend the IND in response to the feedback from the FDA, by including new data from the requested toxicology study, and expects to re-submit to the FDA during the first quarter of 2016. *- D Babra*

EXPERT PICK

RDEB is a terrible rare disease for which there is currently no approved treatments available. So it is good that companies are trying to find a solution to the problem but, unfortunately the development pathway for products in such diseases is not straightforward as there are no specific regulatory disease guidelines to follow. The information that Fibrocell and Intrexon have now released in relation to their interactions with the FDA are therefore very helpful. They have gone public on what the FDA thinks about their preclinical programme and what additional work is now needed to allow their first clinical trial in patients to get the go ahead. From their release it is clear that in such diseases the FDA want proof of the pharmacological activity of the product followed by the more normal toxicology type studies in an appropriate animal species. This is not 'rocket science' of course, but it is useful to see how companies are going about meeting the regulatory requirements and other product developers should take note. ***- Alan Boyd***

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UNIQURE LOOKS FORWARD TO PHASE 3 TRIALS AFTER SUCCESSFUL GENE THERAPY TRIAL

Netherlands-based UniQure is steaming ahead with its gene therapy program after publishing positive results from a small year-long Phase I/II trial – news that sent their share prices up by 20%.

The gene therapy, AMT-110, is intended to treat Sanfilippo B syndrome, a rare and often fatal, genetic disorder caused by a deficiency in the NaGlu protein. In the four-patient Phase I/II trial, a single dose of AMT-110 generated a significant increase in NaGlu activity after 3 months from 0% at baseline to as much as 17%, an increase that persisted through the remainder of the 12-month trial.

At present, limited data is being made available from the trial, but the company is heralding AMT-110's effect as a promising first. No previous gene therapy trials have shown a persistent restoration of NaGlu over a 12-month period, and past studies have indicated that increasing NaGlu activity by as much as 10% is associated with an increase in life expectancy and a better quality of life in children suffering from Sanfillipo B.

"We are gratified that our pursuit of a gene therapy targeting CNS diseases has found this early and promising success" commented Jorn Aldag, CEO UniQure.

Elsewhere, UniQure is pushing forward with other gene therapy programs, including clinical programs in the treatment of hemophilia B and Parkinson's disease, while being involved with a billion-dollar collaboration with Bristol-Myers Squibb, which is researching gene therapy in cardiovascular disease. However, UniQure's lead candidate, Glybera, is at somewhat of a standstill, following regulatory delays in the USA, after requests from the FDA for further Phase 3 data, prior to considering approval. *- D Babra*

UNIQURE: AMT-110 CLINICAL STUDY RESULTS

UniQure has recently announced the results of a very small study involving AMT-110 for treating the rare disease of Sanfilippo B Syndrome. The study only involved four patients but initial results on the positive effect of the replacement gene on NaGlu enzyme activity are very encouraging, particularly as the effect was persistent after just a single administration for at least 12 months. This is a good signal for the approach and it will be interesting to see what the design of the next study will be. *- Alan Boyd*

PHASE 2 TRIAL OF DNA-BASED THERAPY IN MERKEL CELL CARCINOMA SHOWS PROMISE

OncoSec Medical Inc has announced results from a Phase 2 clinical trial utilizing their DNA-based immunotherapy – ImmunoPulse Interleukin (IL)-12 – in Merkel cell carcinoma (MCC). The results demonstrate that the immunotherapy promotes tumor-specific, anti-tumor immune responses in sufferers of this rare skin cancer.

The outcomes of the trial, headed up by Dr Shailender Bhatia, at the University of Washington School of Medicine, support the hypothesis that "intratumoral IL-12 DNA with electroporation promotes tumor immunogenicity."

Tumor biopsy samples taken from patients subjected to the treatment demonstrated an increase in IL-12 protein levels, indicative of successful DNA transfection and protein expression. No adverse events above Grade 2 were observed in patients, with the most commonly reported being procedure-associated transient pain.

