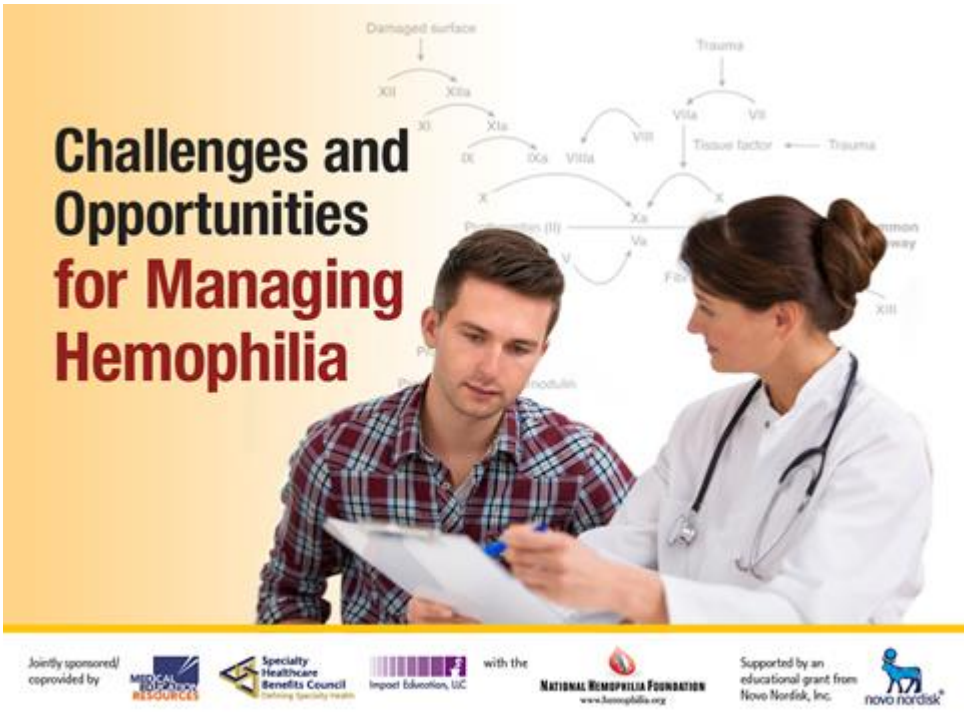
Challenges and Opportunities for Managing Hemophilia 2014

Track 3: Access, Coverage, and Care Issues

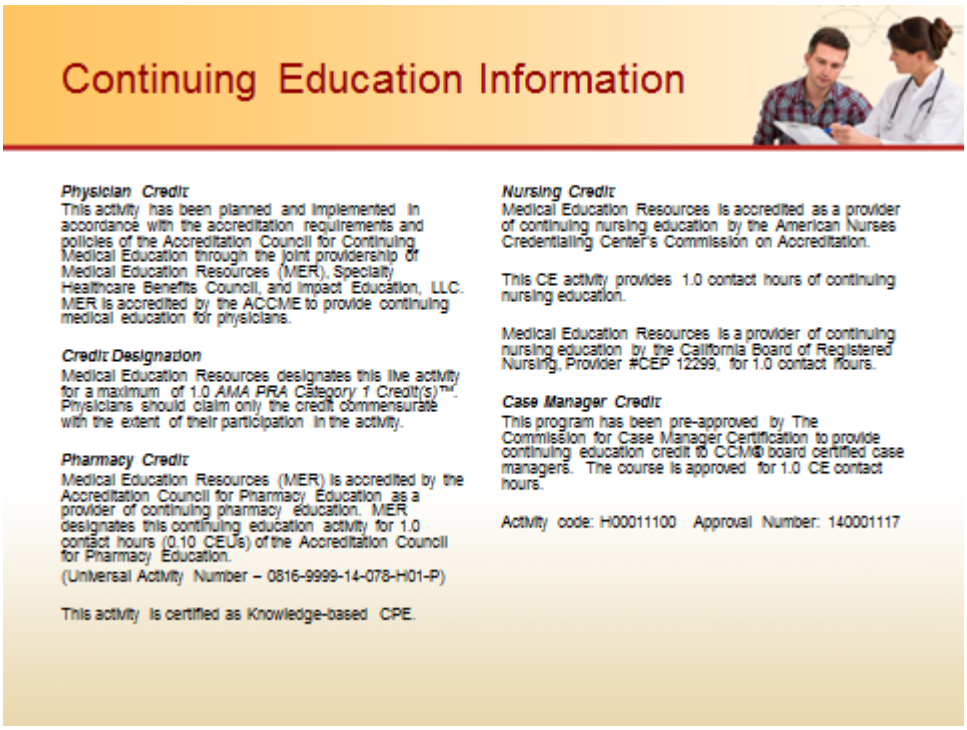


Slide 1: Challenges and Opportunities for Managing Hemophilia

Welcome to Challenges and Opportunities for Managing Hemophilia 2014 Continuing Education Series.

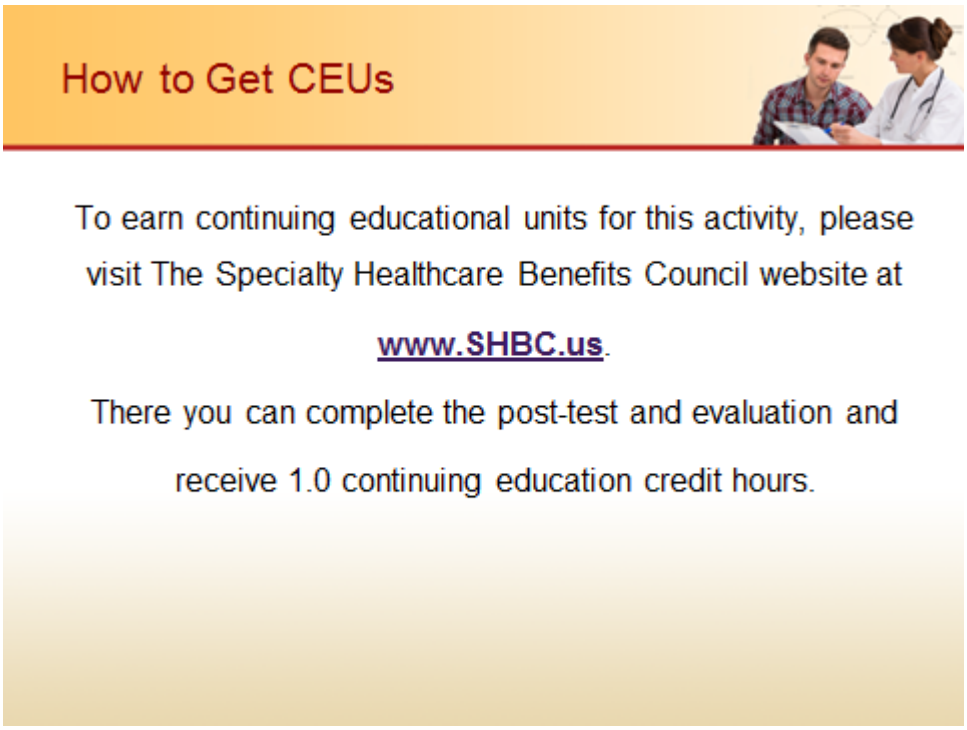
This series of three tracks is jointly sponsored by Medical Education Resources, the Specialty Healthcare Benefits Council, and Impact Education, LLC, in collaboration with the National Hemophilia Foundation.

This activity is supported by an educational grant from Novo Nordisk, Inc. and we would like to thank them for their support.



Slide 2: Continuing Education Information

Continuing education for this activity is provided by Medical Education Resources, or MER. They have designated this activity for one (1) credit hour for each track, or a potential total of three (3) hours if all three tracks are completed. This credit is for physicians, pharmacists, nurses, and case managers.



Slide 3: How to Get CEUs

To earn continuing educational units for this activity, please visit The Specialty Healthcare Benefits Council website at www.SHBC.us. There you can complete the post-test and evaluation and receive 1.0 continuing education credit hours.

Slide 4: Financial Disclosures

The faculty and planners reported the following financial relationships with commercial interests whose products or services may be mentioned in this continuing education activity.

Slide 5: Financial Disclosures (continued)

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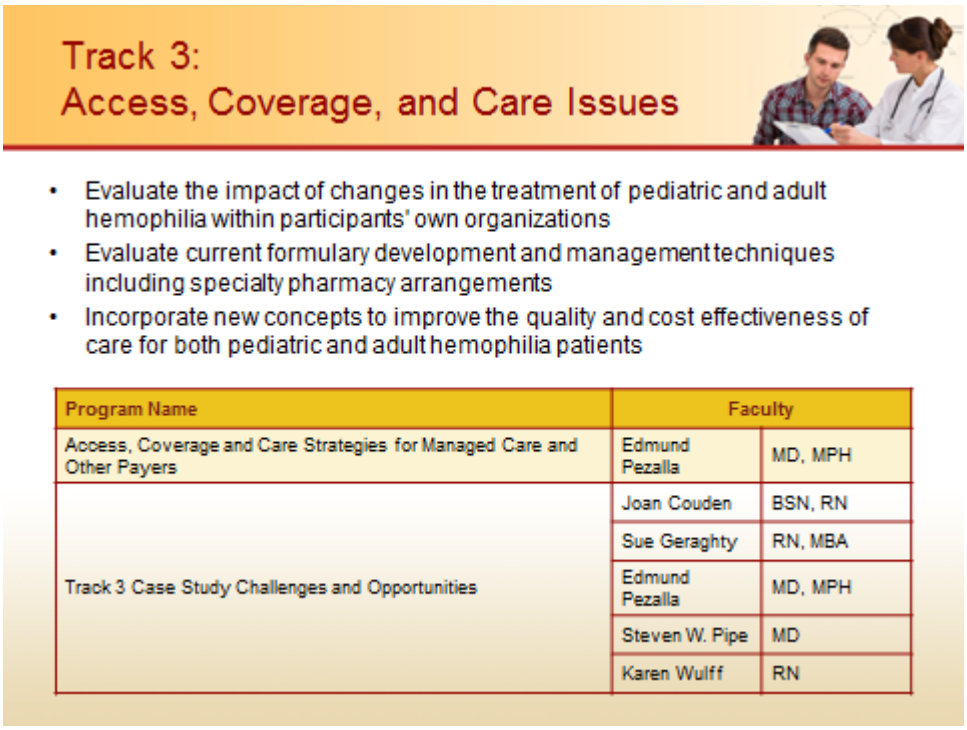


Slide 6: Track 3: Access, Coverage, and Care Issues

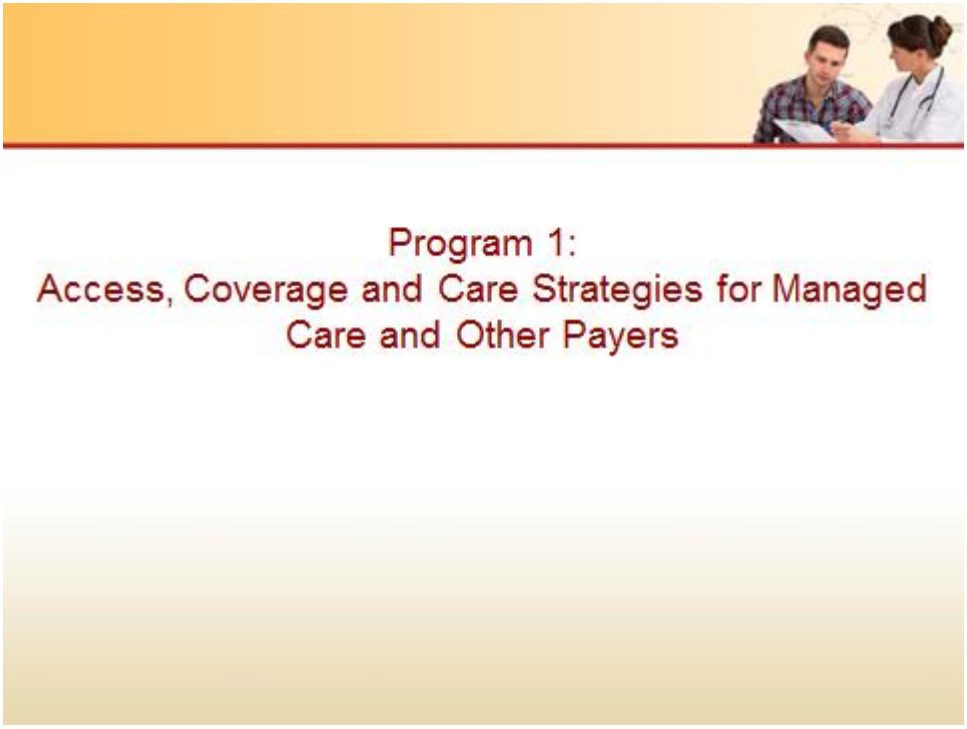
Welcome to Track 3 of a three part series. This track, entitled Access, Coverage, and Care Issues, will focus on the care management strategies for managing hemophilia within a managed care setting.

