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Adapting to Chronic Illness

“ADAPT” IS a word with which most people with a major chronic illness have become intimately familiar. To adapt means to move toward a suitable outcome or resolution. The process of adaptation revolves around reconstructing the aspects of everyday life that have been affected by chronic illness, including relationships, school, work and psychological and physical health.

Staying physically active can seem counterintuitive when feeling ill. When we’re tired or in pain, our bodies tell us to rest. But, incorporating forms of exercise into daily life can actually help to ease symptoms and add more joy to life. And, many find yoga is an excellent way to begin to be active again. In our article “Yoga for Chronic Disease Management” (p.18), physical therapist Matthew Hansen explains the physical and emotional benefits of Hatha yoga that are backed by research. He begins with a discussion of breathing, one of yoga’s most important components, and then describes many postures (asanas) that can be adapted to each individual’s circumstances. Photos of each of the asanas illustrate how they are performed. Hansen wraps up the article with helpful tips for the hardest part: simply getting started.

Attending college is likely one of the biggest life events requiring adaptation for those with chronic illness. But students with disabilities shouldn’t be deterred since laws are in place to ensure their access to education. Indeed, as we detail in our article “Preparing for College with a Chronic Illness” (p.22), people have a civil right to higher education, and they are allowed to request reasonable accommodations to make sure they have the same opportunity to succeed as those who are healthy. Patient advocate Abbie Cornett outlines the types of allowable accommodations and how to register for them. What’s more, many schools have programs in place to assist people with disabilities. Students just need to do their due diligence to determine which school is best suited to their needs.

Sometimes, it can be easy for those affected by chronic illness to forget they are not the only ones who need to adapt to their circumstances; friends and family must adapt as well. In this second installment of our new Therapeutic Helpline column (p.10), which provides advice for coping with chronic illness, licensed clinical psychologist Erika Lawrence explains how patients can get the help and support from family and friends they need and deserve. As Dr. Lawrence notes, it’s important to first dispel the myths that interfere with getting support and then find the courage to ask for what is needed.

As always, we hope you enjoy these articles, as well as the many more educational and insightful topics presented in this issue of IG Living.

Ronale Tucker Rhodes, MS
The Unintended Consequences of Opioid Drug Legislation

By Abbie Cornett

THE MOMENT humans first decided to band together and form social bonds to increase their chances of survival, politics was born. While in the early years of civilization, leadership was determined by who was the best warrior or the best hunter, this simple form of leadership evolved as societies became larger and more complex. How leaders were chosen became dependent on a person’s abilities to solve complex problems that affected the welfare of the people.

Unfortunately, it seems society has moved away from careful consideration of problem topics. Political leaders make promises and introduce legislation based on what’s trending in the media without thoroughly evaluating their impact. A good example of this is current policies adopted to address the very complex issue of opioid addiction.

In recent years, illicit drug and prescription opioid overdoses and deaths have reached epidemic levels. The National Conference of State Legislatures (NCSL) reported 115 people a day died of an opioid overdose in 2018, with nearly half of those deaths occurring as a result of a prescription drug. This was an increase from 78 deaths a day reported by the surgeon general in 2016.

Aside from the grievous loss of life, the economic impact of opioid deaths is staggering. In November 2017, the White House Council of Economic Advisers released a report claiming the crisis had cost the country $504 billion. Due to the problem’s magnitude, the issue has been addressed at both federal and state levels. As of October 2018, NCSL reported 33 states had enacted prescription opioid legislation based on Centers for Disease Control and Prevention guidelines.

Certainly, policymakers need to address the issue of opioid addiction, but not all people who use pain medications are addicted or misuse their medication. While the intent of the new guidelines and legislative restrictions for opioid prescribing is well-intended, they bring unintended consequences resulting in restricted access to medication for patients who need to manage their pain. Indeed, a survey conducted by Practical Pain Management found 18 percent of patients with a chronic pain condition were unable to fill their prescriptions on at least one occasion due to the new rules, as well as due to concerns regarding abuse and misuse. The repercussions of this were demonstrated in an article by Stat News that described how a patient who was suffering from bone cancer was called a “drug seeker” by a pharmacist and sent away. After enduring three days of pain at home, he attempted suicide.

Not only are patients having trouble filling their prescriptions, but many doctors are now hesitant to prescribe opioids for fear of repercussions. This further limits patient access to medications they need, and increases the risk they will be forced to seek illegal drugs to ease their symptoms.

In response to the fear that these denials or delays in treatment place patients at risk, Stefan G. Kertesz, MD, MSc, who teaches addiction medicine at the University of Alabama at Birmingham, submitted a letter to Medicare officials in opposition, which was signed by 220 professors in academic medicine, experts in addiction treatment and pain management, and patient advocacy groups.

Policymakers need to recognize there is no simple solution to the opioid crisis. The current trend in politics is to address the issue that is gaining the most attention. In the case of opioid addiction, that issue is medication abuse, with an afterthought to patients who suffer from pain. Moving forward, policymakers need to recognize not all people seeking opioids are addicts so their “solutions” don’t have the unintended consequence of harming patients. Many people suffering from pain conditions need these medications to have a better quality of life.

ABBIE CORNETT is the patient advocate for IG Living magazine. She can be reached at patient.advocate@igliving.com or (800) 843-7477 x1366.

References
Before my husband died, the doctor gave me a depression screening questionnaire. I knew I wasn’t depressed, just stressed, but it helped talking about it and getting some ideas about how to cope with this worsening chronic condition.

— Harriett HW

Actually, I was misdiagnosed in the emergency room [because there was] no increase in my white blood cell count to show infection, nor was there a fever above 98.6 (my normal body temp is 95.5). I can’t wait for the Immune Deficiency Foundation fever study so I have proof in regard to how [primary immunodeficiency patients] present. Two of perhaps eight or 10 doctors at my local emergency room understand; the others tend to placate me and get me out the door ASAP. I am grateful my general practitioner, immunologist, ENT and gastroenterologist all understand, or I would have died in the emergency room.

— Birgit C

Were you ever misdiagnosed with stress?

Absolutely! [Especially because of] the germ exposure with selective antibody immunodeficiency. I was literally told on more than one occasion: “You can’t have that.” “It’s too rare.” “I’m not treating you for something you can’t have.” Yeah, filing a complaint was a joke.

— Renee WL

Are You Afraid of Going to the Emergency Room?

Yes! The emergency room doctors don’t have a clue when it comes to common variable immunodeficiency! It’s so frustrating to have them call my doctor to confirm I regularly get pneumonia, and if I am not breathing well and coughing up blood with a fever, they should indeed look at me! It’s the same routine every time!

— Carly F

Have you had to fail on step therapy in order to receive a prescribed medication?

Yes. I have had to fail on numerous medications over the years before I could get what the doctor wanted for me. All this failed time equates to several years of my life in pain, misery, missed school (resulting in poor grades at times due to lack of attendance) and more stress as I tried to teach myself, missed work (which means less money, job loss, constant mental and emotional stress, etc.) and lost friendships and relationships, which caused more emotional stress. Once, the insurance company’s constant denials of prescriptions brought me near death. I was clinically dying before my insurance company would approve intravenous immune globulin! Something needs to be done. Now.

— Amanda T

I had to try three different approved nasal sprays before I could use the one the doctor wanted me to be on. I had already used two, so had to go on the third one. I had a sinus infection within a week. That was sufficient failure to let me go on the spray my doctor wanted me on in the first place.

— Lourdes EA
Michelle » A low IgG level is just the first step in determining your need to start IG replacement therapy. A number of other things also should be looked at, including frequency and severity of infections and the results of additional lab tests, which can include your specific antibody responses to the tetanus and diphtheria vaccines. Another vital test reveals how you respond to the pneumococcal vaccine. For this, your blood is drawn and tested, the vaccine is administered and approximately four weeks later, your antibody titers are drawn in a blood test again to see how you responded. If you don’t have a robust response to the pneumococcal vaccine, IG replacement therapy may be considered.

Question » What can be done to reduce pain associated with IVIG treatment?

My son was diagnosed with Guillain-Barré syndrome and has been treated with five doses of 10 mg per day of intravenous immune globulin (IVIG). Prior to each treatment, he was infused with 540 mL of normal saline, and he was given dexamethasone, acetaminophen and Benadryl. After completing the fifth dose, he cried the whole night, and asked me to massage his arm, leg and back. What would you please advise?

Michelle and Leslie » The symptoms your son is experiencing sound like side effects of IVIG. There is a possibility the symptoms will resolve with time. But, if they continue, you should discuss a brand change of IVIG with his treating physician, as well as consider treating the side effects with increased hydration.

Abbie » I spoke with Terry O. Harville, MD, regarding your question. He said part of the process of diagnosing CVID is to get a pneumococcal vaccine to see how your immune system reacts to it. If you are already being treated with IG, receiving the pneumococcal polysaccharide vaccine is not necessary, and could result in a reaction. Receiving the Prevnar pneumococcal vaccine could be helpful, but it may also cause a reaction. However, receiving the annual influenza vaccine, as well as the tetanus/diphtheria vaccine, is a must. And, receiving all other vaccines is likely fine, but the shingles vaccine should be discussed with your doctor. If you are treated with intravenous IG, you should receive immunizations three to seven days before an infusion. If you are treated with subcutaneous IG, you should receive vaccines halfway between infusions.

Question » Should CVID patients receive recommended vaccines for adults?

I have common variable immunodeficiency disease (CVID) and was told not to get recommended vaccinations such as the pneumococcal vaccine because I am being treated with immune globulin (IG). Is that correct?

Michelle and Leslie » The symptoms your son is experiencing sound like side effects of IVIG. There is a possibility the symptoms will resolve with time. But, if they continue, you should discuss a brand change of IVIG with his treating physician, as well as consider treating the side effects with increased hydration.

Have a question? Email us at editor@IGLiving.com. Your information will remain confidential unless permission is given.

ABBIE CORNETT is the patient advocate for IG Living magazine.
MICHIELLE GREER, RN, is senior vice president of sales for NuFACTOR Specialty Pharmacy.
LESLIE J. VAUGHAN, RPh, is senior vice president of clinical programs at NuFACTOR Specialty Pharmacy.
Testing for Infectious organisms and autoimmune disorders has long depended on detecting specific antibodies. For example, following a streptococcal bacterial infection, detecting antistreptococcal antibodies in a patient can confirm the infection. Indeed, this process is used when a person is suspected of having an antibody deficiency. When testing for an antibody deficiency, the pneumococcal vaccine is given, with antibody titers obtained before immunization and four weeks after for comparison. If the person does not produce new antibodies and there is no increase in existing antibodies, it is likely he or she has an antibody deficiency (a concept discussed in previous columns).

Accordingly, in a primary immunodeficiency (PI) patient, the development of specific antibodies following an infection will be impaired, so using a blood test to detect those antibodies is unreliable in this patient. Unfortunately, many healthcare providers are unaware of or do not recognize this anomaly. As a result, when a blood test is ordered, results come back negative and the patient is declared free from the potential offending microorganisms — despite ongoing symptoms. This scenario frustrates both the patient and healthcare providers. To avoid such a situation, PI patients need to remind healthcare providers of the unreliability of using blood tests to detect antibodies against infectious organisms. (Showing them this column may be of benefit.)

The question then is: “How do we detect microorganisms in PI patients?” There are several potential approaches, and more than one may be required. A cough with sputum, a nasal discharge that is purulent (appears to have pus and possibly blood), a wound with pus, urinary discharge or diarrheal stool can be sent for staining and culture for microorganisms. Routine staining and culture should be performed, but there should also be staining and culture for rarer or unusual organisms. These could include opportunistic infectious organisms (those that occur in patients who are immunocompromised, but not in patients with intact immunity) and fungal organisms. Additionally, harder to stain and culture microorganisms, such as mycoplasma and similar, may need further care and preparation since special collection tubes and culture techniques are required for their detection. Thus, the old-fashioned microbiology approach, which preceded the modern blood tests, may be very useful.

A newer approach for detecting microorganisms, including bacteria, fungi and viruses, is with nucleic acid testing (NAT). During the past couple of decades, molecular biologic techniques have been expanded and tweaked to allow for rapid and precise detection of the presence of specific microorganisms — even when they are at very low levels. And, in many instances, the cost for this testing has come down. While NATs are blood tests, they are not looking for antibodies but, instead, for the DNA or RNA that can identify the microorganisms. This type of testing can be performed on tissue, body fluids, mucus, saliva, tears, urine, pus and even stool. Commonly, a special swab to collect specimens from the mouth, nose, skin wounds and rectum or genital region is used, which can be tested for the presence of DNA or RNA from suspected microorganisms.

We will continue next time with more details on NAT, including testing limitations.

TERRY O. HARVILLE, MD, PhD, is medical director of the Special Immunology Laboratory at the University of Arkansas for Medical Sciences and a consultant for immunodeficiencies, autoimmunities and transplantation.
WHILE FAMILY and friends want to provide support, they often don’t know how. In fact, a number of false myths interfere with getting the support we want and deserve:

Myth #1: They know me well enough to know what I need; I shouldn’t have to tell them. While most of us would like this to be true, this belief causes a lot of unnecessary disappointment. Our loved ones aren’t mind readers, and neither are we!

Myth #2: Women want to be listened to and held; men want to fix the problem. This may be true for many men and women, but it is definitely not true for everyone. We all have individual preferences for the types of support we want. We may even have different preferences depending on the problem, our mood or the day.

Myth #3: Help others the way you would want to be helped. This is a noble way to approach helping others, but it can lead to offering help or support that is unwanted or unhelpful. If we choose who to help based on what we would want, we may get it right at times, but we will also get it wrong many times.

Myth #4: More support is always better. Many of us complain we want “more support,” which leads others to think they should just do more of whatever they are already doing. However, too much support can be unhelpful or even cause more problems, just like not having enough support. What we really want is “different support.”

So, how do we get the support we want and deserve?

1) Notice when we want support or help. Often, we are not even aware of the kind of support we want from others until they try to help and get it wrong. There are many different situations in which we might want help and support such as when we have had a bad day or are in a bad mood, are going through a particularly stressful time, are depressed or anxious, are in pain, are physically exhausted, are working toward a personal goal or are having a problem with another person. This may seem like common sense, but most of us do not take the time to be truly aware of what is going on inside of our heads and hearts.

