2016 VASCULITIS FOUNDATION
RESEARCH FUNDING REPORT

ON THE COVER

ABOUT BRANDON HUDGINS

Brandon Hudgins is both a professional athlete and a patient with vasculitis. His record-breaking performances on the track are all the more remarkable because he has simultaneously achieved his goals despite two relapses from granulomatosis with polyangiitis (GPA/Wegener's) over the last few years.

PURSUING HIS DREAMS WHILE INSPIRING OTHERS

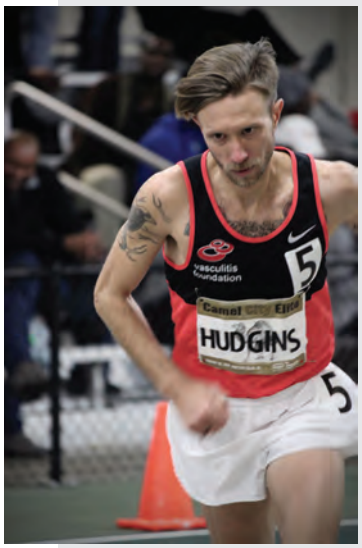
On August 7, 2015, Brandon became the 449th American to run a mile in less than 4-minutes. In 2016, he capped his running season by qualifying for the 2016 Olympic Trials in Eugene, Oregon. Although he didn't qualify for the Olympics, Brandon's real victory was not allowing the disease to hold him back. Moreover, Brandon continues his goal of advocating for vasculitis awareness and research through his work with the **Victory Over Vasculitis: VF Team Brandon Campaign**.

In this campaign, Brandon is an ambassador for the VF through interviews with national media, and with personal visits such as meeting with fifty children in an after-school running club in New York City. He also is the coach and motivator for a team of more than 300 vasculitis patients for the online **VF Team Brandon Challenge** where he publishes a bi-weekly email message designed to inspire and promote an active lifestyle despite the illness.

The Vasculitis Foundation thanks Brandon for his dedication and commitment to raising awareness about vasculitis, and especially the need for continuing research to help patients manage the disease.

"Research is the driving force behind progress. Until we have a cure for all forms of vasculitis, it is imperative that we continue to fund research to improve people's chances at living a normal life. For years the Vasculitis Foundation has been at the forefront of funding research on vasculitis. My goal as an athlete who battles a vasculitis disorder, is to promote awareness about the disease and help the Vasculitis Foundation raise money for research. I know I am a miracle of modern medicine. Without the research that has lead to current treatment options, I wouldn't be able to pursue my dream of Olympic glory. We aren't done though until we can improve the quality of life for all patients who have been diagnosed with vasculitis."

BRANDON HUDGINS



VASCULITIS FOUNDATION
RESEARCH PROGRAM UPDATE

We are pleased to provide this report on the Vasculitis Foundation's Research and Fellowship Programs to our patients, families, physicians, researchers, and, especially, to our many donors. The VF Research program broke the $2,000,000 mark in funding in February 2016, made possible by the generous donations of our members and extended vasculitis community. Since 2002, the Vasculitis Foundation has funded 42 studies in Australia, Austria, Canada, England, Germany, Ireland, Italy, Netherlands, and the United States.

This report details our accomplishments since our Second International Vasculitis Research Consensus Conference held in Chicago, Illinois in 2012. During the conference, participants evaluated programs and explored new areas of inquiry that the Foundation could fund over the next five years.

Thank you for your generous support.

The Vasculitis Foundation acknowledges an educational grant for support of this report from Genentech, Inc., and Biogen, Idec.

THE VF FELLOWSHIP PROGRAM

Our newest and most exciting initiative (and a direct result of the 2012 Consensus Conference) is the formal establishment of the VF Fellowship Program, designed to increase the number of vasculitis experts to ensure that all regions of the world have health care professionals trained in the diagnosis and treatment of vasculitis.

The VF Fellowship Program provides the opportunity for one- or two-year tracks designed to support the training of physician-scientists who wish to gain clinical expertise in vasculitis, and who may also aspire to pursue an investigational research career in this field. Fellowships are conducted through Vasculitis Centers where there has been a track record of training individuals in the specialty of vasculitis. The training will lay the foundation for developing experts and establishing centers in areas of the world lacking vasculitis experts.

Providing financial assistance for fellowships is crucial to enable talented trainees to develop experience and expertise in the diagnosis and management of vasculitis and to learn how to conduct high-quality clinical and translational research in vasculitis.

MEET OUR FELLOWS

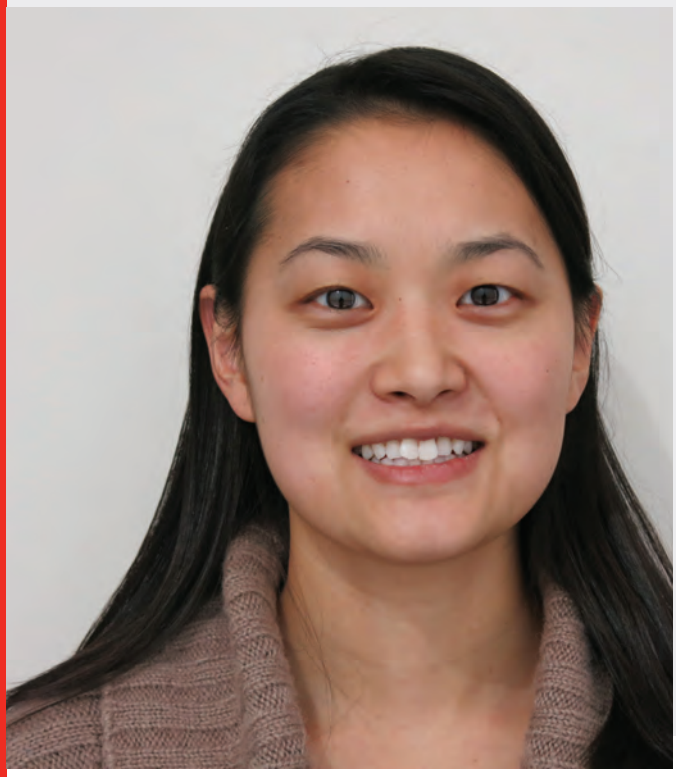
> "As is so often the case, figuring out the basic mechanisms will help us understand other diseases. The more we know, the more avenues of treatment we can pursue. Hopefully, this research will help us identify those at greatest risk of this complication and guide us toward intervention."

