

## 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:** Exciting news this month in the field of hereditary muscle diseases! A collaboration led by the University of Washington has demonstrated pre-clinical proof of concept for systemically delivered, AAV-

based gene therapy in dogs with X-linked myotubular myopathy. In addition, there is good news for boys with Duchenne muscular dystrophy, with Exonics announcing the award of seed funding to support preclinical development of its CRISPR/Cas9 technology, which could offer a one-off, curative treatment for the disease. Other interesting developments include the award of Breakthrough Therapy designation by FDA for UniQure's AAV-5 treatment for hemophilia B, based on emerging clinical data from its ongoing, dose-ranging Phase 1–2 study. It's always encouraging to receive 'validation' of clinical data from a regulatory authority with this type of designation!



**GENE THERAPY**  
**Richard Philipson**  
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**CELL THERAPY**  
**Mark Curtis**  
Financial Portfolio  
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**CELL THERAPY:** Kite Pharma delivered new pivotal data this month that showed that there was a limited drop in complete response rate between 3 and 6 months in ZUMA-1, a study investigating lead candidate axicabtagene



ciloleucel in patients with aggressive NHL. While there was a substantial drop in complete response rate in the first 3 months of the study, this decline softened over time, adding some confidence the therapy can be a cure for this form of lymphoma. While more follow-up will be required to substantiate durability, it certainly strengthens the case for approval at the FDA. While Kite's launch of its first autologous CAR product becomes imminent, other developments continue to appear in off-the-shelf approaches to cell-based immunotherapy.



### ARGOS THERAPEUTICS TO DISCONTINUE ADAPT TRIAL

The Independent Data Monitoring Committee (IDMC) for Argos' Phase 3 ADAPT clinical trial of rocapuldencel-T in combination with sunitinib/standard-of-care for the treatment of metastatic renal cell carcinoma (mRCC) has recommended that the study be discontinued for futility based on its interim data report. The IDMC noted that although rocapuldencel-T was well tolerated in the patients, the combination treatment was unlikely to demonstrate a statistically significant improvement in overall survival in the patients.

In conjunction with its clinical and scientific advisors, the company is analyzing the preliminary ADAPT trial data set and plans to discuss the data with the FDA. The company plans to leave the ADAPT trial open while the company conducts its ongoing data review and discussions with FDA. Based on these analyses and discussions, the company will make a determination as to the next steps for the rocapuldencel-T clinical program.

Argos Therapeutics is a US-based immuno-oncology company focused on the development and commercialization of personalized immunotherapies for the treatment

of cancer, based on its Arcelis® technology platform.

Rocapuldencel-T is a personalized immunotherapy that is designed to capture mutated and variant antigens that are specific to each patient's tumor and induce an immune response targeting that patient's tumor antigens. The randomized Phase 3 ADAPT trial evaluating rocapuldencel-T plus sunitinib/standard-of-care therapy versus standard-of-care therapy alone in newly diagnosed mRCC patients was opened in January 2013 and completed enrollment in July 2015. A total of 462 mRCC patients were randomized to the trial. The primary endpoint of the trial was a statistically significant improvement in overall survival.

Jeff Abbey, president and CEO of Argos commented: "We are extremely disappointed with these results, which included 75% of the targeted events needed to permit the primary analysis and assessment of overall survival in the study. We sincerely appreciate the patients and investigators who have participated in the ADAPT Phase 3 trial, and remain convinced in the ability of precision immunotherapy to improve the lives of patients."



## LYSOGENE'S GENE THERAPY RECEIVES ORPHAN DRUG DESIGNATION FROM EMA

The European Medicines Agency (EMA) has granted orphan drug designation to Lysogene's gene therapy candidate, LYS-GM101, developed for the treatment of GM1 Gangliosidosis (GM1), a severe neurodegenerative disease.

The US Food and Drug Administration (FDA) had also granted an orphan drug designation and a rare pediatric disease designation to LYS-GM101 earlier this year. GM1 is a lysosomal storage disorder caused by the deficiency of beta-galactosidase enzyme, resulting in the accumulation of GM1 gangliosides and related glycoconjugates in the lysosomes. It leads to lysosomal swelling and organ dysfunction. The disease is lethal in the infantile and juvenile forms. Lysogene's LYS-GM101 is an adeno-associated

virus vector containing the human gene for the beta-galactosidase enzyme.

Lysogene is a clinical-stage biotechnology company specialized in the development of adeno-associated virus (AAV)-mediated gene therapy for central nervous system disorders.

