BloodLines

The Official Newsletter of the Hemophilia Association of San Diego County | Volume 39 Issue 4 2021

WEIGHTLIFTING AND JOINT HEALTH

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HEMOPHILIA ASSOCIATION OF SAN DIEGO COUNTY

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Since we do not engage in the practice of medicine, we always recommend that you consult a physician before pursuing any course of treatment.

Information and opinions expressed in this publication are not necessarily those of the Hemophilia Association of San Diego County, or those of the editorial staff.

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DO THE 5:

1. Get an annual comprehensive checkup at a hemophilia treatment center.
2. Get vaccinated – Hepatitis A and B are preventable.
3. Treat bleeds early and adequately.
4. Exercise to protect your joints
5. Get tested regularly for blood-borne infections.

NHF National Prevention Program

Key steps today for giant strides tomorrow.

Collaborating with the CDC, chapters, associations, HTCs, and the community to prevent or reduce the complications of bleeding disorders.

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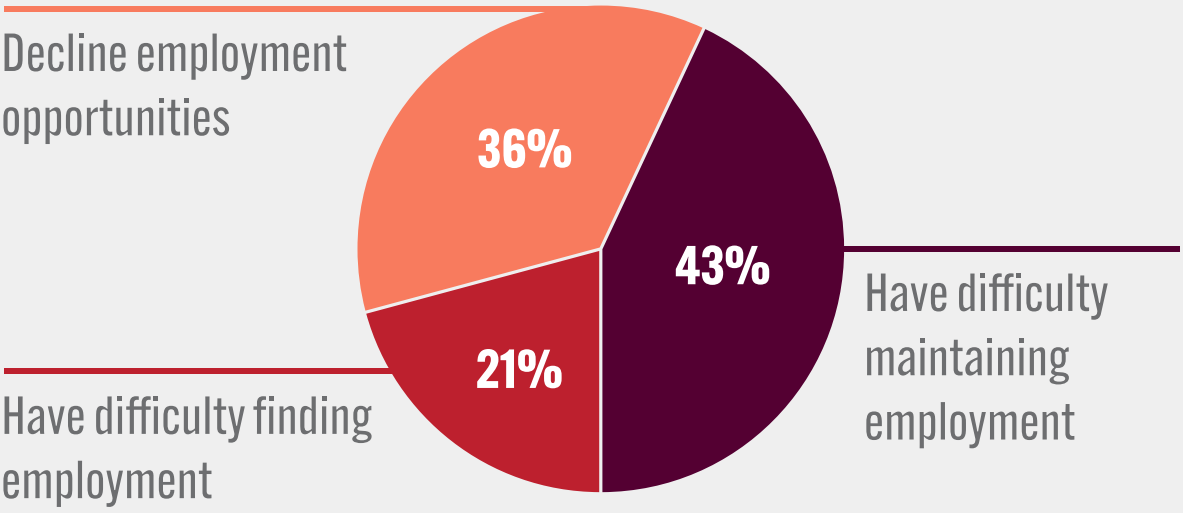
A SAMPLING OF WHAT WE'VE LEARNED SO FAR:

EMPLOYMENT

MY BLEEDING DISORDER HAS CAUSED ME TO:



NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Community Voices in Research

UC San Diego Health Hemophilia & Thrombosis Treatment Center

TRANSITIONING TO ADULT

Back in college, I had an elbow bleed. But this was different than all other prior bleeds. It was my first time going to an adult emergency room instead of a pediatric hospital. I thought I was ready to tackle all the responsibilities associated with my health. After all, I had a lifetime of experience. I knew my bleeding disorder well, my medications, and the correct doses. But just like more than 80% of individuals with chronic health illnesses, I didn't have a "transition plan."

For a teenager to become an adult, a.k.a. "growing up", can be an exciting yet stressful time in life. Some may move to a different town, go to a new college, make new friends, or start a new job. But this can be more complex for individuals with bleeding disorders who will also have to face other changes specific to their health, including transitioning from a pediatric to an adult hematology care team.

What is transition? When does it begin? Who is the new doctor and care team? How will the appointments be made? How will I navigate my health insurance? Many questions may arise for teenagers and their families regarding their transition from a pediatric to an adult hematology care team. A process that can be overwhelming but at the same time it can be made easier with the help of your hemophilia treatment center.

