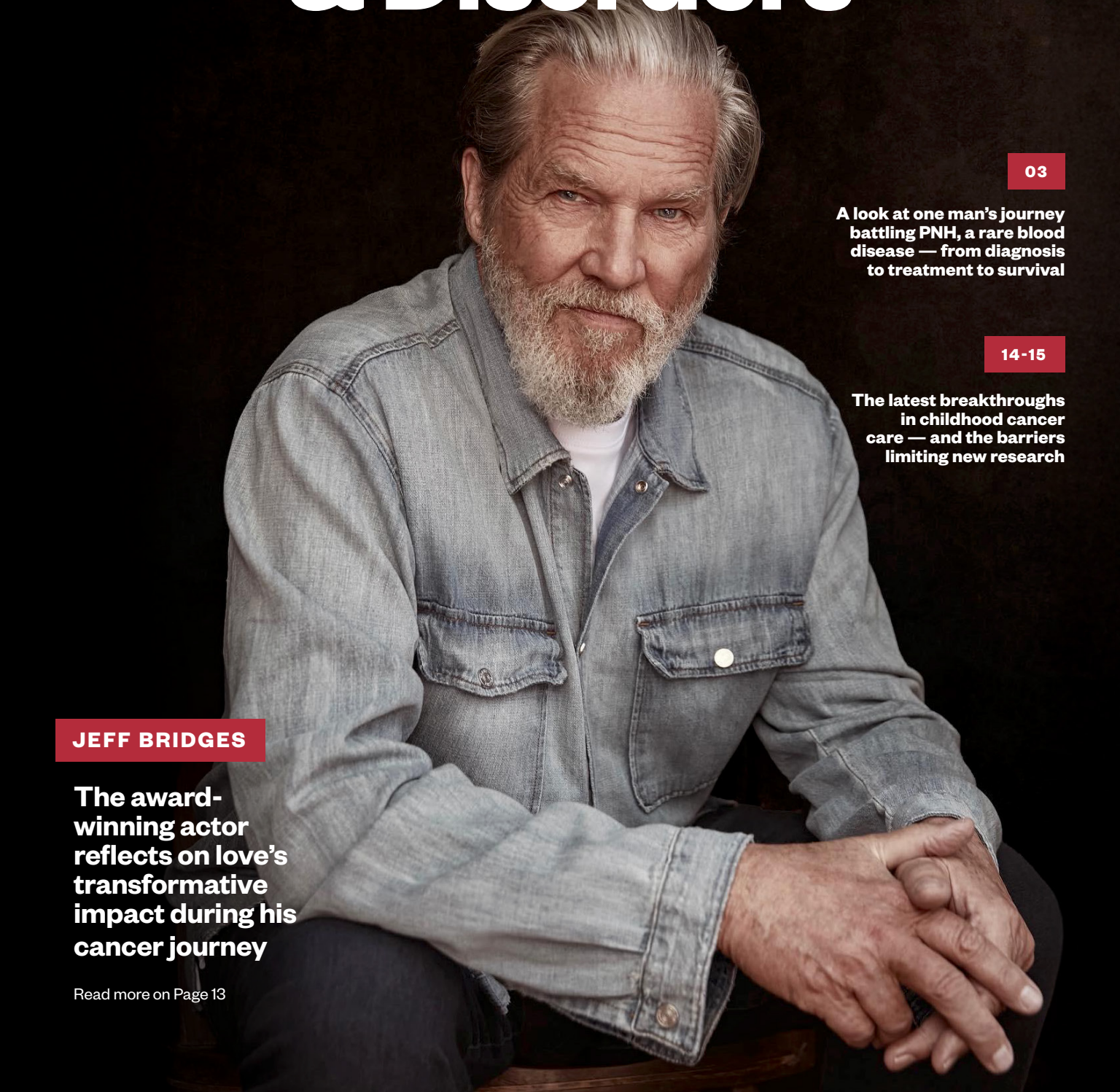
Blood Cancers & Disorders



JEFF BRIDGES

The award-winning actor reflects on love's transformative impact during his cancer journey

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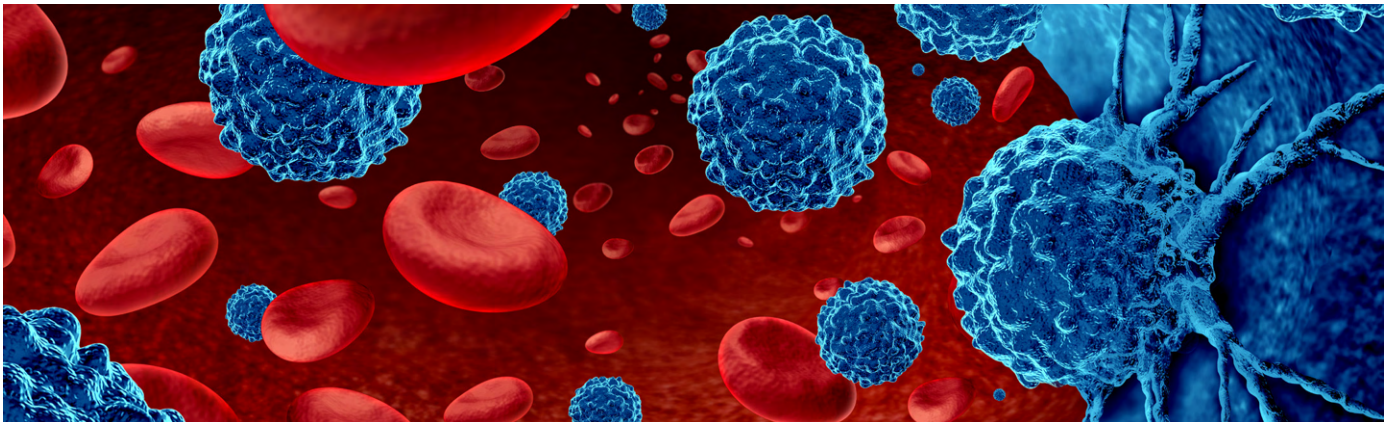


The Pulse of Progress: Blood Cancer Awareness Month

September is dedicated to awareness for blood cancers, life-threatening disorders that impact thousands of Americans and their families.

Each year, more than 180,000 people are diagnosed with blood cancers, such as leukemia, lymphoma, or multiple myeloma. Blood cancers account for 9.4% of all new cancer cases diagnosed in the United States.

The American Society of Hematology (ASH) is made up of over 18,000 hematologists, or doctors who treat blood cancers and other blood disorders. Some of us are clinicians, caring directly for patients, while others are researchers, working to develop the next breakthrough therapy. Some focus on cancers and some on non-cancerous conditions, but all of us are united by a common mission: conquering blood disorders to improve the lives of individuals around the world.

September is Blood Cancer Awareness Month

September is a significant month for ASH, as it is dedicated to raising awareness for blood cancers, which originate in the bone marrow — a spongey material inside the bones that produces blood cells — or the lymphatic system, a part of the body's immune system. These cancers begin when the body overproduces abnormal blood cells, usually the white blood cells that fight off infection. This overproduction can lead to a diagnosis of a form of leukemia, lymphoma, multiple myeloma, myelodysplastic syndrome, or myeloproliferative neoplasm.

Unfortunately, blood cancers are more common than many people realize. In 2024, nearly 1.7 million people in the United States were living with or in remission from a blood cancer. Among children, leukemia is the most common cancer, affecting about 4,000 children each year in the United States and accounting for 1 in 3 pediatric cancer diagnoses.

