COMMERCIAL INSIGHT: MARCH 2018

Commercial insight: cell and gene therapy

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

CELL THERAPY: Allogeneic cell therapies were in the spotlight this past month with Rubius Therapeutics raising an additional $100M to fund its red blood cell platform, and Sigilon Therapeutics securing an Advanced Therapy Medicinical Product Designation from the FDA for its encapsulation technology for transplant. Both companies were birthed in recent years by Flagship Pioneering. On the autologous front JW Therapeutics, a subsidiary of Juno Therapeutics, raised $90M to fund development of CAR-T therapies in Asia, and Bluebird bio and Celgene decided to share the development work to bring their anti-BCMA CAR-T to market in the United States.

GENE THERAPY: This month sees several companies achieve notable regulatory "endorsements", with MeiraGTx receiving EMA PRIME designation for its gene therapy candidate for achromatopsia, AveXis receiving Sakigake designation for its SMA Type 1 gene therapy in Japan and Abeona Therapeutics receiving Rare Pediatric Disease Designation for its gene therapy program in CLN1 disease. Whilst not providing any guarantee of future approval, these designations nevertheless indicate good quality programs underpinned by solid scientific data. Avexis is one of the few companies to seek Sakigake designation in Japan to complement its existing European PRIME and US Breakthrough Therapy designations, indicating its interest in early engagement with all three key regulatory authorities to discuss its clinical plans and data.

GENE THERAPY
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FIRST PATIENT TREATED WITH SPARK'S VISION-RESTORING RPE65 GENE THERAPY

Surgeons at the Children's Hospital Los Angeles (CHLA) have completed the first gene replacement procedure to restore vision in a patient with retinal degeneration using Spark Therapeutics' **LUXTURNA™**, the first FDA-approved gene therapy for a genetic condition in the USA. Spark obtained marketing approval for **LUXTURNA™** from the US Food and Drug Administration in December 2017 for treating children and adults with a defective *RPE65* gene.

RPE65-mediated inherited retinal dystrophy is an inherited retinal disease (IRD) which progresses to complete blindness. Between 1,000-2,000 people in the US is estimated to have vision loss due to biallelic *RPE65* mutations. The gene therapy uses adeno-associated virus vector to deliver *RPE65* gene to the affected patients.

CHLA is one of the seven hospitals approved nationwide to deliver the therapy and it has two full-time pediatric retinal surgeons on site, Drs Aaron Nagiel and Thomas C. Lee. Genetic testing to verify the gene mutation and identify biallelic RPE65 mutations is a pre-requisite before the procedure and CHLA's laboratory is certified to do the same.

The first patient who underwent the surgery on March 20th was an adult who suffered from Leber congenital amaurosis (LCA). Trials performed in LCA patients with RPE65 mutations aged between 4 and 44 had shown the therapy's efficacy in restoring vision in these patients. Following treatment, trial participants reported recognizing faces, seeing stars and being able to read for the first time in years.

FATE ANNOUNCES UPDATES FROM PHASE 1 PROTMUNE TRIAL

New data have been released by Fate therapeutics from the ongoing **ProTmune phase1 trial.** A preventative product to mitigate the incidence of graft versus host disease (GvHD) in patients undergoing an allogeneic hematopoietic stem cell transplant, **ProTmune** is comprised of a hematopoietic cell graft that has been modulated with two small molecules.

The latest results released concern the seven patients initially enrolled in the trial. Of these five are surviving and have not experienced any cancer relapse. Additionally, there have not been any serious adverse events associated with **ProTmune** experienced by any of the participants. The subjects are being followed for a two-year period to assess the incidence of grade 2-4 GvHD, and the safety and tolerability of the treatment.

GvHD currently presents a significant hurdle for cancer patients

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undergoing stem cell transplants; it is the leading cause of post-transplant mortality and is accompanied by heavy immunosuppression that reduces the efficacy of transplants and increases the incidence of relapse. Half of acute GvHD cases are not responsive to treatment. By taking a preventative route, **ProTmune** is therefore tackling the root of issues associated with GvHD.

