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Industry updates from the field of stem cell research and regenerative medicine in June 2025

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INDUSTRY NEWS

Industry updates from the field of stem cell research and regenerative medicine in June 2025

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ABSTRACT
Latest developments in the field of Advanced Therapy Medicinal Products and regenerative medicine compiled from publicly available information and press releases from non-academic institutions in June 2025.

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KEYWORDS
Industry; stem cells; regenerative medicine; cell therapy; gene therapy

1. Business development

1.1. Collaboration agreement: Invetech and AiCella

Invetech (CA, USA; www.invetechgroup.com), a contract manufacturing organization, has announced a strategic collaboration with AiCella (CA, USA; www.aicella.io), a company focused on harnessing the power of artificial intelligence (AI) to optimize the development of cell therapy production processes and maximize patient response to treatment [1]. This partnership brings together Invetech's deep domain expertise in End-to-End custom automation with AiCella's cutting-edge artificial intelligence platform to deliver smarter, faster, and more cost-effective solutions for the industry – accelerating innovation and improving outcomes across the cell therapy landscape.

1.2. Collaboration agreement: Xcellbio and Thermo Fisher

Xcell Biosciences (CA, USA; www.xcellbio.com), an instrumentation company focused on cell and gene therapy applications, has announced a strategic collaboration with Thermo Fisher Scientific (MA; USA; www.thermofisher.com), to advance research in regulatory T cells (Tregs) and tumor-infiltrating lymphocytes (TILs) [2]. This collaboration aims to advance Treg and TIL cell therapies that specialize in combating autoimmune and solid tumor diseases.

1.3. Strategic partnership: Germfree Laboratories and KFSHRC

King Faisal Specialist Hospital & Research Centre (KFSHRC; Saudi Arabia; www.kfshrc.edu.sa) and Germfree Laboratories (FL, USA; www.germfree.com), have announced a strategic partnership to develop Saudi Arabia's first fully integrated, modular Advanced Therapy Medicinal Product (ATMP) Manufacturing Campus [3].

The new facility will be located at KFSHRC's main campus in Riyadh and will serve as a critical milestone in advancing Vision 2030 and the National Biotechnology Strategy, two national initiatives aimed at transforming the Kingdom into a global hub for life sciences, biomanufacturing, and to become a global biotech hub by 2040.

2. Achievements, launches ...

2.1. Akadeum

Akadeum Life Sciences (MI, USA; www.akadeum.com), an advanced cell separation technology company, has announced a groundbreaking workflow that leverages its proprietary microbubble platform to enable instrument-free, large-scale cell isolation for cell therapy manufacturing [4]. This innovative solution empowers researchers and manufacturers to efficiently isolate target cells directly in processing

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bags, eliminating the need for complex instrumentation and streamlining the cell therapy workflow to truly enable decentralized and point-of-care manufacturing models, reduce costs typical of complex cell therapy workflows such as GMP suite time by 80%, labor and training, and enable more broad access to life saving therapies.

The platform supports cell isolation of up to 50 billion cells directly within both sterile processing bags and freshly collected apheresis material, enabling pre-clinical through commercial scale with closed-system operations and the same reagents at each stage of development. This approach eliminates the need for traditional cell washing and isolation-specific instrumentation, minimizing cell handling steps, and reducing the complexity of cell therapy manufacturing.

2.2. Paradromics

Paradromics (TX, USA; www.paradromics.com), a neurote chnology company developing the data-rate brain–computer interface platform, has successfully completed its first-in-human procedure with the Connexus® Brain–Computer Interface (BCI) [5].

The team demonstrated that Connexus can be safely implanted, record electrical brain signals, and be removed intact in less than 20 minutes, using surgical techniques familiar to neurosurgeons worldwide. Connexus BCI was implanted during an epilepsy resection surgery to better understand how epilepsy influences brain signaling. The procedure confirms that the Connexus system can be used in humans, following nearly 3 years of preclinical studies using the same device.

2.3. Samsung Biologics

Samsung Biologics (Korea; https://samsungbiologics.com), a contract development and manufacturing organization (CDMO), has launched Samsung Organoids – advanced drug screening services to support clients in drug discovery and development [6].

Patient-derived organoids are 3D tumor models that mirror individual cancers. The company offers precision drug screening to help identify effective compounds with higher clinical relevance, covering colorectal, lung, liver, gastric, and breast cancers.