2) Figure out what we want from others in that moment. There are five types of support:
   • Emotional support is listening to someone talk, letting them know we hear and understand them and empathizing. It also includes physical comfort such as holding their hand or giving them a hug.
   • Esteem support is expressing confidence in their ability to handle a difficult situation.
   • Informational support is giving advice or guidance about how to solve a problem or gathering information about a new diagnosis.
   • Tangible support is taking care of the problem or taking on other responsibilities (childcare, making dinner, cleaning) so the person has more time and energy to deal with the problem.
   • Network support is encouraging the person to reach out to others (friends, family). It also includes simply spending time with the person or being in the room. Or, it can mean giving the person space when they want it (the absence of network support).

3) Ask for what we want and need. We absolutely have a right to ask for the support or help we need and want. Most of the time, the people who love us will be thrilled to know exactly how to help. Think how often people simply say: “Let me know if there is anything I can do to help.” They want us to be specific.

4) Express gratitude and give feedback. We need to thank people for their efforts, even if they get it wrong. We can then let them know what we would like them to do differently next time. For example: “Thank you so much for doing the dishes and my laundry for me today. I really appreciate you trying to take some things off of my plate. Other times, it might also be helpful if you also just sat and listened while I shared what I’m feeling, even if it doesn’t solve anything.”

The bottom line: We all have a right to ask for what we need, and we deserve to be helped and supported. It takes practice to notice what we are feeling and thinking and to figure out what we want from others. It takes courage and strength to ask for what we need.

ERIKA LAWRENCE, PhD, LCP, is director of translational science at The Family Institute at Northwestern University, Evanston, Ill.
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URTICARIA (from the Latin word meaning to burn or hives) is a kind of skin rash notable for dark red, raised, itchy bumps that affects 15 percent to 20 percent of the population once or more during a lifetime. However, in approximately 30 percent of patients, urticaria attacks often recur for months or years. Chronic urticaria is defined by recurrent episodes occurring at least twice a week for six weeks. Females are more commonly affected than males.¹

Most allergists and dermatologists who see many cases of chronic urticaria would describe it as “easy to diagnose but difficult to treat.” For many years, chronic urticaria was considered a dermatologic disease with an occult (hidden) or idiopathic (unknown) etiology. The occult causes ranged from food allergies, food additives or chemical preservatives; chronic infections (dental or sinus more commonly); endocrine disorders (mainly hypothyroidism); or neurogenic (arising from the nerves or nervous system). However, in more than 90 percent of chronic urticaria cases, no underlying cause was determined (excluding physical urticarias from pressure, cold, heat and aquagenic), which led to changing the terminology to chronic idiopathic urticaria.

Chronic idiopathic urticaria has been an especially difficult disease to treat since the first- and second-line treatments, nonsedating antihistamines and higher doses of antihistamines, respectively, are in many cases ineffective. And, the addition of oral corticosteroids, while effective, has too many acute and chronic side effects to be a viable long-term treatment option.

In the mid-1990s, the discovery of serum IgG autoantibodies against the alpha chain of the Fc epsilon receptor changed the belief that chronic urticaria was an allergic disease but, instead, an autoimmune disease.² This autoantibody discovery was present in about one-third of patients. Indeed, a single case report in a patient with common variable immunodeficiency whose chronic urticaria ameliorated when treated with intravenous immune globulin (IVIG) may have opened the door to IG as a treatment for chronic urticaria.³

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Treating Chronic Urticaria with Immune Globulin Therapy

By Dean Mitchell, MD
IG: A Third-Line Treatment

Immune-modulating treatments have become the third line of treatment for chronic urticaria, and research has shown both high-dose and low-dose IVIG are effective.

Since specific cases of chronic urticaria are autoimmune disease, rather than immune deficiency, it is reasonable to believe low-dose IVIG can be sufficient and as effective as high-dose IVIG. The main goal of autoimmune treatment is to dampen the immune response, rather than maintain serum immunoglobulin levels. As an allergist/immunologist, I have found GamaSTAN S/D given intramuscularly has been effective in many cases. Similar to subcutaneous administration of IG, the use of an intramuscular preparation has fewer side effects (headaches, thrombosis, aseptic meningitis) than IVIG. In addition, its cost is significantly lower than IVIG products.4,5,6

GamaSTAN S/D is a human IG treated with solvent/detergent for intramuscular administration in either the upper lateral thigh or the deltoid muscle in the upper arm. Passive immunization with GamaSTAN S/D modifies hepatitis A and prevents or modifies measles. It is also an option for varicella (chickenpox) in an immunocompromised patient if varicella-zoster IG is unavailable. GamaSTAN S/D should not be given to persons with an IgA deficiency, thrombocytopenia or any coagulation disorder that would contraindicate intramuscular injections.7

Omalizumab: A New Option for Chronic Urticaria

In 2015, the U.S. Food and Drug Administration (FDA) approved omalizumab (XOLAIR) to treat chronic idiopathic urticaria (it was originally approved for moderate to severe asthma). Omalizumab is a recombinant humanized anti-IgE antibody directed against the Fc IgE receptor on basophils and mast cells (central effector cells in allergic inflammation and innate and adaptive immunity). The benefit of omalizumab to patients is that the course of treatment is typically one or two injections. The concern, however, is that patients have developed anaphylaxis after the injections.8 In the randomized double, placebo-controlled ASTERIA II study, doses of 150 mg and 300 mg of omalizumab were found to be effective in 33.7 percent of patients treated with the drug versus 4 percent of those treated with a placebo.9

GammaSTAN S/D More Effective and Well-Tolerated

While GamaSTAN S/D is not approved by FDA for chronic urticaria, it can be used off-label in appropriate chronic idiopathic urticaria patients who have not responded to antihistamines, oral corticosteroids or omalizumab. In my practice over the past 10 years, I have used both omalizumab and GamaSTAN S/D, and have found the latter to be more effective and well-tolerated by patients.10

DEAN MITCHELL, MD, is a clinical assistant professor at Touro College of Osteopathic Medicine in New York and chief medical consultant for MRS Allergy.

References
3. Abschle, A, and Cunningham-Burrous, C. Chronic Urticaria and Angioedema as the First Presentation of Common Variable Immunodeficiency. Journal of Allergy and Clinical Immunology, 2002; 110:664-5.
8. Wu, KC, and Jabbar-Lopez, ZK. Omalizumab, an Anti-IgE mAb, Received Approval for Treatment of Chronic Idiopathic Spontaneous Urticaria. Journal of Investigative Dermatology, 2015; 135:131S.
A new study shows OTL-102, an autologous ex-vivo lentiviral gene therapy that utilizes self-inactivating lentiviral vector, is successful in producing sustained corrected neutrophil function for 12 months or more in severely affected X-linked chronic granulomatous disease (X-CGD). In a study of six of seven evaluable X-CGD patients, all showed persistence of 16 percent to 46 percent (mean 30.2 percent) functioning neutrophils 12 or more months after treatment. Prior publicly available data suggests this is above the 10 percent minimum threshold necessary to show potential clinical benefit and restoration of both biochemical function and immunity. Two additional patients were treated but died within three months of treatment from complications deemed by the investigator to be related to pre-existing disease-associated comorbidities due to advanced disease progression. During the 12-month follow-up, patients continued to produce corrected neutrophil function.

“X-CGD is a severe, life-threatening disease that leads to a significantly reduced quality and length of life in affected patients,” said Donald B. Kohn, MD, Orchard Therapeutics Scientific Advisory Board member. “Current treatment options, including prophylactic antibiotics, antifungals and hematopoietic stem cell transplants, all have significant associated risk and limitations. By providing the first-ever demonstration that autologous hematopoietic stem cell gene therapy has the potential to lead to sustained levels of functioning neutrophils and, thereby, a long-term clinical benefit, we are hopeful that OTL-102 may provide a new treatment options for X-CGD patients to improve the quality and length of their lives, escaping the chronic infections and inflammation associated with the disease.”

Orchard Therapeutics intends to meet with regulatory authorities this year to discuss the clinical development path forward for the OTL-102 program in patients with X-CGD.

**Research**

**Mini-Pools of IVIG Found as Effective as Standard IVIG to Treat Pediatric ITP**

A new study shows mini-pools of intravenous immune globulin (IVIG) from as few as 20 donations are just as sufficient in treating pediatric immune thrombocytopenia (ITP) as standard high IVIG doses. In the study, 72 patients ranging in age from 1 year to 18 years with newly diagnosed ITP who had low platelet counts and no serious bleeding were randomized into three treatment groups: Group A (24 patients) received 1 g/kg of mini-pool IVIG; Group B (24 patients) received standard IVIG treatment of 1 g/kg; and Group C (24 patients) received no platelet-enhancing treatment. Results showed 16.6 percent of patients in Group A had a response, and 58.8 percent had a complete response. Comparably, in Group B, 16.6 percent had a response, and 66.6 percent had a complete response. Only 33.3 percent of patients in Group C had a complete response. Time to response was eight days in Group A, nine days in Group B and 21 days in Group C.

“Mini-pool intravenous immunoglobulin G was well-tolerated, presented no safety issues and was effective in the treatment of immune thrombocytopenia, with efficacy comparable to that of the standard intravenous immunoglobulin G group, and it was significantly more effective than no treatment,” said study authors. ITP leads to excessive bruising and bleeding as a result of low levels of platelets (cells that help blood clot). Pediatric ITP patients are typically treated with high levels of IVIG, which contains plasma extracted from thousands of blood donations.
IN THE NEWS

The board of directors of the Plasma Protein Therapeutics Association (PPTA), the world’s leading trade association that represents more than 750 human plasma collection centers in North America and Europe, as well as the manufacturers of lifesaving plasma protein therapies, named Amy Efantis as its president and chief executive officer effective Jan. 16. Efantis most recently served as vice president of global public policy and government affairs at Biogen. Previous to her role with Biogen, she held roles with Boehringer Ingelheim, PhRMA, and worked on Capitol Hill as a congressional legislative director and, prior to this role, advised Rep. Thomas Barrett (D-WI) on various House Energy and Commerce Committee issues, primarily in the healthcare policy areas.

“I am thrilled and honored to be joining the PPTA and the vital efforts of the plasma industry,” said Efantis. “To represent companies whose mission is serving patients with rare diseases that can be treated through the development of plasma-derived products is a stewardship role that I embrace with humility and a deeply felt passion.”


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Research

Fostamatinib Shown Effective for ITP

A new study finds fostamatinib produces clinically meaningful responses in adults with immune thrombocytopenia (ITP). In the two parallel Phase III trials conducted at Weill Cornell Medicine in New York City, patients with persistent/chronic ITP (median duration 8.5 years) were randomized in a two-to-one ratio to fostamatinib (101 patients) or placebo (49 patients) at 100 mg twice daily for 24 weeks with a dose increase to 150 mg twice daily after four weeks in non-responders. Results showed stable responses occurred in 18 percent of patients on fostamatinib versus 2 percent on placebo. Forty-three percent of patients on fostamatinib and 14 percent on placebo achieved overall responses (defined as greater than or equal to one platelet count of greater than or equal to 50,000 µL within the first 12 weeks on treatment). With the 100 mg dosage, the median time to response was 15 days, and 83 percent responded within eight weeks. Diarrhea, hypertension, nausea, dizziness and alanine aminotransferase increase were the most common adverse events, all of which were more frequent with fostamatinib versus placebo. However, most adverse events were mild or moderate and resolved spontaneously or with medical management. According to the study’s authors, “fostamatinib is a novel ITP treatment option that targets an important mechanism of ITP pathogenesis.”


Study

Prednisolone Improves Coronary Artery Outcomes in KD Patients at High Risk of IVIG Resistance

A new study shows that a primary intravenous immune globulin (IVIG) and prednisolone combination therapy might prevent coronary artery abnormalities and contribute to lowering medical costs in Kawasaki disease (KD) patients. The study was conducted in response to results of the randomized controlled trial to assess immunoglobulin plus steroid efficacy (RAISE) for KD, which showed additional prednisolone improved coronary artery outcomes in patients at high risk of intravenous immune globulin (IVIG) resistance. However, no studies had been conducted to test the steroid regimen used in the RAISE study.

In the multicenter, prospective cohort study at 34 hospitals in Japan, 2,628 KD patients were enrolled from July 1, 2012, through June 30, 2015, 724 of whom were predicted IVIG nonresponders who received IVIG plus prednisolone as primary treatment. For these, each hospital independently decided whether to add prednisolone (intravenous injection of 2 mg/kg per day for five days) to the primary IVIG treatment. The primary endpoint was the incidence of coronary artery abnormalities determined by two-dimensional echocardiography at one month after the primary treatment in predicted nonresponders. Results showed 132 of the 724 patients did not respond to primary treatment. Among patients with complete data, coronary artery abnormalities were present in 40 of 676 patients, according to American Heart Association criteria, or in 26 of 677 patients, according to Japanese criteria. Serious adverse events were reported in 12 of 724 patients, two of whom had hypertension and bacteraemia that was probably related to prednisolone. One patient died possibly due to severe inflammation from KD.

The researchers concluded primary IVIG plus prednisolone therapy in this study had a similar effect to that seen in the RAISE study in reducing the nonresponse rate and decreasing the incidence of coronary artery abnormalities.


Prednisolone Improves Coronary Artery Outcomes in KD Patients at High Risk of IVIG Resistance
The U.S. Food and Drug Administration has approved Amgen’s romiplostim to treat pediatric patients ages 1 year and older with immune thrombocytopenia (ITP) for a minimum of six months and who have had an insufficient response to corticosteroids, immune globulin therapy or splenectomy.

Approval was based on two double-blind placebo-controlled clinical trials in pediatric patients 1 year and older with ITP for at least six months. In one study, patients whose disease was refractory or relapsed after at least one prior ITP therapy were randomized to receive romiplostim or placebo. Durable platelet response (at least six weekly platelet counts greater than or equal to 50 \times 10^9/L during weeks 18 through 25 of treatment) was achieved in 22 patients (52 percent) who received romiplostim and two (10 percent) on the placebo arm. Overall platelet response, defined as a durable or a transient platelet response, was achieved in 30 (71 percent) and four (20 percent) patients, respectively. Patients who received romiplostim had platelet counts greater than or equal to 50 \times 10^9/L for a median of 12 weeks, compared to one week in patients who received placebo. The results for all three endpoints were statistically significant, with p-values all less than 0.05.