Upon completion of this learning track, the learner will be able to:

1. Evaluate the impact of changes in the treatment of pediatric and adult hemophilia within participants' own organizations
2. Evaluate current formulary development and management techniques including specialty pharmacy arrangements
3. Incorporate new concepts to improve the quality and cost effectiveness of care for both pediatric and adult hemophilia patients

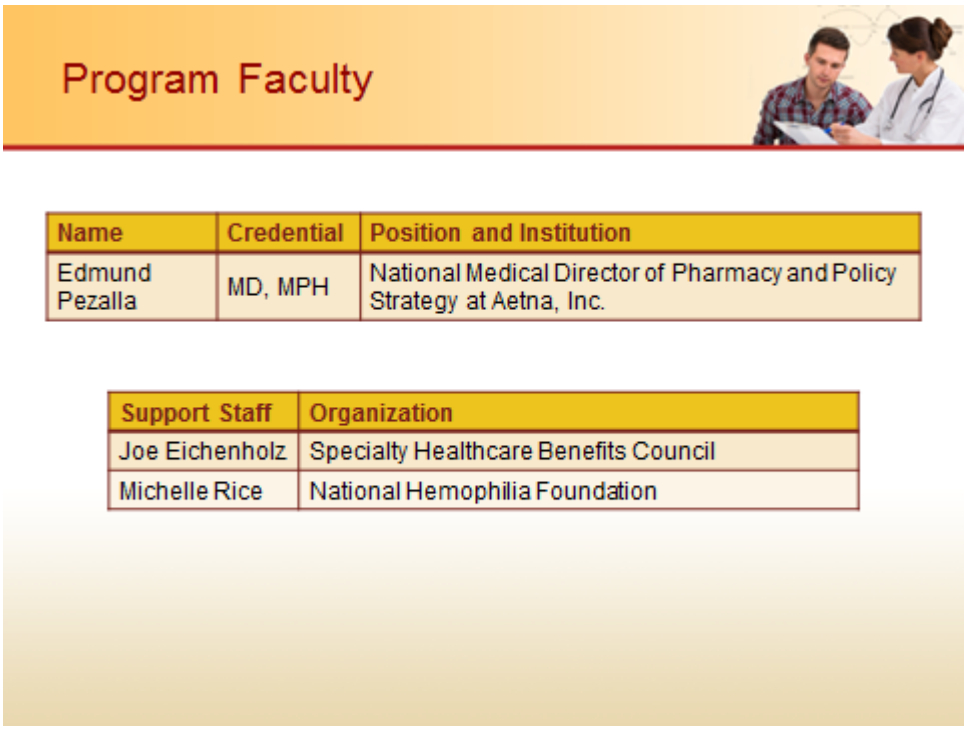
Program 1:

Access, Coverage and Care Strategies for Managed Care and Other Payers



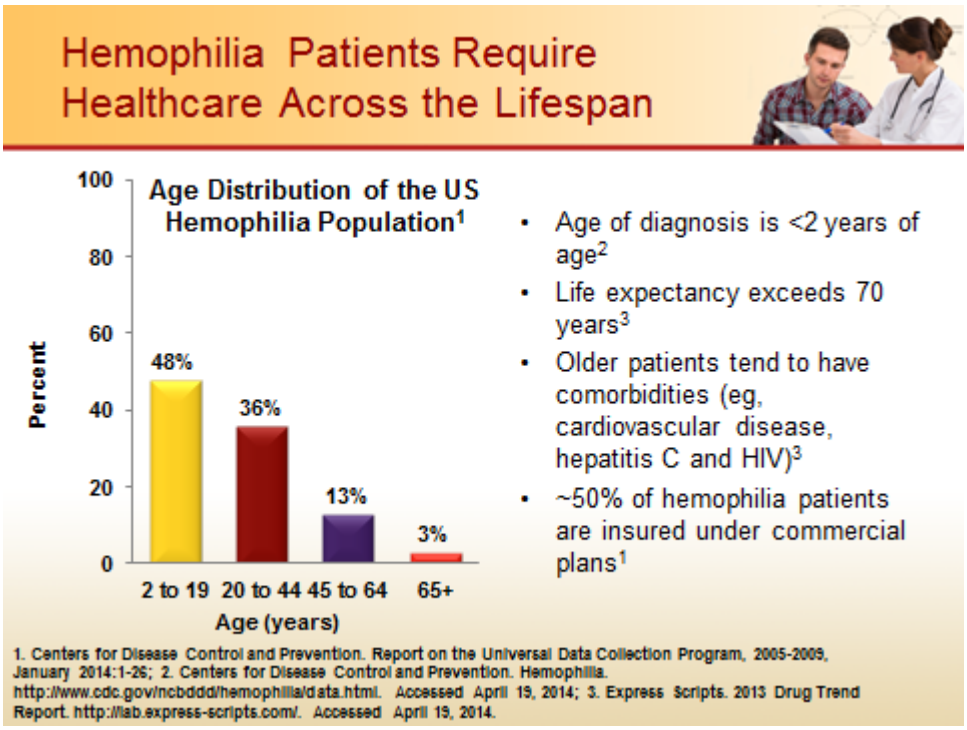
Slide 7: Program 1: Access, Coverage and Care Strategies for Managed Care and Other Payers

The first presentation of Track 3 is entitled Access, Coverage and Care Strategies for Managed Care and Other Payers.



Slide 8: Program Faculty

The faculty for this presentation is Dr. Edmund Pezalla, National Medical Director of Pharmacy and Policy Strategy at Aetna, Inc. Support staff for this presentation includes Joe Eichenholz, Executive Director of the Specialty Healthcare Benefits Council, and Michelle Rice from the National Hemophilia Foundation's Public Policy Team.



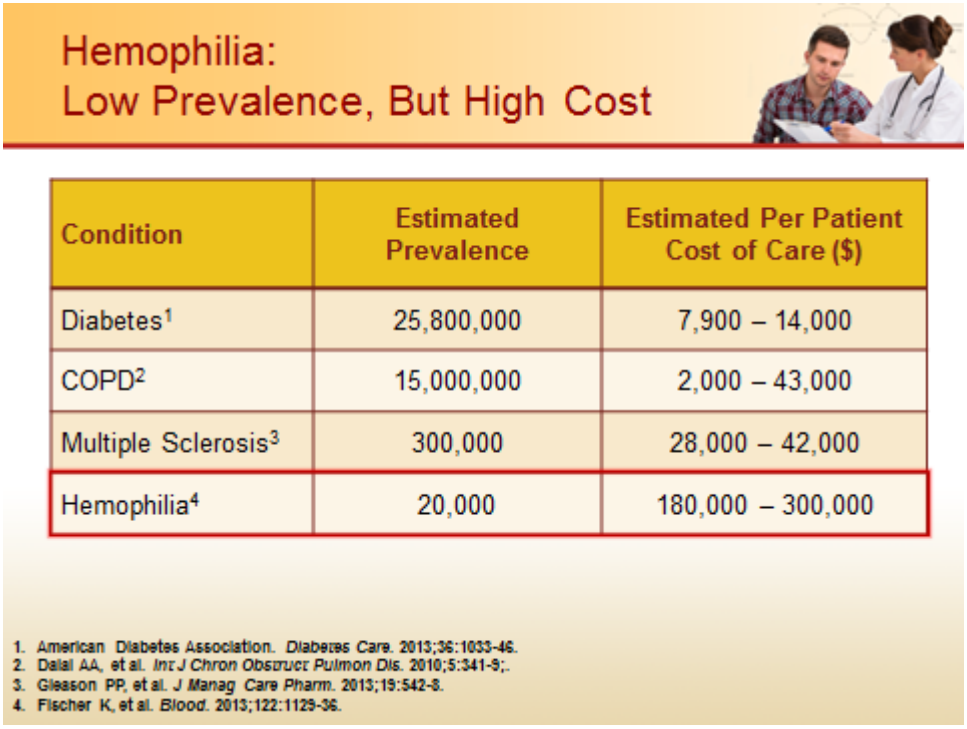
Slide 9: Hemophilia Patients Require Healthcare Across the Lifespan

Hemophilia is not just a problem of the young. Oftentimes what comes to mind when thinking of a person with hemophilia is a young child or an adolescent; and indeed, the average age of diagnosis is less than 2 years of age. However, as life expectancy exceeds 70 years, there are now many hemophilia patients who are surviving far into adulthood where, in addition to dealing with comorbidities acquired earlier in life such as hepatitis C and/or HIV, they are now developing comorbidities such as cardiovascular disease and diabetes. This entails taking other medications in addition to clotting factor concentrate.

Some patients are now in need of thrombosis management at the same time they also have hemophilia. They are entering an age in which we may give them platelet aggregation inhibitors, such as Coumadin®, and other medications to reduce the risk of stroke and to treat them because they have stents. They are becoming more medically complex and certainly they should receive care at the appropriate site with the appropriate specialist.

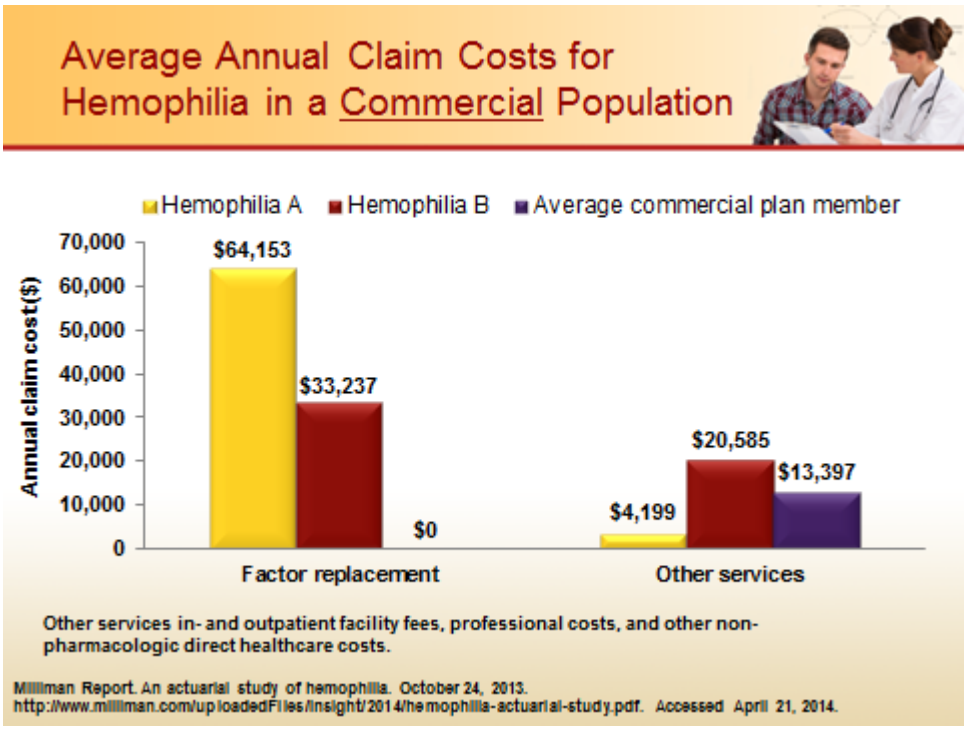
The age distribution reflects that up until now, relatively few persons with hemophilia have survived to age 65. This is not due to a lack of clotting factor concentrate or understanding how to use it, but because there was a significant impact of HIV disease on this population a number

of years ago before effective treatments for HIV came about. Many patients did not survive through that period and it reduced life expectancy for the entire population. Now that the situation has clearly changed, it is critical to recognize that we will be caring for hemophilia patients through the normal life expectancy.



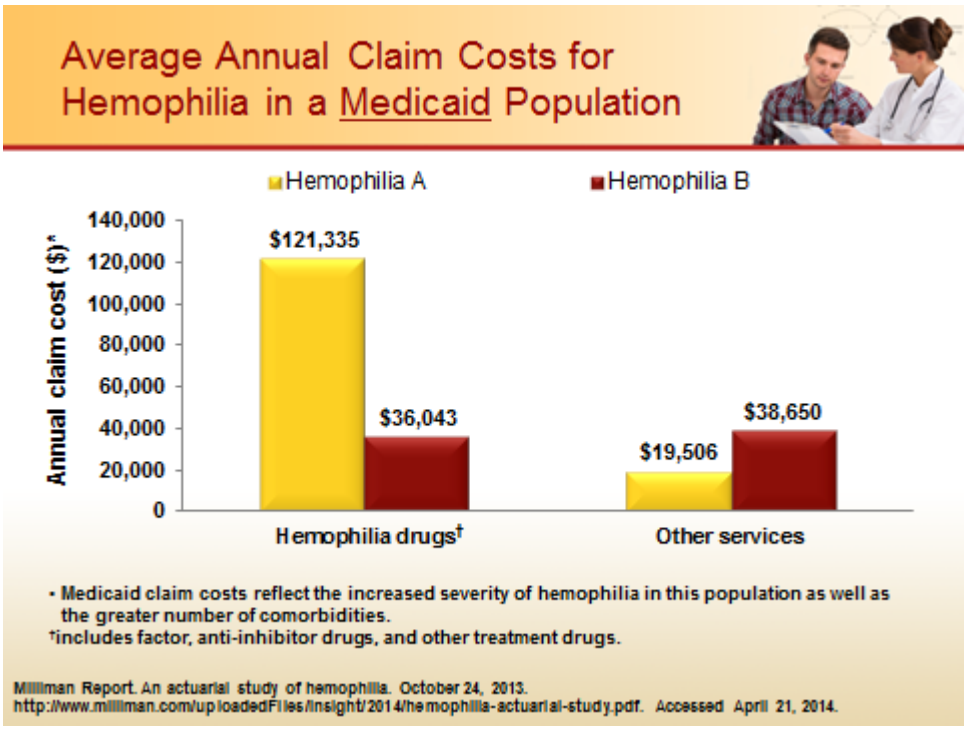
Slide 10: Low Prevalence, But High Cost

Hemophilia is a very low prevalence disorder in the United States, and when broken out into specific types of hemophilia, prevalence rates are even lower. But because of the high cost of care for each patient, the total cost of care can be significant. This means that we should think very hard about managing these patients well. They are a special population. We must ensure that we do not have an unnecessary financial impact on the family, on the hemophilia patient himself, or on those who are paying premiums to their health plans.



Slide 11: Average Annual Claim Costs for Hemophilia in a Commercial Population

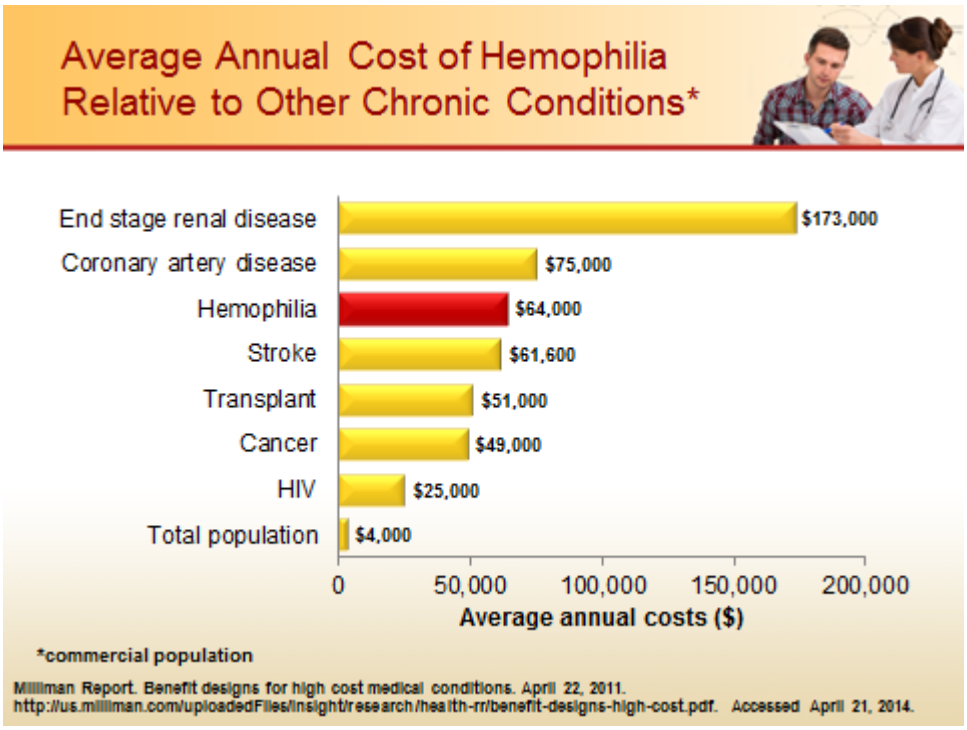
The average claim cost has much to do with clotting factor replacement. Hemophilia patients do use other services. As this slide illustrates, hemophilia B patients tend to be more likely than hemophilia A patients to use other health care services and do so more than the average patient in a health plan. Far and away, however, the most important cost concerns are the use of clotting factor. These costs emphasize that we should be using factor wisely and in the appropriate settings, but they do not suggest that we should avoid the use of factor where it is appropriate for patients, either as prophylaxis or for treatment of bleeding events and other indications.



