



Annual Report

2018

NATIONAL HEMOPHILIA FOUNDATION

THE NATIONAL HEMOPHILIA FOUNDATION IS DEDICATED TO FINDING BETTER TREATMENTS AND CURES FOR INHERITABLE BLEEDING DISORDERS AND TO PREVENTING THE COMPLICATIONS OF THESE DISORDERS THROUGH EDUCATION, ADVOCACY AND RESEARCH. THE PROGRAMMATIC ACTIVITIES AND ACCOMPLISHMENTS IN SUPPORT OF THIS MISSION CONDUCTED IN FY 2018 ARE DETAILED IN THE FOLLOWING PAGES.

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Medical Information

HANDI

Through HANDI, NHF's information resource center, over 1,782 requests for information were answered in 2018. These requests were received from patients, families, healthcare providers and the general public on such topics as hemophilia, von Willebrand disease, healthcare coverage, hepatitis C, HIV, inhibitor formation and school issues. In collaboration with Medscape Education, NHF offered 6 educational programs for healthcare providers reaching over 10,000 learners through online offerings on gene therapy for hemophilia for continuing education credit. We also launched new webinars specifically for bleeding disorders on topics such as inhibitors, women's issues, and von Willebrand disease.

Healthcare Provider Fellowships

NHF offers several fellowships to healthcare providers. The NHF-Shire Clinical Fellowship program is intended to increase the number of skilled clinicians committed to providing comprehensive care for individuals with bleeding disorders. It provides physicians with hands-on clinical training and prepares them for academic careers in bleeding disorders research. The Nursing Excellence Fellowship provides support for a registered nurse at a federally funded hemophilia treatment center (HTC) interested in conducting research or clinical projects. The Physical Therapy Excellence Fellowship provides support for a physical therapist at an HTC to conduct research or clinical projects related to the care of patients with bleeding disorders. The Social Work Excellence Fellowship provides support for a social worker conducting research into psychosocial bleeding disorders care.

NHF-Shire Clinical Fellowships

2018 to 2019

Hanny Al-Samkari, MD

Boston Children's Hospital/Harvard University

While an NHF-Shire Clinical Fellow, will acquire a clinical and research expertise in hemophilia and rare bleeding disorders under the mentorship of Dr. Stacy Croteau at Boston Children's Hospital and Drs. Eric Grabowski and Larissa Bornikova at Massachusetts General Hospital (MGH). Dr. Al-Samkari received his medical degree from Washington University in St. Louis, where he was elected to the national medical honor society, Alpha Omega Alpha (AOA). He completed his residency in internal medicine at the University of Pennsylvania, where he served as Chief Medical Resident. He completed his fellowship in hematology and medical oncology at the Dana-Farber Cancer Institute and Massachusetts General Hospital (MGH) combined program. During this fellowship, he strengthened his passion for classical hematology, and his research was focused on novel indications for thrombopoietin receptor agonists. Dr. Al-Samkari will lead the Hereditary Hemorrhagic Telangiectasia (HHT) clinic at MGH under the mentorship of Dr. David Kuter. He is also a dedicated medical educator, having worked as a member of the American Society of Hematology's Benign Hematology Curriculum Initiative, and he has continued to be active in teaching medical students, residents and physician assistants.

2018 to 2020

Michael H. White, MD

Emory University/Children's Healthcare of Atlanta (CHOA)

Dr. Michael H. White is a pediatric hematology and oncology fellow at Emory University/CHOA. He earned his undergraduate degree in biology from Harding University and completed his medical degree at the University of Texas Southwestern Medical School. He went on to complete his general pediatrics residency training at Vanderbilt University in Nashville, TN. He is currently pursuing a Master of Science degree in Clinical Research at Emory University and has received a TL1 research training grant (also known as a 'Linked Training Award'), through the National Institutes of Health for his focus on multidisciplinary clinical and translational science. Dr. White is also the Chair-Elect of the Hemostasis and Thrombosis Research Society (HTRS) Fellows Network and serves as a board member for the International Health Care Foundation. As an NHF-Shire clinical fellow, Dr. White will receive specialized clinical training in the Comprehensive Bleeding Disorders Clinic and Women and Girls' Bleeding Clinic at CHOA under the mentorship of Drs. Robert Sidonio and Shannon Meeks. In addition to his clinical focus treating patients with disorders of hemostasis and thrombosis, Dr. White will pursue his clinical research characterizing the management and outcomes of heavy menstrual bleeding in adolescents with bleeding disorders and in those who are taking anticoagulant or antiplatelet medications.

Nursing Excellence Fellowship

2018

Kelly Tickle, MSN, APN, PPCNP-BC
Children's Healthcare of Atlanta

Quality of Life and Outcome Improvement in Adolescents with Bleeding Disorders Receiving Medical Management for Heavy Menstrual Bleeding

Heavy menstrual bleeding (HMB) is common in adolescent girls with a bleeding disorder. While there are multiple medical options available for this group of patients, patients must attempt many treatments prior to finding the optimal hemostatic therapy. While it is important to determine which treatment is ideal for those patients, it is also important to look at how these therapies affect their quality of life. Quality of life is the ability to enjoy and participate in the activities of their everyday life. As doctors, nurses, and healthcare providers we look to care for patients' minds and bodies. HMB affects patients' physical, spiritual, mental, emotional, and social wellbeing. Our previous studies have tried to find treatments that help heavy menstrual bleeding. This study would not only continue to find those treatments, but also try to see how treatment that helps the bleeding will potentially improve patients' lives.

Social Work Excellence Fellowship

2018

Sabrina Farina, LMSW
Gulf States Hemophilia Treatment Center

Improving the Quality of Life for the Aging Patient with Hemophilia

As a social worker at Gulf States Hemophilia & Thrombophilia Treatment Center, I have the privilege of serving patients across their lifespan. I would like to initiate grass roots education about hemophilia in Houston, Texas, by offering educational programming to specialized healthcare professionals who work directly with the aging population in nursing homes and assisted living communities. This would include executive directors and administrators of these facilities as well as direct clinical staff.

