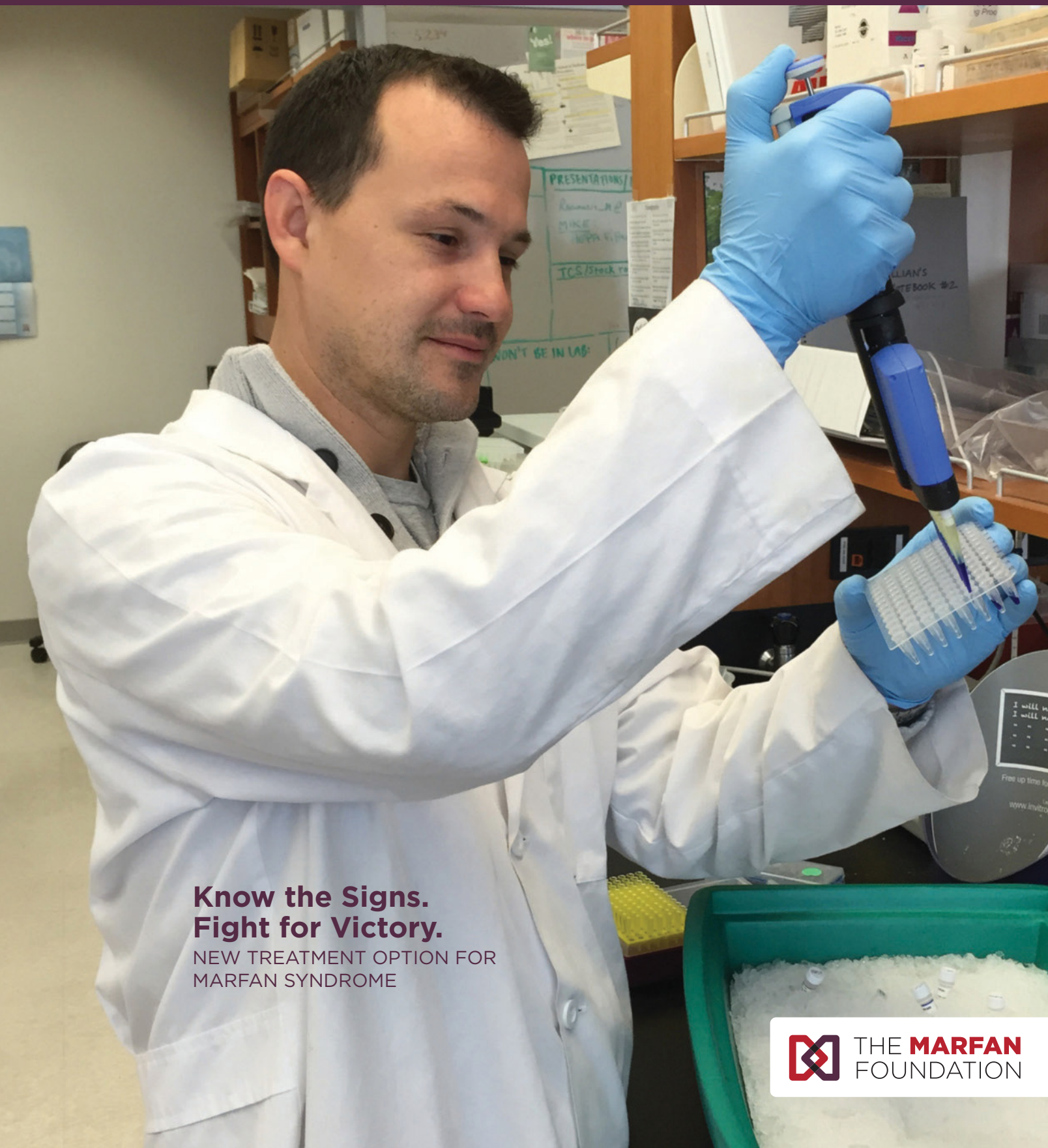
# CONNECTIVE ISSUES

WINTER 2015




## Know the Signs. Fight for Victory.

NEW TREATMENT OPTION FOR MARFAN SYNDROME

THE MARFAN FOUNDATION

THE **MARFAN** FOUNDATION

**The Marfan Foundation creates a brighter future for everyone affected by Marfan syndrome and related disorders. We work tirelessly to advance research, serve as a resource for families and healthcare providers, and raise public awareness. We will not rest until we have achieved victory.**

**Learn more and get involved at Marfan.org.**



## WHAT IS VICTORY?

Victory means something different to everyone in our community—the researchers and clinicians, the individuals and families, and the volunteers. What does victory mean to you? Write your definition on page 15 using a thick marker. Then take a photo with your sign and email it to us at publicity@marfan.org

**TOP LEFT** PRISCILLA CICCARIELLO, CHAIR EMERITUS, THE MARFAN FOUNDATION
**LEFT** DUKE CAMERON, MD, CARDIAC SURGEON, JOHNS HOPKINS