ELIZABETH BRANT, M.D., 2013 - 2015 VF FELLOW

Dr. Elizabeth Brant trained as a nephrologist at the University of North Carolina and completed her vasculitis fellowship under the direction of Drs. Ronald Falk and Patrick Nachman in the UNC Kidney Center. Her research focused on deep venous thromboembolism (VTE) in patients with ANCA-associated vasculitis.

Dr. Brant presented her research at the June 2015 International Vasculitis Symposium, reporting that the incidence of VTE is 8 to 10 percent in ANCA patients – an incidence rate 50 to 100 times greater than in the general population. The results showed a clear association with patients who have MPO and PR3 disease. These clots have a 50 percent chance of causing a pulmonary embolus (PE) and subsequent pulmonary hypertension and right ventricular dysfunction, all of which can be life-threatening.

After completing her fellowship in 2015, Dr. Brant joined the Dartmouth-Hitchcock Medical Center's Division of Nephrology as an assistant professor of medicine. She practices general nephrology and is developing a clinical and research vasculitis program.



> The VF fellowship makes an enormous difference for researchers early in their careers. As a young investigator, studying a rare disease such as vasculitis is challenging, particularly in this financial climate. Obtaining this fellowship provided me the needed training and mentoring to continue contributing to this field and to benefit ultimately the many patients diagnosed with vasculitis. I also had the privilege of working with world-renown experts in the field who trained me in both clinical management and research investigation.

RENNIE RHEE, M.D., MSCE, 2014 - 2016 VF FELLOW

Dr. Rennie Rhee, a rheumatologist, completed her fellowship in June at the University of Pennsylvania, under the mentorship of Dr. Peter Merkel. Dr. Rhee plans a career as a patient-oriented researcher in vasculitis.

Her research interests include the impact of early diagnosis and treatment to improve the survival of end-stage renal (kidney) disease in ANCA-associated vasculitis. Specifically, she is looking at end-stage renal disease, death, or a relapse in five years for patients diagnosed between 1985 and 2009. Her initial data supports that we are indeed extending end-stage renal disease-free survival primarily because of earlier diagnosis of the disease.

Dr. Rhee also found a higher rate of cardiovascular disease – the main cause of death after the first year of diagnosis – in these patients. There was also a higher rate of cancer in these patients, most of which were non-melanoma skin cancers.

NEW VASCULITIS FELLOWSHIP OPPORTUNITIES

THE VCRC-VF FELLOWSHIP

In 2015, the Vasculitis Foundation partnered with the Vasculitis Clinical Research Consortium (VCRC) to combine resources and expand fellowship education. This partnership provides five, one-year fellowships to be conducted at VCRC centers around the world between 2015 and 2020. The VCRC-VF Fellowship is generously sponsored by Dr. Jeffrey Fishbein, a member of the VF Board of Directors, and his family through their annual Chicagoland Golf Outing.

> "Equipped with the knowledge I've gained from the fellowship, I'll be able to devote my career to greater research in the field of vasculitis and the development of educational and awareness programs for members of the community."

MEDHA SOOWAMBER, M.D.
2015 - 2016 VCRC-VF FELLOW
UNIVERSITY OF TORONTO, CANADA

Dr. Medha Soowamber trained in rheumatology at Toronto's Mount Sinai Hospital and completed her VCRC-VF fellowship under the mentorship of Drs. Simon Carette and Christian Pagnoux. She intends to devote her career to research in the field of vasculitis and the development of education and awareness materials for both patients and healthcare providers.

During her fellowship, Dr. Soowamber gained knowledge, expertise, and experience in diagnosing and managing patients with vasculitis. The training also gave her the opportunity to develop a deeper understanding of current vasculitis research being conducted.

Dr. Soowamber's first research project was an observational study in which she compared the clinical characteristics and outcomes in biopsy negative versus biopsy positive data for patients with giant cell arteritis. In a second descriptive study, she explored data on lung involvement across all the primary systemic vasculitides.

> This fellowship provides me the opportunity to improve the care provided to patients with vasculitis, I am fortunate to have many excellent mentors within the field of vasculitis and glomerulonephritis and to be among the many previous successful recipients of this fellowship.

JENNIFER RODRIGUES, M.D.

2016 - 2017 VCRC-VF FELLOW

Dr. Jennifer Rodrigues, a nephrologist, will conduct her VCRC-VF Fellowship under the mentorship of Dr. Michael Walsh at McMaster University. Her interest in nephrology began as a teaching assistant in physiology and grew during the early clinical rotations of internal medicine training where one of her first patients had kidney failure requiring dialysis.

Dr. Rodrigues will focus on glomerulonephritis, diseases that affect the filters of the kidneys, the second most common cause of kidney failure requiring dialysis. The kidneys are frequently affected by vasculitis, and while there are treatments, including immunosuppressive and anti-inflammatory steroid medications, to initially control the disease and prevent flare-ups, physicians often don't know how long patients should remain on these drugs.

During her fellowship, Dr. Rodrigues will conduct a pilot clinical trial examining whether long-term low-dose prednisone is effective at preventing relapse of vasculitis and its impact on kidney function and patients' quality of life. Opinions about whether low-dose medications, particularly prednisone, work in vasculitis vary dramatically around the world so the information from this study will change how vasculitis is treated, no matter what the data shows.

