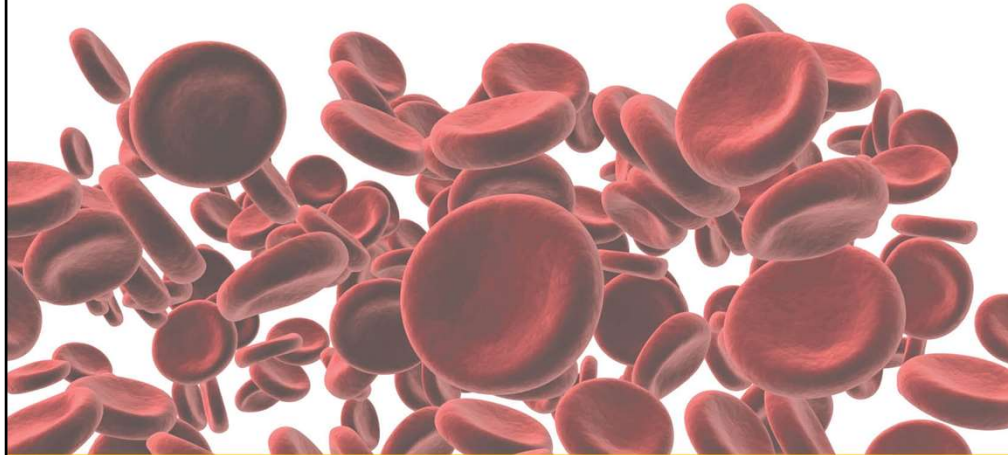


Challenges and Opportunities for Managing Hemophilia



Jointly sponsored/
coprovided by



with the



Supported by an
educational grant from
Novo Nordisk, Inc.



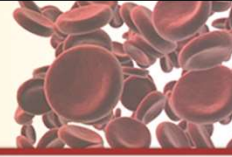
Welcome to the continuing education activity entitled “Challenges and Opportunities for Managing Hemophilia”. We are pleased to provide you with what we hope will be an informative and meaningful program.

We would like to acknowledge that this activity is supported by an educational grant from Novo Nordisk and we would like to thank them for their support.



This presentation, entitled “Opportunities with Specialty Pharmacy and Home Infusion,” will provide information from the Specialty Pharmacy and Home Infusion Services Perspective.

Objectives

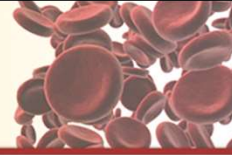


- Define the role of specialty pharmacy (SP) and home infusion (HI) in the management of hemophilia patients
- Describe how SP/HI is best integrated with the Hemophilia Treatment Center (HTC) and payers
- Review the role of the SP/HI in providing patient/caregiver support
- Explain the role of the SP/HI in achieving optimal clinical and economic outcomes
- Identify the challenges and opportunities of the SP/HI in achieving full alignment with the other members of the hemophilia care team

3

The objectives of this presentation are to define the role of specialty pharmacy and home infusion services in the management of hemophilia patients; to describe how specialty pharmacy/home infusion services are best integrated with the Hemophilia Treatment Center, or HTC, and payers; to review the role of specialty pharmacy/home infusion services in providing patient and caregiver support; to explain the role of specialty pharmacy/home infusion services in achieving optimal clinical and economic outcomes; and to identify the challenges and opportunities of specialty pharmacy/home infusion services in achieving full alignment with the other members of the hemophilia care team.

Role of the SP/HI in the Treatment of Hemophilia



- Communication/feedback/collaboration with HTC's
- Support patient adherence to hemophilia therapy
- Teach/reinforce self-care by patients/family
 - *Identify education or language barriers*
- Educate on adjunctive therapies (e.g., RICE, protective gear, etc.)
- Collaborate with HTC's and payers to manage dosing and limit unwarranted use
 - *Waste reduction and inventory management*
- Facilitate pain management plans of care
- Assist patients with understanding insurance/cost-sharing issues and responsibilities

4

Specialty pharmacy/home infusion services can be utilized as an extension of the HTC or prescriber for the 364 days a year that a patient is not seen at the HTC. Ideally, specialty pharmacy/home infusion services can act as a prescriber's eyes and ears in the field and provide valuable information on how the patient is responding to his regimen. Even more ideally, specialty pharmacy/home infusion services can prompt the patient to provide appropriate communication themselves. With the HTC's direction, specialty pharmacy/home infusion services can assist with some of the educational elements that were mentioned from the Nurse Coordinator perspective such as the education of schools, worksites, and coaches.

