June-July 2024

IGLiving

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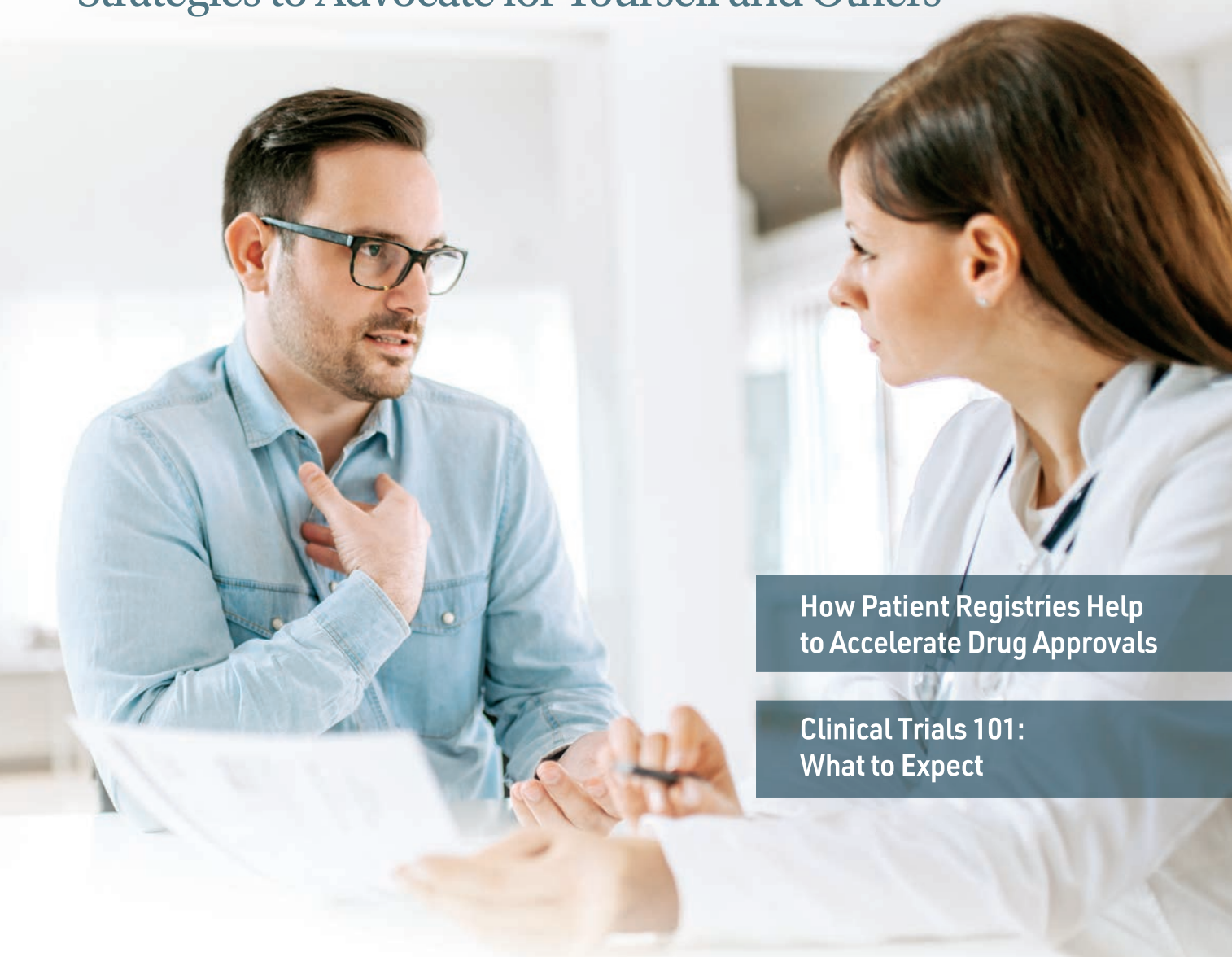
Patient Advocacy

Strategies to Advocate for Yourself and Others



How Patient Registries Help to Accelerate Drug Approvals

Clinical Trials 101: What to Expect

Obtaining Prescriptions After Insurance Denial

The Connection Between Inflammation and Disease

FOR PATIENTS WITH PRIMARY HUMORAL IMMUNODEFICIENCY (PI)

IT'S WHAT'S INSIDE THAT COUNTS

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IMMUNE GLOBULIN INTRAVENOUS (HUMAN) – slra 10% LIQUID

DESIGNED TO DELIVER

Talk to your doctor about whether ASCENIV™ is right for you asceniv.com

Important Safety Information for ASCENIV™

WARNING: RISK OF BLOOD CLOTS (THROMBOSIS), POOR KIDNEY FUNCTION, AND INABILITY TO FILTER WASTE FROM KIDNEYS. BLOOD CLOTS MAY OCCUR WITH INTRAVENOUS IMMUNE GLOBULIN PRODUCTS, INCLUDING ASCENIV.

Before taking ASCENIV, talk to your doctor if you:

- Are of advanced age
- Are unusually sedentary (long periods of sitting down or inactive)
- Are taking estrogen-containing medicines (birth control pills, hormone replacement therapy)
- Have a permanent intravenous (IV) catheter
- Have hyperviscosity of the blood (diseases such as multiple myeloma or other causes of elevated proteins in the blood)
- Have cardiovascular (heart) problems or previous history of stroke

Thrombosis may occur even if you do not have any risk factors.

Serious kidney problems and death can also happen in certain patients who receive such products.

If you are at high risk of thrombosis or kidney problems, your doctor should adjust the dose of ASCENIV and will monitor you for signs and symptoms of thrombosis and viscosity, as well as kidney function.

What is ASCENIV (immune globulin intravenous, human)?
ASCENIV (immune globulin intravenous, human) is a prescription medicine to help adults and adolescents (12 to 17 years old) with primary immunodeficiency fight and prevent infections.

ASCENIV is for intravenous administration only. ASCENIV is made from healthy human blood/plasma.

Who should not use ASCENIV?
ASCENIV should not be used if you had a severe allergic reaction to human immune globulin or if you have been told by a doctor that you are immunoglobulin A (IgA)-deficient and have developed antibodies to IgA and hypersensitivity after exposure to a previous plasma product.

What are possible warnings and precautions with taking ASCENIV?
Hypersensitivity. Severe allergic reactions may occur with immune globulin products, including ASCENIV. If you have a severe allergic reaction, stop the infusion immediately and get medical attention. ASCENIV contains IgA. If you have known antibodies to IgA, you may have a greater risk of developing potentially severe allergic reactions.

If you take ASCENIV or a similar immune globulin product, you could experience a serious and life-threatening blood clot (thromboembolism). This may include pain and/or swelling of an arm or leg with warmth over the affected area, discoloration of an arm or leg, unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, numbness, or weakness on one side of the body. If you are at risk, your doctor may decide to adjust the dose of ASCENIV. Your doctor will monitor you for any signs or symptoms of blood clots or poor blood flow in your arteries.

Always tell your doctor immediately if your medical history is similar to what is described here, and especially if you experience any of these symptoms while taking ASCENIV.

Kidney problems or failure. Kidney problems, kidney failure, and death may occur with use of human immune globulin products, especially those containing sucrose (sugar). ASCENIV does not contain sucrose.

If you have kidney disease or diseases with kidney involvement, your doctor should perform a blood test to assess your hydration level and kidney function before beginning immune globulin treatment and at appropriate intervals thereafter. If your doctor determines that kidney function is worsening, they may discontinue treatment. If your doctor determines you to be at risk, they may start your dose of ASCENIV at a safe level.

People taking human immune globulin products, including ASCENIV, may experience hyperproteinemia (high levels of protein in the blood), hyponatremia (low levels of sodium in the blood), and hyperviscosity (poor blood flow). Your doctor may perform certain blood tests and monitor you to minimize any of the above risks.

Aseptic meningitis syndrome (AMS). Aseptic meningitis is a non-infectious inflammation of the membranes that cover the brain. It causes a severe headache, which may occur with human immune globulin treatment, including ASCENIV. AMS usually happens within a few hours to 2 days after treatment. AMS is more commonly associated with higher doses of treatment and/or after rapid infusion. Your doctor may perform a neurological exam, including spinal tap (sampling fluid which surrounds the spinal cord) to evaluate your condition and to rule out other causes of meningitis.

Hemolysis. Hemolysis refers to the destruction of red blood cells. Immune globulin products, including ASCENIV, may contain certain antibodies that can result in the rupturing of red blood cells. Your doctor should monitor you for signs and symptoms of hemolysis, which may include additional confirmation tests.

Taking intravenous human immune globulin products may cause a build up of fluid in the lungs (pulmonary edema) that is unrelated to heart problems. Your doctor should monitor you for lung-related side effects and may conduct appropriate tests that can detect the presence of certain white blood cells (anti-neutrophils) in the drug or your blood. If needed, your doctor may decide to use oxygen or other respiratory methods to help your breathing.

Transmissible infectious agents. Because ASCENIV is made from human blood, it may carry a risk of transmitting infectious agents such as viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent, and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent. Your doctor will report to the manufacturer any cases of suspected infections spread by the product.

Interference with lab tests. Because ASCENIV contains a variety of antibodies that are infused into your body, blood tests to determine antibody levels may provide misleading interpretations. Be sure to always tell your doctor, nurse, or lab technician of any medicines you are taking and that you are using ASCENIV.

Interactions with medicines. ASCENIV can make vaccines (like measles, mumps, rubella, and chicken pox vaccines) less effective in your body. Before you get any vaccines, tell your healthcare provider that you take ASCENIV.

What are other possible side effects of ASCENIV?
In clinical studies of ASCENIV, some patients experienced the following:

- Headache
- Sinus inflammation (sinusitis)
- Diarrhea
- Intestinal lining inflammation caused by virus (gastroenteritis)
- Common cold (nasopharyngitis)
- Upper respiratory tract infection
- Bronchitis
- Nausea

These are not all the possible side effects of ASCENIV. Talk to your healthcare provider about any side effect that bothers you or that does not go away.

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.

For additional safety information about ASCENIV, please see full Prescribing Information at www.asceniv.com

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IG Living Magazine is read by 30,000 subscribers who are patients that depend upon immune globulin products and their healthcare providers. For information about advertising in IG Living, download a media kit at igliving.com/advertise/advertise.html. Or contact advertising@igliving.com.

About IG Living
IG Living magazine brings together patients, advocates and caregivers in the immune globulin (IG) community.

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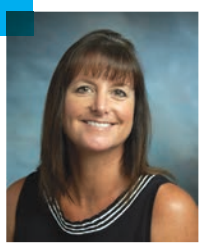
Help Yourself and Others: Get Involved



STUDIES SHOW that when patients take an active role in their healthcare, it not only leads to better health outcomes, but it is key to meeting health goals. Examples of ways to get involved include advocating for yourself and others and participating in patient registries and clinical trials.

Today, patients are viewed as a team player in their healthcare decisions. We discuss the ways in which you can get involved in both your care and others in our article "Is Advocacy Right for Me? Letting Your Voice Be Heard" (p.28). Key aspects of self-advocacy include educating yourself about your illness, its symptoms and treatments; effectively communicating with your doctors; and setting goals for managing your condition both physically and emotionally. We also explore ways in which you can advocate for yourself *and* others through political activism to fight for patient rights and affect healthcare legislation, as well as social activism to raise awareness and share experiences.

In fact, getting involved can actually save lives by better understanding rare diseases and finding treatments. How? By enrolling in patient registries. As we explain in our article "Patient Registries: Key to Accelerating Rare Disease Research" (p.35), there are some 10,000 rare diseases affecting 30 million Americans today, many of which don't have FDA-approved treatments. The only way to get more treatments approved is by increasing patient involvement in clinical trials. Patient registries help to identify eligible participants in clinical trials, as well as garner a better understanding of diseases' history, progression and manifestation. We outline actionable steps you can take to get involved in registries that can be life-changing for you and others. We also provide a list of online sites to locate registries to improve outcomes for your own rare disease.

Many patients can also benefit from getting involved in clinical trials themselves, which can help researchers develop new medications and strategies to prevent and treat diseases. We explain the intricacies of the clinical trials process in our article "Clinical Trials 101: The Basics of Clinical Research" (p.38). Specifically, we outline the different trial types, their phases, the results process, eligibility criteria, the key people involved and how they are regulated. In addition, a list of clinical trial websites is included to help you locate a trial for which you may be eligible.

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

Ronale Tucker Rhodes, MS

IGLiving

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Herd Immunity Safeguards Vulnerable Populations

By Abbie Cornett, MBA

IN AN AGE defined by remarkable strides in medicine, the resurgence of a disease once thought eradicated is deeply troubling. Measles, which the United States declared eliminated as recently as 2000, has resurfaced, and stands out as an example of a preventable affliction now making a troubling comeback. Recent Centers for Disease Control and Prevention data reveals that as of April 18, 2024, 125 measles cases have been reported across 18 states.[1]

Why measles is making a comeback and what the repercussions are is an important topic to investigate. The resurgence of measles in the U.S. can be attributed to various factors, with delayed vaccinations playing a major role. When individuals, particularly children, miss or delay their vaccinations, they remain susceptible to measles and other preventable diseases for extended periods. This susceptibility contributes to outbreaks and an overall increase in measles cases.

Delayed vaccinations create a gap in immunity within communities, allowing the measles virus to spread more easily among susceptible individuals. Furthermore, vaccine hesitancy significantly undermines herd immunity, a vital safeguard against infectious diseases. Herd immunity occurs when a large portion of the population becomes immune, lowering the risk of disease transmission. This indirect protection benefits those without protection, greatly reducing the likelihood of disease spread.[2]

Understanding the threshold for herd immunity is crucial since it varies depending on the disease. Measles, for instance, is highly contagious, requiring an estimated 95 percent vaccination rate to achieve herd immunity. At this level of coverage, the spread of measles within the community becomes significantly unlikely, providing indirect protection to the remaining five percent of the population.[3]

While a handful of cases may not set off alarm bells for most healthy individuals, they pose significant risks for those managing immunodeficiencies or chronic illnesses. This population bears the brunt of the impact when herd immunity is compromised.

Addressing vaccine hesitancy requires a multifaceted approach tailored to individuals' concerns and barriers to immunization. Education is key, with healthcare providers playing a vital role in providing clear, accurate information about vaccine safety, efficacy and importance. Public health campaigns and community outreach are also crucial for disseminating evidence-based information and addressing vaccine concerns.

Ensuring timely vaccinations for all children in the U.S. requires addressing both concerns contributing to delays or missed vaccinations and access issues. Access to healthcare services varies based on factors such as location, socioeconomic status and insurance coverage. Targeted interventions such as offering well-child visits and immunization clinics are crucial in bridging these gaps. Outreach efforts to underserved communities, including mobile vaccination clinics and community health centers, play a vital role in reaching those facing barriers to traditional healthcare settings.[4,5] Collaboration among healthcare providers, public health agencies, community organizations and policymakers is essential for implementing and maintaining these initiatives, ensuring equitable access to essential vaccinations for every child.

In conclusion, widespread vaccination is essential for maintaining herd immunity and protecting public health. It's particularly crucial for those with chronic illnesses, immunodeficiencies or medical reasons preventing vaccination. Herd immunity offers indirect protection to these vulnerable groups, reducing the risk of exposure and complications from infectious diseases. Therefore, addressing vaccine hesitancy and promoting acceptance is not just about individual health but a collective responsibility to safeguard society's well-being.

References

1. Centers for Disease Control and Prevention. Measles Cases and Outbreaks. Accessed at www.cdc.gov/measles/cases-outbreaks.html#:~:text=There%20have%20been%207%20outbreaks,58)%20were%20outbreak%2Dassociated.
2. Desai, AN, and Majumder, MS. What Is Herd Immunity. *JAMA*, 2020;324(20):2113. Accessed at jamanetwork.com/journals/jama/fullarticle/2772168.
3. Macmillan, C. Herd Immunity: Will We Ever Get There? Yale Medicine, May 21, 2021. Accessed at www.yalemedicine.org/news/herd-immunity.
4. Leibowitz, A, Livaditis, L, Daftary, G, et al. Using Mobile Clinics to Deliver Care to Difficult-to-Reach Populations: A COVID-19 Practice We Should Keep. *Preventive Medicine Reports*, 2021 Dec; 24: 101551. Accessed at www.ncbi.nlm.nih.gov/pmc/articles/PMC8428151.
5. Pfizer. Addressing Disproportionate Childhood Vaccination. Accessed at www.pfizer.com/news/articles/addressing_disproportionate_childhood_vaccination.

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

Do You Practice Self-Care Basics for Living with PI?

I do my best to take care of me first. Lots of sleep every night, sometimes during the day, healthy diet, exercise most days and keeping stress under control is very important. I'm thankful for intravenous immune globulin and my medication cocktail.

I live by myself as I am single and I've never had any kids, so I guess you could say that I do this all the time?

Are Gastric Problems a Sign of CVID?

For decades, first I was told I had a nervous stomach. Then I was told I had a spastic colon. Then they said I had irritable bowel syndrome. I had my fourth colonoscopy and second endoscopy this year. They said I had either ulcerative colitis or Crohn's. I did a genetic blood test and then I went to another gastrointestinal doctor. I went for a second opinion, and I had my fifth colonoscopy and third endoscopy, and I had blood tests and stool tests. I was told I have ulcerative colitis with complications. Now, recently, I had another stool test and I was told I now have ulcerative pancolitis, which I think means that it has spread and gotten worse.

Well, I know that I was diagnosed with it, and I've been following a super restrictive diet since my diagnosis earlier this year. I've lost 30-plus pounds. I was on a steroid for eight weeks. I went into remission, and then I was told to get off the medication. I retested in six weeks, and now I'm no longer in remission. Now I have to do another test in six weeks. This is super frustrating. I did hear something recently that having a primary immune deficiency increases a person's risk for inflammatory bowel disease, as well as blood diseases.

What Is the Impact of Alternative Funding Programs on Patients with Chronic Illness?

I'm so thankful for programs by the drug companies, or I wouldn't have made it!

Years ago before I went on a Medicare Advantage plan, I had a plan through Blue Cross and Blue Shield for Illinois. It was called Illinois comprehensive health insurance (Ichip). There were medications I couldn't afford, so I had to get grants through something called the patient advocate foundation in Virginia. I could only get the grant as long as I had the insurance so they would pick up the balance. But then the next year they wouldn't cover the cost, so I couldn't use the pain medication the doctor had prescribed for my complex regional pain syndrome. So the doctor prescribed four other medications to take the place of one medication, and it caused excessive weight gain and all types of other side effects.

Join the conversation! Connect with other immune globulin patients through IG Living's Facebook page at www.facebook.com/IGLivingMagazine. Each day, we post interesting articles and facts, as well as thought-provoking questions you can weigh in on. These are some snapshots of what's being discussed.

Can IVIG Be Infused Somewhere Other Than in My Home to Treat CIDP?

If I want to receive intravenous immune globulin (IVIG) infusions in an infusion center or suite rather than in my home to treat chronic inflammatory demyelinating polyneuropathy (CIDP), would I be able to?

Abbie: Leslie Vaughan, RPh, CSP, IgCP, and Michelle Greer, RN, IgCN, chief operations officer and executive vice president of sales, respectively, at Nufactor, a specialty infusion company, said the answer depends on the insurance provider, but most providers cover IVIG for CIDP in the home setting. However, it is important to know that some insurance providers prefer home infusion over infusion centers, whereas others don't.

Is There a Provider or a Diagnostic Flow Chart to Diagnose Autoinflammatory Conditions?

Is there either an adult medical provider and/or a clear diagnostic flow chart to help diagnose autoinflammatory conditions? We are waiting on the Invitae Autoinflammatory panel for gene testing, but I don't have anything past that or someone who knows how to perform an observation without gene testing, nor use drugs (colchicine, anakinra) for it.

Abbie: I spoke with Marc Riedl, MD, MS, associate professor of medicine in the division of rheumatology, allergy and immunology at the University of California, San Diego, regarding your question. He replied that autoinflammatory conditions can be challenging to diagnose because they rely on pattern recognition and often present with multiple seemingly unrelated symptoms with a fair amount of variability. So, it's about having clinical suspicion based on the history and symptoms and then completing genetic testing since most of the conditions heavily rely on finding an associated mutation.

The Invitae Autoinflammatory panel provides good coverage in that regard. If genetic testing is negative, there are a handful of conditions in which the genetics are not known, but they have established clinical criteria, allowing clinicians to determine if there is sufficient clinical evidence to consider treatment. Treatment varies widely depending on the specific condition and underlying mechanism. Allergists with experience in clinical immunology are typically adept at managing these conditions, and some rheumatologists also handle them well, which is beneficial since autoimmune conditions may present similarly. There are several centers in the U.S. that specialize in this evaluation, so it's worth considering those sites. However, many are located at Children's hospitals, so you would need to locate sites that also see adults.

With regard to the diagnostic flow chart, there is a patient advocacy group that provides a summary chart including some (but not all) autoinflammatory conditions that can be found at saidsupport.org/how-to-use-the-comparison-chart-of-systemic-autoinflammatory-diseases-diagnostic-chart.

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

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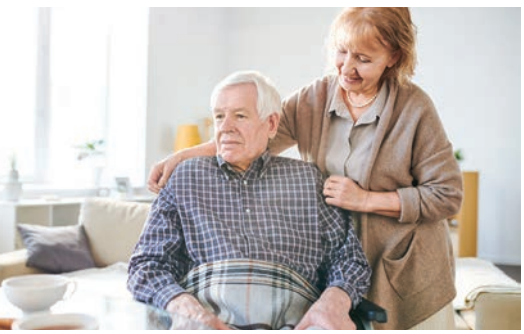
The Patient-Caregiver Relationship

By Mairead McConnell, PhD



HEALTHY relationships are vitally important to our physical and emotional health, but the stress of illness and caregiving can put a strain on even the closest bonds. Whether you are living with chronic illness yourself or supporting a loved one through theirs, navigating the relationship between patient and caregiver presents unique challenges for both individuals.

In this context, I use "caregiver" to refer to any loved one, family member, friend or hired helper who offers short- or long-term emotional and/or instrumental support for someone living with illness or disability. The caregiver need not live with the patient or see them every day, and the patient need not be disabled or critically ill to warrant caregiving. Caregiving looks different in different scenarios. However, many patient-caregiver relationships will face similar challenges. Consider how the themes below may apply to you and help improve your relationships.

• *Navigating changing roles.* Change is a common source of stress, and it can be especially unsettling when close, reliable, intimate relationships undergo a major shift or change. It will take time for both people to adjust to a new reality, to grieve the loss of the way things were and to accept that roles and responsibilities may temporarily — or permanently — be altered. Give yourself grace as you grieve and adjust; consider that your loved one may be struggling with the very same feelings.

• *Communicating about difficult topics.* Communication is the key to successful relationships, but that doesn't make it easy. It can be especially difficult to communicate about important and sensitive topics such as healthcare decisions, emotions, boundaries or finances. Remember that no matter how well someone knows you, they cannot read your mind. Clearly asking for what you need is a gift to both you and them: The other person doesn't have to guess, and you reduce the likelihood that you will be disappointed. Communication doesn't have to be perfect to be effective, and it's OK to ask for a do-over and try again.

• *Engaging in healthy conflict.* Conflict, in any relationship, is normal and necessary. That doesn't mean that all conflict is healthy, but all healthy relationships have some conflict. It is common to focus on the other person's response: how they behaved, why they are wrong, etc. To improve and shift your conflicts, consider how *you* respond when you are upset. Do you tend to fight and criticize? Do you tend to avoid and flee? Do you shut down or try to smooth things over immediately? Understanding how you respond to conflict doesn't mean you are entirely responsible, but it allows you to take responsibility for the only part you can control: yourself.

• *Maintaining your own identity.* It is natural for the role of patient or caregiver to become a part of your identity. While this can be a sign of acceptance, it is important to also maintain parts of your identity that extend beyond just that one domain. Consider engaging in activities that allow you to embody other parts of yourself as a friend, sibling, learner or expert. Perhaps there are moments when the caregiver can become the student and learn from the patient. This may take place within the patient-caregiver relationship or in independent activities that allow each person to explore other aspects that make them who they are.

• *Caring for oneself.* We all need care and support to see that our needs matter. This is true in relationships with others and is just as true in our relationship with ourselves. As a caregiver, caring for yourself can feel like yet another chore on a long to-do list, but it is equally as important as caring for your loved one. Your needs and well-being matter. Give yourself some of the same compassion and consideration that you give to others. As a patient, consider that you also have a responsibility to care for yourself in the ways that you can, even within your limitations and external support. Being kind toward yourself, asking for what you need and actively engaging in the things that help you be and stay well are within your capacity to honor and care for yourself. IG

MAIREAD MCCONNELL, PhD, is a clinical psychologist and assistant professor at Banner University Medical Center in Tucson, Ariz. She specializes in health psychology and is passionate about helping patients live well while navigating the challenges of chronic illness.

Reconnect with volunteer days

People with primary immunodeficiency (PI) who infuse CUVITRU weekly or every other week may be able to experience more of these moments.

What is CUVITRU®?

CUVITRU [Immune Globulin Subcutaneous (Human)] 20% Solution is a ready-to-use liquid medicine that is given under the skin (subcutaneously) to treat primary immunodeficiency (PI) in people 2 years and older.

IMPORTANT SAFETY INFORMATION

What is the most important information I need to know about CUVITRU?

CUVITRU can cause the following serious reactions:

- Severe allergic reactions causing difficulty in breathing or skin rashes
- Decreased kidney function or kidney failure
- Blood clots in the heart, brain, lungs, or elsewhere in the body
- Severe headache, drowsiness, fever, painful eye movements, or nausea and vomiting
- Dark colored urine, swelling, fatigue, or difficulty breathing

Who should not use CUVITRU?

Do not use CUVITRU if you:

- Have had a severe allergic reaction to immune globulin or other blood products.
- Have a condition called selective (or severe) immunoglobulin A (IgA) deficiency.

What should I avoid while taking CUVITRU?

- CUVITRU can make vaccines (like measles/mumps/rubella or chickenpox vaccines) not work as well for you. Before you get any vaccines, tell your healthcare provider (HCP) that you take CUVITRU.
- Tell your HCP if you are pregnant, or plan to become pregnant, or if you are nursing.

What are the possible or reasonably likely side effects of CUVITRU?