THE GARY S. HOFFMAN VASCULITIS FOUNDATION FELLOWSHIP
CLEVELAND CLINIC CENTER FOR VASCULITIS CARE AND RESEARCH
CLEVELAND, OHIO

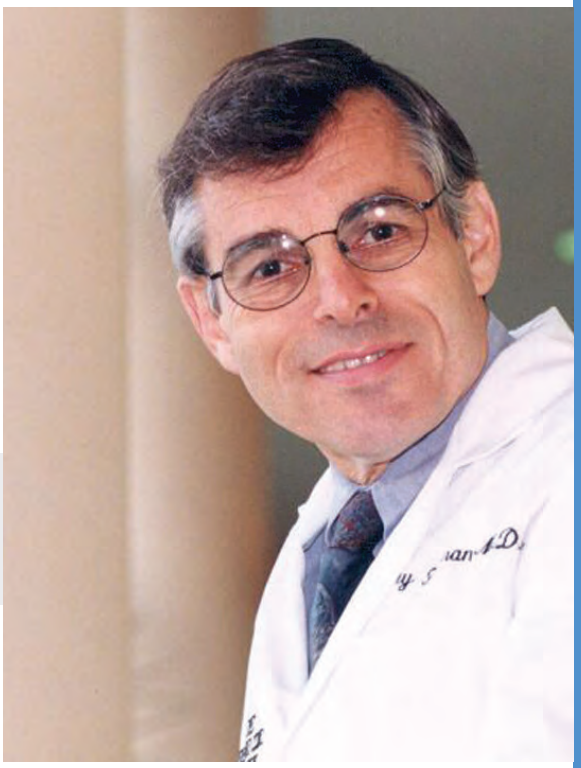
Our most recent fellowship honors Dr. Gary S. Hoffman, who retired from patient care at the Cleveland Clinic in 2014. Dr. Hoffman had a profound influence on the lives of people with vasculitis and is recognized as a world leader in vasculitis clinical care and research.

Dr. Hoffman served as the Harold C. Schott Chair of Rheumatology and Immunologic Diseases at the Cleveland Clinic and founded the Cleveland Clinic Center for Vasculitis Care and Research in 1992. He also founded the International Network for the Study of Systemic Vasculitides, which organized the first international multicenter studies for diagnosis and management of vasculitis.

The successful collaboration with other international vasculitis researchers has allowed large cohorts of patients to be studied at the same time. These numbers are critical to achieving statistically significant results, a particular challenge in studying rare diseases.

Dr. Hoffman generously donated his time as a VF Medical Consultant and helped guide the organization for almost 30 years. He served on the VF Board of Directors from 2010 to 2013 and was instrumental in helping expand the VF Research Program to support fellowships.

Upon the announcement of his retirement, the VF Board of Directors chose to honor Dr. Hoffman by partnering with the Cleveland Clinic Center for Vasculitis Care and Research to fund a yearlong fellowship.



GARY S. HOFFMAN, M.D., M.S.

DRS. HIRO TAMAKI, GARY S. HOFFMAN AND CAROL A. LANGFORD

HIROMICHI "HIRO" TAMAKI, M.D.

2015 GARY S. HOFFMAN VASCULITIS FOUNDATION FELLOW 2016 - 2017

Dr. Hiromichi Tamaki graduated from the University of Tokyo and completed his internal medicine residency at the University of Hawaii followed by a rheumatology fellowship at the Cleveland Clinic. In July 2016 he began the Gary S. Hoffman Fellowship at the Center for Vasculitis Care and Research under the mentorship of Dr. Carol Langford, director of the center. Dr. Tamaki plans to return to Japan for his clinical practice and research after the completion of the second year of his fellowship.

Dr. Tamaki is interested in venous thromboembolism (VTE), a prevalent multifactorial health condition associated with significant morbidity and mortality. Population-based epidemiological studies have revealed an association between systemic autoimmune diseases and deep venous thrombosis.

Dr. Tamaki's research is looking at the safety of simultaneous use of methotrexate and low dose trimethoprim-sulfamethoxazole in patients with granulomatosis with polyangiitis (GPA, Wegener's), microscopic polyangiitis and eosinophilic granulomatosis with polyangiitis (EGPA, Churg-Strauss Syndrome).

Dr. Tamaki shared, "Vasculitis is a chronic disease. We have better treatment strategies and are better able to manage the disease, but the treatments are still not perfect. I hope that I can contribute to improving the quality of life of patients with vasculitis. The most rewarding moment is always when patients feel better, and they can come back to their lives. I love to hear that they are once again enjoying what they used to enjoy before the illness started."

THE VF RESEARCH PROGRAM

"I was a practicing neurosurgeon when I developed atypical Takayasu arteritis in 2001 and became disabled. Through my unique vantage point as both physician and patient, I thought I could best contribute to the Vasculitis Foundation by volunteering to serve on the research committee that I now chair. Research is our hope for the future, and it is exciting to work to fund studies that will help us achieve our goal. The immune system is still poorly understood, and I believe there will be great advances in the near future in this field."

CHRIS COX-MARINELLI, M.D.
CHAIR, VF RESEARCH COMMITTEE

OUR RESEARCH

Through the generosity of our donors, the Vasculitis Foundation continues to be the world's largest private funding source for research covering the broad spectrum of the vasculitides. The VF Research Program annually funds $150,000 in new research through one or two-year grants. Our grants allow researchers to obtain initial data that may then be used to compete for larger grants from the National Institutes of Health or other such institutions.

Once a year the Vasculitis Foundation issues a call for research applications. Physicians and researchers on our scientific advisory committee generously donate their time and expertise to evaluate the applications. Using a standardized scale, reviewers score each application on relevancy to the field of vasculitis, study methods, the likelihood of producing a result, and researchers' qualifications. Comments are encouraged to supplement the evaluations. The VF Research Committee then compiles all the reviews, and chooses the top applications to recommend to the VF Board of Directors for final funding approval.

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Over the past five years we have funded studies in basic science, clinical and diagnostic science, etiology, laboratory and diagnostic findings, and treatment outcomes in vasculitis patients. From frontline genomic techniques to immunotherapy and biomarkers, we have maximized our opportunities to advance the field and, hopefully, lead to a "cure".

Here's a deeper look at a few research accomplishments achieved with your support.