Treatment adherence, or truly getting the family and patient to own their care and fully participate in optimal management is critical, and education is the way to get there. Adherence can be reinforced on every interaction that specialty pharmacy/home infusion services have with their population, which may be more frequently than they are able to get to an HTC based on geography and other variables.

Reinforcing the importance of treatment adherence begins with continuing the teaching that has been initiated by the HTC, understanding what has been taught, and carrying that training forward. It includes identifying both language and educational barriers to teaching, working within those limits when necessary, and using appropriate teaching tools such as arms, chests, or cuffs to support that. Specialty pharmacy/home infusion services usually have bilingual support available 24/7 for the Hispanic population and offer access to language lines for translation.

Specialty pharmacy/home infusion services can help patients to access adjunctive therapies

in support of devices such as CryoCuffs, ice, protective gear for elbows and knees and heads for toddling children. Specialty pharmacy/home infusion services can educate on their appropriate use, avoiding myths and possible complications from following through on information that somebody else from the community may have provided. It was about a year ago one specialty pharmacy got several calls about packing the nose with salt pork. There was an article going around on the Internet about that treatment method reducing nasal bleeding, and the specialty pharmacy worked with the HTC to determine the health professionals' opinion on that treatment method and how appropriate it might be. Sometimes specialty pharmacies are the first to get those calls.

Specialty pharmacy/home infusion services collaborate with HTCs and payers with dosing. There is a real opportunity for specialty pharmacy/home infusion services to manage dosing and limit unwarranted use of clotting factor concentrate. Specialty pharmacy/home infusion services confirm dosing orders and ensure that the patient's request for clotting factor product matches the physician orders and the treatment plan.

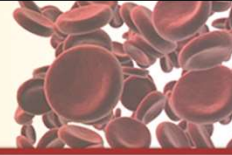
Because hemophilia patients, like all people, are not 100% accurate historians, specialty pharmacy/home infusion services often must find out why a disconnect exists between patients' orders for clotting factor product and physician orders or treatment plans. Was there a bleed that the patient did not report? That could have an impact on their treatment plan, depending on what caused the bleed or whether it was spontaneous. Did they get their vial sizes mixed up? Was there wasted or misplaced product? Was product taken to an alternate residence and left there by accident? Each of these is an educable moment and specialty pharmacy/home infusion services can use these events to facilitate the patient and family buying into their part of responsible product management.

From a pain management perspective, there are many opportunities for specialty pharmacy/home infusion services, HTCs, and patients/families to partner. Specialty pharmacy/home infusion services can work with the patient and prescriber to evaluate how the patient is responding to alternate pain management modalities; investigate pharmacologic rationale for lack of pain control; and work within the boundaries of pain management contracts with patients. Doing home visits can uncover issues of adherence or possible impairment when such issues are suspected by a prescriber.

Also, when it comes to pain, sometimes there is a fine line between chronic pain and pain from an acute bleed. A home visit from a skilled bleeding disorder infusion nurse can help navigate that delicate issue. Certainly pain medication is less expensive than clotting factor concentrate, so identifying a patient who is in a cycle of continuing to treat when bleeding may not be the root cause of pain is another means by which specialty pharmacy/home infusion services can serve as an extension to the HTC.

Finally specialty pharmacy/home infusion services are responsible for investigating and helping patients understand how their product is reimbursed and what their financial responsibilities may be in their therapy. When cost sharing presents a true burden to the patient, specialty pharmacy/home infusion services can work with patients to identify potential sources for assistance, of course working with payer requirements and escalating any issues appropriately.