2.4. STEMCELL Technologies

STEMCELL Technologies (BC, Canada; www.stemcell.com) has commercially launched the STEMprep™ Tissue Dissociator System – a new benchtop instrument that automates, standardizes, and streamlines tissue dissociation, the process of breaking down tissue samples into single-cell suspensions for research purposes [7].

3. Clinical trials

3.1. Pluripotent stem cells

3.1.1. Vertex

Vertex Pharmaceuticals (MA, USA; www.vrtx.com) has announced a publication of updated data from the Phase 1/2 portion of the Phase 1/2/3 FORWARD-101 clinical trial of zimislecel (VX-880), an investigational cell therapy, in people with type 1 diabetes with impaired hypoglycemic awareness and severe hypoglycemic events [8–10].

All 12 participants were free of severe hypoglycemic events and had a glycated hemoglobin level of less than 7%; these participants spent more than 70% of the time in the target glucose range (70–180 mg/dL). Ten of the 12 participants (83%) had insulin independence and were not using exogenous insulin at day 365.

Zimislecel (VX-880) is an investigational allogeneic human embryonic stem cell (hESC)-derived, fully differentiated, insulin-producing islet cell therapy manufactured using proprietary technology.

4. Immune cell therapy

4.1. Bristol Myers Squibb

Bristol Myers Squibb (NJ, USA; www.bms.com) has announced the first disclosure of the primary analysis results of the marginal zone lymphoma (MZL) cohort of TRANSCEND FL, an open-label, global, multicenter, Phase 2, single-arm study evaluating Breyanzi® (lisocabtagene maraleucel; liso-cel) in patients with relapsed or refractory disease [11,12].

The MZL cohort of TRANSCEND FL enrolled adults with relapsed or refractory disease treated with liso-cel in the third-line plus setting. Patients received treatment with liso-cel at a target dose of 100×106CAR-positive viable T cells.

In efficacy-evaluable patients with relapsed or refractory MZL treated with liso-cel ($n = 66$), liso-cel demonstrated clinically meaningful benefit, with high rates of durable responses. The overall response rate (ORR) was 95.5% (95% CI: 87.3–99.1; one-sided $p < 0.0001$), with 62.1% of patients achieving a complete response (CR) (95% CI: 49.3–73.8; one-sided $p < 0.0001$) by independent review committee per CT. With a median follow-up of 21.6, 23.8, and 24.5 months, respectively, the 24-month rates were 88.6% for duration of response, 85.7% for progression-free survival, and 90.4% for overall survival.

Breyanzi is a CD19-directed CAR T cell therapy with a 4-1BB costimulatory domain, which enhances the expansion and persistence of the CAR T cells. Breyanzi is made from

a patient's own T cells, which are collected and genetically reengineered to become CAR T cells that are then delivered via infusion as a one-time treatment.

In a separate press release, the company has announced that the U.S. FDA has approved label updates for both of its CAR T cell therapies, Breyanzi for the treatment of large B cell lymphoma and other lymphomas and Abecma® (idecabtagene vicleucel; ide-cel) for the treatment of multiple myeloma [13]. These label updates reduce certain patient monitoring requirements and remove the Risk Evaluation and Mitigation Strategy (REMS) programs that had been in place since each product was initially approved.

4.2. Cabaletta Bio

Cabaletta Bio (PA, USA; www.cabalettabio.com), a clinical-stage biotechnology company focused on developing and launching targeted cell therapies designed for patients with autoimmune diseases, has announced new clinical and translational data from the ongoing RESETMyositis™, RESET-SLE™, and RESET-SSc™ trials evaluating rese-cel (resecabtagene autoleucel, formerly known as CABA-201) [14–18].

- Seven of eight myositis patients achieved clinically meaningful TIS responses after discontinuation of all immunomodulators, while off or actively tapering steroids; responses were sustained throughout the follow-up period in all responding patients [15].
- All SLE patients without nephropathy achieved the definition of remission in SLE (DORIS) as of the latest follow-up, and all seven SLE and LN patients experienced SLEDAI-2K reductions, while off all immunomodulators and steroid [17].
- Both scleroderma patients demonstrated clinically compelling mRSS improvement after discontinuation of all immunomodulators and steroids.
- In 18 patients with follow-up of 4 weeks or more, 94% had either no CRS or Grade 1 CRS (transient fever) and 89% had no ICANS (2 patients with previously reported ICANS events).
- Two registrational myositis cohorts with ~15 patients each are on track to initiate enrollment this year; registrational discussions with FDA are scheduled for SLE/LN in 3Q25 and anticipated for scleroderma in 4Q25 and myasthenia gravis in 1H26.
- RESET™ clinical trial program enrollment continues to accelerate, with 51 patients now actively enrolled and 24 patients dosed across the US clinical site network as of 30 May 2025.

