



inside

- 3 **As I See It:** Setting Goals
- 4 **Inhibitor Insights:** The First Disruptor
- 5 **Richard's Review:** Surviving with Hemophilia
- 6 **YOU:** SMART Women

What's Your Everest?

Laurie Kelley

It's a new year, often a time when we set goals to improve our lives. Is there something you'd like to improve right now? A dream you want to chase? Setting goals effectively has been proven to increase your chances of making lasting, positive change. I often use mountain climbing as a metaphor for attaining goals and making life changes. Well, this metaphor became a reality for one man with hemophilia!

In May 2017, at age 31, Chris Bombardier of Denver, Colorado, became the first person with hemophilia in history to summit Mt. Everest. Then in January 2018, he summited Mt. Vinson in Antarctica, completing the Seven Summits, the highest peak on each continent. Not only is Chris the first person with hemophilia to achieve this, but he's one of only about 450 people *in history* ever to summit the Seven!

Chris's climbs were groundbreaking and history making. But you don't have to be an elite mountaineer to achieve some pretty awesome accomplishments yourself. Using the mountain climb as a metaphor, you can learn how to set your own goals, and then take steps to make 2018 your year to succeed! Learn from Chris's strategy for turning challenge into triumph. Ask yourself this question: What's *your* Everest?

When a Dream Takes Hold

When you were a child, did you ever daydream? Maybe you thought that one day you'd be a pilot, a US Marine, a race-car driver, a ballerina. What happened when you got older? You may have tried to reach your dream but hit your limit: Bad eyesight prevented you from flying. Growing too tall kept you from dancing professionally. Hemophilia prevented you from joining the military. More likely, you were told you couldn't, you shouldn't, it's not practical. Sometimes, other people squash your dreams, based on their own realities, limits, or insecurities.

As an adult now, do you still have "someday" dreams? Maybe you want to return to school someday and get that degree, or buy your dream house. Maybe you want to start a band someday, or start a family, or run your first 10K or your first marathon! Maybe your dream is just to learn how to infuse your child with hemophilia. How do you make "someday" into "today"?

Take a moment and think: If you could accomplish one thing by December 31, 2018, what would it be? If you could accomplish one thing in your life, knowing you could not fail, what would it be? That's right: imagine that you *cannot* fail—because if you think you'll fail, you might not make the attempt.



Chris is motivated to climb to bring attention to people without treatment



A business colleague once told me that football is the greatest metaphor for life. I don't follow football, so for me, it's mountains. Mountains have always held an allure for me, and they seem like the perfect metaphor for reaching goals in life. A mountain summit is usually slightly out of sight, but you know it's there. You have to train hard, and you have to secure resources. You need a

compass or GPS, a guide, and the right equipment. And you need to prepare for obstacles and setbacks.

We all have dreams, or we should have dreams. But we often lose our way when we try to make our dreams come true, because we don't have a clear vision of what we want in the end, and we don't map out the practical steps to get there.

On top of that, our attention is constantly assaulted by flashes of social media, texting, cell phones, and music. It's a wonder people have any quiet time to think and dream about what they really want to accomplish in life. I love this statement by General Omar Nelson Bradley: "We need to learn to set our course by the stars, not by the lights of every passing ship." We need alone time to think, dream, and then plan.

It's never too late to start. I've accomplished many of my earlier dreams—writing books, starting a nonprofit—but in the past seven years, I've actually begun climbing real mountains, fulfilling a dream I've long had, and which seemed impossible, given my work commitments. Now at 60, I may be too old to plan for Everest, but I plan to summit other mountains soon. I've climbed Kilimanjaro twice, and last May, I hiked to Everest base camp! I accompanied my friend Chris Bombardier, who reveals in our feature article how he conquered the Seven Summits—including Everest—despite having hemophilia. Read the story and be inspired by Chris, who has now gone down in history. Learn how to dream again, how to plan, and then get 2018 going. Make it your best year yet! ☺

Laurie Kelley

inbox

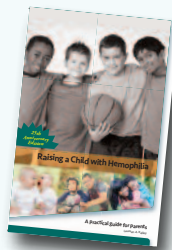
HemaBlog

THANK YOU FOR informing us [about Helixate® FS being discontinued]! Even though we already switched to another product, I didn't know why Helixate FS was being discontinued. I appreciate the clarity and the information you bring to the hemophilia community and your dedication and efforts to all hemophilia patients around the world.

Andrea Carminio
NEW YORK

THANK YOU FOR the book *Raising a Child with Hemophilia*, which was my first educational tool when my now 13-year-old was diagnosed with severe hemophilia A at 18 months. It helped me tremendously on our first trip to the ER for an infusion before we were self-infusing at home. I was actually prepared for the staff that was clueless about hemophilia. I also have a 10-year-old-son with severe hemophilia A, so we're old pros now.

Ann Hodyl
ILLINOIS



»» page 15

PARENT EMPOWERMENT NEWSLETTER | FEBRUARY 2018

EDITOR-IN-CHIEF Laureen A. Kelley

SENIOR EDITOR Sara P. Evangelos • SCIENCE EDITOR Paul Clement

ASSISTANT EDITOR Tara L. Kelley • CONTRIBUTING WRITER Richard J. Atwood

LAYOUT DESIGNER Tracy Brody • PUBLICATIONS MANAGER Jessica O'Donnell

DIRECTOR, PROJECT SHARE Zoraida Rosado

PEN is a newsletter for families and patients affected by bleeding disorders. PEN is published by LA Kelley Communications, Inc., a worldwide provider of groundbreaking educational resources for the bleeding disorder community since 1990.

PEN respects the privacy of all subscribers and registered patients and families with bleeding disorders. Personal information (PI), including but not limited to names, addresses, phone numbers, and email addresses, is kept confidential and secure by the LA Kelley Communications editorial staff in accordance with our privacy policies, which can be viewed in entirety on our website. PEN publishes information with written consent only. Full names are used unless otherwise specified.

PEN is funded by corporate grants or advertisements. Sponsors and advertisers have no rights to production, content, or distribution, and no access to files. The views of our guest writers are their own and do not necessarily reflect the views of LA Kelley Communications, Inc., or its sponsors.