Responsibilities of the Hemophilia Specialty Pharmacist



- Responsibilities of the hemophilia specialty pharmacist include
 - Ensuring the drug received by the patient is appropriate for their diagnosis
 - Validates appropriate dosing, particularly if weight-based
 - Identify comorbidities and patient history
 - Monitors bleed episodes, pain levels and other potential complications and communicates information back to HTC or prescribing physician
 - Identifies potential drug interactions
 - Encourages compliance to treatment plan
 - Provides medication oversight including
 - Storage and reconstitution of factor products
 - Stock rotation and expiration date monitoring

5

The hemophilia specialty pharmacist represents an infusion pharmacy. Pharmacy is their specialty. As such, they do have an obligation to validate orders. They calculate weight-based dosing. If the patient is out of range, they verify there is documentation to support it. Does a patient have an inhibitor? What is their inhibitor level? Is he a rapid metabolizer? Are there pharmacokinetic studies to support a dose higher than a typical range? Is the patient especially active or involved in sports that would indicate a need to maintain a higher factor level? These are issues that payer partners want specialty pharmacy/home infusion services to address when the need for clotting factor is other than expected.

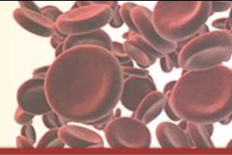
Specialty pharmacy/home infusion services look at the full medication profile and history and help the patient through comorbidity management therapy, ensuring that labs and hepatitis C or HIV regimens are completed, if that is part of the patient's care requirements. Specialty pharmacy/home infusion services look for drug interactions and educate on side effects and over the counter medications to avoid. They inquire about all of these issues prior to each dispensation.

Although at times it is a challenge to get patients to communicate with detail, specialty pharmacy/home infusion services strive to get the full picture, to optimize patients' treatment regimens, and report any key findings to HTCs so that the plan can be updated when needed. Again, adherence to the treatment plan is always the primary goal and education is the best path.

Finally, specialty pharmacy/home infusion services make sure patients and families know the nuts and bolts about their treatment. As mentioned in the Nurse Coordinator perspective, education starts at the HTC, but when there is a change in an infusion schedule or a reconstitution device, when the patient hears about the latest smartphone app to log treatment or bleeding episodes, or changes in room temperature stability of their drugs, or questions like, “What do we do if somebody puts this in the freezer by mistake? Can we still use it? What happens in a power outage?” It is the infusion pharmacy that frequently hears these questions and provides this education. Specialty pharmacy/home infusion services also reinforce how important it is to rotate stock and other important clotting factor management issues.

To ensure the patient uses the correct dose, specialty pharmacy/home infusion services do make sure that doses are bagged together. If more than one vial is necessary to make a specific dose, bagging doses together can minimize the possibility of error and waste. Patients are asked to keep lot numbers and expiration dates logged. Many specialty pharmacy/home infusion services suggest they just tear off the label and stick it to their calendar. This not only keeps expiration dates and stock rotation in mind, but they can be in-tune if there are any issues of product recall.

Responsibilities of the Hemophilia Specialty Pharmacy



- Responsibilities of the hemophilia specialty pharmacy include
 - Timely delivery of factor
 - Home inventory management
 - Coordination of supplies
 - Provide 24/7 access to clinical support
 - Facilitate out-of-network situations
 - Complement the role of HTC's
 - Reimbursement consultation services
 - Nursing services when required: infusion and teaching self-care
 - Maintain current knowledge of therapies and new products

6

How do specialty pharmacy/home infusion services accomplish these responsibilities? First and foremost is timely delivery of clotting factor product, not letting the patient run out, having doses on hand in the event of an unexpected bleed. These provisions are crucial in avoiding unnecessary emergency department visits.

Specialty pharmacy/home infusion services also make sure, for those 20-year-olds whose strong suit is not planning, to nudge patients to make sure they are staying on top of things. Specialty pharmacy/home infusion services also keep emergency doses in the closest infusion office for those last-minute calls when planning has fallen through.