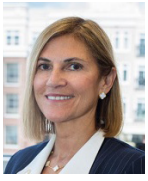
While a person's genetics, age, and ethnicity play a large role in determining whether they'll develop a blood cancer, new research shows that environmental factors play a significant role in triggering these cancers. People who smoke, have previously received cancer treatments, or have been exposed to toxic chemicals in an industry like agriculture, manufacturing, or emergency response are more likely to develop a blood cancer.

Common blood cancer symptoms include chest pain, recurring and unexplained fever, persistent weakness and fatigue, enlarged lymph nodes, and frequent bruising or bleeding. The earlier the diagnosis, the better, so if you suspect that something is awry, talk to your doctor.

Research saves lives

While some blood cancers require more involved treatment than others, common therapies include chemotherapy, radiation, immunotherapy, or bone marrow transplant. Many of these advances have been made possible through support from the National Institutes of Health (NIH), the world's largest public funder of biomedical research and a key driver of discoveries that have transformed care and saved lives.

The progress we've made in treating blood cancers is significant, but it depends on sustained, robust funding for the NIH. ASH is committed to advancing progress and championing the importance of the NIH to ensure patients with blood disorders around the world continuing to see improved outcomes. You can make your voice heard and advocate for continued investment and progress in lifesaving research by joining ASH's #Fight4Hematology.



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Finding His Way Through: **Evan's Story of PNH**

Meet Evan. In a recent conversation with the Aplastic Anemia and MDS International Foundation, he shared his journey from paroxysmal nocturnal hemoglobinuria (PNH) diagnosis through treatment and survival.

Evan | Photos courtesy of Aplastic Anemia and MDS International Foundation

Evan, please tell us a little about yourself.

I'm 31 years old. I'm based in New York City, and I've been living here for around eight years, after graduating from college. I'm working, enjoying life, spending time with friends, and just moving along.

What was your life like before the diagnosis of PNH?

I was biking a lot and extremely active. I had career goals, friends, and was planning on traveling. Pre-PNH, I had a different outlook on what I wanted to do and what goals I wanted to set physically, mentally, and professionally.

What led to the diagnosis?

I went to a Yankees game with my father and my friend. My father is in the medical field; he looked at me and recognized that my coloring looked "off." I kept biking 20 miles daily while I had a blood test and waited for the results.

When the blood test came back, I found out my hemoglobin was under five! During that week, with more testing, I found out I had PNH. I'm extremely lucky to have found out in that short period of time.

It was a very scary time, and a lot of things were happening at once. I found out I had two blood clots in and above my leg, and I thought it was just an injury from biking or exercise.

When you get diagnosed with any disease that is extremely serious or rare, it's a lot of information at once. You're stressed about dealing with the diagnosis, let alone processing everything that's going on and being able to do research.

Even though the medical team gave me information about PNH, I learned more when I got connected with someone else in the community. I was able to talk to him in detail about PNH, what it was, what I can and can't do, how he's surviving, and how he's living his life currently.

Since you've been on treatment, how has life changed?

For me, it's the little things. I prioritize resting and relaxing because I have weddings, bachelor parties, and work events. I'm balancing my energy to go to those and managing everyone's expectations.

I still have the same ambitions and goals that I had before PNH, but it's just going to take longer to do them all. Accepting that was the hardest thing.

Self-Advocacy Helped Me Navigate My Rare Disease

Diagnosed at a young age with a rare and potentially life-threatening disease, one man learned the power of taking control.

Roger | Photo courtesy of Alexion, AstraZeneca Rare Disease

When South Carolinian Roger was diagnosed with paroxysmal nocturnal hemoglobinuria (PNH) at age 26, he was overwhelmed. "Being diagnosed with PNH was shocking," he explained. "It was life-altering. In the beginning, I didn't really know how to prioritize my needs and my health. I was desperate for educational information."

PNH is a rare disease that causes the body's own immune system to attack and destroy red blood cells. It is an acquired disease, which means you don't inherit it. Caused by a mutation in the body's stem cells, it affects about 12-13 people per million each year. "When I was diagnosed back in 2011, there weren't a lot of treatment options," Roger explained. "But you've got to take some action. You can't just sit by and let it take control."

For Roger, that meant leaning into self-advocacy around managing his PNH. And that has made all the difference.

Self-advocacy

Initially, Roger was treated with blood thinners and steroids — but managing his condition was stressful and destabilizing, especially as a young man with an active life between work, family and friends and hobbies like golf and playing the guitar and piano. "I was getting frustrated," he said. "I was tired of taking pills twice, three times a day. I said, 'enough is enough.'"

With the help of his family — especially his mother, who actively researched PNH and available treatment options — Roger identified a less-frequent treatment plan to address his disease. But he felt trapped in a retail job that allowed him the time off he needed to manage his health but left him feeling unfulfilled.

"It was easy to take off time working in retail," he explained, "but I wanted to advance my career further and have more freedom."

Roger and his family continued to educate themselves and track the development of new PNH treatments. That paid off for Roger in 2018 when he asked his doctor about ULTOMIRIS® (ravulizumab-cwvz), an FDA-approved medicine used to treat adults and children 1 month of age and older with PNH.

Sustained disease control

ULTOMIRIS is designed to work by targeting one of the immune system proteins that is attacking red blood cells in the body. Two weeks after an initial loading dose, ULTOMIRIS is infused intravenously every eight weeks for most people. The most common side effects of ULTOMIRIS are upper respiratory tract infection and headache. Roger and his doctor discussed the benefits and risks of treatment including that he would need meningococcal vaccinations at least two weeks before his first dose of ULTOMIRIS due to the risk of serious meningococcal infections. Roger liked that ULTOMIRIS allowed him more time between infusions, which fit his lifestyle and career goals better. "I left retail and pursued a more professional role in staffing and recruiting, which I'm passionate about," he explained. "With ULTOMIRIS, I'm able to really plan my life, my free time, my vacations. I'm able to schedule things that I previously couldn't. It gave me a real sense of freedom. This was my own experience and may be different for others."

Roger credits the doctors and nurses he's worked with over the years for the success of his current care plan — but also himself and his proactive push to ask questions, initiate conversations, and advocate for himself.

"For me, self-advocacy is really taking back the control that you feel you lost with the diagnosis," he said. "The first thing I would say to someone facing a diagnosis like this is, life is not over. There is hope. Find the right doctors, but you're going to have to do some of the work on your own. You can do it."

Written by **Jeff Somers**

To learn more, visit **ultomiris.com/pnh**

ALEXION® AstraZeneca Rare Disease

IMPORTANT SAFETY INFORMATION

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

ULTOMIRIS is a medicine that affects your immune system and may lower the ability of your immune system to fight infections.

- **ULTOMIRIS increases your chance of getting serious meningococcal infections that may quickly become life-threatening or cause death if not recognized and treated early.**

  1. You must complete or update meningococcal vaccine(s) at least 2 weeks before your first dose of ULTOMIRIS.
  2. If you have not completed your meningococcal vaccines and ULTOMIRIS must be started right away, you should receive the required vaccine(s) as soon as possible.
  3. If you have not been vaccinated and ULTOMIRIS must be started right away, you should also receive antibiotics for as long as your healthcare provider tells you.
  4. If you had a meningococcal vaccine in the past, you might need additional vaccines before starting ULTOMIRIS. Your healthcare provider will decide if you need additional meningococcal vaccines.
  5. Meningococcal vaccines do not prevent all meningococcal infections. **Call your healthcare provider or get emergency medical care right away if you get any of these signs and symptoms of a meningococcal infection**: fever, fever with high heart rate, headache and fever, confusion, muscle aches with flu-like symptoms, fever and a rash, headache with nausea or vomiting, headache with a stiff neck or stiff back, or eyes sensitive to light.

