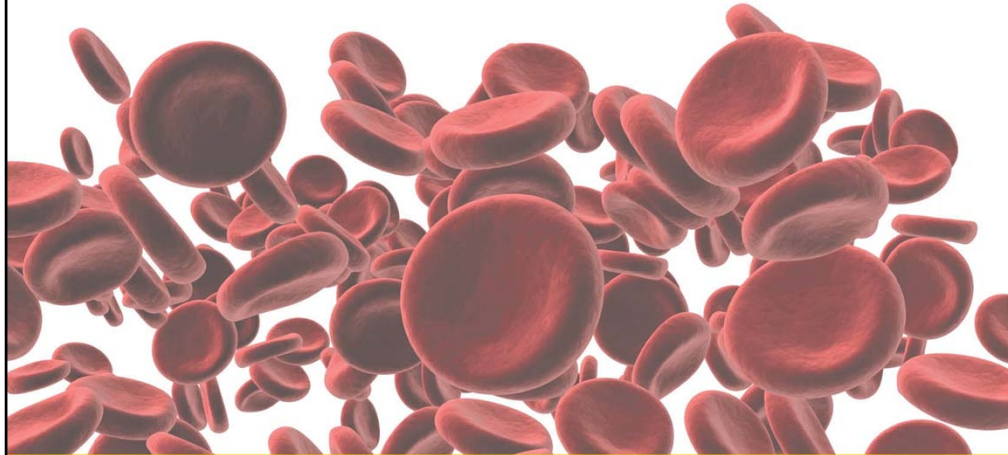


Challenges and Opportunities for Managing Hemophilia



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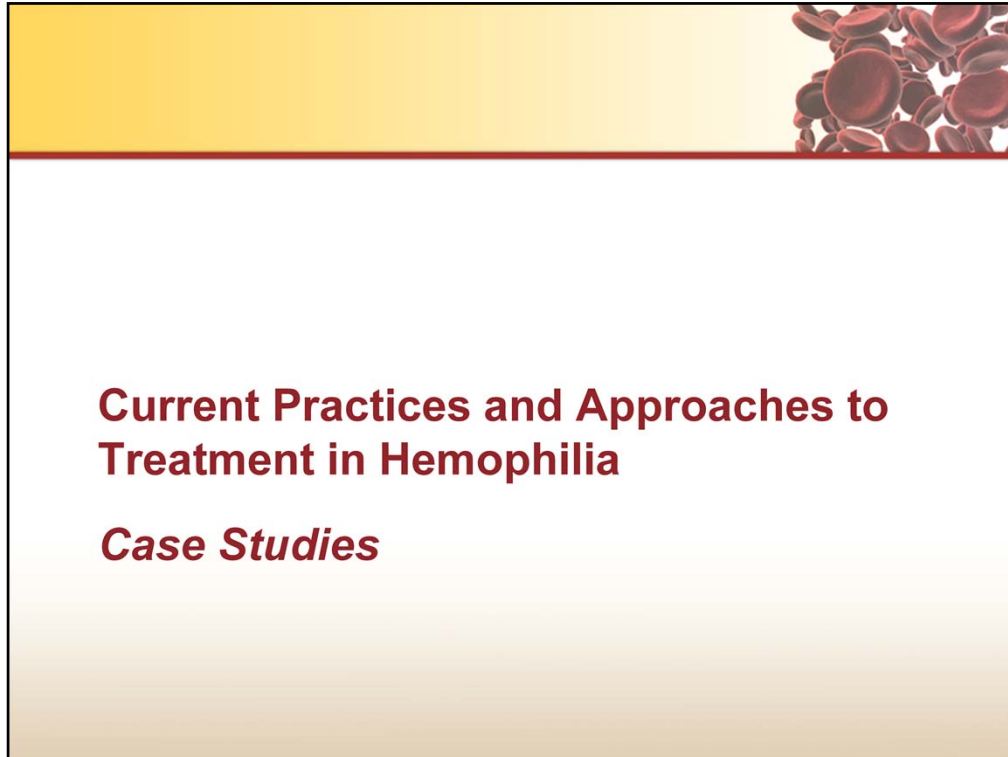


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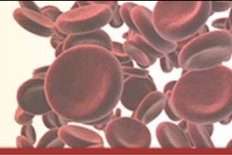
Welcome to the continuing education activity entitled “Challenges and Opportunities for Managing Hemophilia”. We are pleased to provide you with what we hope will be an informative and meaningful program.