Additional data suggests that ImmunoPulse IL-12 can elicit a tumor-specific T-Cell response with an increase in tumor-infiltrating lymphocytes and $CD8^+$ T-cells, further highlighting the potential of this therapy as a viable solution to sufferers of MCC.

"These results are consistent with what we have previously observed in metastatic melanoma and underscore the broad-reaching potential of ImmunoPulse IL-12 in driving immunogenicity" commented Mai H Le, CMO, OncoSec. - *M Jayawardana*

ONES TO WATCH

INTERLEUKIN-12 REVIVAL

Interleukin-12 (IL-12) was first investigated in clinical studies in the mid-90's to see if its myriad immunoregulatory activities could be leveraged to thwart cancer. However, limited efficacy along with a range of serious adverse events led to the withdrawal of IL-12 from clinical development. Due to significant advances in our ability to engineer cells, the biotechnology field is showing a renewed interest in IL-12. OncoSec Medical is farthest along in clinical development having released results from a Phase 2 study investigating its ImmunoPulse IL-12TM technology (IL-12 plasmid injected intratumorally and electroporated into cells in situ) in patients with Merkel cell carcinoma. In this study, 30% of patients showed regression in distant, non-electroporated lesions, showing that IL-12 can modulate the tumor microenvironment and induce tumor immunogenicity, while 80% of patients exhibited increased IL-12 protein levels in tumor biopsies. Ziopharm is also investigating IL-12 in the clinic and is currently enrolling patients in two Phase 1/2 studies investigating an adenovirus expressing IL-12, under the control of an inducible promoter, for use in patients with breast and brain cancer. - ***Mark Curtis & Rahul Sarugaser***

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IMMUNOTHERAPIES FOR GVHD TO BE DEVELOPED BY INTREXON & ZIOPHARM

Synthetic biology company, Intrexon Corporation, announced a new Exclusive Channel Collaboration (ECC) with ZIOPHARM Oncology, Inc, a biopharmaceutical company whose primary focus is the development of new cancer immunotherapies for the treatment and prevention of graft-versus-host disease (GvHD). GvHD represents a major and potentially fatal complication of allogeneic hematopoietic stem cell transplantation (HSCT).

Allogeneic HSCTs are currently used to treat various diseases that include hematological malignancies, immunological deficiencies and non-malignant conditions. GvHD develops in 40–60% of HSCT recipients when the grafted cells in recognize the host as foreign and begin attacking the patient's own cells. Current treatments for GvHD include immunosuppressive agents and systemic steroids, which have limited efficacy and toxicity.

The collaboration between the two companies will address the need for more efficient, safer treatments through the use of engineered cell platforms to express and deliver the cytokine IL-2, which is critical for the modulation of the immune system. Strategies for targeted GvHD treatment include:

- The infusion of regulatory T cells (Tregs) expressing IL-2 utilizing proprietary gene control approaches (such as Intrexon's Rheoswitch® platform).
- Orally-delivered microbe-based ActoBiotics® therapeutics expressing IL-2.

The agreement sees Intrexon received a technology access fee of $10 million in cash as well as reimbursement of all R&D costs.

Samuel Broder, Senior Vice President and Head of Intrexon's Health Sector stated that "adoptive therapy with gene-modified T cells may offer an exciting alternative approach for restoring 'immune homeostasis' and countering the destructive pro-inflammatory mediators of GvHD." - *M Jayawardana*

"Adoptive therapy with gene-modified T cells may offer an exciting alternative approach for restoring 'immune homeostasis' and countering the destructive pro-inflammatory mediators of GvHD." Samuel Broder, SVP & Head of Intrexon Health Sector.

LICENSING AGREEMENT BETWEEN SERNOVA AND UHN OF TORONTO

Sernova Corp, a clinical stage company that develops medical technologies for the long-term treatment of chronic metabolic diseases including diabetes and hemophilia has announced a licensing agreement with the University Health Network (UHN) of Toronto, Canada providing them with exclusive worldwide rights to patent-pending technologies developed by UHN researchers.