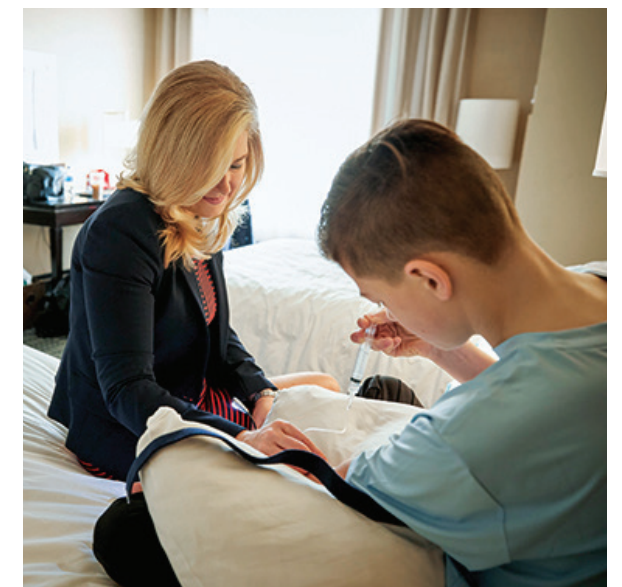
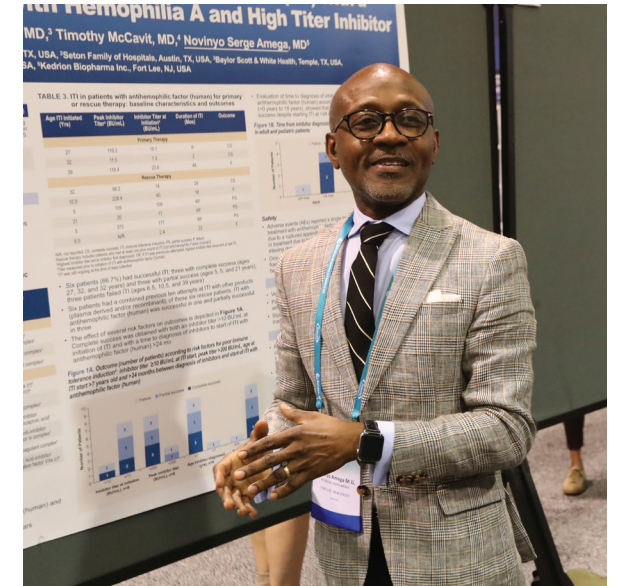
Physical Therapy Excellence Fellowship

2018

Laura Fox, PT, DPT, PCS
University of Colorado Hemophilia & Thrombosis Center

Women with Hemophilia: Gender-based Differences in the Delivery of Comprehensive Care

This study will evaluate hemophilia treatment center (HTC) services provided to women with hemophilia A or B (Factor VIII or Factor IX level \leq 50%). The American Thrombosis and Hemostasis Network (ATHN) maintains a confidential national database for patients with bleeding and clotting disorders. Utilizing this existing ATHN dataset, the study will analyze the effect of gender on the delivery of comprehensive care in patients with hemophilia A and B. The project will focus on how gender impacts three specific components of care: identification of patients with factor VIII or IX deficiency, inclusion of patients in the comprehensive care model, and monitoring of joint bleeding as a key component of comprehensive care provided by HTCs. Demonstrating gender-based disparities in comprehensive care would provide evidence for making changes to improve the clinical care provided to women with hemophilia. This study will add to the knowledge regarding the care of women with hemophilia, helping to inform future studies of this under-researched population.



Research

General Updates

Fellowships and Awards: The application process was streamlined, updated and transferred to an electronic format. All documents for application and instructions were updated on the website. NHF began two annual cycles of award applications with coordinated media notification to notify all HTC providers of upcoming awards in the hopes of increasing awareness within and outside of our community.

The **Judith Graham Pool (JGP) Fellowship** was increased from \$42,000/year for 2 years to \$52,000/year for 2 years.

An additional **Career Development Award** was offered in 2018 (it wasn't scheduled to be awarded until 2019).

The **Innovative Investigator Award** was started. This peer-reviewed award is intended for any member of the multidisciplinary team within the federally funded hemophilia treatment center (HTC). \$60,000 is awarded for 12-18 months for an innovative project.

The **Bridge Grant** was started. This award grants \$125,000 over 12-18 months to a physician investigator who has applied for a National Institutes of Health (NIH) grant and was scored but not funded.

Obesity Study: The research department assisted Novo Nordisk in recruiting participants for their Hope-to-Action Obesity Study by coordinating ebcasts and consumer participant payment.

Camp survey: A first-ever survey of camp administrators and health care professionals was developed and implemented. Results have been analyzed and a resultant manuscript initiated.

Telegenetic Counseling: Based upon the My Life, Our Future pilot group of women carriers of hemophilia, a study was developed to evaluate their access and response to genetic counseling utilizing telemedicine protocols. A coordinated awareness campaign to reach potential participants was initiated with treatment centers and chapters. Several expert hemophilia genetic counselors participated in this pilot study. Results are being analyzed.

Pain Study: In conjunction with MASAC, a first ever survey of the alignment of multidisciplinary HTC pain practices with the 2016 CDC Pain Guidelines was developed and sent to all federally-funded multidisciplinary team members. The MASAC Pain Initiative Sub Committee is currently writing a manuscript based on the results as well as recommendations to be forwarded to the larger MASAC group.

My Bleeding Disorders Community (MyBDC): Research staff worked with a consultant to develop an overall strategic plan for the research department. In preparation for MyBDC, focus groups were held throughout 2018 with consumers, chapters, our federal partners (CDC, HRSA), regional coordinators, providers, and industry to understand the wants and needs of the community regarding research as well as their desire to participate in a consumer-powered registry. A platform vendor was chosen after an intensive RFP and interview process.

BDC2018: Awardees were invited to the 2018 BDC, many to present their research findings.

WFH 2018: Research staff attended WFH Congress in Scotland and presented to nursing and general audiences.

Fellowships and Awards

The Judith Graham Pool Postdoctoral Research Fellowship supports basic science and pre-clinical research in bleeding disorders. The NHF/Novo Nordisk Career Development Award supports innovative projects that promote the development of novel technologies and/or therapies that would advance the field of bleeding disorders research. The Innovative Investigator Award supports projects that demonstrate a commitment to NHF's goal of developing outcomes-related data. The Bridge Award supports hematology investigators focused on bleeding disorders who applied for an NIH grant and was scored but not funded.