Rese-cel is a 4-1BB-containing fully human CD19-CAR T cell investigational therapy for patients with autoimmune diseases where B cells contribute to the initiation and/or maintenance of disease. Following a one-time infusion of a weight-based dose, rese-cel is designed to transiently and deeply deplete all CD19-positive cells in both the peripheral circulation and within tissues. Cabaletta believes this approach has the potential to reset the immune system and result in profound clinical responses without chronic therapy requirements in patients. Cabaletta is currently evaluating rese-cel in the RESET™ (REstoring SElf-Tolerance) clinical development program, which includes multiple disease-specific, company-sponsored clinical trials across expanding portfolios of autoimmune diseases in a broad range of therapeutic areas, including rheumatology, neurology, and dermatology.

4.3. CARsgen

CARsgen Therapeutics (China; www.carsgen.com/en/) a company focused on developing innovative CAR T-cell therapies, has published the results of a pivotal Phase II clinical trial in China investigating satricabtagene autoleucel (satri-cel, CT041), a CLDN18.2-specific autologous CAR T-cell product candidate, in patients with CLDN18.2-positive, advanced gastric/gastroesophageal junction cancer refractory to at least two prior lines of treatment [19–21]. CLDN18.2 is a splice variant of *CLDN18* primarily expressed in gastric mucosa but aberrantly re-expressed in various cancers.

Satri-cel was associated with a statistically significant increase in progression-free survival and clinically meaningful increase in overall survival compared with control, along with a manageable safety profile in patients with previously treated, advanced, CLDN18.2-positive gastric or gastro-esophageal junction cancer. Globally, this is the first randomized controlled study of a CAR T-cell therapy in solid tumors and the first to demonstrate the superiority of CAR T-cell therapy versus standard of care.

4.4. Diakonos Oncology

Diakonos Oncology (TX, USA; www.diakonosoncology.com), a clinical-stage biotechnology company developing a new generation of immunotherapies to treat challenging and aggressive cancers, has announced promising results from its Phase I clinical trial of Dubodencel (DOC1021), a patient-derived dendritic cell (DC) therapy for the treatment of patients with glioblastoma [22,23]. The trial included patients with particularly challenging disease characteristics and demonstrated a favorable safety profile along with early signs of clinical activity. These results support advancement to a randomized Phase II trial.

The study evaluated Dubodencel (DOC1021), a patient-derived immunotherapy prepared from mobilized peripheral blood mononuclear cells, loaded with autologous tumor lysate

and amplified tumor mRNA, and administered near deep cervical lymph nodes. Sixteen newly diagnosed patients, with a median age of 61 years (range 47–73), of which 94% were *MGMT* unmethylated, and 25% with subtotal resection, completed three courses of the injection every 2 weeks alongside weekly pegylated interferon (peg-IFN), following chemotherapy and radiation. Four dose levels, ranging from 3.5 to 36 million total cells, were tested. Two additional patients with recurrent glioblastoma were also treated. *MGMT* unmethylated means that the *MGMT* gene's promoter region is not methylated, which typically leads to higher expression of the MGMT protein. This higher expression of the MGMT protein can make the tumor more resistant to chemotherapy, specifically alkylating agents like temozolomide (TMZ), because MGMT can repair the DNA damage caused by these drugs.

The 12-month overall survival (OS) rate was 88%, significantly higher than the expected ~60% for standard of care, and four patients are still alive at 22–33 months of follow-up. Recurrent glioblastoma patients survived 10–12 months.

4.5. Johnson & Johnson

Johnson & Johnson (IL, USA; www.jnj.com) has announced new long-term follow-up data from the Phase 1b/2 CARTITUDE-1 study demonstrating 33% ($n = 32$) of patients in the study ($n = 97$) with relapsed or refractory multiple myeloma (RRMM) treated with CARVYKTI (ciltacabtagene autoleucel; cilta-cel) achieved progression-free survival of 5 years or more with a single infusion and no maintenance or subsequent anti-myeloma therapy [24,25].