CUVITRU can cause serious side effects. If any of the following problems occur after starting CUVITRU, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain.
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem.
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot.

Cuvitru
[Immune Globulin Subcutaneous (Human)] 20%

Proven protection from infection

In the North American (NA) study, there were 0.012 acute serious bacterial infections (ASBIs) per patient-year.*† This exceeds the FDA standard for effectiveness, which is one serious ASBI per year.

Nearly all infusions (99.8%) were completed without reduction, interruption or discontinuation due to tolerability

No patients discontinued due to local adverse reactions (ARs) and 0 serious ARs related to CUVITRU were reported.

The most common adverse reactions observed in clinical trials in ≥5% of patients were: local adverse reactions including mild or moderate pain, erythema, and pruritus, and systemic adverse reactions including headache, nausea, fatigue, diarrhea, and vomiting.

Flexible administration that can be tailored to fit your lifestyle‡§

CUVITRU can be infused at the fastest rates and highest volumes with the fewest infusion sites of any subQ IG.§

In the NA clinical study, CUVITRU was studied in 77 people with PI ≥2 years of age. The main goal of the study was to measure how many acute serious bacterial infections (ASBIs) were experienced over the course of 1 year. ASBIs are short-term but serious infections that require immediate medical care. ASBIs were evaluated in 74 people taking CUVITRU for an average of 380.5 days (range, 30-629 days).
*One ASBI that occurred during the study was a case of pneumonia in a 78-year-old person.
†A patient-year is a patient experience in a clinical trial over the course of 1 year. One patient-year is equal to, for example, the experience of 2 patients for 6 months, or 12 patients for 1 month each.
‡In the NA study, the average infusion time was 0.95 hours (range 0.2-6.4 hours) and most (84.9%) used 1 to 2 needlesticks.
§You'll infuse your first 2 infusions at 10 to 20 mL/hr/site. After that, you'll be able to increase your rate up to 60 mL/hr/site as tolerated. Infuse at up to 4 sites simultaneously.
SubQ IG=subcutaneous immune globulin.

IMPORTANT SAFETY INFORMATION (continued)

- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem.
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem.
- Fever over 100°F. This could be sign of an infection.

The following one or more possible side effects may occur at the site of infusion. These generally go away within a few hours, and are less likely after the first few infusions.

- Mild or moderate pain
- Redness
- Itching

The most common side effects that may occur are:

- Headache
- Nausea
- Fatigue
- Diarrhea
- Vomiting

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away.

Please see Important Facts about CUVITRU on the following page.

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.

Up to 100% of out-of-pocket co-pay costs could be covered.

Scan the QR code to learn more about CUVITRU, including co-pay costs.

Takeda

IMPORTANT FACTS about CUVITRU (CUE-vih-troo) [Immune Globulin Subcutaneous (Human)] 20% Solution

What is the most important information I need to know about CUVITRU?

CUVITRU can cause the following serious reactions:

- Severe allergic reactions causing difficulty in breathing or skin rashes
- Decreased kidney function or kidney failure
- Blood clots in the heart, brain, lungs, or elsewhere in the body
- Severe headache, drowsiness, fever, painful eye movements, or nausea and vomiting
- Dark colored urine, swelling, fatigue, or difficulty breathing

What is CUVITRU?

CUVITRU is a ready-to-use liquid medicine that contains immunoglobulin G (IgG) antibodies, which protect the body against infection. CUVITRU is used to treat patients with primary immunodeficiency diseases (PI).

There are many forms of PI. The most common types of PI result in an inability to make a very important type of protein called antibodies, which help the body fight off infections from bacteria or viruses. CUVITRU is made from human plasma that is donated by healthy people. CUVITRU contains antibodies collected from these healthy people that replace the missing antibodies in PI patients.

Who should not use CUVITRU?

Do not use CUVITRU if you have a known history of a severe allergic reaction to immune globulin or other blood products. If you have such a history, discuss this with your healthcare provider (HCP) to determine if CUVITRU can be given to you. Tell your HCP if you have a condition called selective (or severe) immunoglobulin A (IgA) deficiency.

How should I use CUVITRU?

CUVITRU is given under the skin (subcutaneously). Most of the time, infusions under the skin are given at home by self-infusion or by caregivers. Instructions for giving CUVITRU under the skin (subcutaneously) are provided in the FDA-approved patient labeling (Information for Patients and Instructions for Use). Only use CUVITRU by yourself after you have been instructed by your HCP.

What should I avoid while taking CUVITRU?

CUVITRU can make vaccines (like measles/mumps/rubella or chickenpox vaccines) not work as well for you. Before you get any vaccines, tell your HCP that you take CUVITRU.

Tell your HCP if you are pregnant, or plan to become pregnant, or if you are nursing.

What are the possible or reasonably likely side effects of CUVITRU?

The following are one or more possible reactions that may occur at the site of infusion. These generally go away within a few hours, and are less likely after the first few infusions.

- Mild or moderate pain
- Redness
- Itching

The most common side effects of CUVITRU are headache, nausea, fatigue, diarrhea, and vomiting.

If any of the following problems occur after starting treatment with CUVITRU, stop the infusion immediately and contact your HCP or call emergency services. These could be signs of a serious problem.

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation of the lining around your brain.
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem.
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot.
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem.
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem.
- Fever over 100°F. This could be a sign of an infection.

These are not all the possible side effects. You can ask your HCP for a physician's information leaflet. Tell your HCP about any side effect that bothers you or that does not go away.

Whenever giving yourself treatments at home, you should have another responsible person present to help treat side effects or get help if you have a serious adverse reaction occur. Ask your HCP whether you should have rescue medications, such as antihistamines or epinephrine.

How do I store CUVITRU?

Store CUVITRU refrigerated or at room temperature.

- You can store CUVITRU in the refrigerator (36°F to 46°F [2°C to 8°C]) for up to 36 months or
- You can store CUVITRU at room temperature (up to 77°F [25°C]) for up to 24 months.
- Do not return CUVITRU to the refrigerator if you take it out to room temperature.
- Do not freeze.
- Do not shake.
- Check the expiration date on the carton and vial label. Do not use CUVITRU after the expiration date.
- Protect from light. You can use the original CUVITRU containers to protect it from light.

How do I get more information about CUVITRU?

The risk information provided here is not comprehensive. To learn more, talk about CUVITRU with your HCP or pharmacist. The FDA-approved Full Prescribing Information, including Information for Patients, can be found at www.CUVITRU.com or by calling 1-877-TAKEDA7 (1-877-825-3327).

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SARS-CoV-2 and COVID-19: Why Does Our Immune System Have a Problem with This Virus?

By Terry O. Harville, MD, PhD

AS MENTIONED in the last issue, SARS-CoV-2 (SARS2 for convenience) is a zoonotic virus, a type of virus endemic in a specific type of animal that through chance encounters can infect humans. A common example of a zoonotic virus we encounter each winter is influenza. It is likely that most have heard of swine flu and bird flu, the latter of which has more recently devastated the poultry industry. Further, this recent bird flu has been harming endangered bird species in the wild. Since humans may occasionally encounter an infected bird, a susceptible person and/or a bird flu virus that has undergone some mutations may allow for the virus to infect the person, rather than remain specific for the endemic host species. If there is pre-existing sufficient immunity, for example from prior vaccinations and exposures, illness could be brief or even not noticed. If there is not prior immunity and the person is immunocompromised, there could be severe, even life-threatening, illness. This also depends on the "virulence" of the virus. A mildly virulent virus may not cause much disease, even in someone who is immunocompromised, whereas a highly virulent virus could cause deadly disease even in someone with fully intact immunity and with prior immunizations and exposures.

Even though we speak of swine flu and bird flu as though they are each specific for the endemic species, influenza can easily mutate and reassort its gene segments, allowing for it to more readily infect humans or even pass from birds to swine and vice versa, and even infect other species. Further, this has been occurring for thousands and perhaps millions of years of human evolution. A "new" influenza virus would develop, and Darwinian evolution would take place, allowing those with a more robust immunity to the new form to survive and pass on the genetics responsible for survival. Alternatively, those whose immune systems could not provide the correct protection would not survive, thus those "poorer-protective" genes would not pass on. Additionally, since for most of human evolution we lived in small and isolated groups, worldwide pandemic would not occur. Unfortunately, though, isolated and susceptible populations of humans may not have survived and been lost in time.

Examples of more recent tragic zoonotic viruses include HIV and Ebola. These have been endemic in specific animal species for a very long time, and while over the millennia of time, these viruses may have occasionally infected humans, they do not appear to have infected large populations of humans in the past. Only in the recent historical time frame have they made their way into large numbers of humans. Fortunately, we have medications that can help. And for Ebola, vaccines have been produced that work. Unfortunately with HIV, it may ultimately be impossible to produce a good universal vaccine.

This begs the question: "Why can we not develop an easy and good vaccine for HIV, since most other infections can have useful vaccines?" The answer, in some respects, mirrors the problem with SARS2 (I will be discussing this more thoroughly). HIV binds to CD4 on T lymphocytes to enter into and infect this specific subpopulation of T lymphocytes, commonly known as "helper" T lymphocytes. To create a "neutralizing antibody" from a vaccine to try to prevent infection with HIV means that the vaccine must be to the part of HIV that attaches to CD4. As discussed last time, for every antibody we produce, we produce a corresponding antibody, which is against the target to which the original antibody was binding (so-called anti-idiotypic antibody). Therefore, to make an antibody to HIV to prevent it from binding to CD4, our immune system will subsequently make an antibody directed against CD4. In essence, this antibody may actually help HIV infect CD4 T lymphocytes, and the anti-CD4 antibody can further diminish the CD4 T lymphocyte count and accelerate the development of AIDS.

In the next column, I will discuss these concepts in detail regarding SARS2.

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.

Understanding the FDA Approval Process for New Drugs

By Michelle Greer, RN, IgCN

THE U.S. FOOD and Drug Administration (FDA) is a federal regulatory authority whose general purpose is to protect public health by "ensuring the safety, efficacy and security of human and veterinary drugs, biological products and medical devices; and by ensuring the safety of our nation's food supply, cosmetics and products that emit radiation."[1] FDA safeguards consumers by enforcing strict guidelines for many consumer products, from over-the-counter pain relievers and poultry to perfume and pet food (Table). Here, we look specifically at the role FDA plays in the drug development process.

The Preapproval Process

In all, there are five steps in the drug development process, and the first two of them (discovery and development and preclinical research) take place before the FDA approval process begins. During a new drug's early development, the main goal is for the drug sponsor (manufacturer or potential marketer) to investigate whether the drug product is reasonably safe for initial use in humans, and whether it shows pharmacological promise that justifies commercial development. When the new drug product is deemed to be a suitable candidate for further development, the sponsor gathers data to show it will not pose an unreasonable risk to humans when used in limited, early-stage studies.[2] That's where FDA comes in.

Over the course of the next three steps (clinical research, FDA drug review and FDA post-market drug safety monitoring), FDA enforces rules and regulations to ensure the drug product is reasonably safe for consumers.

Investigational New Drug Applications

After the drug has been tested in a living organism and is ready to move on to human trials (under the Federal Food, Drug and Cosmetic Act), its legal status changes, and the molecule is legally considered a new drug that must follow specific requirements of the drug regulatory system.[2] Federal law stipulates a drug cannot be distributed across state lines until it has an approved marketing plan. However, since a new drug product will likely be used at multiple sites around the country during a clinical trial, a provision for legally distributing it across state lines during clinical trials is necessary.

An investigational new drug application (IND) helps get an exemption to this law approved. FDA gets involved during this mandatory part of the clinical research phase, specifically when the drug's sponsor completes an IND application. The IND application is quite extensive and includes the data collected during steps one and two of the drug development process. An IND must also be completed for an approved drug for a new indication. There are three types of INDs:[2]

1) Investigator-initiated IND: This is submitted to study a new compound or an approved drug to get a new indication of that drug.

2) Emergency IND: This allows approval of an unapproved drug in situations in which the intended subject does not meet the criteria of an existing study protocol. Or, if an approved study protocol does not exist,

the usual procedure is to contact the manufacturer to determine if the drug or biologic can be made available for emergency use under the company's IND.[3]

3) Treatment IND: This is submitted for experimental drugs showing promise in clinical testing for serious or immediately life-threatening conditions while the final clinical work is conducted and the FDA review takes place.

Once a non-urgent IND is submitted, FDA has 30 days to review the application. From there, the trial is either approved to continue, or a clinical hold is placed to delay or stop the trial because there is something in the trial that could produce risk to participants. (A clinical hold is rare; instead, FDA often provides comments intended to improve the quality of a clinical trial.) In most cases, if FDA is satisfied the trial meets federal standards, the applicant is allowed to proceed with the proposed study.[2]

New Drug Applications

Once the researchers have concluded the drug is safe and effective, a new drug application (NDA) can be submitted to market that drug. This is the way the researchers and/or drug manufacturer make their case for FDA approval. All the data and information provided help FDA reviewers decide whether or not:[4]

- the drug is safe and effective in its proposed use(s), and the benefits of the drug outweigh the risks;
- the drug's proposed labeling (package insert) is appropriate, and what it should contain;
- the methods used in manufacturing the drug and the controls used to maintain the drug's quality are adequate to preserve the drug's identity, strength, quality and purity.

Table. What Does FDA Regulate?

Product	Examples
Foods and beverages	Bottled water Dietary supplements Food additives Food products* Infant formulas *The U.S. Department of Agriculture plays a lead role in regulating aspects of some meat, poultry and egg products.
Drugs	Over-the-counter (non-prescription) drugs Prescription drugs (both brand name and generic)
Biologics	Allergenics Blood and blood products Cellular and gene therapy products Tissue and tissue products Vaccines for humans
Medical devices	Complex technologies such as heart pacemakers Dental devices Simple items such as tongue depressors and bedpans Surgical implants and prosthetics
Electronic products that give off radiation	Laser products Mercury vapor lamps Microwave ovens Sunlamps Ultrasonic therapy equipment X-ray equipment
Cosmetics	Color additives found in makeup and other personal care products Nail polish and perfume Skin cleansers and moisturizers
Veterinary products	Livestock feed Pet food Veterinary drugs and devices
Tobacco	Cigars Cigarettes Cigarette tobacco E-cigarettes (tobacco and non-tobacco-derived nicotine) Hookah Roll-your-own tobacco Smokeless tobacco

Drug Development Designations

For each NDA, FDA assigns designations including:[5,6]

- *Fast track.* This facilitates development and expedites review of drugs that treat serious conditions and fill an unmet medical need.
- *Breakthrough therapy.* This expedites development and review of drugs that may demonstrate substantial improvement over available therapy.
- *Accelerated review.* This allow drugs

for serious conditions that filled an unmet medical need to be approved based on a surrogate endpoint.

• *Priority review.* This indicates FDA's goal to take action on the application within six months.

• *Standard review.* This indicates FDA's standard timeline for application review of 10 months.

Once the researchers have concluded the drug is safe and effective, a new drug application (NDA) can be submitted to market that drug.

Orphan Drug Designations

More than 7,000 rare diseases affect more than 30 million people in the United States.[7] Many are life-threatening and most do not have treatments, but research and development costs to find drugs to treat rare diseases have historically deterred pharmaceutical companies from pursuing them. However, the Orphan Drug Approval Law of 1983 incentivized pharmaceutical companies to develop drugs for rare diseases.

When a new drug for a rare disease is trialed and subsequently approved by FDA, it can apply for something called an orphan drug designation (ODD). Drugs for rare diseases must go through the same approval process as other drugs, so the process for seeking ODD is separate from and in addition to an FDA approval. If ODD is approved, the manufacturer receives tax credits for qualified clinical trials, exemption from user fees and a potential seven years of market exclusivity after FDA approval.

FDA's Post-Market Involvement

Once an FDA approval is granted, the label and prescribing information are specified. The label describes what the drug is intended to treat, and the prescribing information gives the details on how clinicians are to prescribe and utilize the new medication for their patients.

FDA continues to oversee the safety and efficacy of a drug product after it is brought to market. FDA does this through:

• *Safety monitoring.* FDA conducts post-market safety monitoring, since the only way to really know how effective a new medication is and how it is tolerated is through ongoing observation and tracking after the drug hits the market.

• *Change oversight.* In addition, FDA requires the manufacturer to complete a supplemental application for review and approval if it makes any significant changes from the initial NDA.

• *Routine inspections.* Periodic inspections of manufacturer facilities are also conducted. These are routine, but they also occur if any problems arise. They can be announced or unannounced.

• *Marketing regulations.* FDA regulates drug advertisement and promotion post-market. Manufacturers may not promote the drug in any way other than for what the drug was approved.

• *Patent protection.* New drugs are patent-protected for a period of time. Once the patent expires, other companies can develop a similar version of that drug. This is known as a generic version, which must go through its own FDA approval process. (However, the FDA approval process for generics is less complicated and shorter since the original company conducted all the research for that drug.)

A Long Journey to FDA Approval

FDA approval is just one step of a new drug's development process and is interdependent upon clinical trials. For in-depth information on the clinical trials process, including a breakdown of the various phases involved, see "Clinical Trials 101: The Basics of Clinical Research" on p.38.

References

1. U.S. Food and Drug Administration. What We Do. Accessed at www.fda.gov/about-fda/what-we-do.
2. U.S. Food and Drug Administration. Investigational New Drug (IND) Application. Accessed at www.fda.gov/drugs/types-applications/investigational-new-drug-ind-application.
3. U.S. Food and Drug Administration. Emergency Use of an Investigational Drug or Biologic, January 1998. Accessed at www.fda.gov/regulatory-information/search-fda-guidance-documents/emergency-use-investigational-drug-or-biologic.
4. U.S. Food and Drug Administration. New Drug Application (NDA). Accessed at www.fda.gov/drugs/types-applications/new-drug-application-nda.
5. U.S. Food and Drug Administration. Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review. Accessed at www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review.
6. U.S. Food and Drug Administration. Development and Approval Process I Drugs. Accessed at www.fda.gov/drugs/development-approval-process-drugs.
7. U.S. Food and Drug Administration. Rare Diseases at FDA. Accessed at www.fda.gov/patients/rare-diseases-fda.

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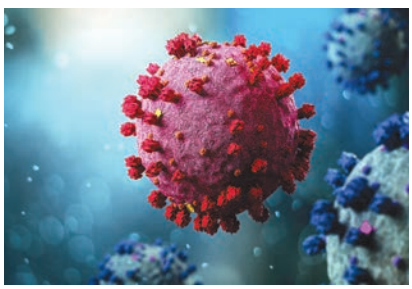
MEDICINES

New Drug Approved to Protect Immune Compromised from COVID-19



Invivyd's PEMGARDA (pemivibart), formerly VYD222, a half-life extended monoclonal antibody (mAb), has been approved by the U.S. Food and Drug Administration (FDA) to protect immunocompromised individuals against COVID-19. It is the first such drug to become available since the agency pulled AstraZeneca's Evusheld off the market in January 2023 after new Omicron variants had rendered Evusheld ineffective. The emergency use authorization (EUA) from FDA is for the pre-exposure prophylaxis (prevention) of COVID-19 in adults and adolescents (12 years of age and older weighing at least 40 kg) who have moderate-to-severe immune compromise due to certain medical conditions or receipt of certain immunosuppressive medications or treatments and are unlikely to mount an adequate immune response to COVID-19 vaccination. However, the drug should not be used in individuals currently infected with or have had a known recent exposure to an individual infected with SARS-CoV-2.

The EUA of PEMGARDA is based on the totality of scientific evidence available such as data showing that immunobridging was established in the CANOPY clinical trial and that the calculated serum neutralizing antibody titers against JN.1 were consistent with the titer levels associated with efficacy in prior clinical trials of adintrevimab (ADG20), the parent mAb for VYD222, and other monoclonal antibody products. JN.1 is currently the dominant variant circulating in the U.S., according to estimates from the Centers for Disease Control and Prevention. PEMGARDA (pemivibart) (4,500 mg) is administered as an intravenous infusion.

"The PEMGARDA EUA marks a transformational moment for Invivyd and for the many moderately to severely immunocompromised people who are vulnerable to COVID-19 disease in the U.S. This EUA milestone represents strategic proof-of-concept for our company and platform, affirming the unique strategy we embarked on over a year ago: to use rapid innovation and surrogate markers to bring new antibodies to market repeatedly," said Dave Hering, chief executive officer of Invivyd. "PEMGARDA is the first authorized monoclonal antibody from our proprietary platform approach. We are committed to ongoing process improvement while working with global regulatory agencies with the aim to increase the speed and efficiency of new mAb candidate development even further. Additionally, we are planning to explore the protective clinical benefits of mAb prophylaxis for symptomatic COVID-19 disease in future studies." IG

Invivyd Announces FDA Authorization for Emergency Use of PEMGARDA™ (Formerly VYD222) for Pre-Exposure Prophylaxis (PrEP) of COVID-19. Invivyd press release, March 22, 2024. Accessed at kdvr.com/business/press-releases/globenewswire/9068086/invivyd-announces-fda-authorization-for-emergency-use-of-pemgarda-formerly-vyd222-for-pre-exposure-prophylaxis-prep-of-covid-19.

RESEARCH

KORU Signs Agreement for Phase III Trial of SCIG Drug

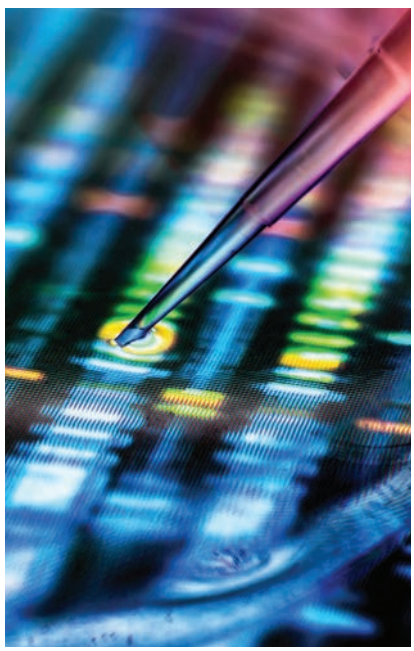
KORU Medical Systems has entered into a clinical supply agreement related to a novel subcutaneous immune globulin (SCIG) drug now proceeding to a Phase III trial. The Phase III trial will evaluate the drug's safety, efficacy and performance across a variety of medical conditions, including autoimmune diseases, primary immunodeficiency disorders and neurological disorders. KORU Medical has developed a custom device for the trial, which is based on the company's existing Freedom System but tailored to the specific requirements of the new drug.

Linda Tharby, president and CEO of KORU Medical, expressed pride in the company's ability to meet the needs of pharmaceutical partners and patients. She highlighted the partnership's role in advancing healthcare through innovation. Tharby also indicated that, contingent upon successful trial results, the company anticipates filing for U.S. Food and Drug Administration clearance to commercialize the SCIG drug. IG

Abdulazez, A. KORU Medical Inks Deal for Phase 3 Trial of SCIG Drug. Investing.com, March 6, 2024. Accessed at www.investing.com/news/stock-market-news/koru-medical-inks-deal-for-phase-3-trial-of-scig-drug-93CH-3326507.

RESEARCH

Study Finds Inborn Errors of Immunity in Children with Sepsis



According to a recent study, whole-exome sequencing helped physicians identify undiagnosed primary immunodeficiency diseases in children with community-acquired sepsis (CAS).

The study comprised 34 children aged 1 month to 14 years (mean age, 46.5 months; 18 girls) admitted to the ICU at Children's Medical Center in Tehran, Iran, with suspected CAS and symptoms or signs that suggested inborn errors of immunity (IEI), but without an IEI diagnosis. Common clinical manifestations that suggested IEI in the cohort included recurrent respiratory infections (35 percent), chronic or recurrent diarrhea and gastroenteritis (32 percent), failure to thrive (23.5 percent), recurrent skin or perianal abscesses (15 percent), recurrent oral aphthous (12 percent) and candidiasis (6 percent). The cohort also included 22 (65 percent) patients with a family history of consanguinity and 10 (29 percent) with a family history of IEI. Two of the patients were diagnosed with hemophagocytic lymphohistiocytosis instead of CAS but remained in the cohort due to their personal histories of recurrent infections and family histories of IEI.

Whole-exome sequencing identified causative gene variants in 29 (85 percent) of the patients. Overall, the researchers said, 28 had primary immunodeficiencies and one had interstitial lung and liver disease. Seven (21 percent) patients had afflicted cellular and humoral immunity, including two (6 percent) with severe combined immunodeficiency (SCID) and four (15 percent) with combined immunodeficiencies (CIDs). Additional diagnoses included:

- CIDs with associated or syndromic features: six (18 percent) patients;
- congenital defects of phagocyte number or function: four (12 percent) patients;
- defects in intrinsic and innate immunity: four (12 percent) patients;
- immune dysregulation: six (18 percent) patients;
- autoinflammatory disorders: one patient; and
- predominantly antibody deficiency: one patient.

Five patients did not have any IEI. Average ages of admission included 43.6 months overall, 41.6 months for patients with an IEI and 55 months for patients who did not have an IEI. Other average ages of admission included:

- CID: 36.8 months;
- SCID: 4.5 months;
- CIDs with associated or syndromic features: 60.3 months;
- predominantly antibody deficiency: 120 months;
- disease of immune dysregulation: 38.7 months;
- congenital defects of phagocyte: 51.5 months;
- defects in intrinsic and innate immunity: 10.5 months; and
- autoinflammatory disorders: 29 months.

Further, the researchers identified pathogenic variants in 26 different genes, including 25 genes associated with IEI in the International Union of Immunological Societies 2022 classification. The remaining gene was not associated with IEI.

Based on these findings, the researchers concluded that whole-exome sequencing had high diagnostic value for detecting IEI in children with a history of recurrent infections or a family history of IEI who were admitted to the ICU for CAS. However, the researchers noted that the high proportion of patients from consanguineous marriages, which are more prevalent in Iran than in Western countries, may have contributed to the high rates of IEI diagnosis in this cohort.

The researchers called for additional studies with larger, multicentric patient populations to confirm these findings, with an emphasis on cost-effectiveness to support its use in low-resource settings, along with other genetic testing approaches, including whole-genome sequencing and targeted gene panel testing.

