AMCP Webinar
Market Insights
Future Treatments in Hemophilia

AMCP

Optimizing medicine.
Improving lives.

Guest Speaker

Michelle Rice

Chief External Affairs Officer

National Hemophilia Foundation

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

eye for pharma
AWARDS 2019
Dec 11, 2019 | Bellevue Hotel, Philadelphia

FINALIST

Patient Champion Award: Patient Advocate

National Hemophilia Foundation

Mission Statement

The National Hemophilia Foundation is dedicated to finding the cures for inherited bleeding disorders and to preventing and treating the complications of these disorders—through education, advocacy, and research.

CCSC: A Hemophilia Quality Improvement and Cost Management Initiative Sponsored by NHF

- Formed in 2014 with a prominent group of HTC directors, clinicians, and administrators along with payer/managed care medical and pharmacy directors
- CCSC's vision is to augment the sustainability of HTCs through the following:

Comprehensive Care Sustainability Collaborative
National Hemophilia Foundation

- Overcome communication gaps to increase connectivity between payers, purchasers, and providers
- Broaden access to extensive hemophilia-related outcomes data from HTCs
- Disseminate rigorous standards of care, quality, and cost management for hemophilia
- Improve the recognition of HTC-derived value
- Obtain advanced analytics on a rare, high-cost disease and gain insight on actionable best practices

Rationale for the CCSC Initiative

HTC

NHF has long since recognized the HTC integrated comprehensive care model as the gold standard of clinical management for patients with rare, chronic bleeding or clotting disorders

340B

Many HTCs depend on 340B program revenue from the sale of clotting factor replacement products to maintain and grow integrated care programs

SPP

HTCs are challenged to access specialty pharmacy contracts due to current trends:

- Factor coverage is increasingly being shifted from the medical to the pharmacy benefit
- Pharmacy benefit managers (PBMs) are acquiring their own specialty pharmacies (SPs) and encouraging plan sponsors to allow the SPs to become the exclusive provider of specialty drugs

CCSC Bridges the Communication Gap Between Providers and Payers/Purchasers

HTCs

Advocacy

Outreach

Health Plans

Employers

How CCSC is Helping HTCs Address Market Trends

Value-Based Contracting	• Provide data demonstrating improved outcomes and lower costs
Copay Accumulator Adjustment Programs	• Leverage CCSC payer education materials and outreach
Gene Therapy	• Become centers of excellence for the administration and follow-up of gene therapy

AMCP Market Insights- Future in Hemophilia Treatment Executive Summary

Market Insights Meeting Overview

- Clinical Overview: Hemophilia A—Evolving Role of Factor
- Clinical Overview: Hemophilia A—Inhibitor Management
- Clinical Overview: Emergence of NonFactor Treatments
- On the Horizon—Pipeline and Gene Therapy
- Employer Perspective- Self Insured and Reinsurance
- Impact of New Treatments on Delivery of Care
- Partnering in Hemophilia

Expert Presenter:
Tammuella Singleton, MD
Assistant Professor of Pediatrics
Department of Pediatrics
Children's Hospital
New Orleans, LA

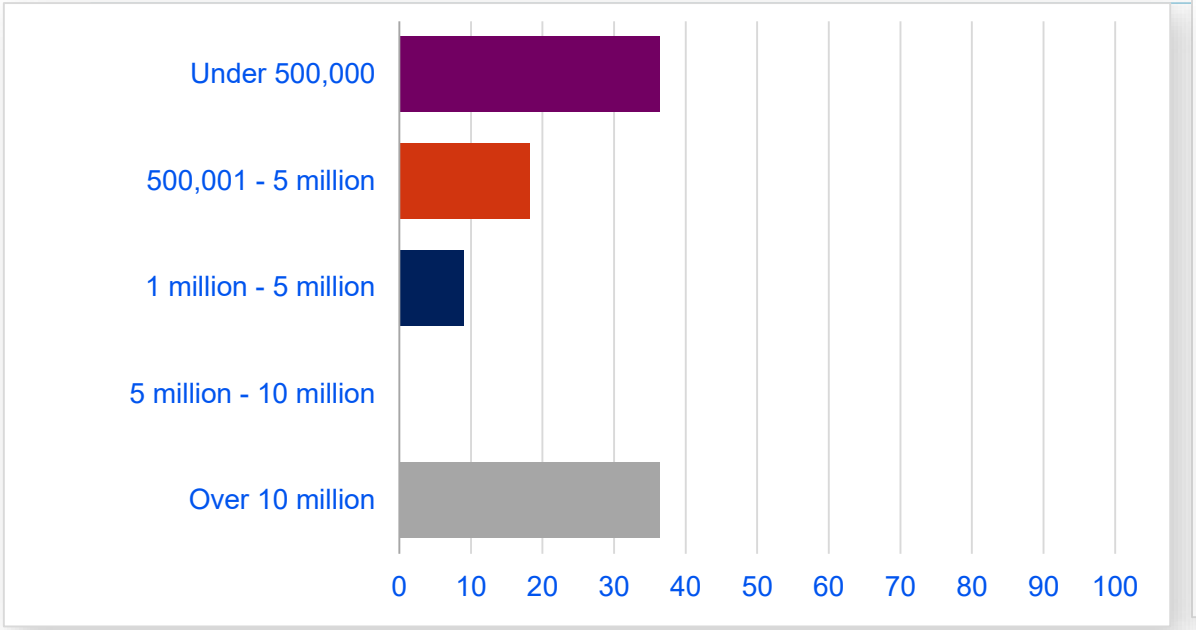
Expert Presenter:
Michael Baldzicki
Executive Vice President, Growth & Strategy
AscellaHealth

Objectives

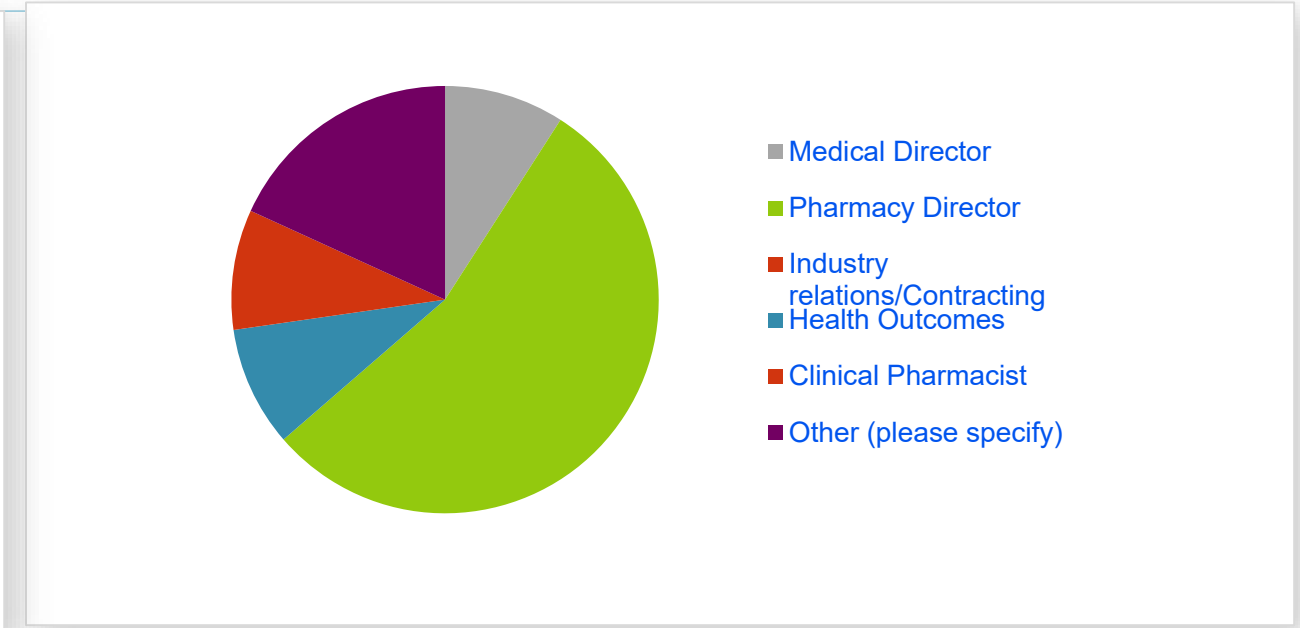
- Collect Insights to help payers manage access and product utilization amidst the introduction of new hemophilia treatments
- Define the role of factor replacement, emicizumab and future gene therapies in patient outcomes
- Understand changing business models for care management as non-factor product use increases
- Collect and disseminate best practices for evaluating hemophilia outcomes and value of specific therapies and interventions