PEN is in no way a substitute for medical care or personal insurance responsibility. Parents or patients who question a particular symptom or treatment should contact a qualified medical specialist.

Parents or patients with personal insurance questions should contact their employer's human resource department, Medicaid or Medicare caseworker, payer representative, or HTC social worker.

Articles may be reprinted from PEN only with express written permission from the editor, and with proper citation. PEN and/or its articles may not be published, copied, placed on websites, or in any way distributed without express written permission.



37-39 West Main Street #8

Georgetown MA 01833 USA

978-352-7657 • fax: 978-352-6254

info@kelleycom.com • www.kelleycom.com

as i see it

Changing Your Life: Setting Goals

L.A. Aguayo and Chad White

Motivational guru Tony Robbins says, “If you talk about it, it’s a dream. If you envision it, it’s possible. But if you schedule it, it’s real!”

How do you make a dream become reality? When we set a goal, no matter what it is, we focus on achieving it 100%. Creating Hemolife, bodybuilding, a competition, whatever the goal may be. We start by setting the target. Next, we want to document it: tell people our plans. This creates accountability. We would feel terrible, and would lose credibility and self-respect, if we told everyone we were doing something and then quit. Documenting our goal makes us work that much harder at it, even on days when we don’t feel up to it. We can reach our goal only by making ourselves vulnerable and accountable. So we show our journey toward our goal in the raw—on Facebook, Instagram, Snapchat—as it unfolds. We want to let people know that we are no different than they are, and that they, too, can reach their goals. This also can help inspire others. Goals take work, long and hard work, 24/7! We must do whatever needs to be done.

A great way to get started on a goal is to ask the most influential people you know for help. Get a team together if you can, and work together on your goal.

One important aspect of goal setting is to constantly reset your goals. When you achieve one goal, make another! Don’t be afraid to fail; if you do fail, then adjust your goal as you go along. Maybe aim a little lower to create some small wins, or change up your schedule. Remember, if you do the same thing you’ve always done, you’ll get the same thing you’ve always gotten. If you’re experiencing difficulties repeatedly, then stop, evaluate, and make big changes. Failure isn’t an option with this mentality. It’s like climbing a hill or mountain that you thought was too high. Goals are a nonstop journey. If the goal is big enough, there will always be adversity and setbacks. That’s part of growing. Goals are not meant to be easy. Most people don’t even take the first step because it seems too hard and the results aren’t guaranteed.

If you’re experiencing success, great! Now double up on whatever it is you are doing.

L.A. and Chad’s Formula for Success!

1. Choose a goal (no matter how big).
2. Define why you want it (your purpose).
3. Define what your desired outcome will be.
4. Decide on a timetable, and schedule it.
5. Find influential people who have achieved what you want, and ask for advice.
6. Make a detailed plan to reach your goal, and write it down.
7. Tell people your goals, and document your journey.
8. Take massive action!
9. Don’t be afraid of failure, and be ready to adjust along the way.



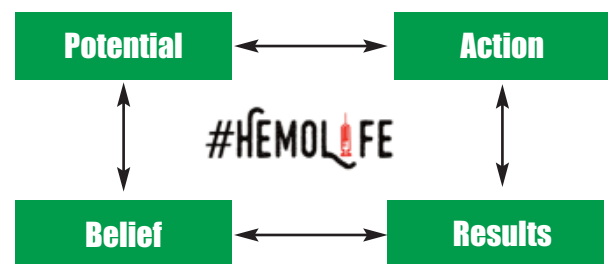
L.A. Aguayo



Chad White

Hemophilia is an obstacle, but not one that has the power to stop us. Our minds are stronger than that! Remember, the body can do anything the mind can convince it to do. If you have hemophilia, factor up regularly! Reaching a goal is worth it, and once you do, you’ll be addicted to success. Nothing is more gratifying than setting a tough goal and achieving it. Make a solid plan on how to go through each step to the finish, and then knock off each step, one by one. Adversity along the way will create opportunity at the end.

Here’s a mental framework we use to achieve our bodybuilding goals.



We all start off with some type of potential. Each person will be on a different level, but the potential is there; you just need to tap into it. When you realize you have that potential, you can take an action toward achieving your goal. And with that action, you’ll get results. When the results are positive, you’ll create new belief in yourself about the possibilities of achieving your goal. This new belief in yourself will increase your level of potential in return, giving you the ability to take a *bigger* action next time. The bigger action you take will get you bigger results, and the bigger results will create even more belief in yourself!

What if the results are negative? Go back to the action you took, reevaluate it, and take a new action. Keep the cycle going! If you keep repeating this process, you’ll discover you’re capable of accomplishing incredible things, because once your belief increases, you begin to develop new vision, which will create new goals.

Being with Hemolife is a dream come true. Not by magic, but by setting goals, and by hard work. Our next big goal is to speak at the World Federation of Hemophilia Congress! We believe it. So we know it’s within our reach. @

L.A. Aguayo, 29, has severe hemophilia B and lives in St. Louis. He has four children and works for Heritage Biologics. He created Hemolife.org, a website for leaders with bleeding disorders who share their stories of adversity to inspire the hemophilia community. Hemolife’s mission is to inspire and motivate people who are going through extreme adverse situations.

Chad White, 44, has hemophilia A and lives in Windsor, Ontario. He is married to Jocelyn, has four children, and works at Ford Motor Co. He’s a member of the Hemolife team and Hemophilia Ontario Adult network.

ACE910: The First Disruptor

Disruptive innovation: an innovation that creates a new market and eventually disrupts an existing market, displacing established market-leading firms, products, and alliances.

The standard of care for people with hemophilia in developed countries is clotting factor replacement therapy, and for those with inhibitors, therapy with bypassing agents (BPAs). These therapies have created an industry worth about \$11 billion. That's all about to change, especially for people with inhibitors, because of new "disruptive therapies"—which do not require factor replacement therapy or the use of BPAs. In addition to gene therapy, which will soon become a reality, several other disruptive therapies are poised to enter the market, and shake it up (see box, page 13).

