ANNUAL REPORT 2019

NATIONAL HEMOPHILIA FOUNDATION

for all bleeding disorders

WE SERVE PEOPLE AND FAMILIES WHO LIVE WITH HEMOPHILIA, VON WILLEBRAND DISEASE, AND RARE BLEEDING DISORDERS, AND THE HEALTHCARE PROFESSIONALS AND CHAPTERS WHO PROVIDE TREATMENT, CARE, AND SUPPORT.

NHF 2019 BY THE NUMBERS

BUILDING GRASSROOTS ADVOCACY

1,500+
community members trained as advocates for their states

450+
came to Capitol Hill to advocate for people with bleeding disorders

IMPROVING CARE THROUGH RESEARCH

9
fellowships and grants given to increase knowledge of bleeding disorders

EDUCATING PEOPLE TO LIVE HEALTHY LIVES

4,500+
people and families with bleeding disorders provided education and training

SUPPORTING CHAPTERS

$138,000
given in grants to build chapter capacity

SPREADING THE WORD

152
In-person workshops for 2,900 + participants

50,000+
monthly visitors to hemophilia.org

VictoryforWomen.org
7200+ visitors

32 families attended NHF's BDC through Connections for Learning grants

27
young community members trained in youth leadership

NYLI
National Youth Leadership Institute

STEPS FOR LIVING TRAINING
NHF trained **20** participants in the English track and **15** in the Spanish track in 2019.

86
attendees at the Rare Bleeding Disorders Converence

MEDICAL INFORMATION
Educated 22,000+ healthcare providers on gene therapy and emergency department management

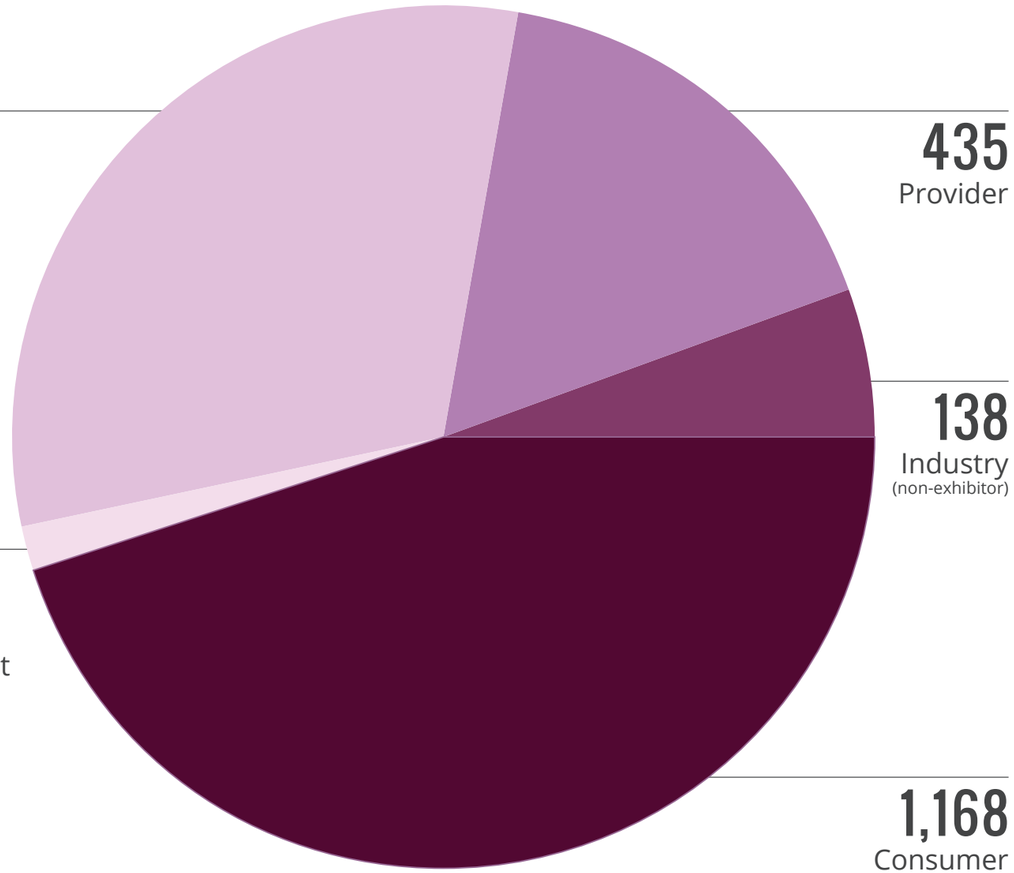
INHIBITOR EDUCATION

646
attendees at national summit.

58
attendees at *Viviendo con Inhibidores*

2019 BLEEDING DISORDERS CONFERENCE

CONFERENCE ATTENDANCE: 2,590



WE EDUCATE

IN-PERSON WORKSHOPS

NHF brings high-quality in-person education to local chapter or HTC events through our Education for Empowerment and Collaborating in Care workshops. Our workshops cover all aspects of living with a bleeding disorder, from advocating for care in the emergency room to relationships and disclosures to gene therapy and are available in English and Spanish. We also continued our commitment to healthy lifestyles with two nurse-led workshops, "Half-Life in Your Life," and "It's Not Too Late to Save Your Joints," as well as a PT-led workshop, "Playing It Safe: Bleeding Disorders, Sports & Exercise."