Your healthcare provider will give you a Patient Safety Card about the risk of serious meningococcal infection. Carry it with you at all times during treatment and for 8 months after your last ULTOMIRIS dose. Your risk of meningococcal infection may continue for several months after your last dose of ULTOMIRIS. It is important to show this card to any healthcare provider who treats you. This will help them diagnose and treat you quickly.

ULTOMIRIS is only available through a program called the ULTOMIRIS and SOLIRIS Risk Evaluation and Mitigation Strategy (REMS). Before you can receive ULTOMIRIS, your healthcare provider must: enroll in the REMS program; counsel you about the risk of serious meningococcal infections; give you information about the signs and symptoms of serious meningococcal infection; make sure that you are vaccinated against serious infections caused by meningococcal bacteria, and that you receive antibiotics if you need to start ULTOMIRIS right away and are not up to date on your vaccines; give you a **Patient Safety Card** about your risk of meningococcal infection.

ULTOMIRIS may also increase the risk of other types of serious infections, including *Streptococcus pneumoniae*, *Haemophilus influenzae*, and *Neisseria gonorrhoeae*. Your child should receive vaccines against *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib) if treated with ULTOMIRIS. Certain people may be at risk of serious infections with gonorrhea.

Who should not receive ULTOMIRIS?
Do not receive ULTOMIRIS if you have a serious meningococcal infection when you are starting ULTOMIRIS.

Before you receive ULTOMIRIS, tell your healthcare provider about all of your medical conditions, including if you:

- have an infection or fever
- are pregnant or plan to become pregnant. It is not known if ULTOMIRIS will harm your unborn baby.
  - Pregnancy Registry: There is a registry for pregnant women who take ULTOMIRIS to check the health of the pregnant mother and her baby. If you are pregnant or become pregnant while taking ULTOMIRIS, talk to your healthcare provider about how you can join this registry or you may contact the registry at 1-833-793-0563 or www.UltomirisPregnancyStudy.com to enroll.
- are breastfeeding or plan to breastfeed. It is not known if ULTOMIRIS passes into your breast milk. You should not breastfeed during treatment and for 8 months after your final dose of ULTOMIRIS.

Tell your healthcare provider about all the vaccines you receive and medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements which could affect your treatment.

If you have PNH and you stop receiving ULTOMIRIS, your healthcare provider will need to monitor you closely for at least 16 weeks after you stop ULTOMIRIS. Stopping ULTOMIRIS may cause breakdown of your red blood cells due to PNH. Symptoms or problems that can happen due to red blood cell breakdown include: drop in your red blood cell count, tiredness, blood in your urine, stomach-area (abdomen) pain, shortness of breath, blood clots, trouble swallowing, and erectile dysfunction (ED) in males.

What are the possible side effects of ULTOMIRIS?

ULTOMIRIS can cause serious side effects including infusion-related reactions. Symptoms of an infusion-related reaction with ULTOMIRIS may include lower back pain, stomach (abdominal) pain, muscle spasms, changes in blood pressure, tiredness, feeling faint, shaking chills (rigors), discomfort in your arms or legs, or bad taste. Stop treatment of ULTOMIRIS and tell your healthcare provider right away if you develop these symptoms, or any other symptoms during your ULTOMIRIS infusion that may mean you are having a serious infusion-related reaction, including: chest pain, trouble breathing or shortness of breath, swelling of your face, tongue, or throat, and feel faint or pass out.

The most common side effects of ULTOMIRIS in people treated for PNH are upper respiratory tract infection and headache.

Tell your healthcare provider about any side effect that bothers you or that does not go away. These are not all the possible side effects of ULTOMIRIS. For more information, ask your healthcare provider or pharmacist. Call your healthcare provider right away if you miss an ULTOMIRIS infusion or for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

INDICATION

What is ULTOMIRIS?

ULTOMIRIS is a prescription medicine used to treat adults and children 1 month of age and older with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH). It is not known if ULTOMIRIS is safe and effective in children younger than 1 month of age.

Please see full Prescribing Information and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious meningococcal infections.

ULTOMIRIS® (ravulizumab-cwvz) INJECTION, FOR INTRAVENOUS USE
BRIEF SUMMARY OF FULL PRESCRIBING INFORMATION

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS
See full prescribing information for complete boxed warning.

ULTOMIRIS increases the risk of serious and life-threatening infections caused by *Neisseria meningitidis*.

- **Complete or update meningococcal vaccination at least 2 weeks prior to the first dose of ULTOMIRIS, unless the risks of delaying ULTOMIRIS outweigh the risks of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients receiving a complement inhibitor. (5.1)**
- **Patients receiving ULTOMIRIS are at increased risk for invasive disease caused by *N. meningitidis*, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of meningococcal infections and evaluate immediately if infection is suspected. (5.1)**

ULTOMIRIS is available only through a restricted program called ULTOMIRIS and SOLIRIS REMS. (5.2)

1 INDICATIONS AND USAGE

1.1 Paroxysmal Nocturnal Hemoglobinuria

ULTOMIRIS is indicated for the treatment of adult and pediatric patients one month of age and older with paroxysmal nocturnal hemoglobinuria (PNH).

4 CONTRAINDICATIONS

ULTOMIRIS is contraindicated for initiation in patients with unresolved serious *Neisseria meningitidis* infection *[see Warnings and Precautions (5.1)]*.

5 WARNINGS AND PRECAUTIONS

5.1 Serious Meningococcal Infections

ULTOMIRIS, a complement inhibitor, increases a patient's susceptibility to serious, life-threatening, or fatal infections caused by meningococcal bacteria (septicemia and/or meningitis) in any serogroup, including non-groupable strains. Life-threatening and fatal meningococcal infections have occurred in both vaccinated and unvaccinated patients treated with complement inhibitors. The initiation of ULTOMIRIS treatment is contraindicated in patients with unresolved serious *Neisseria meningitidis* infection.

Complete or update meningococcal vaccination (for serogroups A, C, W, Y and B) at least 2 weeks prior to administration of the first dose of ULTOMIRIS, according to current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of ULTOMIRIS therapy. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent ULTOMIRIS therapy is indicated in a patient who is not up to date with meningococcal vaccines according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer meningococcal vaccines as soon as possible. Various durations and regimens of antibacterial drug prophylaxis have been considered, but the optimal durations and drug regimens for prophylaxis and their efficacy have not been studied in unvaccinated or vaccinated patients receiving complement inhibitors, including ULTOMIRIS. The benefits and risks of treatment with ULTOMIRIS, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by *Neisseria meningitidis*.

Vaccination does not eliminate the risk of meningococcal infections, despite development of antibodies following vaccination.

Closely monitor patients for early signs and symptoms of meningococcal infection and evaluate patients immediately if infection is suspected. Inform patients of these signs and symptoms and instruct patients to seek immediate medical care if these signs and symptoms occur. Promptly treat known infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider interruption of ULTOMIRIS in patients who are undergoing treatment for serious meningococcal infection, depending on the risks of interrupting treatment in the disease being treated.

ULTOMIRIS is available only through a restricted program under a REMS *[see Warnings and Precautions (5.2)]*.

5.2 ULTOMIRIS and SOLIRIS REMS

ULTOMIRIS is available only through a restricted program under a REMS called ULTOMIRIS and SOLIRIS REMS, because of the risk of serious meningococcal infections *[see Warnings and Precautions (5.1)]*.