Methodology

- 7-hour live meeting on November 1st at 2019 AMCP NEXUS, National Harbor, MD
- Roundtable format, with presentations and group discussion



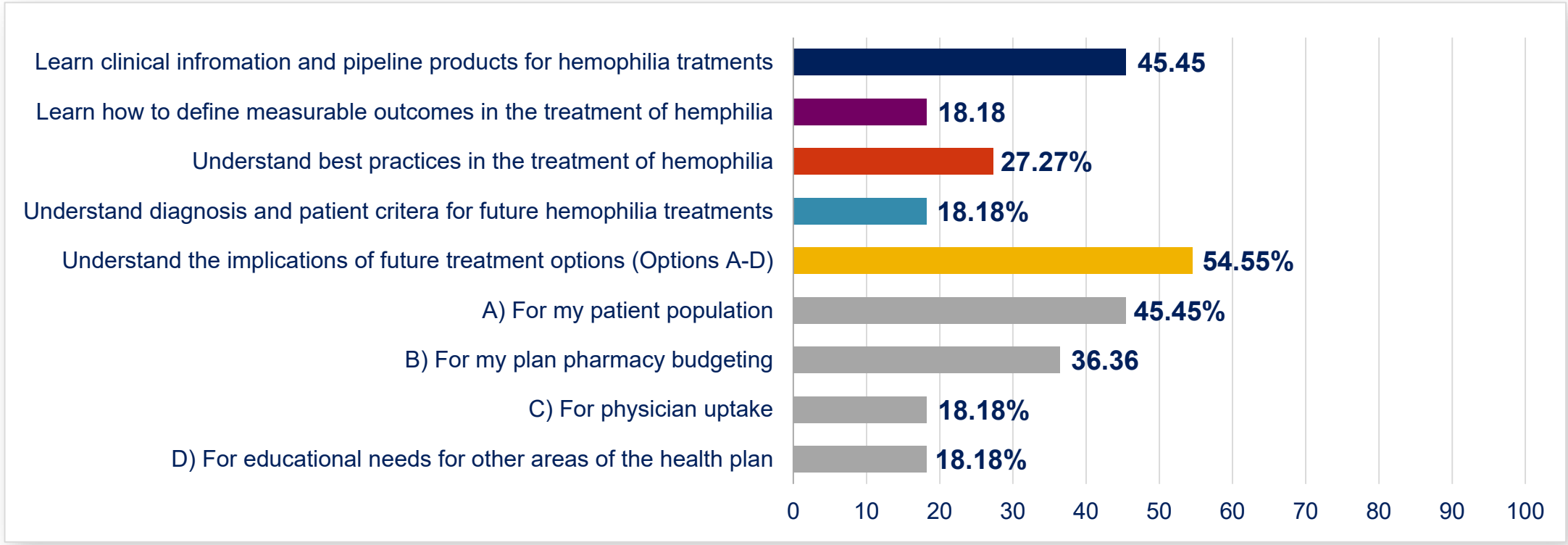
Lives covered ranged from 135,000 to 35 million.



Pharmacy directors, medical directors, clinical pharmacist, Hemophilia Alliance, Specialty Pharmacy, National Hemophilia Foundation

Participant Informational Needs

With respect to the upcoming AMCP Market Insights program on hemophilia, what are your key objectives in attending this program? (Select all that apply)



Payers identified numerous needs for increased knowledge about hemophilia

Key Questions from Participants

- How do we integrate gene therapy within existing benefit structures, and how do you integrate it functionally?
- How do you manage costs and patient expectations?
- How do you manage emicizumab and other products in the pipeline, and how will they all fit together?
- How can all stakeholders collaborate?

"I hope to gain a better understanding of how collectively everyone is going to approach the shift in management of hemophilia."

"What's the role of all of the different players, and how does that change with this moving into the gene therapy realm?"

"There's just no standard of care in what you're supposed to do in hemophilia, which makes it difficult."

Participant Perspectives on Factor Utilization Management

Payers-identified issues

- ✓ Factor usage and spend
- ✓ Many use PA for extended half-life products
- ✓ High levels of month-to-month variability makes utilization unpredictable
- ✓ Starting to look at pharmaco-kinetics for dosing
- ✓ Majority monitor wastage through assay management
- ✓ Many participants can link and analyze pharmacy and medical claims
- ✓ Participants may adjust plan decisions as new products enter the market
- ✓ There are no established standards of care

Evolving Issues With Factor Replacement Treatment

Treatment must be individualized based on specific patient characteristics

Issues impacting factor use

- Patients can have the same genotype but different phenotypes in severity of bleeding
- Early and appropriate treatment of each bleeding episode is critical to minimize complications
- Consider impact of weight on dosing on amount of replacement required
- Early refills may be appropriate, especially after bleeding episodes
- Bleeding episodes can result in significant and permanent consequences for patients—treatment goal should be no bleeds
- Products are not interchangeable- not a lot of data for switching

Who should receive prophylaxis

- All severe patients are candidates for prophylaxis but not all receive it
- Self-infusion with factor is a goal, but access can pose challenges, particularly for young patients and those with psychosocial issues
- Risk of microbleeds resulting in joint damage must be considered for mild and moderate patients
- Typically don't start prophylaxis until after a severe hemorrhage, a joint bleed, or certain other situations, such as intercranial hemorrhage

"90% of those with severe hemophilia have chronic degenerative changes due to recurrent hemarthrosis in at least one joint by age 25."

