Commercial insight: cell and gene therapy

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

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

CELL THERAPY
Mark Curtis
Financial Portfolio Manager,
Emerging Technologies
Lonza AG
Switzerland

GENE THERAPY: This month sees plenty of activity in the start-up arena, with new companies being formed to advance gene therapy treatments for alpha-1 antitrypsin deficiency (Apic Bio), Parkinson's disease (Prevail Therapeutics) and complement-mediated diseases (Aevitas Therapeutics), and an IPO filed by Krystal to support its epidermolysis bullosa gene therapy; a joint venture between Agilis Biotherapeutics and Gene Therapy Research Institution Company Ltd completes the picture, although the disease targets for this collaboration are not disclosed. There certainly seems to be no lack of investment funds, even for the most challenging of targets like Parkinson's disease, or for targets like alpha-1 antitrypsin deficiency, where an enzyme replacement therapy is already available.

CELL THERAPY: it was a celebratory month for the cell therapy industry. Novartis received the world's first approval for a CAR-T therapy. Ctl019, which will be marketed under the tradename kymriah (tisagenlecleucel), it was approved for the treatment of B-cell acute lymphoblastic leukemia in children and

young adults. As the body of clinical evidence grew over the last few years, it became increasingly clear that the therapy would offer patients a giant leap in standard-of-care. The decision from the FDA was a resounding – yes. The therapy will initially be priced at US$475,000. Just days later, in one of the most significant deals in recent times in the biotech industry, Novartis' rival Kite pharma was acquired by Gilead, in an all-cash deal, for a staggering US$11.9 billion. The deal is positive for patients overall, as the deep pipeline of car and tcr candidates assembled by Kite is now backed by the financial might needed to bring them to market.

FIRST LOT OF ASTERIAS' AST-VAC2 FOR LUNG CANCER MANUFACTURED SUCCESSFULLY

Cancer Research UK, supported by Asterias technical personnel has completed the manufacture of current Good Manufacturing Practice (cGMP) clinical grade AST-VAC2, which meets all specifications for release. This first lot of Asterias' non-small-cell lung cancer gene therapy is intended for use in the first clinical study of the drug.

AST-VAC2 is an allogeneic treatment comprising of dendritic cells derived from pluripotent stem cells, more specifically human embryonic stem cells. The cells are engineered to express a form of telomerase, which is commonly found in tumors but rarely occurs in normal cells. This is intended to enhance the immune system response to tumors, in conjunction with other immunotherapies.

Under the company's 2014 agreement with Cancer Research UK, Asterias has transferred its innovative laboratory scale AST-VAC2 manufacturing process to Cancer Research UK's Biotherapeutics Development Unit. Cancer Research will also conduct the initial trialing of the therapy.

"The successful production of this first cGMP lot of AST-VAC2 is a major step towards initiating the upcoming study in non-small-cell lung cancer," said Mike Mulroy, President and CEO. "With its potential as a ready-to-use, off-the-shelf cancer immunotherapy, AST-VAC2 represents an exciting opportunity for Asterias in the rapidly evolving cancer immunotherapy sector."

ABEONA THERAPEUTICS TO EXPAND MPS III AAV GENE THERAPY TRIAL

Abeona therapeutics has announced the expansion of its clinical trial for the gene therapy ABO-102 to treat the mucopolysaccharide disease MPS III, also known as Sanfilippo syndrome. An additional eight to

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ten patients are expected to enrol onto the trial by the first quarter of 2018.

ABO-102 is an adeno-associated virus (AAV) vector based therapy that delivers a corrective copy of the gene underlying MPS III. It is administered via a single intravenous injection and encouraging results from ongoing trials have prompted Abeona to initiate this acceleration of their plans.

Patients will be enrolled in Europe and Australia and will be monitored for safety data, bipotency and clinical activity. Of particular note is the successful delivery of ABO-102 to the central nervous system – one of the therapy's target tissues.

Chief Medical Officer Juan Ruiz commented, "We have completed the necessary regulatory and ethical committee approvals and site initiations in Europe and Australia in order to accelerate enrollment. We remain very encouraged by the improvements observed in clinically relevant biomarkers post-dosing of ABO-102, including durable reductions in heparan sulfate measured in the CNS, reduction of organ disease pathology, and signals of CNS improvement or stabilization at one-year follow-up in Cohort 1 subjects, and look forward to providing a more fulsome clinical update at important clinical conferences, including ESGCT, this fall."