CMO Chris Storgard commented,

'The primary objective of hematopoietic cell transplantation for cancer patients is disease-free survival. We are very encouraged that no events of cancer relapse have occurred with ProTmune in the Phase 1 stage of PROTECT. Our clinical findings underscore the compelling safety profile of ProTmune and suggest that ProTmune has the unique potential to attenuate early, life-threatening events of acute GvHD and promote the curative potential of allogeneic transplant. We continue to follow Phase 1 subjects and look forward to assessing key one-year outcomes with ProTmune, including incidence and severity of chronic GvHD, cancer relapse and disease-free survival.'

KRYSTAL FILES IND APPLICATION FOR DEB GENE THERAPY KB103

Pittsburgh based Krystal Biotech has submitted an Investigational New Drug (IND) application to the FDA for the initiation of human trials of allogeneic gene therapy candidate **KB103**. Targeting the serious skin disease Dystrophic Epidermolysis Bullosa (DEB), **KB103** is being developed as an off the shelf product for intradermal or topical administration.

KB103 has been developed on Krystal's proprietary STAR-D platform; a dermatologically targeted vector based on the HSV-1 virus. In **KB103**, this is used to deliver *COL7A1* genes to the skin cells of patients with the DEB, to compensate for the lack of collagen protein that is the cause underlying the disease's blistering symptoms. **KB103** has been engineered to have low immunogenicity, and the capacity to mediate the transfer of large or numerous genes.

*'**KB103** has the potential to become a first-in-class "off-the-shelf" topical gene therapy treatment for DEB. It is the result of an extensive research and preclinical effort by our internal team that included*

The announcement of Krystal Biotech's IND submission for its HSV-based gene therapy treatment brings another player to dystrophic epidermolysis bullosa – currently a very active field of R&D. The company's approach of using a topical gene therapy is certainly novel, and complements other treatments in development such as injectable gene modified fibroblasts, gene modified epidermal grafts and various skin dressings. Whether a topical gene therapy can tackle this devastating disease that causes widespread, severe skin blistering remains to be seen – the outcome of FDA's review of the IND submission will be interesting to see, but news of another company bringing a new treatment to patients is to be applauded.
- Richard Philipson

engineering, screening and testing a library of in-house constructed vectors and complementing cell lines. This reflects our deep expertise in our proprietary STAR-D Platform. As we look ahead, we believe that the productive STAR-D platform and our intent to bring GMP manufacturing in-house by the end of 2018 will support rapid advancement of clinical programs to treat debilitating skin diseases'

commented CEO Suma Krishnan.

BELLICUM'S T CELL THERAPY SHOWS POSITIVE SIGNS IN PEDIATRIC LEUKEMIA PATIENTS

Interim data from Bellicum Pharmaceuticals' adjunct T cell therapy trial of **BPX-501** in pediatric patients with acute myeloid leukemia (AML) and primary immunodeficiencies (PIDs) shows high rates of disease-free survival and overall survival.

BPX-501 comprises of engineered donor T cells which incorporate Bellicum's CaspaCIDe® safety switch. This facilitates the elimination of alloreactive **BPX-501** T cells (via administration of activator agent rimiducid) should uncontrollable GvHD occur. Intended for administration after an allogeneic hematopoietic stem cell transplant (HSCT), the therapy enhances immune reconstitution and viral infection control.

Data from the ongoing BP-004 trial of **BPX-501** conducted at various transplant centers in the US and Europe suggests that **BPX-501** T cells contributes to a durable anti-leukemic effect in patients with AML. 38 pediatric AML patients in their first (n=13) or second (n=25) complete response underwent a haplo-HSCT followed by treatment with **BPX-501**. Treatment with **BPX-501** resulted in 91.5% relapse-free survival and 97.3% overall survival in AML patients, with a median follow-up of one year. This is the highest one-year rate reported thus far in pediatric AML patients.

Dr Neena Kapoor, Director of the Blood and Marrow Transplantation Program at Children's Hospital of Los Angeles and an investigator in the BP-004 trial commented:

"The recurrence of cancer is one of the most serious risks to AML patients receiving a stem cell transplant. These impressive results in children with AML suggest that BPX-501 may be effectively reducing or eradicating residual cancer cells following a haplo-transplant procedure."

Of the 59 pediatric PID patients who underwent haplo-HSCT and treatment with **BPX-501**, disease-free survival and overall survival was reported at 88.1% and 88.6%, respectively, with a median follow-up of one year.

Bellicum is now working with the investigators and the US FDA to develop a protocol for a potential US registration study in pediatric patients. Pending regulatory clearances, the company expects to initiate the study by the end of 2018.