BLEEDING DISORDERS CONFERENCE

Every year, we bring together nearly 3,000 people with bleeding disorders, family members, chapter staff, healthcare providers and industry partners for a three-day educational conference that offers education and support for the bleeding disorders community. With over 60 educational sessions for patients, families, and chapters, and accredited tracks for physicians and researchers, nurses, social workers, and physical therapists, NHF provides the bleeding disorders community has the information and resources they need to thrive personally and professionally.

HANDI

Whether it's a family with a new diagnosis of a bleeding disorder, a young adult looking for information on healthcare insurance, or a student researching bleeding disorders, or a healthcare provider seeking the latest research on treatment, HANDI, NHF's information resource center is here to help. Our HANDI staff answers requests for information on topics ranging from hemophilia, von Willebrand disease, other inheritable bleeding disorders, health insurance, women with bleeding disorders, inhibitors, and navigating school issues.

STEPS FOR LIVING

Steps for Living, a life stage education program that combines online educational content with in-person workshops to assist those affected by bleeding disorders and their families not only in the first years of a child's life, but throughout the various stages of his/ her/their development. NHF's Steps for Living Training teaches chapter and HTC staff and HTC staff how to facilitate NHF's workshop curriculum for their local events.

INHIBITOR EDUCATION

NHF's Inhibitor Education Summits are three-day conferences tailored to serve the unique needs of families affected by hemophilia with an inhibitor. These summits provide specialized education and support for families as well as their support network. In 2019, NHF held three National Inhibitor Summits and one Spanish-language summit, *Viviendo con Inhibidores*, NHF also launched a new campaign including videos to promote annual inhibitor testing.

RARE BLEEDING DISORDERS EDUCATION

NHF is committed to serving the entire bleeding disorders community, including those with rare factor deficiencies such as factor VII, X, XIII deficiencies, and Glanzmann's thrombasthenia, and platelet disorders. NHF debuted its first Rare Bleeding Disorders Conference in 2019, held in conjunction with NHF's BDC, allowing families with these ultra-rare disorders to receive specialized education while also finding support and connection with the wider bleeding disorders community.

HEMAWARE MAGAZINE

HemAware magazine, NHF award-winning publication, is a lifestyle magazine for people with bleeding disorders. It offers in-depth profiles of leaders in the community, provides insights into the physical and emotional challenges faced by people living with bleeding disorders, and explores the latest discoveries in treatment and care.

STEPS FOR LIVING

176,000 +
sessions

BETTER YOU KNOW

1,200
assessed

85%
scored at risk for a bleeding disorder.

HANDI

answered **1,126** requests for information

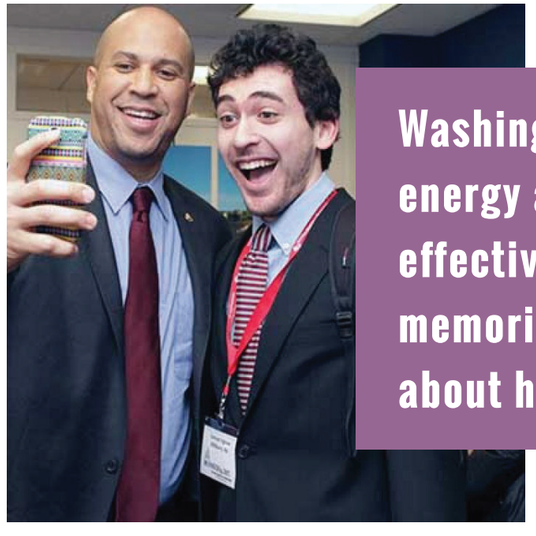
WE ADVOCATE

NATIONAL ADVOCACY EMPOWERMENT PROGRAM (NAEP)

The bleeding disorders community has a long history of advocacy, and access to affordable, quality healthcare continues to be NHF's top advocacy priority in 2019. Our annual advocacy event, Washington Days, drew more than 500 volunteers to Capitol Hill to advocate for the patient protections included in the Affordable Care Act (ACA), and federal funding for the nationwide network of Hemophilia Treatment Centers, and funding for bleeding disorders research at the National Institutes of Health. All of our volunteer advocates are given training on the issues, deepening their understanding of grassroots advocacy and the impact telling their story can have on the legislative process.

STATE-BASED ADVOCACY COALITIONS

Our State-based Advocacy Coalitions (SBACs) comprise 17 NHF chapters and coalitions, working together to create sustainable year-round local advocacy programs, train volunteer advocates, and respond to challenges facing the bleeding disorders community in states, particularly around access to care.



Washington Days is a whirlwind of energy and excitement. Thankfully, effective advocacy doesn't rely on memorizing lots of facts and figures about healthcare policy.

WE EMPOWER

UNITE FOR BLEEDING DISORDERS WALKS

NHF's Unite for Bleeding Disorders Walks, held at 40 NHF chapters across the country, bring together community members, friends, and coworkers for a day to raise awareness and funds for people with bleeding disorders.

BLEEDING DISORDERS AWARENESS MONTH

Since 2016, NHF has been at the forefront of raising national recognition for Bleeding Disorders Awareness Month. In 2019, NHF chapters successfully advocated for official state recognition of Bleeding Disorders Awareness Month in 25 states.

