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Several therapies for hemophilia A, hemophilia B and hemophilia with inhibitors to be launched between 2015-2025

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Decision Resources Group forecasts that several therapies will launch for hemophilia A, hemophilia B and hemophilia with inhibitors in the United States and Europe during our 2015-2025 study period. New factor IX agents will have a significant impact in the hemophilia B space, but new factor VIII agents will fail to make a strong impression on the hemophilia A market. The launch of agents with novel mechanisms of action will provide competition for classic factor replacement therapies in hemophilia A and hemophilia A with inhibitors. Gene therapy for hemophilia B is forecast to launch late in our study period; it will provide another treatment option for these patients.

Key findings from the Niche Markets & Rare Diseases report entitled *Hemophilia:*

- **Extended half-life factor IX products are set to revolutionize the treatment of hemophilia B.** The emergence of longer-acting agents such as Alprolix (Biogen) and CSL654 (CSL Behring) offer significant advantages in dosing intervals (decreased frequency of infusions) and interviewed physicians believe they can support significant improvements in clinical outcomes. By 2025, we forecast Alprolix and CSL654 to capture majority market share amongst the extended half-life FIX products
- **Despite the expected launch of several new drugs for hemophilia A, the very high unmet need for hemophilia A patients will remain**. We do not expect the extended half-life factor VIII concentrates to significantly impact the market. This meager performance will be a result of their only marginal increase in half-life; frequency of infusions with the longer-acting FVIII products is similar to the frequency of infusion given with standard half-life products. We forecast Eloctate (Biogen) and CSL 627 (CSL Behring) to capture majority market share amongst the extended half-life FVIII products. Personalized prophylaxis regimens and pharmacokinetic tailoring are expected to gain traction in severe hemophilia A over our forecast window.
- **Inhibitor patients remain one of the major challenges in hemophilia,** particularly severe hemophilia A patients. Bypass agents are required in 20 to 30 percent of patients in whom immune tolerance induction is inefficacious. Physician concerns around the half-life, clinical efficacy and cost-effectiveness of current agents will be significantly addressed by the launch CSL 689 (CSL Behring), LR769 (rEVO) and ACE910 (Chugai/Roche). ACE910 represents a highly anticipated and novel way of prophylactically treating hemophilia A patients with inhibitors.
- **Gene therapy would make a significant contribution to the presently large unmet need in hemophilia A but it is still a remote option for this patient group,** while in hemophilia B gene therapy may not be necessary. The longer-acting hemophilia B treatments are largely viewed as "safe" and will already have made a significant impact with respect to clinical outcomes by the time the gene therapy BAX 335 launches toward the end of our forecast window.

Comments from Decision Resources Group Business Insights Analyst, Kerri Brown:

- "We anticipate that several additional therapies will receive regulatory approval for hemophilia A, hemophilia B and hemophilia with inhibitors in the United States and Europe during our 2015-2025 window. The emergence of the FIX longer-acting factor concentrates (LAFCs) will revolutionize the treatment of hemophilia B, while the FVIII LAFCs will not make a significant impact in the treatment of hemophilia A. For this reason, we expect to see an increase in personalized prophylaxis and pharmacokinetic tailoring in severe hemophilia A through 2025. Emergence of longer acting agents and transgenically produced agents, for hemophilia with inhibitors, will address physician concerns about the half-life of current bypass agents and cost of therapy, respectively. Launch of the novel agent ACE910 toward the tail end of our forecast window has the potential to revolutionize the treatment of hemophilia A and hemophilia A with inhibitors."

Source:
Decision Resources Group