4.6. Qihan

Qihan Biotech (China; www.qihanbio.com), a genome editing technology, has published a case report study of Qihan's off-the-shelf, dual-targeting iPSC-derived CAR-NK cell product, QN-139b, in treating refractory systemic sclerosis (SSc) [26,27].

QN-139b is a universal, dual-targeting CAR-NK product that eliminates pathogenic B cells and plasma cells by targeting both CD19 and BCMA. The product was developed from Qihan's high-throughput gene editing platform and incorporates advanced features for enhanced safety and persistence. These include edits to nine genes, non-cleaving editing tools to prevent chromosomal rearrangements, transgene insertion into genomic "safe harbors," and production from sequenced monoclonal iPSC lines to minimize genomic toxicity. To further enhance product safety, QN-139b includes a tEGFR safety switch and an innovative CD16 knockout to reduce the risk of disease flares in autoimmune patients.

In the reported study, a 36-year-old female patient with a nearly 20-year history of diffuse cutaneous systemic sclerosis (dcSSc) received four doses of QN-139b (6×10^8 cells per dose) on days 0, 3, 7, and 10. After 6 months of follow-up, QN-139b demonstrated a strong safety profile and remarkable clinical efficacy:

- Significant reduction in autoantibodies and normalization of complement levels
- Dramatic improvement in modified Rodnan skin score (mRSS)
- Enhanced American College of Rheumatology Composite Response Index in Systemic Sclerosis (ACR-CRISS) score
- Histological evidence of B cell clearance, fibrosis suppression, lymphocyte depletion in affected tissues, and skin microvascular remodeling

5. Gene therapy

5.1. Pacira

Pacira BioSciences (CA, USA; www.pacira.com), the industry leader in the delivery of innovative, non-opioid pain therapies to transform the lives of patients, has announced new long-term follow-up data from its Phase 1 clinical trial evaluating PCRX-201 (enekinragene inzadenovec), a novel gene therapy candidate for osteoarthritis of the knee [28]. Results show that a single intra-articular injection of PCRX-201 was well tolerated and produced sustained improvements in pain, stiffness, and function through 156 weeks in patients with moderate-to-severe osteoarthritis of the knee.

PCRX-201 features an innovative design based on the company's proprietary high-capacity adenovirus, or HCAd, gene therapy vector platform. It is injected locally into the knee joint to boost cellular production of the IL-1 receptor antagonist (IL-1Ra) and block interleukin-1 pathway activation to improve chronic inflammation, pain, and function. PCRX-201's unique disease-modifying design also features an inducible promoter to mimic the body's natural response to inflammation by "turning up" the expression of IL-1Ra when inflammation is present in the joint and turning down expression once inflammation is quelled.

Given the promising Phase 1 results, dosing is underway in a Phase 2 study of PCRX-201 (the ASCEND study) for the treatment of osteoarthritis of the knee [29].

5.2. REGENXBIO

REGENXBIO (MD, USA; www.regenxbio.com) has announced new positive interim data from the Phase I/II AFFINITY DUCHENNE trial [30,31]. Updates include positive functional, safety, and biomarker data. At 9 months, RGX-202 participants

demonstrated improvement in function and exceeded external controls on all measures.

RGX-202 is an investigational gene therapy designed for improved function and outcomes in Duchenne. RGX-202 is the only gene therapy approved or in late-stage development for Duchenne with a differentiated microdystrophin construct that encodes key regions of naturally occurring dystrophin, including the C-Terminal (CT) domain.

Additional design features may potentially improve gene expression, increase protein translation efficiency, and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of microdystrophin throughout skeletal and heart muscle using the NAV® AAV8 vector and a well-characterized muscle-specific promoter (Spc5–12). RGX-202 is manufactured using REGENXBIO's proprietary, high-yielding NAVXpress™ suspension-based platform process.

6. Regulations, approvals, acquisitions ...

6.1. Acquisitions

6.1.1. Capstan and AbbVie

Capstan Therapeutics (CA, USA; www.capstantx.com), a clinical-stage biotechnology company dedicated to advancing in vivo reprogramming of cells through RNA delivery using targeted lipid nanoparticles (tLNP), has announced successful dosing of the first participants in its Phase 1 trial of CPTX2309, Capstan's lead anti-CD19 in vivo CAR-T candidate, for the treatment of B cell-mediated autoimmune disorders [32,33].