Notable requirements of the ULTOMIRIS and SOLIRIS REMS include the following:

- Prescribers must enroll in the REMS.
- Prescribers must counsel patients about the risk of serious meningococcal infection.
- Prescribers must provide the patients with the REMS educational materials.
- Prescribers must assess patient vaccination status for meningococcal vaccines (against serogroups A, C, W, Y, and B) and vaccinate if needed according to current ACIP recommendations two weeks prior to the first dose of ULTOMIRIS.
- Prescribers must provide a prescription for antibacterial drug prophylaxis if treatment must be started urgently and the patient is not up to date with meningococcal vaccines according to current ACIP recommendations at least two weeks prior to the first dose of ULTOMIRIS.
- Healthcare settings and pharmacies that dispense ULTOMIRIS must be certified in the REMS and must verify prescribers are certified.
- Patients must receive counseling from the prescriber about the need to receive meningococcal vaccines per ACIP recommendations, the need to take antibiotics as directed by the prescriber, and the signs and symptoms of meningococcal infection.
- Patients must be instructed to carry the Patient Safety Card with them at all times during and for 8 months following treatment with ULTOMIRIS.

Further information is available at www.UltSolREMS.com or 1-888-765-4747.

5.3 Other Infections

Serious infections with Neisseria species (other than *Neisseria meningitidis*), including disseminated gonococcal infections, have been reported.

ULTOMIRIS blocks terminal complement activation; therefore, patients may have increased susceptibility to infections, especially with encapsulated bacteria, such as infections caused by *Neisseria meningitidis* but also *Streptococcus pneumoniae*, *Haemophilus influenzae*, and to a lesser extent, *Neisseria gonorrhoeae*. Children treated with ULTOMIRIS may be at increased risk of developing serious infections due to *Streptococcus pneumoniae* and *Haemophilus influenzae type b* (Hib). Administer vaccinations for the prevention of *Streptococcus pneumoniae* and *Haemophilus influenzae type b* (Hib) infections according to ACIP recommendations. Patients receiving ULTOMIRIS are at increased risk for infections due to these organisms, even if they develop antibodies following vaccination.

5.4 Monitoring Disease Manifestations after ULTOMIRIS Discontinuation

Treatment Discontinuation for PNH

After discontinuing treatment with ULTOMIRIS, closely monitor for signs and symptoms of hemolysis, identified by elevated lactate dehydrogenase (LDH) along with sudden decrease in PNH clone size or hemoglobin, or reappearance of symptoms such as fatigue, hemoglobinuria, abdominal pain, shortness of breath (dyspnea), major adverse vascular event (including thrombosis), dysphagia, or erectile dysfunction. Monitor any patient who discontinues ULTOMIRIS for at least 16 weeks to detect hemolysis and other reactions. If signs and symptoms of hemolysis occur after discontinuation, including elevated LDH, consider restarting treatment with ULTOMIRIS.

5.5 Thromboembolic Event Management

The effect of withdrawal of anticoagulant therapy during ULTOMIRIS treatment has not been established. Therefore, treatment with ULTOMIRIS should not alter anticoagulant management.

5.6 Infusion-Related Reactions

Administration of ULTOMIRIS may result in systemic infusion-related reactions, including anaphylaxis *[see Adverse Reactions (6.2)]* and hypersensitivity reactions. In clinical trials, infusion-related reactions occurred in approximately 1 to 7% of patients treated with ULTOMIRIS *[see Adverse Reactions (6.1)]*. These events included lower back pain, abdominal pain, muscle spasms, drop in blood pressure, elevation in blood pressure, rigors, limb discomfort, drug hypersensitivity (allergic reaction), and dysgeusia (bad taste). These reactions did not require discontinuation of ULTOMIRIS. If signs of cardiovascular instability or respiratory compromise occur, interrupt ULTOMIRIS infusion and institute appropriate supportive measures.

6 ADVERSE REACTIONS

The following clinically significant adverse reactions are discussed in greater detail in other sections of the labeling:
Serious Meningococcal Infections *[see Warnings and Precautions (5.1)]*
Other Infections *[see Warnings and Precautions (5.3)]*
Infusion-Related Reactions *[see Warnings and Precautions (5.6)]*

6.1 Clinical Trial Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

Paroxysmal Nocturnal Hemoglobinuria (PNH)

Adult Population with PNH Treated with ULTOMIRIS

The data described below reflect exposure of 441 adult patients with PNH in Phase 3 studies who received ULTOMIRIS (n = 222) or eculizumab (n = 219) at the recommended dosing regimens with median treatment duration of 6 months for ULTOMIRIS and 6 months for eculizumab. The most frequent adverse reactions (≥ 10%) with ULTOMIRIS were upper respiratory tract infection and headache. Table 10 describes adverse reactions that occurred at a rate of 5% or more among patients treated with ULTOMIRIS in PNH studies.

Serious adverse reactions were reported in 15 (6.8%) patients with PNH receiving ULTOMIRIS. The serious adverse reactions in patients treated with ULTOMIRIS included hyperthermia and pyrexia. No serious adverse reaction was reported in more than 1 patient treated with ULTOMIRIS.

One fatal case of sepsis was identified in a patient treated with ULTOMIRIS.

Table 10: Adverse Reactions Reported in 5% or More of ULTOMIRIS-Treated Patients in Complement Inhibitor Naïve and Eculizumab-Experienced Adult Patients with PNH

Body System Adverse Reaction	Number of Patients	
	ULTOMIRIS (N=222) n (%)	Eculizumab (N=219) n (%)
Gastrointestinal Disorders		
Diarrhea	19 (9)	12 (5)
Nausea	19 (9)	19 (9)
Abdominal pain	13 (6)	16 (7)
General disorders and administration site conditions		
Pyrexia	15 (7)	18 (8)
Infections and infestations		
Upper respiratory tract infection[a]	86 (39)	86 (39)
Musculoskeletal and connective tissue disorders		
Pain in extremity	14 (6)	11 (5)
Arthralgia	11 (5)	12 (5)
Nervous system disorders		
Headache	71 (32)	57 (26)
Dizziness	12 (5)	14 (6)

[a] Grouped term includes: nasopharyngitis, upper respiratory tract infection, oropharyngeal pain, viral upper respiratory tract infection, rhinitis, respiratory tract infection, rhinorrhea, pharyngitis, and upper respiratory tract inflammation

Clinically relevant adverse reactions in 1% of patients include infusion-related reactions.

Pediatric Population with PNH Treated with ULTOMIRIS

In pediatric patients with PNH (aged 9 to 17 years old) included in the pediatric PNH Phase 3 study, the safety profile appeared similar to that observed in adult patients with PNH. The most common adverse reactions (> 20%) were upper respiratory tract infection, anemia, abdominal pain, and headache. Table 11 describes the adverse reactions that occurred at a rate of 10% or more among pediatric patients treated with ULTOMIRIS in Study ALXN1210-PNH-304.

Table 11: Adverse Reactions Reported in 10% or More of ULTOMIRIS-Treated Pediatric Patients with PNH in Study ALXN1210-PNH-304

Body System Adverse Reaction	Treatment Naïve (N=5)	Eculizumab Experienced (N=8)	Total (N=13)
	n (%)	n (%)	n (%)
Blood and lymphatic system disorders			
Anemia[a]	1 (20)	2 (25)	3 (23)
Gastrointestinal disorders			
Abdominal pain	0 (0)	3 (38)	3 (23)
Constipation	0 (0)	2 (25)	2 (15)
General disorders and administration site conditions			
Pyrexia	1 (20)	1 (13)	2 (15)
Infections and infestations			
Upper Respiratory tract infection[b]	1 (20)	6 (75)	7 (54)
Musculoskeletal and connective tissue disorders			
Pain in extremity	0 (0)	2 (25)	2 (15)
Nervous system disorders			
Headache	1 (20)	2 (25)	3 (23)

[a] Grouped term includes: anemia and iron deficiency anemia

[b] Grouped term includes: nasopharyngitis, upper respiratory tract infection, oropharyngeal pain, and viral upper respiratory tract infection

ULTOMIRIS® (ravulizumab-cwvz) INJECTION, FOR INTRAVENOUS USE
BRIEF SUMMARY OF FULL PRESCRIBING INFORMATION (cont'd)

6.2 Postmarketing Experience
The following adverse reactions have been identified during post-approval use of ULTOMIRIS. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to ULTOMIRIS exposure.