VICTORY FOR WOMEN

NHF's online community for women with bleeding disorders, Victory for Women, is a place where women can share their experiences, express themselves, ask questions, and celebrate the victory of standing up and being heard.

UNITE FOR BLEEDING DISORDERS WALKS

40+ WALKS

15,000+ SUPPORTERS

$2.5M RAISED FOR EDUCATION PROGRAMS AND SERVICES

Back when I was diagnosed, I started to go to conferences, and I met a lot of people with bleeding disorders. It made me realize there are so many others out there with issues just like mine. That awareness motivated me to be the best version of myself and to be an example for people who have rare diseases

Taylor D. Flake-Lawson

WE ELEVATE

CHAPTERS OF EXCELLENCE

NHF's chapters are where our community comes together. NHF works to ensure that our chapters are strong, sustainable, and able to bring the programs and support services that are so necessary to the community. That's why NHF created the Chapters of Excellence program. To earn a chapter of excellence award, the chapters must work to the high standards and best practices evaluated through the rigorous chapter review process. Thirty-five chapters earned Chapter of Excellence Awards in 2019 in the areas of programs and services, advocacy and public policy, fundraising and development, governance and board leadership and volunteer management, and NHF partnership.

NATIONAL YOUTH LEADERSHIP INSTITUTE

NHF's National Youth Leadership Institute (NYLI) fosters the development of youth leaders, enhancing their skills in communication, advocacy, facilitation, leadership, and personal development. The two-year program allows members to focus on one of three areas: outreach, advocacy, and nonprofit development. NYLI members assist chapters with youth programming, lead educational sessions, serve as junior board members, lead youth advocacy efforts, and encourage kids to become involved with their chapter and the bleeding disorders community.

CHAPTER SUPPORT

NHF provides support to its local chapters by giving grants to support programs and services, training in fundraising, volunteer recruitment and management, advocacy, and board development. In 2019, NHF gave $138,000 in chapter support.

WE REACH OUT

BETTER YOU KNOW

NHF's Better You Know, developed in partnership with the Centers for Disease Control and Prevention (CDC), is a bilingual initiative designed to guide women with an undiagnosed bleeding disorder to the information and resources they need to receive an accurate diagnosis and live healthy, full lives. At BetterYouKnow.org, women can access a risk assessment tool, learn how to prepare for doctor's visits, and explore additional resources.

GUÍAS CULTURALES

NHF's Guías Culturales are culturally intelligent volunteers who act as "guides" to diverse members of the community, helping those members access services and support.

La parte más gratificante de ser una Guía Cultural es conectarse con tantas familias hispanas y entregar información en español le llena de alegría.

The most rewarding part of being a Guía Cultural is to connect with so many Hispanic families and delivering the information in Spanish fills him with lots of joy.

WE IMPROVE CARE

MYBDC

NHF launched its community-powered registry, MyBDC, to capture the experiences of people with bleeding disorders and their family members. This longitudinal program will help researchers understand what it really means to live with a bleeding disorder and how current treatments, therapies, and policies affect the community. Enrollment began in March 2019; by the end of 2019 there were 488 people enrolled, 270 affected with an inherited bleeding disorder.

PROVIDER EDUCATION

In collaboration with Medscape Education, NHF offered seven educational programs for healthcare providers reaching over 22,000 learners through online offerings on gene therapy for hemophilia and emergency department management for continuing education credit and issuing more than 6,000 certificates. We also launched new webinars, specifically for bleeding disorders on topics such as women's issues, and von Willebrand disease.

MASAC

NHF's Medical and Scientific Advisory Council (MASAC), physicians, scientists, and other medical professionals with a wide range of expertise on bleeding disorders, issues recommendations and advisories on treatment, research and other general health concerns for the bleeding disorders community. The following are the documents that were approved by the council in 2019:

MASAC Document #256
MASAC Recommendation for Liver Biopsies in Gene Therapy Trials for Hemophilia