Gawel, W. Whole Exome Sequencing Finds Inborn Errors of Immunity in Children with Sepsis. Healio, Feb. 21, 2024. Accessed at www.healio.com/news/allergy-asthma/20240221/whole-exome-sequencing-finds-inborn-errors-of-immunity-in-children-with-sepsis.

INDICATIONS AND USAGE

PANZYGA (Immune Globulin Intravenous [Human] – ifas) is indicated for the treatment of primary humoral immunodeficiency (PI) in patients 2 years of age and older, chronic immune thrombocytopenia (cITP) in adults and chronic inflammatory demyelinating polyneuropathy (CIDP) in adults.

PANZYGA is a liquid medicine for infusion that contains immunoglobulin G (IgG), which are proteins that help fight infection. It is made from human plasma that is donated by healthy people and contains antibodies. For patients with PI, PANZYGA helps replace the missing antibodies in the body. For patients with cITP, PANZYGA helps the body produce more platelets (the blood cells that help blood clot) to control or prevent bleeding. For patients with CIDP, PANZYGA may help improve mobility and hand strength.

PANZYGA is given into a vein (intravenously) in a hospital, infusion center, doctor's office, or at home by a trained healthcare provider (HCP).

IMPORTANT SAFETY INFORMATION

WARNING: THROMBOSIS, RENAL DYSFUNCTION, and ACUTE RENAL FAILURE

See full prescribing information for complete BOXED WARNING

- **Thrombosis may occur with immune globulin intravenous (IGIV) products, including PANZYGA. 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.**
- **Renal dysfunction, acute renal failure, osmotic nephropathy, and death may occur with the administration of IGIV products in predisposed patients. Renal dysfunction and acute renal failure occur more commonly in patients receiving IGIV products containing sucrose. PANZYGA does not contain sucrose.**
- **For patients at risk of thrombosis, renal dysfunction, or acute renal failure, administer PANZYGA at the minimum 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.**

Do not use PANZYGA if you:

- Have had a severe allergic reaction to immune globulin or other blood products
- Have a condition called selective (or severe) immunoglobulin A (IgA) deficiency, with antibodies against IgA and a history of hypersensitivity

What should I know before taking PANZYGA?

- PANZYGA can make vaccines (like measles/mumps/rubella or chickenpox vaccines) work less effectively for you. Before you get any vaccines, tell your healthcare provider that you take PANZYGA
- Decreased kidney function and kidney function failure can occur
- Severe headache, drowsiness, fever, painful eye movements, or nausea and vomiting can occur
- Elevated blood pressure can occur particularly in patients who have a history of hypertension (high blood pressure)
- If you are elderly, with heart or kidney problems, discuss with your healthcare provider prior to initiating treatment with PANZYGA
- PANZYGA is made from human blood and therefore may have a risk of transmitting infectious agents, including viruses and, theoretically, the variant Creutzfeldt-Jakob disease (CJD) and CJD agent. The production and manufacturing process reduces this risk, but the risk cannot be eliminated

PANZYGA can cause serious side effects. If any of the following problems occur after starting PANZYGA, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting, or dizziness. These could be signs of a serious allergic reaction
- Bad headache with nausea, vomiting, stiff neck, fever, drowsiness, painful eye movements, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain

Please see Important Safety Information on this and adjacent page of this advertisement and Brief Summary of Prescribing Information.

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  - Patients must have commercial insurance to be eligible
  - Patients are not eligible if they are enrolled in a state or federally funded insurance program

*Depending on the ongoing therapy dose.
†Eligible, commercially insured patients may pay as little as $0 for PANZYGA and may receive a maximum benefit of $12,500 per year or the cost of patient's co-pay in a 12-month period (whichever is less) for claims received by the program. Terms and conditions/eligibility requirements apply. See full Terms and Conditions at PanzygaCoPay.com.

Talk to your doctor about PANZYGA and learn more at PanzygaInfo.com

IMPORTANT SAFETY INFORMATION (continued)

- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem (decreased kidney function or kidney failure)
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot, which could happen in the heart, brain, lungs, or elsewhere in the body
- Brown or red urine, swelling, fatigue, fast heart rate, difficulty breathing, or yellow skin or eyes. These could be signs of a liver or blood problem
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem
- Fever over 100°F. This could be a sign of an infection
- Headache, fatigue or confusion, vision problem, chest pain, difficulty breathing, irregular heartbeat, or pounding in your chest, neck, or ears. These could be signs of high blood pressure

Ask your HCP whether you should have rescue medications available, such as antihistamines or epinephrine.

What are the possible or reasonably likely side effects for PANZYGA?

The most common side effects that may occur with PANZYGA are:

- Headache
- Nausea
- Fever
- Increased blood pressure
- Dermatitis
- Fatigue
- Abdominal pain
- Dizziness
- Anemia

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away.

Tell your HCP if you are pregnant, or plan to become pregnant, or if you are nursing.

Patients should always ask their doctors for medical advice about adverse events.

You may report an adverse event related to Pfizer products by calling 1-800-438-1985 (US only). If you prefer, you may contact the U.S. Food & Drug Administration (FDA) directly. The FDA has established a reporting service known as MedWatch where healthcare professionals and consumers can report problems they suspect may be associated with the drugs and medical devices they prescribe, dispense, or use. Visit www.fda.gov/MedWatch or call 1-800-FDA-1088.

PANZYGA is FDA approved for 3 indications:

- CIDP in adults
- PI in patients 2 years of age or older
- cITP in adults

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Manufactured by Octapharma Pharmazeutika Produktionsges m.b.H. Distributed by Pfizer Labs, Division of Pfizer inc.

PP-PAN-USA-0927

April 2024

panzyga®
Immune Globulin
Intravenous (Human) - ifas
10% Liquid Preparation

CONSUMER BRIEF SUMMARY

(PANZYGA: *pan-zee-guh*)

This brief summary highlights the most important information about PANZYGA. Please read it carefully before using PANZYGA and each time you have an infusion, as there may be new information. This brief summary does not take the place of talking with your healthcare provider about your medical condition or your treatment. If you have any questions after reading this, ask your healthcare provider. For more information, go to www.PanzygaInfo.com.

What is PANZYGA?

PANZYGA is a liquid medicine for infusion that contains immunoglobulin G (IgG), which are proteins that help fight infection. PANZYGA is used to treat primary humoral immunodeficiency (PI) in patients 2 years of age and older, chronic immune thrombocytopenia (cITP) in adults, and chronic inflammatory demyelinating polyneuropathy (CIDP) in adults.

PANZYGA is made from human plasma that is donated by healthy people and contains antibodies. For patients with PI, PANZYGA helps replace the missing antibodies in the body. For patients with cITP, PANZYGA helps the body produce more platelets (the blood cells that help blood clot) to control or prevent bleeding. For patients with CIDP, PANZYGA may help improve mobility and hand strength.

PANZYGA is given into a vein (intravenously) in a hospital, infusion center, doctor's office, or at home by a trained healthcare provider (HCP).

WARNING: THROMBOSIS, RENAL DYSFUNCTION, and ACUTE RENAL FAILURE

See full prescribing information for complete BOXED WARNING

- **Thrombosis may occur with immune globulin intravenous (IGIV) products, including PANZYGA. 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.**
- **Renal dysfunction, acute renal failure, osmotic nephropathy, and death may occur with the administration of IGIV products in predisposed patients. Renal dysfunction and acute renal failure occur more commonly in patients receiving IGIV products containing sucrose. PANZYGA does not contain sucrose.**
- **For patients at risk of thrombosis, renal dysfunction, or acute renal failure, administer PANZYGA at the minimum 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.**

Who should NOT use PANZYGA?

Tell your healthcare provider if you:

- Have had a severe allergic reaction to immune globulin or other blood products
- Have a condition called selective (or severe) immunoglobulin A (IgA) deficiency, with antibodies against IgA and a history of hypersensitivity

PANZYGA can cause serious side effects. If any of the following problems occur after starting PANZYGA, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting, or dizziness. These could be signs of a serious allergic reaction
- Bad headache with nausea, vomiting, stiff neck, fever, drowsiness, painful eye movements, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem (decreased kidney function or kidney failure)
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot, which could happen in the heart, brain, lungs, or elsewhere in the body
- Brown or red urine, swelling, fatigue, fast heart rate, difficulty breathing, or yellow skin or eyes. These could be signs of a liver or blood problem
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem
- Fever over 100°F. This could be a sign of an infection
- Headache, fatigue or confusion, vision problem, chest pain, difficulty breathing, irregular heartbeat, or pounding in your chest, neck, or ears. These could be signs of high blood pressure

Ask your HCP whether you should have rescue medications available, such as antihistamines or epinephrine.

What should I know before taking PANZYGA?

- PANZYGA can make vaccines (like measles/mumps/rubella or chickenpox vaccines) work less effectively for you. Before you get any vaccines, tell your healthcare provider that you take PANZYGA
- Decreased kidney function and kidney function failure can occur
- Severe headache, drowsiness, fever, painful eye movements, or nausea and vomiting can occur
- Elevated blood pressure can occur particularly in patients who have a history of hypertension (high blood pressure)
- If you are elderly, with heart or kidney problems, discuss with your healthcare provider prior to initiating treatment with PANZYGA
- PANZYGA is made from human blood and therefore may have a risk of transmitting infectious agents, including viruses and, theoretically, the variant Creutzfeldt-Jakob disease (CJD) and CJD agent. The production and manufacturing process reduces this risk, but the risk cannot be eliminated

What are the possible or reasonably likely side effects for PANZYGA?

The most common side effects that may occur with PANZYGA are:

- Headache
- Nausea
- Fever
- Increased blood pressure
- Dermatitis
- Fatigue
- Abdominal pain
- Dizziness
- Anemia

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away. Tell your HCP if you are pregnant, or plan to become pregnant, or if you are nursing. If you encounter any problems or experience side effects during or after the infusion, contact your healthcare provider. When doing so, keep your therapy tracker with you to be able to give all necessary information.

Patients should always ask their doctors for medical advice about adverse events.

You may report an adverse event related to Pfizer products by calling 1-800-438-1985 (US only). If you prefer, you may contact the US Food and Drug Administration (FDA) directly. The FDA has established a reporting service known as MedWatch where healthcare professionals and consumers can report problems they suspect may be associated with the drugs and medical devices they prescribe, dispense, or use. Visit www.fda.gov/MedWatch or call 1-800-FDA-1088.

This brief summary is based on the PANZYGA Prescribing Information (February 2021).

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MEDICINES

FDA Approves Extended Room Temperature Storage Conditions for ASCENIV and BIVIGAM

The U.S. Food and Drug Administration has approved ADMA Biologics' supplemental biologics license applications for both ASCENIV and BIVIGAM to extend the approved four-week room temperature (25 degrees Celsius) storage conditions during the first 24 months of shelf life, to allow for a four-week room temperature storage at any time during the entire 36-month approved shelf life. The room temperature approval applies to all existing ASCENIV and BIVIGAM lots currently in the commercial supply chain, as well as to future production of ASCENIV and BIVIGAM. The new approval is effective immediately.

"With the FDA-approved extension of room temperature storage conditions, the company expects to reach more customers who were previously inaccessible due to limited refrigeration space and cold chain capacity constraints," said Adam Grossman, president and chief executive officer of ADMA. "We believe that this added storage flexibility for both ASCENIV and BIVIGAM will meaningfully enhance our products' market offerings, enabling more versatile utilization and better inventory management for providers." IG

ADMA Biologics Announces FDA Approvals of Extended Room Temperature Storage Conditions for ASCENIV and BIVIGAM. ADMA Biologics press release, March 11, 2024. Accessed at www.biospace.com/article/releases/adma-biologics-announces-fda-approvals-of-extended-room-temperature-storage-conditions-for-asceniv-and-bivigam-.

RESEARCH

Newly Discovered Genetic Disorder Causes Immunodeficiency

Researchers have discovered a new genetic disorder that causes immunodeficiency and profound susceptibility to opportunistic infections, including a life-threatening fungal pneumonia.

In-born errors of immunity (IEIs), also known as primary immunodeficiencies, are genetic defects characterized by increased susceptibility to infectious diseases, autoimmunity, anti-inflammatory disorders, allergies and, in some cases, cancer. To date, 485 different IEIs have been identified. It is now thought that they occur in one of every 1,000 to 5,000 births, making them as prevalent as other genetic disorders, including cystic fibrosis and Duchene's muscular dystrophy.

In their study, a consortium of IRF4 experts identified seven patients from six unrelated families across four continents with profound combination immunodeficiency who experienced recurrent and serious infections, including pneumonia caused by the fungus Pneumocystis jirovecii. Each patient had the same mutation in the DNA-binding domain of IRF4. (IRF4 is a transcription factor that is pivotal for the development and function of B and T white blood cells, as well as other immune cells.)

They then conducted extensive phenotyping of patients' blood cells, which revealed immune cell abnormalities associated with the disease, including impaired maturation of antibody-producing B cells and reduced T cell production of infection-fighting cytokines. Two knock-in mouse models, in which the mutation was inserted into the mouse genome, exhibited a severe defect in antibody production consistent with the combined immune deficiency observed in the patients.

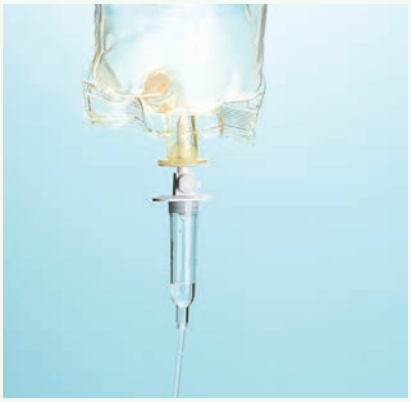
The researchers also discovered the mutation had a "multimorphic" effect detrimental to the activation and differentiation of immune cells. While the mutant IRF4 binds to DNA with a higher affinity than the native form of the protein (in a hypermorphic way), its transcriptional activity in common, canonical genes is reduced (hypomorphic), and it binds to other DNA sites (in a neomorphic way), altering the protein's normal gene expression profile. This multimorphic activity is a new mechanism for human disease. "We anticipate that variants with multimorphic activity may be more widespread in health and disease," the researchers concluded.

The researchers hope their discovery will help identify people who carry this IEI. "Our findings will provide the basis for genetic diagnosis and preventive treatment for these groups of patients," said Rubén Martínez-Barricarte, PhD, an immunogeneticist at Vanderbilt University Medical Center. IG

Genetic Disorder That Causes Immunodeficiency and Susceptibility to Opportunistic Infections Discovered. News Medical Life Sciences, Jan. 23, 2023. Accessed at www.news-medical.net/news/20230123/Genetic-disorder-that-causes-immunodeficiency-and-susceptibility-to-opportunistic-infections-discovered.aspx.

RESEARCH

Groundbreaking Study Demonstrates 20-Year Remission in Pemphigus Vulgaris Patients Treated with IVIG



Research published in the Proceedings of the National Academy of Sciences of the United States of America (PNAS) offers hope for long-term remission in patients with pemphigus vulgaris (PV), a potentially fatal autoimmune blistering disease. Researchers from Tufts University School of Medicine in Boston, Mass., and Sorbonne Université in Paris, France, have demonstrated a 20-year clinical and serological remission in patients treated with intravenous immune globulin (IVIG) therapy.

Twenty-one patients with confirmed PV received IVIG therapy according to a specific protocol. Twelve patients (57 percent) experienced complete remission with no relapses for 20 years. Six patients (29 percent) experienced transient relapses, which were successfully controlled with additional IVIG treatment. No severe adverse events from IVIG were observed in any patients. According to the researchers, this long-term remission suggests that IVIG may help restore immune balance and tolerance in PV patients.

"This study offers compelling evidence for the potential of IVIG as a transformative treatment for PV," stated lead researcher A. Razzaque Ahmed, MD, DSc, MPA, FRCP (Edin), professor of dermatology at Tufts University School of Medicine and director of the Center for Blistering Diseases. "The observed 20-year remission without further medication suggests a profound impact on the underlying immune dysregulation, paving the way for further research into immune tolerance mechanisms in autoimmune diseases." IG

Remission in Pemphigus Vulgaris Patients Using IVIG. Globe Newswire, Jan. 31, 2024. Accessed at www.bakersfield.com/ap/news/groundbreaking-study-demonstrates-20-year-remission-in-pemphigus-vulgaris-patients-using-ivig/article_8df45659-4623-5028-903a-ef081fc94a26.html.

MEDICINES

FDA Approves Acthar Gel SelfJect Injector to Treat Autoimmune, Inflammatory Conditions

The U.S. Food and Drug Administration (FDA) has approved Mallinckrodt Pharmaceutical's supplemental new drug application for Acthar Gel injector for the treatment of several chronic and acute inflammatory and autoimmune conditions.

Expected to launch in the United States in the second half of 2024, the Acthar Gel (corticotropin) single-dose pre-filled SelfJect injector is indicated in acute exacerbations of multiple sclerosis; selected cases of systemic lupus erythematosus and polymyositis; as an adjunctive therapy for psoriatic arthritis, rheumatoid arthritis and ankylosing spondylitis; as well as other conditions.

"We're excited to bring this innovation to U.S. patients with chronic and acute inflammatory and autoimmune conditions," said Peter Richardson, MRCP, executive vice president and chief scientific officer at Mallinckrodt. "This approval reflects Mallinckrodt's long-standing commitment to clinical research and therapeutic modernization efforts providing a new delivery device for patients, caregivers and medical professionals managing these challenging conditions."

The SelfJect injector is prefilled with a prescribed dose for subcutaneous injection, available in 40 units or 80 units, and features a hidden needle to protect against needlestick injury. Mallinckrodt said it will continue to make corticotropin available as a vial-and-syringe injection.

Corticotropin is a "naturally sourced complex mixture of adrenocorticotropic hormone analogs and other pituitary peptides," according to the release. IG

FDA Approves Acthar Gel SelfJect Injector in Autoimmune, Inflammatory Conditions. Mallinckrodt press release, March 1, 2024. Accessed at www.healio.com/news/rheumatology/20240301/fda-approves-acthar-gel-selfject-injector-in-autoimmune-inflammatory-conditions.

RESEARCH

Reengineered Immune Cells Show Promise for Treating Lupus Nephritis and Other Autoimmune Diseases

A new study showed there may be a way to reengineer defective cells that cause lupus nephritis (LN). Researchers hope this new method can lead to a targeted therapy for people living with LN, as well as other autoimmune diseases.

In people with lupus and other autoimmune diseases, the cells that are essential for maintaining immune system balance, regulatory T cells (Tregs), are diminished. Using blood samples from study participants, researchers identified specific protective molecules from people without lupus or autoimmune disease, then reprogrammed the Tregs from people

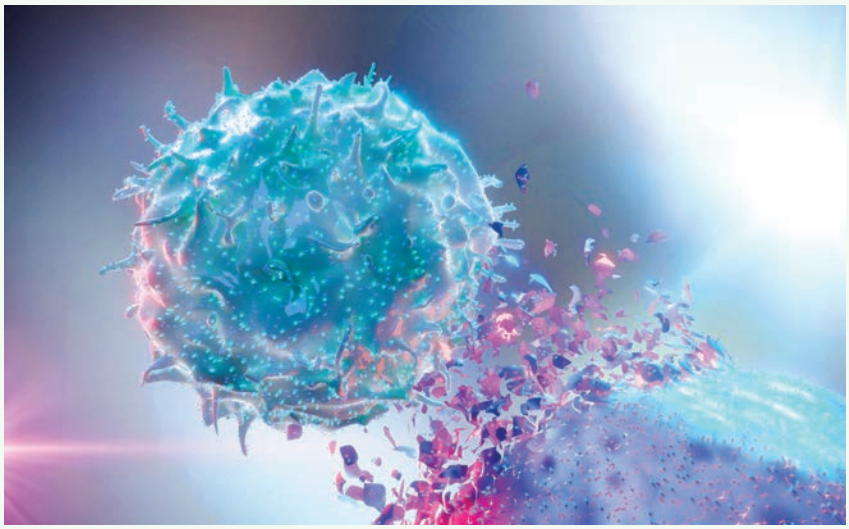
with lupus to specifically target the "Smith protein," which is strongly associated with LN. People with LN typically have antibodies that mistakenly target their own body; however, the reengineered Tregs were able to restore the patients' ability to suppress their overactive immune system. Notably, this approach was effective in preventing the development of LN without the use of immunosuppressant drugs.

A Phase I clinical trial for people with LN is expected to start in 2026, but this promising research illustrates the potential for a new treatment option that does not involve the use of immunosuppressant drugs for many people living with autoimmune diseases. IG

Reengineered Immune Cells Could Offer a Potential Treatment for People with Lupus Nephritis and Other Autoimmune Diseases. Lupus Foundation of America, Feb. 16, 2024. Accessed at www.lupus.org/news/reengineered-immune-cells-could-offer-a-potential-treatment-for-people-with-lupus-nephritis.

RESEARCH

CAR T-Cell Therapy Brings Sustained, Drug-Free Remission in Three Autoimmune Diseases



Single injections of CD19 chimeric antigen receptor T-cell therapy brought long-lasting remission to 15 patients with three different autoimmune diseases, according to data published in *The New England Journal of Medicine*.

"Although antibody-based B-cell targeting certainly improved treatment of autoimmune disease, achieving long-lasting drug-free remission has proven elusive," Fabian Müller, MD, of the University of Erlangen-Nuremberg, in Germany, and colleagues wrote. "CAR T cells could potentially achieve this goal by deep depletion of B cells through the targeting of the surface molecule CD19, which is expressed on a wide spectrum of B cells and plasmablasts."

To analyze their safety and efficacy, Dr. Müller and colleagues used CD19 CAR T cells to treat eight patients with systemic lupus erythematosus (SLE), three with idiopathic inflammatory myositis and four with systemic sclerosis. All participants had previously demonstrated inadequate responses to at least two previous immunosuppressive treatments. CAR T-cell therapy was administered between February 2021 and May 2023. All patients additionally received lymphodepleting chemotherapy with fludarabine and cyclophosphamide.

Each patient's disease activity was monitored every three months for periods lasting between four months and 29 months. More than 1×10^9 CD19 CAR T cells could be produced in each patient, with transduction efficiencies ranging between 18 percent and 44 percent. Following injection, CAR T-cell expansion and clearance of B cells were "highly consistent" across the patients.

According to the researchers, after six months, all patients with SLE met lupus low disease activity state criteria; had entered remission, according to definition of remission in SLE (DORIS) criteria; and had a score of zero on the SLE disease activity index 2K. All patients remained free of SLE disease activity across follow-up periods ranging from six months to 29 months.

At three months, the three patients with idiopathic inflammatory myositis demonstrated normalized creatine kinase levels and "major clinical response" based on American College of Rheumatology-EULAR score, the researchers wrote. Meanwhile, all four patients with systemic sclerosis had reduced global disease activity, according to the European Scleroderma Trials and Research Group activity index, as well as diminished skin activity as measured by modified Rodnan skin score. Every patient was able to discontinue all immunosuppressive medications, including glucocorticoids, as of final follow-up (median, 15 months).

Overall, adverse effects were "minimal," Dr. Müller and colleagues wrote. Recorded infections were mostly mild and in the upper respiratory tract, while one patient with SLE developed pneumonia seven weeks after therapy that resolved with antibiotics. There were no prolonged toxic effects in bone marrow and no moderate- or high-grade instances of cytokine release syndrome or immune effector cell-associated neurotoxicity syndrome.

While the researchers noted "it cannot be ruled out" that chemotherapy contributed to the short-term effects of CD19 CAR T-cell treatment, they wrote that it was unlikely to have induced the "complete B-cell depletion, abrogation of autoantibodies and sustained drug-free remission." They called for controlled clinical studies to investigate further. IG

Cooper, J. CAR T-Cell Therapy Brings Sustained, Drug-Free Remission in Three Autoimmune Diseases. Healio, Feb. 28, 2024. Accessed at www.healio.com/news/rheumatology/20240228/car-tcell-therapy-brings-sustained-drugfree-remission-in-three-autoimmune-diseases.

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- ☑ Strategies to reduce the risk of side effects
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Is Advocacy Right for Me?
Letting Your Voice Be Heard

There are many paths to advocacy — from education to digital platforms and even political activism — that can empower you to take control of your healthcare and advocate for others.

By Abbie Cornett, MBA

A CHRONIC ILLNESS diagnosis marks the beginning of a journey that often feels like navigating through uncharted territory. The complexities and uncertainties inherent in managing a chronic condition can be overwhelming, leaving individuals and their families grappling with a myriad of emotions and challenges. Yet, amid this turmoil, advocacy emerges as a powerful tool for patient empowerment.

Before delving into how advocacy can support you and your family facing chronic illness post-diagnosis, it's important to understand the concept of healthcare advocacy. At its core, healthcare advocacy involves advocating for personalized treatment plans, accessing necessary resources and addressing barriers to care.[1] It encompasses a range of actions, including self-advocacy, where you assert your own

needs and preferences, as well as advocacy on behalf of others or engaging in broader community advocacy efforts.

Healthcare advocacy empowers you and your family to become active participants in your healthcare journey, enabling you to make informed decisions and advocate for your rights within healthcare systems.

The Role of Self-Advocacy

When living with a chronic illness or disability, you need to be able to advocate for yourself to receive the care and resources you require.[2] In fact, advocating for yourself can be a pivotal component of your success in managing your illness. Self-advocacy empowers you and allows you to assert you needs, preferences and rights within healthcare settings.

This proactive role in your own care extends to all areas, including interactions with physicians, employers, hospitals, pharmacies and disability resource offices. Successful self-advocacy ensures needs are identified, personal goals are set and concerns are effectively communicated with healthcare providers. Moreover, it fosters self-confidence, facilitates informed healthcare decisions and empowers you to take control of your ongoing care, resulting in better outcomes.

What Does Effective Self-Advocacy Entail?

Effectively advocating for yourself in healthcare involves several essential elements that empower you to confidently navigate the healthcare system and ensure your needs are addressed. Self-advocacy in healthcare means taking an active role in managing your own healthcare. Here are some critical aspects of effective self-advocacy:

Education. Education is pivotal for empowering you to navigate your healthcare journey effectively. By familiarizing yourself with your disease, including symptoms, treatments and potential complications, you lay the foundation for informed decision-making and proactive engagement with healthcare providers. This knowledge serves as a powerful tool for enhancing communication between you and your healthcare team.