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CONNECTIVE **ISSUES**
WINTER 2015
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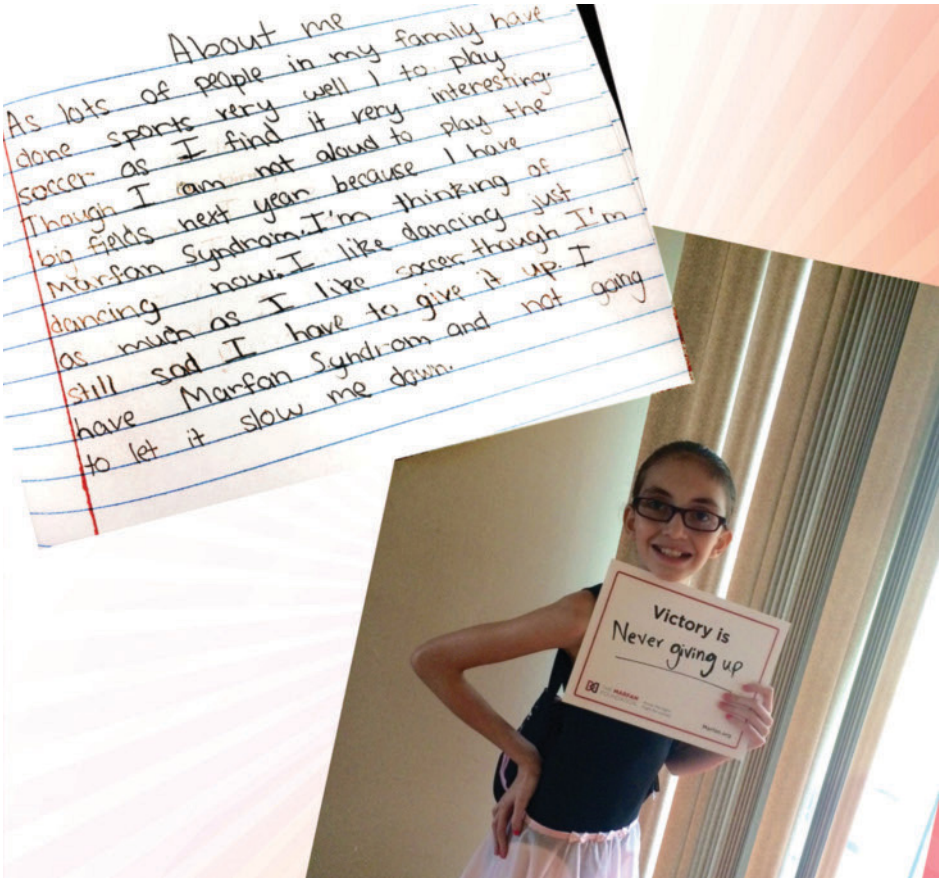
THE MARFAN FOUNDATION
22 MANHASSET AVENUE
PORT WASHINGTON, NY 11050
516-883-8712 | 800-8-MARFAN
WWW.MARFAN.ORG

**COVER:** DAVID ALVARADO, PHD, WASHINGTON UNIVERSITY IN ST. LOUIS, RECIPIENT OF ONE OF OUR 2014 VICTOR MCKUSICK FELLOWSHIP GRANTS.




**ABOVE** "THE SECOND WEEK OF SCHOOL THIS YEAR I GOT THE CHANCE TO VISIT ALANA'S CLASSROOM AND I SAW WHAT SHE WROTE ON HER WRITING JOURNAL. NEEDLESS TO SAY, THE WEEK AFTER THAT WE ENROLLED ALANA IN DANCE CLASSES." – EVA SEIJO, VIRGINIA BEACH, VA

# MESSAGE FROM THE CHAIR

In this issue of *Connective Issues*, we are pleased to share with you extensive research news, including the results of the Atenolol vs. Losartan in Marfan Syndrome clinical trial, conducted by the National Heart, Lung, and Blood Institute's (NHLBI) Pediatric Heart Network and supported by The Marfan Foundation.

The identification of TGF-beta as a possible factor contributing to the characteristics of Marfan syndrome in 2003 unlocked the doors to scientific discovery in Marfan syndrome. The first and largest subsequent initiative was the atenolol vs. losartan clinical trial. It gave us the opportunity to partner with NHLBI, which saw the promise of this breakthrough in Marfan syndrome and was committed to the research.

We are in an enviable position in the rare disease community because we know the pathway and the mechanism believed to cause the life-threatening aspects of Marfan syndrome. Additionally, we are testing existing medications that can alter the pathway and possibly decrease aortic enlargement. We also have a pipeline of researchers who are investigating additional pathways and compounds that represent potential therapies for our community. Plus, we have the full involvement of our patient population; our families are committed to supporting our aggressive pursuit of all potential avenues of scientific inquiry and clinical study.

The atenolol vs. losartan study spawned multiple studies worldwide and, through our robust research program, we are playing a leading role in advancing the science.

We are extremely grateful to our scientists, patient community, partners, and donors for moving so quickly with vision and determination, with belief in the scientific process, and with utmost confidence that as a community we can create a brighter future for everyone living with Marfan syndrome and related disorders.

Karen Murray
Chair, Board of Directors

## COMING TO CHICAGO **AUGUST 6–9, 2015**: THE MARFAN FOUNDATION 31ST ANNUAL FAMILY CONFERENCE

CO-HOSTED BY NORTHWESTERN MEDICINE
AND ANN & ROBERT H. LURIE CHILDREN'S HOSPITAL OF CHICAGO

In December, Ray Chevallier, the chair of our Board of Directors, stepped down from his position due to health concerns. A Foundation member for 30 years, Ray, who has Marfan syndrome, continues to serve as Board advisor. Karen Murray, who was a member of the Board's executive committee, has been named chair through June 30, 2016, when Ray's term ends. The Foundation is grateful to Ray for the leadership he provided during his tenure as chair and values his ongoing dedication to the Foundation.