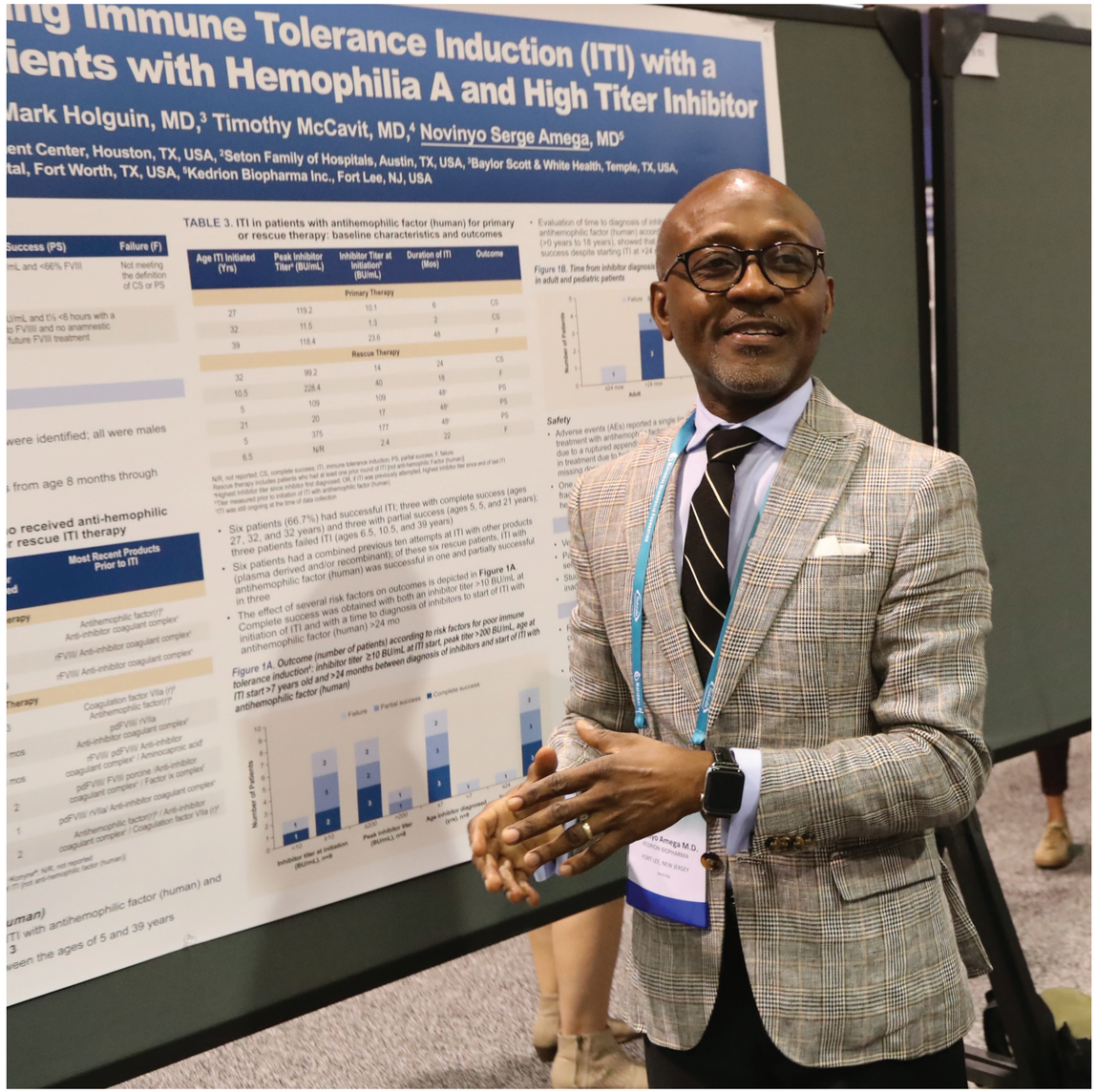
MASAC Document #257
Guidelines for Emergency Department Management of Individuals with Hemophilia and Other Bleeding Disorders

PAIN STUDY

The MASAC Pain Initiative Taskforce completed analysis of the first-ever multi-disciplinary HTC survey evaluating pain management practices. Recommendations based upon the results were submitted to MASAC, approved by the NHF board of directors, and published.

NHF supports the doctors, nurses, social workers and physical therapists who provide care for people with bleeding disorders by providing opportunities for clinical training and supporting research by the comprehensive care team.

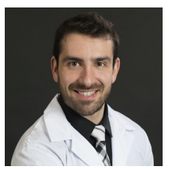
NHF SUPPORTS THE DOCTORS, NURSES, SOCIAL WORKERS AND PHYSICAL THERAPISTS WHO PROVIDE CARE FOR PEOPLE WITH BLEEDING DISORDERS BY PROVIDING OPPORTUNITIES FOR CLINICAL TRAINING AND SUPPORTING RESEARCH BY THE COMPREHENSIVE CARE TEAM.



NHF'S PROVIDER FELLOWSHIPS

NHF-TAKEDA CLINICAL FELLOWSHIP

The NHF-Takeda 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.



2018 to 2019

Hanny Al-Samkari, MD
Boston Children's Hospital/Harvard University

Dr. Al-Samkari received his medical degree from Washington University in St. Louis, where he was elected to AOA (Alpha Omega Alpha is a national medical honor society). 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. As an NHF-Takeda Clinical Fellow, he 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).

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 received 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 (NIH) 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

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.

Lydia Johnson, RN, BSN

Cardinal Glennon
Children's Hospital

EMS Provider Education Regarding Bleeding Disorders and the Treatment Required in an Emergency

People with bleeding disorders are often connected to and with various community members such as local and national foundation/associations, their home health company/nurses, pharmaceutical representatives, etc. While people are often encouraged to wear Medic Alert ID, they do not know anyone in the EMS system who may be caring for the/their loved one in case of emergency. Through this project, EMS providers will receive education regarding bleeding disorders and the treatment required in an emergency. This project will seek to connect the HTCs with the EMS systems locally and regionally to coordinate the education with the orders EMS professionals must have in order to provide care.

SOCIAL WORK EXCELLENCE FELLOWSHIP

The Social Work Excellence Fellowship provides support for a social worker conducting research into psychosocial bleeding disorders care.