Judith Graham Pool Postdoctoral Research Fellowship

2018 to 2020

Laura Haynes, PhD

Life Sciences Institute University of Michigan

Protein Engineering of Plasminogen Activator 1 to Develop Novel Regulators of the Fibrinolytic and Hemostatic Pathways

As the coagulation and fibrinolytic processes are governed largely by serine protease-mediated proteolytic reactions, the central hypothesis of this proposal is that serine protease inhibitors (SERPINs) can be engineered in order to treat bleeding disorders. In order to promote a more procoagulant state, the SERPIN plasminogen activator inhibitor 1 (PAI-1) will be a "backbone" from which variants that can downregulate the fibrinolytic and anticoagulant pathways will be generated with the potential to treat coagulopathies. Phage display technology coupled with modern high-throughput sequencing will be used to characterize simultaneously how the substitution of any amino acid at any position in the PAI-1 primary sequence affects the (1) half-life, (2) kinetics of inhibition, and (3) specificity. PAI-1 is an excellent choice of a prototypic SERPIN to use in these studies as (1) small portions of its mutational landscape have been previously characterized and can be used to validate findings, and (2) as PAI-1 lacks Cys residues/disulfide bonds, it can be readily produced in bacterial expression systems. This experimental tool will be used to generate two distinct PAI-1 variants with therapeutic potential. In Aim 1, a PAI-1 variant with a prolonged half-life will be generated in order to downregulate fibrinolytic pathways. In Specific Aim 2, a PAI-1 variant that inhibits activated protein C will be generated in order to downregulate this anticoagulant pathway.



Kari Lavik, PhD

**Division of Hematology/Oncology Department of Pediatrics and Communicable Diseases
University of Michigan**

Identifying novel hemostatic regulation through species-specific studies using zebrafish.

Identifying novel hemostatic regulation through species-specific studies using zebrafish. Hemophilia is one of the most common bleeding disorders, affecting approximately 400,000 individuals worldwide. Hemophilia A, caused by a deficiency in factor VIII (F8), is the more prevalent form of this bleeding disorder; while, hemophilia B, resulting from a deficiency in factor IX (F9), affects roughly 1/25,000 male births. Hemophilia treatment has dramatically improved patient survival and quality of life, yet accessibility and affordability remain a barrier world-wide. By deepening our understanding of the genetics behind hemophilia, we hope to identify novel mechanisms that can be targeted to improve treatments. Zebrafish demonstrate genetic conservation with humans and exhibit high fecundity, rapid development and optical transparency, making them an ideal system for genetic studies. Advancements in genome sequencing, genome editing, microscopy and related technologies have established zebrafish as a useful model for the study of the genetics of hemostasis and thrombosis. Previously, our laboratory has produced coagulation factor knockout zebrafish (antithrombin and factor X) that successfully model human pathologies. However, our preliminary studies suggest that loss of F8 does not result in a bleeding defect. In zebrafish, f8 exists in a single form, but an evolutionary duplication event has resulted in two orthologs of the f9 gene (f9a, f9b). In order to discover whether the intrinsic pathway is required for coagulation in zebrafish, we will generate f9a and f9b deficient zebrafish and evaluate them for bleeding phenotypes, as well as cross them into the f8 mutant background. We will also perform further studies to determine why loss of F8 has no effect on hemostasis. If complete loss of the intrinsic pathway in zebrafish does not result in bleeding phenotypes, this suggests the existence of species-specific differences between zebrafish and humans which can be exploited to develop novel therapeutics for affected patients.

NHF/Novo Nordisk Career Development Award

2018 to 2021

Moanaro Biswas, PhD

Herman B Wells Center for Pediatric Research Indiana University

Engineered regulatory T cell therapy for tolerance to FVIII

The development of inhibitors against coagulation factor VIII (FVIII) is a critical complication in hemophilia A treatment, as hemostasis can no longer be re-established by FVIII replacement therapy. Although immune tolerance induction (ITI) protocols are in place for elimination of inhibitors, about 30% of hemophilia A patients undergoing ITI fail to control inhibitor titers to manageable levels. Therefore, new and improved tools are needed that can work alone or in conjunction with either prolonged ITI or bypassing agents to improve hemostasis in patients that have failed ITI. There is strong evidence that regulatory T cells (Treg) are an integral part of immune tolerance to coagulation factors in gene and protein replacement therapies. We present adoptive transplant of autologous regulatory T cells (Treg) as a candidate immunotherapy for targeted suppression of immune cells involved in inhibitor formation. Tregs will be engineered for antigen specificity by 2 alternative approaches: 1) Redirecting Treg specificity toward FVIII epitopes via chimeric antigen receptor (CAR) incorporation. If successful, a single CAR construct will be able to confer FVIII specific suppression regardless of MHC or epitope usage. 2) Reprogramming antigen experienced CD4+ T effector cells into Tregs by FoxP3 gene transfer. This will generate a large pool of Tregs enriched for specificity to FVIII. Ex vivo engineered Tregs will be tested for antigen recognition and suppressive responses in vitro. Effectiveness of both these approaches to reverse existing inhibitor titers will be tested in an established murine model of hemophilia A. Mechanisms of tolerance and interaction with critical immune cells as well as efforts to address tonic signaling and cell exhaustion will be tackled. On completion of this project, we hope to optimize a cellular therapy regimen for tolerance induction to FVIII, which can be translated into an alternative ITI approach for hemophilia.

NHF Bridge Grant (new in 2018)

2018 to 2019

Qizhen Shi, MD

Medical College of Wisconsin

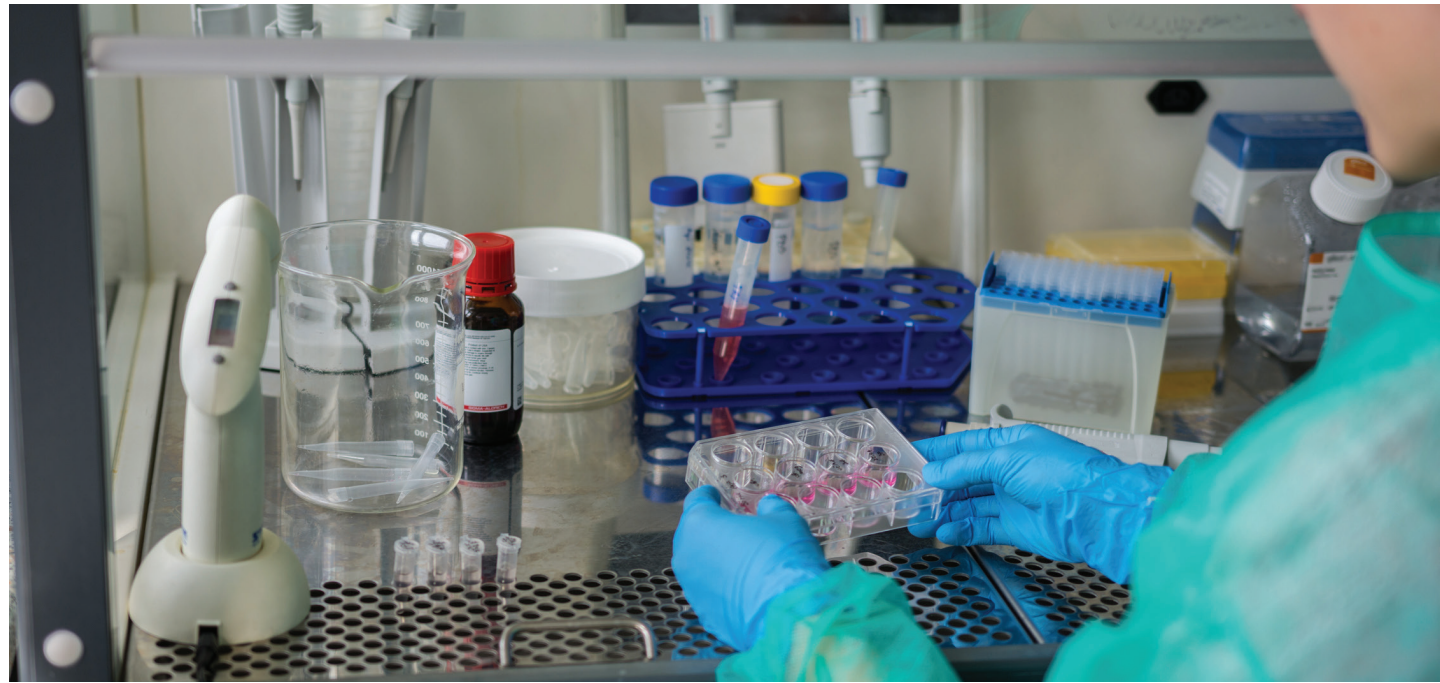
Investigation of VWF as an immunomodulator of the immunogenic response towards FVIII