Prior Authorization and Factor Use

Issues to Consider

- Most PAs follow dosing provided in package inserts- when possible
- Patient type, severity, inhibitors, body weight are considered
- Total factor use and variations in dosing based on individual patient factors

Factor use is highly variable

- Varies from month to month based on frequency of bleeds, prophylactic use
  - Even patients with mild disease may experience a severe bleed
- Consider impact of factor use on medical utilization and ability to review total cost of care
- Most plans cover all products; most HTCs purchase all products to individualize

Category/ utilization review processes

- Annually, quarterly, or monthly
- As part of specialty pharmacy category review
- Assay management reviews

"When we're starting to do any type of budget predictions for the next year, we do look at the factor utilization and if we've had any significant changes in that trend. And we actually look at it on a monthly basis."

Monitoring and Reporting

Real world evidence

- Can be a challenge for payers to manage data
  - Few data are currently available
- Inventory management most common - not much outcome-based analysis available
- Quality of life issues are most common e.g., activity level, psychosocial issues
- Limited ability to separate patients by characteristics (e.g., severity, age, bleeds)

Reporting requirements

- Monitor bleeds
- Behavioral health components of patient care
- Pharmacy reporting of dispensing information- may not match prescribing
- HTC reporting—share various levels of data with plans- however challenges remain

Digital strategies

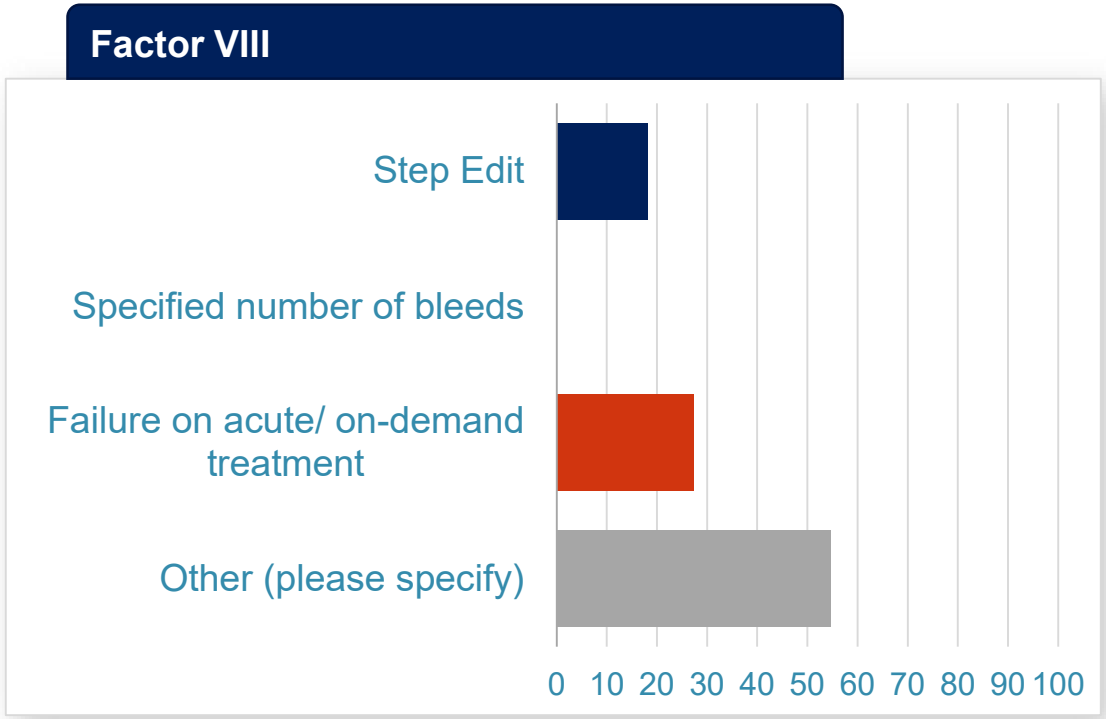
- Can be used to collect data
- Some programs have proprietary software for tracking outcomes
  - Can review dispensing history
  - Patient reported outcomes

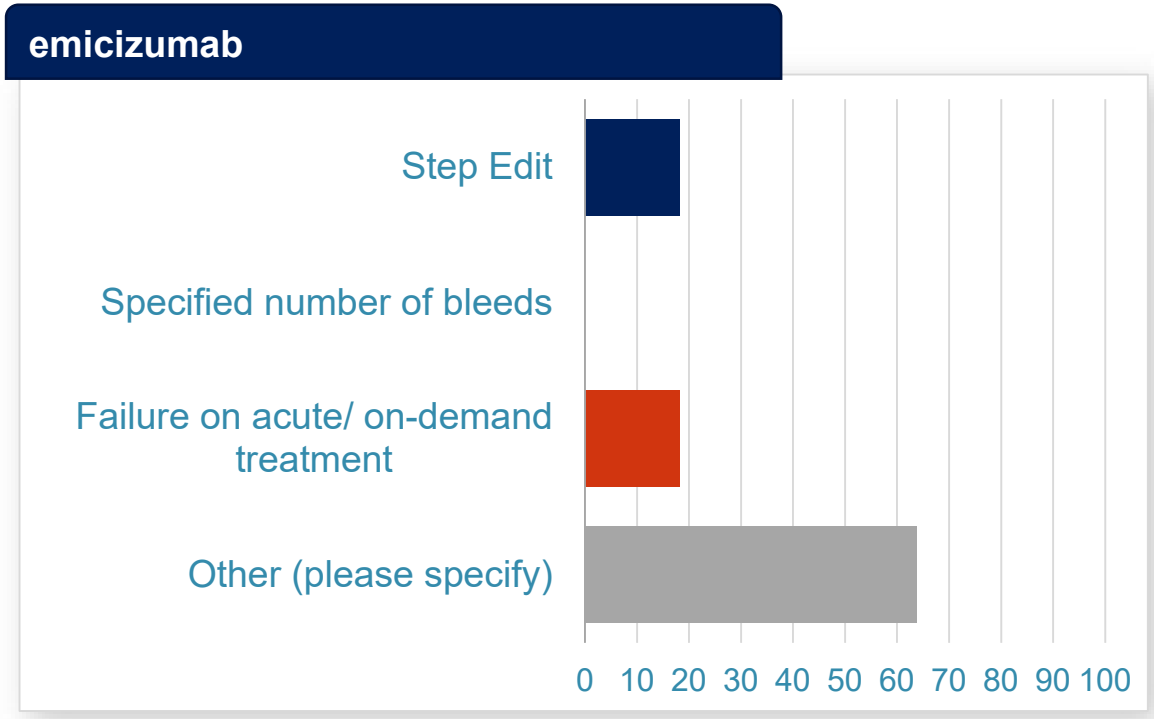
Factor Site of Care Implications

- Many patients receive care through HTCs, other in outpatient treatment centers that also treat benign hematology
- Many patients store factor at home for self-administration—concerns of stockpiling remain
  - Some specialty pharmacies still auto-ship product
  - Patients have reasonable fear about ensuring they have products on hand
  - Should a patient have multiple types of Factor (SA and EHL) at home?
- Impact of 340b pricing on hemophilia treatment centers and ability to provide supportive services
  - Concern about the shift to specialty pharmacies and/or non-factor treatment may impact quality of care and HTC revenue streams
- Patients are encouraged to bring factor with them to ER
- Goal is to optimize treatment regimens and avoid wastage

"There's so much variance in care, it's hard to have management strategies."
"There is no standard of care of what exactly you are supposed to be doing"

Other than confirmation of diagnosis which, if any, restrictions does your organization have on access to products for prevention of bleeds in members with hemophilia? (select all that apply)





On concomitant use of emicizumab with a Factor-replacement therapy?