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 section of the program, entitled “Current Practices and Approaches to Treatment in Hemophilia: Case Studies,” will provide case studies followed by a faculty panel discussion to get their perspectives on the cases.

Case 1: Patient with Multiple Comorbidities



- **Clinical circumstances**

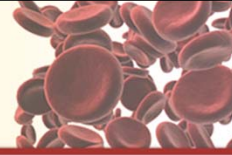
- *40-year-old male with severe factor VIII deficient hemophilia*
- *Intravenous self-infusion of factor VIII concentrate to prevent or treat bleeding*
- *Presence of comorbid conditions including:*
 - *HIV*
 - *Hepatitis C*
- *Patient received all medications necessary to treat hemophilia and comorbidities from the same specialty pharmacy for the past 5 years*
- *In January 2013, he changed to a new insurance plan that required the use of different specialty pharmacies for each of his chronic conditions, resulting in fragmented and poorly coordinated care*

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The first case is a patient who has multiple comorbidities. A 40-year-old gentleman has severe Factor 8 deficiency, hemophilia A. He does intravenous self-infusion of factor 8 concentrate in order to prevent or treat bleeding. He is also old enough to have comorbid conditions that include both HIV and hepatitis C.

The patient has received all of his medications necessary to treat his hemophilia as well as his comorbidities from the same specialty pharmacy for the past five years. But in January 2013 he changed to a new insurance plan that required the use of different specialty pharmacies for each of his chronic conditions, resulting in fragmented and poorly coordinated care.

Case 1: Critical Issues



- **Critical issues**

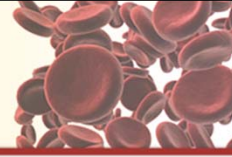
- *A patient with hemophilia with multiple serious comorbidities is not uncommon*
- *Patients with multiple comorbidities require regular monitoring and consistent care*
- *Providers and payers must address the hemophilia as well as the patient's other health issues in an efficient and cost-effective manner*
- *Appropriate treatment for hemophilia and comorbid conditions is critical for best outcomes*
- *Ease of access and timely delivery of prescribed medications enhances compliance and contributes to the maintenance of disease control*

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These are some of the critical issues related to this case. This is a patient with hemophilia with multiple serious comorbidities, and this is not an uncommon scenario in adult Hemophilia Treatment Centers, or HTC's. Patients with multiple comorbidities require regular monitoring and consistent care. Providers and payers have to address the hemophilia as well as the patient's other health issues in both an efficient and cost effective manner. They are all trying to work towards an appropriate treatment for the hemophilia and comorbid conditions in order to obtain the best outcome for this patient.

In addition, ease and access, and timely delivery of the prescribed medications are required to enhance adherence and to contribute to the maintenance of disease control.

Hematologist Perspective, Case 1

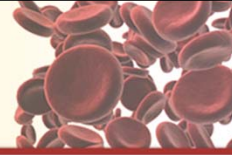


- Adherence with treatment requirements for such complex conditions is critical for overall outcomes
- This would be discussed with the patient at each and every visit
- Discontinuity in care impairing the patient's ability to adhere to treatment recommendations poses a challenge for the physician

From the physician perspective, adherence with treatment requirements for such complex conditions is critical for the overall outcome. This is of utmost importance. This is something that we would be discussing with the patient at each and every visit. If we felt that there was discontinuity in care related to this issue, if this was impairing the patient's ability to keep up with treatment, then this really poses a challenge for the physician.

I'd like to hear from the other faculty about not only how they see this being an issue, but what solutions have they seen for this exact scenario.

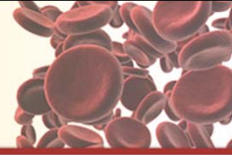
HTC Nurse Perspective, Case 1



- Outcomes in this scenario are dependent on patient and family and how this change affects their ability to manage the conditions
- It is the role of the HTC team, especially the social worker and the nurse, to negotiate with the payer and advocate for the patient to streamline the patient's care for the best outcomes
- This requires an individual approach with the case manager

As the hemophilia nurse coordinator, the outcomes in this sort of scenario really depend on the patient and the family and how this change affects their ability to manage the conditions. If the change does affect the patient's ability to manage the conditions, then it is the role of the HTC team, especially the social worker and the nurse, to negotiate with the payer and to advocate for the patient to streamline the patient's care for the best outcomes. We would take an individual approach with the case manager.