IDENTIFICATION OF NOVEL WEGENER'S GRANULOMATOSIS SUSCEPTIBILITY GENES

2004 - 2016

KATHERINE SIMINOVITCH, M.D.
MOUNT SINAI HOSPITAL
UNIVERSITY OF TORONTO, CANADA

In 2004, Dr. Katherine Siminovitch was well-known as an expert in the genetics of inflammatory bowel disease. She suspected that granulomatosis with polyangiitis (GPA, Wegener's) would also include gene variations as risk factors and that the genetics would be "complex," meaning that many genes would each contribute a small amount to the risk of having GPA.

With support from the Vasculitis Foundation and the participation of doctors and patients throughout North America, she was able to obtain DNA from nearly 500 patients and measure over 300,000 regions of common genetic variation using the most advanced "gene chip" of the time. By that time, however, it had become clear that detection of most genes associated with autoimmune diseases required even larger numbers of patients.

Fortunately, a few other researchers had had the same vision, and neither genetic data nor DNA itself has an expiration date. Through collaborations with Drs. Peter Merkel, Sharon Chung, Paul Monach, and Jeff Edberg, Dr. Siminovitch and her lab at the University of Toronto have made and continue to make important contributions to understanding the genetics of GPA, particularly the HLA region – the "hot spot" of extraordinary genetic variation that underlies every autoimmune disease – and the similarities to and differences from the genes involved in other autoimmune inflammatory diseases.

With rapidly improving technology to identify more gene sequences at lower cost, more advanced "bioinformatics" to accurately analyze massive and complex sets of data, growing interest of researchers to engage in collaborations nationally and internationally, and the interest of ever larger numbers of patients to donate blood samples for this effort without any prospect of personal gain, greater understanding of multiple forms of vasculitis can be expected through genetic studies in the next 5-10 years.

VASCULITIS PREGNANCY REGISTRY: IMPACT OF VASCULITIS ON REPRODUCTION

2010 - 2014

MEGAN E.B. CLOWSE, M.D.
DUKE UNIVERSITY, DURHAM, NORTH CAROLINA

Many patients are diagnosed with vasculitis during their reproductive years, and the effect of vasculitis and its treatment on fertility and the health of both the mother and the fetus during pregnancy are important concerns for many young patients. As of 2010, although all doctors would agree that questions about future pregnancy were among the most important for young women, there was almost no information available. By that year, Dr. Megan Clowse, a rheumatologist at Duke University, had made important contributions to the study of pregnancy in patients with lupus, but she also recognized the great need for information to assist patients with vasculitis, and that such research in these rare diseases would require a novel approach.

With support of a grant from the Vasculitis Foundation, Dr. Clowse developed a survey about reproductive history for both women and men with vasculitis and posted it on the Patient Contact Registry of the Vasculitis Clinical Research Consortium (VCRC). In a short time, 350 women and 114 men had responded. The reassuring news was that most pregnancies proceeded without complications, but the rate of pregnancy loss was 50% higher than it had been prior to diagnosis.

The success of this study has led to an even more ambitious project among the same collaborators: a survey of woman with vasculitis with questions asked while they are pregnant, conducted by Dr. Clowse using the Vasculitis Patient-Powered Research Network, a federally funded joint effort of the Vasculitis Foundation and the VCRC.

DEVELOPMENT OF A UNIFORM HISTOLOGY SCORING SYSTEM FOR SMALL VESSEL CNS VASCULITIS IN CHILDHOOD AND ADULTHOOD

2012 - 2016

SUSANNE M. BENSELER, M.D., M.S.C.E., PHD.
THE UNIVERSITY OF SICK KIDS, TORONTO, CANADA
ALBERTA CHILDREN'S HOSPITAL, CALGARY, CANADA

Dr. Susanne Benseler, chief of paediatric rheumatology at the University of Calgary, has spent most of her career as an expert in vasculitis affecting the brain in children. It might be more appropriate to say "the" expert in this rare condition, since her first study in 2006 included far more patients than all previous published reports combined, and her dedication to maximizing awareness, defining subtypes of the disease that might have different causes and require very different treatments, and perfecting those treatments has never waned since then.

In 2012, when Dr. Benseler was at the University of Toronto, the natural next step in her effort to improve definition of subtypes of "primary childhood central nervous system vasculitis" was to include rigorous analysis of brain biopsies, a study supported by a grant from the Vasculitis Foundation. Even in large cities such as Toronto and Calgary, it would be impossible for a few institutions to assemble enough biopsies for such a study, but the tradition of multi-center collaboration in vasculitis and in pediatric rheumatology has made such studies possible.

Dr. Benseler has also established an international registry of children with the full range of inflammatory diseases of the brain (BrainWorks) and is an integral member of the Canadian network for the study of vasculitis (CanVasc) and of A Registry for Childhood Vasculitis: e-entry (ARChiVe). ARChiVe was initiated by a pilot study funded by a VF grant to Dr. David Cabral in 2005.

RHO-KINASE ACTIVITY IN LARGE VESSEL VASCULITIS
2014 - 2016

LINDSAY S. LALLY, M.D.
HOSPITAL FOR SPECIAL SURGERY
NEW YORK CITY

The diagnosis of giant cell arteritis (GCA) is clear when biopsy of the temporal artery shows vasculitis. However, around 20 percent of patients will have negative biopsies. One of the most difficult tasks of a doctor specializing in vasculitis is determining, among the many elderly patients who develop symptoms that might be due to GCA, which ones truly have this disease.

The need for better diagnostic tests is huge, because GCA is relatively common and treatment begins with high doses of prednisone – which could be dangerous if used for a different disease and always causes unpleasant side effects even when used correctly.

In 2014, Dr. Lindsay Lally, a rheumatologist at the Hospital for Special Surgery in New York City, was awarded a VF grant to study whether activation of a pathway called ROCK in the cells of the artery wall, which she had found preliminary evidence for in GCA, could also be detected in some biopsies in which no inflammation to indicate GCA was visible under the microscope.