No participant had any policy for concomitant use, but were positive about concomitant use, as it confirmed to them that bleeds were being treated

emicizumab and Inhibitor Considerations

Inhibitor development

- Inhibitors may develop more frequently with recombinant products
  - SIPPET trial: recombinant FVIII associated with an 87% higher incidence of inhibitors)
- Immunotolerance therapy is an option to try to eradicate the inhibitor
  - 60% to 70% effectiveness; high cost

Consider use of emicizumab initially to avoid inhibitor development

- Can start emicizumab in newborns
- Can use emicizumab in patients with inhibitors
- May be more appropriate for highly active patients

““emicizumab changes the question about whether to do immunotolerance therapy.”

Possible emicizumab PA requirements

- Documentation of hemophilia diagnosis
- Why they are not a candidate for factor, or
- Why emicizumab is needed with factor

Products on the Horizon

Fitusiran (ALN-AT3)

SC-administered small interfering RNA (siRNA) therapeutic targeting antithrombin (AT)
In phase 3 trials

Concizumab mAB 2021

High affinity monoclonal humanized antibody specific to the KPI-2 domain of TFPI – binds all forms of TFPI in blood and cell bound TFPI
In phase 3 trials

Gene Therapy

Potential approvals in 2020, several additional products in phase 1, 2, 3 trials

Considerations Regarding Gene Therapy

Unmet needs addressed

- Steady, ongoing concentrations of factor
- Reduction or elimination of spontaneous bleeds
- Reduction or elimination of dependence on frequent infusions

Potential Limitations

- Not all Hemophilia A patients will be candidates or will want to receive gene therapy
- Still need treatment for bleeds
- There are viable options for treating patients now
- Patients who receive gene therapy may not be cured in the sense that they may still need treatment with factor under certain conditions
  - Trauma, surgery
- Treatment will not reverse joint damage
- Durability of treatment remains unknown; will patients require more than one treatment? Will antibodies develop?

Patient candidates

- On prophylaxis but having difficulty optimizing
- Able to participate in significant follow up
- BMI? Re-dose?

How will Gene Therapy change Hemophilia A category management?

- Concern about durability of effect
- Also from a patient management perspective, gene therapy was noted as creating a lot of “mild patients with hemophilia”, as such will still have to manage bleeds and be prepared

Financial Implications of Gene Therapy and the Potential for Improved Outcomes and Reduced Health Care Service Utilization

- The specialty drug trend continues to outpace that of traditional pharmaceuticals and remains a key priority of payer management
- Gene therapy forecasts demonstrate a significant cost impact on the specialty trend, including in hemophilia
- Value in health care innovation lies in the result of the innovation rather than the innovation itself
- The juxtaposed needs and concerns of payers, providers, and patients must all be carefully weighed when evaluating the role and coverage of gene therapy in future care interventions
- Consider impact of newly-approved treatments on underwriting assumptions

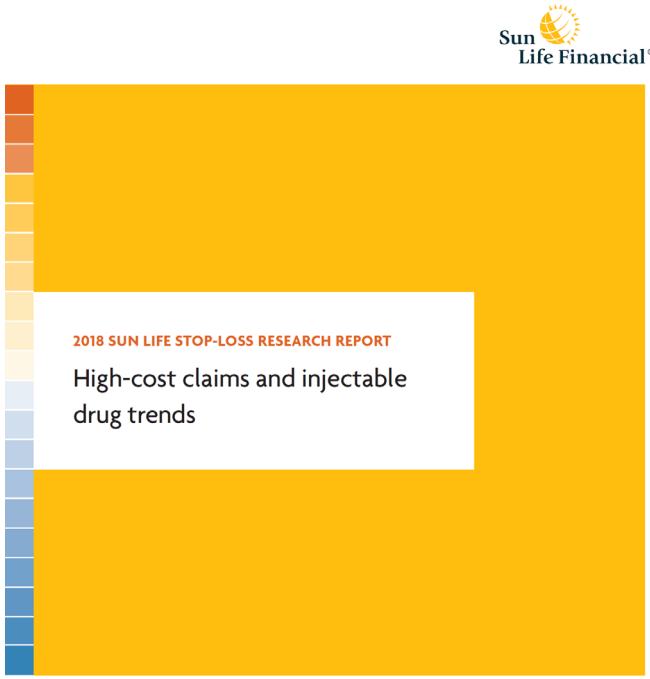
Proposed Payment Models Aligned with Appropriate Use for Hemophilia Gene Therapy

- The anticipated high cost of gene therapy, in addition to the potential for patient migration between health plans, necessitates innovative payment models
  - Consider potential impact of gene therapy cost on access to other treatments
- Potential strategies
  - Outcomes-Based Agreements
    - Consider need for collection and tracking of real-world evidence
    - Gene-therapy manufacturer becomes responsible for other treatment costs (e.g., any necessary Factor costs) within a defined time period
  - Alternative Payment Models: Annuities and/or Risk Pools
    - Some reinsurance providers laser hemophilia
- New types of provider entities are likely to emerge
- The eventual choice of innovative access scheme will ultimately depend on individual health plan environment and characteristics

"There has to be alignment on risk."

Considerations Reinsurance/Stop Loss Payment Options

- Secondary insurers assume financial risk above a specific threshold
  - On average, self insured organizations cover the first $300,000 of a specific claim
- Stop Loss Carriers have realized a significant increase in catastrophic claims, or those above $1M
  - Patients with claims of more than $1 million represented only 2% of the total number of stop-loss claims from 2014 to 2017, but roughly 20%, or nearly $600 million, of the total $3 billion in stop-loss reimbursement
  - In 2018, hemophilia resulted in $67.9 million in stop-loss claim reimbursements
- Stop Loss Carriers utilize several techniques to minimize their risk in high dollar cases which ultimately adversely affect the plan sponsors
  - Techniques may limit access for patients with hemophilia
- Because the drug treatment protocols for conditions like hemophilia are more maintenance-like than curative, those $10 million to $20 million claims are becoming annuities



2019 NHF Goals to Address Gene Therapy

- Community education
- Relationship building – rare disease organizations
- Increase knowledge of the science of gene therapy
- Raising the profile of NHF as an important voice in the rare disease and policy and regulatory space

Educating the Community on Gene Therapy

- Established an External Working Group
  - 4 HTC physicians, 2 patients, 1 caregiver and 1 social worker
- Frequently Asked Questions (FAQs)
- In-depth lexicon of gene therapy terms
- *All About Gene Therapy* Video
- Website strategy outlined
- 3 Sessions at NHF's Bleeding Disorders Conference on Gen Innovative Therapy
  - Multiple sessions in provider track
- Gene Therapy Summit held in November 2019

insights
INNOVATIONS IN TREATMENT

National Hemophilia Foundation
for all bleeding disorders

FREQUENTLY ASKED QUESTIONS ON GENE THERAPY

GENE THERAPY DEFINED:

What is gene therapy?
Gene therapy is designed to introduce genetic material into cells to compensate for abnormal genes or to make a beneficial protein. If a mutated gene causes a necessary protein to be faulty or missing, gene therapy may be able to introduce a normal copy of the gene to restore the function of the protein. https://ghr.nlm.nih.gov/primer/therapy/procedures

What is a vector? Are there different types of vectors?
A vector is a carrier; the DNA of an agent (virus of plasmid) used to transfer the desired gene to a cell. There are a variety of different types of vectors currently used in gene therapy including retroviruses, adenoviruses, adeno-associated viruses & the herpes simplex virus.