These technologies involve the development of stem cells into glucose-responsive therapeutic cells to be used as a treatment for patients with insulin-dependent diabetes.

Although it's an effective therapeutic approach for T1 diabetes, pancreatic islet transplantation is currently restricted to diabetics with severe hypoglycemia awareness and control due to the limited supply of cadaveric donor islets and the need for life-long use of toxic immunosuppressants. The potential to generate a readily available and unlimited source of glucose-responsive cells for transplantation presents a huge opportunity.

> "We believe that a virtually unlimited supply of glucose responsive therapeutic cells could be generated from ethically derived stem cells which, if shown to have positive safety and efficacy in clinical trials, could be used to treat millions of patients," Delfina Siroen, Sernova's head of Research and Development.

By transplanting these cells within Sernova's Cell Pouch, in combination with additional technologies for immuno-isolation, the hope is that they will be protected from immune detection and attack. *- D Babra*

The push to develop a cell therapy for type 1 diabetes continues. Sernova announced completion of a licensing transaction with the University Health Network (UHN) in Toronto to gain exclusive world-wide rights to a protocol for the derivation of pluripotent stem cell (PSC)-derived pancreatic progenitor cells, which differentiate into glucose-responsive cells *in vivo* following transplant. Earlier this year Semma Therapeutics made its debut with a US$44 million Series A financing to fund development of a PSC platform for the derivation of functioning beta cells. The technology, based on the work of Dr Doug Melton at the Harvard Stem Cell Institute, is the only protocol that can turn PSCs into terminally differentiated beta cells *in vitro*. Now that industry has access to commercially viable sources of cells for transplant it must solve the next grand challenge, immune rejection by the host. Novel approaches to encapsulation with biomaterials will be needed to prevent destruction of therapeutic cells. ***Mark Curtis & Rahul Sarugaser***

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COLLABORATION BETWEEN CALADRIUS BIOSCIENCES & SANFORD RESEARCH IN TYPE 1 DIABETES

Caladrius Biosciences, a cell therapy company specialising in the development of immunotherapies has entered into an agreement with Sanford Research to further development of their T-regulatory cell therapy product candidate CLBS03, which aims at treating adolescents with recent-onset type 1 diabetes. CLBS03 is a personalized treatment that consists of a patients' own regulatory T cells that have been expanded and functionally enhanced.

Sanford Research, a non-profit research organisation, will run the Sanford Project Trutina Study, a Phase 2, placebo-controlled randomised clinical trial evaluating the safety and efficacy of CLBS03 in adolescents. Enrolment of 111 patients across 12–15 sites is planned for early 2016 and the current collaboration sees Sanford provide not only funding support, but also logistical services for the trial such as recruitment, treatment and monitoring.

"We are delighted to join forces with Sanford Research and share the goal of identifying and developing new therapies with the potential to become major advances in the treatment of type 1 diabetes".

commented David Mazzo, Caladrius CEO, on the collaboration. - *M Jayawardana*

EXPANDED COLLABORATION BETWEEN KITE PHARMA & NETHERLANDS CANCER INSTITUTE

The announcement came that Kite Pharma has expanded its collaboration with the Netherlands Cancer Institute (NKI). The agreement will allow Kite to receive the exclusive option to licence multiple TCR gene sequences from the NKI. Access to these sequences will allow for the development and commercialization of cancer immunotherapy candidates which target solid tumors.

"Kite's relationship with the NKI, an internationally renowned cancer research and clinical institution, provides an important operational platform, as we advance TCR-based immuno-oncology product candidates"

Arie Belldegrun, Chairman, President and CEO of Kite.