Like an adolescent spending their high school years preparing for college, transitioning to an adult hematologist is a process which takes years. Initial discussions about transition should ideally start when the adolescent is 12 years old. Then, the process includes preparing and teaching the adolescent about their health and how to best take care of themselves independently. While transition to an adult clinic should typically occur around 18-21 years of age, a.k.a. "graduation", sometimes it can occur earlier or later depending on each situation. The preparing and teaching of the young adult will continue at the adult clinic, just like college where one continues to build on what was taught during high school. But do not worry, your doctor will not hand you a pop quiz!

An ineffective transition can lead to a high rate of health-related complications. Thus, transition of care is a very important phase. A successful transition requires the participation of the patient, family, pediatric team, and adult team. Each hematology team has many members from doctors, nurses, social workers, and many others. They work together to create goals to ready the adolescent for transitioning through an individualize approach because each patient is unique. Ultimately, empowering the adolescent/young adult to manage their health with ease, decreasing health-related complications, and becoming independent to achieve their life goals.

Although my elbow bleed got complicated requiring a two-week hospitalization, this experience taught me the importance of a successful transition plan and some of the differences between the pediatric and adult care models. It inspired me to train in both pediatrics and internal medicine to bridge the gap between both worlds and promote collaborative changes to improve the transition of care for individuals with bleeding disorders.

We look forward to helping you continue your journey along our team at the UCSD Hemophilia and Thrombosis Treatment Center.

Akram Mesleh Shayeb MD Hematologist
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University of California San Diego

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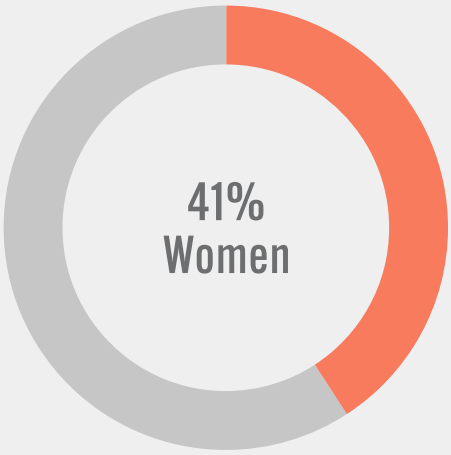
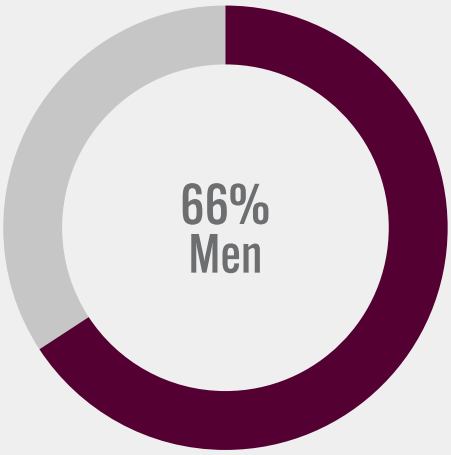
UC San Diego Health

A SAMPLING OF WHAT WE'VE LEARNED SO FAR:

PAIN

41% OF WOMEN EXPERIENCE PAIN MOST DAYS OR EVERY DAY BUT THAT NUMBER JUMPS TO 66% WHEN WE LOOK AT MEN





NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Community Voices in Research

RADY CHILDREN'S HOSPITAL HEMOPHILIA & THROMBOSIS TREATMENT CENTER

Rady Children's Hospital San Diego

BLOOD SUNDAE

A HEMOPHILIA CAMP FAVORITE

Ingredients:

Vanilla Ice Cream
Kix Cereal (platelets)
Mini Marshmallows (white blood cells)
Maraschino cherries (red blood cells)
Multi-colored sprinkles (clotting factors)
Pretzel snaps (fibrin)
Caramel sauce (plasma)

Directions:
Scoop ice cream into dish and add as many toppings as you would like!

FUTURE LEADERS MAKE AN IMPACT VIRTUALLY

Naomi Giron has been a Future Leader in 2019, 2020 and 2021. Her brother Ivan Jr. joined her in 2021. They follow in their father Ivan's footsteps. He was a Future Leader back in 2005. Naomi said that, "[Being a Future Leader] taught me the importance of being part of the bleeding disorder community and advocating for myself." For over 20 years the Hemophilia Council of California has hosted Future Leaders with our founding member organizations to train and inspire young people in the vitally important work of advocacy. While we couldn't gather in person again this year, we were pleased to be able to host 18 Future Leaders for a 7-part webinar series that included 10 guest speakers, a pizza party, packages, cookies and most importantly the opportunity to share their bleeding disorders stories with CA Legislators.