What are the differences among the various approaches to gene therapy: gene editing, gene transfer and cell therapy? Gene therapy involves the transfer of genetic material utilizing a vector. Cell therapy refers to the transfers of cells with the necessary function into the patient. Gene editing targets a specific gene in order to correct its mutation. For more information please go to: https://www.asgct.org/education

What is CRISPR?
Clustered Regularly Interspaced Short Palindromic Repeats is a term used to describe a gene editing technique that can identify and modify specific DNA sequences in the genome of an organism. There is a wide variety of uses for this type of technology including repairing specific disease-causing gene mutations as well as the ability to increase a plant's nutritional value and resistance to climate change.

What vectors are being used in gene therapy for hemophilia?
Gene Therapy that is focused on treating hemophilia currently utilizes viral vectors. The most commonly used are the adeno-associated viruses or AAV. These viruses are modified so they don't cause viral infections and are able to safely deliver the gene of interest into specific cells so that they can start producing factor.

WHAT CAN I EXPECT:

Is gene therapy a cure for hemophilia?
Gene therapy holds the potential for longer term, durable treatments for hemophilia and gives promise for improvements to quality of life. Clinical trials are still underway so there is no definitive answers to length of durability which could determine if it is a cure.

Are there gene therapy treatments for both Hemophilia A and Hemophilia B?
Yes, currently there are several different clinical trials underway at various stages for both types of hemophilia.

What can I expect my factor level to be once I undergo gene therapy?
This depends on many factors. Response to gene therapy is very individual and it can vary over time. Clinical trials to date have demonstrated an increase in baseline factor levels. However, it is hard to predict if it will work on everybody and, if it does, how much it will increase and for how long.

Will my factor level fluctuate in the future if I undergo gene therapy?
Current clinical trials data have shown fluctuations in factor levels over time.

When is gene therapy for hemophilia realistically going to happen?
Currently there are several ongoing clinical trials underway for both Hemophilia A and B which are showing promising results. These trials will continue to be closely monitored to determine safety and efficacy of the therapy. At least one of these trials is nearing completion and is expected to file an NDA New Drug Application with the FDA which means the therapy could be available to patients as early as late 2020.

How long can I expect the effects of gene therapy to last?
At this time, no one really knows the answer to this question. There are several clinical trials underway with data that suggests the possibility of some factor expression in excess of 7 years.

Will I still need factor if I receive gene therapy? What if I have an accident, injury or need surgery?
The need for factor post gene therapy will be dependent upon the amount and duration of factor expression. Each person's response to gene therapy will likely be different and will require an individualized approach. This is a question best discussed with your provider.

If I am deemed ineligible for a particular gene therapy will this mean I am ineligible for any type of gene therapy in the future?
There are many reasons why a person could be deemed ineligible for gene therapy. Current clinical trials do not include males under 18, women, or those with an active inhibitor. Some trials exclude those who have developed antibodies to the vector used in the gene therapy. As the technology matures we will learn more about ways to make this technology available to more patients.

If I undergo gene therapy and it stops working can I try again in the future?
Currently, gene therapy for hemophilia is indicated as a one-time intravenous infusion. There is a possibility that the science will advance and increase options for the future.

Once I receive gene therapy will I still need annual checkups at my HTC?
Yes, you still need to follow up with your health care team after having completed gene therapy at least yearly.

Can I stop or turn off gene therapy?
No, gene therapy is a one-time intravenous infusion which once administered cannot be reversed or undone.

RISKS ASSOCIATED WITH GENE THERAPY:

What is vector shedding?
It is the process by which the viral vector leaves the body through bodily fluids after it is no longer needed by the body.

What are the risks associated with gene therapy?
Some possible risks associated with gene therapy include but are not limited to: an unwanted immune system reaction, targeting of the wrong cell, an infection caused by the virus and the possibility of hepatic carcinomas.

What happens if I undergo gene therapy and my resulting factor levels are higher than the normal range?
In some cases, gene therapy has resulted in factor levels well over the normal ranges. Although this may be associated with increased clotting risks no adverse effects have been reported. However, you need to know that this can happen and if it does you will need to be closely supervised by your hemophilia treatment center.

GENE THERAPY AND REPRODUCTION:

Can I pass the effects of gene therapy to my children?
No. Gene therapy for hemophilia is designed to correct the genetic defect only in the person who receives it. That is, it delivers a functional (or working) copy of the factor VIII or factor IX gene to the liver cells providing them with the instructions of how to produce the missing factor. Gene therapy does not correct the genes that are passed onto the next generation.

Should patients who undergo gene therapy bank sperm?
After the administration of gene therapy the body takes several weeks to months to get rid of the vector used in gene therapy through different bodily fluids including semen, blood, urine, feces and saliva. Although the risk for the vector to integrate into the sperm cell is low, men who undergo gene therapy are being asked to use a barrier contraceptive method (such as condoms) to prevent pregnancies for an extended period of time after the infusion. If you are considering having a baby in the near future, it might be reasonable to think about banking sperm before undergoing gene therapy.

will it last) and the level of factor expression will vary among individuals. Factor levels may be in the moderate, mild or normal range and may fluctuate over time.

These gene therapies are targeted to somatic cells (ex. cells that control biological processes within your tissues, organs, blood) not germline or reproductive cells so you can still pass hemophilia to your offspring. While you may rarely need to do infusions or maybe not at all, you will need to remain diligent with your healthcare to ensure you are on top of things.

Will I still be able to go to the HTC?
Most definitely, in fact please do. It will be important to continue to be monitored to assess any changes in overall health including emotional health as well as factor levels. Any underlying issues you had prior to gene therapy (ex. joint issues) will need to be monitored as well. Your HTC will be an important partner in your follow-up care post gene therapy.

ELIGIBILITY:

Who is eligible for gene therapy?
Currently gene therapy for Hemophilia A and B are in the clinical trial phases. Eligibility criteria varies somewhat between studies but for the most part the criteria is as follows: healthy (no diabetes, history of malignancies, heart disease, glaucoma, high blood pressures etc.) males, age 18 and older, with severe Hemophilia A or B, without inhibitors, no evidence of hepatitis or HIV, no evidence of liver dysfunction and no detectable neutralizing AAV antibodies. Your healthcare provider can explain the exclusion criteria in more detail.

At what age can my child have gene therapy?
Currently gene therapy for hemophilia is not indicated for individuals under 18. Gene Therapy for Hemophilia is targeted towards the liver which continues to grow in humans until they reach at least age 12. As the science is perfected the age could be lowered in the future.

Can females with Hemophilia receive gene therapy? If not why?
Currently gene therapy for Hemophilia is not indicated for female patients. Most female patients who are affected are classified as carriers with mild hemophilia and their factor levels are not low enough to qualify.