When you are equipped with information about your condition, you can articulate your concerns, preferences and treatment goals more effectively. This facilitates the development of personalized treatment plans that align with your unique needs and preferences, ultimately enhancing your satisfaction with your care. Moreover, understanding the medical diagnosis and its implications diminishes fear, confusion, uncertainty and anxiety, fostering a sense of empowerment.[3]

Furthermore, knowledge empowers you to self-manage your care. By understanding the potential complications associated with your condition, you can take proactive steps to mitigate risks and optimize your health outcomes.[4] This may involve adhering to prescribed treatment regimens, making lifestyle modifications and seeking timely medical intervention when necessary.[3]

Communication skills. In the past, communication between physicians and patients followed a one-directional model. The physician would dictate the agenda, define health objectives and make all medical decisions, while patients were typically expected to passively comply with their doctors' orders.[5] Fortunately, this outdated model has evolved, with patients now taking a more active role in their healthcare decisions. Effective communication with the care team is crucial for all patients, particularly those managing chronic conditions. Here are five key strategies through which you can advocate for yourself:

1) Express concerns and preferences: Feel empowered to express your healthcare concerns, preferences and goals. Clear and open communication with the care team is essential, whether it's discussing treatment options,

expressing fears or uncertainties or communicating personal preferences.

2) Ask questions and seek clarification: Actively engage with your care team by asking questions about your condition, treatment plans and any other aspects of your care that you may not fully understand. Asking questions helps you gain clarity, make informed decisions and actively participate in your healthcare journey.[6]

3) Share relevant information: Provide your care team with relevant information about your medical history, symptoms, lifestyle and any changes in your condition. Openly sharing information helps the care team make accurate diagnoses, tailor treatment plans to you and address specific concerns or needs.[3]

4) Be proactive in decision-making: Take an active role in decisions about your healthcare. This involves being informed about treatment options, weighing the risks and benefits, and collaborating with the care team to develop a plan that aligns with your preferences and goals.

5) Advocate for needs and rights: Advocate for your needs and rights within the healthcare system. This may include advocating for timely appointments, access to necessary treatments or medications, accommodations for individual preferences or circumstances, and respectful and compassionate care from the care team.

Educating yourself about your health condition and learning to effectively communicate with healthcare providers are essential aspects of self-advocacy in healthcare.

Setting self-management goals. Educating yourself about your health condition and learning to effectively communicate with healthcare providers are essential aspects of self-advocacy in healthcare. However, setting goals is equally integral to this process. Setting goals enables you to take ownership of your health and well-being by identifying areas where improvements can be made or desired outcomes can be achieved. These goals can range from managing symptoms and adhering to treatment plans to adopting healthier lifestyles and addressing emotional well-being. By setting goals, you can establish clear objectives, track your progress and take proactive steps toward achieving optimal health outcomes.

A good starting point when setting goals is the acronym SMART, which stands for specific, measurable, attainable, realistic and time-based.[7]

- Specific: Start by addressing the five W's — who, what, when, where and why. This detailed approach clarifies the desired outcome, providing a clear understanding of the plan to be achieved.
- Measurable: Identify measurable indicators to gauge progress toward your goals. Whether tracking improvements in health metrics or adherence to treatment plans, quantifiable measures ensure tangible progress can be observed.
- Attainable: Consider the resources and support available to work toward goals. Assess whether you have the necessary tools, knowledge and assistance to accomplish your objectives realistically.
- Realistic: While you may aspire to ambitious goals, it's crucial to set expectations aligned with your capabilities and circumstances. Realistic goals ensure you stay motivated and avoid overwhelm or discouragement.
- Time-based: Establishing a time frame provides structure and urgency to the goal-setting process. Allocate specific deadlines for achieving milestones, breaking down long-term objectives into manageable steps within days, weeks or months.

Let Your Voice Be Heard

In addition to self-advocacy, you can engage in various avenues to advocate for yourself and others with chronic illnesses, including political involvement, advocating for policy changes regarding access to treatment reimbursement, and leveraging social media platforms. Political involvement encompasses a range of activities, from voting for policymakers who prioritize healthcare to actively engaging in advocacy campaigns and contacting elected officials regarding legislation that affects access to treatment and reimbursement policies.

If you are interested in taking a more active role, you can take advantage of patient activism toolkits provided by

various chronic disease groups. These toolkits are designed to empower patients. They typically include resources such as guides to writing impactful letters to newspapers, contact information for legislators and decision-makers in the local area, and information on how to participate in legislative lobbying days at both the state and federal levels. By utilizing these toolkits, you can let your voice be heard, raise awareness about chronic illnesses and advocate for policy changes that improve access to treatment and reimbursement.[8]

Digital Advocacy

If you are not comfortable with or unable to engage in in-person advocacy due to health or personal reasons, social media provides an excellent alternative. Through online platforms such as Facebook, Twitter and Instagram, you can overcome physical limitations and geographical barriers to participate in advocacy efforts from the comfort of your own home. This accessibility proves especially valuable for those with chronic illnesses or disabilities who may encounter difficulties attending in-person events or meetings. By leveraging social media platforms, you can still effectively raise awareness, share experiences and advocate for policy changes concerning chronic diseases.[4]

Participating in online advocacy efforts can take various forms, including starting your own social media page and engaging with existing pages and communities. Starting your own page dedicated to raising awareness about a specific chronic illness allows you to create a platform where you can share information, personal experiences and resources related to the condition. This can attract like-minded individuals and help amplify your advocacy efforts.

The Role of Patient Advocates in Healthcare Advocacy

When patients and their families find themselves unable to advocate effectively, whether due to health challenges or other constraints, the assistance of a patient advocate becomes invaluable. Patient advocates serve as dedicated allies, representing patients' interests and ensuring effective communication within the healthcare system. Most advocates have been trained to navigate complex medical processes, advocate for appropriate care and facilitate communication among patients, families and healthcare providers. Whether addressing reimbursement issues or handling intricate situations, patient advocates uphold the patients' rights and well-being.

In situations involving reimbursement challenges, patient advocates can play a crucial role in advocating for fair and timely reimbursement for medical expenses. They know how to navigate insurance policies, appeal denials and negotiate with payers to ensure patients receive the financial support to which they are entitled. The involvement of a patient advocate not only alleviates the burden on you and your family but also enhances your ability to navigate complex healthcare processes.

Advocacy Is a Formidable Tool

In summary, advocacy can be a formidable tool for empowerment within the healthcare sphere, particularly for those contending with chronic illnesses. Through self-advocacy, leveraging digital platforms and seeking support from patient advocates, you can confidently navigate the intricacies of the healthcare system. By arming yourself with knowledge, setting objectives and effectively communicating your needs, you can assert your independence and strive for optimal health outcomes. Additionally, participation in political activism and utilizing social media platforms provide avenues for raising awareness and advocating for policy changes that enhance access to treatment and reimbursement equity. When faced with barriers to advocacy, the guidance and assistance of patient advocates are crucial in ensuring that patient voices are not only heard but also respected. Ultimately, by embracing advocacy and actively engaging in your healthcare journey, you can contribute to shaping a healthcare environment that prioritizes patient well-being and fosters inclusivity for all. IG

References

1. Hubinette, M, Dobson, S, Scott, I, and Sherbino, J. Health Advocacy. *Medical Teacher*, Vol. 39, 2017. Accessed at www.tandfonline.com/doi/full/10.1080/0142159X.2017.1245853.
2. PainScale. Why Is Self-Advocacy Important? Accessed at www.painscale.com/article/why-is-self-advocacy-important.
3. Global Autoimmune Institute. Effective Communication Strategies for the Doctor's Office for Patients with Autoimmune Disease, Jan. 10, 2024. Accessed at www.autoimmuneinstitute.org/articles/diagnosis-and-treatment/effective-communication-strategies-for-patients-with-ads.
4. Thomas, EN, Edwards, L, and McArdle, P. Knowledge Is Power. A Quality Improvement Project to Increase Patient Understanding of Their Hospital Stay. *BMJ Quality Improvement Reports*, v6(1); 2017. Accessed at www.ncbi.nlm.nih.gov/pmc/articles/PMC5337670.
5. Boxer, H, and Snyder, S. Five Communication Strategies to Promote Self-Management of Chronic Illness. *Family Practice Management*, 2009;16(5):12-16. Accessed at www.aafp.org/pubs/fpm/issues/2009/0900/p12.html.
6. DeSanto, L. Can You Hear Me Now? Health Central, updated Oct. 3, 2023. Accessed at www.healthcentral.com/chronic-health/how-to-communicate-better-with-your-doctor.
7. Morris, S. Setting Goals and Managing Expectations for Chronic Illness. *IG Living*, February-March 2020. Accessed at www.igliving.com/magazine/articles/IGL_2020-02_AR_Setting-Goals-and-Managing-Expectations-for-Chronic-Illness.pdf.
8. Chronic Disease Coalition. Five Resources for Becoming a Chronic Disease Patient Advocate, April 29, 2016. Accessed at chronicdiseasecoalition.org/news/five-resources-for-becoming-a-chronic-disease-patient-advocate.

ABBIE CORNETT, MBA, is the patient advocate for *IG Living* magazine.

gamunex®-C
immune globulin injection (human), 10% caprylate/chromatography purified

Step out of the symptoms of CIDP and back into your life with GAMUNEX-C

GAMUNEX-C helps your body fight the inflammation of CIDP* in multiple ways to protect your nerves from damage.[1-3]

GAMUNEX®-C (immune globulin injection [human], 10% caprylate/chromatography purified) is approved to treat primary humoral immunodeficiency disease (PIDD) in patients 2 years of age and older. If you have PIDD, you may take GAMUNEX-C under the skin (subcutaneously) or in a vein (intravenously). GAMUNEX-C is also approved to treat idiopathic thrombocytopenic purpura (ITP) in adults and children and chronic inflammatory demyelinating polyneuropathy (CIDP) in adults. If you have ITP or CIDP, you may only take GAMUNEX-C intravenously.

Do not take GAMUNEX-C if you have an allergy to immune globulin. Tell your doctor if you have had a serious reaction to other medicines that contain human immune globulin. Also tell your doctor if you have immunoglobulin A (IgA) deficiency. If you have a serious reaction while taking GAMUNEX-C, stop taking it immediately and tell your doctor.

*Chronic inflammatory demyelinating polyneuropathy.

Please see Important Safety Information for GAMUNEX-C on the following page.

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Learn more at GAMUNEX-C.com or call 1-888-MYGAMUNEX (1-888-694-2686)

IMPORTANT SAFETY INFORMATION

GAMUNEX®-C (immune globulin injection [human], 10% caprylate/chromatography purified) is approved to treat primary humoral immunodeficiency disease (PIDD) in patients 2 years of age and older. If you have PIDD, you may take GAMUNEX-C under the skin (subcutaneously) or in a vein (intravenously). GAMUNEX-C is also approved to treat idiopathic thrombocytopenic purpura (ITP) in adults and children and chronic inflammatory demyelinating polyneuropathy (CIDP) in adults. If you have ITP or CIDP, you may only take GAMUNEX-C intravenously.

If you take GAMUNEX-C or a similar immune globulin product, you could experience a serious and life-threatening blood clot (thromboembolism), which may include pain and/or swelling of an arm or leg with warmth over the affected area, discoloration of an arm or leg, unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, numbness, or weakness on one side of the body. You are more likely to develop a blood clot if you have a history of hardening of the arteries (atherosclerosis), stroke, heart attack, or heart failure (low volume of blood pumped by the heart). You may also be more likely to get a blood clot if you are elderly, if you have a blood clotting disorder, if you are inactive for long periods of time (such as long bed rest), if you use estrogens, or if you have thickening of your blood. For patients at risk, GAMUNEX-C should be administered at the lowest dose and slowest infusion rate that is practical. However, blood clots may occur in the absence of any of the known risk factors. Patients should be well hydrated by drinking enough water before GAMUNEX-C is administered. Tell your doctor immediately if your medical history is similar to what is described here, and especially if you start having any of these symptoms while taking GAMUNEX-C.

If you take GAMUNEX-C or a similar immune globulin product intravenously, you could experience serious kidney disease and death. You may have symptoms of decreased urination, sudden weight gain, swelling in your legs (edema), or shortness of breath. You are more likely to develop serious kidney disease if you already have a kidney problem, have Type II diabetes mellitus, or are older than 65. You are more likely to develop serious kidney disease if you are dehydrated, have a blood infection (sepsis), have high protein content in your blood, or if you are receiving other medicines that are harmful to your kidneys. Tell your doctor immediately if your medical history is similar to what is described here, and especially if you start having any of these symptoms while taking GAMUNEX-C.

You are more likely to develop serious kidney disease if you take an intravenous immune globulin product that contains sugar (sucrose). GAMUNEX-C does not contain sugar. If your situation makes you more likely to experience serious kidney disease, you should take GAMUNEX-C at the lowest concentration available and the slowest infusion rate that is practical.

Do not take GAMUNEX-C if you have an allergy to immune globulin. Tell your doctor if you have had a serious reaction to other medicines that contain human immune globulin. Also tell your doctor if you have immunoglobulin A (IgA) deficiency. If you have a serious reaction while taking GAMUNEX-C, stop taking it immediately and tell your doctor.

Periodic monitoring of kidney function and urine output is particularly important in patients more likely to experience severe kidney disease.

You could experience other serious and life-threatening problems due to immune globulin. You could get aseptic meningitis (a type of brain inflammation with symptoms of severe headache, stiff neck, fatigue, fever, sensitivity to light, painful eye movements, nausea, and vomiting), a blood problem called hemolytic anemia (common symptoms include increased heart rate, fatigue, yellow skin or eyes, and dark-colored urine), and/or a lung problem called transfusion-related acute lung injury (commonly referred to as TRALI). TRALI is a condition where you build up fluid in the lungs (called pulmonary edema) that is not the result of heart failure.

If you have higher than normal body fluid volumes or if you have a condition where increasing body fluid volume may be a concern, a higher dose, such as 1g/kg for 1-2 days, is not recommended.

Because GAMUNEX-C is made from human blood, it may carry a risk of transmitting infectious agents such as viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent, and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent.

You may not take GAMUNEX-C subcutaneously if you have ITP. **If you have ITP and take GAMUNEX-C subcutaneously, you could experience a very serious and life-threatening black and blue wound (hematoma, which is a pocket of blood within a tissue).**

After you take GAMUNEX-C, your blood antibody levels may rise, which could cause some blood antibody tests to give false results.

The most common side effects in a clinical study with PIDD patients who got subcutaneous injections of GAMUNEX-C were infusion-site reactions such as redness, swelling, and itching; extreme tiredness; pain in the region of the head or neck; a runny nose, nasal congestion, sneezing, cough, and sputum production; joint pain; loose stools; a sensation of unease and discomfort in the upper stomach; swelling of the tissue lining the sinuses; inflammation of the airways that carry air to your lungs; a feeling of unhappiness, sadness, melancholy, gloom, hopelessness, or low spirits; red rash or bumps, itchy, swollen, and tender skin with or without blisters or a burning feeling; a severe throbbing pain or a pulsing sensation, usually on just one side of the head; muscle pain; familiar infectious diseases such as the common cold or flu; and raised body temperature or fever. In clinical studies with PIDD patients who got GAMUNEX-C intravenously, the most common side effects were cough; irritation and inflammation of the mucous membrane inside the nose; sore throat caused by inflammation of the back of the throat; pain in the region of the head or neck; a condition in which your airways narrow and swell and produce extra mucus; a sensation of unease and discomfort in the upper stomach; raised body temperature or fever; loose stools; and swelling of the tissue lining the sinuses. In a clinical study with CIDP patients who got GAMUNEX-C intravenously, the most common side effects were pain in the region of the head or neck; raised body temperature or fever; abnormally high blood pressure; feelings of coldness accompanied by shivering; a noticeable change in the texture or color of your skin such as your skin becoming scaly, bumpy, itchy, or otherwise irritated; a sensation of unease and discomfort in the upper stomach; joint pain; and abnormal physical weakness or lack of energy. In clinical trials with ITP patients who got GAMUNEX-C intravenously, the most common side effects were pain in the region of the head or neck; a discoloration of the skin resulting from bleeding underneath, typically caused by bruising; vomiting, fever, nausea, rash, abdominal pain, back pain, and a pain or an uncomfortable feeling in the upper middle part of your stomach.

The most serious side effects in clinical studies were a blood clot to the lung (pulmonary embolism) in 1 patient with a history of this condition (in CIDP), a flare-up of an existing type of anemia (autoimmune pure red cell aplasia) in 1 patient (in PIDD), and heart inflammation (myocarditis) in 1 patient (in ITP).

Please see brief summary of the Full Prescribing Information on the following page.

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.

References: 1. Merkies IS, Bril V, Dalakas MC, et al. Health-related quality-of-life improvements in CIDP with immune globulin IV 10%: the ICE Study. *Neurology.* 2009;72(15):1337-1344. **2.** Hughes RAC, Donofrio P, Bril V, et al; on behalf of the ICE Study Group. Intravenous immune globulin (10% caprylate-chromatography purified) for the treatment of chronic inflammatory demyelinating polyradiculoneuropathy (ICE study): a randomised placebo-controlled trial. *Lancet Neurol.* 2008;7(2):136-144. **3.** Dalakas MC, Latov N, Kuitwaard K. Intravenous immunoglobulin in chronic inflammatory demyelinating polyradiculoneuropathy (CIDP): mechanisms of action and clinical and genetic considerations. *Expert Rev Neurother.* 2022;22(11-12):953-962.

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GAMUNEX®-C

Immune Globulin Injection (Human), 10% Caprylate/Chromatography Purified

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use GAMUNEX®-C safely and effectively. See full prescribing information for GAMUNEX-C.

GAMUNEX®-C, [Immune Globulin Injection (Human), 10% Caprylate/Chromatography Purified]

Initial U.S. Approval: 2003

WARNING: THROMBOSIS, RENAL DYSFUNCTION and ACUTE RENAL FAILURE

See full prescribing information for complete boxed warning.

- **Thrombosis may occur with immune globulin products, including GAMUNEX-C. 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 GAMUNEX-C 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.**
- **Renal dysfunction, acute renal failure, osmotic nephrosis, and death may occur with immune globulin intravenous (IGIV) products in predisposed patients.**
- **Renal dysfunction and acute renal failure occur more commonly in patients receiving IGIV products containing sucrose. GAMUNEX-C does not contain sucrose.**
- **For patients at risk of renal dysfunction or failure, administer GAMUNEX-C at the minimum concentration available and the minimum infusion rate practicable.**

---------- INDICATIONS AND USAGE ----------

GAMUNEX-C is an immune globulin injection (human), 10% liquid indicated for treatment of:

- Primary Humoral Immunodeficiency (PI) in patients 2 years of age and older
- Idiopathic Thrombocytopenic Purpura (ITP) in adults and children
- Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) in adults

----------DOSAGE AND ADMINISTRATION ----------

Intravenous Administration Only: ITP and CIDP

Indication	Dose	Initial Infusion Rate	Maintenance Infusion Rate (if tolerated)
ITP	2 g/kg	1 mg/kg/min	8 mg/kg/min
CIDP	loading dose 2 g/kg maintenance dose 1 g/kg	2 mg/kg/min	8 mg/kg/min Every 3 weeks

- Ensure that patients with pre-existing renal insufficiency are not volume depleted; discontinue GAMUNEX-C if renal function deteriorates.
- For patients at risk of renal dysfunction or thrombosis, administer GAMUNEX-C at the minimum infusion rate practicable.

Intravenous or Subcutaneous Administration: PI
DO NOT ADMINISTER SUBCUTANEOUSLY FOR ITP PATIENTS

Route of Administration	Dose	Initial Infusion Rate	Maintenance Infusion Rate (if tolerated)
Intravenous (IV)	300-600 mg/kg	1 mg/kg/min	8 mg/kg/min Every 3 to 4 weeks
Subcutaneous (SC)	1.37 x current IV dose in grams/ IV dose interval in weeks	Adult:† 20 mL/hr/site Pediatric:† 10 mL/hr/site (< 25 kg) 15 mL/hr/site (≥ 25 kg)	Adult:† 20 mL/hr/site Pediatric:† 10 mL/hr/site (< 25 kg) 20 mL/hr/site (≥ 25 kg) Weekly

† Adults: use up to 8 infusion sites simultaneously; pediatric: use up to 6 infusion sites simultaneously; for all ages, ensure infusion sites are at least 2 inches (5 cm) apart.

----------DOSAGE FORMS AND STRENGTHS----------

GAMUNEX-C is a sterile solution for injection supplied in 1 g (10 mL), 2.5 g (25 mL), 5 g (50 mL), 10 g (100 mL), 20 g (200 mL), or 40 g (400 mL) single use vials.

----------CONTRAINDICATIONS ----------

- Anaphylactic or severe systemic reactions to human immunoglobulin
- IgA deficient patients with antibodies against IgA and a history of hypersensitivity

---------- WARNINGS AND PRECAUTIONS----------

- IgA deficient patients with antibodies against IgA are at greater risk of developing severe hypersensitivity and anaphylactic reactions. Have epinephrine available immediately to treat any acute severe hypersensitivity reactions.
- Hyperproteinemia, with resultant changes in serum viscosity and electrolyte imbalances may occur in patients receiving IGIV therapy.
- Aseptic Meningitis Syndrome (AMS) may occur, especially with high doses or rapid infusion.
- Hemolysis, either intravascular or due to enhanced RBC sequestration, can develop subsequent to GAMUNEX-C treatments. Risk factors include high doses and non-O blood group. Closely monitor patients for hemolysis and hemolytic anemia, especially in patients with pre-existing anemia and/or cardiovascular or pulmonary compromise.
- Monitor patients for pulmonary adverse reactions (transfusion-related acute lung injury [TRALI]).
- Volume overload.
- GAMUNEX-C is made from human plasma and may carry a risk of transmitting infectious agents, e.g., viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent.
- GAMUNEX-C is not approved for subcutaneous use in ITP patients. Due to a potential risk of hematoma formation, do not administer GAMUNEX-C subcutaneously in patients with ITP.
- Passive transfer of antibodies may confound serologic testing.

---------- ADVERSE REACTIONS ----------

The most common adverse reactions observed in ≥ 5% patients were:

PI: Intravenous: Cough increased, rhinitis, pharyngitis, headache, asthma, nausea, fever, diarrhea, and sinusitis.
Subcutaneous: Local infusion site reactions, fatigue, headache, upper respiratory tract infection, arthralgia, diarrhea, nausea, sinusitis, bronchitis, depression, allergic dermatitis, erythema, migraine, myalgia, viral infection, and pyrexia.

ITP: Headache, ecchymosis, vomiting, fever, nausea, rash, abdominal pain, back pain, and dyspepsia.

CIDP: Headache, pyrexia, hypertension, chills, rash, nausea, arthralgia, and asthenia.

To report SUSPECTED ADVERSE REACTIONS, contact Grifols Therapeutics LLC at 1-800-520-2807 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

---------- DRUG INTERACTIONS ----------

- The passive transfer of antibodies may transiently interfere with the response to live virus vaccines, such as measles, mumps and rubella.

----------USE IN SPECIFIC POPULATIONS ----------

- Geriatric: In patients over 65 years of age do not exceed the recommended dose, and infuse GAMUNEX-C at the minimum infusion rate practicable.

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Patient Registries: Key to Accelerating Rare Disease Research

Research and development of orphan drugs is time-consuming and costly, and appropriate participants for clinical trials are hard to find. Efficiently connecting rare disease patients with the right clinical trials is a crucial step toward discovering efficient, effective treatments for this population.

By Diane L.M. Cook

THE NATIONAL Institutes for Health (NIH) says there are more than 10,000 identified rare diseases affecting an estimated 30 million people in the United States.[1] According to the Centers for Disease Control and Prevention (CDC), a rare disease is one that affects fewer than 200,000 people; CDC estimates that of the 30 million people affected, about half are children.[2]

What's more, the United States Food and Drug Administration (FDA) says fewer than 10 percent of known rare diseases have an FDA-approved treatment available.[3]

These three government statistics do not paint a very encouraging picture for patients who suffer from rare diseases. Thousands of lives would be positively impacted by orphan drugs (medicines prescribed to treat rare diseases), but the research required to discover and market those drugs is painfully slow. To get more of them approved by FDA and into the hands of the people who need them, research into rare diseases and possible treatments for them must be accelerated. However, for research to be accelerated, rare disease patients need to get involved in clinical trial registries — and that's historically been easier said than done.

Harsha Rajasimha, PhD, understands this problem on a personal level: He suffered the loss of a child to a rare congenital disorder and a brother to a chronic disease. But his years of post-doctoral training at NIH and FDA, as well as his work in the healthcare and life science industry and his service as chairman of the annual Indo US bridging RARE Summit where he works to bring stakeholders together to address challenges, puts him in a unique position to bridge the gap between rare disease patients and clinical trials.

As the founder and CEO of Jeeva Clinical Trials, Dr. Rajasimha is working to accelerate access to treatments for rare and chronic diseases by eliminating bottlenecks in the clinical trial process with the Jeeva eClinical Cloud, an AI-driven platform that accelerates clinical trial timelines, realizes electronic data capture efficiencies and conducts remote clinical outcome assessments of participants in therapeutic areas such as oncology, rare diseases and chronic conditions. We interviewed Dr. Rajasimha to discuss why patient registries like this are key to accelerating rare disease research.

What Are the Current Obstacles Faced in Rare Disease Research?

"One obstacle is the limited understanding of the natural history of a rare disease," explained Dr. Rajasimha. "The often intricate nature of rare diseases makes it challenging to gather comprehensive data, hindering the development of targeted therapies. Another obstacle is the often misdiagnosis

or delayed diagnosis of a rare disease. The lack of awareness among healthcare providers about rare diseases can result in diagnostic odysseys for patients, delaying the initiation of appropriate treatments. The rarity of these diseases compounds the issue, as healthcare providers may not encounter such cases frequently, leading to a lack of familiarity."

How Has the Government Helped in the Research of Rare Diseases?

"Government involvement is integral in the commercialization of FDA-approved drugs, making them accessible as patients who have rare diseases rely on the government for access to and affordability of drugs," said Dr. Rajasimha. "These government initiatives underscore a commitment to knowledge exchange and accessibility, aligning with the evolving landscape of healthcare technologies and minimizing disparities in the research of rare diseases."

How Can Government Organizations and Nonprofits Collaborate for Successful Clinical Trials?