Specialty pharmacy/home infusion services stock a variety of supplies which is important in this population. If an 8-year-old learned to self-infuse at camp with the opaque blue big-ringed infusion device and specialty pharmacy/home infusion services send a translucent one with shorter wings and a SpongeBob Band-Aid instead of Spiderman, the patient can be set back to square one with their self-infusion progress. Specialty pharmacy/home infusion services must send just what is needed, and precisely what is needed.

Like HTC partners, specialty pharmacy/home infusion services are available 24/7 for support with nurses, pharmacists, and delivery staff. Specialty pharmacy/home infusion services communicate issues with HTC's and makes sure they are contacted, either by the patient or by specialty pharmacy/home infusion services when appropriate. Importantly,

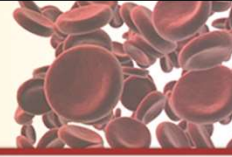
specialty pharmacy/home infusion services do not assume the role of the HTC; they complement it.

If there are payer changes or out-of-network issues in an emergency, specialty pharmacy/home infusion services work to obtain temporary overrides. They provide continuity and coordination of care support for transitions to in-network providers when necessary. A critical consideration in reimbursement consultation services is to do the right thing for both the patient and the payer. There is a reason the payers have networks, and that is the most cost effective thing for both the patient and the payer in those circumstances.

Specialty pharmacy/home infusion nursing services are critical, not just in the pediatric population, but in that mild, moderate, and von Willebrand disease population. Having expert infusion nurses with specific training in bleeding disorders is essential for education and for event-based infusions such as for surgical and dental procedures or injuries.

The hemophilia population is one of the most educated patient populations in the U.S. so it is essential that specialty pharmacy/home infusion services nurses and pharmacists know about changes in product stability, devices, new product pipeline, etc., as these clinicians are the people who get asked the questions.

Interactions of the Specialty Pharmacy with Others Involved in Hemophilia Care

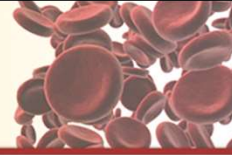


- **Patients**
 - *Support families in living a normal lifestyle*
 - *Provide patient education resources*
 - *Reimbursement consultation services*
- **HTCs**
 - *Support clinical goals of comprehensive care center*
 - *Supply additional perspective on home dynamics*
 - *Provide information and reports to support care management*
- **Payers**
 - *Support payers' objectives to control costs and achieve positive outcomes*
 - *Coordinate with care management*
 - *Provide reports to track utilization*
 - *Assay management performance*

7

Specialty pharmacy/home infusion services can serve as a hub in the network between patients, HTCs, and payers. Similar to the HTCs, many of the services that specialty pharmacy/home infusion services provide are not directly reimbursed; specialty pharmacy/home infusion services are reimbursed for product, and many of the other services are bundled into that cost. Working together, specialty pharmacy/home infusion services, patients, HTCs, and payers can reduce duplication and improve overall effectiveness. From the specialty pharmacy/home infusion services standpoint, partnering with HTCs and payers is a fundamental strategy to doing that successfully.

Specialty Pharmacy and Managed Care



- Home setting is most appropriate site of care for prompt treatment
- SP/HI pharmacy with expertise in bleeding disorder therapies is essential
- SP/HI pharmacy should meet minimum standards of care as established by the industry (e.g., MASAC guidelines)
- Cost savings can be measured
 - Long-term: decreased future costs tied to disease progression
 - Near-term: minimized bleeding episodes or fewer visits to the ER
- Assay management of factor products
 - Assay management is the process of filling the prescription as closely to the prescribed amount with the available assays
 - Goal is to dispense as close to the prescribed target dosing as possible, barring extenuating circumstances
 - Common practice generally permits dosing within $\pm 10\%$ variance of prescribed target dose

8

As described by the Hematologist and the HTC Nurse Coordinator, the home setting is the most appropriate site of care for prompt treatment. It is well-established that people who learn how to treat their bleeds at home are less likely to be admitted to the hospital for bleeding complications, and specialty pharmacy/home infusion services assert that the home infusion nurses have a special role in that facilitating home treatment.