Encouraging news from Abeona Therapeutics with the expansion of its Phase 1/2 clinical trial in Sanfilippo type A (MPS IIIA) to pivotal status. Sanfilippo A, one of four subtypes and the most severe form of the condition, is caused by mutations in the *SGSH* gene, leading to deficiency in heparan N-sulfatase and accumulation of glycosaminoglycans. The condition manifests in early childhood with hyperactivity, sleeplessness, loss of speech and cognitive skills, cardiac issues, seizures and loss of mobility; death usually occurs before adulthood. ABO-102 is an scAAV9 vector administered intravenously to deliver the *SGSH* gene systemically, including the CNS. Preliminary data in three treated children – published earlier this year in abstract form – are encouraging, with reductions in heparan sulfate fragments in urine and CSF, and decreases in liver volume on MRI. The treatment is administered with a minimum 60-day course of oral prednisolone, which appears to minimize immune response to the vector. Serum transaminases remained normal and there were no significant T-cell responses in the first three children studied. – Richard Philipson

BLUEBIRD BIO TO TARGET EUROPE FOR GENE THERAPY FILINGS

bluebird bio will target European markets for their gene therapies before the USA. The strategy is based upon what the company sees as a favorable regulatory pathway that allows companies to submit and have data approved in stepwise stages.

The company has stated that they are undergoing discussions

regarding potential regulatory filings with both the European Medicines Agency (EMA) and the Medicine and Healthcare products Regulatory Agency (MHRA) of the UK. Separate discussions with the two agencies is a cognizant approach with Brexit impending.

bluebird hopes that data from their Northstar trials – which failed to meet primary targets – combined with data from follow-up trial Northstar-2 "could support the filing of a marketing authorization application in the EU" for transfusion-dependent thalassemia.

NOVARTIS WINS THE CAR-T RACE

The US Food and Drug Administration (FDA) has approved Novartis' CTL019 (tisagenlecleucel-T), an investigational CAR-T therapy designed to treat patients with relapsed and refractory B-cell acute lymphoblastic leukemia. The therapy will be marketed as Kymriah.

A ten member expert panel convened by the FDA had unanimously voted in favor of this CAR T therapy in July 2017. Dr Tim Cripe, an oncologist who was part of the FDA advisory committee panel that voted in favor of the approval commented: "I think this is most exciting thing I've seen in my lifetime."

CTL019 was first developed by the University of Pennsylvania, and in 2012 Novartis entered into a strategic license agreement with the University of Pennsylvania to further the research, development and commercialization of CAR-T therapies.

The most important question now is the price of the therapy. Although Novartis hasn't disclosed how much it intends to charge for Kymriah, experts predict it could cost as much as US$700,000 for a course of treatment.

The FDA is also expected to make a decision about another CAR-T treatment from Kite Pharma, which was just acquired by Gilead Sciences, for treating aggressive B-cell non-Hodgkin lymphoma.

FIRST PATIENT TREATED WITH CELLECTIS' ALLOGENEIC CAR T PRODUCT IN PHASE 1 TRIAL

The first patient in a Phase 1 study of Cellectis' UCART123 has been administered with the gene-edited chimeric antigen receptor (CAR-T) cells (UCART) at the MD Anderson Cancer Center in Texas. The treatment is aimed at a rare and aggressive form of acute myeloid leukemia (AML), Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN).

UCART123 is an allogeneic product that employs TALEN® gene-edited CAR T cells derived from donors. The engineered cells

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target CD123, an antigen expressed at the surface of leukemic cells. The Phase 1 trial being led by professors at the MD Anderson Cancer Center will evaluate the safety and efficacy of UCART123. There are currently no treatments for the cancer, which has been compounded by its recent classification as a distinct clinicopathological entity.

Chief Medical Officer of Cellectis Loan Hoang-Sayag commented, "We are eager to progress through clinical trials with UCART123, Cellectis' wholly controlled gene-edited product candidate, next with the treatment of BPDCN, rare but aggressive entity. With this innovative treatment, the hope is that our 'off-the-shelf' approach will transform the way we think about cancer care and serve as the next step in curing this disease through the power of gene editing."

TOUCHLIGHT GENETICS EXPANDS ITS OPERATIONS IN LONDON

Touchlight Genetics Ltd, a biotechnology company focused on developing gene technologies has developed a DNA technology that can synthetically manufacture commercial scale DNA in a 2-week process – a technology that is disrupting the decades old fermentation approach. The process uses two enzymes, and basic benchtop laboratory equipment to produce DNA of any sequence for therapeutic applications. Expansion of Touchlight's DNA platform, which aims to underpin the success of advanced medicines, will directly support this mission to establish the UK life sciences sector as a world leader.