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MEIRAGTX GENE THERAPY CANDIDATE A002 GRANTED PRIME DESIGNATION

A002, a gene therapy product for the eye disorder achromatopsia has been designated as a priority medicine under the European Medicines Agency (EMA)'s PRIME programme. Developed by MeiraGTx, the designation's success was based on data from an ongoing eight patient Phase 1/ 2 trial as well as pre-clinical in vivo data. The company also recently received a Rare Pediatric Disease Designation from the FDA for **A002**.

Arising from an inherited mutation in the *CNGB3* gene, achromatopsia results in blindness, light sensitivity and involuntary eye movement. **A002** is mediated by an AAV vector which delivers a corrective cDNA version of the *CNGB3* gene via a subretinal injection.

MeiraGTx CEO Zandy Forbes commented,

'With receipt of this important designation, we are excited to be begin working closely with the EMA to accelerate A002 as a potential treatment option for patients living with this challenging and painful condition'.

ATMP DESIGNATION FOR SIGILON LIVING THERAPEUTIC CANDIDATE

The EMA has granted Sigilon therapeutics an Advanced Therapy Medicinal Product (ATMP) designation for the product **SIG-003**. The designation is specially intended for tissue engineered, gene therapy, or somatic cell therapy candidates and includes regulatory guidelines as well as development incentives. These include fee reductions on data certifications, recommendations, and evaluation.

SIG-003 has been developed on the company's Afibromer™ Living Therapeutic platform which is biomaterial in which engineered

ONES TO WATCH

Sigilon Therapeutics' Afibromer technology is a novel polymer that the company will deploy to encapsulate engineered cells for transplant. The polymer allows the cells to secrete therapeutic proteins of interest while protecting them from the host's immune system. The platform has broad potential that could make regenerative medicine much more approachable in the future. Sigilon recently signed a deal with Eli Lilly for type 1 diabetes, getting an upfront payment of $63M. The company will compete directly with Semma Therapeutics. Semma is developing a different solution for transplanting beta cells, that will utilize a device that sits below the skin. A key question from a biological perspective will be how effective each approach is in mimicking the in vivo cellular niche. -Mark Curtis

allogeneic cells are embedded. These have been modified to express human factor IX protein for the treatment of haemophilia B.

CEO Paul Wotton stated,

'This first ATMP classification for our platform technology is an important step in engaging with regulatory authorities for this entirely new category of genetically engineered cell factory therapeutics and is a critical step toward commercialization in Europe for advanced therapies like SIG-003. As we proceed in development, it is critical that we work with regulators to define a clear path toward market approval from the earliest stages, which includes satisfying established international guidelines and standards for the future introduction of new Living Therapeutics.'

SOLID BIO HALTS ITS GENE THERAPY TRIAL FOR DMD

The FDA has placed a clinical hold on Solid Biosciences' Phase 1/2 trial of SGT-001, its gene therapy for Duchenne muscular dystrophy. Shares were halted for half an hour following the news and closed down 0.5%, but fell 54% in after-hours trading.

The **IGNITE-DMD** trial was designed to evaluate the safety and efficacy of SGT-001 microdystrophin gene transfer in ambulatory and non-ambulatory children and adolescents with DMD. The first patient dosed was a non-ambulatory adult and received 5E13 vg/kg of SGT-001 in February this year. The patient was however hospitalized a few days later and laboratory findings revealed a decrease in platelet count, red blood cell count and complement activation, a type of immune response. The patient responded well to medical treatment and is currently asymptomatic.

The FDA classed the event as a "Suspected Unexpected Serious Adverse Reaction" and placed a clinical hold on the trial. Following this, the company has halted enrolment and dosing and is now awaiting formal letter from the agency to understand the requirements for resuming the clinical trial.