One of these disruptive therapies has already arrived: in November 2017, the US FDA approved Genentech's Hemlibra® (emicizumab or ACE910) for treating hemophilia A with inhibitors. Two things make this product a standout: it's administered subcutaneously—under the skin—and the clinical results have been remarkable. Genentech describes Hemlibra as the first innovation for inhibitor patients in 20 years.

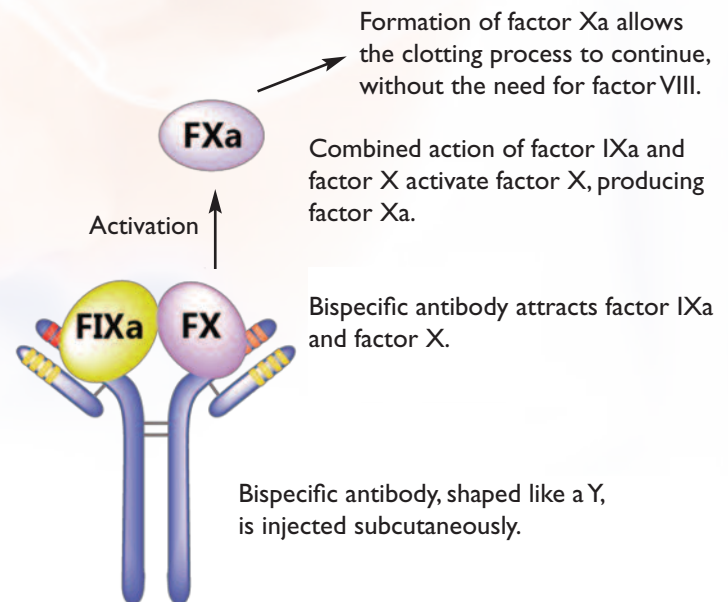
Hemlibra has put the wheels of change in motion.

How Does Hemlibra Work?

Hemlibra is a *bispecific monoclonal antibody* that mimics (copies) the function of factor VIII. What's a bispecific antibody? Antibodies are Y-shaped proteins that serve as the immune system's first line of defense against foreign substances including pathogens such as viruses, bacteria, and fungi. Each antibody is unique in that it recognizes and binds only to a specific antigen: a specific molecule on a specific pathogen. The unique part of the antibody—the binding site that attaches to an antigen—is located near the tips of arms of the Y-shaped antibody. In nature, both arms of the antibody bind to the same antigen. Bispecific antibodies are a little different from natural antibodies—they are artificial antibodies that can simultaneously bind to two different types of proteins (antigens), instead of to a single antigen (as is normal in nature). The adjective "monoclonal" simply means that all the bispecific antibodies are of the same type.

When an antibody binds to an antigen, it may neutralize or inactivate the antigen. Inhibitors, for example, are antibodies that attach themselves to infused factor and inactivate it, preventing it from helping to clot the blood. All antibodies tend to persist for a long time in the blood, from a week to a month (they have long half-lives).

If an inhibitor to factor VIII is an antibody, then how can antibodies be used to treat hemophilia?



Hemlibra, a bispecific antibody, mimics the function of factor VIII by bringing together factors IXa and X to activate factor X (making Xa). This restores the clotting process, so a blood clot can form.

You know that factor VIII is one of more than a dozen clotting factors that participate in the complex blood coagulation process known as the *clotting cascade*. Factor VIII's job is to activate factor X. Factor VIII does this by becoming activated itself (after injury to a blood vessel) and then binding to activated factor IX (written as IXa) and factor X. When factor IXa and X are brought close to each other, the factor X becomes activated (now written as Xa). This allows the clotting process to continue, and eventually to form a clot. When you have hemophilia A, you have little or no factor VIII. With no factor VIII, no Xa is produced, and the clotting process stops.¹ The bleeding continues.

Researchers at Chugai Pharmaceuticals asked: How can we bring factors IXa and X together to activate factor X *without* the need for factor VIII? They settled on investigating bispecific antibodies. And they undertook a daunting molecular engineering task: to design a bispecific antibody capable of activating factor X by simultaneously binding factors IXa and X. The bispecific antibody would act like a person grabbing factor IXa in one hand and factor X in the other and then bringing the two together to create activated Xa.

There would be no need for factor VIII.

» page 13

1. The clotting process does not stop completely in the absence of factor VIII. Another branch of the clotting cascade, the *extrinsic pathway*, does allow for some clotting, but it's inefficient compared to the *intrinsic pathway* that uses factor VIII.



Richard J. Atwood

Surviving with Hemophilia: The Bijl Family

I firmly believe that every memoir written about bleeding disorders deserves to be read. Still, some memoirs just stand out. One outstanding account is *Focus on Survival: A Young Family's Struggle to Survive World War II* (2015) by Julie Bayl.

We know that raising a child with a bleeding disorder can be a challenge. Yet how do you cope when your country is ruled by an antagonistic foreign military, you have two sons with hemophilia under age five, and you don't have enough food? We're fortunate that Em Bayl told her daughter-in-law the story of her family's survival despite hardships during the Nazi occupation of Holland, when even having hemophilia could have dire consequences.

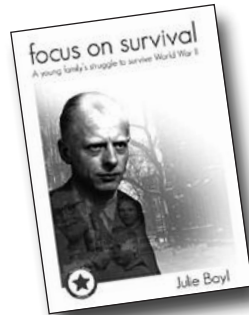
Em was born Maggeltje Flach in 1909, into a Dutch Reformist family. She grew up with six sisters and one brother on the family farm in Chaam, in southern Holland. Em's brother Gerritt, known as a "bleeder," died from a stomach bleed in 1926 when he was only 15. Em had three maternal uncles who exhibited heavy bruising and swollen knees and ankles. They all died young. Em knew her family's history of hemophilia.

Against the wishes of her parents, Em trained to be a midwife in 1933. Practicing midwifery in Amsterdam, Em attended the birth of Maria Bijl's second child, in Baarn in 1934. Maria's husband, Bernard Gerard Heinrich Bijl, or Ben, had been raised in a family of photographers in Amsterdam. Ben and Maria had two sons, Hans and Bennie, and then divorced. Maria had custody of the two boys, who were of Jewish descent but perhaps not considered Jewish by the Nazis. Maria and her sons lived in Amsterdam.