Adverse Reactions from Postmarketing Spontaneous Reports
• Anaphylaxis *[see Warnings and Precautions (5.6)]*

7 DRUG INTERACTIONS
7.1 Plasma Exchange, Plasmapheresis, and Intravenous Immunoglobulins
Concomitant use of ULTOMIRIS with plasma exchange (PE), plasmapheresis (PP), or intravenous immunoglobulin (IVIg) treatment can reduce serum ravulizumab concentrations and requires a supplemental dose of ULTOMIRIS *[see Dosage and Administration (2.5)]*.

7.2 Neonatal Fc Receptor Blockers
Concomitant use of ULTOMIRIS with neonatal Fc receptor (FcRn) blockers (e.g., efgartigimod) may lower systemic exposures and reduce effectiveness of ULTOMIRIS.

Closely monitor for reduced effectiveness of ULTOMIRIS.

8 USE IN SPECIFIC POPULATIONS
8.1 Pregnancy
Pregnancy Exposure Registry
There is a pregnancy exposure registry that monitors pregnancy outcomes in women exposed to ULTOMIRIS during pregnancy. Healthcare providers and patients may call 1-833-793-0563 or go to www.UltomirisPregnancyStudy.com to enroll in or to obtain information about the registry.

Risk Summary
There are no available data on ULTOMIRIS use in pregnant women to inform a drug-associated risk of major birth defects, miscarriage, or adverse maternal or fetal outcomes. There are risks to the mother and fetus associated with untreated PNH in pregnancy *(see Clinical Considerations)*. Animal studies using a mouse analogue of the ravulizumab-cwvz molecule (murine anti-mouse complement component 5 [C5] antibody) showed increased rates of developmental abnormalities and an increased rate of dead and moribund offspring at doses 0.8-2.2 times the human dose *(see Data)*.

The estimated background risk of major birth defects and miscarriage for the indicated populations is unknown. All pregnancies have a background risk of birth defect, loss, or other adverse outcomes. In the U.S. general population, the estimated background risk of major birth defects and miscarriage in clinically recognized pregnancies is 2-4% and 15-20%, respectively.

Clinical Considerations
Disease-associated Maternal and/or Fetal/neonatal Risk
PNH in pregnancy is associated with adverse maternal outcomes, including worsening cytopenias, thrombotic events, infections, bleeding, miscarriages, and increased maternal mortality, and adverse fetal outcomes, including fetal death and premature delivery.

Data
Animal Data
Animal reproduction studies were conducted in mice using doses of a murine anti-C5 antibody that approximated 1-2.2 times (loading dose) and 0.8-1.8 times (maintenance dose) the recommended human ULTOMIRIS dose, based on a body weight comparison. When animal exposure to the antibody occurred in the time period from before mating until early gestation, no decrease in fertility or reproductive performance was observed. When maternal exposure to the antibody occurred during organogenesis, 2 cases of retinal dysplasia and 1 case of umbilical hernia were observed among 230 offspring born to mothers exposed to the higher antibody dose; however, the exposure did not increase fetal loss or neonatal death. When maternal exposure to the antibody occurred in the time period from implantation through weaning, a higher number of male offspring became moribund or died (1/25 controls, 2/25 low dose group, 5/25 high dose group). Surviving offspring had normal development and reproductive function. Human IgG are known to cross the human placental barrier, and thus ULTOMIRIS may potentially cause terminal complement inhibition in fetal circulation.

8.2 Lactation
Risk summary
There are no data on the presence of ravulizumab-cwvz in human milk, the effect on the breastfed child, or the effect on milk production. Since many medicinal products and immunoglobulins are secreted into human milk, and because of the potential for serious adverse reactions in a nursing child, breastfeeding should be discontinued during treatment and for 8 months after the final dose.

8.4 Pediatric Use
The safety and effectiveness of ULTOMIRIS for the treatment of PNH have been established in pediatric patients aged one month and older. Use of ULTOMIRIS for this indication is supported by evidence from adequate and well-controlled trials in adults with additional pharmacokinetic, efficacy and safety data in pediatric patients aged 9 to 17 years *[see Adverse Reactions (6.1), Clinical Pharmacology (12.3), and Clinical Studies (14.1)]*. The safety and efficacy for the treatment of pediatric and adult patients with PNH appear similar. Use of ULTOMIRIS in pediatric patients with PNH aged less than 9 years and body weight < 30 kg is based on extrapolation of pharmacokinetic / pharmacodynamic (PK/PD), and efficacy and safety data from PNH clinical studies *[see Clinical Pharmacology (12.3) and Clinical Studies (14)]*.

8.5 Geriatric Use
Clinical studies of ULTOMIRIS did not include sufficient numbers of subjects aged 65 and over (58 patients with PNH) to determine whether they respond differently from younger subjects.

Other reported clinical experience has not identified differences in responses between elderly and younger patients.

17 PATIENT COUNSELING INFORMATION
Advise the patients and/or caregivers to read the FDA-approved patient labeling (Medication Guide).

Serious Meningococcal Infections

Advise patients of the risk of serious meningococcal infection. Inform patients of the need to complete or update their meningococcal vaccinations at least 2 weeks prior to receiving the first dose of ULTOMIRIS or receive antibacterial drug prophylaxis if ULTOMIRIS treatment must be initiated immediately and they have not been previously vaccinated. Inform patients of the requirement to be revaccinated according to current ACIP recommendations for meningococcal infection while on ULTOMIRIS therapy *[see Warnings and Precautions (5.1)]*.

Inform patients that vaccination may not prevent serious meningococcal infection and to seek immediate medical attention if the following signs or symptoms occur *[see Warnings and Precautions (5.1)]*:

- fever
- fever and a rash
- headache with nausea or vomiting
- fever with high heart rate
- headache and a fever
- headache with a stiff neck or stiff back
- confusion
- muscle aches with flu-like symptoms
- eyes sensitive to light

Inform patients that they will be given a Patient Safety Card for ULTOMIRIS that they should carry with them at all times during and for 8 months following treatment with ULTOMIRIS. This card describes symptoms which, if experienced, should prompt the patient to immediately seek medical evaluation.

ULTOMIRIS and SOLIRIS REMS
ULTOMIRIS is available only through a restricted program called ULTOMIRIS and SOLIRIS REMS *[see Warnings and Precautions (5.2)]*.

Inform the patient of the following notable requirements:

- Patients must receive counseling about the risk of serious meningococcal infections.
- Patients must receive written educational materials about this risk.
- Patients must be instructed to carry the Patient Safety Card with them at all times during and for 8 months following treatment with ULTOMIRIS.
- Patients must be instructed to complete or update meningococcal vaccines for serogroups A, C, W, Y and B per ACIP recommendations as directed by the prescriber prior to treatment with ULTOMIRIS.
- Patients must receive antibiotics as directed by the prescriber if they are not up to date with meningococcal vaccines and have to start ULTOMIRIS right away.