Disappointing news from Solid Biosciences' lead program in Duchenne muscular dystrophy (DMD). **SGT-001** is an AAV microdystrophin gene transfer candidate, which aims to enable the systemic delivery of a synthetic, functional version of the dystrophin gene. An initial partial clinical hold has now been converted to a full hold by FDA, following worrying reports of thrombocytopenia and complement activation in the first patient treated. DMD is a challenging disease to tackle using gene therapy, given the widespread muscle abnormalities and the large size of the faulty dystrophin gene. Whilst the affected adolescent patient is reported to have recovered, this will nevertheless be a significant blow to the program, with no indication of how long the clinical hold will last, and what will be required to lift it. -Richard Philipson

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JAPAN AWARDS AVEXIS' SMA GENE THERAPY SAKIGAKE DESIGNATION

AveXis' lead product candidate AVXS-101 has been granted the SAKIGAKE designation, by Japan's Ministry of Health, Labour and Welfare (MHLW). Akin to the 'breakthrough' and PRIME designations in the US and Europe respectively, the SAKIGAKE designation expediates the reviewing, consultation, and approval processes for selected candidates.

AVXS-101 is intended to treat spinal muscular atrophy Type 1 (SMA) by addressing the underlying monogenetic cause of the condition. SMA is a leading cause of infant mortality; for surviving patients permanent ventilation support is a necessity. This arises from a loss of motor neurons and the subsequent deterioration of muscles.

Sean Nolan, the company's CEO stated,

'This designation by the MHLW underscores the agreement by the Japanese government that there is an urgent need for a new therapeutic approach to treat Japanese patients diagnosed with SMA Type 1 and allows for enhanced discussions between AveXis and the agency to potentially expedite the timeline for approval of AVXS-101'.

ADAPTIMMUNE'S T CELL THERAPY SHOWS POSITIVE RESPONSE IN SOLID TUMOR PATIENTS

Adaptimmune's GSK-partnered T cell therapy has shown partial responses in three of the first four myxoid/round cell liposarcoma (MRCLS) patients treated with its Specific Peptide Enhanced Affinity Receptor (SPEAR™) T cells.

Adaptimmune's NY-ESO T cell therapy targets the NY-ESO cancer antigen present in solid tumors and hematologic cancer types, including synovial sarcoma and multiple myeloma.

Previously in 2015, the company posted positive clinical data of its T cell therapy in treating patients with synovial sarcoma. MRCLS, like synovial sarcoma, is another soft tissue sarcoma but the underlying genetics and clinical manifestations are different in these two tumors.

GSK owns the exclusive license to research, develop and commercialize the NY-ESO SPEAR T-cell therapy program. The therapy is currently being investigated for a range of cancers, including: sarcoma, non-small cell lung cancer, and ovarian cancer. The therapy is also being investigated in combination with Merck's Keytruda (pembrolizumab), an anti-PD-1 inhibitor.

Of the four patients treated thus far, two patients displayed partial response, one showed unconfirmed partial response and the last one displayed stable disease. The treatment

was well-tolerated by all the patients and cytokine release syndrome was managed with standard treatments. Although the size of the dataset is small, the company is hopeful that it's moving in the right direction and the news has increased its shares to nearly 20%.

ABEONA GRANTED RARE PEDIATRIC DISEASE DESIGNATION FOR CLN1 GENE THERAPY PROGRAM

Abeona therapeutics has received a Rare Pediatric Disease Designation for AAV mediated gene therapy **ABO-202** which is in development for the treatment of the lysosomal storage disease Infantile and late infantile onset Batten disease (CLN1). The program already holds an Orphan drug designation which was awarded in February of this year.

CLN1 is a hereditary condition with a rapid progression in newborns that is ultimately fatal and has no current treatment options. This latest designation will facilitate the review process of this and potentially future drugs developed by Abeona.

CEO Timothy Miller commented,

*'This Rare Pediatric Disease designation for **ABO-202** is a significant recognition of the strength of the data supporting a potential treatment for patients with CLN1 and is bolstered by the previous Orphan Drug designation from the FDA. These regulatory designations highlight the urgent need for a treatment for this devastating rare disease, and we look forward to initiating human clinical trials later this year.'*

POSITIVE RESULTS FROM FIRST DTX301 BY ULTRAGENYX PHASE 1/ 2 COHORT

New and encouraging data from the ongoing trial of Ultragenyx's gene therapy **DTX301** has been released. From a cohort of three patients with the target disease: ornithine transcarbamylase (OTC) deficiency, **DTX301** was found to yield clinically meaningful improvements in one subject. This was monitored via the levels of ureagenesis occurring. For the clinically significant patient, initial ureagenesis levels were at 67% of normal, and this peaked at 134% over the course of the treatment. As a phase 1/2 trial, the primary endpoint is safety and tolerability. This was observed in all patients successfully as there were no adverse events beyond Grade 1 or 2.