In a follow-up press release, the company has announced a definitive agreement under which AbbVie (IL, USA; www.abbvie.com) will acquire Capstan, including CPTX2309 and its proprietary tLNP platform technology designed to deliver RNA payloads, such as mRNA, capable of engineering specific cell types in vivo [34].

6.1.2. Eli lilly and Verve

Eli Lilly and Company (IL, USA; www.lilly.com) and Verve Therapeutics (MA, USA; www.vervetx.com), a clinical-stage company developing genetic medicines for cardiovascular disease, have announced a definitive agreement for Lilly to acquire Verve [35].

Verve is developing a pipeline of gene editing medicines designed to address the drivers of atherosclerotic cardiovascular disease (ASCVD) through treatments that may only need to be given once in a lifetime. Verve's lead program (VERVE-102) is a potential first-in-class in vivo gene editing medicine targeting *PCSK9*, a gene linked to cholesterol levels and cardiovascular health. The treatment may be applicable for people who have heterozygous familial hypercholesterolemia, a subset of ASCVD that affects 1 in 250 people in the general population, as well as certain patients with premature coronary artery disease. VERVE-102 is being evaluated in a Phase 1b clinical trial study and has been granted Fast Track designation by the U.S. FDA [36].

6.1.3. STEMCELL and Cellular Highways

STEMCELL Technologies (BC, Canada; www.stemcell.com) has announced the acquisition of Cellular Highways (UK; www.cellularhighways.com), a biotechnology company that specializes in advanced cell sorting technologies with applications in cell and gene therapy, drug discovery, and general cell research, especially where cells are fragile [37]. The acquisition includes the revolutionary Highway1 instrument, which uses Cellular Highways' proprietary Vortex-Actuated Cell Sorting (VACS™) technology. Highway1 enables researchers and cell therapy developers to process cells more efficiently while maintaining high cell integrity for sensitive applications in research and therapeutic development, reducing the timelines and costs for research and development.

7. Green light

7.1. Beam

Beam Therapeutics (MA, USA; https://beamtx.com), a biotechnology company developing precision genetic medicines through base editing, has announced that the US. FDA has granted orphan drug designation to BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD) [38].

The one-time therapy consists of autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) that have been base-edited in the promoter regions of the *HBG1/2* genes and are administered via a hematopoietic stem cell transplant procedure. The BEAM-101 edit is designed to inhibit the transcriptional repressor *BCL11A* from binding to the promoter without disrupting *BCL11A* expression, leading to increased production of non-sickling and anti-sickling fetal hemoglobin (HbF) and thus mimicking the effects of naturally occurring variants seen in hereditary persistence of fetal hemoglobin. HbF is the predominant hemoglobin variant during development and early life. The safety and efficacy of BEAM-101 is being evaluated in the ongoing BEACON Phase 1/2 study, an open-label, single-arm, multicenter trial in adult patients with SCD with severe vaso-occlusive crises [39].

7.2. Capsida

Capsida Biotherapeutics (CA, USA; https://capsida.com) has cleared the Investigational New Drug (IND) application with

the U.S. FDA for CAP-003, its potential best-in-class intravenously (IV) administered gene therapy, to enter clinical trials for Parkinson's disease associated with *GBA* mutations (PD-GBA) [40,41]. Capsida uses a proprietary manufacturing process and CAP-003 is manufactured in Capsida's state-of-the-art wholly owned Good Manufacturing Practice (GMP) facility.

7.3. Cerapedics

Cerapedics (CO, USA; www.cerapedics.com), a commercial-stage orthopedics company dedicated to redefining the path to bone repair, has announced the U.S. FDA premarket approval of PearlMatrix™ *p*-15 Peptide Enhanced Bone Graft as a Class III drug–device combination product for use in single-level transforaminal lumbar interbody fusion surgery in adult patients with degenerative disc disease [42]. *p*-15 Peptide is a 15 amino-acid sequence found naturally in Type-1 collagen, the predominant protein in bone. It serves a crucial role in the bone regeneration process as a powerful cell attachment factor. Cerapedics' pharmaceutically manufactured *p*-15 Peptide is bound onto calcium phosphate particles, creating a *p*-15-enhanced scaffold that provides an abundance of attachment sites for osteogenic, bone-forming, cells. Cell attachment activates pathways that release cell-signaling growth factors and allow bone growth through natural cellular processes.