Maria Iannone, MA, LPC

The University of Arizona

The Prevalence of Depression and Anxiety in Children with Coagulopathies Being Treated at a Hemophilia and Thrombosis Center

The hemophilia and thrombosis centers in Tucson and Phoenix would like to join efforts to make a meaningful contribution to our understanding of the mental health profile of our pediatric population. This knowledge could contribute to a more tailored approach when designing clinics and programming, and, by identifying mental health issues, inform the development of targeted interventions. We hope to look at the prevalence of depression and anxiety in children with bleeding disorders, including hemophilia, von Willebrand Disease, and other congenital coagulopathies. We expect that the existence of a chronic health condition could affect a child's psychosocial development. We are also aware of the critical impact of family culture, so our survey will include data on the mental health of participant's primary caretakers as well as other significant socio-economic markers (e.g. family income, parent's marital status, legal issue, etc.).

PHYSICAL THERAPY EXCELLENCE FELLOWSHIP

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.

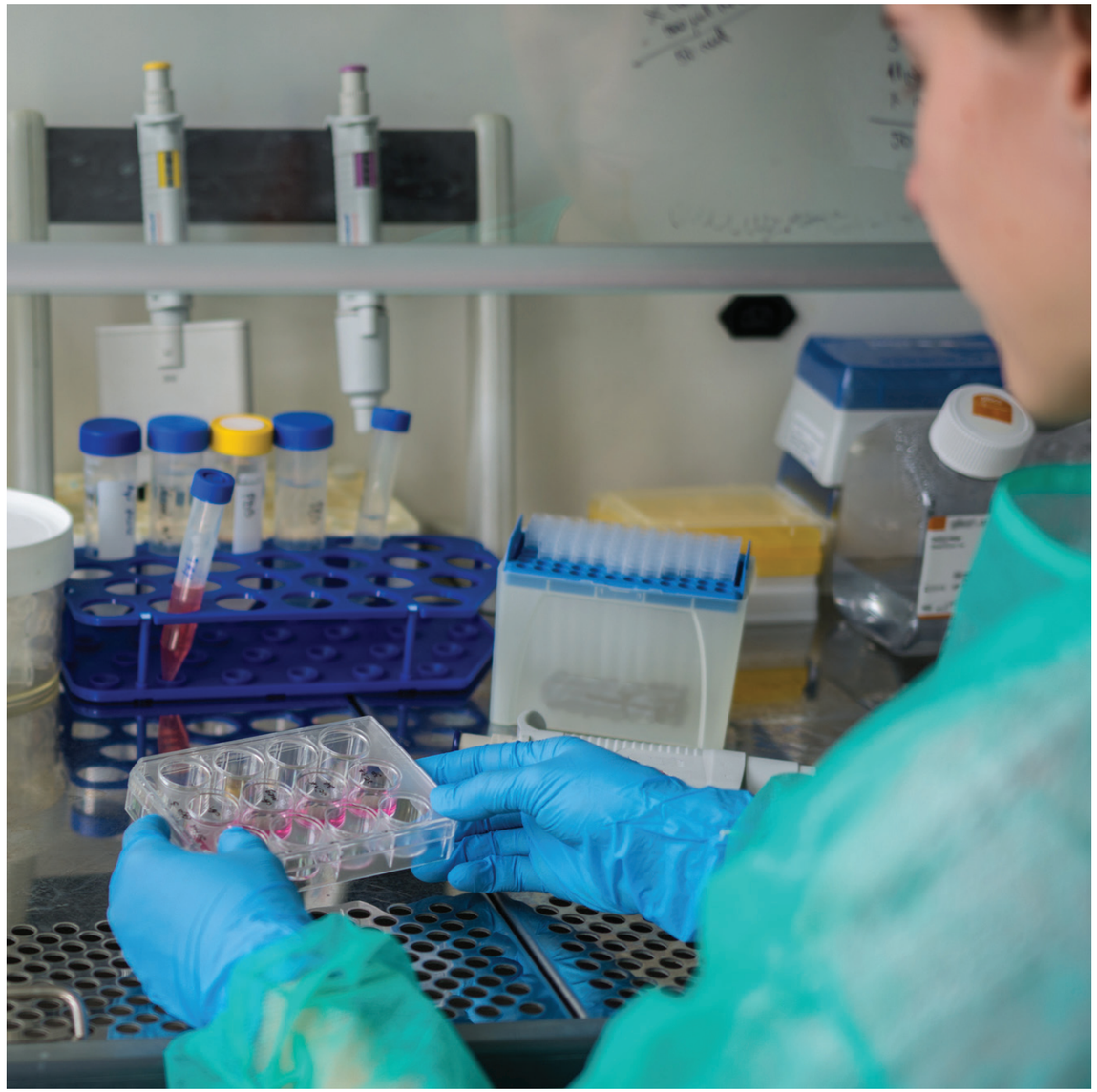
Lena Volland, PT, DPT, OCS

University of St. Augustine

Joint Characteristics, Biomechanics, and Neuromotor Control during Gait in Patients with Hemophilia

Hemophilia causes repetitive bleeding episodes throughout the musculoskeletal system, primarily into joints, such as knees and ankles. This leads to significant joint damage resulting in increased pain reproduction, decreased functional abilities, such as walking, and negatively impacts quality of life. Traditionally the extend of joint damage has been examined via clinical assessments, such as the Hemophilia Joint Health Scores, x-rays, MRIs, and more recently musculoskeletal ultrasound (MSKUS). However, these modalities fail to establish the global impact of joint damage on the entire body of a person with hemophilia and their functional abilities. Analyzing joint motion and forces acting upon the joint during walking has been a widely established technique to gain understanding of abnormal three- dimensional movements and is a key factor in clinical decision making-processes. With the overall goal of establishing better treatment approaches for persons with hemophilia it is vital to understand the underlying functional joint limitations. Therefore, the purpose of this study is to investigate characteristics of damaged joints, joint motion and control as well as forces acting upon the joint during walking in persons with hemophilia.