Touchlight's operations are expanding in order to support their commercial product collaborations with large pharmaceutical and biotechnology companies around the world. Additionally, with the fastest and most scalable DNA technology, Touchlight looks to drive forward the next generation of DNA vaccines, that are safer, more effective, faster to manufacture and easier to distribute to resource-constrained environments.

Touchlight is expanding operations into a tailor-made laboratory, within the restored Hampton Water Works situated on the River Thames. This grade-two listed facility was formed in 1852 by Joseph Quick in response to the London Cholera epidemics, and was a part of some of the greatest life-saving innovation of the era. Operating until 1950, the buildings were left largely untouched until they were purchased in 2012 by Mr Andrew Black, Co-Founder of Betfair.

Andrew's vision is to utilize the 4,000 square meters of Victorian architecture to create a new scientific community. "Science, and biotech as a daughter, is often forgotten as one of the great creative industries. Most other creative industries put great onus on a conducive working environment, but less so in Biotech where laboratories are often subterranean and utilitarian. I wanted to create a light and inspiring atmosphere, one to support great

science, like the work going on at Touchlight."

The first phase of Touchlight's laboratory expansion is now complete, with the second phase to be completed in November. The expansion means that Touchlight is now ideally placed to enable a revolution in medicine with its cutting-edge DNA technology.

FAST TRACK DESIGNATION FOR IOVANCE'S LN-144

Iovance Biotherapeutics has received fast track designation from the FDA for its autologous cell therapy, LN-144, based on tumor infiltrating lymphoctye (TIL) techonology, for the treatment of advanced melanoma.

LN-144 is being trialled in the Phase 2 study C-144-01. Early results from the study found a clinically significant 77% of patients enrolled in the first cohort experienced a reduction in tumor size. The study is expected to enrol up to 60 patients with the second cohort receiving a cryopreserved product.

The FDA's Fast Track process is designed to facilitate the development, and expedite the review of drugs that treat serious conditions and fill an unmet medical need. Fast Track designation allows more frequent meetings and communications with the FDA to discuss the drug's development plans and review process. The Fast Track designation also allows for a rolling review of a company's Biologic License Application.

Maria Fardis CEO commented, "We are pleased that the FDA has granted Fast Track designation to LN-144 for the treatment of advanced melanoma. The Fast Track designation underscores that advanced melanoma remains a serious condition and that LN-144 may have the potential to address this unmet medical need. We look forward to a closer interaction with the FDA as we advance the clinical development of LN-144 for the treatment of advanced melanoma."

KITE SUBMITS IND FOR BCMA TARGETING CAR-T THERAPY

Kite Pharma has applied to the FDA to initiate trialling of another CAR-T candidate – this time to target B-cell maturation antigen (BCMA) in patients with relapsed/refractory multiple myeloma. The investigational new drug (IND) candidate is called KITE-585 and has yielded encouraging results at the pre-clinical stage of testing.

BCMA is expressed on the surface of malignant plasma cells in most patients with multiple myeloma. In addition, it is found on normal plasma cells and certain mature B-cell lineage cells but is absent from other tissues. KITE-585 has been found to induce T-cell expansion in the presence of BCMA, and its activity has not

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been demonstrably affected by soluble BCMA.

Chief development officer David Chang commented, "KITE-585 has the potential to become Kite's next significant advance in cell therapy for patients with cancer. It is the result of an extensive preclinical development effort that included candidate screening, engineering, and testing by Kite's internal research team and it reflects the company's deep experience in CAR design and cellular therapeutics. As we look ahead, we are confident that the cutting-edge design and manufacturing process of KITE-585 together with our proven capability with engineered T cells will support rapid execution of the clinical program."

In additional news this month, Kite has extended the safety expansion cohort of its ZUMA-1 chimeric CAR-T trial into the European Union. Patients with relapsed/refractory diffuse large B-cell lymphoma (DLBCL), primary mediastinal B-cell lymphoma (PMBCL) and transformed follicular lymphoma (TFL) are being enrolled and treated with the company's lead product candidate axicabtagene ciloleucel in centers across the EU. This follows Kite's landmark filing of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for the therapy in July; Europe's first CAR-T application. The first patient in the cohort has been treated at the Academic Medical Center in Amsterdam with the drug, which targets the antigen CD19, a protein expressed on the cell surface of B-cell lymphomas and leukemias, and redirects the T cells to kill cancer cells.