In the other study, patients diagnosed with ITP at least six months prior to enrollment were randomized to receive romiplostim or placebo. Fifteen patients who received romiplostim achieved a platelet count of greater than or equal to 50 \times 10^9/L for two consecutive weeks and an increase in platelet count of greater than or equal to 20 \times 10^9/L above baseline for two consecutive weeks during the treatment period. No patient receiving placebo achieved either endpoint.

In pediatric patients, the most common adverse reactions included contusion, upper respiratory tract infection and oropharyngeal pain.

Yoga for Chronic Disease Management

Research shows yoga is beneficial for chronic disease management, but patients should adapt poses described here to align with their physical abilities.

By Matthew D. Hansen, DPT, MPT, BSPTS
IN TODAY’S WESTERN society, the word “yoga” often invokes images of young and fit participants dressed in vibrantly colored stretchy yoga pants and posed in seemingly unattainable postures. While many consider yoga a textbook example of cultural appropriation, that doesn’t mean everyone shouldn’t be able to benefit from this ancient practice.

Many of the asanas (postures) of Hatha yoga, the most recognizable form of yoga in the United States, can safely be used to improve flexibility. However, yoga’s benefits in its many forms extend far beyond improved range of motion. To obtain these benefits, one must first understand a bit about the history and nature of yoga and, ideally, learn from a yogi who is already practiced in the art.

Most scholars agree that yoga was first developed some 2,500 to 5,000 years ago in India — not as a religion in and of itself (although several religions incorporate its practices) but as a system of spiritual, physical and emotional health. The meaning of the Sanskrit word stems from one of two roots meaning “to concentrate” or “to yoke” (also translated to mean “to unite” or “to control”). Depending on who you ask, there are between four and six main types (paths) of yoga. Hatha yoga, with its familiar “downward-facing dog” (Adho Mukha Svanasana), “child’s” (Balasana) and “warrior” (Virabhadrasana) poses, to name a few, is just one of these paths. One of the newest forms of yoga, Hasyayoga, isn’t one of its main paths, but it is still one of my favorites. If you’re feeling down and need a boost of joy in your day, start by Googling the term “laughter yoga” (Hasyayoga’s English translation). It doesn’t take long to learn the practice, and just watching others do it will put you on the right road.

I recommend learning the basics of all of yoga’s different paths, but for physical and emotional benefits, Hatha yoga is a good place to start. It’s much more than assuming a posture though. It’s meditation and a state of mind that are brought about by one of yoga’s most important components: breathing (also referred to as Pranayama).

Yoga Breathing (Pranayama)

Breathing should be rhythmic and controlled, usually through the nostrils, although it is also appropriate to breath through the mouth if the nasal passage is obstructed, temporarily or otherwise. Breathing will naturally become more labored with increased effort; however, if the quality of breath becomes compromised or if participants were able to breathe through their nose at rest but are no longer able to do so, it is a good indicator they should pause and rest until proper technique can be resumed for yoga breathing.

There are many different yoga breathing techniques, but the following steps for the “victory breath” (Ujjayi Pranayama), although a bit difficult to master at first, describe a technique frequently used during Hatha yoga.

Step 1: First learn the technique by breathing through your mouth. Relax your body, jaw and tongue so your mouth slightly drops open.

Step 2: Inhale and exhale deeply while (here’s the tricky part) slightly contracting the back of your throat and softly whispering the sound “ahh” as you exhale. Some yogis have compared the sensation during inhalation to sucking through a wide straw and the sensation during exhalation to fogging up a window. Your breath should make an “ocean” sound like the tide softly rolling in and out.

Step 3: Once the technique has been mastered, close your mouth and begin breathing through the nose while maintaining soft lips and the same constriction in the throat. The breath should still sound the same.

Step 4: Fully expand the lungs during inhalation, and completely release the air during exhalation.

Step 5: Gradually begin to connect breath with yoga postures. Inhale with the victory breath as you extend into a posture, and exhale as you contract or assume folded postures (i.e., postures that entail bending forward).

Another great yoga breathing technique to use prior to a session or other workout, or simply to clarify the mind and energize the body at any time during the day, is the “bellows breath” (Bhastrika Pranayama). It is also one of the easiest techniques to learn (see steps below), but should not be practiced by someone who has seizures, panic attacks or uncontrolled hypertension, or pregnant women. It’s also not a good idea to perform bellows breathing soon after eating on a full stomach.

Step 1: Inhale deeply through your nostrils, allowing the diaphragm to expand downward and the abdomen to swell outward first, and the chest to expand afterward, followed by a rising of the collar bones.

Step 2: Exhale rapidly through your nostrils (I teach that exhalation should take approximately half the time of inhalation). Feel the collar bones drop, your chest deflate and the abdomen return to its position. Keep the head, shoulders and neck still when breathing.

Step 3: Repeat the process for a cycle of 10 breaths. If you wish to continue, take a 30-second break and either begin a
new cycle of 10 breaths or increase the number of breaths per cycle to 20, and then to 30 after a 30-second break between each cycle.

**Yoga Postures (Asanas)**

Which postures to incorporate into a Hatha yoga routine depends on the intent of the activity and the targeted body part(s). There are many benefits of following a particular yoga sequence (series of related, often progressive postures), but most importantly, a yoga routine should be nonharmful. Some postures for certain people can be outright dangerous. Most postures for the majority of people can be harmful if not performed properly. The self-declared ultimate goal of yoga is liberation (moksha), not mandatory bed rest or a trip to urgent care.

There are many options for adapting a particular posture. Your cobra pose (Bhujangasana) may not look more like Figure 1 than Figure 2, and that’s perfectly all right! With time, your pose may look more like Figure 1 or it may not. Even if you can’t fully lift your head up on your own, that’s perfectly all right, too!

A typical routine consists of pre-yoga stretching, breathing exercises to focus and relax the body, warm-up postures, progressive workout postures and a relaxation (cool-down) period. Participation, mindfulness and proper breathing are just as important to the ultimate benefits of yoga if someone is not able to perform the progressive postures, although tools, including blocks/bricks, straps, bolsters and yoga balls, are available to enhance or assist with assuming and maintaining a posture.

While I would never prescribe specific postures to people without first getting to know them and their particular health situation, the asanas in Figures 1 through 8 are often recommended for beginners due to their adaptability and generally decreased strain on the body. Remember that your performance of the postures doesn’t have to look just like the figures. For example, imagine how you might adapt it to your ability. If being on your knees is difficult, think how you might be able to adapt the pose in a chair.

**Evidence to Support Yoga for Chronic Disease Management**

Numerous studies during the past decade have documented the physical and mental benefits of practicing yoga, including benefits enjoyed specifically by autoimmune disease and immunodeficient patient populations. Benefits may include improved flexibility, body alignment, strength, balance, stamina, proprioception (body awareness) and sleep, as well as reduced stress and pain. Fortunately, there is also specific support for the benefits of yoga on the immune system.

Building on research findings conducted earlier in the decade supporting yoga’s positive effects on several immunological indicators, a 2018 meta-analysis published in the *Journal of Behavioral Medicine* found yoga appears to improve the body’s immune system and decrease chronic inflammation. Another study published in *BMC Complementary and Alternative Medicine* reported yoga breathing for just 20 minutes was enough to lower stress-related inflammation markers. And, a 2013 meta-analysis of 17 different studies that looked at the effects of yoga for rheumatoid arthritis, fibromyalgia and unspecified back pain found yoga significantly improved pain and psychological outcomes in all cases.

**Getting Started**

The greatest challenge to starting any new physical routine for someone living with a chronic health condition is usually simply getting started. Any addition or disruption to someone’s daily routine can cause anxiety. Here are some tips to help:

1) **Prepare.** Yoga doesn’t require much, but you will want to wear nonrestrictive clothing and have a comfortable surface on which to perform postures (e.g., a yoga mat).
2) Start slow. Don’t overdo it! Even starting with one or two postures for a few minutes a day can bring positive results.
3) Be consistent. Creating a habit and subsequent change requires consistency. If you don’t feel up to performing your full routine or even any postures on a given day, consider at least performing breathing exercises. Just about anyone can perform the corpse pose while breathing meditatively, no matter how badly their day is going.
4) Adapt and/or seek help. Adapting postures can take a bit of imagination, but it can also be fun. If you need additional help, find a professional in your area who teaches yoga therapy or who can guide you through a session of restorative yoga. Search for a certified yoga therapist or a physical therapist or certified athletic trainer who has special training in yoga. If you aren’t comfortable trying physical yoga yet or have difficulty with mindfulness meditation, consider trying yoga sleep (Yoga Nidra), a guided meditation through words and imagery that helps relaxation.

Lastly, whatever you do, enjoy your new path to improved wellness. As you begin to practice yoga, you should be left feeling more relaxed and at peace in your life.

Namaste!

MATTHEW DAVID HANSEN, DPT, MPT, BSPTS, is a practicing physical therapist in Utah and president of an allied healthcare staffing and consulting agency named SOMA Health, LLC. He completed his formal education at the University of Utah, Salt Lake City, and has additional training in exercise and sports science, motor development and neurological and pediatric physical therapy.

References
Preparing for College with a Chronic Illness

Chronically ill young adults face many challenges as they plan their postsecondary education, and while these challenges are difficult, they are not insurmountable.

By Abbie Cornett

GRADUATING HIGH school can be one of the most exciting and scary times in young adults’ lives. For most, completing secondary education represents having achieved their first milestone on the road to success as an adult. And, while this triumph culminates years of hard study for all students, it is a particularly impressive accomplishment for graduates who are battling chronic illness.

For those who choose to attend college, the battle to complete their education has just begun. Research has shown that, as a group, children with long-term illness are at very high risk of educational and vocational problems. Not only are they less likely to graduate high school, they are also less likely to attend or graduate college. Failure to address the issues chronically ill young adults face can have long-term employment and financial repercussions. So, what can be done to prevent the college dream from becoming a nightmare?

Understanding the Law

It is likely high school graduates haven’t given much thought to the laws that have protected them up to this point in their education. Access to education has been something their parents have managed. But, upon entering college, that changes. Expectations are that college students have reached a level of maturity to advocate for themselves. Unfortunately, this is frequently untrue due to lack of knowledge and experience. Therefore, the critical first step to success is to become familiar with the laws in place to protect their right to an education.

The Americans with Disabilities Act (ADA) and Section 504 of the Rehabilitation Act of 1973 protect the rights of persons with handicaps in programs and activities that receive federal financial assistance. Further, Section 504 protects the rights not only of individuals with visible disabilities but also those with hidden disabilities (those that are not obvious), including chronic illnesses. And, while the act does not list specific disabilities, its language specifies that individuals are protected if their physical or mental impairment results in a substantial limitation of one or more major life activities.

Understanding Civil Rights

Although college isn’t an entitlement, all individuals do have a civil right to higher education. This means institutions have to provide access and opportunity to participate in college by providing reasonable accommodations. But, it doesn’t mean institutions are obligated to guarantee an education if students don’t meet the criteria for admission, nor are institutions required to guarantee success.

According to Liz Brown, disability services specialist at Bellevue University in Nebraska, educational institutions receiving federal funds cannot exclude otherwise qualified individuals with disabilities. This means those who meet all applicable qualifications cannot be prevented from pursuing postsecondary education based on their disability status. However, they must meet academic and technical standards for the program and/or institution just like any other student.

In addition, students with disabilities are allowed to request reasonable accommodations that create equitable opportunity in the postsecondary setting. But, reasonable accommodations cannot fundamentally alter academic standards or program objectives that are essential for every student. Common reasonable accommodations include attendance flexibility, extended time on exams, reduced-distraction testing environments, note-taking services, preferential seating, alternative format textbooks and use of assistive technology.
It is important for students to understand the ADA defines disability broadly, so those who don’t necessarily identify as having a disability may still qualify. If it is determined reasonable accommodations are warranted, it is then decided what accommodations are appropriate. Examples of accommodations a college might offer include:

- Recording of lectures or having a note-taker with the student in class
- Extending the amount of time needed to take a test, or taking tests in smaller sections over a period of time
- Extending time on assignments
- Using adaptive technology such as a smart pen or screen reader for tests and assignments
- Special seating
- Accommodating absences

Registering for Accommodations

The first step in registering for reasonable accommodations is to contact disability services at the institution to which they are applying and directly disclose a documented disability. Once the disability has been disclosed, students are instructed to submit an intake form, release form and appropriate medical documentation such as the evidence of disability that includes:

- The disability’s diagnosis;
- How the disability limits a major life activity;
- How the disability can affect academic performance; and
- Accommodations requested.

Based on the students’ self-report and medical documentation, the disability services office will determine if reasonable accommodations are warranted and what accommodations are appropriate.

Before any information is sent to instructors, students will be asked to review the assigned accommodations and approve them. Instructors will then be notified of the approved accommodations. No disability-specific information is shared; instructors are only informed that students have been determined eligible for reasonable accommodations under the ADA. All disability/medical documentation is kept in a secure and confidential file that is available only to disability services staff.

The School Matters

When universities look at requests for accommodations, they must determine if the condition substantially limits a major life activity such as learning, says Brown. Universities have come a long way and typically do a good job when it comes to accommodating visible disabilities such as hearing, visual or mobility impairments. Unfortunately, though, many still have room for improvement when it comes to dealing with chronic conditions, autoimmune diseases and rare diseases.

Unlike students with visible disabilities, those with chronic invisible illnesses frequently encounter professors and university personnel who fail to understand the unpredictability of their illnesses. These individuals don’t understand how the students can be well one day and sick the next, which subjects students to disbelief and claims of malingering.

In addition, students with a chronic illness can face many other issues. Often, they are forced to miss class, sometimes for lengthy periods due to medical appointments, illness or hospital stays. They may also be required to take medication that has incapacitating side effects or may make them drowsy or fatigued during class.

Recognizing the many challenges of chronic illness, a number of schools are committed to working directly with students with disabilities to help ensure access to higher education. One such school is Bellevue University, a member of the Association on Higher Education and Disability (AHEAD), an organization of professional members who are involved in the development of policy, as well as the provision of services that meet the needs of people with disabilities, and who are involved in every area of higher education. Formed in 1977, AHEAD began delivering training to personnel who provide higher education via conferences, publications, workshops and consultation. Its members are a diverse network of professionals who actively pursue disability issues on
Important Safety Information

WARNING: Thrombosis (blood clots) can occur with immune globulin products, including Hizentra. Risk factors can include: advanced age, prolonged immobilization, a history of blood clotting or hyperviscosity (blood thickness), use of estrogens, installed vascular catheters, and cardiovascular risk factors.