Ben and Em became secretly engaged in 1936. Then, despite her parents' objection, Em married Ben, and the couple moved to Zeist.

Em had a daughter, Gerda, in 1937. War was declared in September 1939, and three months later, Em had a son, Charles. As a toddler, Charles had a persistent bleeding lip. While hospitalized, Charles received the diagnosis of hemophilia and a transfusion of whole blood donated by his father.

Holland was invaded by Germany in 1940 and surrendered, beginning the Nazi occupation. Ben was a Communist Party



member. Contributing to the resistance, Ben used his photography skills to forge passports for Jewish citizens trying to escape. If caught, Ben could be killed.

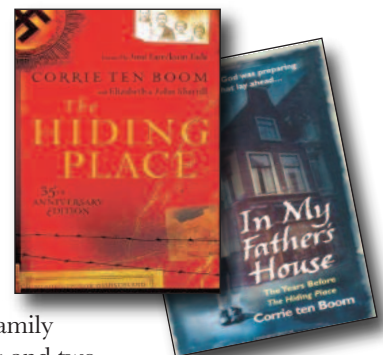
Ben avoided the mass conscription by the Nazis for all Dutch men aged 18 to 45 in 1943. He hid in the attic during searches, and wore women's clothes in public. Em kept her bicycle, despite the forced collection of all types of transportation by the Nazis, because she was still a midwife and needed to reach her patients.

The Bijl family suffered from shortages of food, fuel, electricity, and income. Em was malnourished when her second son, Louis, was born in 1943. Like his brother Charles, Louis bruised easily before he began walking at 16 months, and then he had an ankle bleed. To treat her two young sons with hemophilia, Em used naturopathic remedies to "build up the blood" and get rid of bruises. For external bleeds, she applied ice and wet compresses dipped in arnica.¹ For internal bleeds, Em used witch hazel drops, and Bex powder² and aspirin, when available. Still, the boys experienced pain while bedridden with bleeds.

The Canadians liberated Zeist in May 1945. Em endured another pregnancy while malnourished. Her daughter, Marjolean Anna, was born in freedom two weeks after liberation. Ben and Em had started discussing emigration shortly after their marriage, and the Bijl family moved to Australia in 1953, taking the English name Bayl. After Charles married Julie in 1971, Em told her daughter-in-law the story of the family's survival during the Nazi occupation. As expected from a family of professional photographers, the memoir includes 35 photographs and four house plans showing the hiding place in the attic.

For a similar narrative during the Nazi occupation of Holland, I turned to two books by Corrie ten Boom: *The Hiding Place* (1971) and *In My Father's House* (1976). *The Hiding Place* was made into a 1975 movie with the same title.

The ten Boom family, devout Dutch Reform Church members, were watchmakers in Haarlem, just 10 miles from Amsterdam. During the Nazi occupation, the family assisted and hid four Jewish citizens and two



1. Arnica is a plant extract made from flower heads or roots that is used to treat sprains, bruises, and painful swelling. 2. Bex powder, also available as tablets, is an analgesic containing aspirin, phenacetin, and caffeine. Bex powder was popular in Australia.



SMART Women

Jessica O'Donnell



From personal to professional, the women profiled here turned their connections to hemophilia into real, life-changing achievements. While many of us set out to complete a task, we may not label it as a goal or plot out a deliberate path to achieve it. But each of these women had a vision, implemented a plan, and plotted a path to reach her goal.

Each woman's strategy was unique, but all the goals had something in common: they were all SMART goals. SMART goals are specific, measurable, accountable, realistic, and time-bound. Learn how Mischante, Angelie, Darlene, and Christy and Elise worked hard to get results.

A Better Future for Her Family

After Mischante Cortez's son, Adam, was diagnosed with hemophilia in 2006, Mischante decided to set a goal: to become a registered nurse to better advocate for Adam's healthcare needs. It wasn't easy. Pregnant at age 16, Mischante had dropped out of high school. So before starting her nursing program, she needed to obtain a GED and enroll in community college. After receiving her GED and being accepted into a nursing program, Mischante divided her goal into smaller, specific steps.

But her path wasn't without obstacles. While enrolled in the nursing program, Mischante was often left with no choice but to bring her three young children to school with her because she lacked childcare. Despite the setbacks, Mischante persevered. "I continued to strive toward my goal to be a nurse while raising three kids alone and working full-time as a certified nurse's assistant [CNA]. In May 2010, I graduated from college with my nursing degree. It was the best decision I ever made for my family."

Today, Mischante supports her family as a working nurse and single mother. "I am Adam's number-one advocate, and I get to heal people for a living. I am so grateful that Adam's diagnosis of hemophilia was exactly what I needed to get into gear."

A Son's Diagnosis Prompts a Family Goal

When Angelie Garcia's son Zayden was diagnosed with hemophilia, Angelie recognized that he was unhappy while being infused with factor. So she told her husband, "Once we're taught by home nurses, within two weeks we will start involving Zayden." Then Angelie set a goal to have Zayden take an active role in the infusion process within one year.

Angelie and her husband divided their goal into small, attainable steps: the first was to make sure Zayden wouldn't be afraid of infusions.

On their path to achieving their goal, the family's largest setback was their location. "Our hometown hospitals need

education on hemophilia," explains Angelie. "I'm the only one here that can infuse my son, and it terrifies me because they don't know about his condition and we are three hours away from our HTC [hemophilia treatment center]." Angelie learned very quickly that she had to be Zayden's primary advocate for his hemophilia care. Teaching him to help in the infusion process empowered Zayden, now four years old, to be more aware of his condition. His proud mom says, "He is learning about his condition and will tell you what he can and can't do."

Lobbying for Specialized Emergency Treatment

Darlene Shelton founded Danny's Dose Alliance after her ten-month-old grandson was diagnosed with hemophilia. Although Danny always had his factor with him wherever he went, Darlene's family learned that paramedics and ER workers were not allowed to administer the factor due to conflicting treatment protocols and liability fears. So Darlene sprang to action and formed Danny's Dose with these goals in mind: (1) Raise awareness about the current gap in emergency treatment for people with rare diseases, chronic illnesses, and special medical needs; and see current protocols amended. (2) Assist with specialized education for EMS and ER personnel. (3) Provide education for affected families on how to better advocate for their treatment needs.