Kite submitted an IND this past month for KITE-585, a CAR-T therapy that targets BCMA in multiple myeloma. Kite will go head-to-head with some formidable competition though, once in the clinic. Both bluebird bio and China-based Nanjing Legend are developing CAR-T therapies targeting BCMA, the latter of which has posted some very impressive early clinical efficacy data – a 94% response rate in a group of 35 patients. – Mark Curtis

AGILIS ENTERS JOINT VENTURE WITH GTRI FOR DEVELOPMENT OF AAV VECTORS

Boston-based Agilis Therapeutics has entered a manufacturing and collaboration partnership joint venture with the Japan headquartered corporation Gene Therapy Research Institution (GTRI). Focused on developing gene therapies for diseases which affect the central nervous system, Agilis will look to for support in advancing adeno-associated viral (AAV) vector-based therapies.

Earlier this year, Japanese Ministry of Trade, Economics and Industry (METI) and Japan External Trade Organization (JETRO) awarded a grant for the development of a state-of-the-art AAV manufacturing facility in Japan. The JV between Agilis and GTRI was initiated on the back of this. The process development and production facility in the Tokyo area that will be operated by GTRI is designed to meet international manufacturing standards, and thus is suitable for participation in the manufacturing of Agilis' therapeutics. The JV will be using Sf9 baculovirus and HEK293 mammalian cell systems to develop and optimize AAV vectors, as well as working on expediting the development, approval and commercialization of select gene therapies in specific CNS diseases.

Mark Pykett, Agilis CEO, commented, "We believe that our partnership will enhance the efforts of both organizations, build important shared production capabilities, and accelerate development and commercialization of important gene therapies. We look forward to working with GTRI on a range of initiatives." Co-director of the JV and GTRI CEO Katsuhito Asai further stated, "Our partnership will seek to capitalize on the strong recent progress in the field of gene therapy and expedite the development of innovative gene therapies for patients in need, with a major emphasis on the quality production of safe, effective therapeutics."

SORRENTO CONTRIBUTES TO FORMATION OF PLACENTAL CELL THERAPY COMPANY CELULARITY

Sorrento Therapeutics along with Celgene, Human Longevity Inc., and United Therapeutics among others have collaborated to create Celularity Inc in a recently closed Series A round. The newly formed company will accelerate cell and tissue regenerative therapies to address unmet medical needs in cancer and chronic and degenerative disease.

Contributions of extensive intellectual property, clinical-stage assets, basic and clinical research, and development expertise bolster the company's work into harnessing the potential of the human placenta for cell therapies. Based on the work of former Celgene CEO Dr Robert Hariri, Celularity is equipped to procure placental stem cells, engineer potential therapies and deploy potential treatments.

Dr Hariri commented, "Celularity was formed as a new biotechnology model designed to apply the necessary expertise to harness our placenta discovery platform across a range of unmet medical needs. With the support of our investors, we are assembling proven regenerative medicine technology and expertise with the goal of developing transformative therapies for fatal and intractable diseases."

Dr Henry Ji, President and CEO of Sorrento Therapeutics, said, 'The potential for regenerative therapies in treating a wide array of chronic degenerative conditions is well

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known. We see important synergies for the oncology field and the potential to enhance our fight against malignant cancers. Celularity's technologies, assets and resources will help advance selected Sorrento cellular therapy programs and potentially transform autologous cellular therapies into affordable and accessible allogeneic cell therapies."

Sorrento Therapeutics and Human Longevity have contributed to the formation of Celularity Inc., a company that will be led by Robert Hariri. This month there was an update on the portfolio of assets that have been placed under the company's umbrella. On the regenerative medicine front, Celularity will receive a large portfolio of IP related to the use of placenta stem cells and two commercial assets. The company will also work in the immuno-oncology space, developing fully human CAR-T constructs, an allogeneic immunotherapy platform, and moving a CD38 program into the clinic. It's a diverse mix of technologies that will require some significant funding to move forward at once but there's a lot of potential here. – Mark Curtis

ALPHA 1 GENE THERAPY COMPANY APIC BIO LAUNCHES

A University of Massachusetts spin-off gene therapy company has launched to target the disease Alpha-1 Antitrypsin Deficiency (Alpha 1). Named Apic Bio, the company has been backed by a private investor who lives with Alpha 1, and by the venture philanthropy arm of the Alpha-1 Foundation.