Karen, who is president of VF Sportswear, has been involved with the Foundation—and held many positions on the Board—since her son, Michael, was diagnosed with Marfan syndrome in 1997. An outspoken advocate for the Marfan syndrome and related disorders community, Karen has testified before Congress and been featured in many high-profile media segments on Marfan syndrome. She also has been the corporate host of our highly successful, star-studded Heartworks gala in New York City since its inception 15 years ago.

During this time, the Board continues to search for a new President and CEO following the resignation of John McGrath on October 17. Judy Gibaldi, Chief Operating Officer and Chief Financial Officer, now serves as Acting President and CEO of the Foundation. Carolyn Levering continues to serve the Foundation as Emeritus CEO. Both are working in partnership with Karen Murray.

The Foundation's dedicated staff remains fully committed to our life-saving programs and services. As always, the individuals and families who have Marfan syndrome and related disorders are our top priority. We are confident that we will find the right candidate to join us in our fight for victory.

# LOSARTAN SHOWN TO BE EFFECTIVE IN THE TREATMENT OF MARFAN SYNDROME


THE DOSTALIK FAMILY WAS AMONG THE FIRST TO ENROLL THEIR DAUGHTER, HALEY, NOW 15, IN THE CLINICAL TRIAL. HER MOTHER, KARI, SAID, "HAVING TWO GOOD CHOICES FOR TREATMENT IS A WONDERFUL OUTCOME OF THE TRIAL. WE'RE EXCITED TO SEE WHAT THE FUTURE HOLDS FOR OUR DAUGHTER AND OTHERS WITH MARFAN SYNDROME AS THESE TERRIFIC RESEARCH INITIATIVES CONTINUE!"

People with Marfan syndrome now have expanded therapeutic options for slowing the rate of aortic enlargement, the life-threatening aspect of Marfan syndrome. In the Pediatric Heart Network (PHN) clinical trial of 608 Marfan syndrome patients between the ages of six months and 25 years, losartan (at up to the FDA recommended dose for hypertension) was shown to be equally effective as atenolol (at a dose above the FDA recommended daily dose), with both drugs leading to a significant decline in body size-indexed aortic root dimension over time. This is a new finding for losartan and confirms that adequate dosing of atenolol (titrated to hemodynamic effect) can have a significant impact on the aorta.

Atenolol belongs to a class of drugs called beta-blockers, which are the gold standard for slowing the growth of the aorta in Marfan syndrome. Losartan belongs to a class of drugs known as angiotensin receptor blockers or ARBs. Without any medication, nearly all people with Marfan syndrome experience progressive enlargement of the aorta, the large artery that takes blood away from the heart, leading to a tear or rupture, which can be fatal. This study showed that atypically high doses of atenolol were well-tolerated and that conventional dosing of losartan was equally effective—both victories for the Marfan community. Further study is required to determine if escalation of losartan dose or combined therapy protocols have the potential to further improve patient outcomes.

Interestingly, the study showed that the magnitude of response to therapy was greater in younger age groups, with the greatest apparent benefit in the youngest children. This could change the management of younger patients as some doctors and parents have been hesitant to start medication in young children with Marfan syndrome.

"Research involving Marfan syndrome and related disorders is critical to understanding basic mechanisms of disease and has the possibility to inform treatment and save lives," said Alan Braverman, MD, Professor of Medicine and Director of the Marfan Syndrome Clinic, Washington University School of Medicine, and Chair of The Marfan Foundation's Professional Advisory Board. "The NHLBI, PHN, and Marfan Foundation-sponsored trial of Atenolol versus Losartan in Marfan Syndrome brought together researchers and clinicians from many institutions in a concerted effort to understand the effectiveness of drug therapy for Marfan syndrome. The Marfan Foundation's support and leadership in this enormous endeavor cannot be overstated and is deeply appreciated. This effort provides the structure for further clinical trials with the promise to improve outcomes for our patients."

## Far-Reaching Impact of the Trial

According to Josephine Grima, PhD, Senior Vice President of Research for The Marfan Foundation, the breakthrough research that spurred the National Heart, Lung, and Blood Institute to initiate this trial through the PHN has far-reaching implications. Ten additional trials on losartan or irbesartan (another ARB) were launched around the world, with scientists using slightly different protocols than what was used in the PHN study. Because of the scientific process, each study is limited in the number of questions it can answer, thus a meta-analysis of the results of all the studies—which the Foundation helped to establish—will provide the best information for the Marfan community.

In addition, The Marfan Foundation is supporting supplemental studies to the PHN trial using subsets of the trial population that are looking into:

- The role of genetic factors to determine which patients with Marfan syndrome will respond best and worst to losartan and to atenolol.
- The effect of atenolol and losartan on the musculosketetal aspects of Marfan syndrome, including bone and muscle mass, as well as strength and endurance.
- Quality of life issues, especially related to the severity of Marfan syndrome and the use of atenolol or losartan.
- The potential correlation of circulating TGF-beta levels on clinical outcomes, i.e., the rate of change of aortic size and Z-score (a measure of how much the size of the aorta differs from normal).

PHOEBE JONAS IS ONE OF THE 608 PEOPLE WITH MARFAN SYNDROME, AGE 6 MONTHS TO 25 YEARS OLD, WHO PARTICIPATED IN THE CLINICAL TRIAL.