If you are at high risk of blood clots, your doctor will prescribe Hizentra at the minimum dose and infusion rate practicable and will monitor for signs of clotting events and hyperviscosity. Always drink sufficient fluids before infusing Hizentra.

See your doctor for a full explanation, and the full prescribing information for complete boxed warning.

Hizentra is a prescription medicine used to treat:

- Primary immune deficiency (PI) in patients 2 years and older
- Chronic inflammatory demyelinating polyneuropathy (CIDP) in adults

Treatment with Hizentra might not be possible if your doctor determines you have hyperprolinemia (too much proline in the blood), or are

IgA-deficient with antibodies to IgA and a history of hypersensitivity. Tell your doctor if you have previously had a severe allergic reaction (including anaphylaxis) to the administration of human immune globulin. Tell your doctor right away or go to the emergency room if you have hives, trouble breathing, wheezing, dizziness, or fainting. These could be signs of a bad allergic reaction.

Inform your doctor of any medications you are taking, as well as any medical conditions you may have had, especially if you have a history of diseases related to the heart or blood vessels, or have been immobile for some time. Inform your physician if you are pregnant or nursing, or plan to become pregnant.

Infuse Hizentra under your skin only; do not inject into a blood vessel. Self-administer Hizentra only after having been taught to do so by your doctor or other healthcare professional, and having received dosing instructions for treating your condition.
Immediately report to your physician any of the following symptoms, which could be signs of serious adverse reactions to Hizentra:

- Reduced urination, sudden weight gain, or swelling in your legs (possible signs of a kidney problem).
- Pain and/or swelling or discoloration of an arm or leg, unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, or numbness/weakness on one side of the body (possible signs of a blood clot).
- Bad headache with nausea; vomiting; stiff neck; fever; and sensitivity to light (possible signs of meningitis).
- Brown or red urine; rapid heart rate; yellowing of the skin or eyes; chest pains or breathing trouble; fever over 100°F (possible symptoms of other conditions that require prompt treatment).

Hizentra is made from human blood. The risk of transmission of infectious agents, including viruses and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent and its variant (vCJD), cannot be completely eliminated.

The most common side effects in the clinical trials for Hizentra include redness, swelling, itching, and/or bruising at the infusion site; headache, chest, joint or back pain; diarrhea; tiredness; cough; rash; itching; fever, nausea, and vomiting. These are not the only side effects possible.

Before receiving any vaccine, tell immunizing physician if you have had recent therapy with Hizentra, as effectiveness of the vaccine could be compromised.

Please see brief summary of full prescribing information for Hizentra on adjacent page. For full prescribing information, including boxed warning and patient product information, please visit Hizentra.com.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Why Choose Hizentra?

Choose where you infuse

Self-administration with Hizentra means you and your doctor can decide where you can infuse. Convenient dosing routines mean you won’t have to adjust or cancel your plans due to IV infusion appointments.

No IV infusions

IV infusions can be challenging for people who have hard-to-find or damaged veins. Hizentra allows you to infuse just under the skin, not into a vein, after training from your doctor.

Proven safety

Hizentra has an established safety profile and demonstrated tolerability. In clinical trials, the most common side effects were redness, swelling, itching, and/or bruising at the infusion site; headache, chest, joint or back pain; diarrhea; tiredness; cough; rash; itching; fever, nausea, and vomiting. These are not the only side effects possible.

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Gaylord National Resort & Convention Center
National Harbor, MD | June 20–22

Immune Deficiency Foundation
BRIEF SUMMARY OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use HIZENTRA safely and effectively. See full prescribing information for HIZENTRA.

WARNING: THROMBOSIS

See full prescribing information for complete boxed warning.

- Thrombosis may occur with immune globulin products, including HIZENTRA. Risk factors may include: advanced age, prolonged immobilization, hypercoagulable conditions, history of venous or arterial thrombosis, use of estrogens, indwelling vascular catheters, hyperviscosity, and cardiovascular risk factors.

- For patients at risk of thrombosis, administer HIZENTRA at the minimum dose and infusion rate practicable. Ensure adequate hydration in patients before administration. Monitor for signs and symptoms of thrombosis and assess blood viscosity in patients at risk for hyperviscosity.

- CONTRAINDICATIONS

- Anaphylactic or severe systemic reaction to human immune globulin or components of HIZENTRA, such as polysorbate 80
- Hyperprolinemia (type I or II) (HIZENTRA contains the stabilizer L-proline)
- IgA-deficient patients with antibodies against IgA and a history of hypersensitivity

- WARNINGS AND PRECAUTIONS

- IgA-deficient patients with anti-IgA antibodies are at greater risk of severe hypersensitivity and anaphylactic reactions.
- Thrombosis may occur following treatment with immune globulin products, including HIZENTRA.
- Aseptic meningitis syndrome has been reported with IGIV or IGSC, including HIZENTRA treatment.
- Monitor renal function, including blood urea nitrogen, serum creatinine, and urine output in patients at risk of acute renal failure.
- Monitor for clinical signs and symptoms of hemolysis.
- Monitor for pulmonary adverse reactions (transfusion-related acute lung injury [TRALI])
- HIZENTRA is made from human plasma and may contain infectious agents, e.g., viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent.

- ADVERSE REACTIONS

The most common adverse reactions observed in ≥5% of study subjects were local infusion site reactions, headache, diarrhea, fatigue, back pain, nausea, pain in extremity, cough, upper respiratory tract infection, rash, pruritus, vomiting, abdominal pain (upper), migraine, arthralgia, pain, fall and nasopharyngitis.

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring Pharmacovigilance at 1-866-915-6958 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

- DRUG INTERACTIONS

The passive transfer of antibodies may interfere with the response to live virus vaccines, and lead to misinterpretation of the results of serological testing.

Based on March 2018 revision
campuses they are involved with, as well as in the field of higher education. The association is active in all aspects of promoting equal and full participation by people who experience forms of disabilities in higher education. And, it supports the institutions, systems, professionals and professions that are important for attaining their mission.

But, not all higher-education institutions are members of AHEAD. And, while that doesn’t mean those institutions can’t meet students’ needs, it is important for students to perform due diligence to ensure the school they are considering recognizes the impact of chronic illness on students’ success. To do this, students can start by contacting the school’s disability services office to ask the following questions:

• What services are typically available for students with disabilities?
• Where is the disability office located, and what are its hours of operation?
• Is there a document that describes available services?
• Are there other students enrolled with similar disabilities?

Don’t Be Afraid of the Word “Disability”

When preparing for college, students need to decide early on if they are going to disclose their disability. While the decision to disclose is a personal choice, it is important to keep in mind that disclosure is a requirement for receiving accommodations. Unfortunately, many times students with a chronic illness shy away from the word “disability” or don’t think disability services are relevant to their condition. According to a U.S. Department of Education study, about half of those with disabilities enrolled in postsecondary coursework did not define themselves as disabled, and 40 percent of students in their study did not inform their schools of their disability.9

Unlike the K-12 school system, postsecondary schools aren’t obligated to identify students who need accommodations. And, if students fail to register as disabled before their illness requires the need for accommodations, they can face devastating economic and educational consequences such as the loss of scholarships, automatic withdrawal from classes or failing grades. At the postsecondary level, it is the students’ responsibility to make their disability known and to request academic adjustments.2

Like all other areas of life, success in college is dependent on how well students communicate. Brown’s first recommendation to students is to be willing to ask for help. This could be from anyone with whom students feel more comfortable, including disability services, an academic advisor or an instructor.

Fulfilling the College Dream

College life can be a struggle for anyone with or without a chronic illness. And, for those with a chronic illness, certain rights and laws are in place to help them succeed. But, it’s the students’ responsibility to communicate their disability to the school at the postsecondary level. In addition, students need to be proactive to ensure the schools they are considering are both committed to and have programs in place to assist them. Only then can they have the best chance of fulfilling their college dream.

ABBIE CORNETT is the patient advocate for IG Living magazine.

References

Additional Resources
• Disabilities, Opportunities, Internetworking and Technology (DO-IT) — www.washington.edu/doit
• Going to College: A Resource for Teens with Disabilities — www.going-to-college.org
• The Chronic Illness Initiative: Supporting College Students with Chronic Illness Needs at DePaul University — files.eric.ed.gov/fulltext/EJ825778.pdf
• The Civil Rights of Students with Hidden Disabilities Under Section 504 of the Rehabilitation Act of 1973 — www2.ed.gov/about/offices/list/ocr/docs/hq5269.html
• We Connect Now — weconnectnow.wordpress.com
Properly Disposing of Medicines

Safely disposing of unneeded and expired medicines will help to save thousands of lives each year.

By Ronale Tucker Rhodes, MS

ACCORDING TO RESEARCH, most people understand the importance of disposing of unneeded and/or expired medicines, yet one in five reports being unaware of safe disposal guidelines. Failure to heed the advice of health and government organizations on how to safely dispose of medicines can be dangerous for a variety of reasons. For starters, medications lose their effectiveness after the expiration date, and some may be toxic. More importantly, unneeded medicines create unnecessary health risks for toddlers, teens and even pets accidentally ingesting them. In 2018, a report by the American Academy of Pediatrics found the number of children hospitalized for opioid poisoning increased by three-fold between 1997 and 2012, and the largest overall increase was among toddlers and preschoolers.

And, a U.S. government review shows more than half of all people who first misuse prescription drugs get them from their friends, relatives or simply take them without asking. In fact, more than 115 people die each day from opioid (narcotic) overdoses.

When to Dispose

It’s not uncommon for people to find their cabinets are overflowing with medicines, including prescription and over-the-counter drugs. And, while many consider this just a nuisance, there are many reasons it may be time to dispose of them.

First, if a medication is expired, it may be less effective and can lead to overuse and potential overdose. Over time, the chemical composition of expired medicines can even change,
which can make even a harmless over-the-counter drug very risky to take. According to U.S. Food and Drug Administration pharmacist Lisa Bernstein, “Once the expiration date has passed, there is no guarantee that a medication will be safe and effective.”

Second, people often fail to take all of their medication, and the remaining supply is often allowed to remain in the cabinet. This could happen when a prescription is changed, or the patient felt better and was advised to stop taking the medicine. Or, it could just be some over-the-counter medicines are no longer needed.

Third, medicines sometimes become unidentifiable. When the drug’s name is unfamiliar, especially if it has been years since the prescription was filled or the medicine was purchased, it is no longer useful. Frequently, adults simply fail to remember to take all of their medicines at the right time or in the correct dosages, resulting in leftover doses. Or, it could be because the medicines were transferred into other containers, and are no longer recognizable.

Lastly, medicines can become damaged, which can make people sick. Individuals should always discard medicines that have changed color, texture or smell, even if they aren’t expired.

### Take-Back and Collection Options

The safest solution for disposing of unneeded prescription medicines is community take-back programs. There are two types of take-backs: periodic events and permanent collection sites.

In 2010, the U.S. Drug Enforcement Administration (DEA) began hosting no-questions-asked national drug take-back events twice per year at temporary collection sites in local cities throughout the U.S. for safe disposal of prescription drugs, including opioids. The initiative is one of several strategies to reduce prescription drug abuse and diversion in the U.S. At the DEA’s last take-back event held Oct. 17, 2018, 914,236 pounds (457.12 tons) of prescription medicines were collected. Since 2010, 10,878,950 pounds of medicines have been collected. This year, the first national take-back day will be held April 27 (the fall take-back day has not yet been announced). Collection sites for this first event can be located starting April 1 at the Diversion Control Division website at www.deadiversion.usdoj.gov/drug_disposal/takeback/index.html. See Table 1 for a list of items that can and cannot be disposed of at take-back events.

DEA-registered collectors will also safely and securely collect and dispose of pharmaceuticals containing controlled substances and other medicines. Permanent collection sites consist of retail pharmacies, hospital or clinic pharmacies, and law enforcement facilities. In addition, some authorized collection sites may offer mail-back programs or collection receptacles (also known as drop-boxes). DEA-registered collection centers can be found at the Diversion Control Division website using the controlled substance public disposal locations search utility at apps.deadiversion.usdoj.gov/pubdispsrch/spirut/main?execution=e1s1.

### Table 1. What Can and Cannot Be Accepted at DEA National Take-Back Events

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<th>Can Be Accepted:</th>
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<tbody>
<tr>
<td>Prescription medications, including prescribed controlled substances (DEA Schedule II–V)</td>
</tr>
<tr>
<td>Over-the-counter medications</td>
</tr>
<tr>
<td>Liquid medications (small amounts in original, nonleaking containers)</td>
</tr>
<tr>
<td>Medicated patches such as used Fentanyl and Duragesic patches, which are extremely hazardous (they may also be folded in half, sticky-side together, and flushed down the toilet)</td>
</tr>
<tr>
<td>Medication samples</td>
</tr>
<tr>
<td>Medicated ointments</td>
</tr>
<tr>
<td>Vitamins</td>
</tr>
<tr>
<td>Pet medications</td>
</tr>
<tr>
<td>Unused drug injection cartridges such as unused EpiPens and insulin pens (must be unused with needle still protected inside)</td>
</tr>
<tr>
<td>Unused inhaler canisters such as Advair, Spiriva, ProAir and Ventolin (must be unused, no empty canisters or unneeded plastic holders/mouth pieces)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Cannot Be Accepted:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Marijuana</td>
</tr>
<tr>
<td>Illicit drugs (e.g., DEA Schedule I drugs like heroin, LSD, etc.)</td>
</tr>
<tr>
<td>Needles, syringes and other sharps</td>
</tr>
<tr>
<td>Chemotherapy drugs</td>
</tr>
<tr>
<td>Medical tools and supplies</td>
</tr>
<tr>
<td>Bloody or infectious waste</td>
</tr>
<tr>
<td>Personal care products</td>
</tr>
<tr>
<td>Thermometers</td>
</tr>
<tr>
<td>Empty containers</td>
</tr>
<tr>
<td>Medication wastes generated by healthcare facilities, including nursing homes</td>
</tr>
</tbody>
</table>

Source: Colorado Department of Public Health Information. Items That Can or Cannot Be Accepted. Accessed at www.colorado.gov/pacific/cdphe/items-can-or-cannot-be-accepted.
Donation and Reuse Programs

Pharmaceutical donation and reuse programs are distinct prescription drug programs providing for unused prescription drugs to be donated and redispensed to patients. These drug repository programs began in 1997, and as of fall 2018, 38 states and Guam had enacted laws for donation and reuse. However, more than a dozen of these states do not have functioning or operational programs, which are defined as those that have participating pharmacies, charitable clinics and/or hospitals collecting and redistributing donated drugs to eligible patients. The reason for the lack of operational programs include lack of awareness about the programs, no central agency or entity designated to operate and fund the program, and added work and responsibility for repository sites that accept the donations.