Through these studies, important information about the impact of VWF on FVIII immune responses and tolerance induction in HA with pre-existing anti-F8 immunity will be gained. These studies will aid the design of more effective protocols to prevent FVIII immune responses and to induce FVIII immune tolerance in patients with HA. Thus, these studies will have a significant clinical impact in HA care and will also help researchers understand the biological properties of the interaction between VWF and FVIII.

Research has led to techniques and understanding of pathophysiology (disease-related functional changes) that is applicable to other diseases as well. The successes in hemophilia research pay for themselves several fold.

Paul E. Monahan, MD





NHF's Innovative Investigator Awards are part of NHF's commitment to researchers who are on the forefront of breakthrough research for bleeding disorders.

Michelle Witkop, DNP



Innovative Investigator Award (new in 2018)

2018 to 2019

Shannon Meeks, MD

Aflac Cancer and Blood Disorders Center

Novel Therapeutics for Hemophilia

Managing bleeding in hemophilia A patients with inhibitors remains challenging as the patients' own immune system renders factor VIII (fVIII) inefficacious. To address this critical need of on-demand treatments for these patients, the laboratory of Shannon Meeks, MD has initiated clinical assessment of a novel polyelectrolyte multilayer (polymer) capsule for the targeted delivery of fVIII, recently developed by the laboratory of Wilbur Lam, MD, PhD. This innovative system encapsulates fVIII within a polymer shell, which binds to and hybridizes with the patient's own platelets upon intravenous administration. The hybridized platelets target the capsule to sites of injury and rupture the capsule open through their natural contractile behavior in the clot formation process, facilitating a "burst" release of fVIII. For patients with inhibitors, fVIII is protected during circulation by the polymer shell, so a near-full dose of uninhibited fVIII is delivered at the injury site to quickly rescue hemostasis. Our recent publication (Hansen CE et al., ACS Nano 2017) describes in vitro investigations into the platelet-mediated mechanism. Initial in vivo investigations demonstrate safety and significantly enhanced efficacy of the fVIII loaded capsules compared to infusion of fVIII alone. In this NHF IIR, we propose to continue in vivo evaluation of safety and efficacy of the technology using a murine hemophilia (E16) model towards developing a safe and rapid on-demand treatment for patients with inhibitors. In Aim 1, we will investigate toxicity, biodistribution (whole organ analysis), and immunomodulatory or thrombogenic responses (ELISA assays of anti-fVIII antibodies, TATc, and D-dimer levels). In Aim 2, we propose studies to determine clearance rate (flow cytometry) and a dose-response curve (bleeding times using a tail clip model) in comparison to activated prothrombin complex concentrate. These investigations will be used to approach pharmaceuticals companies for potential partnership to further translational development.

Jill Johnsen, MD

Bloodworks NorthWest

Developing of point-of-care testing for hemophilia

Hemophilia is an inherited bleeding disorder caused by loss of function in the coagulation factors Factor VIII (FVIII), resulting in hemophilia A, or Factor IX (FIX), resulting in hemophilia B. In hemophilia, laboratory measures of factor activity levels inform treatment. The current standard of care in treating patients is to increase the levels of clotting factors to a specific target factor level to prevent or treat bleeding. Coagulation factor level testing informs hemophilia drug treatment dosing and assists in determining drug efficacy. Factor level monitoring is established as an effective strategy for treating patients with hemophilia in tertiary care hospital settings. However, these tests can only be performed in specialty coagulation laboratories due to the reagents and expertise needed to do the tests. Restriction of coagulation factor testing to specialty labs is a significant obstacle to the precise treatment of hemophilia patients using factor levels in routine healthcare settings and at home. We propose a new method for the testing of coagulation factor levels for patients with hemophilia that can be adopted into a point-of-care diagnostic device. This method uses assays that measure the coagulation factor antigen and information about the patient to provide a calculated factor activity level. We propose to optimize our method for testing FVIII and FIX and validate these assays in samples from patients with hemophilia A and B compared to clinical activity assays. The overarching goal of this project is to test performance characteristics of this new method and develop data sufficient to support integration of these tests into point-of-care diagnostic form factors with the long-term goal being incorporation into a handheld point-of-care device that patients or health care providers may use to obtain real time activity data for individualized dosing in the treatment of bleeding episodes.

MASAC

NHF's Medical and Scientific Advisory Council (MASAC) held two meetings, March 10-11, 2018, and October 13, 2018, to update and develop new recommendations regarding the treatment of individuals with bleeding disorders. The following are the documents that were approved by the council in 2018:

MASAC Document #253

MASAC Recommendations Concerning Products Licensed for the Treatment of Hemophilia and Other Bleeding Disorders

MASAC Document #254

MASAC Document Regarding Risks of Gene Therapy Trials for Hemophilia

Recommendation on the Use and Management of Emicizumab-kxwh (Hemlibra®) for Hemophilia A with and without Inhibitors

Chapter Services

Chapter Development

Chapter Reviews & Chapters of Excellence Awards

It is with great pleasure that NHF recognized a select group of chapters during the Bleeding Disorders Conference Awards Ceremony in Orlando as Chapters of Excellence. These awards were given to chapters who demonstrated excellence in the achievement of standards and best practices evaluated through the rigorous chapter review process, scoring 85% or greater in one or more of the six-chapter review areas: NHF Partnership Award, Programs & Services, Advocacy & Public Policy, Fundraising & Development, Governance & Board Leadership & Volunteer Management which was newly added in 2018.