"We are grateful to our physicians and researchers for their dedication to this field of study and for their quest to unravel the complex mechanisms that cause Marfan syndrome and seek new therapies to ensure a long and healthy life for people with this disorder," said Dr. Grima. "We are equally grateful to the Marfan community for their eagerness to enroll in this trial. Their participation is critical to advancing knowledge on the condition.

You can still donate to our Research & Progress Appeal at **Marfan.org/Unlock** and help us prevent any slowdown in research.


THERE WAS EXTENSIVE INTEREST IN THE RESULTS OF THE TRIAL AMONG THE PHYSICIANS AND MEDIA AT THE AMERICAN HEART ASSOCIATION MEETING. HERE, DR. RON LACRO IS INTERVIEWED BY CARDIOSOURCE.

## SPECIAL THANKS FROM OUR COMMUNITY TO **RESEARCHERS**

"Thank you for your tireless work to help save and improve the lives of so many. I was diagnosed in 1989 when not much was known about Marfan. I've learned so much in the last few years and so many things from my childhood now make sense. Thank you, thank you, thank you!"

"I lost my father in 1996, when he was 37 years old and I was 10. Thank you for persevering in your research so that future moms and dads can live long, full lives with their children."

"Thank you for all the continued research that you all do. It's because of you that my quality of life is so much better than it would have been just 20 years ago. I wasn't supposed to make it past the age of 21. Thanks to you, I am 20+ years past that number! So thanks for all that you do. Life wouldn't be the same without you."

"I have Ehlers Danlos Syndrome and anxiously await a treatment or cure. I thank you for your hard work researching Marfan and its related disorders like EDS."

"I am grateful for all of your hard work. My son was just recently diagnosed at 18 months of age. People like you give me hope for his future! A million thanks!"

## SEVERAL STUDIES STILL NEED PARTICIPANTS

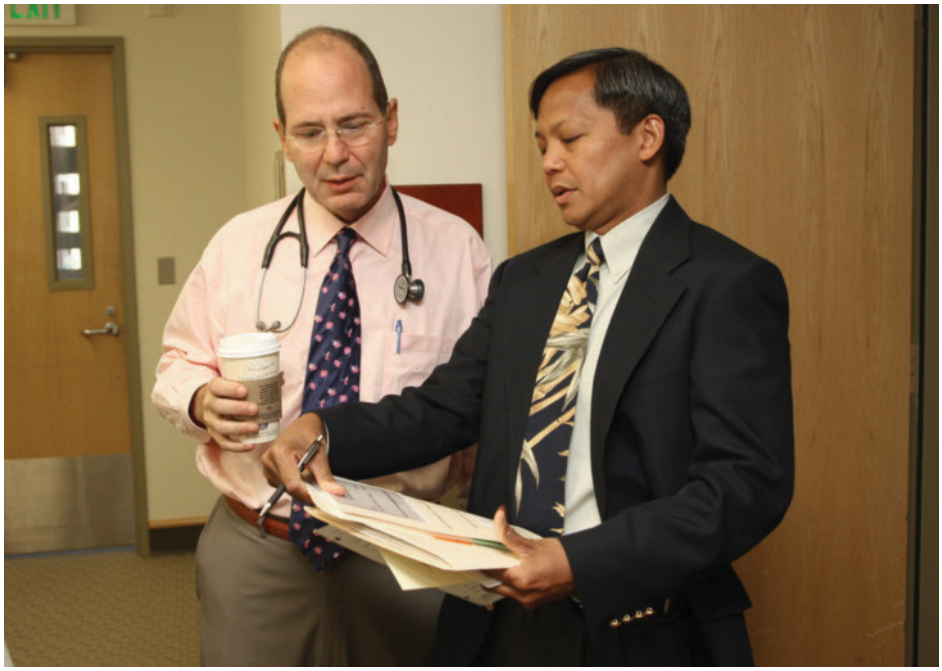
The losartan vs. atenolol trial was only possible because of the 608 people who enrolled in the study. While the results of that study have been announced, there are many more studies underway that need your participation to find answers to questions about Marfan syndrome and related disorders. These include: two studies on pregnancy, two studies that are trying to develop a blood test to detect aortic aneurysms, a study on orthopedic surgery, a pain study, and a study on genetics and aortic aneurysm. In some cases, all that is needed is for you to complete a survey. For other research, a visit to a particular hospital is necessary.

We encourage you to learn more about these studies on our website (Marfan.org/current-studies) and participate if you meet the criteria. Contacts for each study are listed so you can reach out to them with any questions. Your participation is vital. Researchers can only unlock the mysteries of Marfan syndrome and related disorders with your help.

Please check the website frequently as new studies seeking participants are always added.

# CLINICAL TRIAL Q&A

## Researchers answers common questions



DR. HAL DIETZ (LEFT) AND DR. RON LACRO, CO-PRINCIPAL INVESTIGATORS FOR THE LOSARTAN VS. ATENOLOL STUDY.

### What did the trial study?

The trial studied two drugs, atenolol and losartan, at specific doses, to see if they slow aortic growth in people with Marfan syndrome. It also looked for, and compared, any side effects that occurred when a person took either drug.

### What is atenolol?

Atenolol is a medication often used to treat high blood pressure. It is in a class of drugs called beta blockers. It has been used by most physicians at the FDA recommended dose for hypertension to treat people with Marfan syndrome because lowering blood pressure may slow down how fast the aorta grows.

### What is losartan?

Losartan is another medication used to treat high blood pressure. It is in a class of drugs called angiotensin receptor blockers (ARBs). Based on research done in Marfan mice, it is believed that losartan may help manage Marfan syndrome not only by lowering blood pressure, but also in a different way.