The organization's first goal was to amend protocols in the Sheltons' home state of Missouri within two years. Darlene says, "Goals can be lofty, but I believe if you don't set goals high, it removes some of the urgency." After their proposed legislation was passed in Missouri, Darlene looked forward to their next goal. "We passed EMS legislation, assisted with the beginning of paramedic education, and are helping that grow in 2018 and beyond. Of course, we can't be sure we will meet our five-year goal, but it looks promising, and we are determined to push hard." Darlene hopes to have improved treatment protocols for ER and EMS in place across the entire US within five years of their first state, Missouri.

The work of Danny's Dose doesn't just benefit people with hemophilia. "It benefits all individuals with special medical needs," says Darlene, "covering rare disease and chronic illness. This covers those with rare, lifesaving meds, those with particular

The first step in making dreams come true is to clarify and sharpen your current dream. You need to know what that dream is, define it, write it down. You need to make sure your dream is exciting and fills you with passion and drive. And your dream needs to be challenging and slightly out of reach for now, but also achievable.

DEFINE YOUR DREAM

What kind of dream do you have?

Personal: Lose weight, learn how to cook healthy meals, run a 10K race or a marathon, learn how to ski, read 20 books this year, pursue an advanced degree, study a new hobby, learn how to read music.

Professional: Find a better job, get an advanced degree, earn more money.

Hemophilia-related: Learn about genetic transmission, learn how to infuse your child or self-infuse, attend a national meeting, do a fundraiser for your local chapter, get carrier tested.

Chris had a lifelong dream, but not of mountaineering—at first. “I had a dream of playing professional baseball,” he confides. “I worked and trained harder than anyone else on the team. But there’s an innate ability you need to have, and I just didn’t have it. I’m not particularly tall, and I don’t have a good arm for throwing.” Chris’s dream bumped up against reality.

How did Chris turn his baseball disappointment into another dream? “After college,” he says, “I was hanging out with my Uncle Dave in the mountains, to get away. When he started telling stories about climbing Denali, one of the Seven Summits, and talked about mountains all over the world, it sparked my interest. I had read about Everest, of course, but the whole Seven Summits...it just seemed attainable! It’s more about you against *yourself*. A mental challenge. That appealed to me. And the physical challenge really attracted me, too.”

Chris lives in Denver at a high altitude, surrounded by mountains. His body was already somewhat adapted to high-altitude climbing. Chris channeled his dream of playing baseball into something he felt he could attain.

Climbing a mountain is a wonderful metaphor for vision leadership because a mountain is something you can see. The picture of you atop that lofty mountain embeds itself in your mind. Whatever the goal, once you have the picture, the key is to hold it and not give up. —ROYAL ROBBINS, FORMER MOUNTAINEER AND PRESIDENT, ROYAL ROBBINS COMPANY

Visualizing: Believing Is Seeing!

When you have an ultimate dream in mind, you need to visualize yourself achieving it. This might mean seeing your hemophilia organization conduct a successful gala fundraiser. Or completing your first self-infusion. Or finishing a beautiful garden, painting, or piano piece.

Your dream must be visualized and repeated consistently. “The whole idea had started a couple of years before my first summit, after talking to Uncle Dave,” recalls Chris. “The more stories he told me, the more time I spent outside, the more I was falling for this dream.” When Chris reached the first summit, he gained confidence about his dream. “It was on the summit of Kilimanjaro in 2011, when I knew the entire Seven Summits would be possible. That was the moment.”

Your dream should be bold, exciting, inspiring. And visualizing it should evoke emotion and make you *want* to take action. So if making your dream come true seems like a chore, then you need to find a better one! At age 13, Jordan Romero became the youngest person in history to summit Mt. Everest, and also the youngest in 2011 to accomplish all Seven Summits. His dream started at age 9. For years, Jordan and his classmates had passed by a mural of the Seven Summits on their elementary school wall every day: “Other kids hardly seemed to notice it in their rush to play soccer or basketball, but I often stopped and stared, mesmerized by those mountains.” Jordan wondered, “What would it be like to stand on their summits and gaze across entire continents?”¹

Sometimes, even when you find an exciting dream, voices in your head say, “That’s impossible” or “You’re too old” (or short, or weak, or inexperienced). These voices are the limiting opinions you might have heard while growing up—and may still hear when others learn of your dream. Don’t listen to them. Experiment with what *you* desire.

Can you visualize yourself in that dream? Visualization is a technique used to practice “seeing” what you want to achieve. Begin by creating in your mind the desired situation or ultimate goal you want—including color, sound, and emotion. Replay this picture, over and over, in your mind.

Visualization works. Elite athletes have used this technique to push the limits of human physical abilities and improve performance. One of the most successful skiers in history, Lindsey Vonn, says she visualizes running her course 100 times before she gets to the start gate for each race!²

1. Jordan Romero, *No Summit Out of Sight* (New York: Simon and Schuster Books for Young Readers, 2014). 2. www.mindbodygreen.com.



access solutions

Don't let insurance or financial challenges get between you and your treatment



- Trial Program at no cost to you
- Loyalty Program[†]: Assistance during gaps in insurance coverage
- Co-pay support[†]

- Patient support programs
- Live Helpline Support

CALL 1-800-288-8374 8:00 AM–8:00 PM (ET) Monday–Friday. Spanish-speaking Case Specialists are also available.

^{*}Restrictions apply. Please visit KOVALTRY.com, KogenateFS.com, or call 1-800-288-8374 for more information about the restrictions.

[†]Patients who have government insurance are not eligible for the loyalty and co-pay support programs.