National Hemophilia Foundation is proud to recognize the 2018 Chapters of Excellence Awards. There were 42 total awards, and three five-star chapters: Bleeding Disorders Alliance Illinois, Hemophilia of Georgia and Virginia Hemophilia Foundation.

NHF Chapter Partnership Award

- New England Hemophilia Association
- Rocky Mountain Hemophilia & Bleeding Disorders Association
- Nevada Chapter, National Hemophilia Foundation
- Lone Star Chapter of the National Hemophilia Foundation
- New York City Hemophilia Chapter
- Hemophilia Foundation of Oregon
- Virginia Hemophilia Foundation
- Bleeding Disorder Alliance Illinois

Programs & Services

- Nevada Chapter, National Hemophilia Foundation
- Lone Star Chapter of the National Hemophilia Foundation
- Virginia Hemophilia Foundation
- New England Hemophilia Association
- Hemophilia of Georgia
- Northern Ohio Hemophilia Foundation
- Hemophilia Foundation of Oregon
- Bleeding Disorders Alliance Illinois
- New York City Hemophilia Chapter



NHF Chapter Partnership Award

- New England Hemophilia Association
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- Nevada Chapter, National Hemophilia Foundation
- Lone Star Chapter of the National Hemophilia Foundation
- New York City Hemophilia Chapter
- Hemophilia Foundation of Oregon
- Virginia Hemophilia Foundation
- Bleeding Disorder Alliance Illinois

Advocacy & Public Policy

- Virginia Hemophilia Foundation
- Bleeding Disorders Alliance Illinois
- Hemophilia of Georgia
- Northern Ohio Hemophilia Foundation
- Hemophilia Foundation of Oregon
- Tri-State Bleeding Disorders Foundation
- Lone Star Chapter of the National Hemophilia Foundation
- Central California Hemophilia Foundation
- Hemophilia Association of San Diego County
- New England Hemophilia Association
- New York City Hemophilia Chapter

Volunteer Management

(New Category to 2018-2020 Chapter Review Process)

- Hemophilia of Georgia

Programs & Services

- Nevada Chapter, National Hemophilia Foundation
- Lone Star Chapter of the National Hemophilia Foundation
- Virginia Hemophilia Foundation
- New England Hemophilia Association
- Hemophilia of Georgia
- Northern Ohio Hemophilia Foundation
- Hemophilia Foundation of Oregon
- Bleeding Disorders Alliance Illinois
- New York City Hemophilia Chapter

Fundraising & Development

- New England Hemophilia Association
- New York City Hemophilia Chapter
- Hemophilia Foundation of Oregon
- Hemophilia of Georgia
- Bleeding Disorders Alliance of Illinois
- Virginia Hemophilia Foundation
- Oklahoma Hemophilia Foundation
- Nevada Chapter, National Hemophilia Foundation

Governance & Board Leadership

- Virginia Hemophilia Foundation
- Nevada Chapter, National Hemophilia Foundation
- Bleeding Disorders Alliance Illinois
- New York City Hemophilia Chapter
- Hemophilia of Georgia

Board Summits

In 2018, NHF Chapter Services partnered with 11 chapters to host board retreats with 87 board members participating in the training. This is a customized one-day workshop that will leave them with a board development action plan charting a road map for improvement efforts over the 3-6 months following the retreat. During our time together, chapter leadership collectively identified 36 goals that were tracked over time and we are excited to share that 72% of those goals were completed within that 3-6 month window. These retreats ranked highly with chapter staff and their board members ranking items such as: I learned a new idea, concept, or strategy, the retreat met my expectations and the information and resources presented were helpful achieved scores 3.75/4 (94% satisfaction rate).

Development Capacity Building Grants

Chapter services identified five chapters through an application process and committed to financial support grants to create development-centric positions in our local communities. This commitment was a \$70,000+ financial investment in those chapters as well as talent management investment. Through this grant opportunity, NHF is proud to support chapters in the hiring and coaching of their development staff—and helping chapters take this first step in building stronger development systems at the local level.

- New England Hemophilia Association
- Bleeding Disorder Alliance of Illinois
- Oklahoma Hemophilia Foundation
- Virginia Hemophilia Foundation
- Alaska Hemophilia Association



Affiliated Chapters

for Period ending December 31, 2018

Alaska Hemophilia Association

Anchorage, AK

Hemophilia & Bleeding Disorders of Alabama

Wetumpka, AL

Arizona Hemophilia Association

Phoenix, AZ

Central California Hemophilia Foundation

Sacramento, CA

Hemophilia Association of San Diego County

San Diego, CA

Hemophilia Foundation of Northern California

Emeryville, CA

Hemophilia Foundation of Southern California

Pasadena, CA

Colorado Chapter, National Hemophilia Foundation

Denver, CO*

Connecticut Hemophilia Society, Inc.

Windsor, CT

Florida Hemophilia Association

Fort Lauderdale, FL

Hemophilia Foundation of Greater Florida

Winter Park, FL

Hemophilia of Georgia

Atlanta, GA

Hawaii Chapter, National Hemophilia Foundation

Ewa Beach, HI*

Hemophilia of Iowa

Cedar Rapids, IA

Idaho Chapter, National Hemophilia Foundation

Boise, ID*

Bleeding Disorders Alliance Illinois

Chicago, IL

Hemophilia of Indiana

Indianapolis, IN

Midwest Hemophilia Association

Leawood, KS

Kentucky Hemophilia Foundation

Louisville, KY

Louisiana Hemophilia Foundation

Baton Rouge, LA

New England Hemophilia Association

Dedham, MA

Hemophilia Alliance of Maine, Inc.

Brewer, ME

Hemophilia Foundation of Michigan

Ypsilanti, MI

Hemophilia Foundation of Minnesota and the Dakotas

Mendota Heights, MN

Gateway Hemophilia Association

Saint Louis, MO

Rocky Mountain Hemophilia and Bleeding Disorders Association

Bozeman, MT

Hemophilia of North Carolina

Morrisville, NC

Bleeding Disorder Alliance of North Dakota

Fargo, ND

Nebraska Chapter, National Hemophilia Foundation

Omaha, NE*

Sangre de Oro, Bleeding Disorders Foundation of New Mexico

Los Ranchos, NM

Nevada Chapter, National Hemophilia Foundation

Las Vegas, NV*

Bleeding Disorders Association of Northeastern New York

Troy, NY

Hemophilia Center of Western New York

Buffalo, NY

Mary M. Gooley Hemophilia Center

Rochester, NY

New York City Hemophilia Chapter Inc.