### Who was in the trial?

The trial included individuals with Marfan syndrome who were between the ages of 6 months and 25 years, had not had aortic surgery, were not pregnant, and had aortic enlargement that was beyond what is considered normal for their body size (Z-score of 3 or above).

### Do the trial results indicate that losartan is not effective in reducing aortic enlargement?

The trial showed that both losartan, at up to the FDA recommended dose for hypertension, and atenolol, at a dose well above the FDA recommended dose, associate with a reduction in aortic root

Z-score in patients with Marfan syndrome over time, suggesting that body growth is outpacing aortic growth. This suggests that people with Marfan syndrome now have two good choices, but that additional attention needs to be paid to drug dosing.

**Are there any specific guidelines on medications and dosages recommended for Marfan patients now that the trial is over?**

At this point in time, there are no new medication guidelines. This trial's results provide us with some important information—that both atenolol and losartan are options for preventing aortic growth in Marfan syndrome at specific dosages. However, we need to wait for the results of other studies to determine more specific guidelines. At this time, we would recommend that you speak with your physician to determine the best treatment plan for your specific situation.

**Should I take both a beta blocker and losartan?**

This specific trial did not study combination therapy (taking atenolol and losartan at the same time). However, a number of smaller studies have reported a significant reduction in aortic growth rate in people taking both losartan and atenolol, compared to those taking more typical doses of atenolol alone. Additional trials taking place outside of the U.S. are studying combination therapy. This is an option that you should discuss with your/your child's doctor.

**I asked my doctor to put me on losartan and he/she suggested waiting until we know the results of the trial. Now that we have the results, what should I tell my doctor? How should I advocate for myself?**

The results of the trial showed a comparable performance of atenolol and losartan at the doses utilized. You should talk to your doctor about your specific medical history and your specific goals in taking either medication. Depending on your specific situation, one drug may make more sense for you than the other.

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**The clinical trial focused on children and young adults. Can we make any assumptions about adults from this trial?**

This trial focused on patients aged 6 months to 25 years. The COMPARE study in the Netherlands did show a benefit of adding losartan to prior medical therapy (largely atenolol) in adults with Marfan syndrome. There are other studies underway throughout the world; some of those studies are also looking at the impact of losartan in adults with Marfan syndrome. These studies will better help us to determine the effectiveness of losartan in the adult Marfan population.

**What next?**

We keep learning. Any good research study both answers questions and creates new ones. We now believe that there are two medications that show promise for people with Marfan syndrome, atenolol and losartan. This is good news. It is very important to have more than one option for patients because not everyone can tolerate a specific drug. Meanwhile, research continues. Other studies are being done on losartan. The meta-analysis looking at the data from all the losartan trials will be conducted and we are studying other new drug treatments in animal models. Many drug companies have watched and admired the Marfan community for their enthusiastic participation, the professionalism and commitment of the physicians and researchers, and the key support that The Marfan Foundation has provided. As a result, there have already been many shared ideas regarding additional drugs to test and even inquiries about the logistics of launching the next trial.

For a comprehensive list of questions and answers, please go to Marfan.org. You can also access on our website a presentation of the trial results given by Dr. Ron Lacro, one of the principal investigators. Please feel free to contact our help center, support@marfan.org, with additional questions.

GREGORY, NOW 18, AND DOUGLAS, NOW 15, FROM SOUTH GRAFTON, MA, THOUGHT IT WAS "COOL" TO BE PART OF THE CLINICAL TRIAL. IN THE LAST FIVE YEARS, THREE OF THEIR RELATIVES HAVE HAD MARFAN-RELATED SURGERY. THEY ARE HOPING THE TRIAL RESULTS BENEFIT THEIR FAMILY MEMBERS AND OTHERS IN THE FUTURE.

## MARFAN ASSOCIATIONS DISCUSS GLOBAL COLLABORATIONS

Thirty representatives from 10 Marfan associations from around the world met in Paris in September to address their common concerns surrounding patient care and support. All were focused on how to better share information among people with Marfan syndrome in different countries and in several languages. The Marfan Foundation contingent, led by Senior Vice President of Research and Legislative Affairs Josephine Grima, PhD, convened the meeting that included representatives from Denmark, Belgium, Canada, France, Finland, Japan, United Kingdom, Switzerland, Germany, and Australia.

OLGA CHEW AND STEVE LAU, FROM THE HONG KONG MARFAN ASSOCIATION (WITH JENNIFER BUFFONE, SENIOR DIRECTOR OF SUPPORT SERVICES AND VOLUNTEER DEVELOPMENT) VISITED OUR OFFICE IN NOVEMBER AS PART OF OUR ONGOING COLLABORATIONS WITH MARFAN ASSOCIATIONS FROM AROUND THE WORLD.

# RESEARCHERS GATHER TO SHARE ADVANCES

RESEARCHERS, PHYSICIANS-SCIENTISTS, AND REPRESENTATIVES FROM VOLUNTARY HEALTH ORGANIZATIONS FROM ALL OVER THE WORLD ARE COLLABORATING TO CREATE A BRIGHTER FUTURE FOR EVERYONE LIVING WITH MARFAN SYNDROME AND RELATED DISORDERS.

For three days in September, more than 200 researchers and physician-scientists gathered in Paris for the 9th International Research Symposium on Marfan Syndrome and Related Disorders. The symposium provided a meeting ground for basic scientists, applied scientists, and clinicians to better understand the causes of these conditions, the abnormalities produced by the underlying mutations in connective tissue genes, and the effects of medical interventions. In addition to the numerous presentations, there were constructive discussions and debate among the attendees.