Karen Aiach, Founder and CEO of Lysogene, commented: "The EMA Orphan Drug Designation for LYS-GM101 is a key regulatory milestone further validating the medical plausibility of our approach. This designation will further facilitate and accelerate clinical development of our treatment and we look forward to studying this therapy further as we approach our upcoming Phase 1/2 clinical trial (LYS-GM101) in 2018."



## BLUEBIRD BIO TREATS FIRST SICKLE CELL DISEASE PATIENT IN AMENDED PHASE 1 TRIAL

bluebird bio has announced treatment of the first patient with LentiGlobin™ drug product under amended study protocol in HGB-206 Phase 1 study of patients with severe sickle cell disease. The study now incorporates several changes to the study protocol with the goal of increasing production of therapeutic anti-sickling hemoglobin (HbAT87Q).

The study, HGB-206, is an ongoing Phase 1 trial designed to

evaluate the safety and efficacy of gene therapy in subjects with SCD by transplantation of autologous CD34+ cells transduced ex vivo with a lentiviral  $\beta$ -A(T87Q)-globin vector (LentiGlobin™ BB305).

Changes to the study protocol for HGB-206 include increasing the percentage of transduced cells through manufacturing improvements, improving myeloablation (and subsequent engraftment) by increasing the target busulfan area

under the curve, introducing a minimum period of regular blood transfusions prior to stem cell collection, improved cell processing and exploring an alternate hematopoietic stem cell procurement method. To accommodate these changes to the protocol, the study enrolment has been expanded for a total enrolment of up to 29 patients. Subjects will be followed to evaluate safety

and efficacy, which will be measured based on changes in red cell function tests, hemolysis markers and frequency of clinical events secondary to SCD (e.g., vaso-occlusive crises or acute chest syndrome events).

LentiGlobin™ BB305 is currently in three clinical studies for the treatment of transfusion-dependent beta-thalassemia and severe sickle cell disease.



### ONES TO WATCH

*bluebird bio has announced a number of important changes to the company's Phase 1 study of its LentiGlobin Drug Product in patients with severe sickle cell disease (SCD). The changes are clearly intended to improve both manufacturing (through improved transduction efficiency) and bone marrow engraftment (through modifications to busulphan conditioning) to achieve*

*improved gene transfer efficiency. Whilst the outcomes in the first patient with sickle cell disease treated with bluebird's product, recently published in the New England Journal of Medicine (N. Engl. J. Med. 2017; 376: 848–55) were very encouraging, the paper also commented that lower gene transfer efficiency has been observed in the first seven patients treated in HGB-206. The outcomes in patients treated under the modified protocol will be crucial for the long-term success of this product, and are likely to be scrutinized with great interest. – Richard Philipson*



### CELL MEDICA'S LEAD PRODUCT CANDIDATE RECEIVES FAST TRACK DESIGNATION

Cell Medica, a London-based pharmaceutical company specialized in the development of cellular therapeutics for the treatment of cancer and infectious diseases, has announced that the FDA has granted Fast Track designation to its lead oncology product, CMD-003, designed for the treatment of patients with relapsed/refractory lymphoma and post-transplant lymphoproliferative disease associated with the oncogenic Epstein–Barr virus (EBV).

CMD-003, also known as bal-taleucel-T, is an investigational therapy in which the patient's T cells are activated to kill malignant cells expressing EBV antigens. The product has the potential to address a range of EBV-associated lymphomas, nasopharyngeal carcinoma and gastric cancer. CMD-003 is currently being investigated in a Phase 2 CITADEL clinical trial in patients with extranodal natural killer T-cell lymphoma (EN-KTCL), a type of non-Hodgkin

lymphoma. The product is also being tested in a Phase 2 CIV-IC clinical trial in patients with EBV-associated diffuse large B-cell lymphoma, Hodgkin lymphoma, and post-transplant lymphoproliferative disease.

Fast Track designation is granted to facilitate development and expedite review of new therapies that address unmet medical needs. It allows more frequent meetings with the FDA to discuss the drug's

development plan, eligibility for Priority Review if relevant criteria are met, and opportunity for Rolling Review, which allows Cell Medica to submit completed sections of its Biologics License Application (BLA) to the FDA, rather than waiting until every section of the BLA is completed before the entire application can be reviewed. CMD-003 was previously granted Orphan Drug designation from both FDA and European Commission.