Apics's lead product, APB-101, targets the liver via the company's proprietary adeno-associated virus (AAV) delivered Dual Function Vector (df-AAV) platform which silences the Z-AAT protein and augments the M-AAT protein. The therapy has achieved a pre-clinical proof-of-concept with efficacy demonstrated in vitro and in vivo and is currently undergoing pre-clinical GLP toxicology studies in non-human primates. The mechanism of the therapy is potentially significant for Alpha 1 patients who are deficient in the AAT protein, which protects lung tissue from proteases. Mutant AAT proteins also collect in the liver, increasing the risk of cirrhosis.

Dr Chris Mueller, Co-founder and Chief Scientific Officer, said: "We are encouraged by the feedback that we have received during our pre-IND meeting with the FDA that there is a clear path for us to conduct a first-in-human Phase 1/2 clinical study. Furthermore, we are very much looking forward to demonstrating the benefit of APB-101 to patients that have been living with alpha-1 and have had very little hope for a cure. Our data suggests this is a 'liver sparing' approach for gene augmentation, which may exceed the therapeutic and safety margins when compared to a strict gene augmentation without gene

silencing that may exacerbate the underlying liver disease."

Additionally, John Reilly, Co-Founder and President, said: "We are grateful to TAP and A1AT Investors, LLC who have supported the successful start of Apic Bio by providing the first tranche of our seed financing round allowing us to secure key intellectual property rights and operational support. With such strong support from the advocacy and patient community, we are confident that we will identify the right corporate partners to help us achieve our business development goals and bring this exciting new therapy to patients."

SILVERSTEIN-BACKED STARTUP PREVAIL TO DEVELOP PD GENE THERAPY

Regenxbio with investment firm OrbiMed, and nonprofit the Silverstein Foundation, have collaborated to start up Prevail Therapeutics, a company that will focus on new biologics and gene therapies for Parkinson's disease (PD).

Diagnosed with GBA-linked PD in February this year, Silverstein founder and OrbiMed's co-head of private equity Jonathan Silverstein, has already contributed $10 million to foundation. Celgene has recently followed this with a $5 million pledge, and interest is coming from other non-PD companies also.

Regenxbio's NAV AAV9 will be exclusively licensed to Prevail for use in the development of gene therapies. Focus will fall initially on GBA1, the most common of the PD mutations, which is estimated to be present in up to 10% of US PD patients. Silverstein commented that the company will look to expand into other patient groups, "Many of the drugs we are trying for Parkinson's with GBA may work in the broader Parkinson's population."

The announcement of the creation of Prevail Therapeutics offers hope for patients with Parkinson's disease (PD). The company has decided to focus on the gene GBA, which makes sense: a recent multicentre study showed a five-fold increase in risk of PD associated with mutations in GBA, which makes it the greatest genetic risk factor for developing PD. Interestingly, mutations in GBA, which encodes β-glucocerebrosidase, also cause the lysosomal storage disorder Gaucher's disease. Other companies, including Oxford Biomedica (ProSavin) and Ceregene (CERE-120) have conducted gene therapy clinical trials in PD, albeit targeting different mechanisms of the disease; the challenge is delivery of the vector, which to date has been achieved by stereotactic injection into deep brain nuclei. As yet there are no approved disease-modifying therapies, so the announcement of a new company tackling an important genetic mutation associated with the disease is very welcome.
– Richard Philipson

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NEW FORTRESS SUBSIDIARY, AEVITAS THERAPEUTICS, TO DEVELOP COMPLEMENT-MEDIATED DISEASE GENE THERAPIES

New York-based biotech company Fortress has licensed a proprietary AAV-based technology from a top university for the formation of new subsidiary company Aevitas Therapeutics. The company will focus on developing AAV vector-mediated gene therapies to treat complement-mediated diseases by restoring the production of functional proteins.

Complement-mediated diseases include atypical hemolytic uremic syndrome (aHUS) and paroxysmal nocturnal hemoglobinuria (PNH). It is hoped that targeting protein irregularities will prove curative. Further research has also suggested that complement regulator proteins may also have a hand in macular degeneration, which is another potential investigatory route for the company.

CEO of Fortress Lindsay Rosenwald commented, "We are thrilled to work on this potentially groundbreaking technology in numerous areas of unmet need as we continue to establish our capabilities in the gene therapy space."