7.4. Myrio

U.S. FDA has approved an IND application enabling Myrio's (Australia; www.myriotx.com) lead product PHOX PC-CAR T to enter clinical trials for the treatment of neuroblastoma [43]. A functionally relevant and highly specific protein, called PHOX2B, was identified in neuroblastoma cells and the team elucidated that a peptide from PHOX2B could serve as an excellent target for an immunotherapy to treat this devastating disease.

Myrio has developed an entirely novel discovery platform, Retained Display (ReD^{TM}), which is geared toward the discovery of highly stable, full human, scFv binders against peptides presented on the surface of solid cancer cells as presented by the HLA complex. These binders have repeatedly shown their utility in bispecific T-Cell Engager or CAR-T formats to direct cytotoxicity against PHOX2B peptide-human leukocyte antigen (pHLA) specific target tumor cells. In the case of a bispecific T-Cell Engager, the molecule binds to both the pHLA and a T cell – the T cell then kills the tumor cell. In the case of a CAR-T therapy, the antibody is expressed on the surface of a CAR-T cell targeting it against the peptide-major histocompatibility complex (pMHC), directing the killing of the tumor cell.

7.5. Nuevocor

Nuevocor (Singapore; www.nuevocor.com), a biotechnology company developing cures for cardiomyopathies driven by aberrant mechanobiology, has announced that the U.S. FDA has cleared its IND application for NVC-001 [44]. NVC-001 is an adeno-associated virus (AAV)-based gene therapy designed to treat LMNA-related dilated cardiomyopathy (LMNA DCM). The company demonstrated in animal studies that reducing cytoskeletal force transmission to the nucleus by uncoupling the LINC complex appears to be a viable strategy for treating LMNA-linked pathology, and multiple GSLA01 truncation variants can be used to achieve this effect.

7.6. Sarepta

Sarepta Therapeutics (MA, USA; www.sarepta.com), a company developing precision genetic medicine for rare diseases, has announced that the rAAVrh74 viral vector used in the investigational gene therapy SRP-9003 (bidridistrogene xeboparvovec) for the treatment of limb-girdle muscular dystrophy type 2E/R4, has been granted Platform Technology Designation by the U.S. FDA [45–47].

Platform Technology Designation is a program designed to expedite the development and review of drugs and biologics that utilize a well-understood and reproducible technology. This designation recognizes a technology (like a nucleic acid sequence, molecular structure, or delivery method) that has the potential to be used in multiple drug products without compromising safety or quality.

SRP-9003 is intended to deliver a full-length beta-sarcoglycan transgene and uses the *MHCK7* promoter, chosen for its ability to robustly express in the heart which is critically important for patients with limb-girdle muscular dystrophy Type 2E (LGMD2E), also known as beta-sarcoglycanopathy and *LGMDR4*, many of whom die from pulmonary or cardiac complications.

In an unrelated press release, the company provided a safety update regarding Elevidys (delandistrogene moxeparvovec-rokl) in non-ambulatory individuals with Duchenne muscular dystrophy [48,49]. Following the death of a second non-ambulatory patient from acute liver failure, the company is pausing the ENVISION study (SRP-9001–303), a global clinical trial evaluating ELEVIDYS in older ambulatory and non-ambulatory individuals. ELEVIDYS is an adeno-associated virus (AAV) vector-based gene therapy designed to deliver a gene encoding a micro-dystrophin protein [i.e., a shortened (138 kDa) version of the dystrophin protein expressed in normal muscle cells (427 kDa)] to all muscles involved in the pathology of Duchenne muscular dystrophy.

7.7. Senti Bio

Senti Biosciences (CA, USA; www.sentibio.com), a clinical-stage biotechnology company developing next-generation cell and gene therapies using its proprietary Gene Circuit platform, has announced that the U.S. FDA has granted Orphan Drug Designation to SENTI-202 designed to selectively target and eliminate CD33 and/or FLT3-expressing hematologic malignancies [50].

