PRODUCT PIPELINE | GENE THERAPY (DECEMBER 8, 2017)

NAME	COMPANY	TYPE	CLINICAL TRIALS	DESCRIPTION
BMN 270	BioMarin Pharmaceutical, based on a gene therapy program from the University College London and St. Jude's Research Hospital in Tennessee	Gene therapy for hemophilia A	In July 2016, it announced early results from a Phase 1/11 clinical trial in 7 patients who had received the higher dose of 6 X 10^{13} vector particles. 12 to 28 weeks post-infusion, one patient achieved 10% expression of FVIII, while the 6 others achieved levels greater than 50%, with two of them over 200% of normal. Levels have remained high for at least 18 months. In October 2016, Biomarin enrolled 6 more patients in its Phase I/IIa trial. In February 2017, the European Medicines Agency announced it had granted access to its Priority Medicines regulatory initiative, based on early clinical data showing its potential to benefit patients. In August 2017, Biomarin announced it was expanding its research to two Phase III trials with two different doses.	BMN 270 uses adeno-associated viruses (AAV5) as the delivery vehicle, or vector, to carry the genetic codes that prompt the production of the factor VIII protein that is deficient in people with hemophilia A.
SPK-9001	Spark Therapeutics in collaboration with Pfizer	Gene therapy for hemophilia B	In July 2016, Spark announced the early results of a Phase I/II trial. Four patients, 12 to 31 weeks post-infusion with a low dose of vector, exhibited FIX levels of 21 to 42 percent. This dose required no course of steroids to prevent an immune reaction. As of June 2017, 10 patients had been dosed, all achieving levels between 20 and 40 percent. 2 of 10 patients needed a short course of steroids to counteract rejection of the viral vector. Spark Therapeutics and Pfizer Inc. announced that the US Food and Drug Administration (FDA) has granted breakthrough therapy designation to SPK-9001. A Phase III trial will start in 2017.	SPK-9001 is a novel bio-engineered adeno-associated virus (AAV5) capsid expressing a codon-optimized, high-activity human factor IX variant, the Padua gene, enabling endogenous production of factor IX.
AMT-060 and AMT-061	uniQure	Gene therapy for hemophilia B	In July 2016, uniQURE announced early results from its Phase I/II trial of five patients in the low dose cohort who had completed 36 weeks of follow-up and had FIX expression levels between 2 and 7% of normal. None of the 10 had developed antibodies against the vector or the factor IX. In January 2017, uniQURE announced that the U.S. FDA had granted Breakthrough Therapy designation. In October 2017, uniQure announced it would advance its research in 2018 using	AMT-060 consists of a codon-optimized wild type FIX gene and the LP1 liver promoter together with the AAV5 viral vector. AMT-061 consists of a Padua-type FIX gene, with 8 to 9 times the FIX expression of the wild-type, and the LP1 liver promoter together with the AAV5 viral vector.

			the FIX-Padua mutant to achieve higher expression.	
SB-FIX ZFN-mediated Genome Editing: In Vivo Protein Replacement Platform (IVPRP)	Sangamo Biosciences	Gene therapy for hemophilia B	In December 2015, the U.S. FDA approved Sangamo's application for its Investigational New Drug. In 2016, the company will initiate a Phase I/II clinical study to assess safety and potential efficacy in hemophilia B.	The IVPRP uses Sangamo's zinc finger DNA-binding protein (ZFP) genome-editing technology to enable the permanent production of factor IX protein from a specific genomic site in the liver with a single systemic treatment.
SB-525	Sangamo Biosciences & Pfizer	Gene therapy for hemophilia A	Sangamo Therapeutics announced in 2017 that its Investigational New Drug (IND) application for SB-525 had been cleared by the U. S. Food and Drug Administration (FDA) The company expects to start a Phase I/II clinical trial in 2017.	SB-525 is a gene therapy approach to the treatment of hemophilia A that uses a Factor 8 cDNA adeno-associated virus.
DTX101	Dimension Therapeutics	Gene therapy for hemophilia B	In January 2016, Dimension began a Phase I/II clinical trial to enroll 12 patients. However, in May 2017, Dimension announced that it would stop development.	