Other Infections
Counsel patients of the increased risk of infections, particularly those due to encapsulated bacteria, especially *Neisseria* species. Advise patients of the need for vaccination against meningococcal infections according to current medical guidelines. Counsel patients about gonorrhea prevention and advise regular testing for patients at risk. Advise patients to report any new signs and symptoms of infection.

Discontinuation
Inform patients with PNH that they may develop serious hemolysis when ULTOMIRIS is discontinued and that they will be monitored by their healthcare professional for at least 16 weeks for PNH following ULTOMIRIS discontinuation *[see Warnings and Precautions (5.4)]*.

Inform patients who discontinue ULTOMIRIS to keep the Patient Safety Card with them for 8 months after the last ULTOMIRIS dose, because the increased risk of meningococcal infection persists for several months following discontinuation of ULTOMIRIS.

Infusion-Related Reactions
Advise patients that administration of ULTOMIRIS may result in infusion-related reactions *[see Warnings and Precautions (5.6)]*.

Pregnancy Exposure Registry
There is a pregnancy exposure registry that monitors pregnancy outcomes in women exposed to ULTOMIRIS during pregnancy. Encourage participation and advise patients about how they may enroll in the registry *[see Use in Specific Populations (8.1)]*.

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ALEXION®
AstraZeneca Rare Disease

A Thriving Research Lab, Like Healthy Bone Marrow: How Simón Méndez-Ferrer Is Advancing Myelofibrosis Research

The bone marrow is a bustling neighborhood where red, white, and platelet cells mature. However, when communication is thrown off balance, blood production falters, leading to serious disorders and cancer. One such disorder is myelofibrosis (MF), a disease in which the overproduction of fibrous scar tissue cripples bone marrow functioning.

INTERVIEW WITH **Simón Méndez-Ferrer, Ph.D.** Recipient, 2024 MPN Challenge Award

Professor Simón Méndez-Ferrer, a 2024 MPN Challenge award recipient, is a leading authority in MF research, exploring how the bone marrow's environment shifts from supporting healthy blood production to driving disease. However, he's also an expert in fostering a different kind of healthy environment: a thriving research laboratory built to fight blood cancer.

The environment shapes the disease

Bone marrow niches are composed of blood vessels, bone tissue, innervating nerves, and connective tissue, which create pocket environments where stem cells produce blood cells.

"We work at the interface between organs — how these organs communicate and how the environment where stem cells live is controlled," Professor Méndez-Ferrer explained.

> The No. 1 driver for basic scientists is the **joy of discovery**.

His work challenges the longstanding approach that MFs arise solely from genetic mutations in blood stem cells. Instead, his findings demonstrate that the surrounding bone microenvironment (BME) plays a crucial role in either fueling disease or sustaining health.

"This place where stem cells reside can be manipulated in a positive way to prevent the development of cancer or improve treatment," Dr. Méndez-Ferrer said.

Diversity aids production

Much like a healthy bone marrow, where different players work together to maintain balance, Dr. Méndez-Ferrer's research team thrives on collaboration. His laboratory brings together specialists in hematology, molecular biology, immunology, and vascular biology, blending expertise to tackle complex questions from multiple angles.

"It's a very exciting ingredient — people coming from different backgrounds, different ways of seeing life and behaving," he said. This diversity fuels the maturation of ideas and even students in a supportive environment.

When asked which bone marrow cell best described himself, Dr. Méndez-Ferrer did not hesitate with his response: "Definitely mesenchymal stem cell. You know, that's the key cell." Like these lynchpins of the BME, his role as the lead investigator of a laboratory extends to managing talented individuals, fostering communication within his team and beyond, and adapting.

Where questions proliferate

For Dr. Méndez-Ferrer, scientific progress is not just about following a rigid path; it's about correcting his course based on new insights. His team is driven by curiosity, a necessity in a field where unexpected results can redefine entire research directions.

"The No. 1 driver for basic scientists is the joy of discovery," he said. "That can't happen every day, but when you come to understand a question you've been struggling with for years, that is indescribable."

Written by **Amielle Moreno, Ph.D., MPN Research Foundation**

VONJO®
(pacritinib) capsules

When your platelet counts drop and your myelofibrosis starts to progress,

TURN THE PAGE WITH VONJO

When you feel your myelofibrosis may be changing, it's time to move to the next chapter and speak to your doctor about VONJO.

Looking for a myelofibrosis specialist near you? Scan to use our locator tool to find one.

Please see the Brief Summary below of the VONJO Patient Information, which includes information about serious side effects from the full Prescribing Information.

BRIEF SUMMARY

What is VONJO® (pacritinib)?

VONJO is a prescription medicine used to treat adults with certain types of myelofibrosis (MF) who have a platelet count below 50,000 per microliter. This indication is approved under accelerated approval based on spleen volume reduction. Continued approval for this indication may depend on proof and description of clinical benefit in a confirmatory trial(s).

It is not known if VONJO is safe and works in children.

Important Safety Information

Do not use VONJO if you are taking other medications that are strong CYP3A4 inhibitors or inducers.

Before taking VONJO, tell your healthcare provider about all of your medical conditions, including:

- Previous medical conditions such as any other cancers, blood clot, heart attack, other heart problems, stroke, infection, diarrhea, commonly loose stools, nausea, vomiting, liver problems, or kidney problems
- Have active bleeding, have had severe bleeding, or plan to have surgery or invasive procedures. You should stop taking VONJO 7 days before any planned surgery or invasive procedures
- Have a history of low blood levels of potassium. It is important that you get blood tests done during treatment with VONJO to monitor your body salts (electrolytes)
- Smoke or were a smoker in the past
- Are pregnant, plan to become pregnant, are breastfeeding, or plan to breastfeed. It is not known if VONJO will harm your unborn baby or if it passes into breast milk. You should not breastfeed during treatment and for 2 weeks after your last dose of VONJO
- Plan to father a child. VONJO may affect fertility in males. You may have problems fathering a child

Please see Brief Summary continued on the next page.

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Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements and remedies. Especially tell your healthcare provider if you take hormonal contraceptives (birth control).

What are the serious side effects of VONJO?

- **Bleeding.** VONJO can cause severe bleeding, which can be serious and, in some cases, may lead to death.
  - **Stop taking VONJO and tell your healthcare provider right away if you develop any of these symptoms: unusual bleeding, bruising, and fever.** Get medical help right away for any bleeding that you cannot stop
  - You will need to stop taking VONJO 7 days before any planned surgery or invasive procedure (such as a heart catheterization, stent placement in a coronary artery in your heart, or a procedure for varicose veins). Your healthcare provider should tell you when you can start taking VONJO again
- **Diarrhea.** Diarrhea is common with VONJO, but can be severe, and cause loss of too much body fluid (dehydration). Tell your healthcare provider if you have diarrhea and follow instructions for what to do to help treat diarrhea. Drink plenty of fluids to help prevent dehydration.
- **Worsening low platelet counts.**
- **Changes in the electrical activity of your heart called QTc prolongation.** QTc prolongation can cause irregular heartbeats that can be life-threatening. **Tell your healthcare provider right away if you feel dizzy, lightheaded, or faint.**
- **Increased risk of major cardiovascular events such as heart attack, stroke, or death in people have happened, especially in those who have cardiovascular risk factors and who are current or past smokers** taking another Janus associated kinase (JAK) inhibitor to treat rheumatoid arthritis. **Get emergency help right away if you have any symptoms of a heart attack or stroke during treatment with VONJO,** including: discomfort in the center of your chest that lasts for more than a few minutes, or that goes away and comes back; severe tightness, pain, pressure, or heaviness in your chest, throat, neck, or jaw; pain or discomfort in your arms, back, neck, jaw, or stomach; shortness of breath with or without chest discomfort; breaking out in a cold sweat; nausea or vomiting; feeling lightheaded; weakness in one part or on one side of your body; or slurred speech.
- **Increased risk of blood clots.** Blood clots in the veins of your legs (deep vein thrombosis, DVT) or lungs (pulmonary embolism, PE) have happened in some people taking another JAK inhibitor for rheumatoid arthritis and may be life-threatening. **Tell your healthcare provider right away if you have any signs and symptoms of blood clots during treatment with VONJO,** including: swelling, pain, or tenderness in one or both legs; sudden, unexplained chest pain; or shortness of breath/difficulty breathing.
- **Possible increased risk of new (secondary) cancers.** People who take another JAK inhibitor for rheumatoid arthritis have an increased risk of new (secondary) cancers, including lymphoma and other cancers, except non-melanoma skin cancer. The risk of new cancers is further increased in people who smoke or have smoked in the past.
- **Risk of infection.** People who have certain blood cancers and take another JAK inhibitor have an increased risk of serious infections. People who take VONJO may develop serious infections, including bacterial, mycobacterial, fungal, and viral infections. If you have a serious infection, your healthcare provider may not start you on VONJO until your infection is gone. Your healthcare provider will monitor you and treat you for any infections that you get during treatment with VONJO. **Tell your healthcare provider right away if you develop any of the following symptoms of infection:** chills, aches, fever, nausea, vomiting, weakness, painful skin rash, or blisters.