Kite Pharma EU, which comprises a leading team of immuno-oncology researchers and collaborators, will conduct preclinical research on the candidates developed under the agreement with NKI. - *M Jayawardana*

CELGENE'S $25M COLLABORATION WITH RECOMBINANT ANTIBODY NETWORK

Celgene is stumping up $25 million to become the first industry member of a new research consortium – the Recombinant Antibody Network – which aims to find new and novel anti-cancer therapies through antibody engineering.

RAN was co-founded by UC San Francisco's James Wells, and Sachdev Sidhu and Anthony Kossiakoff of Genentech and is focused on systematically targeting the full range of cell-surface protein targets relevant to a number of diseases including cancer, to potentially find the next generation of therapies. Over the past decade, antibodies have emerged as the major breakthrough in targeted cancer therapy and are now the fastest growing class of therapeutic molecules. Unfortunately, antibody development remains an imprecise science, conducted on a case-by-case basis.

"In the future we envision that we will be able to precisely target cancer cells at the molecular levels, which will provide better therapies for patients" commented Dr Sidhu.

In this first industry partnership for the RAN, Celgene agreed to pay $25 million for the option to enter into future license agreements to develop and commercialize promising therapeutic antibodies to cancer-related targets. In having Celgene on board first, the group has gained a high-profile player within the biotech industry, which will only serve to attract even more members of the oncology field to the table. - *D Babra*

CLINICAL TRIAL AGREEMENT BETWEEN ADURO BIOTECH & INCYTE FOR OVARIAN CANCER TREATMENT

An agreement was reached between Aduro Biotech Ltd and Incyte Corporation to investigate the clinical efficacy of a combination of the companies' immunotherapy candidates – Aduro's immunotherapy, CRS-207 and Incyte's oral indoleamine dioxygenase 1 inhibitor, Epacadostat (INCB24360).

These agents are expected to have differing mechanisms that complement each other by enhancing the body's immune system into fighting ovarian cancer. Epacadostat enhances immune activity by reducing immune suppression typically found in the tumor microenvironment, whilst Aduro's CRS-207 stimulates direct immune activity against cells expressing mesothelin, as found in ovarian cancer.

The collaboration sees equal funding of a planned Phase 1/2 trial of the combination therapy, with an initial Phase 1 study to determine optimal dose levels and a follow up Phase 2 comparing the combination therapy against CRS-207 alone. Enrolment plans currently include up to 40 patients for Phase 1 and up to 86 patients for Phase 2, due to begin in early 2016. - *M Jayawardana*

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CIRM GRANT $19.9 MILLION TO FUND PHASE 3 REGISTRATION PROGRAM IN GLIOBLASTOMA

The California Institute for Regenerative Medicine (CIRM) has provided funding to ImmunoCellular Therapeutics, Ltd to support their Phase 3 registration trial of ICT-107 in patients diagnosed with glioblastoma. This award was provided on the basis that ICT-107 has been specifically engineered as an immunotherapy that targets and kills cancer tumor stem cells in hopes of preventing tumor recurrence following treatment.

ImmunoCellular is planning to initiate the phase 3 ICT-107 trial in Q4 2015, involving approximately 120 clinical sites in the USA, Europe and Canada, targeting the recruitment of about 400 patients.

> " Our strategic focus going forward will be on advancing our Stem-to-T-cell immunotherapy platform and pursuing additional related technologies to grow our pipeline – strategies that are key to achieving our goal of building a leading cancer immunotherapy company."

commented Andrew Gengos, ImmunoCellular President and CEO. - *M Jayawardana*

REGENX RAISES $138 MILLION THROUGH IPO

One could quite successfully argue that RegenX Bio, the leader in AAV gene therapy, has given itself a solid financial platform upon which to build its position in the industry following the news of a $138 million IPO, raised through the sale of 6.3 million shares at $22 a piece.

The news added 37% to the price of RegenX's shares, seeing an increase above $30 per share. The success of the IPO has even appeared to quell some recent uncertainty around the biotech market, paving the way forward for the ever-growing number of biotech companies aiming to go public in the last part of this year.