GENERAL INFORMATION:

How is gene therapy administered?
Similar to a factor infusion, gene therapy is a one-time intravenous infusion which can last anywhere from minutes to a few hours. However, unlike factor, it is currently being done in a medical facility by healthcare providers.

Where will I receive gene therapy? An HTC?
Gene therapy is currently being offered to eligible participants who are enrolled in a clinical trial. Currently, infusions occur only in pre-determined sites participating in the clinical trial.

Will my insurance company pay for gene therapy?
This is an important question and much work is being done to determine alternative financing and reimbursement strategies to enable patients to access these promising therapies. The current healthcare system as we know it is not equipped to handle large one-time payments. Additionally, these new treatment options have short treatment regimens (one-time infusion) and benefits that create challenges. These challenges include uncertainty around how long the therapy will last (insurance plans don't want to pay for things that don't work long-term) and uncertainty around an individual remaining on a given insurance plan (think about how often you might change insurance plans – new job, employers change types of plans offered, etc.). We have seen some gene therapies for rare diseases being reimbursed by insurance companies and that is great news for hemophilia patients. The creation of other conditions or comorbidities due to the gene therapy is also an unknown.

We do not currently know if the out-of-pocket costs will continue to be a challenge for the consumer like it is now in our current health care system. For those consumers with comorbidities or lasting physical effects of their primary disease, the out-of-pocket costs could and probably will continue to pay on an ongoing basis as out-of-pocket costs from high deductible health insurance continue to rise year over year.

Some suggested strategies include milestone-based contracts (payments are made when milestones are achieved) and performance-based annuities (a plan would pay an agreed upon amount if the therapy continues to perform well year to year) to name a few. This will require significant changes to our existing healthcare system. Additionally, there will be a need for policy changes at the Federal level to enable these strategies to work. NHF and other rare disease organizations are working with payers and policy makers to ensure access to these therapies becomes a reality. First dollar coverage is one component being discussed to address the affordability from the patient's side of things. Academic entities like the MIT NEWDIGS consortium bring together stakeholders that are working collaboratively (payers, providers, patient advocacy organizations, pharmaceutical developers, academics and others) to come up with innovative solutions.

For more in-depth information please contact:
Brendan Hayes at bhayes@hemophilia.org.

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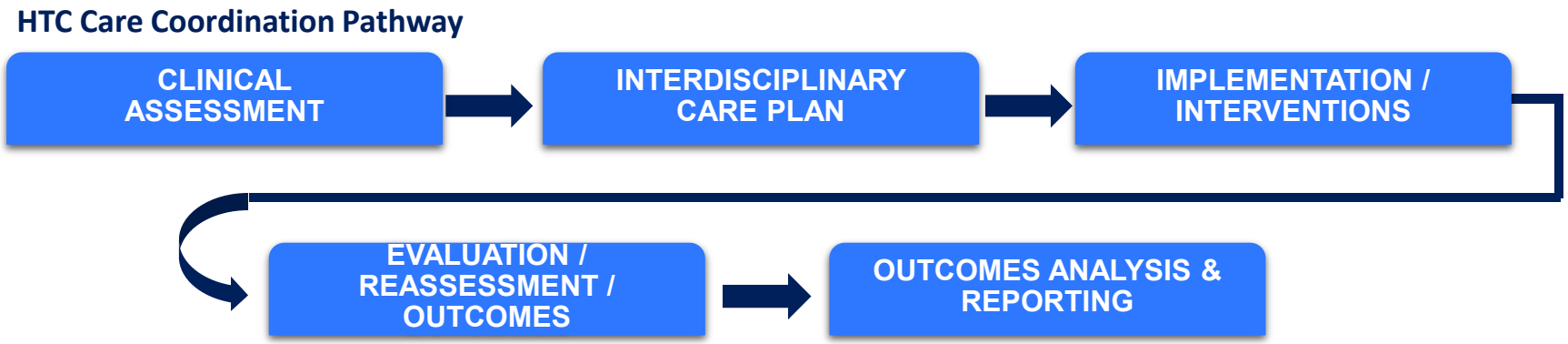
Future in Hemophilia Treatment Impact of Care Delivery Models

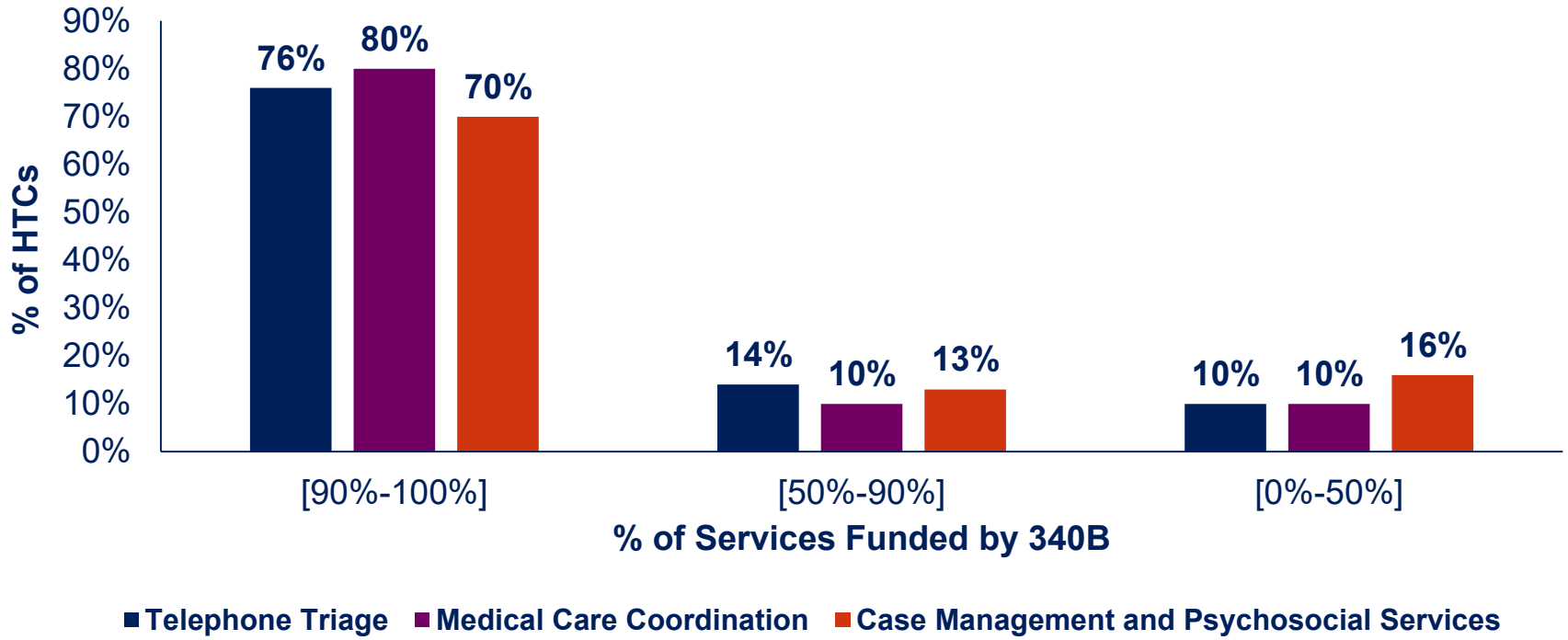
The HTC Model of Care Strives for Greater Integration of Services