Ilusion Perez said, "I liked playing games and that you sent things to our houses so that we could do [Future Leaders], even though it was virtual. I honestly thought compared to last year it was more interactive, which was weird because it was all online but it was fun being in the breakout rooms with everyone." Student's age 14-22 participated in a story telling workshop, practiced sharing their story, and learned about advocacy, the legislative process and policy issues currently facing the bleeding disorder community. They also heard from guest speakers who work in the legislature and in public policy and learned about a variety of internship and leadership programs which they could apply for to further their interest in policy and advocacy.

Naomi, Ivan and Ilusion were joined by Trevor Messerly from HASDC and other youth from around the state. Future Leaders culminated in Legislative Day where 50 advocates met with more than 30 legislators in virtual meetings to advocate for budget requests important to the bleeding disorders community among other things.

We are already making plans for Future Leaders and Legislative Day 2022. Watch for more information and make plans to join us! No one can replace your voice for the bleeding disorder community.

Hemophilia Council of California's Mission: To improve access to care and treatment options in order to advance the quality of life for people with bleeding disorders thorough advocacy, education, and outreach in collaboration with its founding member organizations.

The Executive Director of the Hemophilia Council of California, Lynne Kinst leads a discussion on how to best tell your story to legislators!

VECTOR BIOLOGY AND HEMOPHILIA GENE THERAPY THE FOCUS OF REVIEW

A new review article, "The Intersection of Vector Biology, Gene Therapy, and Hemophilia," was published recently in the journal Research Practice in Thrombosis & Haemostasis (RPTH). It provides a comprehensive overview of the scientific underpinnings of several hemophilia gene therapy platforms, with particular focus on the delivery of genetic payloads at the heart of these technologies.

This review covers both the setbacks and breakthroughs of preclinical and clinical hemophilia A and B gene therapy research that characterize the mass effort to reach a functional cure for hemophilia.

The authors examine the unique biology of viral vectors, including aspects of their inherent design that make them especially effective at transduction, the process by which genetic material (transgene) is introduced into a cell by a virus or viral vector. Over recent decades, researchers have leveraged viral vectors' evolutionary biology and adeptness at transduction to develop and hone investigational gene therapies for hemophilia and other chronic conditions.

The authors describe two primary vector-types at the heart of these advances, including repurposed retroviruses known as lentiviral vectors, and to a greater extent, adeno-associated viruses (AAVs). Engineered recombinant AAVs (rAVVs) have been designed to deliver the genetic messaging that prompt the production of the factor VIII or factor IX in people with hemophilia A and B, respectively. Ideally, rAAVs deliver this genetic material into living cells to achieve a therapeutic effect that is free of unintended adverse reactions in the short and long term.

"rAAV vectors have emerged as the preferred tools for in vivo gene therapy due to their relative safety and ability to transduce a variety of tissue cell types," explain the authors. Liver cells are a particular target for hemophilia gene therapy for their ability to facilitate production factor proteins such as FVIII and FIX.

While rAAVs remain at the forefront of hemophilia gene therapy, their utilization is not without challenges, including the potential for innate and adaptive immune responses to not only the transgene itself but to the capsid—the protein shell of the rAAV. Such immune responses, like the existence of naturally occurring pre-existing neutralizing antibodies (NAbs), could have safety and efficacy implications, inflammatory effects on the liver being one example.

The authors explain the various strategies being investigated and considered to mitigate the possible impacts of NAbs, including procedures designed to remove the circulating antibodies in a patient prior to administration of a gene therapy, and the employment of diagnostic tests to prescreen for these antibodies.

The review also emphasizes concerns over integration of rAAVs into the genome of patients who have received the therapy, such events could have negative safety implications such as the alteration of cell functionality and subsequent development of tumors or malignancies. While random rAAV integration into a host's genome is a rare occurrence, the risks warrant further investigation and proven mitigation strategies, as even proportionally low rates of integration could have harmful, unintended consequences.

In sum, the authors envision a path forward for hemophilia gene therapy, albeit with challenges left to overcome.

"Gene therapy represents a potential functional cure for patients with hemophilia and has the goal of providing safe, durable, and effective factor expression. In vivo investigational rAAV gene therapy has been demonstrated to ameliorate the bleeding phenotype in adults, but long-term. safety and effectiveness remain to be established," conclude the authors. "Current research seeks to improve vector and other gene therapy attributes to achieve treatment success with simpler and more cost-effective protocols and to expand access to patients not currently candidates due to comorbidities, medical history, age, or other factors."