Slide 12: Average Annual Claim Costs for Hemophilia in a Medicaid Population

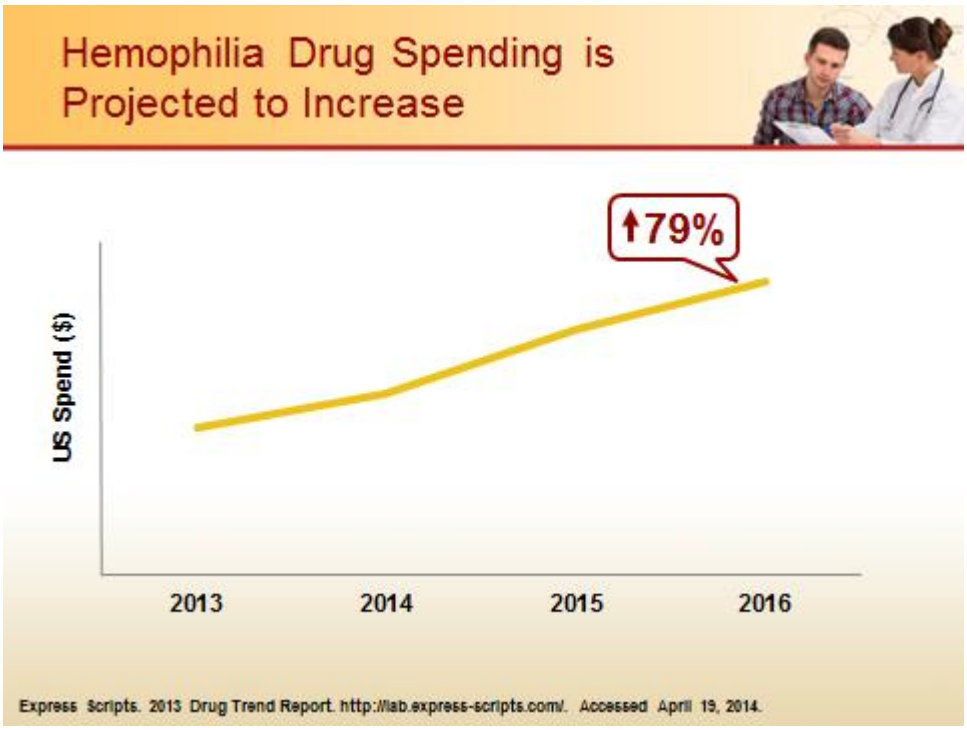
In the Medicaid population we see a similar picture: hemophilia A patients principally require factor and do not utilize the same quantity of other services as do hemophilia B patients, although they do use more than the commercial population. Hemophilia B patients continue to use significant other services; in this case the other services exceeded the hemophilia drug component.

These cost analyses reflect the fact that Medicaid patients tend to be more complex. In part, this may be due to more complex disease with greater complications, which may be the reason they are covered by Medicaid in the first place. However, it may also indicate the important psychosocial variables at play in some portions of the Medicaid population which could, for instance, lead to a patient's hospitalization in a case where, in a commercial population, the patient would have been able to manage care from home.



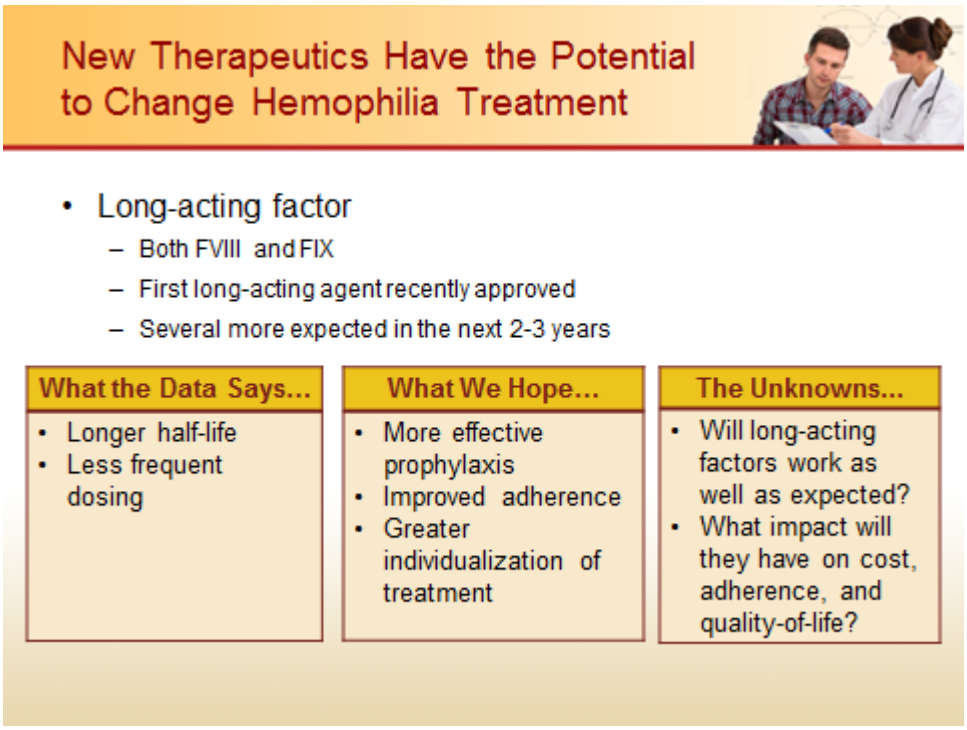
Slide 13: Average Annual Cost of Hemophilia Relative to Other Chronic Conditions

The annual cost of care for hemophilia is on par with other major chronic conditions and is greater than many disorders that receive significant media attention. Patients with end stage renal disease tend to outspend all others due to their need for dialysis, other replacements, and other management issues. Problems and costs among coronary artery disease patients are generally driven by procedures including stents, angiographies, etc. As we have seen, the hemophilia patient cost tends to be driven by medication whereas coronary artery disease costs are not driven by medication, many of which are generic for that disease state.



Slide 14: Hemophilia Drug Spending is Projected to Increase

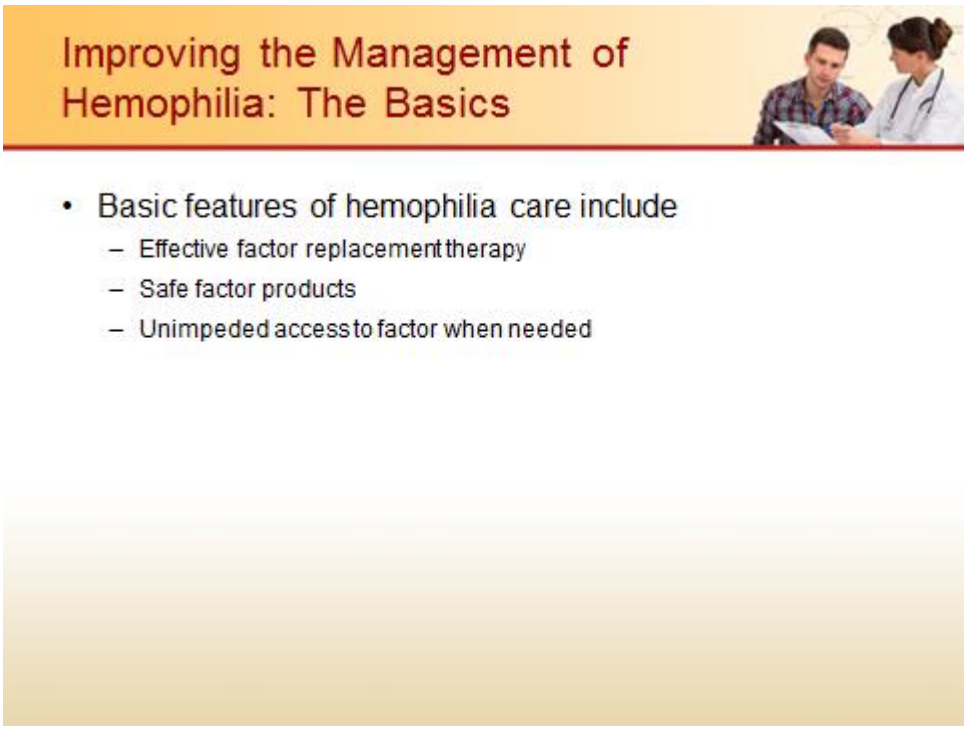
Hemophilia drug spending is expected to increase. This includes not just spending on each individual, but spending overall. More patients are surviving such that the number of patients with hemophilia is fairly large as compared to historic numbers. Variables contributing to the increased life expectancy may include the eradication of HIV in the factor product supply and the increased utilization of prophylactic treatment. Prophylaxis is an interesting therapy in that it may increase drug costs early in the course of the disease, but later in life, might reduce overall costs by decreasing joint disease and disability, reducing dependency on public programs, and enhancing ability to become an active functioning member of the work force.



Slide 15: New Therapeutics Have the Potential to Change Hemophilia Treatment

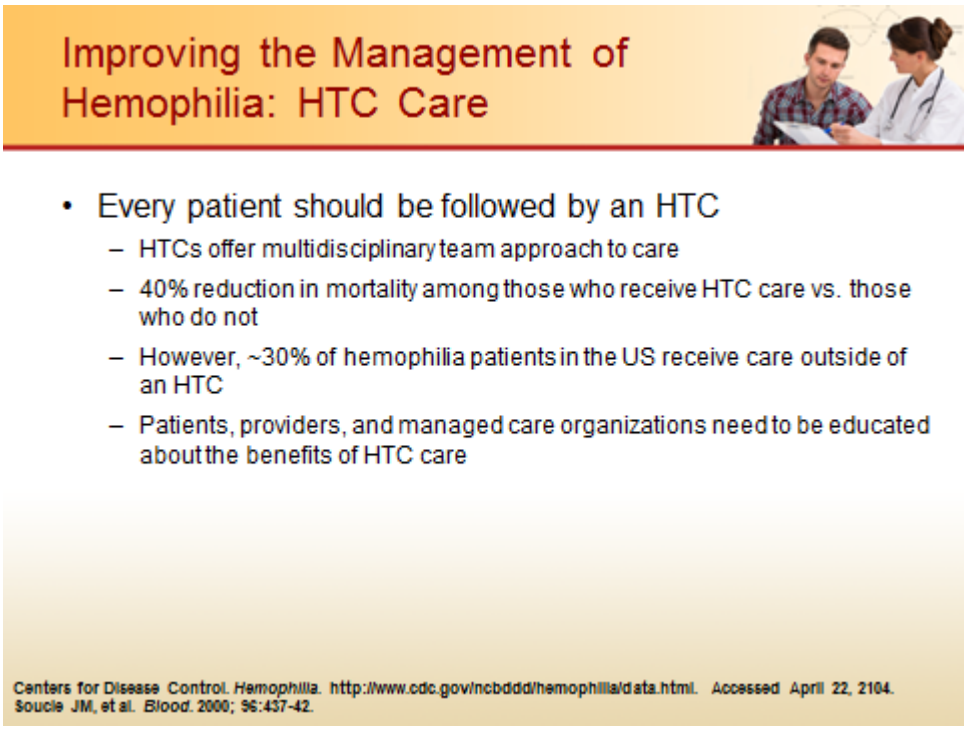
There is tremendous potential in the new treatments that are becoming available such as the long-acting products. Long-acting factor products are now available for both hemophilia A and hemophilia B, and we expect to see several more within the next three years. The longer half-life leads to less frequent dosing, which will ease the burden and increase the effectiveness of prophylactic therapy. We also expect to see improved treatment adherence as patients require fewer infusions. This may also lead to better individualization of treatment.

One consideration to bear in mind is that although we do expect adherence to be better with long-acting agents due to less frequent infusions, if a patient misses a dose, then he may go for a longer period of time without medication, putting him at increased risk for bleeding.



Slide 16: Improving the Management of Hemophilia: The Basics

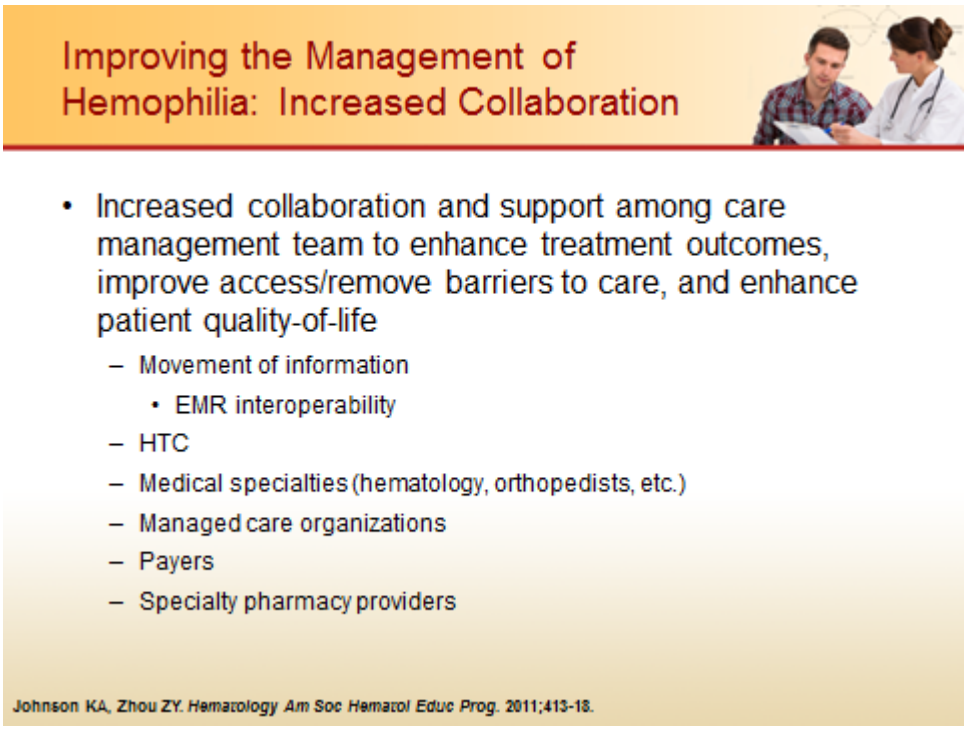
Some of the basic features of managing hemophilia include having the patient on effective factor replacement therapy, ensuring access to safe clotting factor products, and safeguarding unimpeded access to factor product when needed.