"The provision of funding, where government organizations allocate budgets and nonprofits contribute financial support, reduces the financial burden on researchers and institutions," explained Dr. Rajasimha. "Collaborations foster the establishment of patient registries, a critical component for successful clinical trials. By pooling data from diverse sources, including government health records and nonprofit-supported initiatives, researchers gain access to comprehensive and representative datasets. This facilitates participant recruitment, ensures diverse representation and improves the generalizability of trial results.

"Joint initiatives also enhance knowledge-sharing and capacity-building. Government research institutions and nonprofits can organize workshops, conferences and training programs to promote a culture of collaboration and continuous learning within the research community."

How Can Patient Registries Help in the Research of Rare Diseases?

"Patient registries serve as centralized repositories of comprehensive patient data," said Dr. Rajasimha, "offering a collective understanding of the natural history, progression and diverse manifestations of rare diseases. This wealth of information facilitates the identification of patterns, contributing to a deeper comprehension of these conditions.

"Researchers actively seeking to enroll participants for clinical trials often turn to such registries to identify their target populations. Patient registries enhance patient identification and recruitment for clinical trials and research studies. The rarity of these diseases often leads to challenges in assembling a sufficiently large and diverse study population. Registries streamline the recruitment process by providing a centralized platform where eligible participants can be identified and engaged, accelerating the pace of research."

Why Are Patient Registries Key to Accelerating Rare Disease Research?

"Patient registries enhance the identification and recruitment of individuals with rare diseases for clinical trials and research studies. Given the limited number of patients with a particularly rare condition, accessing a centralized database streamlines the process of finding suitable candidates, saving valuable time and resources. This accelerates the initiation and completion of research studies" said Dr. Rajasimha. "These registries enable the identification of specific subpopulations that may respond differently to treatments, identifying factors influencing treatment outcomes. This allows for targeted and personalized approaches by not only enhancing the precision of medical interventions, but also contributing to the development of more equitable healthcare solutions."

Why Is It Important for Patients to Participate in Clinical Trials?

"Participation in clinical trials offers patients access to cutting-edge therapies and interventions that may not be available through standard treatments," said Dr. Rajasimha. "This presents a unique opportunity for patients with rare diseases to explore potentially more effective options that could enhance their quality of life.

"Clinical trials often involve close monitoring and comprehensive healthcare assessments, ensuring that participants receive a high standard of medical care. This level of attention can lead to early detection of health issues, allowing for timely interventions and personalized treatment adjustments for patients."

Steps Patients Can Take to Find Clinical Trials

Actively participating in a clinical trial as a rare disease patient can be more than empowering — it could also

potentially be life-changing. Following are some actionable steps to get started:

• *Engage with patient registries.* Patient registries are invaluable tools for connecting patients with clinical trials. These databases collect detailed information about patients affected by rare diseases and often include trial recruitment notices. Patients can explore registries specific to their disease and consider enrolling to stay informed about relevant trials.

• *Explore disease-specific registries.* There are disease-specific registries such as the National Organization for Rare Disorders and Global Genes for comprehensive listings of trials relevant to a patient's rare disease. Patients can consider enrolling in registries such as the Rare Diseases Clinical Research Network or patient-powered platforms such as PatientsLikeMe to stay informed about ongoing trials and research opportunities tailored to their specific rare disease.

• *Seek guidance.* Patients should not hesitate to reach out to healthcare providers, patient advocacy organizations or support groups for guidance. They can offer insights into available trials, assist with navigating enrollment processes and provide emotional support throughout a patient's journey.

• *Utilize online platforms.* Patients can take advantage of online platforms and forums dedicated to rare diseases. These communities often provide information about clinical trials, share personal experiences and offer support to fellow patients and their caregivers.

• *Consider genetic testing.* Genetic testing can provide valuable insights into the underlying causes of rare diseases and may lead to opportunities for patients to participate in clinical trials targeting specific genetic mutations. Patients can discuss genetic testing options with their healthcare provider to explore potential eligibility for relevant trials.

Patient Registry and Clinical Trial Resources

Patients can keep themselves updated on ongoing clinical trials relevant to their rare disease. These websites provide important information on patient registries and clinical trials:

• ClinicalTrials.gov is a U.S. government website listing of all past, active and upcoming clinical trials globally. All U.S. clinical trials involving new treatments or drug repurposing are required to be listed in this portal.

• NIH Genetic and Rare Diseases Information Center (RareDiseases.info.nih.gov) offers a free patient information service for families battling rare diseases.

• Indo US Organization for Rare Diseases (www.indousrare.org/patient-concierge) offers a free patient concierge service via email, SMS, telephone or video call from anywhere in the world. Patients, or authorized family members of patients, can submit a request for assistance on their online concierge portal.

• Organizations such as IndoUSRare (IndoUSRare.org) invite rare disease patients and caregivers from across the world to join in creating awareness of rare diseases. For caregivers, IndoUSRare has teamed up with RareGivers in supporting their journey. Through initiatives such as IndoUSBridging Rare Summit, hosted each year alternately in India and the United States, patients, caregivers and stakeholders are brought together to join in accelerating the engagement of the global rare disease community in orphan drug research and development. IndoUSRare provides highly informative and educational sessions and presents great networking opportunities.

• Global Genes has a RARE concierge service (globalgenes.org/rare-disease-patient-services) that any patient can request for free assistance globally.

• Contract research organizations (ClinicalResearchNewsOnline.com/news/2023/10/11/concierge-style-services-becoming-a-staple-in-clinical-trials) set up concierge services for patients, specific to a sponsored clinical trial, as approved by a biopharmaceutical sponsor.

• Using Google to search for patient registries and clinical trials can be useful when using the name of a specific rare disease. However, search results must be filtered and evaluated for credibility, security, privacy and data-sharing concerns.

Get Involved!

There is still much work ahead to discover and distribute viable treatment options for the millions of Americans living with rare diseases. The good news is patients can be part of the solution! By actively participating in clinical trials, patients are not just contributing to scientific research, but they are also playing a vital role in advancing treatments and improving outcomes for their own rare disease, as well as for rare disease communities worldwide.

References

1. National Institutes of Health. Genetic and Rare Diseases Information Center. Accessed at rarediseases.info.nih.gov.
2. Roth, A. Research for Rare Disease: Giving Hope to Families Who Are Searching for Answers. National Children's, Feb. 24, 2022. Accessed at www.nationwidechildrens.org/family-resources-education/700childrens/2022/02/research-rare-disease-giving-hope-families.
3. U.S. Food and Drug Administration. Rare Disease Cures Accelerator, May 16, 2022. Accessed at www.fda.gov/drugs/regulatory-science-research-and-education/rare-disease-cures-accelerator.

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Clinical Trials 101: The Basics of Clinical Research

FDA enforces strict procedures to control the processes that occur when conducting clinical trials in the U.S.

By Ronale Tucker Rhodes, MS

CLINICAL RESEARCH is a broad term that refers to the study of health and illness in people. It consists of both controlled clinical trials and observational studies. Whereas observational studies monitor people in normal settings such as asking older adults about their exercise habits and comparing changes over time, clinical trials test medical, surgical or behavioral interventions to determine if a new form of treatment or prevention such as a new drug or medical device is safe and effective in people.[1] Clinical trials answer important questions, including:[2]

- Does the new treatment work in people and, if so, how well does it work? Is it better than treatment now being used, and if not, is it as good and does it cause fewer side effects? Or does it work in some people who aren't helped by current treatments?
- Is the new treatment safe? No treatment is without risk, but do the benefits of the new treatment outweigh the risks?
- Is the treatment better than the standard treatment given for a disease?

Every May, Clinical Trials Day is recognized to celebrate the first "controlled" clinical trial conducted by James Lind, MD, in 1747. In this trial, Dr. Lind's research found a much-needed remedy for sailors suffering from scurvy. But the history of clinical trials extends all the way back to 500 B.C., with the first reference of one recorded in the Bible. This observational trial tested whether people who consumed a diet consisting purely of meat and wine would be significantly healthier than those on a non-meat diet, but in reality found those on the vegetarian diet were significantly healthier than those on the meat diet.[3]

Today, clinical trials have evolved into standardized procedures with strict regulatory and ethical requirements,

and they continue to contribute to scientific and medical breakthroughs. But, because there are both benefits and risks of clinical trials, patients are encouraged to gain a good understanding of the different types, their phases, how eligibility is determined and what regulations govern them. Not only can this knowledge contribute to their confidence in U.S. Food and Drug Administration (FDA)-approved treatments, but also to determine if a trial might be right for them.

Clinical Trial Types

The National Institutes of Health (NIH) lists six types of clinical trials:[4]

- Prevention trials that look for better ways to prevent a disease in people who have never had the disease or to prevent the disease from returning using approaches such as medicines, vaccines or lifestyle changes;
- Screening trials that test new ways for detecting diseases or health conditions;
- Diagnostic trials that study or compare tests or procedures for diagnosing a particular disease or condition;
- Treatment trials that test new treatments, new combinations of drugs or new approaches to surgery or radiation therapy;
- Behavioral trials that evaluate or compare ways to promote behavioral changes designed to improve health; and
- Quality-of-life trials (or supportive care trials) that explore and measure ways to improve the comfort and quality of life of people with conditions or illnesses.

Phases of Clinical Trials

Commonly, only four phases of clinical trials are discussed (Phase I, II, III and IV); however, there are actually five phases of clinical trials that build on one another, each of which is designed to answer specific questions:[2,4]

- Phase 0 clinical trials explore if and how a new drug may work. While Phase 0 trials *are* performed on humans, they are performed mainly to help speed up and streamline the drug approval process by helping researchers find out if the drugs do what they're expected to do. And, this phase uses only a few small doses of a new drug in a few people.
- Phase I clinical trials are conducted to find the highest dose of a new treatment that can be given safely without causing severe side effects. This phase looks at what the drug does to the body and what the body does with the drug. To begin, a very low dose of the drug is given to the first few people who are closely watched, and if only minor side effects occur, the next few participants get a higher dose. This process continues until a dose of the drug that's most likely to work while having an acceptable level of side effects is found. Phase I trials usually include only a small number of people (20 to 80), and they do not include placebos (inactive treatments).

Phase II clinical trials, which typically include a larger group of people (100 to 300), are conducted to determine a drug's effectiveness and to further study its safety. In some Phase II trials, all patients get the same dose of the drug, but other trials randomly assign people to different treatment groups that may get different doses or get the treatment in different ways to see which provides the best balance of safety and response. Phase II trials also do not include placebos.

If a treatment works in a Phase II clinical trial, the treatment must be tested in a Phase III trial before being approved for general use. In this phase, a larger group of participants (1,000 to 3,000) are given the new treatment to confirm its effectiveness; monitor side effects; compare it with standard or similar treatments; and collect information that will allow the new drug or treatment to be used safely. Participants are often picked at random (called randomized) to receive either the standard treatment or the new treatment. When possible, Phase III trials are double-blind, meaning neither the doctor nor the patient knows which of the treatments the patient is receiving.

Phase IV clinical trials are conducted after FDA has approved a treatment and it is available to the public. In this phase, researchers track the treatment's safety in the general population, as well as seek more information about the drug's or treatment's benefits and optimal use, over a long period of time.

Clinical Trials Results Process

NIH explains that once a clinical trial is completed, the researchers examine information collected during the study and then make decisions about the meaning of the findings and about the need for further testing. After a Phase I or II trial, the researchers decide whether to move on to the next phase or to stop testing the treatment or procedure because it was unsafe or ineffective. After a Phase III trial is completed, the researchers examine the information and decide whether the results have medical importance.

Peer-reviewed scientific journals often publish results from clinical trials. Peer review is a process by which experts review the report before it is published to ensure the analysis and conclusions are sound. Results that are particularly important are often featured in the news and discussed at scientific meetings and by patient advocacy groups before or after they are published in a scientific journal. Once a treatment has

been proven safe and effective in a Phase III clinical trial, it may become a new standard of medical practice.[4]

Who Is Eligible to Participate in Clinical Trials?

A clinical trial's protocol describes who is eligible to take part in the research, and eligibility criteria are different for each trial. Eligibility is based on such factors as age, sex, type and stage of disease, previous treatment history, other medical conditions and whether participants are healthy or patient volunteers. The goal is to reduce the variation within the study and to ensure the researchers will be able to answer the questions they plan to study. Therefore, not everyone who applies for a clinical trial will be accepted.[5,6]

Also, some trials include healthy volunteers, whereas others include patients. Those with healthy volunteers are designed to develop new knowledge rather than to provide direct benefit to those taking part, and the data may be used to compare patient volunteers and healthy volunteers. Trials with patient volunteers also help develop new knowledge, and depending on how much is known about the disease or condition, the treatments being studied may or may not benefit patient volunteers.[6]

A clinical trial's protocol describes who is eligible to take part in the research, and eligibility criteria are different for each trial.

Key Players in the Clinical Trials Process

There are key people involved in clinical trials, some of whom are always in the background and others who are patient-facing:[7]

- *Research coordinator.* The research coordinator utilizes the trial protocol to recruit and screen patients and ensure the safety and security of trial medications. He or she also oversees the collection of all patients' data, tests, side effects and outcomes, and pays attention to any adverse events that patients experience.
- *Research assistant.* The research assistant helps the coordinator fulfill the protocol requirements — from recruiting patients to patient interviews and preparing mid-trial reports to helping prepare final reports.
- *Principal investigator.* A principal investigator is a physician who coordinates the medical side of the trial to ensure patients' access to a potentially more effective treatment while watching for adverse events. The investigator also interfaces with the drug company as it evaluates the efficacy and safety of a new treatment.
- *Sponsors.* Sponsors help ensure the necessary funding for the clinical trial and direct the ongoing process of the trial. Sponsors also communicate with FDA, other regulatory agencies and ethics boards over any concerns about the treatment's safety or benefits. Sponsors can include government agencies; biotech, medical device or pharmaceutical companies; healthcare entities; or private individuals or entities.
- *Contract research organizations (CROs).* CROs provide clinical trial management services for biotech, medical device and pharmaceutical companies. They have extensive experience in the day-to-day operation of clinical trials to maintain a well-run trial.

Regulations Governing Clinical Trials

FDA, the regulatory authority responsible for safeguarding public health and human rights, enforces strict guidelines on any investigators researching medicinal products in the U.S. When an investigational new drug or treatment is tested on humans, the sponsor of the trial must submit an investigational new drug application (NDA) to FDA. The NDA provides all relevant information on the sponsor's planned clinical trial, including documents such as the investigator's brochure, study protocol, previous human experiences with the drug and more.

While FDA is reviewing a submitted NDA, an institutional review board (IRB) consisting of at least five qualified members from diverse backgrounds simultaneously conducts a thorough ethics assessment of the proposed investigation. The IRB weighs potential risks with benefits carefully in the interest of ensuring patient safety, particularly with informed consent.

Participants in clinical trials must freely give informed consent based on FDA's Good Clinical Practice (GCP) guidelines, an international standard for ethics in clinical research. According to GCP guidelines, investigators must first clearly provide the details of a research study in simple language to prospective participants in an informed consent form (ICF). Once participants have the information needed to decide to consent, they must be given ample time to do so without pressure or

coercion. As the ICF is revised throughout the study, all patients must be reconsented to the newest version to ensure GCP compliance. And, as with all other study materials that may be provided to participants, ICFs cannot be implemented until they have been approved by the IRB.

A clinical trial can begin only after it receives approval from the ethics board, typically 30 days after the NDA is submitted, although this time frame can be longer. And, once the first human patient is enrolled, the clinical trial must be registered on ClinicalTrials.gov after 21 calendar days.[8]

For more information about this, see the Clinical Brief column titled "Understanding the FDA Approval Process for New Drugs" on p.14.

Resources for Clinical Trials

There are many websites patients can access to find clinical trials. Following is a list of some of the larger clinical research resources:

• ClinicalTrials.gov is perhaps one of the most well-known sites to access information about clinical trials. Maintained by FDA, it is a searchable registry and results database of federally and privately supported clinical trials conducted in the U.S. and around the world. Information is provided about a trial's purpose, who may participate, locations and phone numbers for more details.

• The National Institutes of Health (NIH) (clinicalstudies.info.nih.gov) conducts clinical research trials for many diseases and conditions, including cancer, Alzheimer's disease, allergy and infectious diseases, and neurological disorders. NIH maintains an online database of clinical research studies taking place at its Clinical Center located on its campus in Bethesda, Md. The Clinical Center hosts a wide range of studies from rare diseases to chronic health conditions, as well as studies for healthy volunteers. Visitors can search by diagnosis, sign, symptom or other key words.[9]

• ResearchMatch (www.researchmatch.org) is an NIH-funded initiative to connect people who are trying to find research studies and researchers seeking people to participate in their studies. It is a free, secure registry to make it easier for the public to volunteer and to become involved in clinical research studies that contribute to improved health in the future.

• National Cancer Institute (NCI) Clinical Trials (www.cancer.gov/research/participate/clinical-trials-search) is a federal agency providing funding for most U.S. cancer clinical trials. The site lists open and closed cancer clinical trials sponsored or supported by NCI.

• CISCRP's Search Clinical Trials (www.ciscrp.org/services/search-clinical-trials) is a free service designed to help people find clinical trials relevant to their medical and healthcare needs. The CISCRP team works with individuals to help them understand their options and find local clinical trials in their community, or as far as they are comfortable traveling.

• Antidote match (antidote.me) helps patients find a trial based on their condition, city, age, sex and whether the interest is in a trial for themselves or someone else. Individuals answer a few questions about their condition and medical history, and depending on the condition chosen, questions may pertain to length of diagnosis, medication usage and other information relevant to a study. The results page shows studies that may be a potential match that can be clicked on to learn more about the study's participation requirements, where the study sites are located and what phase the clinical trial is in.[10]

• Cancer.net (www.cancer.net/research-and-advocacy/clinical-trials/finding-clinical-trial) also lists sites for both general and disease-specific clinical trials.

Clinical trials can also be located through patient advocacy organizations, individual medical centers and cancer centers, and pharmaceutical companies.

Clinical Trial Participants Are Pivotal to Saving Lives

Patients can help scientists develop new medications and other strategies to treat and prevent disease by participating in clinical trials. In fact, without research participants, many of the effective treatments available to patients today would not exist. But, prior to getting involved in any clinical trial, patients should understand what the trial entails and what type of commitment will be required. IG

References

1. National Institute on Aging. What Are Clinical Trials and Studies? Accessed at www.nia.nih.gov/health/clinical-trials-and-studies/what-are-clinical-trials-and-studies#participate.
2. American Cancer Society. Types and Phases of Clinical Trials. Accessed at www.cancer.org/cancer/managing-cancer/making-treatment-decisions/clinical-trials/what-you-need-to-know/phases-of-clinical-trials.html.
3. Orlando Clinical Research Center. The Evolution of Clinical Research Through the Years, May 24, 2022. Accessed at ocrc.net/the-evolution-of-clinical-research-through-the-years.
4. National Institutes of Health. NIH Clinical Research Trials and You: The Basics. Accessed at www.nih.gov/health-information/nih-clinical-research-trials-you/basics.
5. U.S. Food and Drug Administration. Basics About Clinical Trials, updated May 8, 2023. Accessed at www.fda.gov/patients/clinical-trials-what-patients-need-know/basics-about-clinical-trials.
6. National Institutes of Health. Clinical Trials: Who Can Participate. Accessed at www.nhlbi.nih.gov/research/clinical-trials/participating.
7. Circuit Clinical. Clinical Trials 101: The Ultimate Guide to Understanding Clinical Research, June 29, 2023. Accessed at www.circuitclinical.com/posts/clinical-trials-101.
8. Vial. Understanding Clinical Trial Regulations (2023). Accessed at vial.com/blog/articles/understanding-clinical-trial-regulations-2023/?utm_source=organic.
9. NIH Clinical Center. Accessed at clinicalstudies.info.nih.gov.
10. Antidote. How to Find Clinical Trials Near You. Accessed at www.antidote.me/blog/how-to-find-clinical-trials-near-you.

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

FDA-approved for adult and pediatric patients aged 2 years and older with primary immunodeficiency (PI)

cutaquig®
Immune Globulin Subcutaneous (Human)-hipp,16.5% solution

Count the reasons

to ask your care team about cutaquig

1 hour or less to **complete infusion***

2 or fewer **infusion sites**†‡

3 **flexible dosing** schedule options§

Not an actual patient.

*The estimated infusion duration for a 13 g (78 mL) weekly dose is approximately 45 minutes in an adult patient using 2 infusion sites, if tolerated, not including setup time.
†Depending on your dose and dosing schedule selected.
‡Most infusions only need 2 or fewer infusion sites.
§Every-other-week, weekly, or frequent dosing (2-7 times a week).

Scan to visit **CutaquigInfo.com** to learn more.

INDICATIONS AND USAGE

CUTAQUIG (Immune Globulin Subcutaneous [Human] - hipp) is a 16.5% immune globulin solution for subcutaneous infusion indicated for treatment of primary humoral immunodeficiency (PI) in adults and pediatric patients 2 years of age and older.

There are many forms of PI. Certain types of PI are associated with low immunoglobulin G (IgG), which are proteins that help fight infection.

CUTAQUIG is a liquid medicine for infusion that contains immunoglobulin G (IgG), which are proteins that help fight infection. It is made from human plasma that is donated by healthy people and contains antibodies that replace the missing antibodies in patients with PI.

CUTAQUIG is given under the skin (subcutaneous). Most of the time, infusions under the skin are given at home by self-infusion or by a caregiver. Only use CUTAQUIG by yourself after you have been instructed on use by a healthcare provider (HCP).

IMPORTANT SAFETY INFORMATION

WARNING: THROMBOSIS

See full Prescribing Information for complete BOXED WARNING

- **Thrombosis may occur with immune globulin products, including CUTAQUIG. 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 CUTAQUIG 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 of hyperviscosity.**

What is the most important information I need to know about CUTAQUIG?

CUTAQUIG can cause the following serious reactions:

- Severe allergic reactions causing difficulty in breathing or skin rashes
- Blood clots in the heart, brain, lungs, or elsewhere in the body
- Severe headache, drowsiness, fever, painful eye movements, or nausea and vomiting
- Decreased kidney function or kidney failure
- Dark colored urine, swelling, fatigue, or difficulty breathing

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

What should I know while taking CUTAQUIG?

- CUTAQUIG can make vaccines (like measles/mumps/rubella or chickenpox vaccines) not work as well for you. Before you get any vaccines, tell your HCP that you take CUTAQUIG
- Tell your HCP if you are pregnant, or plan to become pregnant, or if you are nursing

CUTAQUIG can cause serious side effects. If any of the following problems occur after starting CUTAQUIG, contact your HCP or call emergency services. If any of the following problems occur during CUTAQUIG infusion, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting, or dizziness. These could be signs of a serious allergic reaction
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem
- Fever over 100°F. This could be a sign of an infection

Ask your HCP whether you should have rescue medications available, such as antihistamines or epinephrine.

What are the possible or reasonably likely side effects of CUTAQUIG?

The most common side effects of CUTAQUIG are:

- Infusion site reactions (including but not limited to redness, swelling, itching, fluid in tissue, pain, mass, bruising)
- Headache
- Elevated body temperature

One or more of the following possible side effects may occur at the site of infusion; these may go away within a few hours and are less likely after the first few infusions:

- Mild or moderate pain
- Redness
- Itching

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away.

Patients should always ask their doctors for medical advice about adverse events.

You may report an adverse event related to Pfizer products by calling 1-800-438-1985 (US only). If you prefer, you may contact the US Food and Drug Administration (FDA) directly. The FDA has established a reporting service known as MedWatch where healthcare professionals and consumers can report problems they suspect may be associated with the drugs and medical devices they prescribe, dispense, or use. Visit www.fda.gov/MedWatch or call 1-800-FDA-1088.

CUTAQUIG® is a registered trademark of Octapharma AG.

Please see brief summary of Full Prescribing Information on following page and Full Prescribing Information, including complete BOXED WARNING and Patient Information and Instructions for Use, at CutaquigInfo.com.

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PP-CQG-USA-0561

March 2023

cutaquig®
Immune Globulin Subcutaneous (Human)-hipp, 16.5% solution

CONSUMER BRIEF SUMMARY

(CUTAQUIG: *cute-ah-quig*)

This brief summary highlights the most important information about CUTAQUIG. Please read it carefully before using CUTAQUIG and each time you get a refill, as there may be new information. This Patient Information does not take the place of talking with your healthcare provider about your medical condition or your treatment. If you have any questions after reading this, ask your healthcare provider. For more information, go to www.CutaquigInfo.com.

What is CUTAQUIG?

CUTAQUIG is a ready-to-use liquid solution of immunoglobulin G (IgG), also called antibodies, which protects the body against infection. CUTAQUIG is used to treat adult patients and pediatric patients 2 years of age and older with primary humoral immunodeficiency (PI).

There are many forms of PI. The most common types of PI result in an inability to make a very important type of protein called antibodies, which help the body fight off infections from bacteria or viruses. Regular administration of CUTAQUIG has been demonstrated to help your body to fight bacteria and viruses that cause infections. CUTAQUIG is made from human plasma that is donated by healthy people. CUTAQUIG contains antibodies collected from these healthy people; these antibodies replace the missing antibodies in patients with PI.

WARNING: THROMBOSIS

See full Prescribing Information for complete BOXED WARNING

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

Who should NOT use CUTAQUIG?

Do not use CUTAQUIG if you have ever had a severe allergic reaction to immune globulin or other blood products.

Tell your healthcare provider if you:

- Ever had any severe reaction to other immune globulin medicines
- Were told that you have a condition called IgA deficiency
- Have a history of heart or blood vessel disease
- Have had blood clots or thick blood
- Have been immobile for some time

CUTAQUIG can cause serious side effects. If any of the following problems occur after starting CUTAQUIG, contact your HCP or call emergency services. If any of the following problems occur during CUTAQUIG infusion, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting, or dizziness. These could be signs of a serious allergic reaction
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem
- Pain, swelling, warmth, redness, or a lump in your legs or arms. These could be signs of a blood clot
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem
- Chest pain or trouble breathing, or blue lips or extremities. These could be signs of a serious heart or lung problem
- Fever over 100°F. This could be a sign of an infection

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

What should I tell my healthcare provider before using CUTAQUIG?

Talk to your healthcare provider about any medical conditions that you have or have had.