Indeed, more than 90 percent of negative biopsies from patients thought to have GCA tested positive for the ROCK pathway, whereas fewer than half of biopsies from patients thought not to have GCA tested positive. This finding indicated that negative testing for ROCK might, if confirmed in larger future studies, give a doctor confidence that a patient does not have GCA.

Support for Dr. Lally's research by the Vasculitis Foundation is also notable because it aided her transition to become an independent physician-scientist with expertise in vasculitis, with the mentorship of Dr. Robert Spiera. This grant thus achieved the goals of the VF Fellowship Program in addition to facilitating a specific research project.

APPENDIX A: VF RESEARCH AWARDEES

2016 AWARDEES

MARK LITTLE

PROFESSOR/CONSULTANT OF NEPHROLOGY, ST. JAMES HOSPITAL, DUBLIN, IRELAND

This project focuses on development of a new biomarker test in the diagnosis and monitoring of crescentic glomerulonephritis (CGN), a severe form of inflammatory kidney injury. The researchers will track urinary sCD163 during the first few months of treatment for CGN to investigate its usefulness as a biomarker to help us decide when to switch to less intensive maintenance treatment.

PETER HEERINGA

PROFESSOR, UNIVERSITY MEDICAL CENTER, GRONINGEN, THE NETHERLANDS

Prof. Heeringa is studying the autoantibodies that cause the inflammation in ANCA-associated vasculitis (AAV). The researchers hypothesize that the autoantibodies in AAV differ from "normal" antibodies with respect to the amount of sugar groups attached and therefore impacts their function. This research will guide efforts to refine laboratory tests for AAV diagnosis and patient monitoring, and in the future, reveal whether interfering with the sugar coating of autoantibodies can be used for therapeutic applications.

ROBERT MICHELETTI, M.D.

ASSISTANT PROFESSOR, DERMATOLOGY AND MEDICINE, UNIVERSITY OF PENNSYLVANIA, PHILADELPHIA

Dr. Micheletti will conduct the ARAMIS Trial for patients with chronic, recurrent skin vasculitis. ARAMIS will test three medications—dapsone, colchicine, and azathioprine—used commonly but never proven to work for vasculitis. Patients will receive one of the three medications titrated to a goal dose and duration designed to evaluate its effectiveness. The results from these two study stages will be combined to determine which medication is best at decreasing skin lesions and improving patient symptoms. If the study is successful, doctors will for the first time have high-quality scientific evidence to guide how they treat skin vasculitis.

2015 AWARDEES

JONATHAN CHOY, PH.D.

MOLECULAR BIOLOGY AND BIOCHEMISTRY, SIMON FRASER UNIVERSITY, BURNABY, CANADA

Dr. Choy is studying the potential mechanisms by which IL-6 affects immune responses in giant cell arteritis (GCA) by examining the relationship between IL-6 expression levels and features of T cell responses that are improperly controlled during the development of immune-mediated diseases. The potential identification of correlations between IL-6 levels and altered properties of T cells will provide insight into how IL-6 alters immune function in GCA and will form the basis for future studies for patients and will have implications for the management of GCA.

SUSAN S. JICK, M.D., DSc.

BOSTON COLLABORATIVE DRUG SURVEILLANCE PROGRAM, BOSTON UNIVERSITY SCHOOL OF PUBLIC HEALTH, BOSTON, MASSACHUSETTS

Dr. Jick is leading a large two-year epidemiological study investigating the longitudinal morbidity and mortality of patients with vasculitis. This critical information remains scanty in the literature. We have few research-based insights into the impact of these diseases on patients. It is of utmost importance to have this information when applying for NIH/other research funding; looking for government support (eligibility for disability and Medicare, for example); and, reimbursement from insurance companies.

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RENATE KAIN, M.D., PH.D.

CLINICAL INSTITUTE OF PATHOLOGY, MEDICAL UNIVERSITY OF VIENNA, AUSTRIA

Despite recent advances in treatment, ANCA-associated vasculitis (AAV) still causes serious morbidity especially after the acute injury has been controlled, emphasizing the need for better ways to monitor disease activity and personalize immunosuppressive therapy. Dr. Kain is examining why autoantibodies to LAMP-2 and MPO and PR3 are found so commonly together, and whether they synergize to cause injury.

DAVIDE MARTORANA, Bsc, PH.D.

DIAGNOSTIC DEPARTMENT, MEDICAL GENETICS UNIT, UNIVERSITY HOSPITAL OF PARMA, ITALY

Eosinophilic granulomatosis with polyangiitis (EGPA, Churg-Strauss Syndrome) is a multisystem disorder, belonging to the small vessel anti-neutrophil cytoplasmic antibody ANCA-associated vasculitides (AAVs). Specific aims of this study are to develop newer potential EGPA diagnostic and prognostic biomarkers by exome sequencing and computational modeling, clinical subset stratification of EGPA patients on the basis of the genetic study and to ascertain the diagnostic-prognostic role of the whole findings. The ultimate goal is the development of personalized medicine, in order to find rational means to optimize drug therapy, with respect to the patient's genotype, to ensure maximum efficacy with minimal adverse effects and saving costs for the health system.

PARAMESWARAN NAIR, M.D., PH.D., FRCP, FRCPC

MCMASTER UNIVERSITY, ONTARIO, CANADA

Eosinophilic granulomatosis with polyangiitis (EGPA, Churg-Strauss Syndrome) is a serious rare autoimmune condition that affects multiple organs, primarily lung, peripheral nerves, heart, gastrointestinal tract and skin. The research team is studying the presence of antibodies against MPO, their relation to severity of the disease and a possible involvement of EPX in the underlying autoimmune mechanism. This novel idea has therapeutic implications in that these autoantibodies may determine the severity of disease and allow us to better manage and treat these patients.

2014 AWARDEES

KIMBERLY LIANG, M.D.