The paper, which was published online in RPTH on September 1, 2021, is open access – https://onlinelibrary.wiley.com/doi/10.1002/rth2.12586

Lisowski L, Staber JM, Wright JF, Valentino LA. The intersection of vector biology, gene therapy, and hemophilia. Res Pract Thromb Haemost. 2021 Sep 1;5(6):e12586. doi: 10.1002/rth2.12586. PMID: 34485808; PMCID: PMC8410952.

Hemophilia Foundation of Southern California

Central California Hemophilia Foundation

HASDC

WELLNESS CORNER

BY AARON SEAN PENTZ

WEIGHTLIFTING AND JOINT HEALTH

There's a common myth that lifting weight will ruin your joints, but there's a growing body of scientific evidence that shows the opposite. Many studies have shown that there is a link between strength training and muscle strength, joint strength and bone density. Over time regular strength training can provide you with decreased pain even if you have a form of arthritis (which many with a bleeding disorder develop due to joint bleeds). While some soreness is normal from strength training, in total the net outcome of strength training is positive as long as you are using proper methods.

WHY DO MY JOINTS HURT WHEN I LIFT WEIGHTS?

This is a common complaint from individuals who have never lifted weights before. The two most common mistakes when beginning weight training are using too much weight on a given lift or using incorrect form when executing the lift (sometimes the issue is both).

When beginning your weightlifting regimen, it's extremely important to listen to what your body is telling you. Pain is your body's way of telling you that there is a problem somewhere. If you're completing a lift and you feel muscle or joint pain it's important to not just 'tough it out.' If you feel joint pain after a workout, it's probably a good idea to review your workout and how much weight you're lifting and reduce either the amount of weight used for your lifts or reducing some of the volume of each exercise. If you put too much stress on your joints and don't make the adjustment by either reducing weight or volume there's a good chance this can lead to pulling a muscle or damaging your cartilage and tendons in your joints. The more complex the lifts you try to complete the more risk there is for injury if you try to lift too heavy a weight.

Another common problem that novice weightlifters encounter is incorrect form when executing a lift. With even a slight shift in balance one can put themselves in a compromised position and risk joint injury. If you're new to lifting it's important to seek out help in the beginning for teaching proper lifting technique and workout pace. Most gyms offer personal training, which I would recommend to any individual to utilize in the beginning of their strength training journey to learn how to lift. Also, make sure to reach out to your Hemophilia Treatment Center to coordinate ideas with a physical therapist (especially if you're having trouble putting together a workout routine due to mobility constraints), or contact your hematologist

continued on next page

WELLNESS (WEL-NIS): THE QUALITY OR STATE OF BEING HEALTHY IN BODY AND MIND, AS THE RESULT OF A DELIBERATE EFFORT.

if you're not seen at a Hemophilia Treatment Center. Because everybody is different, something as simple as a slight adjustment in body position can be very beneficial for injury prevention.

Credit ender if needed

HOW DO I AVOID JOINT INJURIES WHILE LIFTING?

Here are some simple steps to help avoid injury when lifting weights:

1. **Warm-up!** Start by moving around. Walking, jogging or jumping jacks. Then, once you break a sweat, stretch to loosen up your muscles.
2. **Don't train too hard in the beginning.** If you feel too tired, that's your body's signal to either rest or completely stop working out. If you're too exhausted to perform a lift with good technique you greatly increase your chance of joint injury.
3. **Get enough rest!** Make sure to give your body plenty of rest in between lifting sessions. You should not be training the same muscle group strenuously two days in a row. If you like to do more full body weight training then stick to 2-3 days a week to guarantee enough rest in between training sessions.
4. **Don't rush.** Make sure that you take your time when weightlifting and be very controlled with your movements to ensure good technique. If you're trying too hard to get through the workout you run the risk of improper lifting technique which can lead to serious injury.
5. **Listen to your body.** If something begins to hurt and you're exhausted simply stop the workout. Do not try to be tough and push through the pain, because this could end up making an injury worse and sidelining you away from the gym (and many other life activities) for months.

While lifting itself will not outright cure arthritis, it does have many long-term benefits to overall joint health. Studies show that those who lift weights living with arthritis experience less pain than those who do not. The muscles that surround a joint also play a part in reducing pain. By strengthening those muscles, it allows them to work at their full capacity. With weak surrounding muscle due to inactivity and atrophy, your joints take on undue burdens that can lead to long-term joint damage. All too often there's a misconception that joint pain and weightlifting go hand in hand, but we now know that to not be true. Consistent strength training will build muscle all over your body, but most importantly for those of us in the bleeding disorders community, those muscles around our joints. This increase in strength in your joint muscles will be a boost to your overall lifestyle since it will prevent your joints from deteriorating, allowing them to be without pain and functional. I know this seems counter intuitive, but the more you use your joints the better off they will be over your lifetime. However, the positive effect from weightlifting can only be attained if done with proper technique to avoid injury. We're all built a little differently so paying attention to our bodies and lifting with proper technique is essential to truly reaping the benefits of strength training. When executed correctly and regularly, weightlifting can provide joint pain relief and overall strength.