New York, NY

Central Ohio Chapter, National Hemophilia Foundation

Columbus, OH*

Northern Ohio Hemophilia Foundation

Independence, OH

Northwest Ohio Hemophilia Foundation

Toledo, OH

Southwestern Ohio Hemophilia Foundation

Moraine, OH

Tri-State Bleeding Disorder Foundation

Cincinnati, OH

Oklahoma Hemophilia Foundation

Owasso, OK

Hemophilia Foundation of Oregon

Corvallis, OR

Eastern Pennsylvania Chapter of NHF

Springfield, PA

Western Pennsylvania Chapter of NHF

Cranberry Township, PA

Hemophilia of South Carolina

Greenville, SC

Lone Star Chapter of NHF

Houston, TX

Texas Central Hemophilia Association

Dallas, TX

Hemophilia Association of the Capital Area

Springfield, VA

Virginia Hemophilia Foundation

Richmond, VA

Bleeding Disorders Foundation of Washington

Edmonds, WA

Great Lakes Hemophilia Foundation

Milwaukee, WI

West Virginia Chapter, National Hemophilia Foundation

Morgantown, NY*

*: Chapters owned by The National Hemophilia Foundation

Education

Steps for Living

In 2018, the National Hemophilia Foundation continued to grow Steps for Living, a life stage education program that combines online, life stages education content with in-person workshop curriculum to assist those affected by bleeding disorders and continue supporting families not only in the first years of a child's life, but throughout the various stages of his/her/their development. NHF launched new educational content on sex and intimacy with a bleeding disorder, with web content and educational videos addressing community-sourced questions. Chapter and hemophilia treatment center (HTC) staff and volunteers participated in NHF's Steps for Living Training where participants learned how to run some of our on-the-ground programming themselves for their local events. NHF trained 50 participants in 2018 at the first-ever training in both English and Spanish.

On-the-Ground Programs

Beginning in 2015, NHF made a commitment to bring our high quality in-person programming to local chapter or HTC events through our Education for Empowerment and Collaborating in Care workshops. NHF staff, speakers, and youth leaders facilitated 24 workshops across the country in 2015 and grew this to 146 workshops in 2018. These workshops were tailored for youth, adults, and women and were also facilitated in Spanish where requested.

Young Adults

NHF's National Youth Leadership Institute (NYLI) fosters the development of youth leaders to help NHF chapters and associations across the country build strong youth programs. The NYLI program in 2018 had 27 members ages 18-24. This three-year program includes general leadership development in year one, and opportunities for NYLI members to select a track to focus on for years two and three to apply their leadership skills in a more in-depth way. The tracks include nonprofit development, outreach, and advocacy. NYLI members continue to participate in Washington Days and lead sessions at NHF's Bleeding Disorders Conference. Through these activities the NYLI program builds five core competencies for the participants: communication, self-management, career skills, emotional intelligence and meeting & group facilitation skills.

Women with Bleeding Disorders

NHF's programming for girls and women with bleeding disorders has two main goals: 1) to increase awareness of women's bleeding disorders so that girls and women receive early, accurate diagnosis, leading to better health outcomes, and 2) to provide women affected by bleeding disorders with the education, support, skills and resources they need to advocate for their healthcare, financial and social support needs.

Victory for Women (V4W), NHF's initiative for women affected by bleeding disorders, focused on building an online community for women on victoryforwomen.org in 2018 and saw an 82% increase in users compared to 2017. NHF also hosted an in person follow up training for those who had previously completed its V4W Ventures Program, an eight-week webinar series to develop leadership skills in women with bleeding disorders, and chapter and HTC staff who will run women's programming.

As part of a five-year cooperative agreement with the Centers for Disease Control and Prevention (CDC) to increase awareness of bleeding disorders so women can receive timely and accurate diagnosis and lead healthy full lives, NHF launched a campaign for undiagnosed women with symptoms of a bleeding disorder to get the information and care they need. Women can go to betteryouknow.org to take a risk assessment tool, find more information on next steps to finding a diagnosis and treatment. NHF produced a series of videos of women sharing their stories to ensure that women don't feel alone and get the care they need, as well as created resources for chapters to use in local outreach for undiagnosed women. From 2017 to 2018 NHF's outreach efforts have more than doubled the number of risk assessment tool takers, and of those who took the assessment, 84% scored at risk for a bleeding disorder.



Von Willebrand Disease (VWD)

NHF continues to offer dynamic educational programming for those living with VWD, from in person workshops delivered at chapters to the VWD track and pre-conference at NHF's Bleeding Disorders Conference.

Reaching Our Diverse Community

The NHF Cultural Diversity Working Group advised on content in both English and Spanish for the 70th Bleeding Disorders Conference, and reviewed applications for NHF's Connections for Learning Program, that provides assistance to individuals and/or families affected by bleeding disorders who would have been unable to attend due to financial constraints or had never attended. Individuals from 35 families were awarded educational participant scholarships to attend. The families were very appreciative of the opportunity to attend and meet other families with bleeding disorders. Evaluations from these families showed how vital the information gathered at Bleeding Disorders Conference was to their ability to manage bleeding disorders.

NHF continued to promote its *Guías Culturales* program. The *Guías* are culturally intelligent volunteers who are the path to service to the bleeding disorders community, acting as "guides" to diverse members of the bleeding disorders community. NHF recruited and trained nine *Guías* in 2017 who presented at two chapter events in 2017, increasing to eight in 2018.

Healthy Lifestyles

As part of a Cooperative Agreement with the CDC, NHF continues to offer two new nurse-led workshops that were facilitated at local chapter events in both English and Spanish: "Half-Life in Your Life," and "It's Not Too Late to Save Your Joints," as well as a PT-led workshop on Playing It Safe: "Bleeding Disorders, Sports & Exercise." NHF also added two new physical therapist led webinars in 2018: "Meet the Joint: Elbows" and "Meet the Muscle Bleed" to its online educational offerings, leading to more than 1,600 views of its online content for healthy joints in 2018.

Inhibitor Education

NHF hosted three National Inhibitor Summits in 2018 for those with an active or tolerized inhibitor and their support network. The three-day educational conference reached 859 participants in 2018. In addition, NHF hosted one Spanish Inhibitor Summit, *Viviendo con Inhibidores*, in 2018, offering educational workshops all in Spanish. Fifty-eight participants attended the Spanish summit.