Recent years have seen the success of a huge number of IPOs, which have brought billions of dollars into the industry; recent assessments suggest even more growth, with 55 new IPOs this year. *D Babra*

Last month I commented on the fact that RegenX were planning to go to IPO to raise funds for their own in-house development programmes. For this month it is therefore good to see that they made it and raised $138 million followed by over a third in the increase of their share price on the day of the float. Clearly the IPO window is still firmly wide open and having obtained this funding now the pressure will be on RegenX to deliver their in-house programmes. Watch this space! - ***Alan Boyd***

AVEXIS RAISES IN $65M IN SERIES D

Gene therapy company, AveXis, has raised $65million in funds to help push forward with a novel gene therapy targeted at Spinal Muscular Atrophy (SMA), a rare and serious condition, for which there are currently no approved therapies.

SMA affects approximately 1 in 10,000 babies, and is non-discriminatory when it comes to gender or race. The disease is caused by a deficiency in the SMN protein, resulting in the destruction of motor neurons, leading to gradual disability, with many losing the ability to walk, eat or breathe.

The funds will be used to continue work focusing on AveXis' lead candidate, scAAV9.CB.SMN, a novel Phase I gene therapy for SMA Type 1. The therapy, which utilizes AAV technology, pioneered by RegenX Bio, is designed to deliver corrected forms of the SMN gene, and thus stop progression of the disease.

The Series D, led by T Rowe Price-managed funds, saw the return of past investors Deerfield Management, Roche Venture Fund and Venrock, as well as the arrival of some new financiers, including Janus Capital, RA Capital and Foresite Capital. This most recent fundraise follows a $10million C-round, which closed in January 2015.

The investment from Roche support's its commitment to finding an effective treatment for this unmet medical need and follows its earlier partnership with PTC Therapeutics, valued at $490 million, and the purchase of Trophos in January, for $545million – both companies with a focus on SMA.

> "We are now well-positioned to continue development of our novel gene therapy for patients suffering from SMA Type 1, a rare genetic disease and the most common genetic cause of infant mortality."

commented AveXis CEO Sean Nolan.

Other companies in the SMA space include Isis Pharmaceuticals who published promising news from its ongoing mid-stage program earlier this year (in collaboration with Biogen), while Pfizer and Repligen entered into a $70million partnership.- *D Babra*

ONES TO WATCH

AVEXIS – BACKING BY ROCHE

AveXis is developing a gene therapy approach to treat the rare condition of spinal muscular atrophy and has just completed a successful Series D fund raise of $65 million. The interesting point here is that big pharma are also involved again in the deal in the form of Roche Ventures. I noted last month that big pharma's interest in the gene therapy space has increased enormously over the past couple of years and here is proof that they continue to be interested even for rare diseases. Long may this continue. ***- Alan Boyd***

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DIMENSION THERAPEUTICS EYEING UP $115M IPO

Dimension Therapeutics, a Cambridge, MA-based gene therapy company, have filed the terms for a $115million IPO. The IPO looks to further strengthen their financial stability with Dimension having reported at least $80 million in the bank in June this year having raised funds from investors and through a partnership with Bayer.

The IPO comes just days after the FDA accepted an IND from the company for their lead gene therapy candidate – a genetic cure for hemophilia B. Hemophilia B, an x-linked recessive hematological disorder caused by the absence of Factor IX, leads to abnormal clotting. Dimension's lead candidate, DTX101, uses an AAV vector, technology pioneered and outlicensed from RegenX Bio, to deliver Factor IX to hemophiliacs, and therefore restore the normal clotting process. *- D Babra*

Plans for IPO: With the IPO window firmly open at the moment it comes as no surprise that Dimension is planning to seize the opportunity and go for an IPO. Having successfully raised $65 million from investors in April plus the money from the deal with Bayer, raising the $115 million that they are expecting to do from the IPO should secure their ability to take their three development programs forward. ***- Alan Boyd***