Specialty Pharmacy/Home Infusion Services Perspective, Case 1



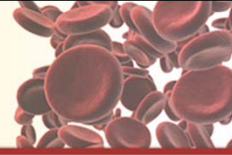
- SP/Hi uniquely situated to coordinate a case such as this
- Planning is key
 - *Can be sensitive*
 - *Some patients do not want to receive hepatitis C/HIV medications from community retail pharmacies because pharmacy staff know them from other circumstances*
- SP/Hi may be able to work with insurer to override multiple pharmacies requirement
- SP/Hi may coordinate amongst multiple pharmacies to assemble all medications in one place and deliver them at once.

Specialty Pharmacy/Home Infusion Services would take a similar approach as that described by the HTC Nurse Coordinator. Specialty pharmacy/home infusion services are in a unique situation because of the different potential sites to coordinate a case such as this.

It really starts with planning. It might be that the patient is new to the specialty pharmacy/home infusion services provider and has grown accustomed to getting everything from one provider and now they have a new hemophilia provider, and in addition their hepatitis C and HIV medication may need to be obtained at a retail pharmacy. Sometimes that is really sensitive. Some patients do not want to go into their community retail pharmacy and pick up medications, because the pharmacy staff may know them from other circumstances. So it is a sensitive issue.

Specialty pharmacy/home infusion services can attempt to work with the insurance plan to override the multiple pharmacies requirement in specific situations where adherence to a treatment plan is essential. Specialty pharmacy/home infusion services may also coordinate among the multiple pharmacies to get all medications assembled in one place and delivering them at the same time. That capability may be unique to specialty pharmacy/home infusion services.

Managed Care Perspective, Case 1



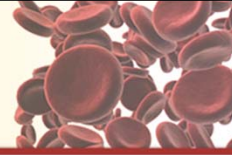
- Payers are open to working with hematologists, HTC nurses, and SP/Hi on these particular issues
 - *Must recognize that the new carrier does not know the patient's history*
 - *Early contact, before filling prescription and processing claims, is helpful*
- Moving prescriptions from retail pharmacies to mail order may be advisable
 - *Might be less expensive for the patient*
 - *Mail pharmacy may easily coordinate multiple prescriptions that are used chronically*

Payers are open to working with the other groups on these particular issues. When a patient changes carriers, the most important thing is to recognize that just because everything went smoothly with the last carrier does not mean the new carrier will know anything about what happened. Early contact before you try to fill a prescription, before claims are processed can be really helpful here. Many times it is a good idea to talk to somebody at the new health plan to inform them that the patient has used care management services in the past because of hemophilia. That can help get things to go more smoothly and make it less of a panic for the patient.

Health plans understand that care can become fragmented in ways that they did not expect, so they can be open to things like asking one specialty pharmacy to take on the entire work. This depends on the capabilities of the pharmacies.

Certainly there are ways to deal with retail pharmacies and move things to mail order pharmacy. Most health plans have a relationship with a mail pharmacy, and so they may be able, for example, to change an HIV medication from retail to the mail pharmacy. This might actually be less expensive for the patient, and it also allows the mail pharmacy to coordinate multiple prescriptions that are used chronically. Mail pharmacies usually do not manage clotting factor concentrates but they may be able to coordinate the small molecules.

Case 2: Prior Authorization (PA)



- **Clinical circumstances**

- *9-year-old male with severe factor VIII deficiency was playing outside in the snow and experienced acute bleeding. His monthly supply from a pharmacy provider had run out and his mother had neglected to order additional factor or have extra factor on hand for emergencies.*
- *Patient goes to the local ER during a snowstorm on a Saturday evening but the ER did not have any recombinant factor VIII on hand and contacted the HTC*
 - *HTC then needed to obtain a PA for an emergency dispensation*
- *HTC was unable to reach the payer for approval but went ahead and made factor available to patient within 3 hours, however, treatment of his bleed was delayed by at least 8 hours*
- *HTC was not reimbursed for emergency dispensation of factor because of failure to obtain PA*

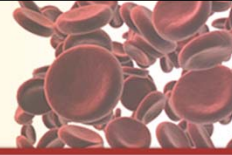
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A nine-year-old boy with severe factor 8 deficiency is playing outside in the snow and he gets an acute bleed. His monthly supply comes from a pharmacy provider, but had run out and his mother had neglected to order additional clotting factor concentrate to deal with this issue; nor did they have extra clotting factor concentrate on hand for an emergency like this.