For those that are operational, there are substantial restrictions on who can donate and what types of prescription products may be donated, as well as strict safety rules intended to protect patients who ultimately obtain and take the drugs. In many states, all donations must meet standards such as:

- Only professionally designated persons are allowed to make a donation, although some states do allow individual patients to donate directly;
- Pills in opened or partly used bottles are never accepted;
- Old drugs are never accepted, and expiration dates (often at least six months later than the date of donation) must be visible;
- Commonly, donated drugs must be delivered to a specific type of medical or pharmacy facility, and some may require the donor to sign a form or waiver; and
- Financial compensation or payment to the donor is usually prohibited, although donations may be tax-deductible if paid for by the individual patient and taxpayer. Beyond donation programs, patients and other individuals may not sell any prescription drugs; such transactions are strictly regulated by State Boards of Pharmacy and other state and federal laws.

In addition, while DEA take-back events do not allow cancer-related prescription drugs, drug depository programs do. In fact, the following 13 states accept these drugs for distribution: Colorado, Florida, Kentucky, Michigan, Minnesota, Montana, Nebraska, Nevada, Ohio, Pennsylvania, Utah, Washington and Wisconsin.

Flushing Options

When take-back and donation options are unavailable, FDA recommends flushing certain potentially dangerous medicines (Table 2). However, some have raised concerns about these medicines’ impact on the environment and

<table>
<thead>
<tr>
<th>Active Ingredient</th>
<th>Found in Brand Names</th>
</tr>
</thead>
<tbody>
<tr>
<td>Benzhydrocodone /Acetaminophen</td>
<td>Apadaz</td>
</tr>
<tr>
<td>Buprenorphine</td>
<td>Belbuca, Bunavail, Butrans, Suboxone, Subutex, Zubsolv</td>
</tr>
<tr>
<td>Fentanyl</td>
<td>Abstral, Actiq, Duragesic, Fentora, Onsolis</td>
</tr>
<tr>
<td>Diazepam</td>
<td>Diastat/Diastat AcuDial rectal gel</td>
</tr>
<tr>
<td>Hydrocodone</td>
<td>Anexia, Hysingla ER, Lortab, Norco, Reponexin, Vicodin, Vicoprofen, Zohydro ER</td>
</tr>
<tr>
<td>Hydromorphone</td>
<td>Dilaudid, Exalgo</td>
</tr>
<tr>
<td>Meperidine</td>
<td>Demerol</td>
</tr>
<tr>
<td>Methadone</td>
<td>Dolophine, Methadose</td>
</tr>
<tr>
<td>Methylenphenidate</td>
<td>Daytrana transdermal patch system</td>
</tr>
<tr>
<td>Morphine</td>
<td>Arymo ER, Embeda, Kadian, Morphabond ER, MS Contin, Awinza</td>
</tr>
<tr>
<td>Oxycodone</td>
<td>Combunox, Oxydo (formerly Oxecta), OxyContin, Percocet, Percodan, Roxicet, Roxicodone, Roxbybond, Targiniq ER, Xartemis XR, Xtampza ER</td>
</tr>
<tr>
<td>Oxymorphone</td>
<td>Opana, Opana ER</td>
</tr>
<tr>
<td>Tapentadol</td>
<td>Nucynta, Nucynta ER</td>
</tr>
<tr>
<td>Sodium Oxybate</td>
<td>Xyrem oral solution</td>
</tr>
</tbody>
</table>

contamination of surface and drinking water supplies. Therefore, FDA investigated the ecological and human-health risks associated with the environmental release of the 15 active pharmaceutical ingredients (API) in the medicines FDA recommends flushing. In the investigation, researchers found “even when highly conservative assumptions are used, including that the entire API mass supplied for clinical use is flushed, all relevant sources in addition to clinical use of the API are considered, and no metabolic loss, environmental degradation or dilution of wastewater effluents are used in estimating environmental concentrations, most of these APIs present a negligible eco-toxicological risk, both as individual compounds and as a mixture.” However, the researchers did say additional eco-toxicological data will need to be developed for a few of these APIs. But, their final conclusion was all 15 APIs present negligible risk through ingestion of water and fish.8

**Personal Disposal of Medicines**

Individuals can also dispose of most medications on their own. If there are any specific instructions for disposal on the label, package or package insert, those instructions should be followed. Otherwise, disposal into the household trash is the common method. FDA recommends following these specific steps when disposing of medicines in the trash:6

- Mix medicines (do not crush tablets or capsules) with an unpalatable substance such as dirt, cat litter or used coffee grounds;
- Place the mixture in a container such as a sealed plastic bag;
- Throw the container into the household trash; and
- Delete all personal information on the prescription label of empty pill bottles or medicine packaging, and dispose of the container.

There are also technologies that have been developed that can be used to dispose of medicines that come in the form of pouches and liquids.

**Medicine Disposal Resources**

- Environmental Protection Agency: How to Dispose of Medicines Properly (go.usa.gov/xNwXc)
- U.S. Food and Drug Administration: Disposal of Unused Medicines: What You Should Know (go.usa.gov/xNw9z)
- How to Dispose of Unused Medicines (go.usa.gov/xNw95)

For instance, Cardinal Health developed the Deterra Drug Deactivation System, a bag containing a carbon that bonds to pharmaceutical compounds and neutralizes the active ingredients when water is added. According to Hooshang Shanehsaz, RPh, DPH, director of pharmacy at Cardinal Health, the system neutralizes 98 percent of the medication using a form of activated charcoal, whereas cat litter or coffee grounds only absorb between 15 percent to 23 percent of the active ingredients. The bag is then disposed of in the trash without risk of entering the water supply or landfill. There are many other similar products on the market.

In addition, eco-friendly disposable bottles can be purchased from several different manufacturers that contain a ready-to-use chemical digestion solution. Medications are inserted into the bottle and the bottle is inverted twice to mix and wash the solution over them. The bottle can be used again and again until it is full and then disposed of in the trash.

**Eliminating the Unintended Consequences**

Too often, unused and unneeded medicines pile up in people’s homes, resulting in unintended consequences. To quell this, the government has established the national take-back program, as well as other laws, to enable individuals to safely dispose of or donate these medicines. If these avenues aren’t an option, following FDA guidelines for flushing or placing medicines in the trash, or purchasing some of the new technologies that destroy the medications’ active ingredients are good alternatives. It’s worth the effort, as these many options for disposing of medicines can help to save thousands of lives each year.

**RONALE TUCKER RHODES,** MS, is the editor of IG Living magazine.

**Sources**

The “Right to Try” Law: What Does It Mean for Patients?

By Jim Trageser

While this new law offers hope for terminally ill patients who have exhausted approved treatment options, the jury is out on whether it will help patients until more questions are answered.

ON MAY 30, 2018, President Trump signed into law SB 204, the “right to try” law allowing terminally ill patients the legal right to request access to experimental drugs not yet approved by the U.S. Food and Drug Administration (FDA). While much discourse has been made by activists on both sides of the political debate surrounding the new law, the few neutral analyses indicate the law is more of a symbolic victory for “right-to-try” advocates that will likely have relatively small practical ramifications for patients and their families.

The uncertainty of its value to patients is due to the fact that even though Congress approved the bill and the president signed it, until lawsuits are filed and the courts decide exactly what the law means, the legal landscape surrounding it will remain murky — leaving many pharmaceutical companies reluctant to take part.

Unfortunately, relatively little is written about the new law in the medical or legal literature. Most of the analysis is found in political outlets, both conservative and liberal, with both claiming to represent the true interests of patients and their families.

What the Law Says

The law, formally known as the “Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017,” removes certain federal regulations regarding unapproved drugs for terminal patients who have exhausted all existing approved options. It amends Chapter V of the Federal Food, Drug and Cosmetic Act that allows a patient who has been diagnosed with a terminal illness by a licensed physician to apply for access to unapproved drugs. The patient must sign a letter of consent, accepting the risks inherent to using a nonapproved drug, and only drugs that have passed Phase I clinical trials can be requested or provided.

The law specifically does not require pharmaceutical manufacturers to provide drugs to patients who request them. Further, while the law stipulates manufacturers and physicians are largely protected from being sued for providing or prescribing an experimental drug under the law, it does allow lawsuits for reckless and willful misconduct, but it does not define what constitutes either.

Another provision in the law says, except in narrowly defined cases, the outcome of a patient’s use of the drug will not be used by FDA when weighing approval of a drug once its clinical trials are completed. The law also directs the Department of Health and Human Services (HHS) to report to Congress each year on how many requests are made under the law, and it requires pharmaceutical companies to provide that information to the HHS.

Left unaddressed is whether any such drug requested and provided will be covered by a patient’s insurance carrier.
What Supporters Say

Supporters argue it is cruel and makes no sense to deny experimental drugs to terminally ill patients. They contend if doctors are allowed to prescribe drugs to let patients take their own lives in some states (known as medically assisted suicide), then doctors should be allowed to prescribe drugs that may save or prolong their lives.

Proponents also argue that FDA’s approval process can take years to complete (about 10 years, on average), and any drug that has passed Phase I clinical trials is known to not be immediately dangerous (i.e., poisonous). They also point out that 38 states already have right-to-try laws on the books, and they argue the federal law evens the playing field for patients living in the other 12 states.

What Opponents Say

Critics argue the law was a political stunt designed to generate positive headlines without doing anything to truly help patients. They point to an existing FDA regulation known as the expanded access rule (sometimes called compassionate use) that has already allowed almost 9,000 patients to receive experimental drugs that have passed Phase I approval. Opponents of the law also argue that allowing patients to take experimental drugs outside the normal testing protocols will hamper the ability of pharmaceutical companies to derive meaningful knowledge from controlled tests.

Critics such as Steven Joffe, MD, professor in medical ethics and health policy at the University of Pennsylvania’s Perelman School of Medicine, have expressed concern that the law may be a first step in deregulating the drug industry and exposing patients to dangerous, untested drugs.

What Lawyers Say

Almost a year after its passage, there is surprisingly little written on the right to try law in legal journals. In one article published in the American Society of Clinical Oncology, law professor Thaddeus M. Pope of the Mitchell Hamline School of Law in Saint Paul, Minn., interpreted the law as a somewhat more streamlined application process than FDA’s expanded access rule, but requiring less disclosure to the physician by the drug manufacturer. He argued this is a trade-off (less knowledge to guide their treatment of patients) that many physicians may find troubling.

In June, the Connecticut law firm Shipman & Goodwin LLP analyzed the law in a white paper that included bullet points of incentives and risks for drug companies. Incentives included generating good will and social capital, gaining additional data and increasing brand awareness for any new drug. But the law firm cautioned the law is vague on what defines “written informed consent” and “life-threatening disease or condition.”

Will Insurance Cover It?

The pro right-to-try website, righttotry.org, has a frequently asked questions section that states the law will work the same as FDA’s expanded access program for insurance coverage. In short, it’s up to insurance companies, including Medicaid and Medicare, to determine whether to cover the cost of experimental treatments.

But, this is also true when doctors prescribe a drug “off label” (to treat a disease for which the drug is not FDA approved). For a drug to receive approval for treating a new condition, it has to complete Phase II and III trials for that disease. Reviewing the roster of ongoing trials at clinicaltrials.gov, many drugs already on the market are back in additional trials to see if they are effective at treating other, often related conditions or diseases.

Supporters argue it is cruel and makes no sense to deny experimental drugs to terminally ill patients.

Still, any drug already on the market would not be governed either by FDA’s existing expanded access policy or the right to try law, even if it is undergoing additional clinical testing. A physician can prescribe a drug legally even though an insurance company may decline to cover it. (One caveat to this is federal law requires Medicare to cover off-label cancer prescriptions if there is evidence to support such use.)

How the FDA Approval Process Works

When a pharmaceutical company or academic research institution discovers a new substance or develops a new compound
that it believes can treat a disease or other condition, it files an investigation new drug (IND) application with FDA for permission to conduct human tests. With the IND, researchers must submit results of any lab tests they’ve conducted, often introducing the new compound into test tissue with the virus, bacteria or cancer they’re looking to treat, or in some cases testing it on animals. If the new compound appears safe and shows promise for treating or curing a disease, it will be approved for clinical testing, which has four phases. Phase I, as mentioned, is designed to study the drug’s safety to determine whether it will cause immediate side effects when given to human patients. FDA says about 70 percent of prospective drugs pass Phase I, which normally involves two dozen to as many as 100 volunteers, and lasts a few months.

Phase II of clinical testing generally takes a few months to a couple of years to complete. This is where the rubber starts to meet the road. The purpose is to determine whether the drug actually treats or cures the disease in question, and if so, in a safe manner. Depending on the disease or condition targeted, the manufacturer or university conducting the research will recruit as many as a couple of hundred volunteers who have the disease or condition. The research typically divides research subjects into two groups with some receiving the new drug and others a placebo. Patients have their condition monitored over a course of months or even years, and are observed for any side effects. Roughly one-third of drugs submitted for Phase II testing pass. (This means two-thirds of drugs available under the right to try law, as well as FDA’s expanded access rule, will eventually fail to do what they were intended to do, or will pose so much adverse risk that they are deemed unsafe.)

Phase III is a continuation of Phase II. The volunteer pool is expanded to up to several thousand test subjects. The drug continues to be studied to see if it is effective in treating a disease or condition. Phase III can last as long as four years, and during this period, researchers watch for any long-term adverse reactions that may not have manifested during the earlier, shorter phases. Only about 25 percent to 30 percent of drugs submitted for study pass Phase III. (Thus, of all potential drugs submitted for FDA clinical trials, only about 7 percent will ever make it to market.)

Phase IV is an ongoing study, again looking to ensure the new drug is safe over the course of years. Phase IV trials can continue even as the drug is submitted to FDA for permission to market and sell, and even after approval.

Once Phase III clinical trials are successfully completed, the manufacturer then submits a new drug application to FDA. FDA then has up to 10 months to review all study results and any other documentation to decide whether the drug can be sold. (Drug companies often express frustration about the length of time it can take to complete preclinical testing and the three required phases of FDA clinical tests before gaining approval on new drugs. This is because a patent for a new drug lasts only 20 years, and it usually takes eight to 10 years from developing a new drug to getting it on the market. Once the patent expires, competitors are free to sell generic versions.)

