Cost Drivers

Three forces will drive costs for hemophilia treatment going forward. The first is the rapid rise in life expectancy for hemophiliacs as improved treatment regimens successfully confront the disease. The second is the continued role that inhibitors play. The third will be the appearance of new drugs which are already proving to be more expensive.

Driver 1: Longer lifespans

This long-term cost driver is just beginning to come in for closer scrutiny. The appearance of safe, reliable factor replacement therapies have helped to stabilize hemophilia patients and give them much more normal lives. As a result, we face a demographic shift with increasing numbers of persons afflicted with the disease who are now living far longer than they once did, even just a few decades ago.¹

In the 1800’s, patient life expectancy was only about 13 years.² By the 1960’s it had improved somewhat, to about age 20.² Today, a baby born with hemophilia in one of the advanced economies can expect to live a normal lifespan (with proper treatment).¹,³

This sudden advance in longevity has both obvious and non-obvious implications.

At the obvious level, while the cost to treat hemophilia shows no signs of coming down, we shall need to treat these patients for many years. And, drug makers are increasingly turning their attention to newer drugs that will seek to improve patient quality of life with simpler, longer-lasting treatments. We can expect these newer drugs to be more expensive.¹
At the non-obvious level, as patients’ lives become more normal, they increasingly have opportunities to develop problems that previous generations of hemophiliacs did not. For example, until recently little was known about low-bone density problems (osteopenia) among hemophilia patients. No one had the time to study it. Other problems are similarly gaining new-found attention, like obesity and aging issues.

Take obesity. Now that hemophilia patients are living long enough to become obese, about 50 percent of them have done so. This is important, because factor concentrate dosing (and cost) is based on body weight. However, while all heavier patients receive higher doses of factor, it is not clear that obese patients actually need it, because fatty tissue has less blood supply than lean tissue.

More research is needed in order to fully understand the implications of these changing demographics. However, it seems clear that, as a result of these and other challenges, the cost of care for hemophilia will continue to rise.

Driver 2: Inhibitors

Some patients develop a resistance to clotting factor infusions. Essentially, the body develops an allergic reaction to the clotting factors. The immune system begins to consider the factor as a foreign substance and tries to destroy it using inhibitor antibodies.

About one-third (30 percent) of patients with severe Type A and up to 5 percent of those with Type B develop a resistance after receiving clotting factor infusions over a period of time. About 1,200 Americans with hemophilia have inhibitors.

When people develop inhibitors it becomes extremely difficult to control their bleeding episodes. These patients must receive additional therapy to help them overcome that resistance. These are some of the most expensive cases to treat.
One treatment for inhibitors involves infusing the patient with special "bypass agents." Bypass agents avoid the immune system response by using a different chemical pathway to support clotting that "bypass" the mechanism controlled by the clotting factor concentrate.

Bypass agents can be effective to control bleeding, but they can also be difficult to use and have side-effects of their own. Other treatments require patients to receive very large amounts of clotting factor concentrates every day for many weeks or months in an effort to "teach" the body to accept clotting factor concentrate treatments.

All of these treatment options – plus additional ER visits and hospitalizations due to increased bleeding – can push overall treatment costs up very high for inhibitor patients. The cost for bypass agents alone averages around $300,000 per year. CDC researchers found that the average yearly health care bill for patients with inhibitors was nearly five times higher than for patients without inhibitors (see above graphic).

**Driver 3: New drugs**

The market for hemophilia drugs is seeing an influx of new drugs. Some, with slightly longer periods needed between treatments, have already arrived. Other, even longer-lasting clotting factors are also on their way.

Two new Biogen products (Aprolix® for type B & Eloctate® for type A) are longer-acting than traditional treatments. They require only one to two infusions per week, as opposed to the commonly used regimen of three infusions per week. Even this relatively small change in frequency translates into 50 to 100 or fewer infusions per year and a correspondingly smaller number of units infused per patient.

When the new concentrates were announced there was speculation that the combination of longer treatment intervals and similar overall cost would mean that existing patients would quickly move toward the new Biogen products. This has not occurred.