The patient goes to local emergency department during a snowstorm. It is a Saturday night. The emergency department did not have on hand any of the recombinant factor 8 that this patient used, and they contact the HTC. The HTC needs to obtain a prior authorization for an emergency dispensation. However, the HTC is unable to reach the payer for approval; the HTC went ahead and made clotting factor concentrate available to the patient within three hours. However, treatment of his bleed ended up being delayed by at least eight hours.

In addition, the HTC was not reimbursed for the emergency dispensation of the clotting factor concentrate because of the failure to properly obtain the prior authorization.

Case 2: Critical Issues

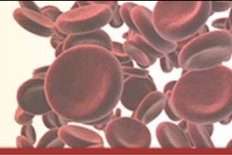


- **Critical issues**
 - *PA is designed to encourage appropriate utilization of medications yet patients and providers may have limited or no knowledge of the process*
 - *PA requirements vary between managed care plans and may be difficult to navigate, particularly in an emergency*
 - *PA may require a signed statement of medical necessity for each prescription*
 - *PA may take up to 24 hours to obtain, may be non-binding, and thus may delay delivery of care*
 - *Delays or potential delays caused by the need to obtain a PA for every dispensation may be life-threatening in the event of an acute bleeding episode*

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Let us look at the critical issues in this case. The prior authorization is designed to encourage appropriate utilization of medications, yet patients and providers may have limited or no knowledge of the process. The prior authorization requirements vary between managed care plans and they may be difficult to navigate, particularly in an emergency, as presented in this case. Some may require a signed statement of medical necessity for each prescription. These can take up to 24 hours to obtain, they may be non-binding, and may delay delivery of care. As in this case, these delays caused by the need to obtain prior authorization for every dispensation may be life-threatening if this is really an acute bleeding event that cannot be dealt with more promptly.

Hematologist Perspective, Case 2



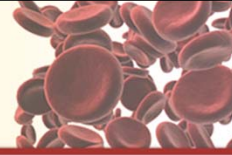
- Hematologists and HTC's strive to anticipate this situation with families
 - Engender a principal of always maintaining an emergency dose on hand
 - Ask patients to bring their own supply of clotting factor concentrate with them to emergency department
- Not uncommon that emergency departments and hospitals do not have clotting factor concentrate available to treat patients with hemophilia
- Even if HTC can get product to patient in short order, prior authorization may delay things further
 - A true detriment to the patient

First of all, as hematologists, we strive to anticipate this situation with our families from the time patients are infants engaging with the HTC. We want them to have a principle of always maintaining an emergency dose on hand. When patients have to use an emergency department, we ask them to bring their own clotting factor concentrate with them. We often provide them printed instructions to let the emergency department know that it is okay for them administer the emergency dose that the patient provides.

But every once in a while it happens that a patient allows an inventory to lapse and does not have an emergency dose available.

From a medical perspective, it is not uncommon that these patients show up and the emergency department literally does not have any product available in the hospital to treat hemophilia. For life-threatening bleeds this is a terrible situation. In addition, even if the HTC can get product out to the patient quickly, to have to go through prior authorization steps that might indeed delay things further is really a detriment to the patient.

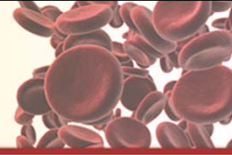
HTC Nurse Perspective, Case 2



- HTC nurses spend a lot of time with patients and families educating and preparing for these types of situations
- Even patients who are not on home infusion are asked to keep an emergency dose of factor at home for just this scenario
- Lack of product on hand needs to be avoided at all costs

From the HTC nurse perspective, we spend a lot of time with patients and families educating and preparing for these types of emergencies. Even for patients who are not on home infusion, we try to ensure that they have an emergency dose at home for just this scenario. Lack of product on hand needs to be avoided at all costs. As in this case study, prior authorization can be an issue in an emergency situation. At this nurse's HTC, however, to be honest, we usually do what we need to do and then worry about the cost later.

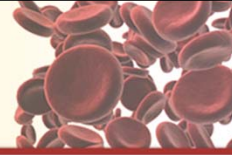
Specialty Pharmacy/Home Infusion Services Perspective, Case 2



- There are payer relationships where failure to appropriately plan and having a patient appear at an emergency department due to not having product available is a penalty to a specialty pharmacy
 - *In some cases, the onus is on SP/Hi to make sure patient has factor on hand*

There are payer relationships where failure to appropriately plan and having a patient appear at an emergency room due to not having product available is a penalty to a specialty pharmacy. So the onus is on specialty pharmacy/home infusion services to maintain contact with the patient to ensure this does not happen. As both of my previous colleagues mentioned, however, the best laid plans may go astray.