Tell your healthcare provider:

- That you are taking CUTAQUIG before you get a vaccination, as vaccines may not work while you are taking CUTAQUIG.
- About all of the prescription and non-prescription medicines you take, including over-the-counter medicines, dietary supplements, or herbal medicines.
- If you are pregnant, plan to get pregnant, or if you are nursing because CUTAQUIG might not be right for you.
- If you have diabetes. If you need to do glucose testing, your healthcare provider may tell you to use a different way to monitor your blood sugar levels on the day that you receive a CUTAQUIG infusion. Some types of blood glucose testing systems (glucometers) can falsely interpret the maltose contained in CUTAQUIG as glucose. If you are uncertain, ask your healthcare provider which glucose testing system you can use while using CUTAQUIG.

The most common side effects that may occur with CUTAQUIG are:

- Infusion site reactions (including but not limited to redness, swelling, itching, fluid in tissue, pain, mass, bruising)
- Headache
- Elevated body temperature

One or more of the following possible side effects may occur at the site of infusion; these may go away within a few hours and are less likely after the first few infusions:

- Mild or moderate pain
- Redness
- Itching

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away. If you encounter any problems or experience side effects during or after the infusion, contact your healthcare provider. When doing so, keep your treatment diary or logbook with you to be able to give all necessary information.

Patients should always ask their doctors for medical advice about adverse events.

You may report an adverse event related to Pfizer products by calling 1-800-438-1985 (US only). If you prefer, you may contact the US Food and Drug Administration (FDA) directly. The FDA has established a reporting service known as MedWatch where healthcare professionals and consumers can report problems they suspect may be associated with the drugs and medical devices they prescribe, dispense, or use. Visit www.fda.gov/MedWatch or call 1-800-FDA-1088.

This brief summary is based on the CUTAQUIG Prescribing Information (October 2021).

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What to Do When Insurance Won't Cover Your Prescriptions

Here are important action items for getting the medicine you need, even when your insurance initially denies coverage.

By Rachel Maier, MS

YOUR PRESCRIPTION isn't covered. Now what?

It's a question I found myself frantically asking when my insurance company denied coverage for a necessary medication. I asked my husband for advice — he is a healthcare provider, after all, and he prescribes drugs for patients all the time. "What should patients do when their insurance won't pay for the prescriptions they need?" I asked.

Here's what he told me: Step one: Don't panic. Step two: Panic a little. Step three: Refer to step one. Then he told me to talk to my doctor.

His advice made me laugh, but he was on to something. There certainly are steps you can take to get the medicine you need, and your doctor is there to help you (so don't panic!), but much of the onus is on you to get it sorted out, and it can be a time-consuming and challenging process (so panic a little).

It's disconcerting to be told a necessary medication isn't covered by your insurance, especially when you pay top dollar for your premium. You expect insurance will pay for the medicines you need, but that just isn't always the case, and it's frustrating. You pay for insurance so you don't have to deal with this issue in the first place!

Sometimes your insurance doesn't cover a prescription your doctor writes for you, and you might not know that until you try to fill it. (For example, you might not have had high blood pressure when you enrolled in your insurance plan, but your doctor recently diagnosed you with it and wrote you a prescription for a beta blocker.) Other times, your formulary changed without your knowledge, and you don't know your drug isn't covered anymore until you go to the pharmacy to pick it up (such as Prilosec, which is now available over the counter).

The list of drugs your insurance covers changes all the time: New drugs enter the market, old drugs leave the market and some drugs become available over the counter, and amid all the flux, your coverage changes. Your pharmacy will dispense a prescription drug if/when you have a valid prescription for it, but when your insurance won't cover its cost (or even a portion of its cost), the responsibility to pay is completely on your shoulders, and most Americans just can't pay the out-of-pocket prices for their necessary prescriptions. However inconvenient and annoying, here are steps you can take to work with your doctor and insurance company to get the medicine you need.

1) Ask Why the Drug Is Denied

First and foremost, ask why the prescription is being denied: The answer may be far simpler than you imagine.

For example, perhaps your insurance coverage changed but your pharmacy still has your old insurance information. In that case, updating the pharmacy's system with your new information may do the trick. Or, maybe your pharmacy is missing information from your doctor (i.e., dosage or duration, physician signature, etc.) so the prescription is automatically denied. Other reasons may include exceeding the allowed amount of the drug, an expired prescription or a coding mistake. Small errors can cause big problems, so double-check your pharmacy is using the most up-to-date, complete information, or make a quick phone call to your doctor to get any omissions fixed.

However, even when all the information is correct and accounted for, your prescription may still be denied on other grounds. Other questions to ask include: Is a prior authorization required? Does the medication require step therapy? Is the drug in question no longer included in your insurance formulary? Did your insurance company move your drug to a higher tier and now will only cover a portion of the cost? The answer to these questions will inform what you do next.

2) Ask for Alternatives

Your insurance provider will deny coverage for drugs that are excluded from its formulary. (A formulary is a list of all the prescription drugs covered by your insurance plan. Sometimes it's called a "drug list.") Check your insurance formulary to see if your prescription is included or excluded.

If the drug is excluded (e.g., not covered by insurance), ask your doctor if there is an alternative in the same drug class that can be prescribed in its place. In many cases, there is. For example, in our example above, a beta blocker is prescribed to treat high blood pressure. There are many beta blockers that treat high blood pressure such as atenolol, metoprolol and carvedilol, so if one particular type isn't covered by your formulary, chances are good that a different one is covered.

Another option is to ask if there is an alternative medicine in a different drug class that will treat the same condition. Back to our example: Calcium channel blockers also treat high blood pressure, so a doctor may recommend it as an alternative to a beta blocker. If your doctor recommends an alternative drug that treats the same condition, you can check their recommendations against your formulary.

3) Consider Step Therapy

Sometimes insurance denies covering a drug because it requires the patient to complete step therapy first; it may agree to cover the cost of the drug after the patient tries (and fails) step therapy.

In short, step therapy, or the "fail first" approach, blocks patients from newer, more expensive treatment options prescribed by their doctor, and instead mandates that patients try and fail older, cheaper medicines first. If the older drug is not effective, then the patient "steps" to a different treatment, which eventually may include the drug the patient's doctor prescribed in the first place.[1]

Talk to your doctor about whether step therapy is right for you. In some cases, your doctor will support step therapy and recommend you give it a try. Then again, there are some instances in which your doctor will not recommend step therapy, including 1) contraindication or likelihood of adverse reaction or harm; 2) step therapy drug is expected to be ineffective based on the patient's clinical characteristics; 3) the patient has previously tried the step therapy drug but it was ineffective or caused an adverse event; 4) the step therapy drug is not in the best interest of the patient due to medical necessity; or 5) the patient is stable on the drug originally prescribed by the doctor while on a different insurance plan, and the doctor reports it is in the patient's best interest to continue on the effective drug.[2]

Your insurance provider will deny coverage for drugs that are excluded from its formulary.

4) Ask for an Exception

If an alternative drug isn't possible or your doctor recommends bypassing step therapy, you can ask him or her to help you file an exception request with your insurance provider for the drug that was originally denied. Formularies are strict, but again, they may grant exceptions on compelling, reasonable grounds. Your doctor can submit paperwork to your insurance company documenting why you cannot take the formulary's preferred medication and must have one that does not appear on it.[3] Insurance companies will often grant an exception when it is medically necessary.

One example is in the case of brand name versus generic drugs. Insurance companies generally prefer to cover generic drugs rather than their brand name counterparts. However, your doctor may write a prescription for a brand name drug; when your pharmacy fills the prescription, it will typically substitute the generic version instead of the brand name. However, there may be instances in which your doctor believes the brand name is in your best interest. If your pharmacy substitutes the generic for the brand name, ask your doctor to resubmit the prescription noting "do not substitute" or "brand name only" (or something similar) on the prescription. Then, ask your doctor to help you file a brand exception request with your insurance, as the brand name is deemed medically necessary by your doctor.

Other scenarios in which formulary exceptions are common include having an allergy to the medication in the formulary, the alternate medication not working with your condition, your doctor deeming the medications available in the formulary aren't appropriate for you, or your doctor believing the use of the formulary medication could escalate your medical condition.[1]

5) Initiate an Internal Appeal or External Review

If you've done everything right — asked for an alternative, tried step therapy, filed exception request(s), etc. — and your insurance provider still denies the request to cover the drug you need, you can file an internal appeal. This means you are requesting that your insurance company conduct a full and fair review of its decision. In urgent cases, your insurance company must speed up the process. Work with your doctor to either fill out the necessary forms or write a letter. Make sure it indicates the name of the drug and the reason you need it covered, and include supporting documentation from your doctor.

Then, if your insurance company denies your appeal, you have the right to file an external (independent) review with your state's insurance regulator, which will make the final decision.[4] This means that your insurance does not make the final decision and must abide by the decision of the external reviewer. If the external review determines your insurance must cover your drug, then your insurance company must cover it.

6) Ask About Patient Assistance

Although insurance is meant to help patients pay for necessary medications, there is often a gap between what insurance covers and what patients can afford. Many pharmaceutical manufacturers, non-profits and government agencies offer patient assistance programs (PAPs) that help people afford medications by providing drugs to patients at reduced or no cost — even for people with commercial insurance. (In fact, in some cases, certain PAPs are only available to people with commercial insurance.) Benefits and eligibility requirements vary by PAP, but they are worth looking into. They can significantly lower the cost to the consumer, and even provide the medication free of charge to qualifying patients. Work with your doctor to find out if there is an assistance program for the drug he or she prescribed. If there is, your doctor will help you get the forms filled out and submitted to the PAP for review.[5]

7) Try Manufacturer Co-Pay Cards or GoodRX

If your insurance won't cover or contribute to the cost of the drug you need, you can still have the pharmacy fill the prescription; you'll just have to pay for it yourself. GoodRX and manufacturer co-pay cards may lower the out-of-pocket costs to consumers. However, while you are not required to have insurance to use GoodRX, co-pay cards are only available to those with commercial or private insurance (and not Medicare or Medicaid).[6]

Co-pay cards typically offset costs of brand name medications that do not have a generic alternative, while GoodRX offers coupons for both brand names and generics. Co-pay cards may have limits to how many you may use and how much you can save annually, but there is no limit to using GoodRX.

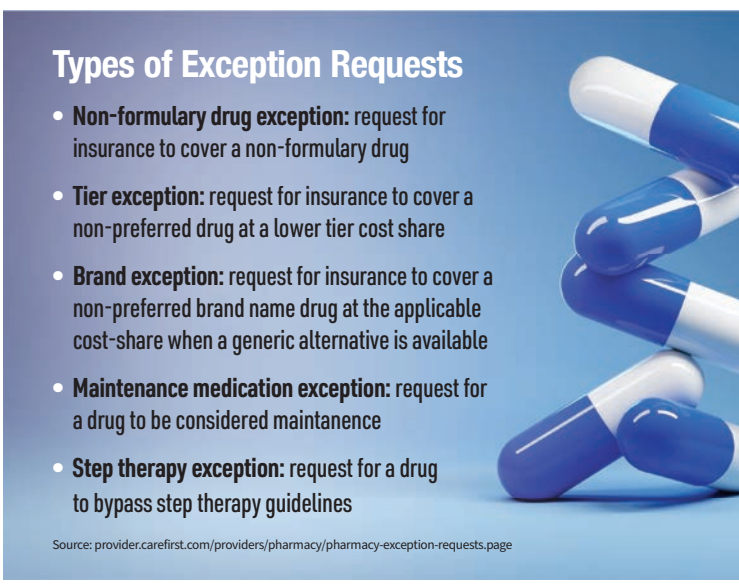
And, for a $10 monthly membership fee, you can enroll in GoodRX Gold. GoodRX Gold offers more than 1,000 prescriptions for less than $10 and free home delivery on more than 950 prescriptions. For example, with GoodRX Gold, a 60-capsule supply of Gabapentin is only $5.12 (as opposed to $77.25). That's a 93 percent savings! (Note: you can use GoodRX or GoodRX Gold even if you are insured. In some cases, you may save more money using it rather than using your insurance.)[7]

8) Switch Insurance Providers

If all else fails, consider switching insurance providers at the next open enrollment opportunity. Find a policy with a formulary that covers your medication. Keep in mind, however, that formularies change all the time, and while they may cover your medication today, they might not cover it tomorrow.

Talk to Your Doctor

Paying for prescriptions gets complicated and can be very frustrating, no doubt about it. The best thing you can do is to explore your options with your doctor.



When I faced a drug denial, I talked to my doctor about it and we decided the best course of action was to pursue a PAP. It turned out to be a great solution to my problem, but it's definitely not the only solution out there. If you're facing a drug denial, remember: It could be due to a small error. If not, switching to a comparable, covered medicine may be in order. Then again, step therapy could be the right choice for you, or perhaps filing an exception request is best. Talk to your doctor, and discuss the best course of action to get you the treatment you need. IG

References

1. Patients Rising. Step Therapy: Everything You Need to Know About "Fail First" Insurance Policy, Aug. 23, 2016. Accessed at www.patientsrising.org/step-therapy-explained/?gad_source=1&gclid=CjwKCAiArLyuBhA7EiwA-qo80KTqLZn6bUFlg2C0oobBcZ1OWNeUAotiZaz6gAt5f4O8_BjHMOirdhoCpRcQAvD_BwE.
2. NORD. Step Therapy (Fail First). NORD State Report Card. Accessed at rarediseases.org/policy-issues/step-therapy.
3. Patient Advocate. Filing a Formulary Exception. Accessed at www.patientadvocate.org/explore-our-resources/insurance-denials-appeals/filing-a-formulary-exception.
4. Skinner, G. 4 Ways to Get Insurance to Cover Your Prescription Drugs. Consumer Reports, Feb. 12, 2016. Accessed at www.consumerreports.org/health/4-ways-to-get-insurance-to-cover-your-prescription-drugs.
5. Chase, L. What Are Manufacturer Copay Cards? GoodRx Health, April 29, 2022. Accessed at www.goodrx.com/drugs/savings/what-are-manufacturer-copay-cards.
6. GoodRX. How GoodRX Works. Accessed at www.goodrx.com/how-goodrx-works.
7. GoodRX. What Is GoodRX Gold? Accessed at www.goodrx.com/gold.

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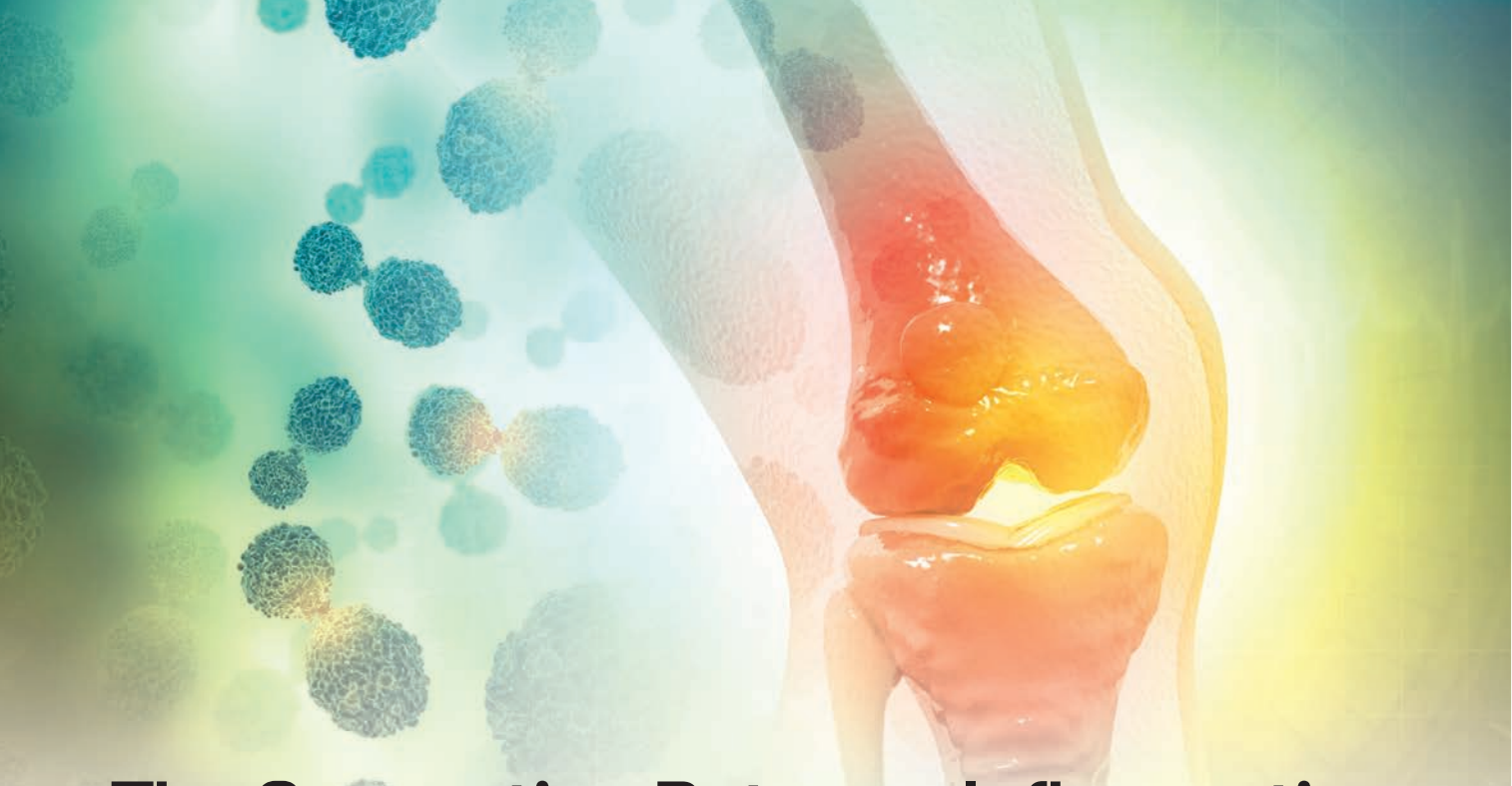

The Connection Between Inflammation and Stress-Related Diseases

Chronic inflammation poses an invisible threat to health and longevity, but lifestyle changes and stress management can help you maintain optimal health.

By Trudie Mitschang

INFLAMMATION, typically considered the body's defense mechanism against harmful stimuli, plays a complex role in health and disease. While acute inflammation is crucial for tissue repair and pathogen clearance such as when you are injured or fighting off a virus, chronic inflammation can lead to a myriad of health problems, including stress-related diseases.

Inflammation can be triggered by any number of factors, including persistent infections, environmental toxins and lifestyle habits such as poor diet, lack of exercise and stress. When inflammation becomes chronic, it can damage healthy tissues and organs, leading to the development of potentially life-threatening conditions.

According to a Harvard Medical School report, chronic inflammation plays a central role in some of the most challenging diseases of our time, including rheumatoid arthritis, cancer, heart disease, diabetes, asthma and even Alzheimer's.[1] In fact, inflammation has gotten a lot more attention in the past 10 years from the medical community, but a root problem still exists: Modern medicine focuses more on treating symptoms rather than addressing underlying causes of many health issues, which in numerous cases is inflammation.

"Inflammation is largely the body's defense mechanism against things that should not be in the body," says Eduardo Marbán, MD, PhD, executive director of the Smidt Heart Institute. "But as with any complicated defense system, any misstep can lead to friendly fire. The same physiological process that reddens the skin around an insect bite and causes swelling in a bum knee can also lead to a host of ailments, ranging from cancer and depression to diabetes and severe cases of COVID-19."[2]

Stress, whether physical, emotional or environmental, is a common trigger for inflammation. When the body perceives a threat or stressor, it activates the stress response, also known as the "fight or flight" response, which involves the release of the stress hormones cortisol and adrenaline. While this response is essential for survival in threatening situations, chronic or excessive stress can dysregulate the immune system and promote inflammation.

In essence, stress can induce chronic inflammation, which in turn leads to stress-induced conditions, creating a vicious cycle.

Diseases Linked to Chronic Inflammation

One of the most important medical discoveries of the past two decades has been that the immune system and inflammatory processes are involved in not just a few select disorders, but a wide variety of mental and physical health problems that impact life expectancies worldwide. According to a study published in *Nature Medicine Journal*,[3] three chronic inflammatory diseases have been recognized as the most significant cause of death in the world today, with more than 50 percent of all deaths being attributable to inflammation-related conditions, including:

• Cardiovascular disease: Chronic inflammation contributes to the development and progression of atherosclerosis, a leading cause of heart attacks and strokes. Research confirms that persistent, low-level inflammation plays a part in every stage of heart disease, including increasing the risk of plaque rupture, which leads to heart attacks. The 2017 Canakinumab Anti-Inflammatory Thrombosis Outcomes Study[4] showed that treating low-level inflammation in people who experienced prior heart attacks (correcting for differences in other factors such as blood cholesterol, diabetes and high blood pressure) lowered patients' risk of heart attacks, stroke and even death from cardiac events.

• Diabetes: Inflammation is involved in the development of insulin resistance, a key feature of type 2 diabetes. Several pathophysiological studies have strengthened our understanding of insulin resistance and secretion in the course of disease onset and progression. Chronic inflammation disrupts insulin signaling pathways, leading to impaired glucose uptake by cells and elevated blood sugar levels.[5]

• Autoimmune disorders: Chronic inflammation plays a central role in autoimmune diseases, in which the immune system mistakenly attacks healthy tissues. For example, rheumatoid arthritis is a chronic inflammatory autoimmune disease characterized by joint swelling and pain, cartilage erosion and injury, and is accompanied by a persistent inflammatory state.[6] Conditions such as lupus and inflammatory bowel disease are also a result of persistent inflammation and tissue damage.

• Neurodegenerative diseases: Inflammation has been implicated in the pathogenesis of neurodegenerative diseases such as Alzheimer's disease. Inflammation in Alzheimer's disease has emerged as a central pathology that likely plays a role in onset and progression. Numerous investigations have highlighted that the sustained inflammation in the brain accelerates other core pathologies, making inflammatory mechanisms viable targets for future therapeutic developments.[7]

• Cancer: Chronic inflammation creates a microenvironment that promotes cancer initiation, progression and metastasis. Over time, chronic inflammation can cause DNA damage and lead to cancer. For example, people with chronic inflammatory bowel diseases such as ulcerative colitis and Crohn's disease have an increased risk of colon cancer.[8]

"Chronic inflammation can also cause threat sensitivity and hypervigilance, which gives rise to anxiety disorders and PTSD, as well as fatigue and social-behavioral withdrawal, which are key symptoms of depression," says George Slavich, PhD, associate professor of psychiatry and biobehavioral sciences at UCLA. He also notes that anyone concerned about developing chronic inflammation (and its correlated diseases) can take affirmative steps to prevent it. "If we make people aware of these risk factors, our hope is that individuals will reduce the factors that apply to them."[9]

How Diet and Lifestyle Can Help

Understanding the complex interplay between inflammation and stress is essential for implementing effective strategies to promote health and well-being. By adopting lifestyle modifications, practicing stress-management techniques and exploring targeted interventions, individuals can mitigate the adverse effects of chronic inflammation and stress-related diseases, leading to improved quality of life and longevity. Experts believe individuals can reduce their risk by embracing lifestyle changes such as eating a healthy diet, improving sleep, exercising regularly, quitting smoking and decreasing both stress and exposure to environmental pollutants.

"Diet is one of the key factors that influences inflammation in the body," says Dr. Slavich. "Whereas fried foods, red meat, sodas and white bread and pastries that have refined carbohydrates tend to increase inflammation, fruits, nuts, green leafy vegetables, tomatoes and olive oil tend to reduce inflammation. Therefore, while diet is not the only factor that can be targeted to improve immune health, it is an important one."[9]

HyQvia

[Immune Globulin Infusion 10% (Human) with Recombinant Human Hyaluronidase]

What is HyQvia®?

HyQvia [Immune Globulin Infusion 10% (Human) with Recombinant Human Hyaluronidase] is a liquid medicine that is given under the skin (subcutaneously) to treat primary immunodeficiency (PI) in people 2 years and older.

IMPORTANT SAFETY INFORMATION

What is the most important information that I should know about HyQvia?

- HyQvia can cause blood clots.
- Call your healthcare professional (HCP) if you have pain, swelling, warmth, redness, or a lump in your legs or arms, other than at the infusion site(s), unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, numbness or weakness on one side of the body.
- Your HCP may perform blood tests regularly to check your IgG level.
- Do not infuse HyQvia into or around an infected or red swollen area because it can cause infection to spread.

Who should not take HyQvia?

Do not take HyQvia if you:

- Are allergic to IgG, hyaluronidase, other blood products, or any ingredient in HyQvia.

What should I avoid while taking HyQvia?

- HyQvia can make vaccines (like measles/mumps/rubella or chickenpox vaccines) not work as well for you. Before you get any vaccines, tell your HCP that you take HyQvia.

What should I tell my HCP before I start using or while using HyQvia?

Tell your HCP if you:

- Have or had any kidney, liver, or heart problems or history of blood clots because HyQvia can make these problems worse.
- Have IgA deficiency or a history of severe allergic reactions to IgG or other blood products.
- Are pregnant, trying to become pregnant or are breast feeding. It is not known whether HyQvia can harm the unborn baby or breastfed infant.

What are the possible or reasonably likely side effects of HyQvia?

HyQvia can cause serious side effects. If any of the following problems occur after starting HyQvia, stop the infusion immediately and contact your HCP or call emergency services:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain.
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem.
- Pain, swelling, warmth, redness, or a lump in your legs or arms, other than at the infusion site(s). These could be signs of a blood clot.
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem.

Meet the only monthly* subQ IG treatment and say hy to more of what you love.

0.025 infections per year

This is equivalent to 25 acute serious bacterial infections (ASBIs) out of 1,000 patients over the course of the 12-month study period.

The FDA standard for efficacy—that is, if an immunoglobulin works—is less than 1 ASBI per year. In the clinical trial, people taking HyQvia experienced significantly less than that.

0 days in the hospital per year

There was a mean of 0.037 days spent in the hospital due to infection during the study.

<4 days off work or school per year

On average, patients taking HyQvia missed 3.31 days of work or school due to an infection.