Key Learnings of HTC Role in Managing Care and Costs
• Most centers fund unbilled care services such as telephone triage, medical care coordination, and case management/psychosocial services almost entirely through 340B
• Greater frequency of education with upstream touchpoints (PCP level and other specialties) to identify bleed prevention, including with social workers and ancillary care providers to stem costs   • Once controlled, live visits to HTCs decrease to 1-2x annually, and the majority of care is teleservice
• Currently, approximately 30% of patients do not go to HTCs; these primarily consist of mild disease and older members; geographic challenges within state and lack of local hematologist availability avail HTC as a resource

340B Program Income Supports Integrated Care Coordination and Unbilled Ancillary Services

Most HTCs fund unbilled care such as telephone triage, medical care coordination, and case management/psychosocial services almost entirely through 340B

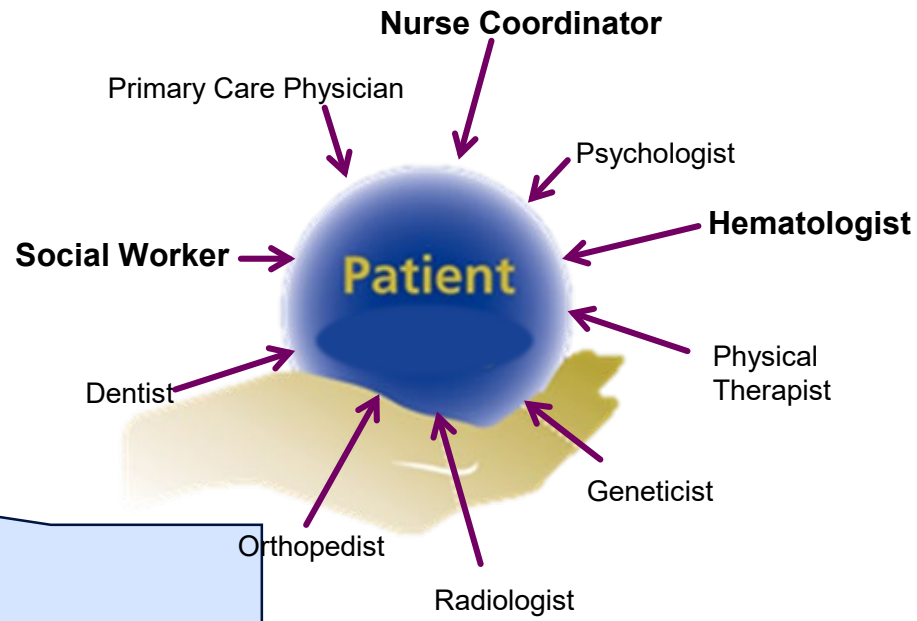


N=31 HTCs with established 340B programs.

Trujillo M, Forsberg AD, Drake J, Cheng D, McLaughlin K, McKernan L. National Survey of the 340B Drug Pricing Program: Quantitative Evaluation of the Services Provided by the U.S. Hemophilia Treatment Centers. Presented at: WFH 2016 World Congress; July 24-28, 2016; Orlando, FL.

Care Models for Hemophilia Therapy

- Persons with hemophilia receive the best care when seen annually at an HTC, in addition to their primary hematologist
  - A CDC study of 3000 people with hemophilia found that patients seen at an HTC were:
    - 40% less likely to die of a hemophilia-related complication
    - 40% less likely to be hospitalized for bleeding complications
- HTCs are seeing margins from 340b products shift due to increased used of non- factor products
- Novel treatments that reduce factor use may further impact HTCs
- Need to consider new care and reimbursement models
  - How to continue to support high quality care?

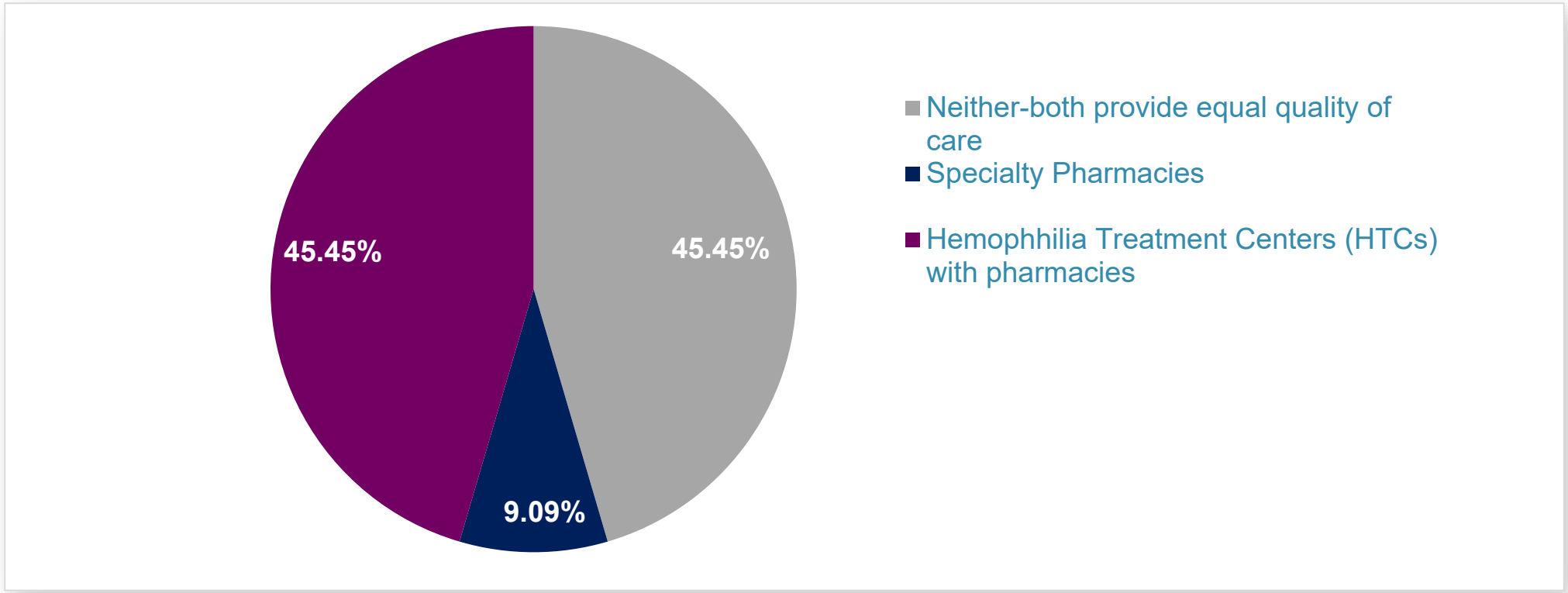


"HTCs need to come up with a new model- everyone else is changing"

Centers for Disease Control and Prevention. Hemophilia Treatment Centers. https://www.cdc.gov/ncbddd/hemophilia/htc.html#ref. Accessed June 25, 2018.