Expertise in therapies is essential. There are guidelines established by the Medical and Scientific Advisory Council, or MASAC, of the National Hemophilia Foundation for specialty pharmacy/home infusion services providers that emphasize responsiveness, leaving the responsibility for product choice with the prescriber, having the availability of all products and all assay sizes and managing assay tightly.

Payers want measurable cost savings. From the Hematologist Perspective, we saw the long-term costs and savings associated with prophylaxis treatment, but it is challenging to capture these cost dynamics in the traditional managed care quarterly reports. Appropriate treatment now generates long-term savings. As managed Medicaid and Accountable Care models in the commercial payer space become more prevalent, this population is going to be evaluated more from a longer-term perspective, and cost savings may be more easily demonstrated and appreciated by payers.

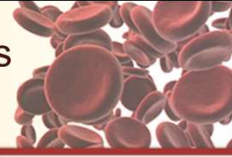
The data that is more easily measured and quantified in those traditional quarterly reports

to payers includes decreasing bleeding episodes and emergency department visits and working with the treatment protocol to improve plans of care to reduce costly events.

For those who work in specialty pharmacy/home infusion services, assay management is an important concern. Assay management involves dispensing product in vial sizes that come as close as possible to the prescribed international units per kilogram, within plus or minus 10 percent variance of prescribed dosage. Assay management is really only a beginning. More importantly, specialty pharmacy/home infusion services have a responsibility to manage not just the assay but the dose. What is the desired order in international units per kilogram? If a dose is sent from the prescriber that simply says "2000 units", what is the real target? Specialty pharmacy/home infusion services can challenge the standard practice of dose +/- 10%. Today there are so many assay sizes of products available, that a 5 to 10% variance should be an exception.

Of course there are exceptions, especially with very small children. Working together, specialty pharmacy/home infusion services and HTC's can treat the patient, not the number, and collaboration and open communication are essential to that end.

Best Practices for Communication Across the Continuum of Hemophilia Care



- Reinforce delivery of the care plan established by the HTC
- Align with requirements of payer (performance standards, reports, authorizations, audits)
- Direct patient questions regarding product choice, therapy regimen, and other clinical decisions tied to the care team at the HTC
- Maintain the highest standards regarding legal statutes, HIPAA compliance, and OIG guidelines
- Provide relevant education resources to patients
- SP/HI reimbursement specialist to act as liaison to communicate patient financial responsibilities to members
- Establish and pursue mutually agreed upon goals

9

We have covered well the collaboration between specialty pharmacy/home infusion services and the HTC, the reinforcing role that specialty pharmacy/home infusion services can play in delivery of care. When it comes to payers, specialty pharmacy/home infusion services do not simply align with payer standards, but work together with payers to hone those standards and further develop them to improve both financial and population outcomes. Specialty pharmacy/home infusion services need to provide reports that paint a picture of that population to help payers better understand their unique needs.

Authorizations and audits should not be emergent or cause for alarm. Planning and documentation are critical in this area.

From an educational perspective, certainly specialty pharmacy/home infusion services should take advantage of opportunities for updates with payers to provide education. But when it comes to patient education, it is not just written materials, but tools as well, such as Arthur Arm and Chester Chest, and other kinds of characters and educational tools to help patients practice self-infusion safely and in the way their HTC taught them.

A presentation regarding the specialty pharmacy/home infusion services community would not be complete without talking about ethics and integrity. Specialty pharmacy/home infusion services must truly be ethical in all that is done, maintaining strict adherence to all legal guidelines, especially Office of the Inspector General guidelines. This has been an area

of particular concern in this industry.

In summary, specialty pharmacy/home infusion services, HTCs, and payers can learn a lot from each other. What is learned can help create positive outcomes for this patient community and responsible cost management, which is imperative. With the goals of being a better partner, improving outcomes, and helping patients find a way to long term wellness, specialty pharmacy/home infusion services, HTCs, and payers can find common ground. Everyone can win.