GILEAD SCIENCES BUYS KITE PHARMA IN $11.9 BILLION DEAL

Leading CAR-T company Kite Pharma will be acquired by Gilead Sciences in an $11.9 billion deal. The agreement won unanimous approval from the directing boards of both companies and is expected to close later this year.

The timing of the deal comes as Kite is poised for US approval of the first CAR-T treatment for refractory aggressive non-Hodgkin lymphoma. This is expected to be followed by marketing approval in Europe. Kite has additional candidates in clinical trials in both hematologic cancers and solid tumors, including multiple myeloma.

"The acquisition of Kite establishes Gilead as a leader in cellular therapy and provides a foundation from which to drive continued innovation for people with advanced cancers," said John F Milligan, Gilead's President and CEO. "The field of cell therapy has advanced very quickly, to the point where the science and technology have opened a clear path toward a potential cure for patients. We are greatly impressed with the Kite team and what they have accomplished, and share their belief that cell therapy will be the cornerstone of treating cancer. Our similar cultures and histories of driving rapid innovation in order to bring more effective and safer products to as many patients as possible make this an excellent strategic fit."

Kite CEO Arie Belldegrun commented, "From the release of our pivotal data for axi-cel, to our potential approval by the FDA, this is

a year of milestones. Each and every accomplishment is a reflection of the talent that is unique to Kite. We are excited that Gilead, one of the most innovative companies in the industry, recognized this value and shares our passion for developing cutting-edge and potentially curative therapies for patients. CAR-T has the potential to become one of the most powerful anti-cancer agents for hematologic cancers. With Gilead's expertise and support, we hope to fulfill that potential by rapidly accelerating our robust pipeline and next-generation research and manufacturing technologies for the benefit of patients around the world."

PFIZER'S GENE THERAPY INVESTMENT PLANS IN NORTH CAROLINA

Pfizer is advancing $100 million investment plans for a clinical and commercial gene therapy manufacturing facility in Sanford, North Carolina. The facility will be based around a technology created at the University of North Caroline in Chapel Hill and is expected to create around 40 new jobs.

Further support for the expansion will come from the One North Carolina (NC) fund if Pfizer is successful in meeting job creation and capital investment targets. The conditional $250,000 grant will be awarded to the wholly owned Pfizer subsidiary Wyeth Holdings. One NC grants also depend upon local government matching the investment, a further source of funding for the preliminary stage plans.

Additionally, Pfizer has pledged $4 million to support post-doctoral fellowships in gene therapy research to be based at universities in the state.

KRYSTAL FILES FOR IPO TO FUND DYSTROPHIC EPIDERMOLYSIS BULLOSA IND

Krystal Biotech has filed an initial public offering (IPO) with hopes to raise in the region of $35 million. This is intended to go towards the filing of an Investigational New Drug (IND) application for the company's dystrophic epidermolysis bullosa gene therapy.

The gene therapy KB103 targets the COL7A1 gene mutation in the rare and debilitating skin disease using a HSV-1-based vector. The company has run preclinical trialling of the candidate, and met with the FDA to sketch out the development of the therapy. Success at the IND stage will enable KB103 to progress to the clinic.

More than two-thirds of the company is currently owned by the family trust of CEO and COO Krish and Suma Krishnan. Sun Pharma owns a further 16.5% share, following a recent Series A round worth $7 million. The company plans to put remaining cash from the NASDAQ listing towards initiating their follow-up program, KB104.

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NIGHTSTAR APPOINTS TUYEN ONG AS CHIEF DEVELOPMENT OFFICER

Nightstar has appointed Dr Tuyen Ong, most recently of PTC Therapeutics, as its new executive vice president and chief development officer. With 20 years of drug development experience, Dr Ong will work from the company's US headquarters in Lexington, MA.

CEO David Fellows commented, "Tuyen joins Nightstar at a very exciting time for us. As we advance our lead product candidate, NSR-REP1, into a planned Phase 3 clinical trial for the treatment of choroideremia in the first half of 2018 and conduct the ongoing Phase 1/2 clinical trial of our second product candidate, NSR-RPGR, for the treatment of X-linked retinitis pigmentosa, expanding our development capabilities in the US will be critical. Tuyen's experience in clinical development will help us as we seek to develop and commercialize novel, one-time gene therapy treatments for patients suffering from these rare inherited retinal diseases that would otherwise progress to blindness."

Dr Ong completed his MD at University College London and holds an MBA from New York University Stern School of Business. He is a member of the Royal College of Ophthalmologists.