PRODUCT PIPELINE | FACTOR CONCENTRATES (FEBRUARY 22, 2018)

NAME	COMPANY	TYPE	CLINICAL TRIALS	DESCRIPTION
Bax 111 (vonicog alfa) (Vonvendi in the U.S.)	Shire	Recombinant von Willebrand factor	In August 2015, Baxalta (now part of Shire) announced the publication of pivotal Phase III study results of Bax 111 with 37 patients with Type 3 VWD. Bleeding was treated successfully in all patients. In December 2015, the U.S. FDA granted Baxalta a biologics license for Vonvendi. In June 2017, the European Medicines Agency approved Vonvendi for use in adults with VWD. Shire submitted an application for approval from Health Canada in early 2018.	This is a recombinant von Willebrand factor that preserves ultra-high molecular weight multimers for the treatment of VWD. Its mean half-life is 21.9 hours.
N8-GP (turoctocog alfa pegol)	Novo Nordisk	Recombinant factor VIII	The pathfinderT2 Phase III trial for hemophilia A patients aged 12 years or older was completed in March 2014. 175 patients were treated prophylactically (50 IU/kg every 4 days, resulting in a mean trough level of 8%) and 11 patients were treated on demand. One inhibitor developed among the 186 patients treated.	This glycopegylated rFVIII was shown to have a half-life of 18.4 hours, approximately 1.5 times most current treatments.
BAY94-9027	Bayer	Recombinant factor VIII	In February 2014, Bayer announced positive results from its *Protect VIII* Phase III trial in 134 adolescents and adults, who received FVIII every 7, 5 or 3.5 days. The study met its primary objective of protection from bleeds with fewer infusions. No inhibitors were reported. In October 2017, Bayer submitted a Biologics License Application to the U.S. FDA, which is now being reviewed.	This is a pegylated, long-acting plasma/albumin free, full-length rFVIII. The goal is to increase half-life and reduce the frequency of infusions.
BAX 826	Shire	Recombinant factor VIII	In March 2016, Baxalta (now part of Shire) reported dosing of the first patient in its Phase I clinical trial of BAX 826. In May 2017, Shire announced a pre-defined once-weekly dosing target was not met.	BAX 826 uses proprietary polysialic acid (PSA) technology to extend its circulating half-life.

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CB2679d/ISU304	Catalyst Biosciences, Inc. & ISU Abxis	Recombinant factor IX	In April 2017, the company announced it had received approval from the Korean Ministry of Food and safety to begin human clinical trials for its new investigational drug CB2679dISU304. In September 2017, the company reported on Phase I/II trial results, which showed a potency 22 times that of Benefix in IV transfusions, demonstrating proof-of-concept.	The company claims that CB 2679d/ISU304, a highly potent next-generation coagulation Factor IX variant, has demonstrated the potential to normalize human Factor IX levels with a daily subcutaneous injection in preclinical studies.
Coagadex (Factor X)	Bio Products Laboratory (BPL)	A plasma-derived factor X concentrate to treat hereditary factor X deficiency	In April 2015, BPL announced results from its pivotal Phase III trial. Control of bleeding was excellent or good in 98% of bleeding episodes in 16 patients. No adverse events caused withdrawal from the trial. In October 2015, the U.S. FDA approved Coagadex for use. In February 2016, the European Medicines Agency recommended to grant market authorization.	Human coagulation factor X is a protein derived from human plasma.