The most common side effects of VONJO include:

Low platelet count (thrombocytopenia), nausea, vomiting, low red blood cell counts (anemia), and swelling of your ankles, legs, and feet.

Your healthcare provider will do blood tests and an electrocardiogram (ECG) before you start treatment with VONJO and as needed during treatment to check for side effects.

Your healthcare provider may change your dose or how often you take VONJO, temporarily stop or permanently stop treatment with VONJO if you have certain side effects.

These are not all the possible side effects of VONJO. Call your doctor for medical advice about side effects. You can report side effects to the FDA at 1-800-FDA-1088.

Talk to your healthcare provider or pharmacist to learn more about VONJO. For more information visit www.vonjo.com or call +1 781-786-7370.

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Care Partner vs. Caregiver: Why Language Matters in Lymphoma Support

When a loved one is diagnosed with lymphoma, the individuals who support them play a crucial role in their journey. Historically, these individuals have been referred to as "caregivers," but in recent years, the term "care partner" has emerged as a more empowering and inclusive alternative.

In the United States, about 53 million people provide unpaid care to a loved one and more than 2.8 million are caring for someone with cancer. With this many care partners in the cancer community working tirelessly to support their loved ones, the words we use to describe roles in the cancer community matter. That is why the Lymphoma Research Foundation supports the use of a term for them that is as meaningful and reflective of their experience as possible. Here is why we believe "care partner" fits the bill:

The power of partnership

The term "caregiver" suggests a one-way relationship — someone giving care to another who passively receives it. While lymphoma can be physically and emotionally demanding, patients remain active participants in their own care, while care partners often carry more responsibility. Responsibilities of care partners may vary but can include attending doctor visits, keeping track of medication, providing transportation to treatments, assisting with childcare responsibilities, and providing emotional support. The term "care partner" acknowledges the mutual exchange of support, respect, and decision-making between the patient and their loved one.

Building a supportive mindset

Just as much as patients need a support system, so do care partners. Care partners often experience stress, anxiety, and emotional fatigue. While it seems counterintuitive, a diagnosis of cancer can be harder on the care partner than it is on the patient. According to a study, cancer care partners spend an average of 32.9 hours per week providing care to their loved one. As a result, care partners reported experiencing increased anxiety, depression, fatigue, and other emotional stress, with more than 50% of care partners rating their stress level as "highly stressed."

Recognizing them as partners rather than sole providers of care reinforces that they, too, deserve support, resources, and self-care. It is also one of the many reasons why the Lymphoma Research Foundation offers specialized tools and education programs just for care partners.

The Lymphoma Research Foundation's shift to "care partner"

While we understand "caregiver" is still a widely used term that is recognized by many as standard terminology, the Lymphoma Research Foundation will begin to make the shift from "caregiver" to "care partner" in the hope of creating a more compassionate and inclusive environment for both patients and those who care for them. We believe it reflects a more accurate and compassionate understanding of the relationship between people living with lymphoma and those supporting them.

When navigating lymphoma, no one should walk alone, and all those impacted by a diagnosis should know that resources exist to support them on their journey. That is exactly what being a care partner is all about.

Written by **The Lymphoma Research Foundation**

Liam Hendriks | Photo by Rachel O'Driscoll/Boston Red Sox

From Diagnosis to Advocacy: **Liam Hendriks Champions Blood Cancer Awareness**

Diagnosed with stage 4 non-Hodgkin's lymphoma while playing for the Chicago White Sox, Liam Hendriks now raises awareness about blood cancers and disorders, encouraging open discussions and active lifestyles.

Tell me about your initial diagnosis and how it shaped the beginning of your journey with blood cancer.

I noticed lumps on the back of my neck while playing with the White Sox. I got a blood test in May, which came back clear, so I chalked it up to stress. But the lumps never went away. By November, they recommended more testing. I saw an ENT who did a needle biopsy, and it came back inconclusive. Eventually, I had a lymph node removed, and it came back as cancerous.

My oncologist had gone through leukemia herself, which was comforting. She told me, "It's stage 4, but I'm not worried." That was huge. People hear "stage 4" and assume the worst, but mine was non-aggressive and very treatable. We started a treatment plan right away.

What was the most challenging part of your diagnosis and treatment?

It's tough because you Google everything. My type of cancer is usually diagnosed in people over 65, and the fatality rate is higher due to age. That scared me. I found comfort in researching athletes and public figures who had been through it: Mario Lemieux, Michael C. Hall, Jane Fonda. It helped to see people bounce back and return to what they love.

The White Sox were amazing. I was treated at Mayo Clinic in Phoenix, and they adjusted everything during Spring Training so I could still work out, rehab, and maintain a routine. That was important for my mental state.

The Red Sox have also been fantastic. They set up meet-and-greets with patients through Dana-Farber and the Jimmy Fund. Talking to others has helped my mental health. Removing the stigma from words like "cancer" or "chemo" is powerful. Just talking about it lightens the load.

> Removing the stigma from words like "cancer" or "chemo" is powerful. **Just talking about it lightens the load.**

What message do you hope to share with others facing blood cancer, especially young patients or fans who look up to you?

Talk about it. Especially young adults — age 18 to 40 — often don't know how to open up, and neither do their friends, so it just bottles up. But the more you talk about it, the lighter it becomes.

Do you have any final thoughts you'd like to share?

I got a message from another player who'd had testicular cancer. He told me, "Don't let anyone tell you what you can and can't do." That stuck with me. Everyone's limits are different. If you can work out and recover well, do it. The more active you are, physically and mentally, the better your outcome can be.

It's easy to say, "I have cancer, so I'm just going to lie on the couch." But get up. Walk the dog. Move around. Push yourself a bit — just enough to feel like you.

Jeff Bridges Finds Gratitude and Love After Lymphoma

Actor Jeff Bridges reflects on his lymphoma journey, sharing how love, resilience, and perspective helped transform his cancer experience.

Jeff Bridges with family | Photo by Audrey Hall

What were some of the hardest parts of your lymphoma journey?

My hospital experience was during COVID, so my family couldn't visit me. It was tough being away from them. I remember being rolled over to the window, and they would be out in the street waving, and I would wave back, but I was so wiped out that I could only get in that chair and raise my hand for two or three minutes.