Even after a drug is approved for marketing and sale, FDA continues to monitor its safety.

**What It All Means**

For patients whose prognosis is grim, who have exhausted all existing treatments and for whom only a drug not yet available through normal channels offers any hope, the right to try law may offer a last chance at a cure and a shorter process to gain access to experimental drugs than FDA’s expanded access program. But, with many questions about liability, cost recovery and use of data generated by out-of-trial use unanswered, it is unclear how many pharmaceutical companies will be willing to honor requests for experimental drugs — no matter how urgent the plea. Until more data is available about how many patients and their physicians request new drugs, and whether those requests are honored, it will be impossible to know whether the law is accomplishing the goals of its supporters.

**JIM TRAGESER** is a freelance journalist in the San Diego area.

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**References**


ONCE KNOWN AS idiopathic thrombocytopenic purpura (ITP), the name for this rare condition has evolved over time as understanding about its mechanisms has advanced. ITP was first described almost a thousand years. But, it wasn’t until 1915 that controversy arose regarding the mechanism of ITP. It was then that German physician Ernest Frank suggested ITP was caused by suppression of megakaryocytes (cells present in bone marrow) by a substance produced in the spleen, whereas Czech scientist Paul Kaznelson suggested it was caused due to increased destruction of platelets in the spleen. When, in 1916, Kaznelson performed a splenectomy on a patient with chronic ITP resulting in the patient’s platelet count increasing and the purpura resolving, splenectomy became the prevailing treatment for ITP for years.

Then, in 1950, the Harrington-Hollingsworth experiment determined the cause of ITP was a factor in blood rather than bone marrow that destroyed platelets. Harrington and other colleagues self-infused the blood of an ITP patient, which caused their platelet count to plummet, a major seizure and bruising and petichiae. When examining the bone marrow, no effect of megakaryocytes was discovered, which suggested an effect of the platelets rather than the marrow. These results, along with other reports published in 1951, led to a name change from idiopathic thrombocytopenic purpura (meaning the cause is unknown) to immune thrombocytopenic purpura, since the role of the immune system was then recognized as the cause of the disease.1

In the U.S., the incidence (number of people diagnosed each year) of ITP is estimated to be 3.3 per 100,000 adults per year. The prevalence (how many people have ITP at any time) is 9.5 cases per 100,000 adults and 5.3 cases per 100,000 children (since children with ITP usually recover, the number of children who have ITP at one time is about equal to those diagnosed annually). The incidence of ITP increases with age. Among adults age 30 years to 60 years diagnosed with ITP, there are 2.6 cases among women for every male case. In older adults, about the same number of men and women are diagnosed with ITP. And, among children diagnosed with ITP, the male to female ratio is almost equal: 52 percent and 48 percent, respectively. About 40 percent of all patients diagnosed with ITP are children younger than 10

Because much more is known about what causes this rare disease today, new treatments continue to evolve.

By Ronale Tucker Rhodes, MS

Managing and Treating ITP

Because much more is known about what causes this rare disease today, new treatments continue to evolve.

By Ronale Tucker Rhodes, MS
years of age. And, among children, the incidence is greatest between ages 2 years and 4 years old. ITP is also more common in white than in black children, and its severity and duration may differ among geographic areas.

**What Is ITP?**

ITP is an autoimmune bleeding disorder characterized by abnormally low levels of blood cells called platelets that maintain the integrity of blood vessel walls and help prevent and stop bleeding by accelerating clotting. ITP occurs when platelets are attacked and prematurely removed by the body’s immune system (with autoimmunity, the body’s immune system mistakenly attacks a part of a person’s own body). A normal platelet count ranges from approximately 150,000 to 400,000 per microliter of blood depending on the laboratory. If a person has a platelet count lower than 100,000 per microliter of blood with no other reason for low platelets, then he or she has thrombocytopenia and may have ITP.

There are three types of ITP: acute (present for less than one year), chronic (present for more than one year) and recurrent (episodes at intervals of more than three months). Eighty percent of children with ITP have the acute form that resolves six months after diagnosis independent of treatment, and the younger the child, the less risk of developing chronic ITP. In children with chronic ITP, complete remission is gained in 90 percent within three years to seven years. Recurrent ITP occurs in between 1 percent and 4 percent of children. More than 50 percent of adults have chronic ITP.

ITP can be further broken down into either primary or secondary. If ITP develops for no known reason, it is considered primary. On the other hand, if ITP is associated with illnesses such as an infection or other autoimmune disease, or if it occurs after a transfusion or after taking certain drugs (such as cancer drugs), it is considered secondary.

**Causes of ITP**

While it is unclear what causes ITP, it is believed multiple factors are involved, including environmental (infections or drugs), self-marker molecules and heredity. For both primary and secondary ITP, it is known that the body’s natural immune defenses inappropriately act against its own cells or tissues, which causes the destruction of the body’s platelets. However, in some cases, platelet production by megakaryocytes in the bone marrow is impaired. So, the causes of ITP can be due to increased platelet destruction, reduced or inadequate platelet production, or both.

In primary ITP, it is unknown what is causing this autoimmune reaction. But, with secondary ITP, the cause is due to another condition. These include inherited immune disorders (such as autoimmune lymphoproliferative syndrome), systemic autoimmunity (such as systemic lupus, ongoing infections such as HIV, hepatitis C and Helicobacter pylori) and lymphoproliferative disorders (such as chronic lymphocytic leukemia). In addition, some cases resembling ITP can result from the use of certain drugs, after a viral or bacterial infection or after vaccination for measles, mumps and rubella.

When ITP is caused by an infection, it is believed the infection generates antibodies that cross-react with platelet antigens or immune complexes that bind to platelet receptors, thereby impairing platelet production due to infected megakaryocyte bone marrow-dependent progenitor cells, decreased production of thrombopoietin (TPO), and splenic sequestration of platelets secondary to portal hypertension (HCV). For instance, sudden and severe onset of thrombocytopenia has been observed in children after vaccination for measles, mumps and rubella, or natural viral infections, including Epstein-Barr virus, cytomegalovirus and varicella zoster virus.

Familial ITP (when more than one family member is affected) is rare, and its inheritance remains unclear.

**Symptoms of ITP**

In some cases, people with ITP have no signs or symptoms. However, when symptoms do occur, they can vary greatly from person to person. In general, the lower the platelet count, the more symptoms, including:

- Easy or excessive bruising (purpura)
- Petechiae (tiny red dots on the skin caused by broken blood vessels or leaks in a capillary wall)
- Bleeding from the nose, mouth and gums, and digestive and urinary tracts
• Blood in urine or stools
• Unusually heavy menstrual flow
• Feeling tired or fatigued

Rarely, bleeding within the brain occurs, but it can be life-threatening if it does. According to a study conducted in 2001 of 2,031 children, life-threatening bleeding occurs in 0.2 percent within the first 12 months, and the risk can be greater during the initial phase of ITP and if the platelet count is below 10,000 x 10^6 per liter, but it can occur at any time in ongoing ITP.

**Diagnosing ITP**

It’s first necessary to exclude other possible causes of bleeding and a low platelet count when diagnosing ITP. Then, in addition to looking at the medical history and performing a physical exam, the following tests can be performed. A complete blood count can determine the number of blood cells, including platelets, in a sample of blood. With ITP, the white and red blood cell counts are usually normal but the platelet count is low. A blood smear, in which a sample of blood is placed on a slide and observed under a microscope, can be used to confirm the number of platelets observed in a complete blood count. And, a bone marrow exam (the American Society of Hematology doesn’t recommend this test for children) can verify there are adequate platelet-forming cells (megakaryocytes) in the marrow to rule out other diseases such as metastatic cancer and leukemia.

In some cases, a bone marrow biopsy (in which a sample of bone tissue and the enclosed marrow is removed) and/or a bone marrow aspiration (in which the liquid portion of the marrow is removed) are performed. These can determine if the bone marrow is normal, which will signal the low platelet count is caused by the destruction of platelets in the bloodstream and spleen, rather than due to a problem with the bone marrow.

**Treating ITP**

While there is no cure for ITP, treatment is determined by the severity of symptoms. The key is to find a treatment that works without unwanted side effects.

In some cases, treatment is not needed. Most children with acute ITP do not require treatment because their condition resolves spontaneously. The American Society of Hematology (ASH) recommends children who have no bleeding or mild bleeding be managed with observation alone regardless of platelet count. This recommendation is based on a study that found observation alone did not lead to an increase in later treatment or an increase in delayed bleeding symptoms. For pediatric patients requiring treatment, ASH recommends a single dose of 0.8 g/kg to 1.0 g/kg of intravenous immune globulin (IVIG) if a more rapid increase in the platelet count is desired, or a short course of corticosteroids, as first-line treatment.

While it is unclear what causes ITP, it is believed multiple factors are involved, including environmental (infections or drugs), self-marker molecules and heredity.

In one newly completed study, mini-pools of IVIG from as few as 20 donations have been found just as significant in treating pediatric ITP as standard high IVIG doses (see Study Shows Mini-Pools of IVIG as Effective as Standard IVIG to Treat Pediatric ITP, p.14). With the high cost of IVIG, the study suggests this first-line treatment can be much more cost-effective for patients and payers.

Because of the significant risk of hemolysis (destruction of red blood cells) with IV Rho(D) immune globulin (RhIG, anti-D immune), ASH advises against its use in children with a hemoglobin (protein found in red blood cells) concentration that is decreased because of bleeding, or in those with evidence of autoimmune hemolysis. However, a single dose of IV RhIG can be used as first-line treatment in Rh-positive, nonsplenectomized children with a negative direct antiglobulin test who require treatment.

Children who have significant ongoing bleeding despite treatment with IVIG, RhIG or conventional doses of corticosteroids can be treated with rituximab or high-dose dexamethasone as second-line treatment. Rituximab or high-dose dexamethasone may also be considered as an alternative to splenectomy or as treatment for children who do not
respond favorably to splenectomy. It should be noted that splenectomy as a therapeutic option in children is restricted to those with uncontrollable bleeding.3

Recommended treatment for adults is similar to that for children by using drugs that alter the immune system’s attack on platelets. First-line treatment initially consists of high-dose corticosteroids with a goal of impairing the production of antiplatelet antibodies so platelet count will remain elevated after ceasing treatment. If corticosteroids fail to improve platelet levels or if severe bleeding persists, patients are treated with monthly IVIG infusions. IV RhIG can also be administered in patients who are Rh positive and who have not received a splenectomy.2

Second-line therapy includes rituximab (anti-CD antibody), which reduces IgG antibody production; splenectomy, which improves platelet count in approximately 70 percent of cases and can achieve remission in 50 percent to 60 percent of patients; immuran (azathioprine); Cytoxan (cyclophosphamide); Sandimmune (cyclosporine); Danocrine (danazol); Cellcept (mycophenolate mofetil); and Vincristine (vinca alkaloids).2

Lifestyle adjustments may also be required for managing ITP. For instance, those whose platelet counts are less than 50,000 are recommended to wear protective gear such as helmets and to avoid contact sports such as boxing or football that carry a high risk of injury. Alcohol should also be consumed in moderation because it slows production of platelets. Over-the-counter medications such as aspirin and ibuprofen should be avoided as they can impair platelet function. And, those who have had their spleen removed need to watch for signs of infection, including fever, and seek immediate medical attention.7

Looking Ahead

Efforts to understand more about ITP are ongoing. As of this writing, there were 234 studies listed on ClinicalTrials.gov. Following is a brief look at some of these:

• Researchers are studying whether intravenous corticosteroids and IVIG administered together will increase platelet count faster, minimize adverse effects of IVIG and lead to a more sustained increase in platelet count. If it is shown the combined therapy results in a quicker rise in platelet count, this would support and justify the use of the combination therapy in emergency situations, which is often used today when children present with a life-threatening bleed.12

• Another study is researching whether eltrombopag can be used instead of IVIG in patients with ITP to adequately raise their platelet count when undergoing minor or major surgery. The randomized controlled trial will involve 74 adult patients in Canada. In addition to evaluating the efficacy and safety of eltrombopag bridging therapy compared with IVIG bridging therapy, the study will evaluate bleeding, adverse events and patient-reported treatment satisfaction.13

• Eltrombopag is also being studied to determine the Fc
gammaR IIIA gene (V158F) genetic predisposition with treatment outcome of the drug in ITP refractory patients. Patients will be assessed by collection of blood samples from 50 controls (treated with standard immunosuppressive first- and second-line treatments) and 25 steroid-refractory patients at the time of enrollment to the trial and then subsequently at three months and six months after treatment.

- A two-phase study known as the PROLONG Trial will evaluate if low-dose rituximab maintenance therapy may prolong the effect of the drug in ITP. In the first phase, patients will be randomized into a rituximab-only or rituximab-plus-dexamethasone group to determine if the response to rituximab can be improved by the addition of dexamethasone. In the second phase, patients will be randomized into a low-dose rituximab or placebo group to determine if the response achieved in the first phase can be prolonged by administering maintenance treatment with low-dose rituximab.15

- And, an observational registry study will document serious adverse events in ITP patients treated off-label with rituximab.16

Because ITP is a rare disorder, information about patients with ITP is being collected in many registries with the help of medical centers. One of these is the ITP Natural History Study Registry, an international patient-consented registry that aims to collect, store and retrieve data on the natural progression of ITP, enabling collection of data on diagnosis and treatment, management of care, quality of life, clinician reporting and characterization of the ITP population as a whole. It is hoped these registries will provide information that can lead to a greater understanding of the disease and help to design future studies.17

ITP’s Impact on Patients

ITP is a rare autoimmune disorder that leads to easy or excessive bruising and bleeding caused by increased platelet destruction, reduced or inadequate platelet production, or both. In some cases, it is unknown why this occurs, yet in others, it can be linked to another condition. Symptoms vary among those affected, with some requiring no treatment and others requiring extensive treatment. While much has been learned about the disease since its first description more than 1,000 years ago, research is ongoing to find additional more effective treatments.

Despite advances in our knowledge and treatments, however, ITP has a significant impact on patients’ quality of life. In June, Novartis, which markets Promacta, released results of its survey titled I-WISh (ITP World Impact Survey). Findings from more than 1,300 ITP patients across 13 countries showed the disease had especially high impact for many patients on emotional well-being (36 percent) and ability to work (28 percent). About two-thirds of patients reported fatigue as the most severe symptom at diagnosis (71 percent) and survey completion (64 percent). Overall, two main treatment goals reported by patients were achieving healthy blood counts (79 percent) and increasing their energy levels (55 percent).