Managed Care Perspective, Case 2



- A specialty pharmacy can help ensure that prior authorization is obtained
- Plans should cover enough medication that patients have doses of clotting factor concentrate available at home
- Oftentimes, prior authorization is not required for emergency situations when the claim comes from an emergency provider
- This is an opportunity to see that the specialty pharmacy ensures that the patient does not run out of clotting factor concentrate
- The best way to make sure the patient has access to product is to make sure that the patient actually has the drug

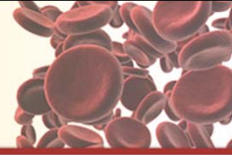
If prior authorization is required, and it often is in this care setting, prior authorization can be granted for all of the products that the patient uses either on a regular basis or an interim basis and can be renewed every six to 12 months, depending on the plan's rules. A specialty pharmacy can help to make sure that prior authorization is obtained.

Plans should cover enough medication that patients can have their doses available at home. In this case study for example, the plan would have probably paid for the clotting factor concentrate if the patient was being treated in an emergency situation, because oftentimes prior authorization is not required for emergency situations. But because the billing came from a non-emergency provider, there was some trouble here in this example.

This is an opportunity to see that the specialty pharmacy ensures that the patient does not run out of clotting factor concentrate and that they have the appropriate medications with them, because a lot of emergency departments just do not have these medications available. The emergency departments are too small to use them. The products expire on their shelves.

This is a matter of coordinating between the specialty pharmacy, the HTC, the patient, and the health plan to make sure things are taken care of and that the patient has access to the necessary medications.

Case 3: Prophylaxis



- **Clinical circumstances**
 - 19-year-old student moves out of state to attend college
 - His severe factor VIII deficiency is well controlled with an ongoing prophylactic regimen
 - Patient is compliant with his treatment regimen with only rare breakthrough bleeds in the year prior to leaving for school
 - Patient reports increased bleeds and factor usage during the first 2 months at college
 - Patient's supply of factor concentrate is only sufficient to cover his prophylactic regimen, without doses for breakthrough bleeding episodes
 - Patient is unaware of insurance carrier's PA process for doses needed beyond routine prophylaxis
 - Dispensing agency was prohibited from providing further doses that month due to having to obtain a PA for prophylaxis
 - The patient was instructed to go to the ER for factor

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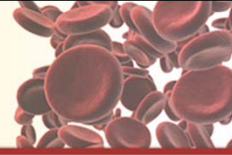
In the third case, a 19-year-old student moves out of state to go to college. He has severe factor 8 deficiency. He has been well-controlled on a regular prophylactic regimen. The patient has been adherent with his treatment with only rare breakthrough bleeding and has no joint disease to speak of.

During the first two months of college, the patient reports some increased bleeds and factor usage. We often see this. Perhaps it is due to increased activity, maybe there is more walking involved.

The patient's supply of clotting factor concentrate is only sufficient to cover his prophylaxis regimen, which does not include doses for breakthrough bleeding episodes. The patient is also unaware of the insurance carrier's prior authorization process for doses needed beyond his routine prophylaxis. The dispensing agency was prohibited from providing further doses that month due to having the prior authorization approved just for the prophylaxis.

The patient is instructed to go to the emergency department for clotting factor concentrate.

Case 3: Critical Issues



- **Critical issues**
 - *Care providers require flexibility in their prescriptions for patients to allow sufficient product availability to treat according to standard regimen and for acute bleeding episodes*
 - *Patient needs proactive planning to maintain uninterrupted supply of product and to establish a relationship with an HTC in his college town*
 - *Local HTC providers need to communicate with the patient's hometown hematologist to consult on possible adjustments to the patient's treatment to achieve optimal control and minimize avoidable resource consumption (i.e., hospital, ER, factor, etc.)*
 - *Importance of prophylaxis is increasingly recognized but individual treatment challenges remain*

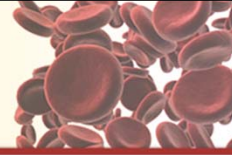
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A critical issue in this case is that the care providers must permit flexibility in their prescription for patients to allow for sufficient product availability to treat, not only their standard regimen, but also for acute bleeding episodes. Even though the average patient who is well-controlled on prophylaxis may have less than one bleed per year, we nevertheless build into the prescription anticipation for trauma or unusual circumstances where they might have breakthrough bleeding.