## UNIQUIRE'S GENE THERAPY RECEIVES BREAKTHROUGH THERAPY DESIGNATION

uniQure, a biopharmaceutical company specialized in the development of gene therapies for severe genetic diseases, has announced that the FDA has granted Breakthrough Therapy Designation for its gene therapy candidate, AMT-060, for the treatment of patients with severe hemophilia B.

FDA's Breakthrough Therapy designation is a process designed to accelerate the development and review of drugs that are intended to treat a serious condition. The current designation for UniQure's gene therapy is based on results from the ongoing, dose-ranging Phase 1–2 study that show sustained increases in Factor IX (FIX), reductions in FIX replacement usage and a near cessation of spontaneous bleeding in patients with severe disease at up to 12 months follow-up.

Updates on clinical data from the ongoing, two-cohort Phase 1–2 trial of AMT-060 were presented

at the *58th American Society of Hematology (ASH) Annual Meeting*. The data included up to 52 weeks of follow-up from the low-dose cohort and up to 31 weeks of follow-up from the second dose cohort. Data from the second-dose cohort showed a dose response with improvement in disease state in all five patients, including the discontinuation of precautionary FIX infusions in all four patients that previously required chronic replacement therapy. All five patients in the low-dose cohort, whose bleedings were previously uncontrolled despite being managed with prophylactic therapy, continued to maintain clinically meaningful levels of FIX activity for up to 52 weeks post treatment, resulting in a complete cessation of spontaneous bleedings in the last 14 weeks of observation. AMT-060 was generally well tolerated and no severe adverse events were reported.



## FDA GRANTS FAST TRACK DESIGNATION TO VERICEL'S IXMYELOCEL-T

Vericel Corporation, a pharmaceutical company specialized in the development of autologous expanded cell therapies for the treatment of patients with serious diseases, has announced that the FDA has granted Fast Track designation to ixmyelocel-T, for the reduction in the risk of death and cardiovascular hospitalization in patients with chronic advanced heart failure due to ischemic dilated cardiomyopathy.

Ixmyelocel-T is an investigational autologous expanded multicellular therapy manufactured from the patient's own bone marrow using Vericel's automated and fully closed cell-processing system. This process selectively expands the population of mesenchymal stromal cells and alternatively activated macrophages, which are responsible for production of

anti-inflammatory and pro-angiogenic factors known to be important for repair of damaged tissue. Ixmyelocel-T has previously been designated as an orphan drug by the FDA for use in the treatment of dilated cardiomyopathy.

Nick Colangelo, president and CEO of Vericel, commented: "Receiving Fast Track designation highlights both the unmet medical need for improved therapies to treat advanced heart failure due to dilated cardiomyopathy and the significance of the results from the ixmyelocel-T Phase 2b ixCELL-DCM clinical study. We believe that achieving important regulatory milestones such as Fast Track designation enhances the value of ixmyelocel-T and our efforts to partner the further development of this program."



## GENSIGHT BIOLOGICS COMPLETES ENROLLMENT OF REVERSE PHASE 3 STUDY

GenSight Biologics, a clinical-stage biotechnology company specialized in developing gene therapies for retinal diseases and diseases of the central nervous system, has announced the completion of enrollment in its REVERSE study, a Phase 3 clinical trial designed to evaluate GS010 in the treatment of Leber's Hereditary Optic Neuropathy (LHON).

LHON is a rare genetic disorder affecting the retinal ganglion cells

leading to a persistent and severe bilateral loss of visual acuity within weeks or months. The disease is caused by point mutations in the mitochondrial DNA. GenSight's GS010 uses a mitochondrial targeting sequence (MTS) proprietary technology platform which, when associated with the gene of interest, allows the platform to specifically address defects inside the mitochondria using an adeno-associated vector.

REVERSE is the first of two parallel randomized, double-masked, sham-controlled, multi-center pivotal Phase 3 trials. REVERSE enrolled 36 patients with an onset of vision loss of 7–12 months, while RESCUE, the second trial, is currently enrolling 36 patients with an onset

of vision loss of less than 6 months. Both studies are being conducted in 7 centers in Europe and in the USA. The RESCUE trial is expected to complete enrolment by the end of first half of 2017. GS010 has been granted Orphan Drug Designation both in the USA and in Europe.



## KITE'S CAR-T THERAPY SHOWS POSITIVE RESULTS IN NON-HODGKIN LYMPHOMA PATIENTS

Kite Pharma has announced positive data from the primary analysis of its ZUMA-1 trial in patients with chemorefractory aggressive B-cell non-Hodgkin lymphoma (NHL). The study met the primary endpoint of objective response rate (ORR), or rates of tumor response (complete response plus partial response) recorded after a single infusion of axicabtagene ciloleucel.

