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HEMLIBRA: A CLINICAL AND FINANCIAL GAME CHANGER FOR TREATMENT OF HEMOPHILIA

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For the first time in nearly 20 years, a new hemophilia A treatment is available that carries with it the potential to be both a clinical and financial game changer. On November 16, 2017, the Food and Drug Administration (FDA) approved Hemlibra (Hem-LEE-bra), a subcutaneous injection for routine prophylaxis, to prevent or reduce the frequency of bleeding episodes in hemophilia A patients with factor VIII inhibitors. The prevalence of hemophilia A in the US is estimated to be 15,500, of which 950 are currently estimated to have inhibitors. The development of inhibitors makes it more difficult and expensive to stop a bleed because the factor replacement administered to the patient is neutralized and the patient's blood does not clot. Inhibitor patients require either very large doses of traditional factor therapy or expensive "bypassing" agents in order to control or prevent bleeds.

The clinical and financial implications for Hemlibra are significant, as this drug restores the function of missing factor VIII without introducing antibody (e.g., inhibitor) development. Patients who develop inhibitors frequently experience joint damage and reduced mobility are twice as likely to be hospitalized for a bleeding complication and are at an increased risk of death. The Centers for Disease Control estimates the average annual health care expenditures for a patient with inhibitors are about five times higher than for a patient without inhibitors. To control a bleeding episode, inhibitor patients can utilize bypassing agents, which go around the factor to help form a clot. To put this into financial perspective, treating a single bleeding episode with a bypassing agent can cost $50,000 or more, and some patients with severe disease can generate bypassing agent costs from $300,000 to $2.5 million each year.

As is the current trend in newly approved specialty and orphan drugs, therapeutic advancements often come at a cost. Wholesale acquisition cost for Hemlibra is estimated to be $482,000 for the first year of therapy for an average patient. The institute for Clinical and Economic Review recently released a final report on the cost effectiveness of Hemlibra. Findings note that despite Hemlibra's high price tag, there is an expected annual average cost savings of $720,000 or $1.85 million per patient, for ages greater than or equal to 12 years and less than 12 years, respectively. When compared to bypassing agent prophylaxis, Hemlibra would still generate cost savings even if the unit priced increased by more than 500%.

So what is next on the horizon for this groundbreaking drug? Aside from the high list price and potential savings compared to standard therapies, Hemlibra has the potential to disrupt the current hemophilia treatment distribution ecosystem. At this time, Hemlibra is not considered factor therapy in the Drug Data Reporting for Medicaid system; therefore, it will be subject to the standard federal drug rebate rather than the lower rebate, which applies to hemophilia factor products. Due to the lower hemophilia drug rebate and

the availability of sub-ceiling discounts for factor products in the 340B program, some state Medicaid programs have been able to save costs on factor therapy through mutually-beneficial arrangements with 340B eligible hemophilia treatment centers (HTCs). It remains unclear at this time if significant sub-ceiling discounts on Hemlibra will be available for 340B providers. States with high utilization of HTC providers will need to consider this provider impact as Hemlibra has the potential to shift utilization away from factor therapy. Given the complexity of both clinical and financial variables in the treatment of hemophilia A, one thing for certain is that states will need to closely evaluate their current policy and reimbursement strategy. One last item for those who enjoy speculation, the FDA recently granted breakthrough therapy designation for use in hemophilia A patients *without* inhibitors. An accelerated approval for this indication has the potential to greatly expand the hemophilia population who could be candidates for Hemlibra from 950 patients to a whopping 15,500 patients.