Slide 17: Improving the Management of Hemophilia: HTC Care

Hemophilia Treatment Centers, or HTCs, are an important part of hemophilia care in the United States. They offer multidisciplinary care teams and have providers who are familiar with hemophilia patients and are expert in their care. The multidisciplinary team at an HTC can provide services to the patient as well as advice to his family and to other health care providers throughout the community.

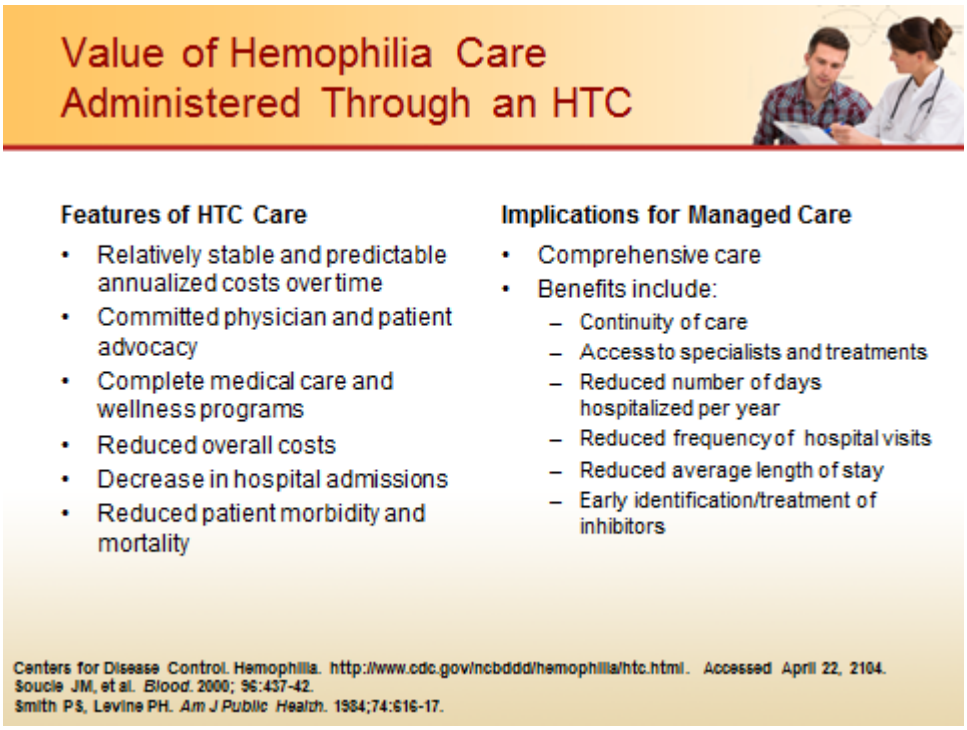
There is a 40% reduction in mortality among those who receive care at an HTC compared to those who do not. However, about 30% of patients do not receive care at an HTC. This may be due to personal preference or to access issues. Some patients live in remote areas and the nearest HTC is far away. In all cases, patients, providers, and managed care organizations need to be educated about the benefits of HTC care. In cases where the patient lives at a distance from an HTC, it is recommended that the patient's local provider, whether it is a hematologist, internist, pediatrician, etc., have some discussion with physicians and nurses at the HTC to become educated about hemophilia.



Slide 18: Improving the Management of Hemophilia: Increased Collaboration

If a patient does attend the HTC for an annual visit, but otherwise utilizes a local provider for treatment, it is most useful to be able to move their information between the HTC and the local provider. In real life, the responsibility for movement of information between providers often falls on the patient. Providers can take on the responsibility of proactively documenting for the patient any important information such as diagnoses, treatments, including prescribed drugs and dosages, and contact information. Over time, as electronic medical records, or EMRs, become more accessible, they will be an enormous help to patients with complex disorders who receive care from more than one provider or specialist. With EMRs, we must be vigilant about HIPAA, or the Health Insurance Portability and Accountability Act, and other privacy issues. We also need to ensure interoperability, or the capacity for exchanging health information across various health care systems' electronic platforms, as this remains a barrier to effective collaboration among providers.

When discussing the goal of increased collaboration to improve the management of hemophilia, there are many players. These include the HTC, medical specialties such as hematology and oncology, managed care organizations, payers, and specialty pharmacy providers.

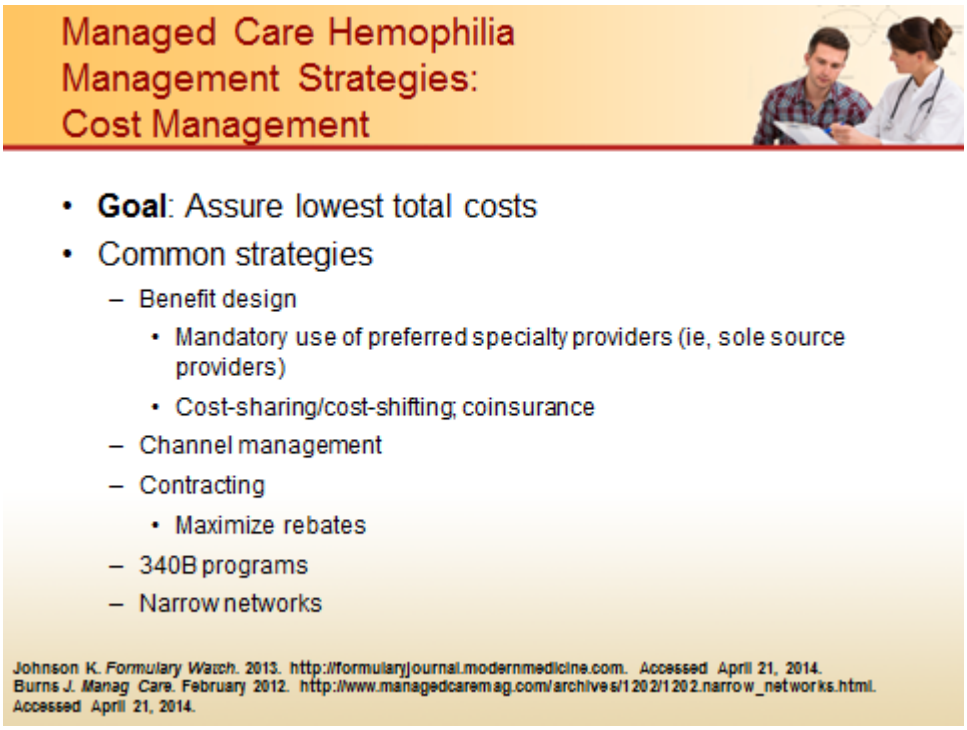


Slide 19: Value of Care Administered Through an HTC

The value of care administered through an HTC is immense. HTCs are accustomed to caring for patients with hemophilia. They are familiar with different types of hemophilia, different prognoses, and differences in the types of problems that may present. They also understand how to coordinate some of the challenging situations that may arise such as severe injuries, dental procedures, and surgeries.

Excluding the cost of clotting factor, HTCs offer relatively stable and predictable annualized costs over time. Managed care companies are wise to pay for the care provided and for coordinating with HTCs because it is more comprehensive and affordable care than is going to be found in most other places. When HTCs provide outstanding care at relatively stable costs over time, and managed care companies pay those costs, there is care continuity for the patient.

Other features of HTC care include a committed physician, patient advocacy, comprehensive medical care and wellness programs, reduced overall costs, decrease in hospital admissions, and reduced patient morbidity and mortality, as we have already discussed. These features result in many benefits for the managed care company.



Slide 20: Managed Care Hemophilia Management Strategies: *Cost Management*

Managed care companies have a number of responsibilities, but a primary goal is to assure that money is well spent resulting in the lowest total costs. Managed care companies do not want to pay more than necessary and they want to ensure that patients do well with money that is spent.

Strategies that are generally employed begin with benefit design. Here we ask, What is a covered benefit for this particular member? Are there any mandatory providers? For example, is the patient required to use a particular specialty pharmacy?

In many states there are any-willing-provider laws requiring managed care organizations to grant network participation to health care providers willing to join and meet network requirements. However, even in any-willing-provider states, the managed care company can deem certain providers "out of network," and use of these providers might result in a higher co-pay or co-insurance for the patient. This is an example of cost-sharing or cost-shifting and it can create significant financial burden for the patient. One of the most important things health care entities can do to help patients and their families is to verify that they are using in-network providers wherever possible to reduce their out-of-pocket expenses.

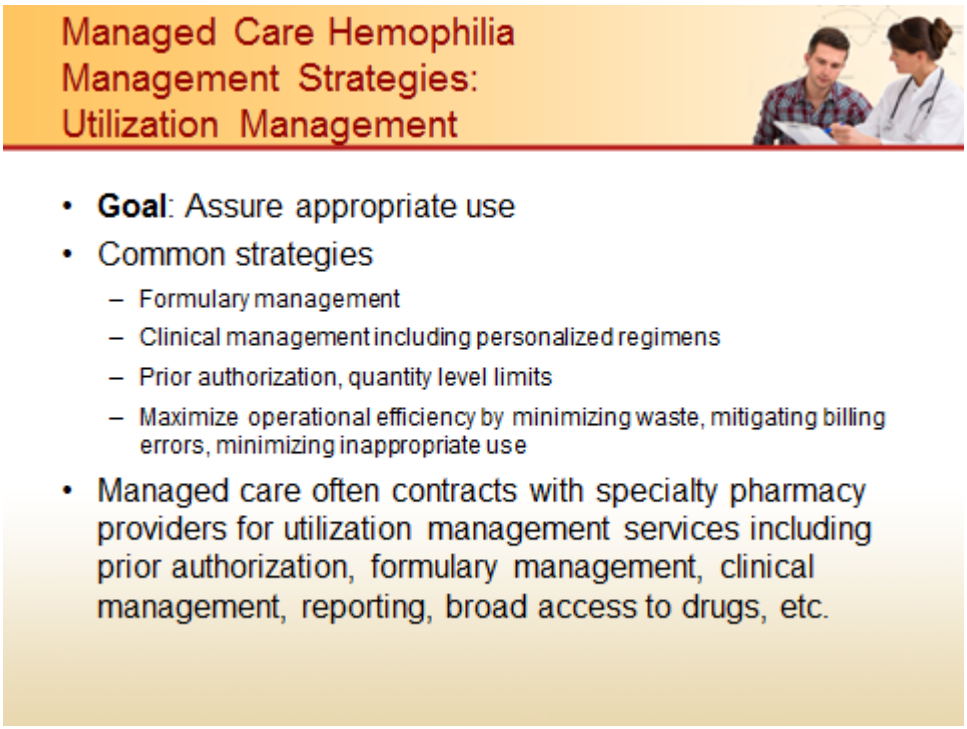
Channel management is the term for obtaining medications, devices, and other objects used by the patient through the best source in terms of cost. Managed care companies take measures to procure items that hemophilia patients need, including clotting factor concentrate and bypass agents, through a channel that warrants them the best price. Many times this means using the specialty pharmacy that is either owned or contracted by the payer.

Another cost management strategy is contracting with specialty pharmacies and manufacturers. Many times, if you have a formulary, you receive rebates for medications on that formulary, and that discount is proportional to how much of that medication you purchase. If you have preferred agents, these are what you try to get patients to use. Most plans have rules around how to do that. Sometimes the strategy is simply to bring up the preferred product, sometimes it is to reduce the amount of cost-sharing that the patient has for the preferred product, and sometimes there are actually requirements to use one medication before another.

The 340B drug purchasing program allows eligible entities, including specialized clinics such as HTCs and certain hospitals, to purchase drugs at or below government mandated ceiling prices. The intent of the 340B program is to further stretch the resources of the participating entities. This may result in a lower billed cost to the payers; however, passing the savings along is not a requirement of the program.

The term “narrow networks” means that not all providers in an area are in a network. It is not always possible to have narrow networks because, as mentioned previously, many states have any-willing-provider laws. However, in states that do not have such laws, the purpose of narrow networks is to drive business towards certain providers and for the resultant increased volume, the payer receives a discount. We often see the strategy of narrow networks used in hemophilia with specialty pharmacies where there might be only one or two pharmacies used by an insurance plan because they are able to get good pricing by having more of their patients use those particular pharmacies.

With regard to the narrowing of networks, it is the National Hemophilia Foundation's position on network adequacy that there should be more than one qualified in-network pharmacy provider per health plan.



Slide 21: Managed Care Hemophilia Management Strategies: *Utilization Management*

Appropriate use is an extremely important aspect of the utilization management strategy for managed care and this is especially true in hemophilia. Here we want to see that patients are receiving the right doses of clotting factor concentrate at the right intervals.

Many managed care companies have formularies for medications, and the formulary management usually has a utilization management component that stipulates that for patients starting a new drug, or for certain situations, patients must use one medication before others. There are usually exception rules and allowances to request a deviation from the protocol. Rules vary widely from payer to payer.