The ZUMO-1 trial uses Kite's lead product candidate, axicabtagene ciloleucel (previously referred to as KTE-C19), an investigational therapy in which a patient's T cells are genetically modified to express a chimeric antigen receptor (CAR) designed to target the antigen CD19, a protein expressed on the cell surface of B-cell lymphomas and leukemias.

The results demonstrate the treatment effect of axicabtagene ciloleucel in a patient population with multiple types of aggressive NHL, including diffuse large B-cell lymphoma (DLBCL) enrolled in cohort 1, as well as primary mediastinal B-cell lymphoma (PMBCL) and transformed follicular lymphoma (TFL) enrolled in cohort 2.

101 patients were treated in ZUMA-1. 82% of treated patients achieved an ORR and 41% of treated patients achieved a response at 6 months, including 36% in CR. 5% continued to experience highly significant and durable partial responses (PR) and one of these PRs converted to a CR at month 9.

The most common grade 3 or higher adverse events included anemia, neutropenia, decreased neutrophil count and febrile neutropenia. As compared to the interim analysis, grade 3 or higher cytokine release syndrome decreased from 18 to 13% and neurologic events decreased from 34 to 28%. There were no cases of cerebral edema.

Kite aims to seek regulatory approval of axicabtagene ciloleucel in aggressive NHL based upon the combined data from all 101 patients and plans to complete its rolling submission of the Biologics License Application (BLA) by the end of the first quarter of 2017. In addition, Kite plans to submit a marketing authorization application for axicabtagene ciloleucel for the treatment of relapsed or refractory DLBCL, PMBCL and TFL with the EMA in 2017.

KTE-C19 was granted Breakthrough Therapy Designation by the FDA in 2015 for the treatment of DLBCL, PMBCL and TFL. It

also gained access to the EMA's Priority Medicines (PRIME) support, for the treatment of DLBCL earlier this year.



### CELLECTIS RECEIVES IND CLEARANCE FOR UCART123

Cellestis, a biopharmaceutical company specialized in developing immunotherapies based on gene edited CAR T-cells (UCART), has announced that it has received IND approval from the FDA to proceed with the clinical development of UCART123 in patients with acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN).

UCART123 is a gene edited T-cell investigational drug that targets CD123, an antigen expressed on the surface of leukemic cells in AML and other tumoral cells in BPDCN. It is the first gene edited off-the-shelf CAR T cell product candidate developed in the USA.

The company plans to initiate the trial in the first half of 2017. The clinical research for AML will be led at Weill Cornell and that for BPDCN will be led at the MD Anderson Cancer Center.

AML is a devastating hematopoietic stem cell neoplasm that is characterized by uncontrolled proliferation and accumulation of leukemic blasts in bone marrow, peripheral blood and other tissues. BPDCN is a rare and aggressive hematological malignancy of bone marrow and blood cells but often affects skin and lymph nodes as well.

Stephan Reynier, Chief Regulatory and Compliance Officer of Cellestis, commented: "After the National Institutes of Health's Recombinant DNA Advisory Committee (RAC)'s unanimous approval of two Phase 1 study protocols for Cellestis' UCART123 in December 2016, the FDA's approval of Cellestis' IND is a new major regulatory milestone achieved, for having UCART123 proceed into clinical development and reaching cancer patients in need".



#### EXPERT PICK

*In 2016, a pediatric ALL patient in Europe was treated with the first allogeneic CAR product to receive approval for clinical development, UCART19. Cellestis' approach to cell-based immunotherapy, involving gene editing, aims to circumvent the critical issues associated with the more prolific autologous*

*immunotherapies, namely cost and patient variability in starting material, the latter of which can make it difficult to deliver product of consistent character and quality. This past month Cellestis announced an FDA greenlight to move its second allogeneic product, UCART123, into the clinic in the USA, where it will be investigated at Cornell and MD Anderson Cancer Center. Now that there is proof of principle in humans for an allogeneic T-cell product, we must wait to see how efficacy compares to autologous approaches. - Mark Curtis*



## UNIVERSITY OF WASHINGTON MEDICINE INSTITUTE FOR STEM CELL & REGENERATIVE MEDICINE USES GENE THERAPY TO TREAT DOGS WITH INHERITED MUSCLE DISEASE

Scientists at the University of Washington Medicine Institute for Stem Cell and Regenerative Medicine, in collaboration with five other academic institutions, have replaced the faulty gene that causes myotubular myopathy and successfully restored muscle strength in dogs with the disease.