Source: "How Does Weightlifting Impact Your Joints," Mufaddal Gombera, MD (August 10, 2020)

TREVOR MESSERLY

TEEN TALK

HI HASDC COMMUNITY,

I hope you are all having a great start to the school year. I am currently a freshman at San Diego Mesa Community College so I am going to share a couple of tips I learned along the way. If you are a senior in high school it's time to start applying for colleges. Last year for UCs the deadline to apply was November 30th and for CSUs it was December 5th. To qualify for financial aid either state or federal, you will need to apply for FAFSA by February 1st, 2022. Around this time you should also start to apply for scholarships. Being in the bleeding disorder community you will be able to qualify for a ton of scholarships if you have a bleeding disorder or are the sibling of someone with a bleeding disorder. If you want to attend a community college in San Diego you can apply anytime before the fall semester or even during it. If you want to qualify for your tuition to be paid for at community college you will need to apply for the San Diego Promise Program. This covers two years of tuition and 200 dollars per semester for books. To receive this you will need to apply before the fall semester of 2022 and it must be your first semester at community. Once you find out which University you are attending you should find out what services they offer. For example at Mesa College because of my severe Hemophilia I qualify for Disabled Support Programs and Services. With this I get accommodated with extended testing times, excused medical absences, digital copies of books, first chance for registering for classes, and extensions of deadlines for homework when needed. Almost any college should offer some form of this and if you qualify you should definitely take advantage even if you do not feel as if you need it. You never know when you may have a bleed that may cause you to miss a deadline and these programs would definitely help to accommodate you. There is a lot more in detail that I can help with the college process so feel free to reach out to me. At Mesa College I am a Outreach Ambassador so that means it's literally my job to help students with their college questions all day long. If you have any questions just shoot send me a message on instagram @trevor__messerly. I hope you all have a great beginning of the school and see you next time!!

SAY HELLO TO JAMES

He has hemophilia A and has gone through two major surgeries while keeping to his factor regimen with the support of his hemophilia care team

"RECOVERY WAS TOUGH, BUT I LEARNED I HAD MORE SUPPORT THAN *I THOUGHT POSSIBLE*."

HELLO FACTOR

Read stories like James' in *Hello Factor* magazine:
BleedingDisorders.com

2021 CALENDAR OF EVENTS

DATES SUBJECT TO CHANGE | All EVENTS ARE CURRENTLY VIRTUAL

JANUARY

1 | New Year's Day (Office Closed)

FEBRUARY

27 | Advocacy Forum

16 | Educational Dinner Program (Bayer)

MARCH

1 | Bleeding Disorders Awareness Month

11 | Educational Dinner Program (Takeda)

25 | Educational Dinner Program (CSL Behring)

APRIL

10-16 | The Amazing Goosechase

17 | World Hemophilia Day

13 & 20 | Educational Dinner Programs (Genentech)

MAY

1 | Family Education Day

20 | Educational Dinner Program (Novo Nordisk)

JUNE

30 | Wellness Wednesdays: Joint Health

Programs highlighted in yellow are currently scheduled for in-person.

JULY

7 | Wellness Wednesdays: It's About Bloody Time

17 | Wellness Wednesdays: Yoga in the Park

20-22 | Camp Firefly (virtual)

24 | Wellness Wednesdays: Hike the Coast

28 | Wellness Wednesdays: Dental Health

AUGUST

2-4 | Camp Dragonfly (virtual)

25-28 | NHF's Bleeding Disorders Conference

SEPTEMBER

12-15 | NHF's State of the Science Conference

17-19 | Familia de Sangre Conference

30 | Educational Dinner Program (Novo Nordisk)

OCTOBER

2 | Women's Retreat

5 | Educational Dinner Program (BioMarin)

23 | Unite for Bleeding Disorders Walk

27 | Educational Dinner Program (Pfizer)

NOVEMBER

6 | Industry Symposium

10 | Educational Dinner Program (CSL Behring)

DECEMBER

TBD | Educational Dinner Program

12 | Factor & Frost (Westin SD Gaslamp)

23-31 | Holidays (Office Closed)

TO REGISTER FOR A PROGRAM VISIT:
WWW.HASDC.ORG

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