The sponsors of the meeting were The Marfan Foundation, French Marfan Association, Genzyme, Ghent University, March of Dimes, VWR, and INSERM.

All major disciplines were covered, with sessions specifically dedicated to clinical information, such as surgery, ophthalmology, orthopedics, and other areas that directly impact the quality of life of affected people. Here is a sample:

Alex Pitcher, BM, BCh, University of Oxford, UK, presented research on the risk of cardiac complications (aortic dissection, abdominal aortic aneurysm rupture, and stroke) in a national cohort of patients with Marfan syndrome in England. Dr. Pitcher and his colleagues were able to quantify the risks of these serious medical consequences for people with Marfan syndrome in what was the largest trial possible in a single location.

Steven Bassnett, PhD, Washington University School of Medicine, discussed his research on the gene mutations in Marfan syndrome, congenital contractural arachnodactyly (CCA or Beal's syndrome), and Weil Marchesani syndrome, and how they cause ocular problems in these conditions. Using mice models, Dr. Bassnett and his colleagues are gaining more insights into the pathology of the eye.

Claudia Campbell, PhD, Johns Hopkins Hospital, presented research on pain and psychosocial aspects of having Marfan syndrome. Her research, based on a patient survey conducted through The Marfan Foundation, validated the significant pain that affected people's experience and confirmed that treating the psychosocial aspects of Marfan syndrome is an unmet medical need.

# 2014 RESEARCH GRANT RECIPIENTS

The Marfan Foundation awards a total of $737,375 this year to eight researchers

## Victor McKusick Fellowship Grants

**David Alvarado, PhD, Washington University in St. Louis,** is studying the role of fibrillin gene variations in a range of human spinal disorders, including infantile scoliosis, kyphosis, and adolescent idiopathic (unknown cause) scoliosis. The objectives are to determine the extent to which specific mutations in FBN1 and FBN2 are associated with the risk of scoliosis and Marfan features, understand the mechanism by which FBN1 and FBN2 contribute to scoliosis susceptibility, and develop zebrafish models to further study these spinal problems.

**Elona Gavazi, MD, Columbia University,** is studying the vision-threatening changes that are caused by Marfan syndrome by fully characterizing the eye features of the condition in the Marfan mouse model. In addition, the study is looking at novel treatments for the eye problems of Marfan syndrome and determining whether or not they are safe and effective in the mouse model.

## Early Investigator Grants

**Dirk Hubmacher, PhD, Cleveland Clinic Foundation,** is studying a protein called ADAMTSL2, which has previously been shown to increase the amount of microfibrils made by cells. This is important in Marfan syndrome because an increased amount of microfibrils in the aortic wall would prevent the enlargement of the blood vessel. This research focuses on the study of ADAMTSL2 and how it affects microfibrils form the wall of the aorta both in isolation and in a Marfan mouse.

**Douglas Y. Mah, MD, Boston Children's Hospital,** is studying the dangerous heart rhythms that sometimes accompany Marfan syndrome and looking into whether or not abnormal findings can be seen on an echocardiogram. This study will use data already gathered as part of the National Heart, Lung, and Blood Institute's Pediatric Heart Network study on losartan vs. atenolol in patients with Marfan syndrome.

**Francesca Seta, PhD, Boston University School of Medicine,** is studying an enzyme called SirT1, which plays a role in cellular function. The research, using Marfan mice, will look at whether or not SirT1 has a protective effect against aortic dissection and death induced by angiotensin II (a molecule that constricts blood vessels and causes aortic aneurysm and dissection). In addition, the study aims to develop screening assays to test for SirT1 activators and other potential drug candidates for people with Marfan syndrome.

## Faculty Grants

**Steven Bassnett, PhD, Washington University in St. Louis,** is creating a mouse model to study the eye in Marfan syndrome and two related conditions, Weill Marchesani Syndrome and congenital contractural arachnodactyly. The research focuses on how fibrillin mutations affect the eye, including its role in the formation of the eyeball and the structural failures that lead to lens dislocation.

**Silvia Smaldone, PhD, Icahn School of Medicine of Mount Sinai,** is studying a mouse model of Marfan syndrome to better understand the causes of bone overgrowth, which affects the quality of life of people with the disorder. In addition, the research is testing the efficiency of TGF-beta blockade in improving the orthopedic problems associated with Marfan syndrome.

**Dudley Strickland, PhD, University of Maryland-Baltimore,** is studying LRP1, a protein that has been shown to play a role in aneurysm formation. By examining how LRP1 affects the growth of the aorta in Marfan mice and Loeys Dietz syndrome mice, the researchers will have a better understanding of the pathway leading to aortic enlargement and how to treat it.