DRIVEN BY RESEARCH, TREATMENT FOR BLEEDING DISORDERS IS IMPROVING. WE CONTINUE TO INVEST IN RESEARCH INTO BETTER TREATMENTS AND CURES FOR ALL INHERITABLE BLEEDING DISORDERS.



NHF'S RESEARCH GRANTS

JUDITH GRAHAM POOL POSTDOCTORAL RESEARCH FELLOWSHIP

The Judith Graham Pool Fellowship offers $52,000/year for two years to a post-doctoral candidate for basic science and pre-clinical research.

2019 to 2021

Seema Patel, PhD
Emory University

The Epitopes Recognized in the Early Immune Response to Factor VIII

Exposure to factor VIII (fVIII) can lead to formation of neutralizing anti-fVIII IgG (inhibitors) that can mitigate the therapeutic benefits of fVIII by impeding activity. However, no strategies currently exist to block inhibitor development, likely due to the lack of understanding of the immune response to fVIII. While the antibody response is polyclonal and multiple exposures are required to develop inhibitors, whether this response begins polyclonal or a single epitope is responsible for initiating the response remains unclear. Our preliminary data demonstrates that anti-fVIII IgM is detectable after 2 fVIII exposures and those detected following multiple exposures react primarily to the C1 domain of fVIII. Accordingly, the current proposal will identify the initial epitope(s) recognized by fVIII specific B cells, as this is a critical step in understanding the mechanisms of the humoral immune response to fVIII. We hypothesize that the early IgM response to fVIII is directed to a single immunodominant B cell epitope within fVIII. To accomplish this, we will test the following aims. Aim 1: Characterize the epitope specificity of the anti-fVIII IgM response directed to the C1 domain. Using hybridoma technology, we have identified hybridoma clones producing IgM antibodies reactive to the C1 domain of fVIII. To map the specific epitopes of these antibodies, we will sequence and clone the variable regions of the IgM onto an IgG backbone to create anti-C1 domain IgG that will then be used in our well-established epitopemapping assay. Aim 2: Define the epitope specificity of the early IgM response to fVIII. Following each fVIII exposure, fVIII reactive hybridomas will be created and domain specificity of anti-fVIII IgM producing clones will be determined. fVIII reactive IgM will be purified and epitope mapped as in Aim 1. Successful completion of these studies will provide for the first time a clear idea of the epitope specificity of the early immune response to fVIII.

2019 to 2021

Calvin Stephens, PhD
Stanford University

Preclinical Development of Nuclease-free Gene Editing for Lifelong Treatment of Bleeding Disorders

Recent clinical trials established that adeno-associated virus (AAV) can mediate years-long correction of hemophilia B (HB) in adult patients. However, these gene replacement approaches are not applicable to young patients due to a loss of therapeutic gene expression. Loss of therapeutic expression results partially from vector dilution as a young patient grows. Treating young patients may help avoid inhibitor antibodies and other considerable symptoms, while reducing the life-long cost burden for a patient treated with protein replacement therapy. As such, approaches to overcome loss of gene expression in young patients have been investigated, such as the use of integrating viral vectors. These vectors, such as retro- and lentiviral vectors, achieve stable expression due to genomic integration of the therapeutic gene. However, long-term correction is also accompanied by genotoxicity and can result in oncogenesis. Thus, there is a substantial need for vectors capable of genomic integration with minimal genotoxicity. To this end, the Kay lab recently conceived a unique AAV-based approach to integrate therapeutic genes into genomes in a targeted fashion, without exogenous promoters and nucleases (deemed AAV-HR). Importantly, AAV-HR targets the Albumin gene, with minimal disruption by targeting integration to the 3' end of the gene and permits production of therapeutic genes without an exogenous promoter. Despite establishing proof-of-principle that AAV-HR can therapeutically treat HB, the underlying mechanisms governing AAV-HR remains to be elucidated. In this proposal we propose independent experiments with the shared goal of developing AAV-HR to become a one-shot curative treatment for bleeding disorders. To do so, we hypothesize that investigation into inherent limitations of the system, methods to influence extrinsic factors, and application of the system to human cells in vivo will allow fulfillment of the significant potential this platform technology embodies.