The patient also needs proactive planning in order to maintain an uninterrupted supply of product. Running so close to the edge just to cover his prophylaxis doses is not appropriate. The HTC should work with him, particularly when he moves on to his new setting and maybe try to coordinate his care with a local HTC in his college town. The local HTC providers need to communicate with the patient's own established hematologist to consult on any needed adjustments to the regimen. This is in order to maintain the optimal control that the patient has had up to this point.

It is also important to recognize that individual treatment plans can change. They change with different life circumstances, a new environment, etc.. We are always revising prophylaxis regimens. Just because a patient has been on 15 doses a month at a particular dose, does not mean that cannot change at a moment's notice when the patient is in a new setting.

Hematologist Perspective, Case 3

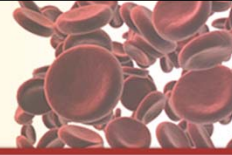


- HTC should provide contact information and a summary of medical care to patients when traveling out of state
 - *Despite this proactive approach, the HTC cannot guarantee that patients always follow through*
- HTC should always write prescriptions for patients on prophylaxis to allow for doses required to treat bleeding events experienced outside of planned therapy
 - *Current constraints with prior authorizations may place some patients in jeopardy if the number of bleeding events exceeds the prescription for that month*

The HTC should always provide contact information and a summary of medical care to patients when traveling out of state, especially when patients plan to reside in another area for extended periods of time. Despite this proactive approach, the HTC cannot guarantee that patients always follow through.

In addition, the HTC should always write prescriptions for patients on prophylaxis to allow for doses required to treat bleeding events experienced outside of planned therapy. However, current constraints with prior authorizations may actually place some patients in jeopardy if the number of bleeding events experienced exceeds the prescription for that month. This possibility needs to be addressed proactively to protect the patient and support quality care.

HTC Nurse Perspective, Case 3

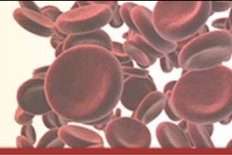


- Problematic that prescribers are unable to get approval for episodic doses for breakthrough bleeding for patients who are on prophylaxis
 - *Prophylaxis doses and vials are not necessarily the appropriate doses for bleeds*
- HTC Nurse would like to advocate for approval for everyone with hemophilia to have doses set aside at home for potential bleeding episodes

The prior authorization may not be the most common problem. But it is a problem that we are not able to get approval for episodic doses for breakthrough bleeding for patients who are on prophylaxis.

The prophylaxis doses and vials that they have at home are not necessarily the appropriate doses for bleeds. This is something that is a relatively new issue that we have been facing. I would like to advocate for approval for everyone with hemophilia to have doses set aside at home for potential bleeding episodes.

Specialty Pharmacy/Home Infusion Services Perspective, Case 3

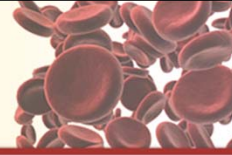


- It is concerning that other providers are reporting difficulties obtaining authorization for extra doses of clotting factor concentrate
- Specialty pharmacy/home infusion services are typically planning well in advance to make sure adequate clotting factor concentrate is available
- One important consideration with this population is finding creative ways to keep in touch
 - *Emailing*
 - *Text messaging*

This specialty pharmacy/home infusion services representative has not yet experienced being unable to get extra doses authorized yet. It is concerning that other providers are reporting this. Specialty pharmacy/home infusion services are typically planned well in advance to make sure an adequate supply of clotting factor concentrate is available.

One important consideration with this population is finding a way to get in touch with them. We have got to be creative. What is most effective? Is it emailing; is it text messaging? Finding creative ways to communicate with this population can eliminate barriers for us.

Managed Care Perspective, Case 3



- Patient care and safety absolutely must come first
 - *Emphasizes the need to work with one specialty pharmacy that has the expertise that can meet quality standards*
- It is best to work with a specialty pharmacy that has expertise and good patient-provider relationships that can quickly take action when something does occur to help navigate the process

Patient care and safety absolutely need to come first and that is what managed care would want to occur for hemophilia patients. This really emphasizes the need to work with a specialty pharmacy that has the expertise that can meet quality standards, and can coordinate effectively with one or more HTC's.

As we discussed in Case 1 regarding the importance of working with a single pharmacy for all prescription medications, from the managed care perspective, it is best to work with someone that has expertise and good patient-provider relationships that can quickly take action when something does occur to help navigate the process.