PRECISION BIOSCIENCES UPDATE MANAGEMENT TEAM

Precision BioSciences, a North Carolina-based company specialising in genome editing, have announced some high-profile additions to their executive team. The company have appointed two new Senior Vice Presidents to the management team – **Gene Liau** as Senior Vice President of Gene Therapy, and **Bruce McCreedy**, as Senior Vice President of Cell Therapy. CEO Matthew Kane has cited their leadership qualities and wide experience as key factors in their appointments, and has high hopes for their abilities in the company's product development programs.

CYTORI APPOINTS VP & GM OF CELL THERAPY

Cytori Therapeutics Inc. announced that **Mr John D Harris** has been appointed as Vice President and General Manager of Cell Therapy, a newly created role to be based in Tokyo, Japan. The role will focus on growth and development of the company's existing and future global cell therapy commercial operations. Harris joins Cytori from Becton Dickinson, where he served as President of their Japanese operations, which include a production facility, over 550 employees, and approximately $315 million in revenues.

ADAPTIMMUNE EXPANDS SENIOR TEAM

Adaptimmune Therapeutics plc, a clinical stage biopharmaceutical company focused on the use of T-cell therapy to treat cancer, have added a trio of new members to their management team to support the Company's growth.

Trupti Trivedi joins as Vice President, Head of Biometrics and brings over 20 year's experience in pharmaceutical and biotech development having worked in companies such as GSK and Roche.

Michael Blackton joins as Vice President, Quality Assurance and CMC, having previously worked at Eli Lilly for 11 years.

Joseph Apostolico has joined as Vice President and Global Director, Human Resources, bringing with him over 28 years' of experience.

NEW SVP OF BUSINESS DEVELOPMENT AT ONCOSEC

OncoSec Medical Incorporated, a company developing DNA-based intratumoral cancer immunotherapies, has appointed **David P Meininger** to the newly created position of Senior Vice President of Business Development. Meininger will be responsible for developing new business and strategic partnerships as well as potential licensing opportunities.

"I'm thrilled to welcome David to the OncoSec team. David brings more than 20 years of experience in basic and applied scientific research, and has led growth-oriented teams at prominent biotech and pharmaceutical companies, including Merck said Punit Dhillon, CEO and President of OncoSec.

KITE BOLSTER BOARD OF DIRECTORS WITH APPOINTMENT FORMER ROCHE CHIEF EXEC

Kite Pharma, Inc announced that **Dr Franz B. Humer,** the former Chairman and Chief Executive of Roche Holding Ltd., has been appointed to the Company's Board of Directors.

Dr Humer stated, "Kite Pharma is at the forefront of advancing cancer T cell immunotherapy which, in my view, is one of the most promising new approaches in oncology. Kite's programs have the potential to change the paradigm of cancer treatment, and I look forward to working closely with the accomplished team at Kite."

NANTKWEST BOLSTERS SENIOR MANAGEMENT TEAM WITH THREE NEW APPOINTMENTS

NantKwest, Inc. a clinical-stage immunotherapy company has appointed three senior-level executives.

Andreas Niethammer joins as executive medical director having previously held the position of VP clinical development at Poseida Therapeutics; **Dvorit Samid** joins as senior vice president of medical affairs with over 30 years' experience in oncology R&D; and **Jerel A Banks** joins as senior vice president M&As having previvously served as VP, portfolio manager and research analyst at Franklin Templeton Investment.

"With these appointments, we have substantially augmented and strengthened our senior management team with accomplished executives," said Patrick Soon-Shiong, chairman and CEO of NantKwest. "Drs Niethammer, Samid and Banks bring deep and relevant expertise, knowledge and experience in critical roles to our organization, as well as strong leadership and strategic direction to help propel NantKwest forward."

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