SENTI-202 is a novel NK cell product candidate to be engineered with both OR and NOT logic gated CAR gene circuits, wherein the OR gate is designed to increase AML LSC/blast tumor clearance (to prevent relapse), and the NOT gate is designed to protect healthy HSCs from off-tumor toxicity, enabling regeneration of a healthy hematopoietic system and mitigating the need for a bone marrow transplant.

7.8. YolTech

YolTech Therapeutics (China; www.yoltx.com), a clinical-stage biotechnology company developing in vivo genome editing therapies, has announced that the U.S. FDA has cleared the company's IND application for YOLT-101, an in vivo based editing therapy targeting *PCSK9* for the treatment of heterozygous familial hypercholesterolemia [51].

YOLT-101 is based on YolTech's proprietary adenine base editor, YolBE – specifically hpABE5—which comprises nCas and a novel deaminase evolved from *Hafnia paralvei*. For delivery, YOLT-101 utilizes YolTech's innovative lipid nanoparticle delivery system. Unlike traditional CRISPR/Cas9 systems that rely on DNA double-strand breaks (DSBs), hpABE5 enables precise A•T to G•C base conversion without introducing DSBs, thereby significantly reducing the risk of chromosomal abnormalities and off-target effects.

8. Capital market and finances

8.1. AvenCell

AvenCell Japan, a wholly owned subsidiary of AvenCell Therapeutics (MA, USA; https://avencell.com), a private, clinical-stage biotechnology company developing CAR-T therapies for hematologic cancers and autoimmune diseases has been awarded a grant of up to US$40 M from the Japan Agency for Medical Research and Development (AMED) [52]. This non-dilutive funding will support the worldwide development of AvenCell's AVC203 candidate – an IND-stage, dual-antigen (CD19 and CD20) allogeneic CAR-T therapy for applications in B-cell Lymphomas.

AvenCell's unique and proprietary allogeneic technology is differentiated from numerous previous cell engineering approaches by applying multiple gene editing steps that ensure a patient's immune system (both innate and adaptive components) is left with no ability to reject the donor cells. Importantly, AvenCell's approach also assures that the healthy donor T-cell fitness and potency are not compromised during the cell manufacturing process.

8.2. BioNtec

With its oncology pipeline increasingly focused on bispecifics and antibody-drug conjugates, BioNTech (Germany; www.biontech.com) has limited the scope of its sole CAR-T cell therapy program [53]. The company had planned last year to soon move BNT-211, a CLDN6-directed CAR-T, into a pivotal Phase II study for relapsed or refractory testicular cancer/germ cell tumors. Now, BioNTech is discontinuing the candidate's development in that indication after "a thorough assessment of data from a signal-seeking Phase I clinical trial. As a result, BioNTech is shutting down CAR-T manufacturing at a site in Gaithersburg, MD, USA, leading to a headcount reduction in the cell therapy technical operation team.

According to the state Work Adjustment and Retraining Notification (WARN), 63 employees at the Gaithersburg site have been let go.

8.3. Portal

Portal Biotechnologies (MA, USA; https://portal.bio), a cell engineering platform company, announces that it has been awarded an $8 M contract with the Defense Advanced Research Projects Agency (DARPA)'s Biotechnology Office (BTO) for its Red Blood Cell (RBC) Factory Program [54]. The program's mission is to develop a compact, portable system capable of rapidly loading diverse therapeutic cargo into red blood cells (RBCs) at the point of care, unlocking field-deployable, personalized, and scalable cell therapies.

8.4. SpliceBio

SpliceBio (Spain; https://splice.bio), a clinical-stage genetic medicine company pioneering Protein Splicing to address diseases caused by mutations in large genes, today announced the close of a US$135 M Series B financing [55]. The funding will be used to advance the clinical development of SpliceBio's lead gene therapy candidate, SB-007 for Stargardt disease, including the ongoing interventional Phase 1/2 ASTRA study and the observational POLARIS study. SB-007

is the first dual adeno-associated viral (AAV) gene therapy cleared by the U.S. FDA to enter clinical development for Stargardt disease. SB-007 has also received regulatory clearance for clinical development from the UK Medicines and Healthcare products Regulatory Agency (MHRA).

SB-007 is designed to address the underlying genetic cause of the disease by producing a functional copy of the full-length ABCA4 protein with the potential to treat all patients, regardless of their specific ABCA4 mutation.

Disclosure statement

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