DIRK HUBMACHER, PHD, CLEVELAND CLINIC FOUNDATION, RECIPIENT OF ONE OF OUR EARLY INVESTIGATOR GRANTS

THE MARFAN FOUNDATION

# HEARTWORKS *Gala*

*Honoring*

HERO WITH A HEART
**ISAIAH AUSTIN**
Former Baylor University Basketball Star

HERO WITH A HEART
**CAROLYN LEVERING**
Emeritus CEO, The Marfan Foundation

THURSDAY
**APRIL 16, 2015**

**CIPRIANI 42ND STREET**
110 E. 42ND ST.
NEW YORK CITY

**6:00 PM**
Cocktails & Silent Auction
**7:00 PM**
Dinner, Awards & Entertainment

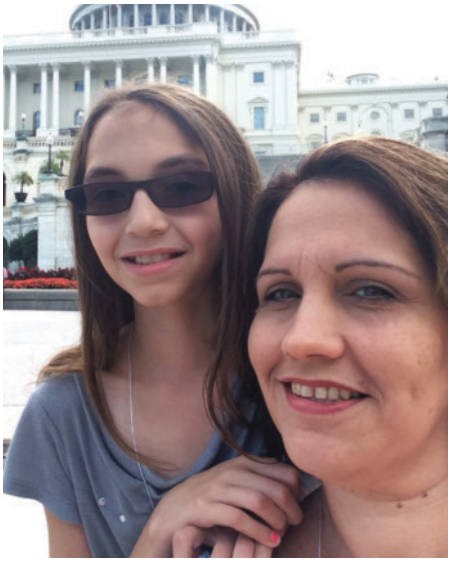
## MARFAN COMMUNITY GOES TO SCHOOL

### Reaching Teachers and School Nurses with the Adopt-A-School Victory Challenge

Thanks to you, our members, more than 75 schools were reached this year with information about Marfan syndrome and related disorders through the Adopt-A-School Victory Challenge. Parents and other volunteers met with teachers and school nurses at schools in 25 states to share our resources and improve the educational experience for affected children. The kickoff of the challenge featured a contest to win a mini iPad. The winner, Eva Marie Seijo, of Virginia, told us:

*During the first week of the school year, I requested a meeting with [my daughter] Alana's teacher (like every other year) to discuss Alana's condition and anything I thought the teacher should know. This year I asked the nurse and school counselor to be present at that meeting also. I provided them with information printed out from the Foundation's website and we discussed questions and concerns that may arise during the year. I think the meeting was beneficial to all parties. As a mother and educator, I feel it is important to do this for as many years as possible and next year I may try to add other schools in the community.*

Any time is a good time to educate your school about Marfan syndrome and related disorders. To find out how, and to make sure your efforts are acknowledged in our initiative to reach schools across the country, please visit bit.ly/MarfanSchoolVictory.



EVA MARIE SEIJO AND HER DAUGHTER, ALANA

# AN EVENT THAT NEVER GRADUATES

## March for Marfan still going strong

In 2006, Maya Brown-Zimmerman, a member of our Board of Directors, was a college student at Case Western Reserve University in charge of community service for Alpha Phi Omega (Theta Upsilon Chapter), a national service fraternity. A charity 5K was planned and The Marfan Foundation was ultimately selected as the beneficiary. Maya was not the only one touched by the disorder. Another member had lost her sister due to Marfan complications and still another was going through the diagnostic process. The first March for Marfan was held in memory of Steve Jerkins, a local resident who passed away from Marfan just before the event.

Maya chaired the March for Marfan for two years, until she graduated from college. She left step-by-step instructions for the fraternity and offered to provide assistance. She hoped that her fraternity brothers would annualize the event. And they have. Over the past nine years, the March for Marfan has raised nearly $15,000.

This year's event, on March 21, will be the 10th Annual March for Marfan.

"I think the chapter feels a real connection to the cause and to the Foundation," said Maya, who is gratified by both the money and awareness raised. "People are learning what Marfan is, what the signs are. At least one student with Marfan has come forward after seeing the event on campus."

Sophia Senderak, a sophomore from Barberton, OH, and Evelyn Rueda, a sophomore from Mason, OH, are the co-chairs this year.

Sophia, who was diagnosed with Tourette's syndrome at the age of 8, knows that increasing awareness is half the battle. "The March for Marfan has the power to greatly improve awareness of the syndrome," said Sophia. "Some service events help indirectly, and it is sometimes difficult to see the end result of your efforts. With the March for Marfan, we are able to see the impact immediately."

Plans are underway for the 2015 event. Said Evelyn, "This year, we hope to increase attendance by attracting participation from alumni from our chapter, students on campus, and residents in the surrounding Cleveland area."

**What's the key to the ongoing commitment to the March for Marfan?**

"A take-away from the success of this event is that passion and knowledge can be transferable to keep an event going," said Maya, who encourages volunteers to overcome any reservations they have to start a new fundraiser. "By engaging other people and organizing yourself—even writing a 'how-to' —events can continue long after you've moved on."

For more information on the March for Marfan, go to the fraternity's website, apo.cwru.edu, or check the community calendar at Marfan.org.

Interested in starting a fundraiser in your area? Please send an email to volunteer@marfan.org.



**ABOVE**: RUNNING FOR VICTORY IN 2014
**LEFT**: MAYA BROWN-ZIMMERMAN AT THE SECOND MARCH FOR MARFAN, WHICH SHE HELPED LAUNCH WHILE A STUDENT AT CASE WESTERN, IN 2007.

## FEBRUARY IS MARFAN AWARENESS MONTH

February is our month, the time for all of us in the Marfan syndrome and related disorders community to amplify our efforts to raise awareness of Marfan syndrome and related disorders. Here's why:

- There are still people who have Marfan syndrome and are not diagnosed. Without treatment, they are at risk of a sudden early death from a tear of their aorta. With a diagnosis and treatment, their medical issues can be managed and they can take steps to avoid a tragedy. You can save a life.
- There are people with a diagnosis who still feel alone. They don't know that we have a warm and welcoming community ready to provide support, share information, and help them on their journey. You can be the one who connects them to The Marfan Foundation.
- There are people in your community who don't know about Marfan syndrome or related disorders. By helping them to understanding these conditions, you can make your community a better place for you and your family to live with your diagnosis.
- There are doctors who are not familiar with the latest on Marfan syndrome and related disorders; perhaps they are your doctors or other doctors in your community. By providing them with new information, you can improve your care and the care of others.