UNIVERSITY OF PITTSBURGH, PENNSYLVANIA

Dr. Liang investigated new methods for following patients with large vessel vasculitis using ultrasonography. Angiography has been the standard, so a simpler, noninvasive study is extremely valuable to giant cell arteritis and Takayasu's arteritis patients. The study distinguished between acute and active disease from the inactive scarring of previous vascular damage.

SUSANNE SCHINKE, M.D.

UNIVERSITY OF LÜBECK, GERMANY

Dr. Schinke received funding to study a distinct pattern revealing a specific dysregulation of miRNA that only occurs in GPA patients to understand granulomatous and necrotizing vasculitis. The research forms the basis for future development of miRNA-based therapeutic interventions. This was the first analysis that attempted to correlate GPA-associated miRNA expression patterns in tissue with the ones in serum and urine and was the first description of a miRNA (miR-184) that seems to regulate the expression of the GPA autoantigen PRTN3.

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CAROLYN T. THORPE, PH.D., MPH

SCHOOL OF PHARMACY, UNIVERSITY OF PITTSBURGH, PENNSYLVANIA

Dr. Thorpe is conducting a multi-year study to determine annual utilization and costs across the full range of healthcare services for systemic vasculitis patients enrolled in fee-for-service Medicare in the U.S., and to characterize variations in costs and mortality by patient socio-demographic, clinical, and geographic factors. In addition, Dr. Thorpe is using online surveys and validated measures with patients, family members, and friends, to describe the nature and impacts of informal caregiving (physical, emotional, social, and financial) and to examine predictors of greater subjective caregiving burden and financial impact.

2013 AWARDEES

PAUL BROGAN

PROFESSOR OF VASCULITIS, UCL GREAT ORMOND STREET INSTITUTE OF CHILD HEALTH, LONDON, ENGLAND

Dr. Brogan's team looked at the therapeutic impact of plasma exchange in patients with granulomatosis with polyangiitis (GPA, Wegener's) to determine which of the microparticles removed during plasma exchange could be responsible for causing the inflammation. They examined a subpopulation of white blood cells to see if they can target the specific ones that trigger the vasculitic reaction. The results could lead to a therapeutic intervention for GPA.

RITU CHAKRAVARTI, PH.D.

LERNER RESEARCH CENTER, CLEVELAND CLINIC, OHIO

Dr. Chakravarti studied giant cell arteritis and Takayasu's arteritis associated with immune-mediated injury that targeted modified aorta14-3-3 proteins. The goal was to identify a surrogate measure of disease activity and provide insight into pathways that may become targets for new therapies. Dr. Chakravarti's research is now supported by a Scientist Development Grant from the American Heart Association until December 31, 2017.

CAROLA VINUESA, PH.D.

THE AUSTRALIAN NATIONAL UNIVERSITY, ACTON, AUSTRALIA

Dr. Vinuesa's team investigated how particular genes control the expression of particular mRNA and protein, and as a result, control the function and development of particular immune cells and how these mechanisms lead to vasculitis, with the aim of identifying new treatment targets.

2012 AWARDEES

SHARON CHUNG, M.D., MAS

UNIVERSITY OF CALIFORNIA - SAN FRANCISCO

Dr. Chung conducted a search for rare genetic variations in GPA patients by "exome" sequencing. The preliminary data generated by the project were instrumental in Dr. Chung securing a five-year K23 award from the National Institute of Arthritis, Musculoskeletal, and Skin diseases. The aims of the K23 award are to broadly assess the contribution of genetic mutation, epigenetic dysregulation, and aberrant gene expression to disease susceptibility and activity in GPA.

CORNELIA WEYAND, M.D., PH.D.

STANFORD UNIVERSITY, CALIFORNIA

Dr. Weyand's study focused on large vessel vasculitis with a special emphasis on giant cell arteritis. She examined how immune cells are activated in the first place; how they respond to activation and how immune activation translates into the injury in the blood vessel. The study gave special attention to a new type of immuno-modulatory drugs, called the JAK inhibitors, which can suppress cellular activation. Experiments explored the potential of such new inhibitors as a new treatment approach in GCA.

APPENDIX B: GLOSSARY OF TERMS

ANCA: Anti-neutrophil cytoplasmic antibodies. The abbreviation is pronounced just like the last name of the singer, Paul Anka. These antibodies are found in patients with some forms of vasculitis, particularly granulomatosis with polyangiitis (GPA, Wegener's), microscopic polyangiitis, and the Eosinophilic granulomatosis with polyangiitis (EGPA, Churg-Strauss Syndrome).

Basic Research: The goal of basic research is broad – to learn the biological rules of life. The applications of basic research are similarly wide-ranging, including: New and improved medicines, Diagnostic tests, Body-imaging technology and better organ donor matching.

Cutaneous: Relating to, existing on, or affecting the skin.

Cyclophospamide: An alkylating agent used in combination with corticosteroids (such as prednisone) to treat severe cases of vasculitis.

Cystoscopy: The inspection of the interior of the bladder using a lighted tubular endoscope, inserted through the urethra. The major reason for performing cystoscopy in patients with vasculitis is to screen for bladder injury caused by cyclophosphamide.

Dermis: The layer of the skin located below the epidermis, containing nerve endings, sweat and sebaceous glands, and blood and lymph vessels. Small and medium-vessel forms of vasculitis affect the dermis and sometimes the layer just below the dermis; the subcutaneous fat.

Epidermis: The protective outer layer of the skin.

ESR: Erythrocyte Sedimentation Rate is the rate at which red blood cells settle out in a tube of blood under standardized conditions; a high rate usually indicates the presence of inflammation.

Etiology: The cause or origin of a disease.

Glomerulonephritis: Inflammation in the kidney, characterized often by decreased production of urine and by the presence of blood and protein in the urine.

IgA: Immunoglobulin A. A specific subcategory of antibodies (which all individuals have). For reasons that are not understood, IgA deposits within the blood vessels of the skin, joints, kidney, and GI tract in Henoch-Schönlein purpura, leading to vasculitis.

Ischemia: A decrease in the blood supply to an organ, tissue, or body part caused by constriction or obstruction of the blood vessels.