“These initial data from the I-WISh survey reveal how a rare blood disease like ITP can significantly affect a patient’s ability to live and function in their day-to-day life,” said Samit Hirawat, MD, head of Novartis oncology global drug development. “We believe these results demonstrate that, even beyond medicine, ITP patients are seeking compassion, support and understanding from family, friends so they can strive to live the best lives they can. These are important insights, and we will look to build them into the programs and services we develop to better support this community.”18

RONALE TUCKER RHODES is the editor of IG Living magazine.

References

Profile: Autumn Bousquette

By Trudie Mitschang

As a busy college student and foster mom, Autumn Bousquette was accomplished at juggling activities. But her life took a sharp detour when an unexpected health crisis led to a diagnosis of common variable immune deficiency (CVID). Autumn knew she could either wallow in self-pity or totally throw herself into living a full life again. After seeking out other CVID patients online, Autumn not only discovered a new community of friends, she also met the man who would become her husband and soul mate.

Trudie: Tell us about your life before CVID.

Autumn: I have always been in good shape. I was a gymnast for 13 years, a cheerleader and I ran track. I also worked on the assembly line at Ford Motor Company in Michigan. When the economy crashed, I took a buyout from Ford and, during that time, I worked out daily and walked. I eventually moved to Arizona to attend college at Arizona State University, and I became a foster mom to three kids under 5 years old.

Trudie: When did your symptoms start?

Autumn: While living in Arizona, I began to get sick more frequently. I was suffering bouts of bronchitis, but nothing too severe. I was given an inhaler, and my doctors were concerned because my sodium level was extremely low, putting me at risk of a seizure. During that time, I was frequently hospitalized, where I was given sodium IVs. Even with all of those challenges, I never worried about anything being seriously wrong. Countless times after my blood was taken by the nurse, the doctor would walk in and joke: “Sorry, you must be the wrong patient; you don’t look this sick!” Life was busy, and I have always been a fighter, so I just kept trucking along.

Trudie: What happened the day you collapsed on the stairs?

Autumn: The day I collapsed was a typical morning. I remember feeling grateful I was still under contract with Ford and had insurance until college graduation. After a normal morning at school, I made my way back home to my upstairs apartment. It was a hot day, but it was typical for Arizona. When I climbed up the stairs, I suddenly started to have breathing issues. Within four to five steps, I could not go any farther. I sat on the stair and called my friend who drove me to the hospital. After checking my vitals, they immediately checked me into a room, and a team of doctors and nurses came running in. I was terrified. The next thing I remember was waking up strapped to a bed in the ICU with a breathing tube down my throat. During my time there, they ran a lot of tests. It was brutal.

Trudie: What were you tested for?

Autumn: They were trying to figure out if I had pulmonary fibrosis since it runs in my family. My uncle, and we assume my mother, had pulmonary fibrosis and possibly CVID.

Trudie: What was your first diagnosis?

Autumn: When I was finally discharged from the hospital, I went home to Michigan with a misdiagnosis of pulmonary fibrosis. I saw a couple of pulmonary doctors, but I was not very happy with any of them. I was not willing to give up, so I found a new doctor.

Trudie: When were you finally
Autumn: On a routine visit to the pulmonologist, one of my doctors sent me to an allergist and immunologist for additional testing, and written on the referral were the letters “CVID.” That was the first time I had heard of common variable immune deficiency. Although I didn’t understand it, that finally led to a diagnosis.

Trudie: What is your treatment plan?

Autumn: Immediately after my diagnosis, I was prescribed home infusions of subcutaneous immune globulin (Hizentra). I educated myself with every bit of knowledge I could gather, and I still learn more daily. These days, I infuse Hizentra monthly at home while watching Ellen DeGeneres.

Trudie: How did you meet your husband?

Autumn: It worked to my advantage that CVID did not make me look sick, so I joined an online dating site and a Facebook group. I was asked out quite a bit, but once my dates found out about my health conditions, they ran. Ironically, Dave, who is now my husband, asked me out constantly, but he was a single dad with two teenage daughters, and that scared me. Thankfully, I finally said yes, and we hit it off within minutes. We talked for six hours before he went home. I don’t think he truly understood how sick I was at the time, but of course, he soon found out. My husband is an incredible man. I don’t know how I got so lucky. These days, we have date nights, lazy nights, vacations and so much more. We are avid gardeners. We go to a ton of live concerts/shows. Life is great! He is my best friend.

Trudie: What advice do you have for other patients?

Autumn: Do your homework. Ask questions and read everything you can to educate yourself. You are your own advocate. Listen to your body and stay zebra strong!

Trudie: Besides helping you meet your husband, how has social media helped you cope with CVID?

Autumn: I had always been active on social media, and I was so thankful that I typed in CVID in the search bar of Facebook. That led me to connect with several support groups for our rare community, and it was a lifesaver. I have always wanted to help others, and social media became an outlet to encourage others and let them know they are not alone.

Trudie: What has CVID taught you about yourself?

Autumn: This journey has taught me that life is short. It really is important to embrace every moment like it is your last. You never know how fast it can be cut short. It has reminded me to tell everyone how much they truly mean to me and to endeavor to live life to the fullest. In many ways, this diagnosis also taught me how strong I really am. For that, I am grateful.

Trudie: What are your goals for the future?

Autumn: I hope to go back to work one day, and I hope to outlive my mom who died at age 47. I am 44 now, and there’s still so much I’d like to accomplish. I could write a book on my hopes and dreams, but it would never have an ending.

TRUDIE MITSCANG is a contributing writer for IG Living magazine.

This journey has taught me that life is short. It really is important to embrace every moment like it is your last.
PATIENT PERSPECTIVE

Defined by Illness?
By Stacey Philpot

HOW MUCH of our identity is defined by our illnesses? Who or what has the power to decide this? Have you ever felt like illness with all of its far-reaching tentacles was taking over your life, bit by bit, until even your identity was consumed by illness? Instead of defining yourself by your strength, tenacity, bravery, humor, intelligence or capability, you began to define yourself by your fight or struggle? Perhaps when you looked in the mirror, all you saw were shortcomings. You felt certain others saw these shortcomings when they looked at you as well. They too, wished you were stronger, more capable, the person you once were.

Last year, my husband and I moved from sunny Florida to the Washington, D.C., area. It’s been quite a transition. Not only did I have to adjust to snow and actual seasons, but I also had to acclimate to a faster pace of life surrounded by goal-focused, high-achieving people. I quickly tired of the baby-voiced apologies issued upon explaining my physical limitations and conditions to new friends, so I stopped. Therefore, I frequently felt like an imposter living among them. While they went to the gym, took on the promotion at work and volunteered for the kid’s party at school, I tried to not set off flares or end up at the hospital.

In the beginning, I found myself not wanting these new, high-achieving people to know I was somehow flawed. Next to them, my life felt so small. My 30s had been consumed by illness. The things these people had already accomplished took my breath away at times. When I compared myself, my accomplishments and my body to them, I sometimes felt the urge to cry. Would I have been like them if illness had not wrapped its tentacles around me? Would I have gone to work in the Pentagon each day or spoken on Capitol Hill? Would I at least have had the energy to put makeup on and leave the house each day?

One day as I stood in the shower overanalyzing a simple conversation with a fellow mom, I realized the fatal error I was making by comparing myself to these people. It’s true the last 10 years of my life have been consumed by illness. In March, I turned 40 years old. I have no great career accomplishments to mark. Yet, I look back and celebrate who I have become in these years and all that my illness has taught me. I choose to celebrate growth and gratitude. I will mark with thankfulness each day I have walked on this earth.

This year, I embarked on a master’s degree program in counseling because these last 10 years have taught me how important it is to cheer one another along on the journey. The high-achievers in my community have reminded me how important it is to never stop learning and growing. Illness is a part of our story, but it is not the whole story. There are exciting pages yet to be written. May we write them well, bringing what only we can bring to the world.

The truth is, everyone is fighting something. For some of the high-achievers in my area, their struggle is with sexism, racism or ableism. Perhaps some of them have an illness I can’t see. There is no shame in our struggle. Nor does our struggle define us. Instead, we are defined by what we do with the struggle. How do we respond to the things in our lives over which we have no control? Do we allow them to help us grow? To make us better?

Stacey Philpot is an author, goofball and avid reader. You can find her blog at chronicallywhole.com, where she shares her journey of making the most of a life touched by common variable immunodeficiency, Lyme disease and rheumatoid arthritis.
The Cost of Chronic Illness: Tips to Save and Receive

By Ilana Jacqueline

MAMA SAID there’d be days like this, but she didn’t mention the credit card bills that would show up on those days!

Recently, I was speaking with a fellow friend and patient about a trip home she was taking for the holidays. She had done everything in her power to prepare for what she knew would be a day of painful traveling. As her joints slipped out of place and the stress on her body became unbearable, she asked a flight attendant if she could lie down in a row of empty seats. The flight attendant refused. If she wanted to lie down, she’d have to pay for all three seats. She quietly sat back down and prayed her body would hold up until the flight was over.

We often talk about the emotional, physical and social aspects of living with a chronic illness. But, does anyone ever talk about the expense? I’m not just talking about medical bills. We all have them, and we let the debt collectors know they can threaten us all they want, but that won’t magically make our bank accounts any bigger. I’m talking about the other costs of living with chronic illness: the ever-changing (yet always more expensive than fast food) diets, the alternative therapies like massages, salt rooms and gentle exercise classes. My nightly routine involves using four different massage devices (each costing between $50 and $200), a TENS unit, medical marijuana (not covered by insurance), compression socks, gloves and a Tempur-Pedic wedge pillow and mattress cover.

Sometimes I feel like my doctors may as well make out my prescriptions to “The Princess and the Pea.” When the credit card balances start rising and I need to get a handle on my finances, I try to keep in mind some of these tips for managing finances and activities:

**Cut unnecessary costs.** Sometimes those $15 entertainment subscriptions can add up and the money could go toward more essential bills. Take a look through your bank statements and see how much you’re spending on things like Netflix, Hulu, Amazon and excess beauty subscriptions like make-up boxes.

**Pay the minimum.** Some months will be more financially defeating than others. It’s OK to just pay the minimum on your credit and debit cards as long as you don’t fall too far behind on your payments. You can also call the bank and let them know you’re having a medical crisis to see if they can pause interest or delay payment for the month. The same goes for your car insurance, car payment and ongoing medical debt collections.

**Ask for a discount.** In some cases, doctors, therapists and other practitioners will offer their services on a sliding scale based on your income. This can drastically reduce co-pays and appointment costs. Pharmacies have similar assistance, and if a medication is too expensive, you can ask them to use a co-pay discount card on your prescription. Some prescriptions will be ineligible, but I always ask!

**Create an Amazon Wish List.** I’m not a child or a beggar, but when I’m in bad shape (a long hospital stay, an unexpected surgery, etc.) and I need a few items not covered by insurance that can help keep me functional, I will share my private Amazon Wish List with those who ask how they can help. I always appreciate when people send flowers in the hospital, but sending something practical (a couple of boxes of tissues, replacement pads for my TENS unit, post-surgical care items) can be a more practical option.

**Use sites like Care Calendar.** Care Calendar is a site that allows friends and family to volunteer to drive you to doctor appointments (saving on gas or Ubers), bring you meals when you can’t cook or get to the store, help run errands or take care of animals or children (saving on dogwalkers or babysitters). Remember, the people around you want to help; they often just don’t know how. Care Calendar is a great way of truly asking for what you need and allowing others to help in a way that’s convenient for their schedule.

The most important rule of thumb, though, is to remember financial insecurity is an issue for most patients no matter how hard they work or save. There is no shame in asking for help when you’re struggling financially, physically or emotionally.

ILANA JACQUELINE is a 29-year-old dysautonomia and primary immune deficiency disease patient from South Florida. She’s been writing professionally since 2004 on everything from health and wellness to celebrities and beauty. Her blog www.letsfeelbetter.com is both a personal collection of anecdotes about life with chronic illness, as well as a resource for patients of all ages.
The Benefits of Summer Camp for Chronically Ill Kids

By Jessica Leigh Johnson

WITH SUMMER right around the corner, many parents are wondering how to fill their children’s days with meaningful activities that keep them active and “unplugged” — engaged in life outside, not just life as it appears on a screen. One way to ensure kids have fun new experiences, take part in physical activities and socially interact is to send them to summer camp. It’s a time that is sure to be a highlight of their summer.

Unfortunately, summer camp can be a scary prospect for parents of children with a chronic illness, so sending them to a sleep-away camp for a week may not be the best option. Luckily, no two summer camps are the same, and they don’t all involve staying overnight. There are daytime-only camps and countless other options available to children with any number of health conditions. My own three boys suffer from a primary immunodeficiency, and they’ve each attended summer camp every year since the summer after second grade. What’s more, they’ve never had a bad experience; it’s something they look forward to every year.

Before looking at the different types of camps, parents should first know the many benefits of sending their children to summer camp, even if they suffer from a chronic condition.

Benefits of Summer Camps

Camps build leadership skills. Even if children can attend only a one-day camp, parents can be sure they will take part in several team-building exercises. It’s one of the main focuses of any camp’s curriculum. Whether it’s a ropes course, climbing wall or crate-stacking challenge, camp counselors and leaders get kids involved in working together toward a common goal. These types of team-building exercises promote leadership ability, assertiveness and self-confidence, and help children learn to work with others. All the while, no child is left out of the group, and everyone plays a part.

Camps promote social interaction. One of the best parts of summer camp is the opportunity for children to meet kids from other towns who don’t attend the same school. When they arrive at their cabins, children may be introduced to a room full of unfamiliar faces, but they’ll spend the rest of the time getting to know these boys and girls with whom they will form lasting friendships. Making new friends forces kids to step out of their comfort zone while building valuable social skills.

Camp activities build self-esteem. When children are engaged with their new friends and facing new challenges, positive self-esteem develops. Each exercise or group activity is designed to encourage creative thinking and problem solving, which helps kids realize they have valuable ideas and opinions and can contribute to a group in a meaningful way.

And, the time spent away
from home gives children confidence they can be away from their parents and familiar surroundings and still have fun.