2019 to 2021

Azhwar Raghunath, PhD
Bharathiar University

Identification of a Potential Novel Role for Factor IX Using a Zebrafish Model

Hemophilia is an inherited bleeding disorder with life-threatening bleeding and other additional complications, including joint pathology. Hemophilia A and B are caused by defects in clotting factors VIII (F8) and IX (F9), respectively. Prophylaxis using recombinant factors has been the major treatment option for many years. However, a significant number of patients develop alloantibodies and inhibitors against these factors, and this has led to novel treatments for hemophilia. One concern is whether treatment with new non-factor therapies may result in the loss of other potential functions of coagulation factors not related to clotting. For example, there are some data suggesting that F8 is necessary for proper bone development. Our laboratory has established multiple zebrafish models of coagulation disorders using genome editing technologies and shown that there is a high degree of genetic and functional conservation with humans. As a result of an ancestral gene duplication event in zebrafish, 30-40% of the genome is duplicated, resulting in sub- and neo-functionalization. For some genes this may result in separation of functions that are combined in one gene in mammals, making it easier to discern these additional functions. F9 has two paralogs, F9a and F9b, and our preliminary data indicate that only one of them (F9b) has clear clotting activity. This suggests that F9a may reveal a previously unknown function for mammalian F9. We will dissect the roles of these factors, which may identify new pathogenic mechanisms and/or therapeutic targets for patients affected by hemophilia.

CAREER DEVELOPMENT AWARD

The Career Development Award provides $70,000/year for three years to a mid-level investigator at a federally funded hemophilia treatment center.

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

The Bridge Grant provides $125,000 over 12-18 months to an experienced physician investigator who has applied for a National Institutes of Health (NIH) grant and was scored but not funded and needs additional data to resubmit.

2019 to 2020

Janice Staber, MD
University of Iowa

Understanding of a Neurophenotype in Hemophilia A

Understanding the impact of CMBs and neuroinflammation will elucidate the mechanism underlying neuropathogenesis and could alter treatment approaches for PWH. This will reduce costs of the disease and improve quality of life, improve outcomes, and decrease mortality.

INNOVATIVE INVESTIGATOR AWARD

The Innovative Investigator 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.

2019 to 2010

Andrew Yee, MD
Baylor College of Medicine

Functional Interpretation of Genetic Variants in von Willebrand Factor

Evaluation for von Willebrand disease (VWD) involves multiple laboratory assays of von Willebrand factor (VWF) functions. Diagnosis of a specific VWD subtype is often confirmed with genetic analysis which provide critical guidance for therapeutic management. However, variants of uncertain significance (VUS) can complicate the interpretations of the genetic analysis, obscure diagnostic outcomes, and cloud clinical decision making. To clarify the clinical significance of VUS in VWF, we propose an innovative approach that defines comprehensive sets of VWF missense variants according their functional capacity – binding to the platelet VWF receptor, glycoprotein Ibα (GPIbα), or to coagulation factor VIII (FVIII). We will construct and assess comprehensive libraries of single missense variants in the GPIbα-binding or FVIII-binding domains of VWF for their respective binding capacities. Using next generation DNA sequencing with technologies that link proteins to their encoding DNA (i.e., phage display and mammalian cell display), we will quantify each variant's ability to alter VWF function. These results will stratify all variants according to their capacity to strengthen or weaken VWF/platelet interactions (i.e., define the likelihood of variants to cause VWD subtype 2B or 2M, respectively) and identify all variants that inhibit FVIII- binding (i.e., VWD subtype 2N). Preliminary results demonstrate that our methods recapitulate the etiology underlying specific VWD subtypes. Application of our functional, genotype-to-phenotype approach for all single missense variants within the platelet-binding or FVIII-binding domains will provide comprehensive knowledge that may guide clinical management of VWD more precisely.

2019 to 2010

Tam Perry, PhD
Wayne State University School of Social Work

Navigating Time and Space: Experiences of Aging with Hemophilia

For the first time in human history, individuals with hemophilia are living beyond their 30s and 40s. Aging persons with hemophilia (APWH) have witnessed unprecedented treatment changes including administering treatment at home due to factor concentrate availability (1970s) and accessing synthetic factor (mid 1990s) developed to counter dependency on a knowingly contaminated blood supply (1980s) which resulted in HIV and hepatitis contraction for some. Given these contexts, this cohort continues to face unique challenges as they age with hemophilia and other conditions. The proposed study provides knowledge unavailable currently to the field, informed by established gerontological theories that will provide insight into the strategies and time horizons of individuals that are not only living with hemophilia, but productively aging with the disease. These frameworks (Selection, Optimization with Compensation and Socioemotional Selectivity Theory) will provide perspectives to examine four domains of health and well-being: 1) self-care, 2) social networks, 3) the meaning of home and 4) contributions. This collaborative project involving academic and clinical social workers will holistically capture what it means to age for persons with hemophilia. This project also focuses on California's unique healthcare landscape available to this population. Study aims are: 1) To examine the lived experiences and time horizons of APWH in order to characterize this community and 2) To enhance service delivery practice after examining needs, facilitators and barriers experienced by APWH. This project will use a range of qualitative methods including secondary data analysis of already rich interviews, original data collection of semi-structured interviews, fieldnotes and focus groups with a non-probability sample of 40 California residents to include 30 individuals with hemophilia aged 50 or older and 10 service providers. Transcribed data will be analyzed for emergent themes.