One big reason is that the new concentrates may not actually represent a significant clinical advantage; rather, they may simply be more convenient to use. Based on a clinical review of available evidence, UnitedHealthcare has determined that Eloctate may not be medically necessary in all cases. While it is covered under our fully-insured contracts, patients must first demonstrate that the existing alternatives are not sufficient. (ASO and carve-out clients make their own coverage determinations. Contact your consultant, broker or representative for details.)
Another reason for the slow uptake appears to be the highly individual nature of hemophilia and its treatment. Patients who respond well to one treatment may not fare well with another. Consequently, hemophilia patients who are stable in their treatments are reluctant to change treatments and their doctors are equally reluctant to recommend a change.\textsuperscript{11} One indication that this is happening is that, during the last quarter of 2014, when Eloctate was available on the market, its sales hardly moved, while an older product from Bayer (Advate\textsuperscript{®}) actually saw increased sales (+ seven percent).\textsuperscript{11}

**Increased competition?**

One thing we know for sure is that there will be no shortage of new products coming on the market. The Canadian Hemophilia Society (CHS) recently counted no fewer than 29 new therapies in development, including 11 for type A, eight for type B, eight new bypassing therapies, plus two others.\textsuperscript{12}

There are two main categories for new products:

- Greater clotting potency
- Extended factor half-life

Greater clotting potency: This can be especially important for patients with inhibitors.\textsuperscript{15} Unlike drugs that simply add convenience, a clotting factor with dramatically improved clotting potency would probably be covered by insurance. That’s a necessary improvement — maybe even at a premium price.

Extended factor half-life: These improvements seek to increase the time the clotting factors remain effective in the patient’s bloodstream after being infused. Current treatments can fall by half in the body in a matter of hours after infusion. New versions hope to extend the time between infusions from every two or three days to once every 10 days or two weeks, or even longer.\textsuperscript{12} (See chart, below.)

But we’ve already seen how just extending the time between infusions from two or three days to four or five is seen as mainly more convenient; and so may not be covered. But what happens if the time between infusions gets extended to two weeks? Presumably there is some threshold where a long enough interval attains a clinical or cost significance and begins to warrant coverage. It is at this point that higher drug prices will begin to have their impact.

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**Longer-Lasting Clotting Factors On the Way?**

Those with type B have far better prospects

Clotting factor effectiveness is limited by its half-life — i.e., how long half of it remains in the body after infusion. New versions of factor are on the way, however they promise to be much more effective for type B.

<table>
<thead>
<tr>
<th>Current Factor Half-Life</th>
<th>New Factor Half-Life*</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Type A:</strong></td>
<td><strong>Type A:</strong></td>
</tr>
<tr>
<td>Up to 12 Hours</td>
<td>Up to 20 Hours</td>
</tr>
<tr>
<td><strong>Type B:</strong></td>
<td><strong>Type B:</strong></td>
</tr>
<tr>
<td>Up to 24 Hours</td>
<td>Up to 110 Hours</td>
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</tbody>
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Care support

While there is no cure for hemophilia, many people with the disease can lead fairly normal lives by taking a few common sense precautions. For example:

- While regular exercise can help to build up muscle and protect joints, contact sports — such as football or hockey are not safe for people with hemophilia.¹³
- Hemophiliacs should avoid certain pain medications that can aggravate bleeding, including aspirin and ibuprofen and also blood-thinning medications such as heparin, Coumadin®, Plavix® and Effient®.¹³

Hemophilia is a complex disease, so many patients require extra support in order to manage their symptoms. Many patients and their families rely on specialized hemophilia treatment centers (HTC) to help. HTCs are specialized health care centers that bring together teams of doctors, nurses, and other health professionals who are experienced in treating hemophilia.¹⁴

The OptumRx Specialty Pharmacy Program devoted to hemophilia makes a special point to connect patients with the nearest local HTC. In addition to ongoing consultations with one of our Specialty Pharmacists, the HTC teams help monitor each patient’s treatment to avoid complications.

What to do

Conditions like hemophilia offer one of the best illustrations of the principal that all health care is tightly connected. Pharmacy is the most often used benefit by consumers and has the greatest impact on all the various areas of spending.

For example, staying adherent to factor replacement therapy is critical for managing overall hemophilia spending, yet overall only about 40 percent of patients do so. A PBM that attempts to manage just the cost of the clotting factor is missing the bigger picture of managing the overall condition and spend.

OptumRx does this with penetrating insights into all dimensions of spending – not just pharmacy. Find out more about our approach and about our comprehensive, patient-centered approach that we know to be effective and successful in helping members manage this condition in a cost effective way with all the resources they need.

OptumRx Hemophilia Insight Report

An in-depth look at the treatment, cost and key strategies of this rare disease. Download the full insight report.
References


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