- HyQvia was studied in a clinical trial of 83 people with PI, with the main goal of measuring how many acute serious bacterial infections (ASBIs) they experienced over the course of 1 year
- ASBIs are short-term but serious infections caused by bacteria that require immediate medical care
- ASBIs included 2 episodes of pneumonia, both treated as outpatients with oral antibiotics. An additional episode of pneumonia requiring hospitalization occurred during the ramp-up
- The most common general (systemic) side effects were headache, antibody formation against hyaluronidase (Hy), fatigue, nausea, fever, and vomiting. The most common side effects at the infusion site (local) were pain, redness, swelling, and itching

*Between infusions, based on administration every 3 or 4 weeks.
subQ IG=subcutaneous immune globulin.

IMPORTANT SAFETY INFORMATION (continued)

- Chest pain or trouble breathing, blue lips or extremities. These could be signs of a serious heart or lung problem.
- Fever over 100°F. This could be a sign of an infection.

After HyQvia infusion a temporary, soft swelling may occur around the infusion site, which may last 1 to 3 days, due to the volume of fluid infused. The following possible side effects may occur at the site of infusion and generally go away within a few hours, and are less likely after the first few infusions.

- Mild or moderate pain
- Redness
- Swelling
- Itching

The most common side effects of HyQvia are:

- Headache
- Fatigue
- Nausea
- Fever
- Vomiting

Antibodies to the hyaluronidase component of HyQvia were formed in some patients taking HyQvia. It is not known if there is any long-term effect. In theory, these antibodies could react with your body's own hyaluronidase (PH20). PH20 is present in the male reproductive tract. So far, these antibodies have not been associated with increased or new side-effects.

These are not all the possible side effects. Talk to your HCP about any side effect that bothers you or that does not go away.

Please see Important Facts about HyQvia on the following page.

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.

What makes HyQvia different? Scan the code!

You can always visit **HyQvia.com/why-hyqvia** to learn what makes HyQvia different, and so much more.

IMPORTANT FACTS about HYQVIA (Hi-Q-via) [Immune Globulin Infusion 10% (Human) with Recombinant Human Hyaluronidase] Solution, for subcutaneous administration

What is the most important information I should know about HYQVIA?

- HYQVIA can cause blood clots.
- Call your healthcare provider (HCP) if you have pain, swelling, warmth, redness, or a lump in your legs or arms, other than at the infusion site(s), unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, numbness or weakness on one side of the body.
- Your HCP may perform blood tests regularly to check your IgG level.
- Do not infuse HYQVIA into or around an infected or red swollen area because it can cause infection to spread.

What is HYQVIA?

HYQVIA is a liquid medicine containing immune globulin and Recombinant Human Hyaluronidase. HYQVIA is given under the skin (subcutaneously) to treat primary immunodeficiency (PI) in people 2 years of age and older. HYQVIA contains IgG antibodies, collected from human plasma donated by healthy people.

- The antibodies help your body to fight off bacterial and viral infections.
- The hyaluronidase is found in your body naturally. It's the first part of your two-part infusion. It temporarily opens the space under your skin (the subcutaneous space), allowing a larger amount of IgG to reach your subcutaneous tissue and be absorbed into your bloodstream.

What should I tell my HCP before I start using or while using HYQVIA?

Tell your HCP if you:

- Have or had any kidney, liver, or heart problems or history of blood clots because HYQVIA can make these problems worse.
- Have IgA deficiency or a history of severe allergic reactions to IgG or other blood products.
- Are pregnant, trying to become pregnant, or are breastfeeding. It is not known whether HYQVIA can harm the unborn baby or breastfed infant.

Who should not take HYQVIA?

- Do not take HYQVIA if you are allergic to IgG, hyaluronidase, other blood products, or any ingredient in HYQVIA.

How should I take HYQVIA?

- HYQVIA is infused under the skin (subcutaneously) up to once every 4 weeks.
- You can get HYQVIA at your HCP's office, clinic, or hospital.
- You can use HYQVIA at home. You and your HCP will decide if home self-infusion is right for you.

What are the possible or reasonably likely side effects of HYQVIA?

After HYQVIA infusion a temporary, soft swelling may occur around the infusion site, which may last 1 to 3 days, due to the volume of fluid infused.

The following local reactions may occur at the site of infusion and generally go away in a few hours. Local reactions are less likely after the first few infusions.

- Mild or moderate pain
- Redness
- Swelling
- Itching

The most common side effects of HYQVIA are: headache, fatigue, nausea, fever, and vomiting.

Antibodies to the hyaluronidase component of HYQVIA were formed in some patients taking HYQVIA. It is not known if there is any long-term effect. In theory, these antibodies could react with your body's own PH20. PH20 is present in the male reproductive tract. So far, these antibodies have not been associated with increased or new side effects.

Call your HCP or go to your emergency department right away if you get:

- Hives, swelling in the mouth or throat, itching, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of irritation and swelling of the lining around your brain.
- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem.
- Pain, swelling, warmth, redness, or a lump in your legs or arms, other than at the infusion site(s). These could be signs of a blood clot.
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a liver or blood problem.
- Chest pain or trouble breathing, blue lips or extremities. These could be signs of a serious heart or lung problem.

These are not all of the possible side effects for HYQVIA. You can ask your HCP for information that is provided to HCPs. Talk to your HCP about any side effects that bother you or that don't go away.

How do I store HYQVIA?

Store HYQVIA refrigerated or at room temperature.

- You can store HYQVIA in the refrigerator (36°F to 46°F [2°C to 8°C]) for up to 36 months.
- You can store HYQVIA at room temperature (up to 77°F [25°C]) for up to 3 months during the first 24 months from the date of manufacturing (Mfg Date) printed on the carton.
- Do not return HYQVIA to the refrigerator if you take it out to room temperature.

Check the expiration date on the carton and vial label. Do not use HYQVIA after the expiration date.

Do not freeze.

Protect from light. You can use the original HYQVIA containers to protect it from light.

How do I get more information about HYQVIA?

The risk information provided here is not comprehensive. To learn more, talk about HYQVIA with your HCP or pharmacist. The FDA-approved Full Prescribing Information, including Information for Patients, can be found at www.HYQVIA.com or by calling 1-877-TAKEDA7 (1-877-825-3327).

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It is estimated that 60 percent of chronic diseases, including many of the health problems listed above, could be prevented by a healthy diet. Not only can eating the right foods reduce the occurrence of inflammation in the first place, but it can also help to reduce and resolve inflammation that is already occurring. In terms of simple dietary changes that pack a big health punch, consuming omega-3 fatty acid-rich foods like salmon and other fatty fish (such as anchovies, halibut, sardines and tuna) can reduce inflammation.

"Omega-3s disrupt the production of chemicals that cause inflammation by certain immune system cells. They may even help lower the risks for stroke and for the type of brain inflammation associated with Alzheimer's disease," says Dr. Robert Shmerling, a rheumatologist and medical editor of a Harvard special health report on fighting inflammation. "But our body doesn't make omega-3s. We need to get them from food."[10]

Exercise has also been shown to reduce inflammation, and people who get regular physical activity tend to have lower levels of inflammation than their more sedentary counterparts. Aerobic exercise — the kind that gets your heart and lungs working such as brisk walking, is one of the easiest ways to begin seeing the benefits. "[Exercise] helps reduce body fat, which contains inflammation-promoting substances. Exercise may also increase the production of hormones that help keep inflammation in check," says Dr. Shmerling.

Being diligent about your oral health is also proven to minimize your risk of chronic inflammation. Regular brushing and flossing, it turns out, fights more than cavities. "Evidence suggests bacteria can travel to the heart, lungs and even the brain. Inflammation in the gums also is strongly associated with diabetes," says Dr. Tien Jiang, a prosthodontist in the department of oral health policy and epidemiology at the Harvard school of dental medicine.[10]

Finally, mind-body interventions such as mindfulness, meditation, yoga and deep breathing exercises have been shown to reduce stress levels and dampen inflammatory responses. These practices promote relaxation, regulate the stress response and modulate immune function, thereby mitigating the detrimental effects of chronic stress and inflammation on health. "Studies have found that people who take part in those types of exercise have less stress and fewer inflammatory markers in the blood," Dr. Shmerling says.[10]

In addition to lifestyle interventions, pharmacological approaches targeting inflammation and stress pathways may also be beneficial in certain cases. Nonsteroidal anti-inflammatory drugs, corticosteroids and immunomodulatory agents are commonly used to manage inflammation in conditions such as arthritis and autoimmune disorders. Likewise, medications that modulate the stress response such as antidepressants and anxiolytics (a class of medications aimed at treating patients with panic disorders, generalized anxiety and various other uses) may help alleviate stress-related symptoms and reduce inflammation.

An Ongoing Endeavor

Research is increasingly making the connection between chronic inflammation and disease. At Cedars-Sinai Medical Center in Los Angeles, Calif., scientists across a variety of disciplines, including cardiology, endocrinology, neurology, oncology and more, are dissecting the role inflammation plays in every organ system and disease state.[2] This research opens up the possibility that a class of inflammation-thwarting remedies could address nearly every disease.

As we consider the high levels of stress that is commonplace in our 21st century lifestyles, the health threats posed by hidden inflammation become a top priority for medical professionals, researchers and the general population. Exposing this invisible but harmful force can significantly change how we view chronic disease and disease treatment and management. IG

References

1. Axe, J. Chronic Inflammation at the Root of Most Diseases and How to Prevent! Dr. Axe, Dec. 12, 2018. Accessed at draxe.com/health/inflammation-at-the-root-of-most-diseases.
2. Paturel, A. A Double-Edged Sword: Inflammation and Your Health. Cedars-Sinai, Feb. 12, 2021. Accessed at www.cedars-sinai.org/discoveries/inflammation.html.
3. Cimons, M. Chronic Inflammation Is Long Lasting, Insidious, Dangerous. And You May Not Even Know You Have It. *The Washington Post*, Jan. 20, 2020. Accessed at www.washingtonpost.com/health/chronic-inflammation-is-long-lasting-insidious-dangerous-and-you-may-not-even-know-you-have-it/2020/01/17/93ab0fa2-316f-11ea-9313-6cba89b1b9fb_story.html.
4. Crossman, D, and Rothman, A. The Canakinumab Antiinflammatory Thrombosis Outcome Study Trial — The Starting Gun Has Fired. *Journal of Thoracic Disease*, 2017 Dec;9(12):4922-4925. Accessed at www.ncbi.nlm.nih.gov/pmc/articles/PMC5756992.
5. Tsalamandris, S, Antonopoulos, AS, Oikonomou, E, et al. The Role of Inflammation in Diabetes: Current Concepts and Future Perspectives. *European Cardiology Review*, 2019 Apr;14(1):50-59. Accessed at www.ncbi.nlm.nih.gov/pmc/articles/PMC6523054.
6. Xiang, Y, Zhang, M, Jiang, D, et al. The Role of Inflammation in Autoimmune Disease: A Therapeutic Target. *Frontiers in Immunology*, 2023 Oct;14:1267091. Accessed at www.frontiersin.org/journals/immunology/articles/10.3389/fimmu.2023.1267091/full.
7. Kinney, JW, Bemiller, SM, Murtishaw, AS, et al. Inflammation as a Central Mechanism in Alzheimer's Disease. *Alzheimer's and Dementia* (New York, NY), 2018 Sept. 6;4:575-590. Accessed at www.ncbi.nlm.nih.gov/pmc/articles/PMC6214864.
8. National Cancer Institute. Chronic Inflammation, April 29, 2015. Accessed at www.cancer.gov/about-cancer/causes-prevention/risk/chronic-inflammation.
9. Ravella, S. Inflammation May Be the Culprit Behind Our Deadliest Diseases. *Time Magazine*, April 11, 2023. Accessed at time.com/6269070/inflammation-deadly-diseases.
10. Godman, H. Easy Ways to Keep Inflammation in Check. Harvard Health Publishing, Feb. 1, 2023. Accessed at www.health.harvard.edu/staying-healthy/easy-ways-to-keep-inflammation-in-check.

TRUDIE MITSCHANG is a contributing writer for *IG Living* magazine.

Profile: Rory Duckworth

By Trudie Mitschang

AS THE FOUNDER and owner of the Salt Lake Triathlon Club, Rory Duckworth has defied the odds when it comes to what is possible when living with an immune disease. He is a USA Triathlon-certified coach and Ironman-certified coach, with a passion for swimming, cycling, running and helping others. Rory has many local triathlon victories, including competing in the Ironman World Championships in Kona, Hawaii, and he is ranked gold in the Ironman All World Athlete program. He is also an All-American Triathlete for the United States of America. Through his work as a coach and event organizer, Rory aims to create inclusive, supportive environments where everyone can experience the joy and personal growth that comes from crossing a finish line.

Trudie: What is your diagnosis story?

Rory: My journey to diagnosis was both challenging and enlightening. For years, I experienced symptoms that were difficult to explain and often dismissed by healthcare professionals. It was a cycle of doctor visits, tests and still no clear answers until my [former] pediatrician, whom I hadn't seen for many years since I was a 30-year-old adult at the time, advised my mother (who had a discussion with him about my symptoms) that I see an immunologist. Finally, after a thorough investigation and the insight of a specialist, I was diagnosed with common variable immune deficiency (CVID). This diagnosis was very depressing but also a relief and a turning point, offering clarity to the myriad of symptoms I had been experiencing.

Trudie: What were your symptoms prior to being diagnosed?

Rory: Prior to my diagnosis, I struggled with recurrent infections, fatigue and other symptoms that significantly impacted my daily life. These weren't just ordinary colds; they were severe and prolonged, and often led to complications. My energy levels were consistently low, making it difficult to maintain an active lifestyle, which was frustrating given my passion for triathlon and fitness.

Trudie: What is your treatment plan, and how has it evolved?

Rory: My treatment plan is centered around immune globulin replacement therapy, which helps to bolster my immune system. This therapy has been a cornerstone of managing my condition, alongside other supportive treatments and lifestyle adjustments to minimize exposure to pathogens (as best I can do with two small children in school). Over time, the treatment has become more tailored to my specific needs, incorporating feedback from my healthcare team and my own experiences living with the condition. Apart from that, fitness and clean eating are the bedrocks of my treatment plan.

Trudie: Tell us more about your clean eating regimen.

Rory: My clean eating regimen focuses on nourishing my body with whole foods. I prioritize fruits, vegetables, lean proteins and whole grains, making an effort to avoid processed foods, excessive sugars and artificial additives. I also avoid seed oils whenever possible. For example, a typical day might include oatmeal or eggs for breakfast, a salad with grilled chicken for lunch, and fish with quinoa and vegetables for dinner. This approach supports my immune system, reduces inflammation and sustains my energy levels for an active lifestyle.

Trudie: Do you have a daily fitness regimen?

Rory: Yes, maintaining daily physical activity is crucial, even on non-training days. My routine may include a walk or bike ride with my kids and dogs, ice baths, sauna and hot tubs. These activities help me keep a balanced state of mind and manage stress, which is just as important as physical health.

Trudie: How is your health today?

Rory: Today, my health is stable thanks to a combination of regular treatment, vigilant self-care and a proactive approach to managing my condition. While there are still challenges and days of struggle, I have learned how to adapt and maintain a balance that allows me to pursue my passions and career ambitions. My V02 max (the maximum amount of oxygen my body can absorb and use during exercise) and overall fitness are at their best, and I compete competitively in triathlons.

Trudie: How did you overcome physical limitations and become a triathlete?

Rory: Becoming a triathlete despite my physical limitations was a journey of perseverance, adaptation and resilience. It required a deep understanding of my body's signals, careful management of my energy levels and a commitment to training within my limitations. Support from the triathlon community such as the Salt Lake Triathlon Club and my healthcare team was invaluable, allowing me to continue to build my strength and endurance.

Trudie: What is the biggest challenge you've faced, and how did you overcome it?

Rory: The biggest challenge I've faced was accepting my condition and its implications on my life and dreams. Spending two hours a week administering expensive drugs can take a toll on a person mentally. Overcoming it required a shift in perspective, focusing on what I could do rather than what I couldn't. It meant finding new ways to pursue my passions, advocating for my health and learning to see my condition not as a limitation, but as a part of my journey that has taught me resilience, empathy and the importance of community.

Trudie: What is your career focus today?

Rory: Today, my career is focused on making triathlon and endurance sports accessible and enjoyable for people of all backgrounds and abilities. Through my work as a coach and event organizer, I aim to create inclusive, supportive environments where everyone can experience the joy and personal growth that comes from crossing a finish line. I make a living as an insurance agent but give back to others through triathlons.

Trudie: What has living with chronic illness taught you?

Rory: Living with a chronic illness has taught me the value of resilience, empathy for others, the power of community and the importance of advocating for oneself. It's shown me that strength isn't just physical; it's the courage to face challenges head-on and the determination to keep moving forward, even when the path is uncertain.

Trudie: What advice do you have for others living with an immune deficiency?

Rory: My advice is to embrace your journey, including the challenges, as part of what makes you unique. Seek support from healthcare professionals, peers and community resources; stay active physically and mentally as much as possible, even if it's just a short walk to the mailbox. Never underestimate the importance of self-care and listening to your body. And most importantly, hold onto your passions and pursuits — they can be a powerful source of motivation and joy, even in the face of adversity.

Trudie: Are you now or were you ever part of a support group?

Rory: Indeed, I've participated in a support group for individuals with primary immunodeficiency on Facebook. The sense of community, shared experience and mutual support has been invaluable, offering both practical advice and emotional solidarity.

Trudie: How does chronic illness make parenting two young children a challenge?

Rory: Parenting with a chronic illness involves navigating extra considerations, like ensuring I maintain my energy and health. I manage by practicing self-care, being realistic about my capabilities and fostering open communication with my family. My wife and I approach it as a team effort, which helps us adapt and support each other.

Trudie: What do you know now that you wish you'd known before?

Rory: I wish I had understood the significance of self-advocacy in healthcare sooner and the impact of a positive mindset. Learning to seek better care and maintain an optimistic outlook have been pivotal in my journey.

Trudie: What is your proudest accomplishment?

Rory: Completing my first Ironman triathlon stands out as my proudest achievement, as did qualifying for and participating in the Ironman World Championships on the Big Island in Kona, Hawaii. It represented not just a physical feat but the culmination of overcoming numerous challenges related to my health. It was a powerful affirmation of my resilience and determination.

Trudie: How do you maintain a positive attitude?

Rory: A positive attitude for me comes from practicing gratitude, recognizing small victories and staying connected with my supportive network. Focusing on the progress I've made rather than the obstacles ahead keeps me motivated and hopeful.

Trudie: What are your goals for the future?

Rory: Looking forward, I aim to further advocate for those with chronic illnesses, broaden my reach as a coach to inspire more individuals and possibly author a book to share my experiences and insights. Professionally, I aspire to expand my triathlon event company to offer more inclusive and supportive experiences for athletes at all levels.

TRUDIE MITSCHANG is a contributing writer for *IG Living* magazine.


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Do you utilize Ig therapy?
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Whether you've been recently diagnosed, have been living with a primary immunodeficiency (PI) for years, or just think you might have a PI, The Immune Deficiency is here to help.

While PI has no cure, there are lifesaving treatments available that can improve your quality of life. Our programs are meant to connect, engage, and empower families to live longer, stronger, healthier lives.

SCAN ME

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Beginner's Guide to Primary Immune Deficiency

By Michelle Searle

RECEIVING a primary immune deficiency (PI) diagnosis is overwhelming! Even if you're relieved to finally have an answer for why you're always getting sick, maybe you feel like you will never get a handle on everything involved. I understand what you're going through, and it's completely normal! I hope this article serves as a starting point and leaves you with some hope and confidence that you can continue down this road.

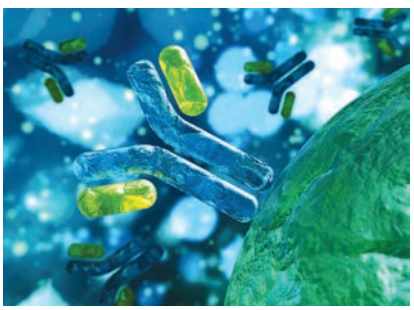
Acronyms

There are a lot of acronyms in the PI world, so buckle up. Here are just a few:

- PI: primary immune deficiency. PI is an umbrella term because there are more than 500 different identified types.
- CVID: common variable immune deficiency. CVID is one type of PI. It's most commonly pronounced by saying each letter "C-V-I-D," but sometimes is pronounced like a word "c-vid."
- IgG: Immunglobulin G, the most common immunoglobulin. Immunoglobulins are the same things as antibodies (proteins that your immune system makes to fight germs).
- IVIG: intravenous immune globulin. IVIG is a medicine made up of pooled antibodies administered through an IV.
- Sub-Q/SCIG: subcutaneous (under the skin) immune globulin. Sub-Q/SCIG delivers IG medicines through a needle in the subcutaneous tissue (the deepest layer of skin).

Research

It's overwhelming how much there is to learn about life with PI, but Google is not your friend. Don't make it harder on yourself by going down the web's



dark rabbit hole. Instead, visit sites such as the Jeffrey Modell Foundation (www.info4pi.org), Immune Deficiency Foundation (IDF; www.primaryimmune.org) and MyIgSource (www.myigsource.com) to learn about PI and how to manage and live with it. Also, talk to your doctor, who can recommend books and other websites.

Resources

Since you're reading this article in *IG Living*, you've already found a great resource! The websites listed above are also great; they provide some of the best resources I have ever used, including books for adults and children; support groups, walks and conferences; help with insurance and finding doctors; regular peer mentor check-ins by phone; volunteer opportunities; and so much more! If I had to pick one thing for you to do right now, it's to go to the IDF website and sign up for a Get Connected group that interests you. The groups meet virtually once a month, and you'll have a chance to connect and relate to others who are going through what you're going through right now.

Fun Stuff

A lot of unique opportunities and fun can come from having PI, too! In fact, I've had the chance to travel throughout the United States, visiting cities and doing things I otherwise might have never done. I got to visit Capitol Hill in Washington, D.C., along with other people in the PI community, to meet with members of Congress and share our stories, influence policy and make a lasting impact. I've also made a lot of friends around the country.

Also, people with PI can use accommodations designed to make things a little easier for the disabled community such as Disney's Disability Access Service, which allows guests with a disability to avoid extended wait times. (As a big Disney fan, this is one of my favorite accommodations!)

Final Pep Talk

You can do this! I promise that infusions will become second nature, and you might begin to look forward to them because they give you energy and protection. You will find a doctor who you like. You will figure out a system that works for you. You will meet others like you. You will learn how strong and special you are. Do not close yourself in, but open yourself up to what's available. Ask for help and speak your truth. Breathe, and take it one hour and one day at a time.

MICHELLE SEARLE is a teacher from South Florida who was diagnosed with common variable immunodeficiency at 11 years old. She is currently living in New York where you will most likely find her eating pizza or trying to make friends with the local cats.

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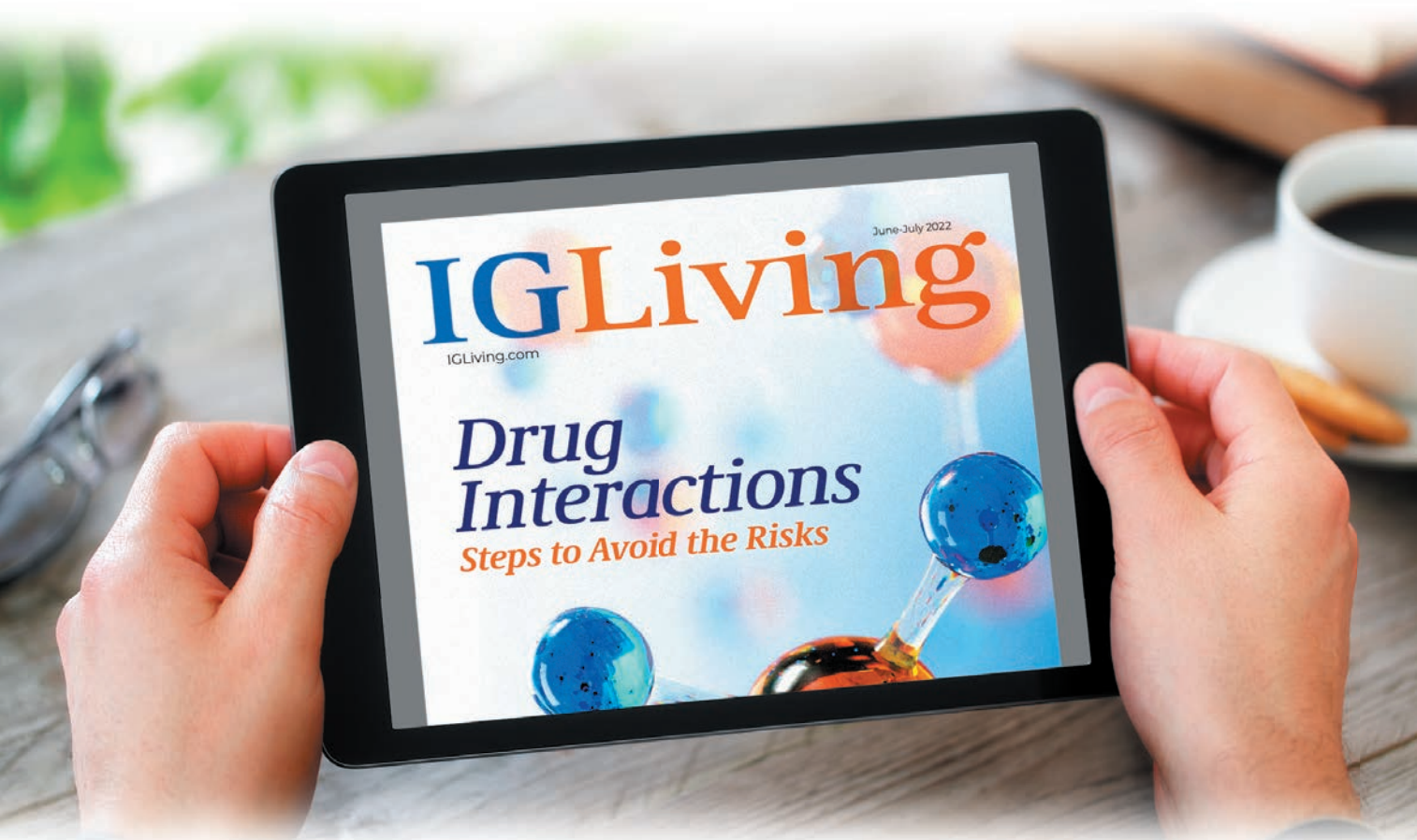

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Immune Globulin Subcutaneous (Human) 20% Liquid

PREFILLED SYRINGES

ELEVATE YOUR EXPERIENCE

Discover proven protection with a first and only for Ig—
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Important Safety Information

Hizentra®, Immune Globulin Subcutaneous (Human), 20% Liquid, is a prescription medicine used to treat:

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

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.

