In a blockbuster deal, Astellas Pharma (Tokyo, Japan; https://www.astellas.com/) agreed to acquire Audentes Therapeutics (San Francisco, CA; https://www.audentestx.com/) for ~$3 billion cash, which the companies said is intended to create a top-tier gene therapy developer.

Upon completion—expected in early 2020—Astellas would add to its four primary focus areas with a fifth that will focus on genetic regulation, a category broad enough to include gene therapy, which Astellas and Audentes asserted “will be a key driver of the company’s future growth.”

“By joining together with Audentes’ talented team, we are establishing a leading position in the field of gene therapy with the goal of addressing the unmet needs of patients living with serious, rare diseases,” Astellas president and CEO Kenji Yasukawa said in a statement.

At least one group of analysts agreed with Yasukawa’s ambitious vision for the combined company, under which Audentes would continue to operate as an independent subsidiary of Astellas.

“We believe that the combined entities are expected to provide an industry-leading gene therapy company with [Audentes’] growing neuromuscular portfolio expected to complement Astellas’ existing pipeline,” Joseph P. Schwartz, managing director, rare diseases with SVB Leerink, and two of the firm’s equity research associates, Dae Gon Ha and Joori Park, concluded in an investor note.

Investors responded with a buying spree that more than doubled Audentes’ share price on the Nasdaq market, up to $58.93 on December 3 from the previous day’s closing price of $28.61. Shares of Astellas on the Tokyo Stock Exchange slipped 1% at the close of trading on December 3, 2019.

PIPELINE PROMISE

Audentes’ gene therapy pipeline is led by AT132, an adeno-associated virus 8 (AAV8) vector containing a functional copy of the MTM1 gene, as a treatment for X-linked myotubular myopathy.

The companies say they can accelerate development of a pipeline of new potentially best-in-class genetic medicines for rare neuromuscular diseases by combining Astellas’ scientific expertise and global resources with Audentes’ AAV technology platform, neuromuscular development expertise, and in-house large-scale current good manufacturing practice (CGMP) manufacturing.

THERMO MANUFACTURING

Thermo Fisher Scientific (Waltham, MA; https://www.thermofisher.com/us/en/home.html) has completed two facilities to enable its viral vector contract development and manufacturing organization (CDMO) business to satisfy growing demand for gene therapies for clinical and commercial use.

On December 4, 2019, Thermo Fisher Scientific officially opened a $90-million commercial facility in Lexington, MA, created to support the development, testing, and manufacture of viral vectors. The company plans to base >200 jobs at the 50,000-square foot site, a new CGMP manufacturing and quality control (QC) operations facility that will include commercial-ready grade 2 clean rooms for viral vector manufacturing.

Meanwhile, in Alachua, FL, Thermo Fisher unveiled a $6-million expansion of its gene therapy and viral vector services site. The investment doubled the site’s laboratory and warehousing capacity for upstream process development and QC testing of gene therapy products.

Thermo Fisher committed to investing $270 million in 2019 to expand its capabilities and talent globally, with the aim of enabling customers to develop and deliver high-quality medicines to patients. The investment followed the company’s acquisition of Brammer Bio for ~$1.7 billion, which closed last May.

BOSTON’S BIOLOGICAL INNOVATION

A group of Massachusetts academic, health care, biotech, and biopharma industry leaders have established a new $50-million center for advanced biological innovation and manufacturing, with the intent of addressing the bottleneck in the discovery of novel gene therapies and other emerging treatments.

The new center will cultivate innovations in cell and gene therapy, as well as advance biologic innovation and manufacturing, and accelerate
developments in immunotherapy, cell therapies, gene editing, and other technologies that show promise toward impacting human health globally and boosting the local economy.

By fostering collaboration and innovation, the center aims to speed innovation and broaden the universe of patients who can be treated with emerging therapies. More than 60,000 patients globally now participate in clinical trials for new cell and gene therapies, the leaders said, including those that apply gene editing.

Leaders from Harvard University, MIT, Fujifilm Diosynth Biotechnologies, GE Healthcare Life Sciences, and Alexandria Real Estate Equities will comprise the board of directors, whereas other contributing members will include Beth Israel Deaconess Medical Center, Boston Children’s Hospital, Brigham, and Women’s Hospital, the Dana-Farber Cancer Institute, Massachusetts General Hospital, MilliporeSigma, and the Commonwealth of Massachusetts.

The center will be an independent nonprofit organization located in the greater Boston area and will be named, and formally incorporated, in 2020.5

 FUJIFILM GOES BIG IN TEXAS

Fujifilm (Tokyo, Japan; https://www.fujifilm.com/) said its Fujifilm Diosynth Biotechnologies business (https://fujifilmdiosynth.com/) has committed ~$120 million toward expanding its capabilities in gene therapy development—nearly half of which will fund a new Gene Therapy Innovation Center the company plans to establish next to its CGMP gene therapy manufacturing facility in College Station, TX.

The $55 million Gene Therapy Innovation Center is expected to begin operation in the fall of 2021. The center will be ~60,000 square feet and will house upstream, downstream, and analytical development technologies, Fujifilm Diosynth Biotechnologies said.

Fujifilm has identified gene therapy investment as a priority growth area in its CDMO business. Fujifilm established a standalone biopharma CDMO operating unit in 2017, separating that business from its pharmaceutical products division, and announced plans to expand capacity at its U.S. and U.K.-based CDMO facilities.6,7

SICKLE CELL

Joining the promising clinical news released recently by CRISPR Therapeutics and the Food and Drug Administration approval of Global Blood Therapeutics’ lead molecule, bluebird bio (Cambridge, MA; https://www.bluebirdbio.com/) has announced positive data from its investigational Lentiglobin gene therapy for sickle cell disease (SCD) in three treatment cohorts of patients.

As of the cutoff date of August 26, 2019, all 17 patients in group 3 were treated with Lentiglobin, of which 9 had at least 6 months of follow-up who had four or more vaso-occlusive crises (VOCs) or acute chest syndrome (ACS) events in the 2 years before treatment.

Among those nine patients, there was a 99% reduction in annualized rate of VOC and ACS, bluebird bio said. There were no reports of ACS or serious VOC at up to 21 months post-treatment in these patients. As previously reported, there was one nonserious grade 2 VOC that was observed in a patient ~3.5 months post-LentiGlobin for SCD treatment. In addition, seven out of nine total patients in groups 1 and 2 (five out of seven in group 1; all two in group 2) did not require regular red blood cell transfusions post-treatment.

All seven group 1 patients had at least 3 years of post-treatment follow-up. Levels of HbAT87Q and total hemoglobin remained durable in all seven. At last evaluable visit, median HbAT87Q levels were 0.9 g/dL and total hemoglobin was 9.0 g/dL.

For both patients in group 2, levels of HbAT87Q and total hemoglobin remained durable at 2 years of post-treatment follow-up. At last visit, HbAT87Q levels were 3.6 and 7.1 g/dL, and total hemoglobin was 11.3 and 13.0 g/dL.

Overall, patients in groups 1 and 2 experienced a reduction of VOC and ACS events at 2 years post-treatment, but not complete elimination.

The data were presented at the 61st American Society of Hematology Annual Meeting and Exposition, held last month in Orlando, FL.8,9

REFERENCES