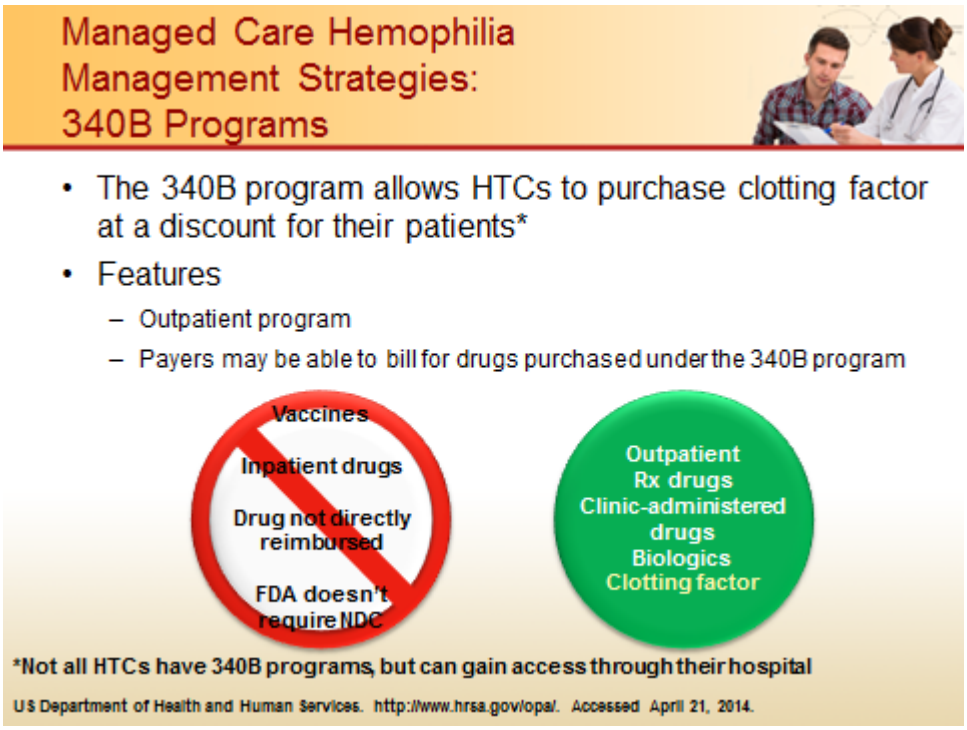
There may be some personalized clinical management, and certainly a connection between the managed care plan and an HTC can help to maintain patients on their personalized treatments and can help to overcome prior authorization problems.

Generally speaking, there are quantity or cost limits on almost everything in managed care. However, the reality is that patients have special requirements and there are occasions when large volumes of factor are needed, such as when a patient has a surgery or serious injury. Quantity limits can be exceeded through a prior authorization process or, in some cases, through negotiation after the factor has been dispensed, though this is not the recommended approach.

Managed care plans want to maximize operational efficiency by minimizing waste, minimizing billing errors, and minimizing inappropriate use of clotting factor concentrate. Thus, specialty pharmacies are expected to be able to dispense factor within reasonable ranges of the dose expected for the patient. This is called assay management. Managed care does not want wasteful or excessive use of factor. At the same time, managed care does not want inappropriately low use of factor that is not effective for the patient.

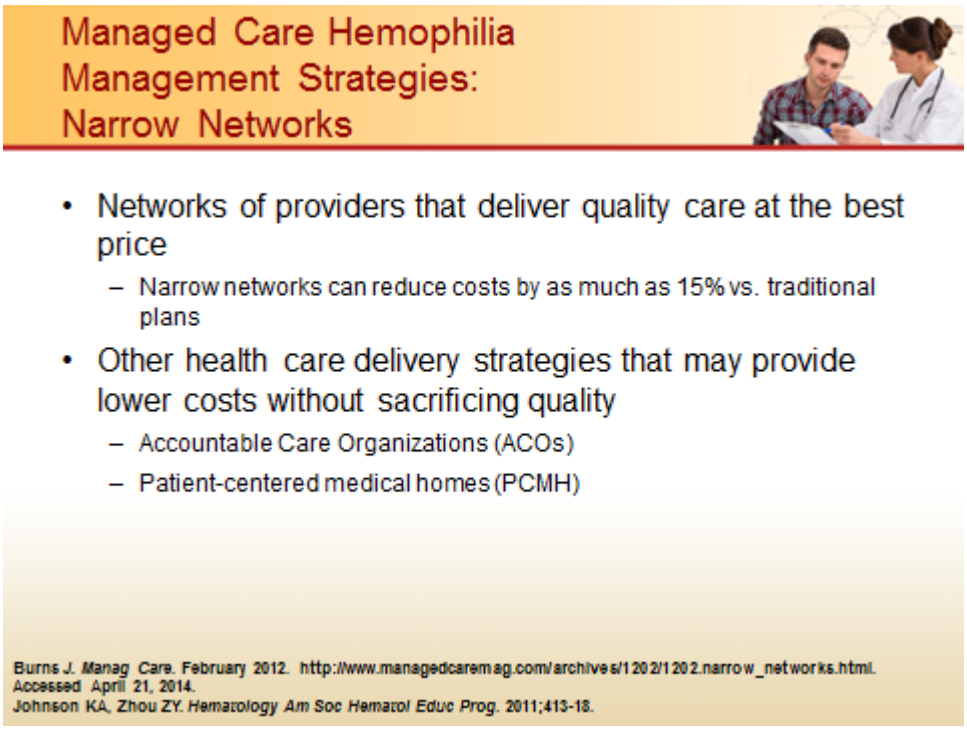
Finally, billing is commonly a problem. To start with, most plans require the J-codes, which are Healthcare Common Procedure Coding System, or HCPCS, reimbursement codes used to identify injectable drugs that are associated with the medications. National Drug Codes, or NDC, which are unique product identifiers used in the U.S. for drugs intended for human use. Some additional codes may be useful to understand what is going on with that particular patient. Of course, plans also require an explanation as to the amount of clotting factor and the weight of the patient to calculate factor units per kilogram.

For all of these strategies, managed care often contracts with specialty pharmacy providers.



Slide 22: Managed Care Hemophilia Management Strategies: *340 B Programs*

As stated earlier, 340B programs allow eligible entities, including specialized clinics such as HTCs and certain hospitals, to purchase drugs at lower prices. 340B programs at HTCs are allowed to purchase clotting factor for their patients only. Not all HTCs have 340B programs, but may be able to gain access through their affiliated hospital if it is eligible. Eligible hospitals are generally disproportionate share hospitals: they may have a high number of patients who are in Medicaid programs. They may treat specialized disease states like oncology. They may be hospitals located in service areas where they are the only provider, such as suburban, exurban, or especially rural areas where there are not many other hospitals nearby. 340B programs can usually purchase outpatient drugs including biologics such as clotting factor concentrate.



Slide 23: Managed Care Hemophilia Management Strategies: *Narrow Networks*

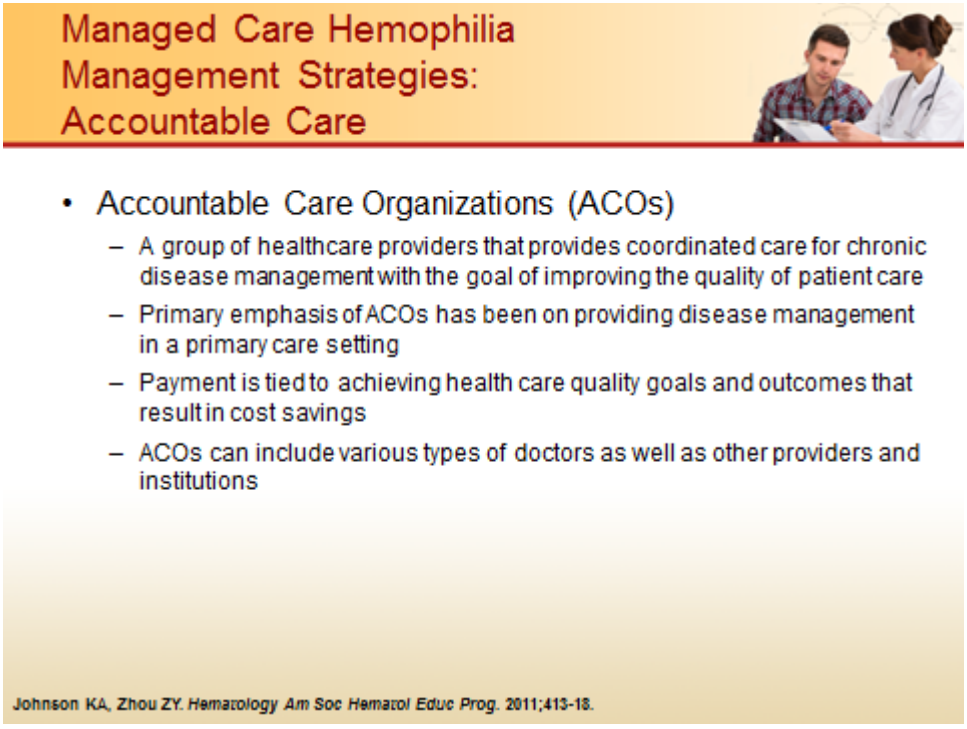
In this program, we have defined the term "narrow networks" to mean networks that do not include all providers in an area. These networks of providers must deliver quality care at the best price. In most cases, patients do not notice the narrow network except when they get to a corner pharmacy and find that the particular pharmacy does not accept their insurance.

Narrow networks can reduce costs by as much as 15% versus traditional plans. The improvement in cost comes because you are able to deliver a certain volume to that provider. Knowing they will receive that volume, they are willing to give a discount on clotting factor products.

In order to be a provider in a narrow network, the provider must demonstrate a certain level of quality. There are various quality measures and performance metrics, including patient outcomes that an in-network provider must meet, whether they are a doctor, hospital, pharmacy, or specialty pharmacy. It is not our goal to save money by sending patients to a provider who cannot provide competent care; it is best practices by quality providers that save money in the long run, so those elements are part of creating a narrow network.

With regard to the narrowing of networks, it is the National Hemophilia Foundation's position on network adequacy that there should be more than one qualified in-network pharmacy provider per health plan.

The expected growth over the next few years of accountable care organizations, or ACOs, and patient-centered medical homes is an extremely important development in hemophilia management. The difference between ACOs and patient centered medical homes is that ACOs tend to be more comprehensive, offering hospitals and many different specialists. They take more risk for each patient as compared to patient-centered medical homes, which tend to be provider-centric, may not involve hospitals, and may provide mostly primary care so they take only partial risk. However, we do see patient-centered medical homes in special circumstances, such as oncology.



Slide 24: Managed Care Hemophilia Management Strategies: *Accountable Care*

An ACO is a group of healthcare providers that provides coordinated care for chronic disease management with the goal of improving the quality of patient care. The primary emphasis of ACOs has traditionally been on providing disease management in a primary care setting. In these

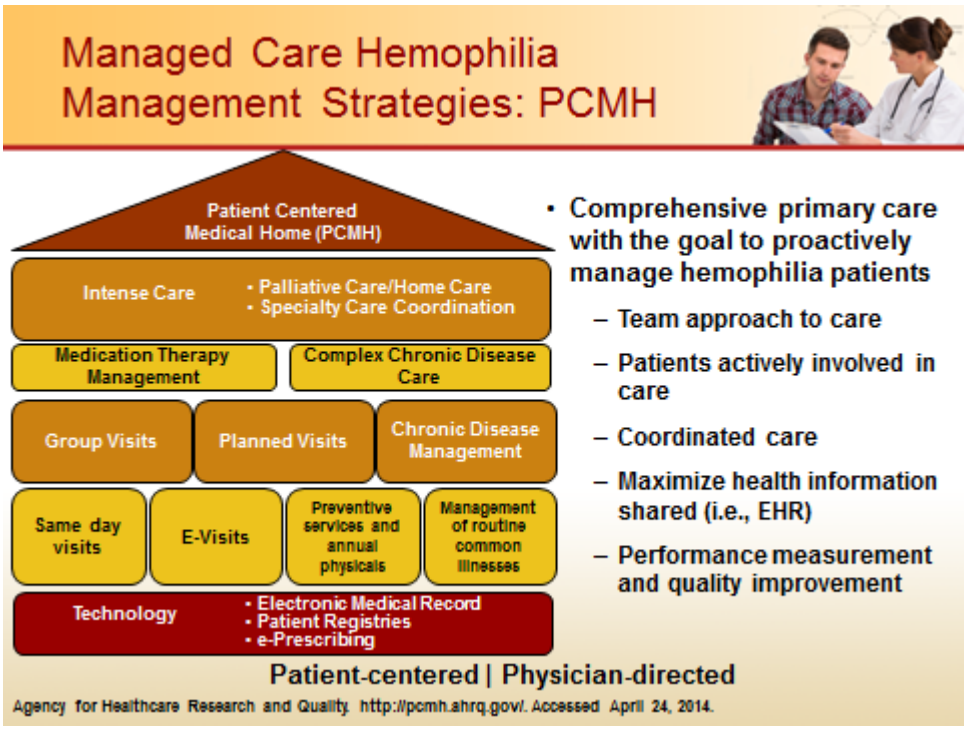
organizations, payment is tied to achieving health care quality goals and outcomes that result in cost savings.

There are two types of ACOs: the Medicare ACO and the commercial ACO. In order to be a Medicare ACO, the entity must meet a number of requirements and provide data to the federal government. Commercial ACOs are not obliged to meet these requirements so long as they are not caring for Medicare patients.

In an ACO, arrangements are typically made between the hospital physician organization and a managed care plan. The risk is shared between the ACO and the managed care plan or health insurer, and then the health insurer may provide various services behind-the-scenes, like case management, claims payment, perhaps reinsurance, etc.

The ACO also has a fairly large amount of risk for an entire population of patients, so how they manage specialty patients such as those with hemophilia will depend on what sort of specialists they have available. If they have specialists who are hemophilia experts or hematologists with training in hemophilia, and they have an adequate number of patients to establish a clinic, then they may do so. Alternatively, they may have just a few hemophilia patients and elect to collaborate with an HTC.

These decisions are entirely at the discretion of the ACO and will not be driven by the health plan. HTCs should consider the option of partnering with a number of ACOs in their catchment area to continue to provide access to care.

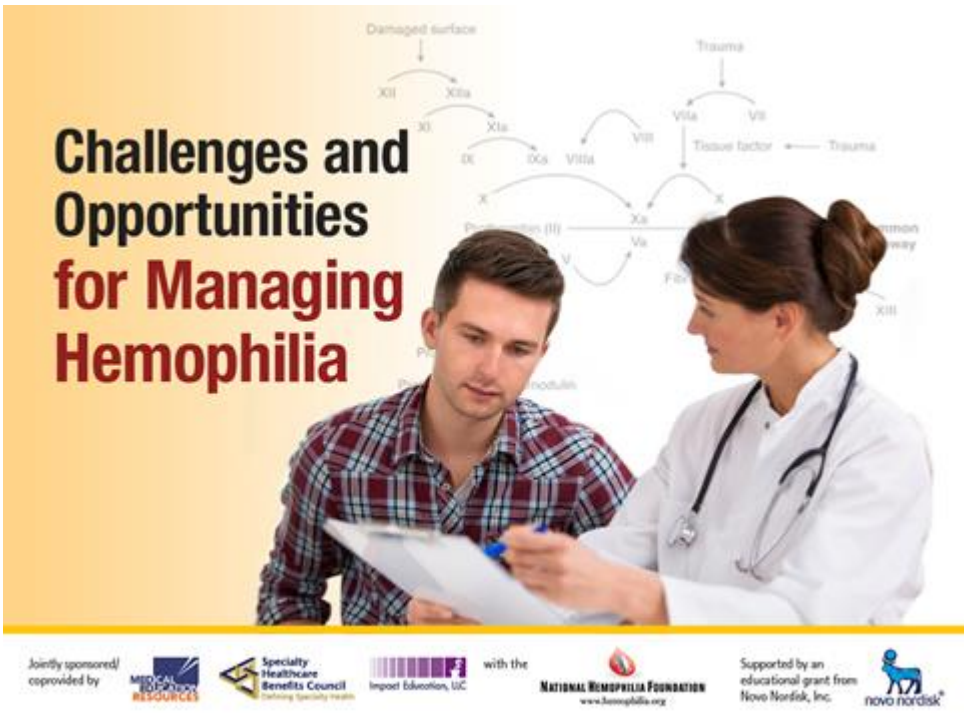


Slide 25: Managed Care Hemophilia Management Strategies: *Patient-Centered Medical Home*

The patient-centered medical home typically provides primary care and sends patients with specialty problems to the appropriate specialist. Thus, we do not often see patients with hemophilia in these settings. Patient-centered medical homes have carve-outs and risk corridors that allow them to move patients with very complex disorders to other sites of care.