Bayer, the Bayer Cross, KOVALTRY, and KOGENATE are registered trademarks of Bayer.
© 2017 Bayer. All rights reserved. Printed in USA 07/17 PP-775-US-0795

Kogenate FS
antihemophilic factor
(recombinant)

Kovaltry
Antihemophilic Factor (Recombinant)

Chris used visualization constantly to prepare himself for the summits. “I pictured each climb in my mind. I read a lot about each climb, and kept thinking of the route, visualizing it. By doing that, I definitely envisioned myself standing on the top. And I wondered, how will it *feel* being on top?”

Use visualization to accomplish three tasks that will help you achieve your goal. The first task is to create a sense of *urgency*. Your vision should excite and motivate you—and also make you slightly uncomfortable. Why? Because you want to make it happen now, not ten years from now. Not someday. Now. You can imagine how excited Chris felt!

The other two tasks require a bit more explanation: finding the best mentors, and designing a long-term strategy. Let’s take a look at those tasks.

“If your dreams do not scare you, they are not big enough.”
—LIBERIAN PRESIDENT ELLEN JOHNSON SIRLEAF

Your Mentor: Find an Uncle Dave

It’s hard to find the right path, stay on that path, and achieve your goals without support. Try to find mentors who will inspire you, goad you on, maybe even train you. Use your vision as a measuring stick when you look for coaches or teammates. If their ideas about your vision differ from your own, then these people could demoralize you, sabotage you, or make you doubt yourself.

Chris had several mentors. “Uncle Dave was my first mentor. He took me out to do activities and didn’t question whether hemophilia would affect my dream. He exposed me to the mountains. Previously, I had spent time outside, but not hiking and climbing. All I did was play baseball!” Uncle Dave was the catalyst for forming Chris’s dream, and he wholeheartedly embraced it when Chris made his decision.

Next, Ryan Waters, Chris’s first professional guide, became “a huge mentor.” Chris says simply, “I trust him with my life on the mountain.” Ryan owns and operates Mountain Professionals, headquartered in Boulder. Starting with Mt. Aconcagua in South America, Chris’s second climb, Ryan guided him on five of the seven summits. Ryan helped transform Chris’s now defined dream into reality.

Finally, Tashi Sherpa, the *siddhar* (lead Sherpa) on Everest, was Chris’s third and very special mentor. Tashi is a sinewy, fit, gifted leader and motivator. “His viewpoint on the world is amazing and infectious,” Chris says with admiration. “Maybe it’s his Buddhism, or simply just who he is, but he emanates respect for nature and people, and even in extreme places, has a calming spirit. He makes you feel good—that you can do anything. When I doubted myself and wanted to quit, just 1,000 feet from Everest’s summit, he reminded me that I had a higher mission and purpose, and he knew I could do it. Then I believed in myself again, and banished negative thoughts.” Chris has no doubt that he succeeded in his goal to summit Everest because of Tashi’s mentoring.

Find yourself a mentor, perhaps one who is an expert in the field. If you want to learn self-infusion, ask your HTC nurse to train you. If you can’t find a mentor in person, you can start by reading books on goal setting, and books on people who have achieved amazing accomplishments through dreaming, visualizing, and then setting goals.

Design a Long-Term Strategy

So you have your dream, you’ve created your vision, and you’ve even found mentors. But you still aren’t ready to achieve a goal. Not yet. Now you need a strategy, a way to divide the journey to your dream—the summit—into manageable tasks. Imagine trying to climb Everest *without* a strategy! You can’t reach the summit simply by climbing hard. You may be the most skilled mountain climber in the world, but you’ll never summit if you don’t have a plan.

Your strategy is like a map that charts your course up the mountain toward your vision. On the map, you draw the known landscape: Are you physically and mentally ready? What obstacles block your route? How far must you go? How long will it take? Do you have enough resources? What will you do if you feel defeated or face disaster?

You’ll need to do some assessment here. First, determine your capabilities and skills. Then evaluate your environment and prepare for setbacks. Break down your journey into goals. Finally, design and implement your action plan. Let’s do it!

COMPONENTS OF A STRATEGY

- Determine your capabilities: What are your strengths and weaknesses?
- Evaluate the environment: What are your challenges?
- Prepare for setbacks: What will you do when you hesitate or doubt?
- Identify and prioritize goals: What measurable steps are achievable?
- Implement an action plan: What steps can you take now?

Determine Your Capabilities

It’s important to know yourself—your preferences, aptitudes, abilities. It might be hard to learn to sea kayak if you get seasick easily. It might be hard to do much hiking if you have a target joint in your knee. Getting an advanced degree might be challenging if you have several little kids at home. Even learning to infuse your child becomes difficult if you have a needle phobia.

So, depending on your dream, ask yourself...What strengths do I have that can make my dream a reality?

- Do I have technical or academic strengths, like graphic design, writing, website creation, math skills, good study habits?
- Do I have social and personal strengths, like being a skilled speaker, feeling empathy, or having athletic prowess in a sport?
- Do I have professional abilities, like business connections, legal or financial skills, administrative or managerial experience?

Finally, budget is important. You can take up running by investing in a good pair of running shoes for about \$100. But...skiing, piano lessons, starting a business...all these require much more funding. Compare your dream to your current resources. If you don’t have the resources or skills you need, can you secure them?

One way to secure resources is through an old principle called the Law of Attraction, which states that focusing intensely and daily on what you want—your dream, your Everest—will marshal the universe into action for you. Leadership coach Denis Waitley writes, “When you visualize, then you materialize.”³ Using this technique, you’ll be amazed at the resources that start coming

3. Denis Waitley, *The Psychology of Winning* (New York: Berkley, 1984).

your way. Visualizing consistently puts your unconscious mind at work throughout the day, so you begin noticing new things that can help you and urge you to take action. Plus, people will be moved by your commitment and your dream, and this may help bring you the resources you need, including funding.

How did Chris afford to do all Seven Summits? He scraped and saved, reached out to business contacts, and started a GoFundMe campaign. With Everest, the funds he needed were astronomical, far beyond his means and out of reach for everyone he knew. So he asked colleagues if they knew someone who could help. Chris sits on the board of Save One Life,⁴ and Octapharma has been one of Save One Life's biggest donors. The chairman of Octapharma, Wolfgang Marguerre, learned of Chris's dream, believed in it, and approved funding of the Everest climb. Chris's successful mountain climbs helped pave the way for others to believe in his dream. After Chris successfully summited Everest, Octapharma also funded his Mt. Vinson climb.