What can you do? Go to Marfan.org and click on Get Involved then Volunteer. You can also email volunteer@marfan.org.

# ST. LOUIS WALK: A VICTORY FOR THE COMMUNITY

"MY FRIENDS AND CO-WORKERS KNEW I HAD A HEALTH PROBLEM, BUT DIDN'T KNOW MUCH ABOUT IT. THROUGH THE WALK FOR VICTORY, THEY LEARNED MORE ABOUT MARFAN SYNDROME, AND ARE EVEN MORE SUPPORTIVE OF ME," SAID LAURA TORODE, BOTTOM ROW, LEFT, WITH TEAM TORODE.

The Marfan Foundation's St. Louis chapter has a long history and has been energized over the years by supporting the annual family conference twice (when it was hosted by Washington University School of Medicine) and the Heartworks St. Louis gala, an annual event. Still, according to Laura Torode, the chapter president, it is challenging to bring the local Marfan syndrome and related disorders community together for informal gatherings where they can share experiences and bring new people in.

Enter The Marfan Foundation's Walk for Victory, which was held in St. Louis in October, and attracted more than 100 people from near and far. Dawn Pulliam, a long-time member of the chapter, said, "What makes the walk so special is that it is a way to bring our family and friends, who make up our own support networks, into the fold. I was honestly shocked by the number of people who came and was thrilled to meet new people from our area."

The Walk for Victory works, says Laura, because it is fun for a variety of age groups. And if you've never been to a local event before, it's completely comfortable. "It's so powerful to be part of the Walk for Victory with others in our community and your own friends and family," said Laura. "You can really feel the energy when we're all together."

The Walk for Victory is coming to these states in 2015:

- Arizona (Scottsdale)
- Georgia (Roswell)
- New York (Woodbury)
- Massachusetts (Salem)
- New Jersey (Paramus)
- Missouri (St. Louis).

Check our website for details.

DAWN PULLIAM, PICTURED HERE WITH HER HUSBAND, ROB, AND DAUGHTERS RILEY AND BROOKE, WHO HAS MARFAN SYNDROME LIKE HER MOM.

# MARFAN SYNDROME AT CENTER COURT IN BOSTON

Marfan syndrome was at center court at the TD Garden on October 29 as the Boston Celtics and Shamrock Foundation proclaimed the Celtics' home opener as Marfan Night. More than 150 members of the Marfan syndrome and related disorders community were on-hand for the evening, which included a special tribute to Isaiah Austin, the NBA hopeful who was diagnosed with Marfan syndrome a few days before the draft in June and then became a spokesperson for The Marfan Foundation. In addition, a new Marfan syndrome public service announcement featuring Isaiah—and created by the Celtics—debuted on the Jumbotron during halftime.

One of the highlights of the evening was the special High Five Kids Tunnel that welcomed the Celtics as they took the court. Twenty children from our community were on court, high-fiving the players as they entered to start the season. At halftime, the Celtics honored Isaiah Austin with their "Hero Among Us" Award.

Rod Gray, a member of The Marfan Foundation from Houston, traveled to Boston for the game with his 13-year-old son, Owen, who was diagnosed with Marfan syndrome earlier this year. "For me, the most important thing was to connect with other families and kids with Marfan syndrome," he said. "We loved being there to support Isaiah Austin and The Marfan Foundation. Watching the Celtics, Owen's favorite team, play was icing on the cake."

Victory is having the Boston Celtics as partners in our fight for victory over Marfan syndrome and related disorders. We are so grateful to the entire Boston Celtics organization for helping to raise awareness of Marfan syndrome. Knowing the signs saves lives.

**TOP**: ISAIAH AUSTIN CHATS WITH OWEN GRAY, 13, WHO TRAVELED FROM HOUSTON TO PARTICIPATE IN OUR HIGH FIVE KIDS TUNNEL.
**ABOVE**: ISAIAH AUSTIN WITH ALL OF THE KIDS FROM OUR MARFAN SYNDROME AND RELATED DISORDERS COMMUNITY WHO PARTICIPATED IN THE HIGH FIVE KIDS TUNNEL.
**LEFT**: THE KELLER FAMILY, OF EAST PROVIDENCE, RI: JONATHAN AND VERA, WITH MELISSA, 7, WHO HAS MARFAN SYNDROME, AND HER BROTHER, ANDRE, 10. BOTH MELISSA AND ANDRE GOT TO PARTICIPATE IN ON-COURT ACTIVITIES.


# WE REMEMBER AND HONOR

We are grateful to our members and friends who have made contributions in memory of, or in honor of, the following individuals. These donations are fully appreciated and support our programs and services that create a brighter future for all those living with Marfan syndrome and related connective tissue disorders.

**Donations In Memory of:**

Adrian, Carol, & Elias Adame
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Owen Gray and Carson Flanagan, of Texas, and Aaron Childress, of Arkansas, were among the 150 members of our community who got together as we raised awareness of Marfan syndrome and related disorders at the Boston Celtics home opener, which was Marfan Night.

Details and more pictures are on page 13.