The researchers used an adeno-associated virus serotype 8 (rAAV8) to deliver a healthy canine version of

the MTM gene in dogs that were 10 weeks old and already showing symptoms. After a single infusion of the treatment, dogs were followed-up for 1 year and were indistinguishable from healthy dogs.

“This regenerative technology allowed dogs that otherwise would have perished to complete restoration of normal health,” said Dr Martin K ‘Casey’ Childers, UW Medicine researcher and physician.



### EXPERT PICK

*News that researchers at the University of Washington have successfully treated dogs with myotubular myopathy using gene therapy brings renewed hope for patients with hereditary muscle diseases. Myotubular myopathy affects approximately 1 in 50,000 newborn males worldwide, causing myopathy and hypotonia. The condition presents at birth, can be complicated by bone deformities, scoliosis and joint contractures, and usually results in death in early childhood. Results of the study, recently released in Molecular Therapy, indicate that the systemic gene therapy was well tolerated, prolonged lifespan in the dogs, and corrected the skeletal musculature throughout the body in a dose-dependent manner. The treatment uses a recombinant AAV8 vector expressing canine myotubularin, and provides preclinical proof of concept in myotubular myopathy, and more broadly shows the promise of systemic therapies in hereditary muscle diseases. Commercial rights to the treatment, which is currently in at the IND-enabling stage of development, are held by Audentes Therapeutics. – Richard Philipson*



## BROAD INSTITUTE WINS CRISPR PATENT FIGHT

The US Patent and Trademark Office (USPTO) has issued a favorable decision and supported Broad Institute in their legal battle with University of California (UC) over CRISPR gene editing.

Following the news, shares in Editas Medicine, which has licenses to the Broad’s patents, rose 30%. However, stock prices of CRISPR Therapeutics and Intellia Therapeutics, biotechs based on the work

## LICENSING AGREEMENTS & COLLABORATIONS

of UC Berkeley fell by 8 and 9%, respectively.

The patent board ruled that the inventions claimed by the Broad Institute's patents and applications, which specify that the CRISPR-Cas9 system is used in eukaryotic cells (such as human cells), are sufficiently distinct as to be separately patentable from the claims of the UC Berkeley's patent application, which cover the use of CRISPR-Cas9 in any setting, including eukaryotic cells and other cell types. Although UC Berkeley argued use in eukaryotes was an obvious extrapolation of its work, the patent board disagreed.

It is unclear about UC Berkeley's next steps. It might appeal as the university thinks its patent covers the use of CRISPR-Cas9 in all cells, including eukaryotes. Alternatively, CRISPR and Intellia might license

CRISPR for use in eukaryotes from Editas, which, in turn, would pick up the right to use single-guide and tracrRNA from UC Berkeley.

Katrine Bosley, President and CEO of Editas, commented: "We are pleased with the USPTO's decision of 'no interference in fact' for the patents that have been granted to the Broad Institute for their innovative and fundamental work on CRISPR-Cas9 genome editing. This important decision affirms the inventiveness of the Broad's work in translating the biology of the natural world into fundamental building blocks to create unprecedented medicines. At Editas Medicine, we are continuing to invest in this technology to build our business for the long-term and to create genome editing therapies for patients suffering from genetically-defined and genetically-treatable diseases."



### FATE PARTNERS WITH UMN TO ADVANCE NK CELL IMMUNOTHERAPY

Fate Therapeutics has announced the expansion of its research collaboration with the Regents of the University of Minnesota (UMN) to initiate the clinical translation of an off-the-shelf targeted NK cell cancer immunotherapy derived from an engineered induced pluripotent stem cell (iPSC) line.

The company aims to produce the product candidate from a single iPSC that is first genetically engineered to express a high-affinity, non-cleavable CD16 (hnCD16) receptor and then is clonally-expanded to generate a master engineered pluripotent cell line. Similar to master cell lines used for the manufacture of therapeutic antibodies,

a master engineered pluripotent cell line can be used to repeatedly create clonal populations of effector cells to enable off-the-shelf treatment of many thousands of patients. Preclinical production runs have shown that a single iPSC can yield a homogeneous population of over one million iPSC-derived NK (iNK) cells.