Lack of resources will make your climb to success harder. When you carefully assess your strengths and weaknesses, you'll see where you must focus. For many professional dreams, funding is often the biggest challenge. But focus on your dream daily, and let the Law of Attraction work for you, too.

Evaluate the Environment

Environment refers to circumstances and events that lie outside your control but can shape or influence your success: national economics, political climate, injuries, time, even other people's opinions of your dream. If you know the environment, then you



Your dreams.
Our dedication.

Our ultimate goal:
a life full of dreams
and free of bleeds.

bleedingdisorders.com

Shire

Alex
Hemophilia A
Massachusetts

©2017 Shire US Inc., Lexington, MA 02421. All rights reserved. 1-800-828-2088. SHIRE and the Shire Logo are registered trademarks of Shire Pharmaceutical Holdings Ireland Limited or its affiliates. 533614 07/17

can plan a way to overcome it. What will you say to those who doubt you can run a 10K race? If your job is downsized, how will you still pursue your dream with less personal funding? Will a target joint or surgery change your dream's deadline?

No surprise that for Chris, when trying to summit mountains, hemophilia was a major concern. He wondered, "How do I carry factor, and protect it on the journey? I was bringing factor higher on earth than anyone. Could I infuse? The weather was subzero." Chris had other concerns, too. "The cost of each trip, time off from work—these are real-world aspects that get in the way of dreams. And most difficult, actually, was training and then getting bleeds. I had to take time off to let them heal. I had left glute pain right before leaving for Everest. You must be healthy when you attempt Everest."

Try to consider as many scenarios as possible when creating your strategy. Not to worry about them, but to think *around* them. Ask yourself: Are these boulders on my path to the summit obstacles—or stepping-stones?

Prepare for Setbacks

Even when everything falls into place perfectly for your journey to your dream, you'll face unexpected setbacks. You get sick or injured, you get a bleed, you're told "No," or your child's veins collapse repeatedly. Maybe you decide you didn't really want this dream, and you want a different one instead.

Chris faced one huge setback. After bagging five summits successfully, his strategy was next to climb Mt. Vinson in Antarctica as his sixth. He wanted to save Everest, biggest and baddest, for last. But it didn't work that way. Chris was not issued a permit to climb Vinson, because he has a "disability." He was devastated. How could this be allowed? He had already climbed five of the highest summits on earth!

Vinson was a setback, but Chris made it work for him. He made a positive out of a negative. He switched up his strategy. He was given a permit to climb Everest, so now he made Everest his sixth summit. "When the Vinson people saw that I had climbed Everest," says Chris, "they couldn't deny me a permit any longer. Vinson was a go!"

Like Chris, you'll experience setbacks and failures. But see them as opportunities for a strategy change and improvement. Be solution oriented.

Thinking positively is a major aspect of reaching goals, Chris believes. "If you doubt yourself, then you can make excuses for why you *can't* do it. I have a quote on my wall: Whatever your excuse is, time to stop believing it. You can create your own setbacks and obstacles just by thinking negatively."

Believe that even setbacks can become successes.

Set Goals

The backbone of your strategy is the tangible goals you set. Goals are your dream broken down into concrete targets that are measurable and incremental. These targets build a visible step-by-step path to success, bringing you to each "camp" on your journey to the summit. Here's the first thing you need to know about goals: *Write them down.*

4. Save One Life, Inc., is a registered international nonprofit dedicated to providing financial aid to those with bleeding disorders in developing countries, through sponsorships, scholarships, microenterprise grants, and camp funding. It was founded in 2001 by Laureen A. Kelley, president of LA Kelley Communications, Inc. www.saveonelifenet.net.

How can you write effective goals? Make your goals SMART:

Specific. Your goal must be focused, clear, and simple. “Be healthier” is vague. Instead, try this: “Lose 20 pounds. Stop drinking soda.”

Measurable. Use numbers. Instead of “Make more money,” try this: “Increase my income by 20%.”

Accountable. Share your goal. It doesn’t do any good if you’re the only one who knows you want to hike the Pacific Crest Trail one day! Post your goal on Facebook, and on your fridge—someplace where everyone will see it. Chris is by nature a shy, humble person. He admits, “Going public with my initial climbs wasn’t the intention. At first, I just shared my dreams and climbs with friends, family, and a few colleagues. After a couple of summits, the hemophilia community was noticing! Knowing people were watching me kept my summits in the forefront of my mind, so that kept me focused on my goal.”

Realistic. Strive for the achievable. Small wins are best. Don’t set such lofty goals, expect such high standards, or establish such short deadlines that failure is inevitable.

Time-bound. Goals are your dream with a deadline. A deadline creates urgency and should motivate you. Chris had small windows of weather when he could climb some of his peaks. For Everest, there were only a few weeks every year when he could climb. And while summiting, only a few days when the weather was ideal. Chris had to be prepared and ready to move at the right time.

Now break your goal down into smaller steps—objectives—and assign each objective a priority: high, medium, low. With priorities, you’ll know where to devote your greatest time and resources. *Spend most of your time on your highest-priority objectives.*

Ironically, Chris is not a big writer of goals! “I thought about my goals a lot,” he laughs. “They were always in my mind. I was always reading books and articles about the Seven Summits. That’s how I kept my focus. And always setting little objectives—to summit Denali, I needed to learn rope skills, crevice rescue, and winter camping. For Everest, I had to practice winter climbs.”

Using his objectives strategically helped Chris attain his ultimate goal to summit the Seven. “I purposefully climbed each mountain in a sequence that helped me to learn skills while I was climbing,” he shares. “Kilimanjaro was the easiest and first. Carstensz Pyramid was out of order, but only because it was cost-challenging. Even climbing Everest as the sixth summit helped me achieve a permit to climb Vinson, the last summit.”