I like to change positions while I sleep, but turning over took about 15 minutes and was strenuous and exhausting. So, I couldn't sleep. The nurse would kick up oxygen to 100 percent so my body would have enough to make the initial turn, which was very painful and difficult.

Who or what helped you get through treatment?

For starters, the wonderful nursing staff, my doctors, and having the oxygen necessary to do basic things. I had to call in the troops to go to the loo; we would get in a huddle and go very fast, like a football team making a play. It took teamwork to avoid having an accident. It was like a sporting event. That stuck with me.

Of course, my family was my biggest source of support. Even though they were far away, I would talk to them on the phone, FaceTime them, and thank God for their presence.

I felt love from all the nurses and doctors, too. Even though their faces were hidden behind masks, I could still feel love radiating and being supported by them — and the universe, really. It feels funny to say it, but it was an experience I'm glad to have had, because I received gifts that I could only receive in that situation. The gift of love was so present.

I remember one of my wonderful doctors saying, "Jeff, you're not fighting. You've got to fight." I had no idea what he was talking about. Fight what? I was in total surrender mode, figuring that everyone dies, and this could very likely be me dying, so I was surrendering to that. I didn't want to fight death in that way. I think of death as part of life. We're all going to die, you know?

I would have contests with myself to see how long I could stand up. My first attempt lasted 45 seconds. Then I would say, "I can beat that record," and I would stand up for a minute, then gradually increase the time. The last contest that I gave myself was to be able to stand and dance with my daughter Hayley on her wedding day. I'm so grateful for my physical therapist and trainer, Zack Wermers. We trained like it was a sporting event. I made it.

What would you say to someone battling blood cancer right now?

Train and do your best. When I say train, I mean be open to all the love and information that's coming at you, and glean what you can from it. Be as present as you can, and receive it all as a gift. It doesn't seem logical, but being alive, we go through all sorts of experiences, and here's one that you're going through right now. So, just do your best. Be open to all the gifts that you can receive in this situation.

Every Child With Cancer **Deserves a Lifetime**

Despite advances in pediatric cancer treatment, limited funding and access challenges continue to threaten progress and children's long-term outcomes.

Pediatric cancer remains the leading cause of death from disease in children and adolescents. Despite remarkable progress, nearly 16,000 children and adolescents in the United States will be diagnosed with cancer this year, and 1 in 5 will not survive. Further, survivors of childhood cancer face significant late effects from their cancer or its treatment. Studies have shown that by age 50, pediatric cancer survivors have a 99% cumulative incidence of developing at least one chronic health condition.

Breakthroughs in pediatric cancer treatment

Pediatric oncologists are committed to finding better therapy options for the 20% of children we are not yet able to cure and are striving to find more targeted and less toxic therapies with fewer late effects. Recent breakthroughs in precision medicine and immunotherapy are helping us come closer to achieving these goals.

Technological advances, such as genome sequencing, allow physicians to rapidly evaluate approximately 20,000 genes in cancer cells within a few weeks and determine if treatment can be customized to improve outcomes. Sequencing is also helping us identify children who are at increased risk of developing cancer, allowing us to monitor and intervene early.

In addition to the role of precision medicine in enhancing outcomes, we have made remarkable strides in harnessing the immune system to help combat cancer. Medicines like PD-1 inhibitors help activate the immune system and prevent cancer cells from evading detection. Additionally, cellular therapy, such as CAR-T cell therapy, has been especially impactful for children with relapsed and refractory acute lymphoblastic leukemia, the most common type of childhood cancer. CAR-T cell therapy involves collecting T cells from the patient's blood and genetically modifying these immune cells to seek out and destroy the leukemia cells. Remarkably, many children with leukemia who have failed all other treatments have achieved sustained remissions with this new therapy.

The importance of continual funding

Despite these advances, pediatric cancer research remains woefully underfunded, creating barriers to realizing the potential of our recent medical discoveries. In fact, only about 4% of the National Cancer Institute's annual budget is dedicated to childhood cancers.

Even with exciting advancements in diagnostics and therapeutics, a question we must contend with is access, particularly for children dependent on Medicaid. Medicaid and the Children's Health Insurance Program cover nearly 50% of all children nationwide. Medicaid provides vital access to pediatric oncology specialists, clinical trials, cutting-edge cancer drugs and treatments, bone marrow transplants, and cancer survivorship programs. Evidence shows that pediatric patients who experience disruptions in Medicaid coverage have worse survival rates than patients without disruptions.

September is Childhood Cancer Awareness Month. While cancer awareness is important, it must be accompanied by action to address crucial issues. Protect Medicaid and simplify enrollment. Fund pediatric cancer research. Enable cancer providers and researchers to discover and advance more treatment options for children with cancer. Give every child with cancer the opportunity for a lifetime.

Written by **Doug Graham, M.D., Ph.D., Past President, American Society of Pediatric Hematology/Oncology; Chief, Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta**

A Mom's Mission for a **Match and Cures**

The onset of the global COVID-19 pandemic in the spring of 2020 was an unimaginable time. But for Courtney Addison, she was facing something even more unthinkable when she was told, "Your child has cancer."

Courtney's vibrant, 3 year-old son, Cayden, had been experiencing leg pain and developed an unusual limp. The pain progressed, and his typically ravenous appetite diminished. After multiple trips to the doctor and a slew of tests, Cayden was diagnosed with a rare, high-risk form of blood cancer called Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ ALL). From that moment forward, Courtney was on a mission.

Committed to supporting Cayden through something no child should have to endure — numerous surgeries, a slew of treatments, including aggressive chemotherapy, and countless overnight hospital stays — she set out to learn everything she could about blood cancer. Shortly after his diagnosis, Courtney connected with Blood Cancer United, formerly The Leukemia & Lymphoma Society, for disease and treatment information and support.

Eventually, she became an ambassador for the organization's Dare To Dream Project, dedicating herself to helping accelerate cancer treatments and policies that could one day mean that other kids and their families wouldn't have to face a blood cancer diagnosis.

Bone marrow donor registries

Cayden completed treatment in April 2022 but relapsed the following year. He underwent CAR T-cell therapy. Although Cayden responded well to treatment, he remains in need of a bone marrow transplant and is waiting for a donor. Courtney has exhausted options within her community and beyond, desperately searching for a match. Because African American and Black individuals are significantly underrepresented in bone marrow donor registries, it's more difficult for patients like Cayden to find compatible matches for transplants.

Cayden just turned 9 and recently started third grade. He is active and enjoys all the things a kid his age should be doing. While they are living their lives to the fullest, Courtney continues to tirelessly help other families and search for a match for Cayden.

"You never think about cancer happening to you and your family," Courtney said. "I will continue to fight not only for Cayden but also for the kids and families affected by a cancer diagnosis, and I continue to educate people about how important it is to consider registering to be a donor. It saves lives."

Written by **Blood Cancer United ® (formerly, The Leukemia & Lymphoma Society)**

Courtney and Cayden Addison | Photos courtesy of Blood Cancer United

> **I will continue to fight not only for Cayden but also for the kids and families affected by a cancer diagnosis,** and I continue to educate people about how important it is to consider registering to be a donor. It saves lives.


Actor Portrayal.
HERE WE USED GENOMIC SEQUENCING
TO FIND THE BEST TREATMENT
FOR QUINN'S CANCER
SO SHE CAN DEFY THE ODDS
Children's Hospital Colorado
Here, it's different.™
BEST CHILDREN'S HOSPITALS
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Innovative cancer care.
Scan to learn more.