Camps build new skills and hobbies. The activities in which kids participate help to develop life skills such as teamwork, responsibility, showing respect, living with integrity and critical thinking. Because of the wide variety of activities available, kids will pick up new hobbies and fine-tune their existing skills and abilities. During free time, children are encouraged to take part in optional activities such as arts and crafts, music, sports, swimming, canoeing, hiking and many other activities. Even if none of these things turn out to be a life-long passion, the exposure to a wide variety of activities helps children add to or improve their skills and makes them more well-rounded individuals.

Camps provide physical activity. One of the biggest challenges for parents today is getting their children outside and away from the screen. Many children spend far too much time indoors, especially during the school year, when kids spend six hours a day sitting at desks, trying to be quiet and still. But all that can change when summer comes. Whether it’s a sports camp, acting camp, Bible camp or boot camp, most promote physical activity. With a multitude of outdoor activities such as paintball, laser tag, challenge courses, sports and water-related activities, children will be worn out by the time they go to bed, and parents will gain the peace of mind knowing their children are learning how fun it can be to lead an active and healthy lifestyle.

What Type of Camp Is Best?

With so many camps and programs available, there really is a camp to fit the needs of all children. Depending on their health, strengths, interests and maturity level, it’s up to parents to decide which camp is right for their children. But with so many options, the choice can be overwhelming.

If the children’s illness can be easily managed with medications given by a camp nurse, an overnight weeklong camp can be a very enriching experience. Kids who take monthly intravenous or weekly subcutaneous immune globulin infusions should have no problem fitting a week of camp in between treatments, making it easier to focus on having fun, rather than on their condition. Any unexpected illnesses or injuries can be treated by the staff nurse, and if need be, parents can be contacted to pick the children up. Hopefully, with fewer illnesses circulating in the summer, this won’t be a concern!

For parents who’d like to provide their children with the excitement and rewards of camp, but don’t think their children are ready for an overnight camp, day camp is always an option. Many day camps last a week or more and take place in the children’s hometown, so parents can drop them off in the morning and pick them up at suppertime. Keeping the children’s needs and interests in mind, there are many types of day camps to choose from.

Many day and overnight camps, including most faith-based camps, are general in their offerings, with a range of different, mostly outdoor, activities. Specialty camps, however, focus on a particular activity or skill such as certain sports (basketball, volleyball, baseball, horseback riding, etc.), robotics or engineering, arts and dance, theater, foreign language, or wilderness and adventure. Other specialty camps include those geared toward kids with special needs, including certain health or mental conditions, enabling them to experience the joys and excitement of camp like healthy children.

For parents who don’t feel comfortable having their children away for a week or even a weekend, many camp organizations offer family camps. At these, children and parents stay in cabins or lodges together for several days and take part in large family-building activities involving both children and adults, as well as breakout activities that are kids-only (while parents get a little break and a chance for adult conversation).

The Opportunities Are Endless

When it comes to summer camp, the opportunities are endless. Parents need only to decide what activities or experiences their children would enjoy most and what limitations, if any, they may have because of health issues. Then, they can choose a camp that best fits their needs. To get started, visit www.summercamps.com for a list of camps across the nation based on location and activities of choice.

JESSICA LEIGH JOHNSON is a stay-at-home mom and mother of four kids, three of whom have X-linked agammaglobulinemia. She is a member of American Christian Fiction Writers and has written one book about the loss of her son to a primary immunodeficiency.

References
Living in an AI World

By Heather Bremner Claverie

FOR MANY, the words artificial intelligence (AI) may conjure up images of a rogue world-eviscerating robot or HAL, the murderous computer from “2001: A Space Odyssey.” But, the reality is much less sinister. AI isn’t just about asking Google to turn on the lights; it has turned the medical world upside down — in a good way.

What Is AI?
The introduction of AI into healthcare has revolutionized the industry. Defined as “a device or product that can imitate intelligent behavior or mimics human learning and reasoning” by the U.S. Food and Drug Administration (FDA), these AI-enabled computers, instruments and networks diagnose and detect disease, assist with surgery and use specialized algorithms to assess and process patient samples.

One sector of AI that is rapidly growing is so-called machine learning, according to FDA. This smart technology can develop a procedure without specific programming by using automated data or adaptive algorithms. The machines can then continue to adapt as they essentially learn more. It does sound quite futuristic and maybe a little closer to that rogue robot scenario, but this technology is a lifesaver. Some examples include an imaging system that uses algorithms to detect skin cancer in patients or an electrocardiogram device that assesses the probability of a patient going into cardiac arrest.

Automating Healthcare
It wasn’t long ago that repetitive stress injuries were just a normal byproduct of life in a laboratory. Repetitive tasks such as pipetting can cause a host of disorders affecting nerves, tendons and muscles. In addition, since lab technicians usually sit or stand in awkward positions while completing their work, they often suffer from back and neck pain. Due to these repetitive tasks, the most common condition among lab technicians is carpal tunnel syndrome, a nerve disorder in the wrists that causes pain, numbness and tingling in the hands and fingers.

Carpal tunnel syndrome is not only painful, it’s costly. As the most expensive musculoskeletal disorder in existence, employers pay more than $28,000 per employee in carpal tunnel syndrome-related costs, according to the Occupational Safety and Health Administration. In addition to the costs, employers suffer loss of productivity and indirect costs involved with hiring and training new employees.

Enter automation. Mechanizing or automating mundane tasks in the healthcare industry is helping to lessen these workplace injuries. Many labs, hospitals and physicians are turning to AI to lower costs, decrease turnaround time and lessen the likelihood of human error. In addition, increased workloads, labor shortages and the impending retirement boom of medical technologists is compelling labs to look to AI to process an influx of lab samples.

A Shifting Landscape
AI in the healthcare industry has evolved drastically in the past decade. With the sudden influx of digital technology, FDA started regulating these devices under the Digital Health Technology program. Any software “intended for one or more medical uses that may run on different operating systems or in virtual environments” falls under the regulation’s umbrella.

And, many of the fears associated with AI have dissolved as patients, physicians and technicians have witnessed its benefits. In the lab, automation frees up technicians, allowing them to focus on more complex work instead of repetitive tasks. In the workplace, smart technology helps physicians anticipate patient needs, spend more time with patients and less time processing paperwork, and prioritize patient care. Essentially, they can all put their brains to better use.

But, don’t worry: AI can’t replace the brain. AI is an asset to human intelligence and has the potential to continue to drastically improve patient care on all levels, bringing more efficient, higher-quality and accessible healthcare to all.

HEATHER BREMNER CLAVERIE is a contributing writer for IG Living magazine.
It’s not a crystal ball, but Medial EarlySign’s algorithms can help foretell the future. Its three founders started the company nearly a decade ago with the idea of using AI technology to detect early warning signals and health risks. Today, those algorithms can predict the likelihood of a patient developing an illness within 12 months or less. Armed with this information, physicians can intervene earlier and potentially delay or prevent an illness from progressing.

earlysign.com

When a woman was hit by a car and the surgery went well but she still died, Chris Mansi was left wondering why. The neurosurgeon discovered there was a four-hour delay transporting the woman from the accident to the emergency room. That lapse in treatment meant they didn’t see the medical scan showing a large blood clot in her brain for hours. Avoiding these scenarios is what led Mansi to help set up Viz.ai. The San Francisco start-up’s main purpose is to use AI to immediately analyze brain scans and decipher which patients need urgent attention.

svin.viz.ai

Smart home-dwelling devices can do more than play a curated selection of dinner music. With Amazon Echo Dot’s AI technology, help is just a voice command away. In addition to calling anyone hands-free, Alexa, the device’s moniker, can lock doors, track fitness, turn lights on and off, adjust the thermostat and more. The device can be paired with others to add even more smart technology to a home.

www.amazon.com

Automating the patient experience is the future of healthcare. Heading to the doctor’s office for a weird skin reaction or a lingering cough may soon be relegated to the past thanks to MTBC’s smart software that allows practitioners to practice and deliver patient care anywhere at any time with its intuitive iEHR. Scheduling appointments and prescribing medications can already be accomplished with the click of a button.

www.mtbc.com

The number of individuals 60 years and older is projected to double by 2050. With more older Americans choosing to live independently, that means AI-enabled devices are bound to become even more valuable. TruSense’s innovative technology helps give individuals and their family members more peace of mind. Kits are customized depending on health concerns and can include products ranging from diabetes monitoring to GPS technology, motion sensors and respiratory health monitors. TruSense products are medical-grade and HIPAA-compliant.

mytrusense.com

Scalpel. Scissors. Robot? Robotic-assisted surgery isn’t just the future of surgery, it’s happening now. With a motto of “we believe in healing through innovation,” Mazor Robotics has successfully brought AI into the operating room. The Orlando-based company develops AI-assisted robotic techniques that help reduce surgical complications. The smart technology improves spine and brain surgery with its state-of-the-art procedures.

www.mazorrobotics.com/en-us
This book is meant as an educational tool and comprehensive guide for those living with a chronic illness, or perhaps supporting someone who has a chronic illness. Written by a chronic illness sufferer who could not get a diagnosis for many years, the author describes her experience with chronic illness and the medical professionals who were meant to be of assistance. It explains how to live and deal with stress, pain and fatigue, how to better communicate with medical professionals and how to plan ahead effectively.

This guide explains the symptoms and diagnosis of PANDAS (pediatric autoimmune neuropsychiatric disorders associated with strep), PANS (pediatric acute-onset neuropsychiatric syndrome) and related conditions, with treatment options and recommended strategies for supporting children at home, school and in community settings. It covers key symptoms, including obsessive-compulsive disorder, tics, anxiety, sensory issues and personality changes, and it offers practical advice on medical management, nutrition, lifestyle and addressing social and behavioral needs. Each chapter also includes sidebars with key information to remember, and action steps for overcoming challenges, managing relapse, family self-care and providing children with the best possible support.

This book uses the novel Robinson Crusoe as an archetypal metaphor for patients who must learn to survive on their own isolated “island” of chronic pain. This unique style is combined with a variety of in-session approaches and other tools that clients have found helpful in identifying their goals and progress. By emphasizing the importance of self-care, the authors hope to diminish the sense of helplessness felt by both the patients and their loved ones.
Download the *IG Living* eBook today—now available for iPad, Nook and Kindle!

“You can lament what is lost to you, whether it’s opportunity, a person or your health, but clinging to anger is no way to experience life.” — Rebecca Zook in “Life Lessons,” excerpted from *Chronic Inspiration*.

Download a daily dose of inspiration with this heartfelt compilation of writings on life with chronic illness. From coping strategies and parenting tips to “from the trenches” advice on dealing with family and friends who simply don’t get it, these personal stories are sure to uplift, challenge and inspire. Honest and candid, *Chronic Inspiration: Heartfelt Perspectives on Life with Chronic Illness* gives voice to those who refuse to let their diagnosis define who they are or what they can accomplish.

“For the patient community, this was invaluable. When I downloaded it, I knew this would be something I would refer to over and over again.”

— Jenny Gardner

*Chronic Inspiration* can be purchased on iTunes, Amazon and Barnes and Noble.com
### RESOURCE CENTER

#### RESOURCE CENTER

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</tbody>
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**Ataxia Telangiectasia (A-T)**
- A-T Children’s Project: www.atcp.org

**Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)**
- GBS/CIDP Foundation International: www.gbs-cidp.org
- The Foundation for Peripheral Neuropathy: www.foundationforpn.com

**Evans Syndrome**
- Evans Syndrome Research and Support Group: www.evanssyndrome.org

**Guillain-Barré Syndrome (GBS)**
- GBS/CIDP Foundation International: www.gbs-cidp.org
- The Foundation for Peripheral Neuropathy: www.foundationforpn.com

**Idiopathic Thrombocytopenic Purpura (ITP)**
- ITP Support Association – UK: www.itpsupport.org.uk
- Platelet Disorder Support Association: www.pdsa.org

**Kawasaki Disease**
- American Heart Association: www.heart.org/HEARTORG/Conditions/More/CardiovascularConditionsOfChildhood/Kawasaki-Disease_UCM_308777_Article.jsp#.T1T2boePWE0
- American Academy of Family Physicians: aafp.org/afp/2006/1001/p1141.html
- Kawasaki Disease Foundation: www.kdfoundation.org
- KidsHealth: kidshealth.org/parent/medical/heart/kawasaki.html

**Mitochondrial Disease**
- United Mitochondrial Disease Foundation: www.umdf.org
- Mitochondrial Disease Action: www.mitoaction.org

**Multifocal Motor Neuropathy (MMN)**
- The Foundation for Peripheral Neuropathy: www.foundationforpn.com

**Multiple Sclerosis (MS)**
- All About Multiple Sclerosis: www.mult-sclerosis.org/index.html
- Multiple Sclerosis Association of America: mymsaa.org
- Multiple Sclerosis Foundation: www.msfocus.org
- National Multiple Sclerosis Society: www.nationalmsociety.org

**Peripheral Neuropathy (PN)**
- Neuropathy Action Foundation: www.neuropathyaction.org
- Western Neuropathy Association: www.mpneuropathy.org
- Neuropathy Alliance of Texas: neuropathyalliance.org
- The Foundation for Peripheral Neuropathy: www.foundationforpn.com

**Primary Immune Deficiency Disease (PI)**
- Immune Deficiency Foundation: www.primaryimmune.org
- Jeffrey Modell Foundation: www.info4pi.org
- The National Institute of Child Health and Human Development (NICHD): www.nichd.nih.gov/Pages/index.aspx
- American Academy of Allergy, Asthma & Immunology: wwwaaa.org
- International Patient Organisation for Primary Immunodeficiencies (IPOD) — UK: www.ipoi.org
- New England Primary Immunodeficiency Network: www.nepin.org
- Rainbow Allergy Immunology: www.uhospitals.org/rainbow/services/allergy-immunology

**Scleroderma**
- Scleroderma Foundation: www.scleroderma.org
- Scleroderma Research Foundation: www.srfcure.org
- Johns Hopkins Scleroderma Center: www.hopkinsscleroderma.org

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- Johns Hopkins Scleroderma Center: www.hopkinsscleroderma.org

**Pemphigus and Pemphigoid**
- The International Pemphigus and Pemphigoid Foundation: www.pemphigus.org

**Stiff Person Syndrome (SPS)**
- American Autoimmune Related Diseases Association Inc.: www.aarda.org
- Genetic Alliance: www.geneticalliance.org
- Living with Stiff Person Syndrome (personal account): www.livingwithspss.com
- Stiff Person Syndrome: www.stiffpersons.net
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