CEO Emil Kakkis stated,

'Longer term data from the first, lowest-dose cohort show that patient 1 maintained and substantially increased levels of ureagenesis through 24 weeks and we view these results as a promising indication of the potential

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*and durability of **DTX301**. Based on these positive results at 24 weeks, this patient opted to discontinue all alternate pathway medication three weeks ago per the trial protocol and continues to do well. The data monitoring committee has completed a favorable review of the current data for this dosing cohort. We were encouraged by the initial signs of efficacy with an acceptable safety profile in the first cohort, which is at the low end of the expected dosing range. We are pleased to advance this study to the second, higher-dose cohort'.*

FIBROCELL'S IND APPLICATION SUCCESSFUL FOR FIBROBLAST GENE THERAPY

Fibrocell has been cleared to begin clinical trialling of gene therapy **FCX-013** for localized scleroderma due to the acceptance of the company's IND application. An autologous treatment, **FCX-013** is comprised of an engineered fibroblast which expresses the matrix metalloproteinase 1 (MMP-1) protein. This addresses excessive collagen deposition, which results in localized scleroderma's symptoms, as the protein MMP-1 is responsible for the breakdown of collagen. The approach has already garnered rare pediatric disease and orphan drug designations. This is in part due to current treatments only targeting inflammation, with the growth and mobility effect on limbs left untreated.

Trials are being planned for the third quarter of this year with adult patients being assessed initially. Fibrocell plans to include pediatric patients at a further stage.

CEO John Malowski commented,

'We are pleased the FDA has granted allowance of our IND for FCX-013 to begin clinical trials for the treatment of moderate to severe localized scleroderma, offering patients the potential for relief from this chronic, painful and debilitating disease'.

PHASE 3 TRIALLING BEGINS FOR NIGHSTAR'S CHM GENE THERAPY

Nighstar Therapeutics has initiated Phase 3 trialling of gene therapy **NSR-REP1** in its STAR trial. The London-based company is assessing the safety and efficacy of the AAV mediated therapy in patients with the retinal disorder choroideremia (CHM). Previous studies in Phase 1/2 reported visual acuity improvements or maintenance in over 90% of the subjects treated.

CHM is a genetic, degenerative disorder that eventually results in blindness. Experienced by males, the mutation underlying the

condition leads to an absence of the REP1 protein which is vital for protein trafficking and the removal of waste in the retina. **NSR-REP1** thus targets the condition by delivering a functional copy of the CHM gene which codes for REP1.

The Phase 3 trial in question will enrol in the region of 140 patients across three continents. Patients will be divided into three cohorts of high, low, and no dose recipients, and will be primarily monitored against the improvement in ETDRS letters from baseline.

Nightstar CEO Dave Fellows stated,

*'The initiation of this first-ever Phase 3 trial for the treatment of choroideremia is a major milestone for Nightstar and a tremendous step forward for patients otherwise at risk of blindness due to this devastating disease. We are very encouraged by the responses we have seen to-date following treatment with **NSR-REP1**. This accomplishment demonstrates our team's ability to successfully advance important gene therapies. We are thankful to our academic and advocacy partners, as well as the many patients who have participated in our studies, all of whom have been instrumental in helping us to achieve this milestone.'*

CALADRIUS BAGS EXCLUSIVE LICENSE TO SHIRE'S CELL THERAPY PROGRAM FOR REFRACTORY ANGINA;

New Jersey-based **Caladrius Biosciences** has announced that it has acquired exclusive worldwide rights to **Shire's CD34+ cell therapy program** developed for the treatment of chronic myocardial ischemia targeting refractory angina.

Under the terms of the agreement, Caladrius has received global rights to the data sets, including preclinical, Phase 1, Phase 2 and Phase 3 clinical study data, and all the regulatory filings for the CD34+ cell therapy program for the treatment of no-option refractory angina. In return, Shire will receive undisclosed up-front consideration, milestones and a royalty on product sales.

Data supporting the use of autologous CD34+ cell therapy for refractory angina come from a pooled analysis of three randomized placebo controlled clinical trials. Tested in over 300 patients, the study published in European Heart Journal reveals clinically meaningful improvement in exercise capacity, chest pain frequency and mortality.