Slide 26: Summary

In summary, hemophilia is a low prevalence, high cost disease. Each patient can be managed individually by their providers as well as by the case management staff and others at their payer. Therapeutic advances may lead to better outcomes and quality of life; but can be costly. Due to the potential benefit and in spite of cost, managed care plans want to cover new technologies and treatments. Hemophilia care management requires collaboration and support among providers to enhance treatment outcomes, improve access and remove potential barriers, and enhance patient quality of life. Sharing of data, treatment plans, and benefit designs among the patient, family, different providers, and managed care plan is extremely important. Finally, the managed care plan can deploy various strategies including collaboration with case managers and specialty pharmacies to help control costs while at the same time maintaining access to the medication that patients need.



Slide 27: Challenges and Opportunities for Managing Hemophilia

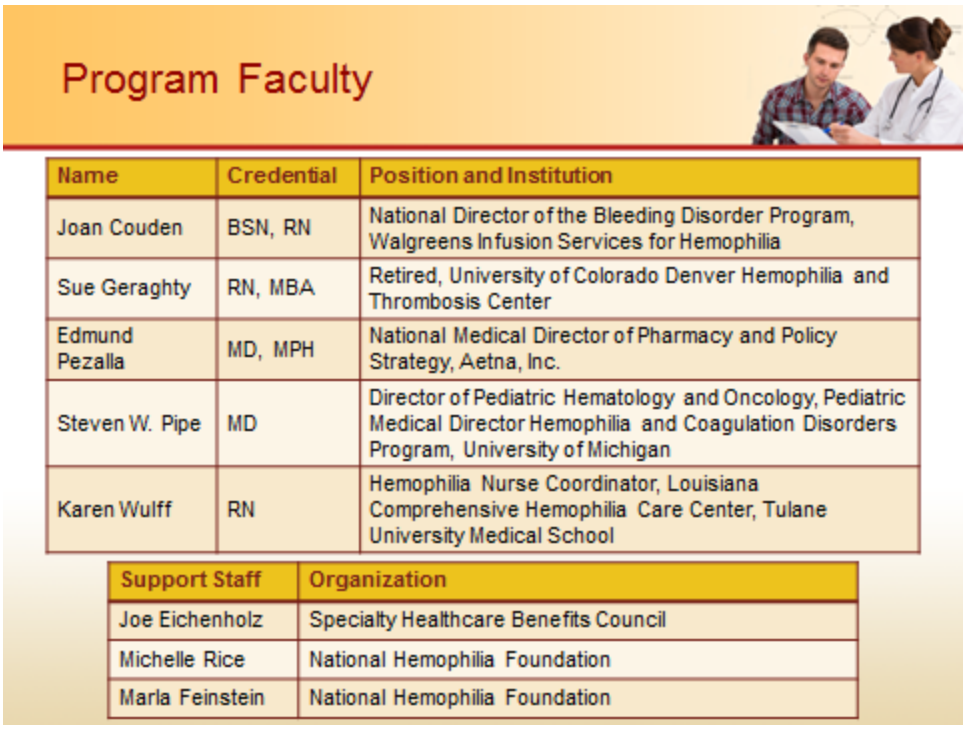
This concludes Program 1 of Track 3, entitled Access, Coverage and Care Strategies for Managed Care and Other Payers.

Program 2:

Track 3 Case Study Challenges and Opportunities

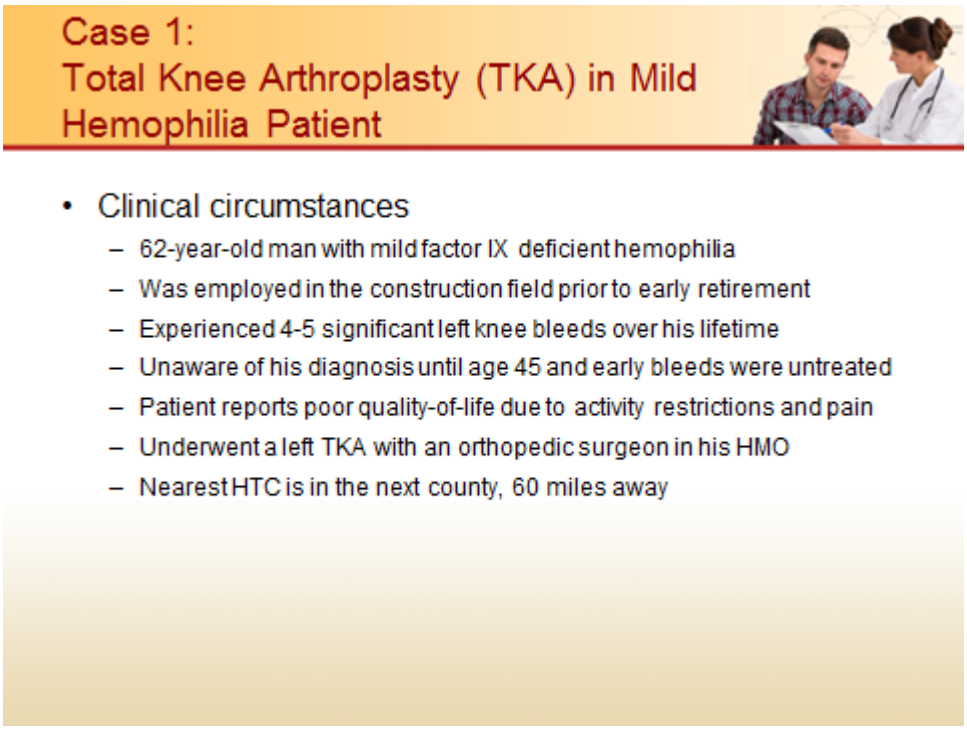
Slide 28: Track 3 Case Studies Challenges and Opportunities

Now that we have had an opportunity to hear about strategies for managed care and other payers, let us move on to examine three case studies.



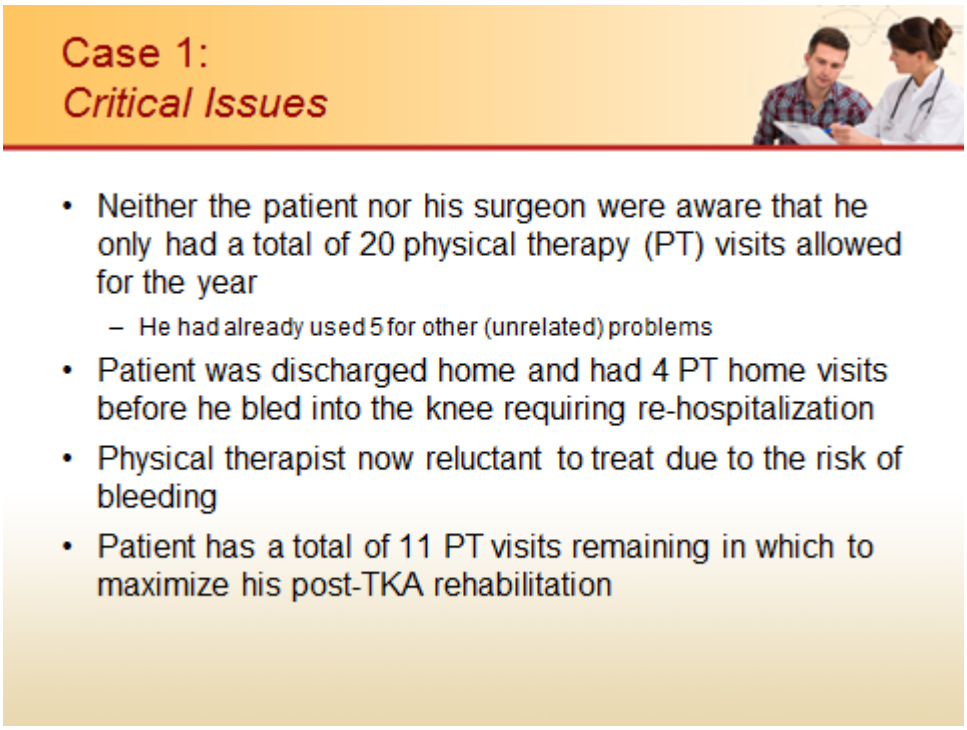
Slide 29: Program Faculty

The faculty for this presentation includes Joan Couden, National Director of the Bleeding Disorder Program for Walgreens Infusion Services for Hemophilia; Sue Geraghty, retired nurse from the University of Colorado Denver Hemophilia and Thrombosis Center; Dr. Edmund Pezalla, National Medical Director of Pharmacy and Policy Strategy at Aetna, Inc.; Dr. Steven Pipe, Director of Pediatric Hematology and Oncology and Pediatric Medical Director of the Hemophilia and Coagulation Disorders Program at University of Michigan; and Karen Wulff, Hemophilia Nurse Coordinator at Louisiana Comprehensive Hemophilia Care Center at Tulane University Medical School. Support staff for this presentation includes Joe Eichenholz, Executive Director of the Specialty Healthcare Benefits Council, and Marla Feinstein and Michelle Rice, from the National Hemophilia Foundation's Public Policy Team.



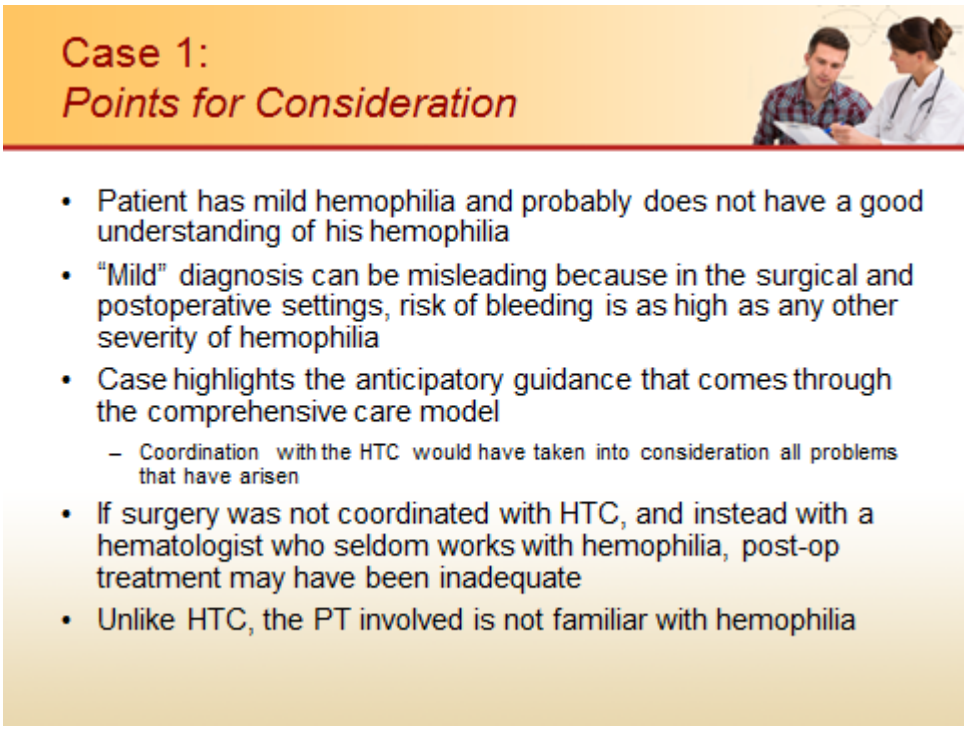
Slide 30: Case 1: ***Total Knee Arthroplasty (TKA) in Mild Hemophilia Patient***

The first case is a 62-year-old male with mild FIX deficiency. He was employed in the construction field prior to early retirement. He experienced 4 to 5 significant left knee bleeds over his lifetime, however, he was undiagnosed until age 45 and his early bleeds were untreated. The patient reports poor quality of life due to activity restrictions and pain. He underwent a total knee arthroplasty, or TKA, with an orthopedic surgeon in his health maintenance organization, or HMO. His nearest Hemophilia Treatment Center, or HTC, is in the next county, 60 miles away.



Slide 31: Case 1: *Critical Issues*

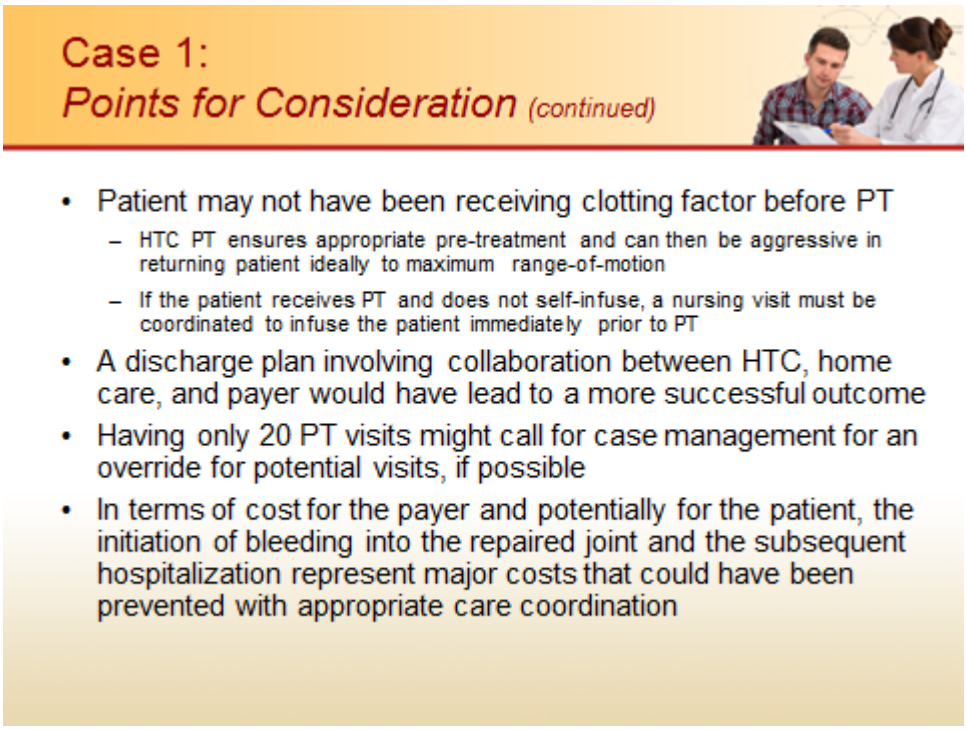
There are some critical issues for this patient. First, neither the patient nor his surgeon were aware that he only had a total of 20 physical therapy, or PT, visits allowed for the year. He had already used 5 for other unrelated problems. Secondly, the patient was discharged home and had 4 PT home visits before he bled into the knee requiring re-hospitalization. Thirdly, PT is now reluctant to treat due to the risk of bleeding. Finally, the patient has a total of 11 PT visits remaining in which to maximize his post-TKA rehabilitation.