Mimicker: A disease process that imitates or simulates another. For example, the lung lesions of granulomatosis with polyangiitis (GPA, Wegener's) may be mimickers of tuberculosis.

MRI Magnetic Resonance Imaging: Another fancy x-ray, similar to a CT scan. MRI scans also provide cross-sectional images of body organs. Because MRI technology involves the use of a large magnet, people with pacemakers, metallic aneurysm clips, and other metallic inserts are not eligible to have these studies.

Myeloperoxidase Abbreviated MPO: An enzyme found in many tissues and cells throughout the body. For reasons that are unknown, many patients with granulomatosis with polyangiitis (GPA, Wegener's) and microscopic polyangiitis make antibodies to this protein.

Prevalence: The total number of cases of a disease present within a given population at a particular time. The prevalence of giant cell arteritis in the United States, for example is estimated to be 160,000.

Proteinuria: The presence of excessive amounts of protein in the urine. Proteinuria is usually caused by damage to the kidneys.

Purpura: A condition characterized by small amounts of bleeding into the skin and mucous membranes that result in the appearance of purplish spots or patches.

Sclera: The white part of the eye. "Scleritis" is a type of inflammation that occurs in the sclera in some forms of vasculitis.

Stenosis: A constriction or narrowing of a blood vessel.

Subcutaneous: Underneath the skin. Some medications, for example, are injected under the skin.

Teratogenic potential: The risk of causing birth defects. Some medications are said to have "teratogenic potential".

Translational Research: The process of turning observations in the laboratory, clinic and community into interventions that improve the health of individuals and the public – from diagnostics and therapeutics to medical procedures and behavioral changes.

Thrombosis: A blood clot.

Uveitis: Inflammation within either the anterior (front) or posterior (back) part of the eye.

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VASCULITIS FOUNDATION GRANTS (2002 - 2016)

1. **Short-Term Cyclophosphamide Therapy in a Cohort of Over 200 Patients**
   Gary S. Hoffman, M.D., MS and Alexandra Villa-Forte, MD, MPH, The Cleveland Clinic Foundation

2. **Host-Microbial Interactions in Wegener's Granulomatosis: the Role of ANCA and S. Aureus Persistence**
   Robert Inman, M.D., University of Toronto, Canada

3. **The Role of Shear Stress in Neutrophil Proteinase-3 Expression and its Importance in Vascular Injury Sites in Wegener's Granulomatosis**
   Deborah Stearns-Kurosawa, Ph.D., Oklahoma Medical Research Foundation

4. **Analysis of NKG2D Expression on CD28- T-Cells and Non-Classical MHC-Class I Antigen Expression on Antigen-Presenting Cells as Clues to an Antigen-driven Process and New Targets of Treatment in Wegener's Granulomatosis**
   Wolfgang L. Gross, M.D., Ph.D., and Peter Lamprecht, M.D. University Hospital of Schleswig-Holstein, Campus Lübeck, and Rheumaklinik Bad, Bramstedt, Germany

5. **Identification of Novel Wegener's Granulomatosis Susceptibility Genes**
   Katherine Siminovitch, M.D., FRCP(C), ABIM, Mount Sinai Hospital, Canada

6. **Wegener's Granulomatosis and Microscopic Polyarteritis Case Control Study in Western Montana**
   Andrew Zeft, M.D., University of Washington

7. **Development of a Measure to Assess Patients' Adherence to Vasculitis Treatment Regimens**
   Robert DeVellis, Ph.D., University of North Carolina

8. **Environmental Factors in the Pathogenesis of Wegener's Granulomatosis**
   Daniel A. Albert, M.D., University of Pennsylvania

9. **Localized Wegener's Granulomatosis: Epidemiological, Clinical and Histopathological Characteristics**
   Peer Malte Aries, M.D., and Wolfgang L. Gross, M.D., Ph.D. University Hospital of Schleswig-Holstein, Campus Lübeck, and Rheumaklinik Bad Bramstedt, Germany

10. **A Pilot Project Towards Establishment of a US/ Canadian Diagnostic Registry of Children with Wegener's Granulomatosis and Related Vasculitides**
    David A. Cabral, MBBS, FRCP(C), BC Children's Hospital, Canada

11. **Renal Transplantation in Wegener's Granulomatosis: Mortality, Allograft Outcomes and Incidence of Malignancies from the United Network for Organ Sharing Database 1988-2005**
    Nadine Tanenbaum, M.D., Duke University

12. **Gene Expression Profile of Temporal Arteries of Giant Cell Arteritis**
    Rula Hajj-Ali, M.D., The Cleveland Clinic Foundation

13. **Identification and Characterization of Immunodominant, Conformational Epitopes of Antineutrophil Cytoplasmic Antibodies**
    Antje Mueller, Ph.D., Elena Csernok, M.D., Peter Lamprecht, M.D., University Hospital Schleswig-Holstein, Campus Lübeck, Germany

14. **ANCA Vasculitis: Autoimmune B Cell Dysregulation and its Clinical Impact**
    Patrick Nachman, M.D., University of North Carolina

15. **Expression and Activation of P38MAPK Isoforms in ANCA-associated Renal Vasculitis**
    Jochen Zwerina, M.D., Immunology, University of Erlangen, Germany

16. **Vasculitic T Cells in Giant Cell Arteritis**
    C.M. Weyand, M.D., Ph.D., Emory University

17. **An Investigation of the Role of the Innate Immune Response in Kawasaki Disease**
    Neil Bowles, Ph.D., University of Utah

18. **Investigating the Role of the Novel Th17 cells in the Pathogenesis of ANCA-associated Vasculitis and Their Potential for Immunomodulation**
    Alan Salama, MBBS, Ph.D., FRCP, Imperial College London, England

19. **PARP-1 in CSS-associated IL-10 Promoter Polymorphisms**
    Jianguo Liu, M.D., Ph.D., Saint Louis University School of Medicine

20. **Reproductive Health in Young Men and Women with Vasculitis**
    Megan E. B. Clowse, M.D., Duke University