Rare Factor Deficiency Programs

NHF offers unique educational programs for families affected by rare bleeding disorders. For those with factor XIII deficiency, NHF hosted its 4rd annual Strength in Numbers: Factor 13 Family Conference. NHF also hosted its second The Power of Ten conference, for those with factor X deficiency. Both programs were held as pre-conferences to NHF's Bleeding Disorders Conference, allowing families to attend all the education content of the larger meeting as well. NHF was pleased to be able to bring 44 factor XIII participants and 41 factor X participants together for education and connections at these programs in 2018.



Public Policy

2018 National Advocacy Empowerment Program (NAEP)

The NHF public policy team continues to monitor emerging trends and identify potential barriers to access to care and share this information with stakeholders (patients, HTC's, chapters, specialty pharmacy providers and manufacturers). It is the team's responsibility to educate and inform chapter leaders and consumers about the issues and how, when and where to engage to help insure that payers and state and federal legislators are aware of the needs of this community. This education is targeted and presented in various formats, including webinars, fact sheets, PowerPoint presentations, etc. In March of 2018, we had a record-breaking 500+ attendees at Washington Days, which was an essential step towards ensuring patient protections in the Affordable Care Act were not eliminated. We also supported state advocacy days in nearly 30 state capitols, with trainings held prior to each event.

We have learned that advocacy programming is executed best with adequate training prior to the events. Prior to Washington Days, we provide comprehensive training via webinars to explain what participants should bring with them for maximum success during their Hill visits. We also provide onsite training the evening prior to the Hill visits to ensure that participants have all the information readily available and have ample opportunities to ask questions of NHF staff and federal policy consultants.

Similarly, with state advocacy days, we strongly encourage chapters to host a training the evening prior to the event. For some chapters/states, this can be difficult because volunteer advocates cannot take another day away from work or other responsibilities. In these instances, we encourage chapters to host a webinar a few days before the advocacy day.

Since 2016, NHF has promoted March as Bleeding Disorders Awareness Month. In addition to this designation at the federal level, in 2018, more than 20 states also recognized March as Bleeding Disorders Awareness Month. These designations have come about either as a Legislative Resolution or a Gubernatorial Proclamation. Many of these actions have coincided with a state advocacy day and/or a photo opportunity with the respective legislators and/or Governors. We have found these opportunities to be tangible measures of success for volunteer advocates. For example, the advocacy committee of the New England Hemophilia Association (NEHA) challenged each state lead from the six New England states to pursue such a designation. In 2018, all six New England states officially declared March as Bleeding Disorders Awareness Month.

State-Based Advocacy Efforts

Advocacy efforts within the bleeding disorders community will continue to be a priority. With emerging treatments constantly on the horizon, ongoing vigilance will be key to ensure patient access to care. In 2018, advocates across the country visited 30 state capitols. These events are an essential component of year-round advocacy efforts we expect of all our chapters.

We also will continue to expand our reach through our State-Based Advocacy Coalition (SBAC) program, which became available to all chapters in 2018. Note that three new grantees became a part of the SBAC program in 2019 (Hawaii, Nevada and Virginia), which brings the total number of SBAC states to 24.

NHF policy staff also presented the success of the SBAC program via poster presentations at three conferences in 2018: the WFH World Congress, the NHF Bleeding Disorders Conference, and the NORD Conference in November.

Our industry partners contributed to the program's success in multiple ways. First, we invite industry partners to our two annual in-person State-Based Advocacy Coalition (SBAC) meetings. The first happens just before Washington Days begins in March. The second in-person SBAC meeting coincides with the beginning of the NHF Bleeding Disorders Conference which was in October 2018. Second, we hosted an advocacy stakeholder meeting at the Bleeding Disorders Conference that was three hours long. Finally, NHF policy staff hold recurring conference calls with industry partners throughout the year to share information, updates and opportunities.

Meeting your elected representatives and telling your story can make a valuable impression. This is usually just a one day commitment.

Nathan Schaefer



Conference & Travel Services

The Conference and Travel Services department (C&TS) of NHF is an internal professional conference organizing team in charge of the management of all NHF multi-day conferences. The Conference and Travel Services team handles a wide range of events every year, from two-day meetings to the complete management of a three-day conference for as many as 3,000 participants.

Bleeding Disorders Conference

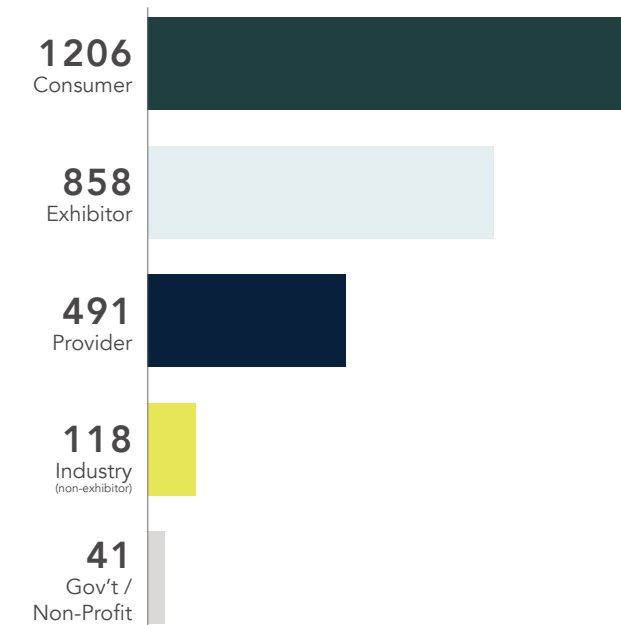
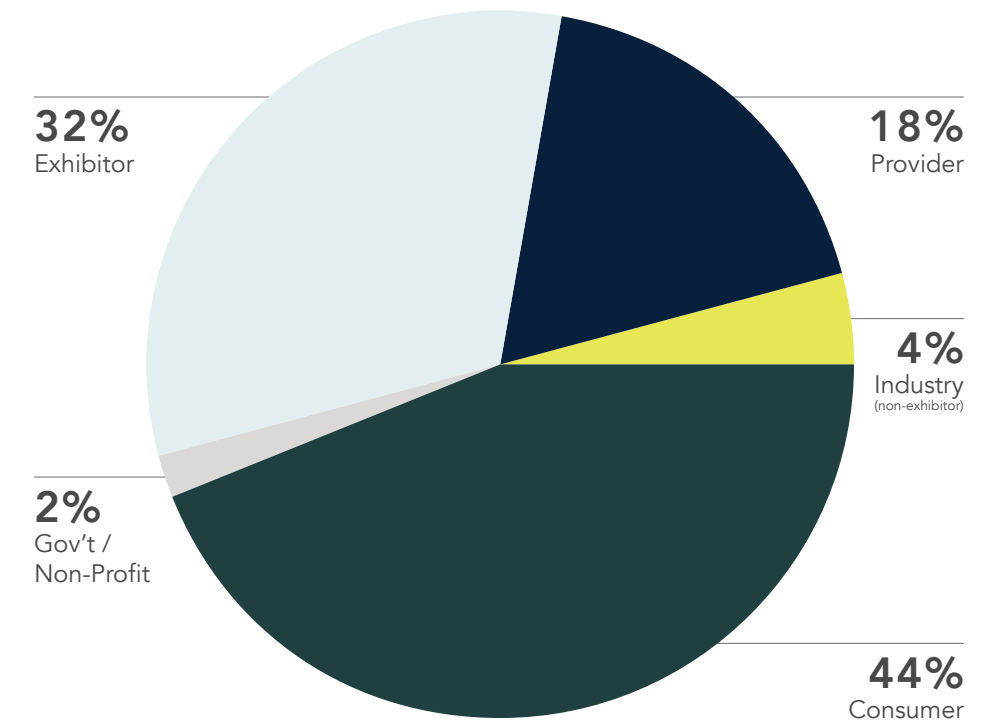
NHF Bleeding Disorders Conference (BDC) is the national education event of the year for the combined audiences of people with bleeding disorders and their families, healthcare providers, industry representatives and government agencies. Unique among annual meetings of national voluntary health agencies, NHF brings together at one conference these varied, but connected, constituencies. The Bleeding Disorders Conference is one of the methods NHF uses to deliver prevention education messages to the bleeding disorders community.