Slide 32: Case 1: *Points for Consideration*

This case raises a multitude of points for consideration. The mild diagnosis presents challenges as, for one thing, the patient may not have a good understanding of his hemophilia, and this may be particularly true since he was not diagnosed until age 45. Also, the "mild" diagnosis can be misleading because, in the surgical and postoperative settings, this patient's risk of bleeding is as high as any other severity of hemophilia; thus he deserves the same oversight and care with factor replacement as any other hemophilia patient would require.

This case also highlights the anticipatory guidance that comes through the comprehensive care model featured at HTCs. Coordination with the HTC would have taken into consideration all problems that have arisen in this case. Of utmost importance, if the surgery was not coordinated with the HTC and was instead managed by a hematologist who seldom works with hemophilia, post-operative clotting factor coverage may have been inadequate. Also, unlike the HTC PT, the PT involved in this case is clearly not familiar with hemophilia.



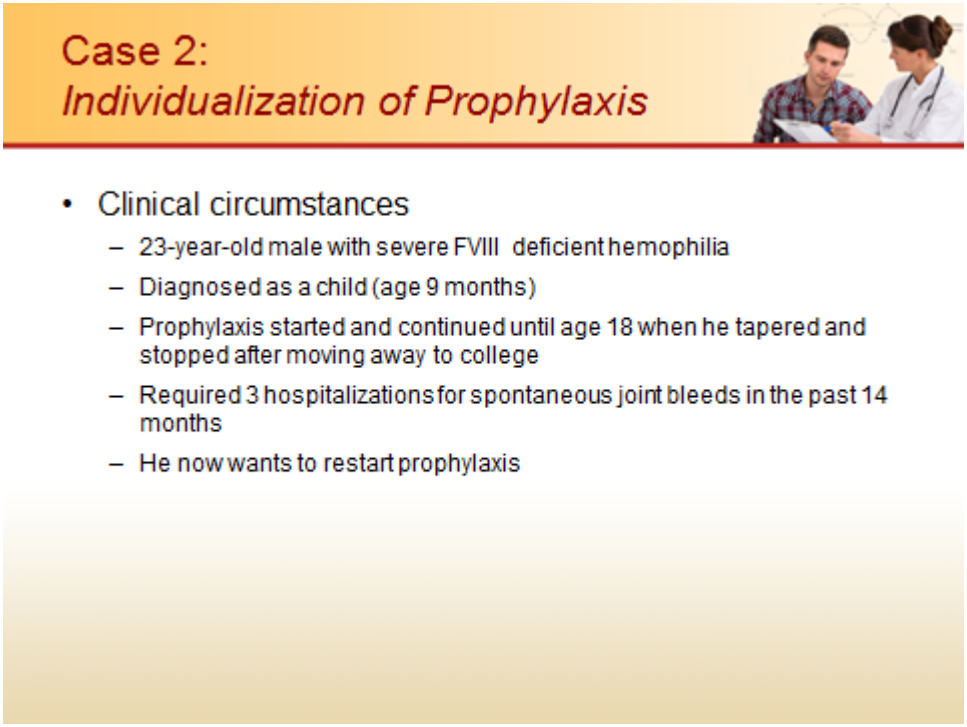
Slide 33: Case 1: *Points for Consideration* *(continued)*

Importantly, the patient may not have received clotting factor before his PT appointments, which may have led to bleeding in the replaced joint. The HTC PT ensures appropriate pre-treatment and can then be aggressive in returning the patient ideally to maximum range-of-motion. If the patient receives PT and does not self-infuse, then a nursing visit must be coordinated to infuse the patient immediately prior to PT. If the patient attends PT at the HTC, he can be infused on-site if the payer covers that service.

This case also highlights the importance of collaboration between the HTC, the home care company, and the payer. Such collaboration would have led to a more successful outcome. For instance, having only 20 PT visits might call for case management for an override for potential visits, if possible. In this case study, such coordination of care was absent.

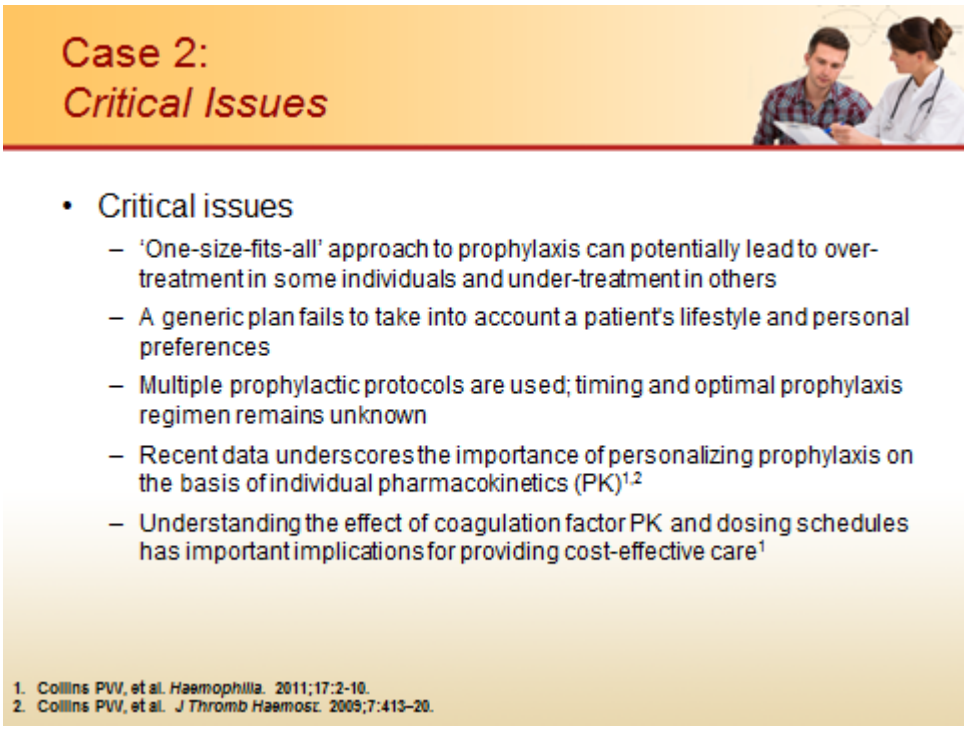
Finally, in terms of cost for the payer and potentially for the patient, the initiation of bleeding into the repaired joint and the subsequent hospitalization represent major costs that could have been prevented with appropriate care coordination. With an appropriate post-operative care plan, the patient may have used daily infusions, continuous infusion, or a long-acting product to

maintain adequate factor levels and prevent bleeding. This case demonstrates how the lack of care coordination can result in negative and costly outcomes for the patient and payer



Slide 34: Case 2: *Individualization of Prophylaxis*

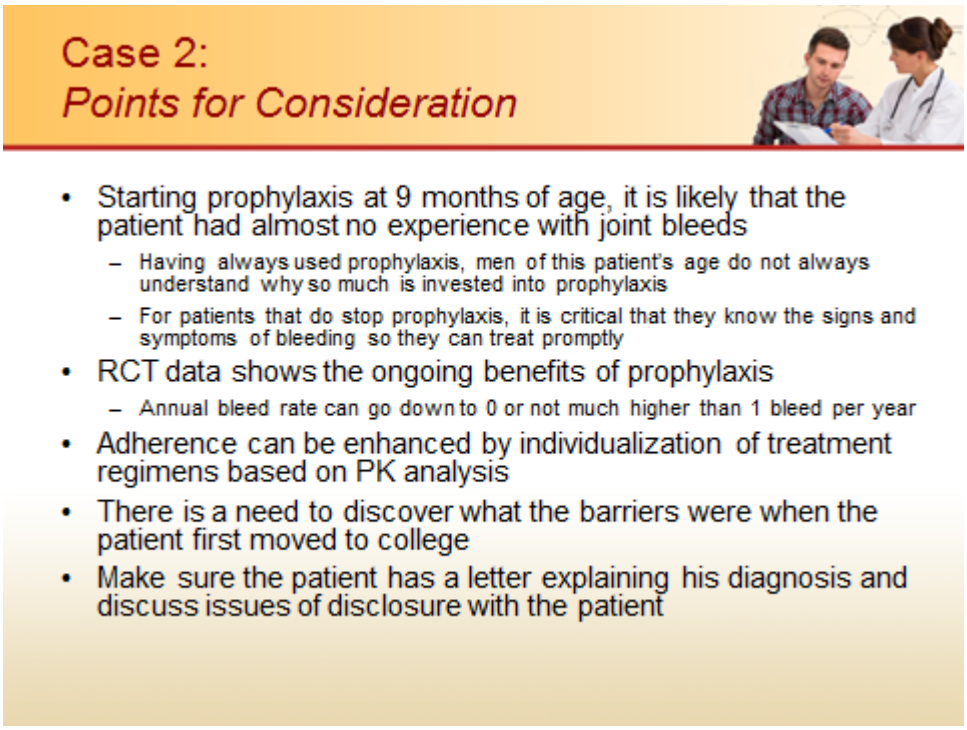
Our second case involves a 23-year-old male with severe FVIII deficient hemophilia who was diagnosed at 9 months of age. He started prophylaxis at that age and continued until age 18 when he tapered and stopped after moving away to college. He subsequently required three hospitalizations for spontaneous joint bleeds in a period of 14 months. He would like to restart prophylaxis.



Slide 35: Case 2: *Critical Issues*

The critical issue here is how to tailor this patient's prophylaxis. A one-size-fits-all approach to prophylaxis can potentially lead to over-treatment in some individuals and under-treatment in others. Also, a generic plan fails to take into account a patient's lifestyle and personal preferences. Patients who are not at home while attending college are going to have a different lifestyle than they had previously while living with their parents and attending high school.

In practice, multiple prophylactic protocols are used, though timing and the optimal prophylaxis regimen remains unknown. Recent data from Collins et al. underscores the importance of personalizing prophylaxis on the basis of individual pharmacokinetics, or PK. Understanding the effect of coagulation factor PK and dosing schedules also has important implications for providing cost-effective care.



Slide 36: Case 2: *Points for Consideration*

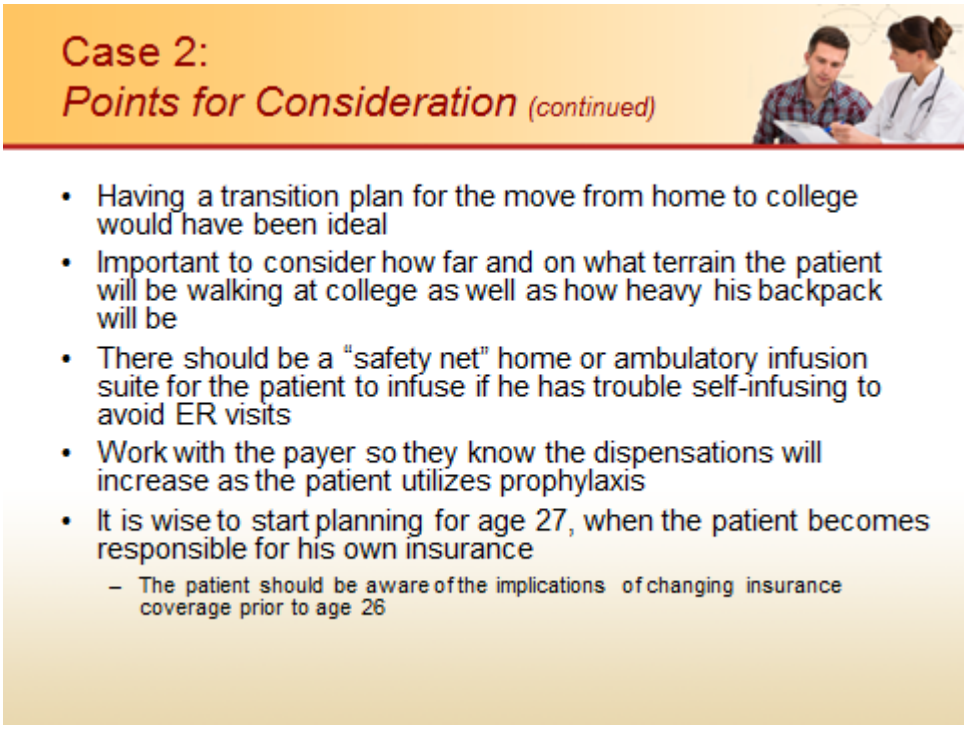
Starting prophylaxis at 9 months of age, it is likely that the patient has had almost no experience with joint bleeds. This can be a blessing and a curse. Having always used prophylaxis, men of this patient's age do not always understand why so much is invested in prophylaxis. It is to this patient's advantage that his experience with bleeding has given him the motivation to reinstitute prophylaxis.

For patients that do stop prophylaxis, it is critical that they know the signs and symptoms of bleeding so they can treat promptly.

We now have randomized control trial, or RCT, data that definitively shows the ongoing benefits of prophylaxis into adulthood. Annual bleed rates can actually go down to 0 or not much higher than 1 bleed per year with effective prophylaxis. Treatment adherence can be enhanced by individualization of treatment regimens based on PK analysis of that patient, looking for an optimal dose and interval regimen to meet his needs. The differences in half-life for FVIII deficient patients are about four-fold, meaning half-life can be as short as 6 hours or as long as 24 hours with the same product. Thus, there is a strong rationale for understanding the individual

PK and devising a dose and interval regimen that has been tailored to them and their activity. This would aid in getting the patient's buy-in and improving his adherence.