Dr David J. Mazzo, President and CEO of Caladrius commented:

"This transaction offers an ideal opportunity for Caladrius to obtain a promising late-stage development asset complementary to our existing pipeline of CD34+ cell therapy development programs in ischemic repair. This program represents a large potential commercial opportunity as refractory angina afflicts approximately one million people in the U.S. alone, with an incidence rate of 50,000 to 100,000 annually."

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BLUEBIRD BIO PARTNERS WITH CELGENE TO DEVELOP ANTI-BCMA CAR T THERAPY

bluebird bio has entered into an agreement with **Celgene** to co-develop and co-promote bb2121, an investigational anti-B-cell maturation antigen (BCMA) chimeric antigen receptor (CAR) T cell therapy for treating patients with relapsed/refractory multiple myeloma in the United States.

The two companies initially entered into a global strategic research collaboration in 2013 to advance gene therapy technologies for treating various cancers. This agreement was amended in 2015 and under the terms of the new three-year collaboration, the focus was to develop product candidates for targeting BCMA. bluebird bio retained rights to develop all of its other CAR T cell programs for solid tumors and hematologic malignancies.

Under the terms of the new agreement, bluebird and Celgene have joint responsibility for developing, manufacturing and commercializing bb2121 in the US. Celgene holds the responsibility for drug product manufacturing and commercialization outside the US for which bluebird will receive milestones and royalties. The companies are also working together on a second anti-BCMA CAR T program, bb21217.

bb21217 complements bb2121 but is thought to be more potent than the latter. This is because for bb21217, the CAR T cell product is enriched for memory T cells – a long-lived, more potent T cell subtype for increased anti-tumor efficacy.

Dr Joanne Smith-Farrell, bluebird bio's Senior VP of corporate development and strategy commented:

"Entering into this co-development and co-promotion partnership with Celgene is a significant step forward in building a fully integrated oncology franchise for bluebird and together, we are committed to rapidly advancing development of bb2121 for patients. The collaboration builds upon our extensive research and development capabilities in oncology and is a testament to the strong partnership that exists between our two companies."

NOHLA THERAPEUTICS COLLABORATES WITH WUXI APPTEC FOR OFF-THE-SHELF CELL THERAPIES

Nohla Therapeutics, a Seattle-based clinical stage cell therapy company has entered into a commercial agreement with **WuXi AppTec** for the production of its hematopoietic stem and progenitor cell product.

WuXi AppTec is a Contract Development Manufacturing Organization that supports the development and manufacturing of various advanced therapy programs. Situated in Philadelphia, its 55,000

square foot GMP-compliant commercial manufacturing facility will support the development, manufacturing and quality control testing of Nohla's lead candidate, NLA101.

Launched in 2015, Nohla Therapeutics is developing universal donor, off-the-shelf cell therapies to redefine clinical outcomes in patients with critical diseases. Nohla's products are based on the cell therapy research at Seattle's Fred Hutchinson Cancer Research Center with which it has a 20-year exclusive licensing agreement.

NLA101 is a universal donor, off-the shelf ex vivo expanded hematopoietic stem and progenitor cell product that provides transient hematopoiesis *in vivo*. Intended to provide temporary bone marrow function for 2-3 weeks, these cells are aimed to boost the immunity by preventing infection until the patient's bone marrow can recover.

Currently NLA101 is the subject of two Phase 2 clinical trials. It is being tested in patients with high-risk hematological malignancies such as acute myeloid leukemia and in patients receiving high-dose chemotherapy. Clinical results to date shows improved treatment outcome in both the trials. While overcoming the broad safety and logistical risks of patient-customized cell therapies, NLA101 does not require HLA matching.

TORQUE ANNOUNCES NEW ADDITIONS TO DIRECTORIAL AND SCIENTIFIC BOARDS

The board of directors of immune-oncology compant Torque will be joined by **John Hohneker**; and the scientific advisory board welcomes the addition of **Marcela Maus**. Torque is developing its proprietary platform Deep Primed™ cell therapeutics aims to drive immune responses from within the tumor microenvironment.

Dr. Hohneker is presently the CEO of Anokion, and has previously worked at FORMA therapeutics and Novartis. He has significant experience of seeing products through to commercialization from early development. Receiving his MD from Rutgers Medical School, Dr Hohneker's fellowship was completed in the field of medical oncology.