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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
Sandy Springs, GA

Hawaii Chapter,
National Hemophilia Foundation**
Ewa Beach, HI

Hemophilia of Iowa
Iowa City, 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

*Period ending December 31, 2019

** Chapters owned by The National Hemophilia Foundation

OUR WORK IS MADE POSSIBLE THANKS TO THE GENEROSITY OF OUR DONORS. WE THANK EVERYONE WHO DONATED IN SUPPORT OF THE BLEEDING DISORDERS COMMUNITY.

A COMPLETE LIST OF DONORS CAN BE FOUND AT HEMOPHILIA.ORG

AUDITED REVENUE

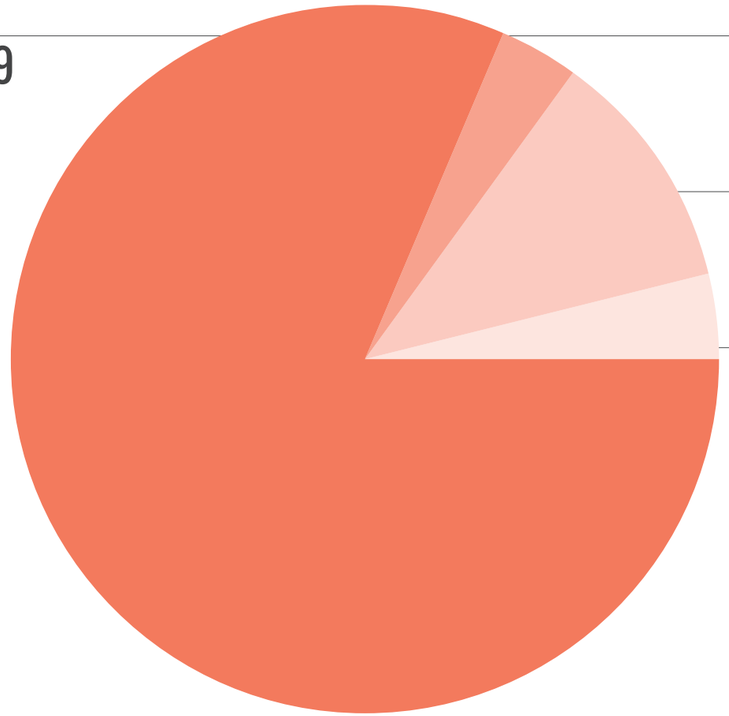
The complete financial statements, from which this financial summary is derived, have been determined to present fairly, in all material respects, the financial position of the National Hemophilia Foundation as of December 31, 2019, in conformity with generally accepted accounting principles. A complete set of audited financial statements for the year ended December 31, 2019 and the 990 are available at www.hemophilia.org.

24,234,309
Grants and Contributions

1,064,749
Program Services

3,223,763
Investments

1,076,278
Special Events



AUDITED EXPENSES

74%
Programing Services

Health Education and Training
7,201,966

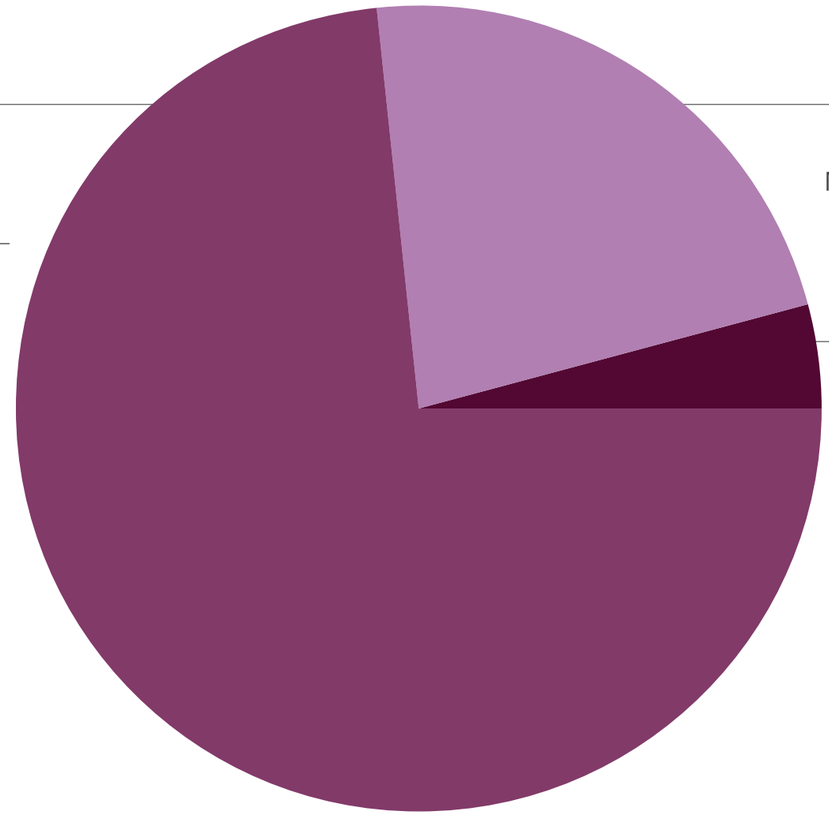
Community Services
4,653,521

Chapter Services
1,476,077

Research
3,111,164

22%
Management & General

4%
Fundraising



$16,442,728
$5,019,380
$882,780

87.7% of revenue comes from pharmaceutical companies The ending net assets for 2019 was $29,249,650

*Numbers based on 2019 audited financial statements