21. **Classification and Diagnostic Criteria in Primary Systemic Vasculitis**
    Raashid Ahmed Luqmani, DM, FRCP(E), Oxford University, United Kingdom

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22. **Identification of Susceptibility Genes in Takayasu's Arteritis**
Amr H. Sawalha, M.D., University of Oklahoma Health Sciences Center

23. **Defining an Animal Model for the Study of Wegener's Granulomatosis**
Felipe Andrade, M.D., Ph.D., The Johns Hopkins University

24. **Exome Sequencing in Granulomatosis with Polyangiitis (GPA, Wegener's)**
Sharon Chung, M.D., MAS, University of California, San Francisco

25. **Investigating the Molecular Mechanism for IL-25-Driven Act1-dependent Eosinophilic Vasculitis**
Shadi Swaidani, Ph.D., Cleveland Clinic Lerner Research Institute

26. **Small Molecule Therapeutics in Giant Cell Arteritis**
C.M. Weyand, M.D., Ph.D., Stanford University School of Medicine

27. **Development of a Uniform Histology Scoring System for Small Vessel CNS Vasculitis in Childhood and Adulthood**
Susanne M. Benseler, M.D., MSCE, Ph.D., The Hospital for Sick Children, Canada

28. **Circulating Microparticles as Therapeutic Targets of Plasma Exchange in Antineutrophil Cytoplasmic Antibody ANCA-associated Vasculitis**
Paul Brogan, Professor of Vasculitis, UCL Great Ormond Street Institute of Child Health, London, England

29. **To Understand the Role of 14-3-3 in Giant Cell Arteritis**
Rita Chakravarti, Ph.D.
Cleveland Clinic Lerner Research Institute

30. **Disease-specific Dysregulation of MiRNA in Granulomatosis with Polyangiitis (GPA/Wegener's)**
Susanne Schinke, M.D., University of Lübeck and Klinikum Bad Bramstedt, Germany

31. **Impact of Healthcare Utilization and Informal Caregiving for Primary Systemic Vasculitis: A National Perspective**
Carolyn T. Thorpe, Ph.D., MPH, University of Pittsburgh

32. **Rho-kinase Activity in Large Vessel Vasculitis**
Lindsay S. Lally, M.D. and Robert F. Spiera, M.D., Hospital for Special Surgery

33. **Microbubble Contrast-Enhanced Vascular Ultrasonography: A Novel Method of Detecting Large Vessel Vasculitis**
Kimberly P. Liang, M.D., University of Pittsburgh

34. **The Role of MicroRNA in a Novel Mouse Model of Vasculitis**
Carola Vinuesa, M.D., MSc, Ph.D., Australian National University, Australia

35. **Immune Regulation by IL-6in Giant Cell Arteritis**
Jonathan C. Choy, Ph.D., Simon Fraser University, Canada

36. **Role of LAMP-2 in Tolerance to ANCA Antigens**
Renate Kain, M.D., Ph.D., Medical University of Vienna, Austria

37. **Longitudinal Study of Morbidity and Mortality in Vasculitis Patients**
Susan Jick, DSc, Boston University School of Public Health

38. **Exome Sequencing in Eosinophilic Granulomatosis with Polyangiitis (EGPA, Churg-Strauss Syndrome) Patients**
Davide Martorana, Ph.D., University Hospital of Parma, Italy

39. **Investigating Local Autoimmune Mechanism Underlying Eosinophilic Granulomatosis with Polyangiitis (EGPA, Churg-Strauss Syndrome)**
Parameswaran Nair, M.D., Ph.D., FRCP, FRCPC, McMaster University, Canada

40. **Rational Treatment of Glomerulonephritis using a Novel Urine Biomarker**
Mark Little, M.D., Professor/Consultant, Trinity Health Kidney Centre, Ireland

41. **Analysis of Glycosylation of Proteinase 3-ANCA IgG Variable Domains**
Peter Heeringa, Professor, University Medical Center, Groningen, the Netherlands

42. **A Randomized Multicenter Study for Isolated Skin Vasculitis (ARAMIS)**
Robert Micheletti, M.D., University of Pennsylvania

VASCULITIS
A FAMILY OF DISEASES

2012 Revised International Chapel Hill Consensus Conference Nomenclature of Vasculitides

LARGE VESSEL VASCULITIS (LVV)
Takayasu Arteritis (TAK)
Giant Cell Arteritis (GCA)

MEDIUM VESSEL VASCULITIS (MVV)
Polyarteritis Nodosa (PAN)
Kawasaki Disease (KD)

SMALL VESSEL VASCULITIS (SVV)
ANCA-Associated Vasculitis (AAV)
Microscopic Polyangiitis (MPA)
Granulomatosis with Polyangiitis (Wegener's) (GPA)
Eosinophilic Granulomatosis with Polyangiitis
(EGPA, Churg-Strauss Syndrome)

IMMUNE COMPLEX SVV
Anti-GBM Disease
Cryoglobulinemic Vasculitis (CV)
IgA Vasculitis (Henoch-Schönlein)(IgAV)
Hypocomplementemic Urticarial Vasculitis
(Anti-C1q Vasculitis)

VARIABLE VESSEL VASCULITIS (VVV)
Behçet's Disease (BD)
Cogan's Syndrome (CS)

SINGLE ORGAN VASCULITIS (SOV)
Cutaneous Leukocytoclastic Angiitis
Cutaneous Arteritis
Primary CNS Vasculitis
Isolated Aortitis

VASCULITIS ASSOCIATED WITH SYSTEMIC DISEASE
Lupus Vasculitis
Rheumatoid Vasculitis
Sarcoid Vasculitis

VASCULITIS ASSOCIATED WITH PROBABLE ETIOLOGY
Hepatitis C Virus-Associated Cryoglobulinemic
Vasculitis
Hepatitis B Virus-Associated Vasculitis
Syphilis-Associated Aortitis
Drug-Associated Immune Complex Vasculitis
Drug-Associated ANCA-Associated Vasculitis
Cancer-Associated Vasculitis

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