There is also a need to discover where the barriers were when the patient first moved to college. For many patients, issues of disclosure to college roommates and other students may be a barrier to sustaining prophylaxis. The HTC can make sure the patient has a letter explaining his diagnosis and can also discuss issues of disclosure ahead of the transition to college to prepare the student for this.



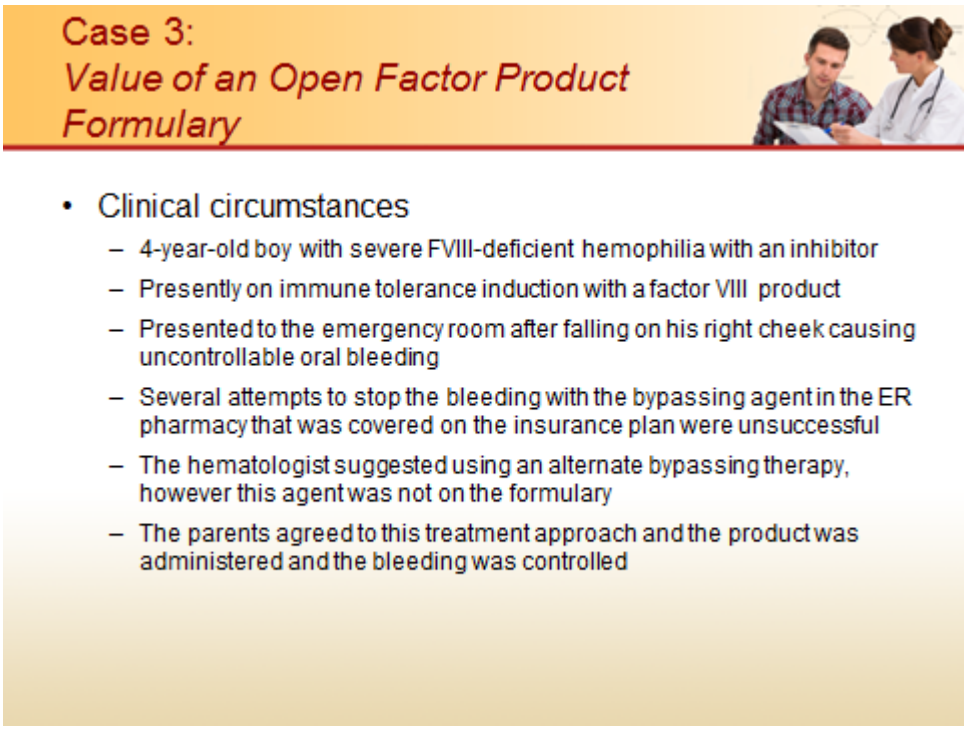
Slide 37: Case 2: *Points for Consideration* *(continued)*

This brings us to the idea of a transition plan. As discussed in Track 1, it is recommended that HTCs have multidisciplinary transition programs aimed at adolescents and young adults, and that special care coordination is provided for those with a major life change, such as going to college. One consideration in such a program is the question of how far and on what terrain the patient will be walking at college as well as how heavy his backpack will be. This may be a variable in determining the patient's prophylaxis dose or interval.

As the patient transitions he may become independent in self-infusion rather quickly. However, it may be helpful for the specialty pharmacy provider to provide a "safety net" home resource or ambulatory suite for the patient to get an infusion if he has trouble self-infusing. This would prevent costly emergency room visits.

In terms of cost, the specialty pharmacy provider and HTC should coordinate to notify the payer that clotting factor dispensations will increase as the patient utilizes prophylaxis.

Finally, it would be wise for this patient to begin planning for age 27, when he becomes responsible for his own insurance coverage. The patient should also be aware of the implications of changing insurance coverage prior to age 26. For instance, some patients will take the insurance from their college. On these plans, coverage may not be provided during summer months. Other students may choose to get a plan on the health exchange. Many of these plans are very high deductible plans. These are important considerations that must be made.



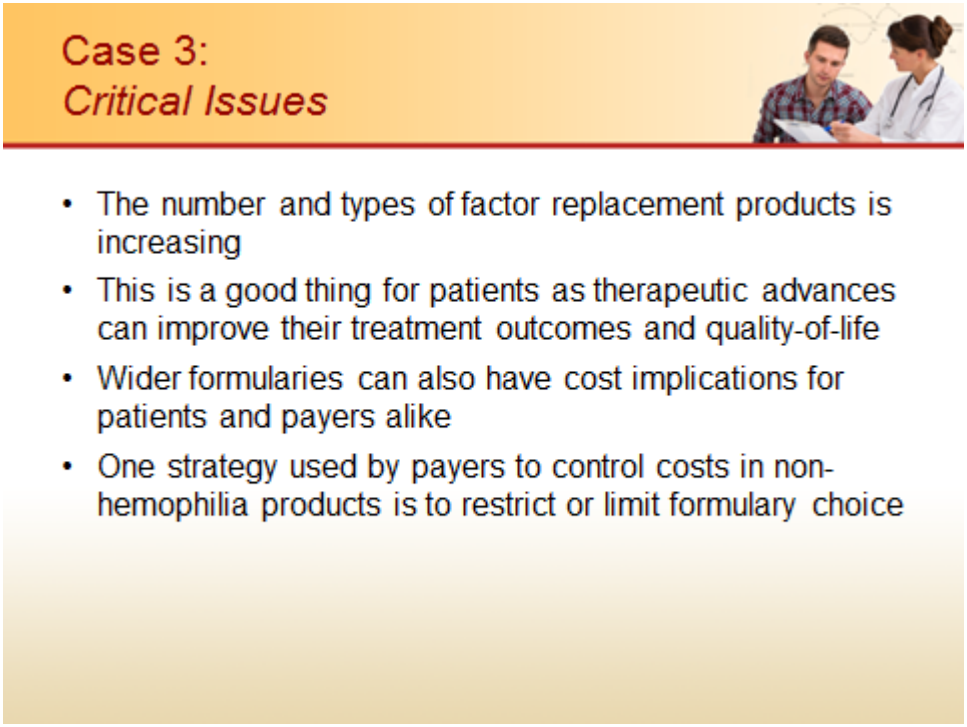
Slide 38: Case 3: *Value of an Open Factor Product Formulary*

This case involves the value of prompt access to clotting factor concentrate. A 4-year-old boy with severe FVIII deficiency with an inhibitor is presently on immune tolerance induction, or ITI, with a FVIII product. With ITI, the patient is getting regular high-dose infusions of FVIII trying to induce tolerance to FVIII so that it has a hemostatic effect. The FVIII treatment that the patient receives for ITI is inadequate to manage bleeding, so the patient needs a bypassing agent as an alternative hemostatic agent.

He presented to the emergency room after falling on his right cheek, causing uncontrollable oral bleeding. An attempt was made to stop the bleeding using the bypassing agent that is authorized by the child's insurance and available in the hospital pharmacy. Because the first bypassing agent did not work to stop the bleeding, the hematologist suggested using an alternate bypassing therapy; however this agent was not on the patient's formulary. The parents agreed to this treatment approach, the product was administered, and the bleeding was controlled.

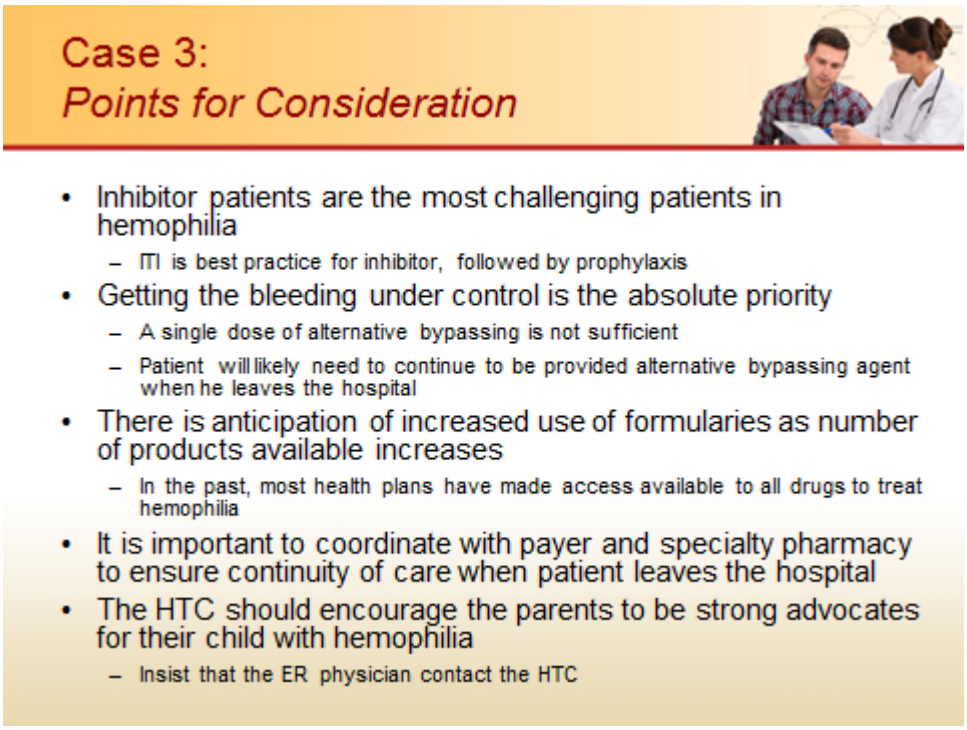
In general, patients are likely to use one particular bypassing agent, whether it is FEIBA® or NovoSeven®. Even though these are both in the same class of therapy called bypassing agents,

they have completely different mechanisms of action and they can be complementary in individual cases.



Slide 39: Case 3: *Critical Issues*

There are several critical issues in this case and these should be considered not just for the emergency room, but for HTCs and hospitals as well. First, let us note that the number and types of factor replacement products is increasing. The proliferation of factor replacement products is a good thing for the patient as therapeutic advances can improve their treatment outcomes and quality of life; however, wider formularies may have cost implications for patients and payers alike. One strategy used by payers to control costs in non-hemophilia products is to restrict or limit formulary choice. With the increasing number of replacement products available, there may be more formularies and more utilization management controls from payers in an attempt to steer patients towards what for them will be the most cost-effective treatment.



Slide 40: Case 3: *Points for Consideration*

Patients with inhibitors are, without a doubt, the most challenging patients that HTCs care for. This boy is on immune tolerance induction, or ITI, which is considered best practice for patients with an active inhibitor. What is becoming a secondary best practice is to use prophylaxis with bypassing agents. That has been most clearly demonstrated with FEIBA® in two randomized controlled trials applied in this setting with quite good results, showing protection from bleeding that is much better than just using a reactive or on-demand approach to bleeding in the inhibitor patients.

In any situation where bleeding was not controlled with the bypassing agent the patient was prescribed, the most appropriate thing would be to try the alternative agent. There is no question that the faster bleeds are controlled in inhibitor patients, the better overall outcome there will be.

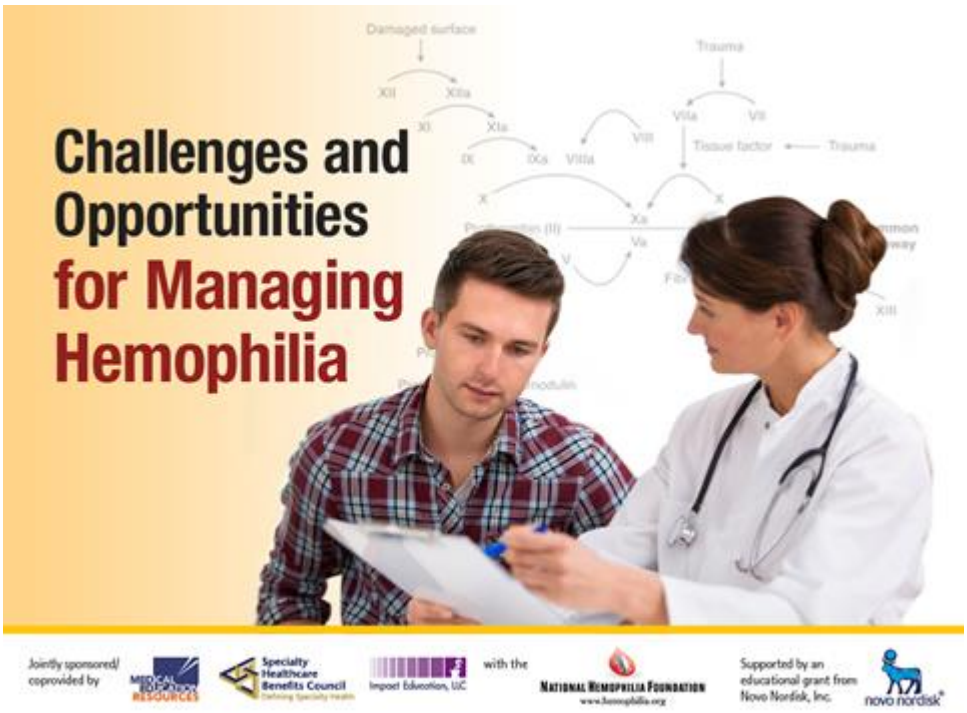
It is detrimental to have any impediments to this child getting the agent that is prescribed by the physician. There is no doubt that for this particular patient, a single dose is not going to be sufficient, and he is going to need continued dosing in the home setting. Removing barriers to the patient continuing the alternative agent in the home after leaving the acute care setting is a

priority. As far as minimizing costs, in this case, the critical point is to get the bleeding under control and get the patient back to the home setting.

A major concern is the increased use of formularies now with additional products coming onto the market. In the past, most health plans have made access available to all drugs to treat hemophilia.

It is also important for the HTC or hematologist to coordinate with the payer and specialty pharmacy to ensure continuity of care when the patient leaves the hospital. While there is a need to attempt to obtain an override for coverage for the bypassing agent, it is also important that the patient does not leave the hospital using a different product than prescribed by the HTC. This emphasizes the need for there to be a clear process regarding who physicians should call to get prior authorization and to ensure everything is in line prior to hospital discharge.

Finally, from the time the child with hemophilia is born, the HTC should encourage the parents to be strong advocates on his behalf. In this case, the parents should insist that the emergency room physician contact the HTC prior to any treatment or as soon as possible once reasonable emergency measures have been implemented.



Slide 41: Challenges and Opportunities for Managing Hemophilia

This concludes Track 3, Access, Coverage, and Care Issues, of the Challenges and Opportunities for Managing Hemophilia 2014 Continuing Education Series. Please visit the Specialty Healthcare Benefits Council at www.SHBC.us to complete the Track 3 post-test and evaluation and receive the appropriate amount of continuing education credit hours. Thank you for your time and attention.