Dr Maus is assistant professor at Harvard Medical School, as well as the Director of Cellular Immunotherapy in the cancer center at Massachusetts General Hospital (MGH). Her research is focused on the development of new CAR T cells, and she has particular expertise in T cell immunotherapies. Previously, Dr Maus has held the position of assistant professor at the University of Pennsylvania, from which she also received her MD and PhD.

"Torque is pioneering a new approach to cell-based therapy for cancer, creating immune cells that carry tightly controlled doses of immunomodulators to direct immune power deep in the tumor,"

said Bart Henderson, CEO of Torque.

"We are breaking new ground, and both John and Marcela bring significant experience and creativity to this effort. John brings expertise in clinical development for breakthrough medicines in immunology and oncology; and Marcela is a leading expert in CAR T cell therapy, now building out the Cell Therapy Program at Massachusetts General Hospital. We are very excited to have these respected leaders join the Torque team."

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RUBIUS RAISES $100 MILLION FOR ADVANCEMENT OF RED CELL THERAPIES

Rubius therapeutics has closed an oversubscribed financing round that realised **$100 million** of funding. The company is developing a portfolio of therapeutic products based on red cells. These are expected to target both cancers and autoimmune diseases due to the variety of beneficial characteristics exhibited by the cells. This latest round of funding brings the company's nine month total to $200 million. This is planned to go towards advancing clinical proof of concept and expanding manufacturing faculties.

'The addition of this funding further strengthens our foundation and enables us to accelerate the development of our first wave of RCT products that are targeting treatment of enzyme deficiencies, cancer and autoimmune disease,'

commented President Torben Straight Nissen.

'We have assembled an extremely talented team of investors, leadership and advisors, which all share the long-term vision of bringing novel cellular therapies to patients.'

PREVAIL RAISES FUND FOR GENE THERAPY OF PARKINSON'S DISEASE

Prevail Therapeutics, a New York-based startup company focusing on developing gene therapies for Parkinson's disease (PD) and other neurodegenerative diseases, has announced that it has raised **$75 million in a Series A financing**.

Prevail will use the funds to advance its pipeline of AAV-based gene therapies for targeting lysosomal dysfunction and treat patients with neurodegenerative diseases. Prevail's lead program PR001 is being developed for treating PD patients with glucocerebrosidase (GBA) mutation, a variant thought to affect around 10% of all patients with PD.

The company was launched in 2017 by OrbiMed's co-head of private equity Jonathan Silverstein along with Regenxbio and The Silverstein Foundation for Parkinson's with GBA, a non-profit research foundation formed by Silverstein after he was diagnosed with Parkinson's in 2016.

The fundraising campaign was supported by OrbiMed, Pontifax Fund, RA Capital Management, EcoR1 Capital, Omega Funds, BVF Partners L.P., Boxer Capital, LLC, Adage Capital Management L.P., and Alexandria Venture Investments.

The company has also taken a license to use an AAV vector developed by Regenxbio called NAV AAV9. This vector will be used across Prevail's gene therapy candidates including PR001.

Jonathan Silverstein commented:

"Prevail's mission to develop gene therapies that address the

underlying genetic causes of neurodegenerative disease is aligned with our commitment to identify treatments that can halt the progression of Parkinson's and other diseases. The Company has assembled an excellent team to accomplish this mission, including executive management, scientific advisors, and board members, and I look forward to working with them to make a difference for patients."

JUNO SPINOUT JW THERAPEUTICS RAISES $90MILLION IN SERIES A ROUND

China based JW therapeutics, which was founded in 2006 in a collaboration between Juno therapeutics and local company WuXi AppTec Group, has secured **$90 million in a Series A round**. The founding companies were joined by Temasek, Sequoia Capital China, and YuanMing Capital aong others as new investors.

JW's focus is on bringing CAR T therapies to China; leveraging both the expertise of Juno and the local manufacturing and technology capabilities of WuXi AppTec. The series A round will go towards the advancement of the company's lead candidate JWCAR029 which is being developed for the treatment of B-cell malignancies. The company plans to establish a therapeutic pipeline, as well as a manufacturing facility with the funding.

CEO James Li commented that the round

"will greatly accelerate the development and commercialization of JW Therapeutics' portfolio to meet the vast unmet medical needs and provide innovative treatment options to patients in China."

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