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.

Please see Brief Summary of full Prescribing Information on reverse.

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- Wide range of sizes for your individual needs
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"I am very grateful for the simplicity that prefilled syringes provide, even for someone like me who has limited strength in their hands."

—**Angela**, patient advocate on Hizentra*

Learn more about Hizentra prefilled syringes at **Hizentra.com/elevate**

*Patient advocates are not healthcare professionals or medical experts. For medical questions, please contact your physician. Patient advocates are compensated by CSL Behring LLC for their time and/or expenses.

Ig=immune globulin.

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. Tell your doctor about any side effect that bothers you or does not go away.

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 full prescribing information for Hizentra, including boxed warning and the patient product information.

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.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

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Satisfaction was high with the first and only Ig prefilled syringes

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Ask your doctor if self-infusing with prefilled syringes might be right for you. Learn more at **Hizentra.com/elevate**

*In an online survey, at least 32 of 33 people who self-infused Ig in prefilled syringes were very/somewhat satisfied on all measures mentioned.

HIZENTRA®, Immune Globulin Subcutaneous (Human), 20% Liquid
Initial US Approval: 2010

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use HIZENTRA safely and effectively. Please see full prescribing information for HIZENTRA, which has a section with information directed specifically to patients.

What is HIZENTRA?
HIZENTRA is a prescription medicine used to treat primary immune deficiency (PI) and chronic inflammatory demyelinating polyneuropathy (CIDP). Infuse HIZENTRA only after you have been trained by your doctor or healthcare professional. HIZENTRA is to be infused under your skin only. DO NOT inject HIZENTRA into a blood vessel (vein or artery).

Who should NOT take HIZENTRA?
Do not take HIZENTRA if you have too much proline in your blood (called "hyperprolinemia") or if you have had reactions to polysorbate 80. Tell your doctor if you have had a serious reaction to other immune globulin medicines or have been told that you have a deficiency of the immunoglobulin called IgA.

Tell your doctor if you have a history of heart or blood vessel disease or blood clots, have thick blood, or have been immobile for some time. These things may increase your risk of having a blood clot after using HIZENTRA. Also tell your doctor what drugs you are using, as some drugs, such as those that contain the hormone estrogen (for example, birth control pills), may increase your risk of developing a blood clot.

What are possible side effects of HIZENTRA?
The most common side effects with HIZENTRA are:

- Redness, swelling, itching, and/or bruising at the infusion site
- Headache/migraine
- Nausea and/or vomiting
- Pain (including pain in the chest, back, joints, arms, legs)
- Fatigue
- Diarrhea
- Stomach ache/bloating
- Cough, cold or flu symptoms
- Rash (including hives)
- Itching
- Fever and/or chills
- Shortness of breath
- Dizziness
- Fall
- Runny or stuffy nose

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.

Tell your doctor right away if you have any of the following symptoms. They could be signs of a serious problem.

- Reduced urination, sudden weight gain, or swelling in your legs. These could be signs of a kidney problem.
- Pain and/or swelling of an arm or leg with warmth over the affected area, discoloration of an arm or leg, unexplained shortness of breath, chest pain or discomfort that worsens on deep breathing, unexplained rapid pulse, or numbness or weakness on one side of the body. These could be signs of a blood clot.
- Bad headache with nausea, vomiting, stiff neck, fever, and sensitivity to light. These could be signs of a brain swelling called meningitis.
- Brown or red urine, fast heart rate, yellow skin or eyes. These could be signs of a blood problem.
- Chest pains or trouble breathing.
- Fever over 100°F. This could be a sign of an infection.

Tell your doctor about any side effects that concern you. You can ask your doctor to give you more information that is available to healthcare professionals.

Please see full prescribing information, including full boxed warning and FDA-approved patient product information. For more information, 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.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

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How to Share Your Story

By Whitney L. Ward

WHEN YOU have a chronic illness, chances are you will be asked to share your story at some point, whether it be for a medical audience, an inspirational event or even a one-on-one. People love to hear a story about perseverance, and when you live with a chronic disease, your whole life is about persevering and overcoming the odds. But how do you know what aspects of your journey to share? What angle should you take? As someone who has shared her story in many places for many audiences, I have learned a thing or two. I am sharing them with you so that when you are asked to share your own story, you will know how to give a relatable and engaging presentation.

Know your audience. The first important step is to know your audience and adjust your message to fit it. The way you share your journey should not be the same at every venue because not every audience is the same: Different demographics have different experiences, so share the part of your story your audience can relate to.

For example, a group of doctors will not respond well to a presentation created for a teen audience. To illustrate, I was invited to speak to a group of doctors at the hospital where I am a patient, and I discussed my objective perspective on ways a doctor/patient relationship can be a partnership. When I addressed a group of teens, I shared more personal stories about how I worked through missing out on a normal childhood and the many memories and milestones that come along with it. The two messages were crafted with different angles that resonated with each audience so they could actually use and implement its information and encouragement.

Choose details that complement your message. When you have a complex medical history, you have years and years of details that are a part of your story, but not every memory or experience can or should be shared. The best way to choose an anecdote to complement your points is to pick something that people in your audience may have experienced themselves and goes well with your message. For example, doctors can relate to stories about interactions with other doctors, how those interactions are positive or negative, and why. Parents of chronically ill kids can relate to stories about ways my parents prepared me for scary unknowns or painful procedures.

Not every memory or experience can or should be shared.

Have a takeaway and a call to action. Most audiences come to presentations to learn something. They want to know how they can improve in an area or have weapons in their arsenals for certain situations. So, it's very important for your presentation to have at least one takeaway that can go home with them, as well as a call to action they can implement immediately.

Aim for three to four points: With each point, include an anecdote along with practical ways people can improve in that area. To illustrate, when speaking to doctors about how a doctor/patient relationship can be a partnership, my points were to 1) always listen to your patients, 2) give your patients 100 percent autonomy over their bodies, 3) remember patient interactions don't always have to be a teaching moment and 4) always give your patients hope. For each point, I shared an anecdote and tips for how doctors can implement the concept into their doctor/patient relationships.

Balance discouragement with encouragement. Everyone knows that when you have a chronic illness you face many discouraging things, but your audience wants to know there is hope! So, balance the negative with the positive. Assure your audience it's OK to grieve the things they can't do, and then redirect them to the many things they *can* do. It's imperative to give people a purpose and a desire to do MORE!

Still have questions and concerns? Great news! I coach people who need help in this area, and I give people with chronic illnesses a major discount. So, shoot me an email because I would love to work with you!

WHITNEY L. WARD was not only the first person in the world diagnosed with MAGIS syndrome, but she also had the honor of naming the new primary immune deficiency. MAGIS means "more" in Latin, and Whitney hopes to instill in her readers the message they are more than their disease. Find out more about Whitney's journey at www.whitneylaneward.com.

Chores and the Chronically Ill Child

By Jessica Leigh Johnson

CHILDREN BENEFIT in many ways from having regular household chores assigned to them. By performing chores, kids not only learn how to care for themselves, their pets and their surroundings, but they also learn valuable life skills that will follow them into adulthood when they have their own home and family to care for. Plus, completing a chore gives a child a sense of accomplishment and satisfaction in having completed something, even if they didn't necessarily enjoy doing it.[1]

For parents of chronically ill children, it might be tempting to demand less of their kids when it comes to doing chores and taking responsibility for things around the house. Since chronically ill children often face adversity and challenges from a young age, chores may seem like one more burden they don't need. But the truth is, doing chores can actually be beneficial for children's development, and not requiring them to take on age-appropriate responsibilities around the house would be a missed opportunity for growth.[2] In fact, assigning household tasks or chores is one of the best ways parents can help their children with chronic conditions develop independence and self-management skills.[3]

Unfortunately, it is not always easy for parents to find the right balance of work and play for their particular child. Parents may expect less of their chronically ill children compared to other children in the household. They might underestimate their children's ability to work or overestimate the limitations caused by the children's condition.[2]

In a recent study, parents of children ages 6 to 17 years who suffered from at least one chronic condition were asked to fill out surveys regarding their children and how household chores impacted their lives.[2] The study found that:

- 85 percent of the children did chores at least once a week.
- There was a positive correlation between performing household chores and healthcare transition readiness.
- Kids who were encouraged to take on greater responsibilities at home had developed skills that will prepare them to transition to managing their own healthcare.
- Children with chronic conditions who were assigned regular chores showed improved levels of communication with their healthcare provider.[2]

After reviewing these results, it's clear that kids and teens who frequently carry out household chores develop competent life-

management skills and are more prepared to transition to adulthood and take on their own healthcare responsibilities.[2]

Household Chores Foster Normalcy

Encouraging household responsibility is one way parents can help their chronically ill child lead as normal a life as possible.[4] Parents should use their judgment to choose appropriate chores that their chronically ill child can carry out successfully. Just like they would with any child, parents need to be consistent in their requirements, and kids should be prepared to face consequences if chores are not completed.[4] In addition, parents should remember to acknowledge chores that have been completed on time and correctly by offering their children praise and encouragement.[4]

Age-Appropriate Chores

Kids as young as 2 and 3 years old can start handling age-appropriate responsibilities. They can start with simple tasks such as cleaning up toys off the floor and putting them away. They can clean up their spills and wipe down their spot at the table or the tray on their highchair after eating (with parental supervision). As children get older, more responsibilities can be added. Tasks like making the bed each morning, picking out clothes for the day, folding laundry after it's been washed and feeding the pets are very reasonable chores for young children. Some chores will need to be supervised by parents to ensure they're being done properly and safely, like mowing the lawn. Once kids have shown competency in carrying out these simple household tasks, they can start dusting, washing dishes, raking leaves, vacuuming and sweeping, taking out the trash — even washing the car. The list is endless.[5]

Have Realistic Expectations

There will be times that children with chronic illness just don't feel up to completing their household jobs, whether they're sick or just run down from the effects of their treatments. If this is the case, they may procrastinate and not do the job when it is expected and will likely complain about having to do the work.[6] If pain, tiredness or illness is truly the reason for their reluctance, that is completely valid. Parents should be realistic about what they are expecting of their children and give them the benefit of the doubt before it turns into a struggle. Remember, adding stress doesn't help a tired body recover any faster.[5]

When Chores Can Cause More Harm Than Good

Sometimes a child's particular chronic illness makes him or her susceptible to certain allergens or infectious agents. Depending on a family's lifestyle — whether they run a farm and have animals, live in a wooded area with certain types of vegetation nearby or have hobbies that put them in contact with inhalable debris — there are chores that could actually be harmful to children with a particular chronic condition and may put them at risk for complications. Parents need to pay attention to those things that their children need to avoid, and make sure they're not asking them to come in contact with something potentially harmful while helping out around the house and yard.

For example, a child with chronic lung disease should avoid contact with particulate matter, so sweeping out the chimney or emptying the ash bucket from a wood-burning furnace would not be an appropriate chore. Even sweeping up dad's workshop full of sawdust could be harmful to a child with compromised airways. Other children who have compromised immune systems might want to avoid mucking out the chicken coop, as chicken manure has been known to cause fungal infections in the lung such as Histoplasmosis in vulnerable people. (My family learned this lesson the hard way.)

The best way parents can encourage their children to take on regular responsibilities around the house is to set a good example. By having an attitude of thankfulness and appreciation for the things they own — the house, the car, the pets, the food — and instilling in their children the value of caring for those things, parents can foster a healthy attitude toward chores in their children.[5] IG

References

1. Raising Children Network. Household Chores for Children and Teenagers. Raisingchildren.net.au, updated May 4, 2023. Accessed at raisingchildren.net.au/preschoolers/family-life/routines-rituals-rules/chores-for-children.
2. Your Therapy Source. How Do Chores Help Children with Chronic Conditions? July 5, 2020. Accessed at www.yourtherapysource.com/blog1/2020/07/05/children-with-chronic-conditions-and-chores.
3. Richards, J, Nazareth, M, Rak, E, et al. Engagement in Household Chores in Youth with Chronic Conditions: Healthcare Transition Implications. *Occupational Therapy Journal of Research*, 2021;41(1):6-14. Accessed at journals.sagepub.com/doi/full/10.1177/1539449220928142?journalCode=otjb.
4. Long, N, and Zolten, K. How Parents Can Help Their Child Cope with a Chronic Illness. Center for Effective Parenting. Accessed at www.nfmidwest.org/wp-content/uploads/2021/04/coping-with-a-chronic-illness.pdf.
5. Seifert, S. Age Appropriate Chores: How to Help Kids Be Responsible. Focus on the Family. Accessed at www.focusonthefamily.com/parenting/parenting-challenges/motivating-kids-to-clean-up/age-appropriate-chores.
6. Kramer, C. Life with Chronic Illness: Your Kids Can Help. HealthCentral, Sept 14, 2018. Accessed at www.healthcentral.com/slideshow/how-your-kids-can-help-with-your-chronic-illness.

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.

Free Healthcare Resources

By Rachel Maier, MS

ASKING FOR help isn't easy. Some of us feel like we should be able to handle our situation on our own, and that if we ask for help, we'll look weak or incompetent. Others have a need for independence that drives them to do things alone. And, sometimes, a little thing called pride gets in the way of asking for help. But when expenses are extraordinarily high, every penny counts, and finding help — especially when it's free — is priceless.

Maybe you make a decent living, but most of your earnings go toward paying your medical bills. Or perhaps money is already tight, and an unexpected diagnosis makes you wonder how you'll pay for necessary medications, let alone keep food on the table. Whatever your situation, help is available! There are many free resources designed to help you meet your healthcare needs and make your pennies stretch.

Is It Really Free?

We all like free stuff, but the old adage "There's no such thing as a free lunch" can make us a little skeptical when the thing that's free is more substantial than a ballpoint pen or a lollipop. Strings seem to always be attached. There's wisdom in being wary: Websites that claim to offer free stuff can come with a catch or even be a scam in disguise.

Your best bet to vet whether claims are legitimate is to dig a little deeper. Is the organization accredited and widely recognized? Is it a nonprofit with a verifiable tax exemption status? Can you read reviews about the company? Is the company asking you for sensitive personal information that does not seem relevant to the resource? Two tools can help you decide if an organization is trustworthy: 1) Charitynavigator.org, which allows you to access data, tools and resources about a given organization and 2) the IRS tax exemption organization search (www.irs.gov/charities-non-profits/tax-exempt-organization-search).

Another consideration is whether you are a Medicaid or Medicare beneficiary. Both come with benefits that you may not know about or understand how to use. For example, your Medicare or Medicaid plan may have an arrangement with your healthcare facility to offer free transportation to and from medical appointments through Lyft; your Medicare plan may cover the cost-eligible over-the-counter health and wellness supplies (such as vitamins, pain relievers, oral care products, cough, cold and allergy medicine and more); or your Medicare Advantage special needs plan might include a monthly grocery allowance. Check your plan for specifics, and make sure you know how to use the benefit. If you have benefits, you'll want to make sure to use them!

Help, Not Hand Out

Getting something for nothing might feel like it's a hand out, and maybe you don't like the way that feels. Example: Maybe your food budget is taking a hit due to medical expenses and you need a little help putting food on the table. Perhaps the thought of going to a food pantry makes you uncomfortable or embarrassed, but did you know that in 2022, 49 million (or one in six) people in the United States relied on food assistance programs?[1] If you're struggling to feed your family, you aren't alone. Feeding America helps bridge the gap between what you have and what you need by connecting you with free food pantries in your own backyard. (Go to www.feedingamerica.org/find-your-local-foodbank to search for services near you.) Whether you need short-term help or long-term assistance, they can make your load a little bit lighter.

Remember: Everyone needs a little help sometimes, and there are some fantastic folks out there providing services to those who need a boost. I recently was prescribed a costly prescription I could not pay for out of pocket, but with a little research and help from my doctor, I enrolled in a patient assistance program and now the cost of the medicine is completely covered. A family friend faced an unexpected cancer battle she couldn't handle on her own. She used CaringBridge to share her story, and the community rallied around her with help. The Shopping Guide gives some great ideas like these for tools and organizations that can also help you when you have a need you can't cover on your own. IG

References

1. Feeding America. Hunger in America. Accessed at www.feedingamerica.org/hunger-in-america.

RACHEL MAIER, MS, is the associate editor of *IG Living* magazine.

GoodRX

Fast, free and easy to use, GoodRX can help you save up to 80 percent on prescription medications. GoodRX is a free, easy-to-use coupon-based system that can help you pay less for the medicine you need, often costing less than it would when using your insurance. Download the app, then search for the lowest price on your prescriptions, show your GoodRX coupon to the pharmacist and save! The best part? There's absolutely no catch. If you would prefer a physical savings card (instead of using the app), you can submit a request for one to be sent to you through the mail for free (goodrx.com/discount-card). *www.goodrx.com*

Caring Bridge

With CaringBridge, you can start a free, ad-free website to document your health journey to keep your loved ones in the loop. Share one quick update, write a long journal entry, post a photo or upload a video. The website also provides tools for you to communicate what friends can do to help you, from providing a ride or running an errand to helping with childcare or household chores. You can even assign a co-author to help with starting a GoFundMe or setting up a Meal Train. *www.caringbridge.org*

Shopping Guide for Free Healthcare Resources

securing safe food

Securing Safe Food

If your health condition is directly linked to the food you eat, and the cost of your special diet prevents you from sticking to your dietary needs, there's help. Securing Safe Food is a national organization working to make no-cost, allergen-free foods available to families and individuals who need it. By partnering with food pantries committed to the mission of providing a diverse range of nutritionally balanced alternatives for allergen-avoidant families, Securing Safe Food makes life a little easier for those with medical-related dietary restrictions. *www.securingsafefood.org/pantry-locations*

I Am Immunocompromised Card

Keep this card in your wallet to alert emergency medical personnel that you have a rare primary immune deficiency (PI), may be more complicated to treat, and may be sicker than you appear. With space for your name, date of birth, preferred language, PI diagnosis, emergency contact information and more, this card communicates your important medical information when you are not able to. Available instantly as a downloadable pdf, but a hard copy is available for order by patients in the United States. *primaryimmune.org/resources/print-material/i-am-immunocompromised-card?utm_source=newsletter&utm_medium=email&utm_campaign=06232023*

AT3 Center

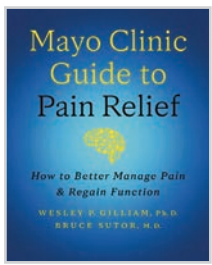
Directory for State AT Act Programs

When your body struggles to perform activities of daily living, assistive technology (AT) devices are a godsend. For information on how to track down free, borrowed or low-cost wheelchairs, power scooters, ramps, adapted kitchen tools or even a robot vacuum, visit the AT3 Center, which connects patients to organizations that offer device demonstrations, loans and acquisitions at little to no cost, as well as options for financing when needed. To find a location near you, click on your state, expand the menu and select the website for your state's AT program. *at3center.net/state-at-programs*

Miracle Flights

MIRACLE FLIGHTS

Miracle Flights provides free commercial airline tickets to pediatric patients in need of life-changing medical care far from home, anywhere in the United States. Qualifying families can receive travel assistance for the child patient and up to two parents or legal guardians. Miracle Flights also provides free flights for individuals to retrieve service dogs and/or attend required service dog training sessions. *miracleflights.org/request-a-flight*



Mayo Clinic Guide to Pain Relief, 3rd Edition: How to Better Manage Pain and Regain Function

Authors: Wesley P. Gilliam, PhD, LP, and Bruce Sutor, MD
Publisher: Mayo Clinic Press

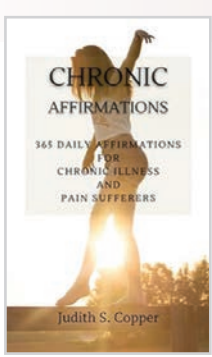
Mayo Clinic Guide to Pain Relief explains how pain develops, how it can become chronic and what can be done to help individuals free themselves from its effects. The book is based on the take-charge pain management program at Mayo Clinic's Pain Rehabilitation Center that has helped thousands of people for more than 40 years. This third edition includes the latest information on pain science and chronic pain management, including the brain's role in the experience of pain; how behaviors and beliefs affect pain; how to shift the focus away from pain and toward a focus on living well; the benefits and risks of medications, injections and other medical treatments; the role of pain specialists, pain clinics and pain rehabilitation programs; and more.

Managing Chronic Pain

Authors: Mary Abraham, MD, and Vandana V. Prakash, MD
Publisher: HarperCollins India

In this book, experienced clinicians Mary Abraham and Vandana V. Prakash uncover the mind-body connection of chronic pain and show how its mental and social processes cannot be separated from the physical. Backed by their years of practice and using case studies, they explain the various relief strategies available for chronic pain — from pharmacological support and interventional procedures to dry needling, and from physical and psychological therapies to complementary and alternative therapies.

New and Useful Reading



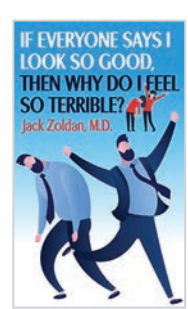
Chronic Affirmations: 365 Daily Affirmations for Chronic Illness and Pain Suffering

Author: Judith S. Copper
Publisher: Independently Published

This book is written to inspire and uplift individuals who are struggling with chronic pain and illness. The daily affirmations provide a powerful tool to help individuals overcome negative thoughts and feelings, boost self-esteem and cultivate a positive and optimistic outlook. Each page offers a unique and encouraging affirmation. Whether readers are dealing with a long-term condition or simply feeling overwhelmed, this book provides the support and guidance needed to help cope with the daily challenges of chronic illness.

If Everyone Says I Look So Good, Then Why Do I Feel So Terrible?: A Book About Fatigue and Health

Author: Jack Zoldan, MD, with Sheldon Reis
Publisher: Independently Published



This book describes unique health insights and concepts from primary care physician Jack Zoldan's 41 years of practice, encountering numerous patients plagued by this baffling ailment. Those grappling with chronic fatigue syndrome will gain new understanding and be rewarded by the wisdom in this easy-to-read book that provides valuable ideas for maintaining lifelong wellness, appealing to both those affected and to individuals keen on safeguarding their health.

Want to Learn About Topics Important to Chronic Illness Patients Living with Autoimmune and Immunodeficiency Disorders?

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Produced by IG Living magazine, written for patients treated with immune globulin therapy and their caregivers

Ataxia Telangiectasia (A-T)
Websites
- A-T Children's Project: www.atcp.org

Chronic Inflammatory Demyelinating-Polyneuropathy (CIDP)
Websites
- GBS/CIDP Foundation International: www.gbs-cidp.org

Evans Syndrome
Online Peer Support
- Rare Connect Evans Syndrome Community Group: www.rareconnect.org/en/community/evans-syndrome/faqs

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

Online Peer Support
- GBS Support Group: www.gaincharity.org.uk
- GBS/CIDP Foundation International Community Forums: forum.gbs-cidp.org

Immune Thrombocytopenia (ITP)
Websites
- ITP Support Association – UK: www.itpsupport.org.uk
- Platelet Disorder Support Association: www.pdsa.org

Kawasaki Disease
Websites
- American Heart Association: www.heart.org/en/health-topics/kawasaki-disease
- American Academy of Family Physicians: www.aafp.org/afp/2006/1001/p1141.html
- Kawasaki Disease Foundation: www.kdfoundation.org
- KidsHealth: www.kidshealth.org/parent/medical/heart/kawasaki.html

Mitochondrial Disease
Websites
- United Mitochondrial Disease Foundation: www.umdf.org
- MitoAction: www.mitoaction.org

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

Multiple Sclerosis (MS)
Websites
- Multiple Sclerosis Association of America: www.mymsaa.org
- Multiple Sclerosis Foundation: www.msfocus.org
- National Multiple Sclerosis Society: www.nationalmssociety.org

Online Peer Support
- Friends with MS: www.FriendsWithMS.com
- MSWorld's Chat and Message Board: www.msworld.org
- Overcoming Multiple Sclerosis: www.overcomingms.org/community

Myasthenia Gravis (MG)
Websites and Chat Rooms
- Myasthenia Gravis Foundation of America (MGFA): www.myasthenia.org
- Myasthenia Gravis Association: mgakc.org

Online Peer Support
- Genetic Alliance: www.geneticalliance.org

Myositis
Websites
- The Myositis Association: www.myositis.org
- International Myositis Assessment and Clinical Studies Group: www.niehs.nih.gov/research/resources/imacs/index.cfm

Online Peer Support
- Juvenile Myositis Family Support Network: www.curejm.org/fsn/index.php
- The Cure JM Foundation: www.curejm.org
- Myositis Association Support Group: www.myositis.org/patient-support/support-groups
- Myositis Support Group – UK: www.myositis.org.uk

Pediatric Autoimmune Neuropsychiatric Disorder Associated with Streptococcus (PANDAS)
Websites
- PANS/PANDAS UK: www.panspandasuk.org
- PANDAS Network: www.pandasnetwork.org
- PANDAS Physician Network Family Resources: www.pandasppn.org/parent-information
- National Institute of Mental Health: www.nimh.nih.gov/health/publications/pandas/index.shtml

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

Peripheral Neuropathy (PN)
Websites
- Neuropathy Action Foundation: www.neuropathyaction.org
- Western Neuropathy Association: www.pnhelp.org
- Neuropathy Alliance of Texas: www.neuropathyalliancetx.org
- The Foundation for Peripheral Neuropathy: www.foundationforpn.com

Primary Immune Deficiency Disease (PI)
Websites
- 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: www.aaaai.org
- International Patient Organisation for Primary Immunodeficiencies (IPOPI) — UK: www.ipopi.org
- Rainbow Allergy-Immunology: www.uhhospitals.org/rainbow/services/pediatric-allergy-and-immunology

Online Peer Support
- IDF Friends: www.idffriends.com
- Jeffrey Modell Foundation Facebook Page: www.facebook.com/JMFworld
- IDF Peer Support Program: www.primaryimmune.org/idf-peer-support-program

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

Online Peer Support
- Scleroderma Support Forum: www.curezone.com/forums/f.asp?=404

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

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