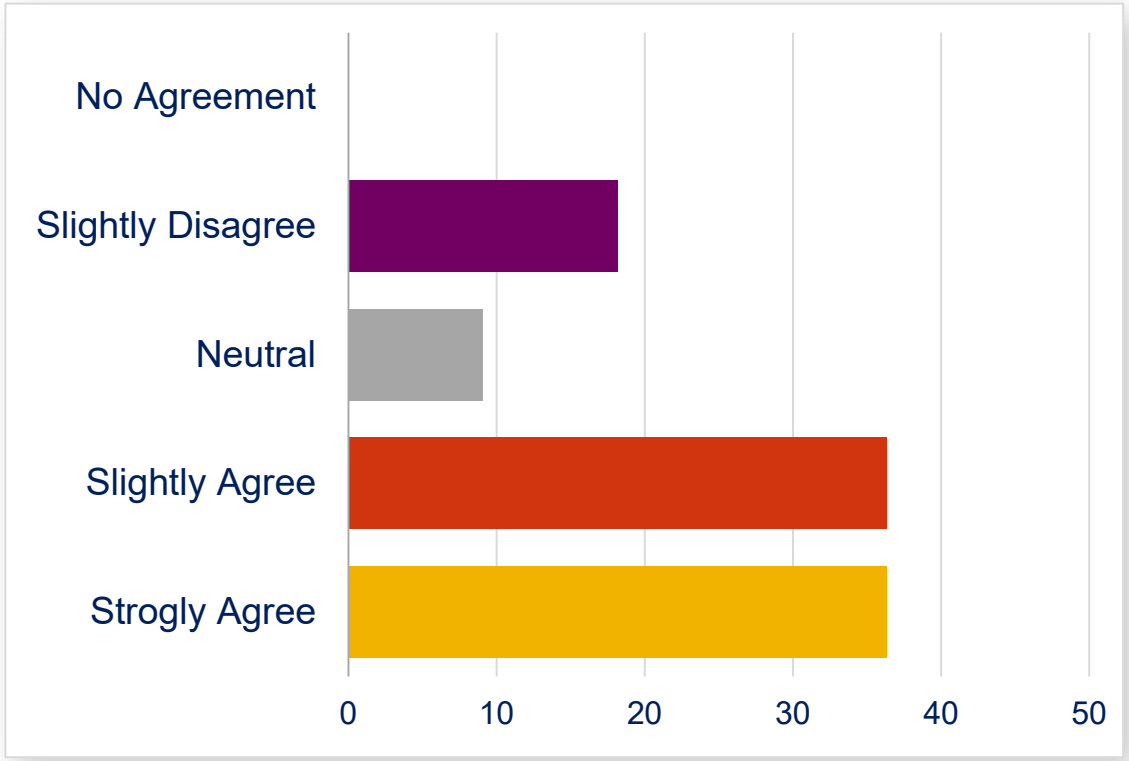
About half of Participants Believe HTCs Provide Better Care than SPs

Which of the following sites of care do you think provides better patient management and outcomes, while dispensing medication, for hemophilia patients?

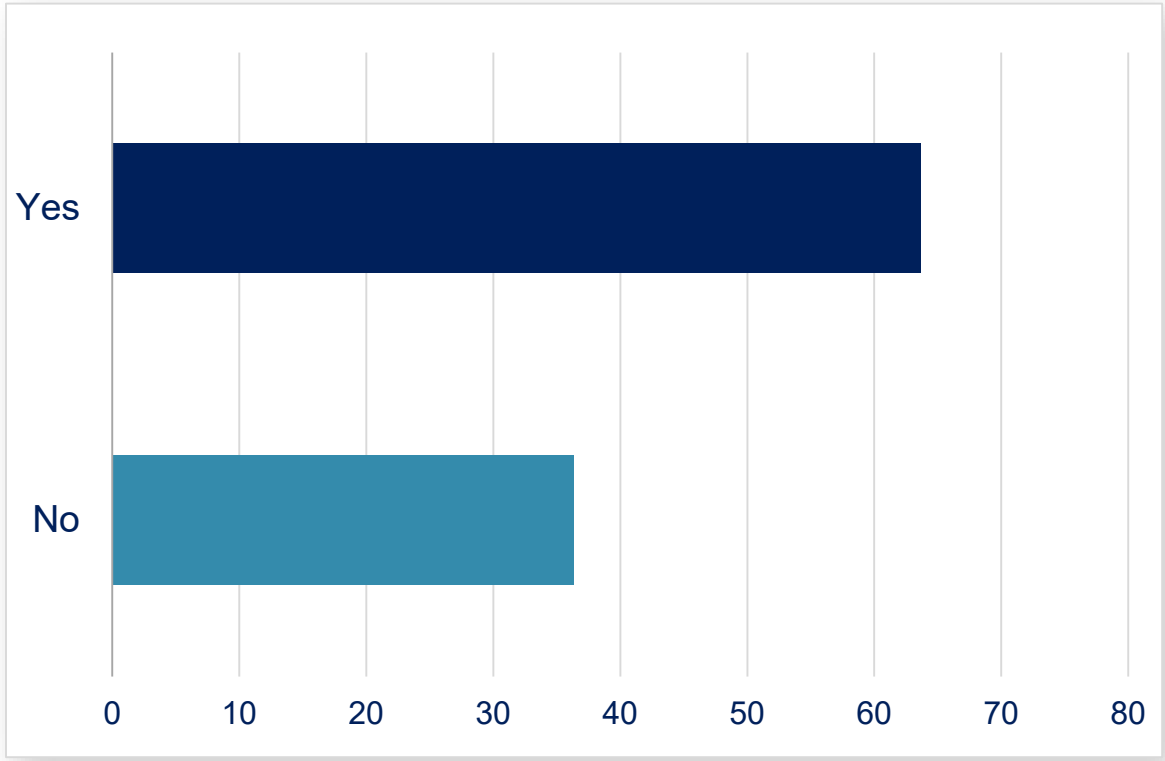


Participant Perspectives on HTC Reimbursement

How strongly to you agree with the statement that HTC pharmacies and SPs should receive the same hemophilia drug reimbursement rates?



Would you pay Hemophilia Treatment Centers directly for patient management / comprehensive care and outcomes separately from drug reimbursement?



Preparing to Manage Patient Needs in an Evolving Treatment Paradigm

- **Treatment Access and Quality**: Health plans should anticipate hemophilia care in network management and medical management strategies, to ensure access to specialized medical and pharmacy providers and support appropriate care as revenue streams shift.
- **Care Management**: Health plans should consider how best to coordinate multi-disciplinary outpatient and home-based services for members with hemophilia, determine what oversight and additional care coordination are needed and clearly designate accountability.
- **Cost Management**: Plans need to consider factor pricing and cost-effective approaches for administration of costly factor replacement products, while allowing for individualized treatment.
- **HTC and SP:** Need to consider new payment reimbursement models- monthly fee per patient, reduce services in rate were noted.
- **Pharmacy Management**: Plans should evaluate the full spectrum of services required to manage hemophilia, and contract with the most appropriate pharmacy to provide cost-effective and timely factor replacement services for routine and emergency needs.
- **Risk Adjustment and Risk Management**: Plans may need to work with the advocacy community and states to anticipate enrollment of members with hemophilia. These stakeholders can proactively recommend financing solutions to ensure member access to appropriate care; this may include risk adjustment or carve outs to avoid risk selection adversely impacting plans and members.

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Questions?

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