The company's hnCD16 receptor, which is licensed exclusively from UMN, incorporates two unique modifications designed to enhance the anti-tumor activity of NK cells. The receptor has been modified to augment its binding affinity to certain antibodies and also to prevent its shedding from the

surface of NK cells upon activation which can otherwise diminish the effector function.

The collaboration is being led by UMN's Dr Jeffrey S. Miller. UMN has granted Fate the exclusive license for intellectual property covering compositions of a modified CD16 as well as certain chimeric antigen receptors and of immune cells expressing such receptors. Fate also maintains an option to exclusively

license all intellectual property arising from research and development activities under the collaboration.

In addition to its collaboration with UMN, Fate has also partnered with Memorial Sloan Kettering Cancer Center (MSKCC) for the development of off-the-shelf T-cell product candidates using engineered iPSCs. Research and development activities are being led by MSKCC's Dr Michel Sadelain.



## ONES TO WATCH

### *Fate Therapeutics Expands iPSC-Derived Programs*

*Like Collectis, Fate Therapeutics envisions a future of frozen, off-the-shelf cell-based immunotherapy products. It recently announced a deal with Sloan Kettering to gain access to technology to derive T cells from pluripotent sources. Now the company*

*has expanded its collaboration with the University of Minnesota, taking an exclusive license to an allogeneic NK cell therapy technology. Unlike T-cell immunotherapies (CAR/TCR), which are designed to target cells directly to elicit a cytotoxic hit to cancer, NK cells act via antibody-dependent cellular cytotoxicity. So, Fate plans to investigate the technology along with antibodies, which form the standard-of-care for many oncology indications today. NK cells are derived from banked iPSCs that are first engineered to express high affinity CD16, a receptor that allows efficient binding to antibody-coated tumor cells. – Mark Curtis*



## EXONICS TO RECEIVE FUNDING FROM CUREDUCHENNE TO ADVANCE TREATMENT FOR DUCHENNE MUSCULAR DYSTROPHY

CureDuchenne Ventures, a subsidiary of the nonprofit CureDuchenne that funds research to find a treatment for Duchenne muscular dystrophy (DMD), have committed \$5 million in seed financing in Exonics Therapeutics, a new biotechnology company focused on utilizing gene editing technologies to advance the development of a DMD therapy.

DMD is an X-linked genetic progressive muscle disease affecting

more than 300,000 boys worldwide. It is caused by a mutation in the gene encoding the protein dystrophin. Loss of dystrophin affects the stability of skeletal muscle, leading to progressive muscle damage and dysfunction. Children with the disease start missing development milestones at age 3 and often lose their ability to walk by age 12. All patients display reduced mobility and ultimately respiratory



## FINANCE

or cardiac failure results in a reduced life expectancy in the mid-20s and currently there is no cure for DMD.

Exonics' CRISPR/Cas9 technology is a potential one-time treatment that would make a permanent correction of the mutation that causes DMD. The technology is licensed from the University of Texas Southwestern Medical Center and is based on the research of its scientific founder and chief science advisor, Dr Eric Olson. The initial seed funding will allow Exonics to advance the preclinical research findings of Dr Olson. Research in Dr Olson's laboratory has demonstrated the potential of using adeno-associated virus (AAV)

to deliver CRISPR/Cas9 to cells, to identify and correct exon mutations that prevent the production of dystrophin. Preclinical data published in *Science* suggests that this gene editing approach has the potential to permanently treat up to 80% of children who suffer from DMD.

Dr Olson, who also serves as Professor at the University of Texas Southwestern Medical Center commented: "This represents the next generation of potential Duchenne muscular dystrophy therapies. By leveraging the revolutionary CRISPR/Cas9 method to permanently correct errors in the DNA sequence, it is our hope that we can develop a one-time therapy that provides lifelong benefit to Duchenne patients."



### JACQUALYN FOUSE RETIRES FROM CELGENE

Celgene has announced that its President and Chief Operating Officer (COO), Dr Jacquelyn Fouse, has decided to retire from Celgene effective June 30, 2017. She will continue to serve as

President and COO until April 1, and through June 30, she will serve as a strategic advisor to the management team. At Celgene, Dr Fouse served as Chief Financial Officer and President of Hematology & Oncology Franchise before her promotion to President and COO in 2016.

In addition, the company has also announced the promotion of Scott Smith to President and COO, which is effective April 1, 2017, and the promotion of Terrie Curran to President, Global Inflammation and Immunology Franchise, which is effective April 1, 2017.

*Written by Applonia Rose, Commissioning Editor, Cell and Gene Therapy Insights*