NHF's Bleeding Disorders Conference continues to grow and is becoming more complex to plan. With each new BDC under our belt, it is essential to review current policies and procedures and adapt, when necessary, so that we remain the single largest meeting in the bleeding disorders community in the USA.

Registration

Attendance 2,714 participants. There were 50 exhibitor groups (10 or more attendees) that registered for BDC, which represented approximately 858 registrations. Once again, all group coordinators were asked to make an appointment just prior to the start of the conference so that they could verify the names of all their guests/ attendees and ensure that all necessary documentation was included in their attendee kit. These appointments were held in a meeting room at the Marriott World Center on October 9 and 10, 2018.

Conference Attendance:
2,714

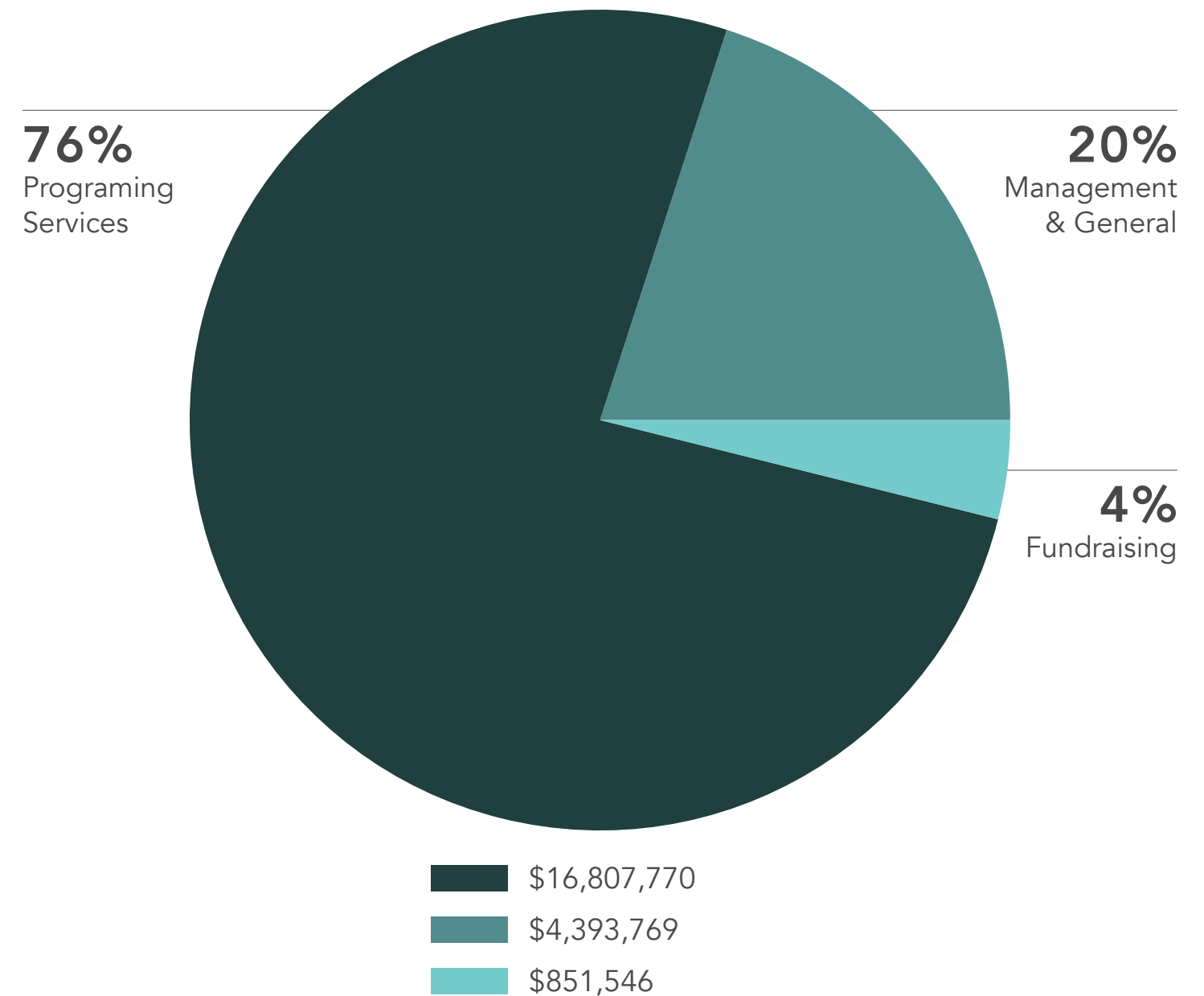


Audited Revenue

The complete financial statements, from which this financial summary is derived, have been determined to present fairly, in all material respects, the financial position of the National Hemophilia Foundation as of December 31, 2018, in conformity with generally accepted accounting principles. A complete set of audited financial statements for the year ended December 31, 2018 and the 990 are available at www.hemophilia.org.



Audited Expenses



86.5% of revenue comes from pharmaceutical companies

The ending net assets for 2018 was \$21,995,439

*Numbers based on 2018 audited financial statements



NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders