NATIONAL HEMOPHILIA FOUNDATION
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The Need For Qualified, Experienced Specialty Pharmacy Providers and Competition

"Competition is not only the basis of protection to the consumer, but is the incentive to progress."
Herbert Hoover

NHF has long advocated for the hemophilia community to have access to more than one qualified specialty pharmacy provider experienced in handling individuals with hemophilia or related bleeding disorders and preferably more than one delivery method for providing these services, specialty pharmacy and 340B. As explained more fully below, having access to more than one provider and delivery method (where available) ensures access, quality services, and patient safety. Thus, NHF will continue to advocate to states to adopt policies that ensure access in such a manner, and to adopt certain quality standards to ensure qualified, experienced providers in the market.

Background on Specialty Pharmacies

Individuals with hemophilia or other inherited bleeding disorders rely on FDA-approved blood clotting factor therapies, either recombinant or plasma-derived, as life-sustaining treatments for the entirety of their lives. These products fall in a class commonly known as "specialty drugs," which because of their properties, require specialized storage and handling. Given these special handling and storage requirements, someone with hemophilia cannot go to a traditional neighborhood pharmacy to obtain their life-saving medications. Instead, they receive their medication from what is known as a specialty pharmacy.

Specialty pharmacies include separate pharmacy divisions owned by your traditional, national retail chains; independent specialty pharmacies; pharmacies at hemophilia treatment centers (HTCs) who have elected to participate in the 340B federal discount drug program (340B pharmacies); and, more recently, specialty pharmacies owned by insurers. Because most individuals self-infuse their medications at home, specialty drugs are typically delivered to patients via mail or other home-delivery options. Unlike your traditional pharmacy, specialty pharmacies provide a range of support services targeted at patient education, care coordination, assay management and disease management.

Because hemophilia requires effective prophylactic treatment, adherence and optimal service from qualified providers knowledgeable about hemophilia, it is important the specialty pharmacy providers serving the hemophilia and related bleeding disorders community meet certain standards. Accordingly, NHF's Medical and Scientific Advisory Council (MASAC)[1] developed MASAC 188: *Recommendation Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders.*[2]

MASAC 188 sets minimum standards that all specialty pharmacies should adhere to in order to ensure: (1) patient safety; (2) avoid unnecessary, adverse health incidents; (3) good health outcomes; and (4) more predictable costs.

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[1] MASAC is comprised of scientists, hematologists other treatment professionals, US government agencies and patient representatives that are internationally regarded as experts in the broad field of bleeding disorders research and care, AIDS, hepatitis, other infectious diseases and blood safety. MASAC recommendations set the standard of care around the world; are referred to by an international array of physicians, medical schools, pharmacists, emergency room personnel, and insurance companies; and address a wide array of treatment issues.

[2] Adopted by MASAC on November 15, 2008. MASAC #188 can be located at: http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=57&contentid=1107

According to MASAC 188, qualified specialty pharmacies serving the hemophilia community will meet minimum standards in six (6) key ways summarized below:

- Be Knowledgeable – Must have staff knowledgeable of clotting factor concentrates, necessary ancillary supplies, and the proper handling of both
- Be Comprehensive – Must be able to provide the full range of available, FDA-approved clotting factor concentrates, ancillaries and supportive services (either directly or through a third party)
- Be Timely & Accurate - Must be able to timely process prescription orders (48-hours or less) and fill prescriptions within a certain, acceptable assay range
- Be Accessible – Must be available to patients within normal business hours, have 24-hour emergency services, have multi-lingual customer service representatives, and maintain necessary contact with the treating physicians
- Be Safe - Must have appropriate, safe, federally-compliant delivery services to ensure timely delivery, a faster process in emergent need cases (less than 12 hours; goal of 3 hours), and a back-up plan for natural disasters
- Be Thorough – Must maintain accurate, up-to-date records meeting all federal and state requirements, be HIPAA compliant; provide patients with accurate information about factor costs per unit and their out-of-pocket responsibilities under their insurance; maintain an accurate process to trace all shipments; and participate in the National Patient Notification System for clotting factor concentrate recalls to ensure patient safety

Sole Source and the Need for Competition

In the recent past, we have seen sole source situations occur in Medicaid programs; however, the trend has started navigating to the private insurance market at a rapid pace, particularly with regard to specialty pharmacy services providers. In fact, a more common variation on this theme is for insurance companies to acquire specialty pharmacies and mandate that all consumers in their plans utilize that particular specialty pharmacy.

While intended as a cost-containment measure, failure to maintain a "competitive market" of qualified specialty pharmacy providers experienced in handling individuals with hemophilia or related bleeding disorders can cause significant risk to patient safety and result in increased costs.

First, there are safety concerns. In some instances, insurers or PBMs have contracted with specialty pharmacy providers who have little to no experience in managing prescribed therapies for individuals with hemophilia or related bleeding disorders. Timely assistance with therapy needs in compliance with prescribed therapy regimens is critical to achieving positive, cost effective, patient outcomes. Specialized knowledge of the unique needs of the hemophilia community, along with timely consultation with the HTC's multi-disciplinary team, is necessary in order to prevent delays/barriers in access to care. Lack of timely or compliant therapy access, in coordination with disease management teams at HTCs, can lead to detrimental, adverse health risks to patients, including increased bleeding episodes, frequent hospitalizations, increased joint disease and in worst case scenarios, premature death. These adverse health risks also lead to higher costs for insurers.

Second, when a contract is sole sourced, the pharmacy services provider may not provide access to the full range of clotting factor products. For example, a sole source provider may attempt to contain costs by limiting the number of products accessible to consumers. While in other patient populations, this may not have detrimental effects, because of the biologic nature of clotting factor therapies and an individual patient's metabolic rate and other reactions to the medications, not every clotting factor product works effectively for every patient.[3] Thus, if patients are forced to use a particular product that is not as effective for them, they could require more medication to prevent or control bleeding

[3] See MASAC #159: *Regarding Factor Concentrate Prescriptions and Formulary Development and Restrictions*; http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=57&contentid=179

episodes, thereby driving up costs; require more frequent hospitalizations for uncontrolled bleeds; or could experience other resulting effects of uncontrolled bleeds such as joint, tissue, or organ damage.

Third, without more than one option, patients in rural areas are potentially denied reasonable access to specialty pharmacy services in already under-served rural and inner-city areas. If a single provider without the ability to effectively manage patients in such populations is the only option, patients will not have access to the ancillary specialty pharmacy services, such as a disease management, care coordination, and patient education services that they require to effectively manage their disease. When insurers sole source and exclude the providers traditionally available and able to assist the communities in the area, this causes unnecessary risk to the patients.

Finally, sole source contracts are typically entered into with specialty pharmacies under the traditional delivery method; however, this does not factor in the 340B delivery method of specialty drugs. In addition to providing the ancillary services that other specialty pharmacy providers provide for patients, 340B pharmacies at HTCs can reinvest the income from their program into services that would not otherwise be reimbursed for patients. This provides for extra, quality care for patients and gives them the convenient option of obtaining their pharmacy and medical need from the same provider. Published data exists documenting the importance of federally recognized hemophilia treatment centers (HTCs) in decreasing the morbidity and mortality of hemophilia patients.[4] The participation of these federally recognized comprehensive teams is a critical component to effective disease management of this population. Disease management with the goal of cost containment alone will prove to negatively impact outcomes and can lead to higher costs for insurers.

Conclusion

In conclusion, denying access to qualified specialty pharmacy providers experienced in managing patients with hemophilia and ensuring quality through a competitive, specialty pharmacy market, payers potentially place patients at risk of adverse health incidences and may possibly increase overall costs in managing the care of individuals with hemophilia.

Accordingly, NHF will continue to advocate that states should adopt legislation aimed to require insurers (both public and private) to:

- Offer covered individuals more than one, but preferably a range, of specialty pharmacy providers who are knowledgeable of and experienced in managing the care for individuals with hemophilia, including an HTC with a 340B program if operating in the state/local area
- Either incorporate MASAC 188 guidelines into their pharmacy provider contracts or pass standards of care/service legislation requiring plans to ensure that all specialty pharmacy providers meet certain minimum standards

[4] See Soucie JM, et al. Blood 2000; 96(2):437-442.