Implement Action Plans

When you’re ready to commit your strategy to paper, you can start designing an action plan. Your plan can encompass a one-year, three-year, or even five-year focus, and should include the following:

- Your vision or dream
- Your strengths and weaknesses
- Your environmental opportunities and challenges
- Your SMART goals, broken down into objectives, with deadlines, priorities, and mentors

Post your action plan where people you trust will hold you accountable and support you! “When I got comments and likes on Facebook,” notes Chris, “it gave me more confidence and made it feel more worthwhile.” When you know that others you respect are watching you and waiting for success, you’ll try harder and more consistently to achieve your goals.

Know that not everyone on Facebook will chime in with “Likes” if they think you are risk-taking, showing off, or being unrealistic. Even Chris had his share of Facebook detractors in the hemophilia community who wanted to know why he was putting his life in jeopardy, why he was doing something they could never dream of, or why he was “wasting” so much money in the hemophilia community.

These comments saddened Chris temporarily, but he rallied and focused on his goal—and he succeeded wildly. His strategic plan, which showcased a stunning dream, along with superb mentors and supporters, helped Chris Bombardier, a person with hemophilia, go down in history.

Conquer, Inspire, Save

Chris’s dream of summiting Everest and completing the Seven Summits was audacious. But he didn’t do it just for himself. He had a higher calling. As a board member of Save One Life, an international child-sponsorship nonprofit for children with bleeding disorders in developing countries, Chris knows well how much children without factor suffer. And he wants to help solve this terrible problem.

NOW AVAILABLE

rebinyn®
Coagulation Factor IX
(Recombinant), GlycoPEGylated

Learn more at
rebinyn.com

Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536 U.S.A.
Rebinyn® is a registered trademark of Novo Nordisk Health Care AG.
Novo Nordisk is a registered trademark of Novo Nordisk A/S.
© 2018 Novo Nordisk All rights reserved. USA17BI003303 January 2018

headlines



Chris Bombardier Achieves a Dream, Makes History!

On January 6, 2018, Chris Bombardier became the first person with hemophilia to summit Mt. Vinson in Antarctica, completing his quest to summit the highest peaks on the seven continents. The Seven Summits have been reached by only about 450 people, and this is the first time in history that anyone with hemophilia has achieved them. Chris chose this quest to raise awareness of the disparity in hemophilia treatment in developing countries, where only 25% of people with hemophilia have access to proper treatment. Chris is a board member of Save One Life, Inc., which gives direct financial aid to families with hemophilia. **Why this matters:** Chris has shown what is possible with appropriate treatment, training, and medical care, and has made history.

For info or to donate: www.saveonelife.net

Follow Chris at "Adventures of a Hemophiliac" on Facebook.

manufacturer

First SubQ Hemophilia Therapy Approved!

A new therapy for hemophilia A with inhibitors was approved by the FDA on November 16. ACE910 (emicizumab), created by Chugai and Genentech (both subsidiaries of Roche Group), will have the brand name Hemlibra® and is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children with hemophilia A with inhibitors.

Why this matters: This is the first new FDA-approved therapy to treat hemophilia A with inhibitors in nearly 20 years, and the first subcutaneous therapy for hemophilia—it is expected to be a market game-changer.

For info: www.gene.com

global

Hemophilia "UN" in Scotland

**World Federation of Hemophilia
World Congress
May 20–24, 2018
Glasgow, Scotland**



Organized by WFH and hosted by Haemophilia Society (UK), the congress unites patients, patient advocacy groups, and healthcare providers from over 120 countries to participate in symposia, social events, and meetings. **Why this matters:** WFH congresses are the largest international meetings for the global bleeding disorder community.

For info: www.wfh.org

A Gift That Gives

Octapharma AG donated 33 million IU of Nuwiq®, a 4th-generation recombinant (produced by a human cell line) factor VIII for treating hemophilia A, to Project SHARE. The donation boosted SHARE's factor inventory to over 43 million IU, shattering its previous annual average of about 7 million IU. Nine developing countries benefited from the donation. The Philippines, recipient of about 6 million IU, said this was its single largest donation ever. **Why this matters:** This is the largest donation received in Project SHARE's 15-year history, and will help thousands of hemophilia A patients who might never receive product.

For info: www.kelleycom.com



science

UniQure is advancing its hemophilia B gene therapy program in 2018. Using an enhanced version of its previous product AMT-060, AMT-061 combines an AAV5 vector with the factor IX-Padua variant. This variant has been reported to provide about an eight- to ninefold increase in factor IX activity as compared to normal factor IX. **Why this matters:** Gene therapy has been in research for years, but is advancing slowly toward a cure.

For info: www.uniqure.com

Factor IX Clinical Studies Moving Forward

After a patient died in a clinical trial of fitusiran (ALN-AT3SC), the FDA cleared Alnylam Pharmaceutical to resume phase 2/3 testing of the drug (on Dec. 15, 2017) after Alnylam agreed on safety measures and a risk mitigation strategy, including education for investigators and patients on using lower doses of factor or bypassing agent to treat breakthrough bleeds while in fitusiran clinical studies. Fitusiran, given subcutaneously once monthly, is an RNA interference inhibitor (RNAi) therapy that targets antithrombin, resulting in a dose-dependent reduction in the circulating antithrombin level and increased thrombin. **Why this matters:** Fitusiran is one of several small-molecule hemophilia therapies in development that are likely to upend the factor industry in coming years.

For info: www.alnylam.com



nonprofit

NHF 70th Annual Meeting in Orlando!

October 11–13, 2018

Orlando, Florida

Registration begins February 1, 2018

National Hemophilia Foundation welcomes all attendees to sunny Orlando for three days of learning, fun, and networking with the national bleeding disorder community. Guest speakers, games, and social events make this a valuable time for new families and regular attendees. **Why this matters:** This is the largest national hemophilia meeting in the world.

For info: www.hemophilia.org



HFA's Symposium at the City of Rock!

April 26–29, 2018

Cleveland, Ohio

Hemophilia Federation of America's annual, community-centered educational event draws hundreds of members from the bleeding disorder community to share information, learn new advancements, and build a support network. Travel scholarships for first-time attendees who need financial assistance. Check out the Rock and Roll Hall of Fame! **Why this matters:** As a grassroots event, HFA's symposium offers a different approach that complements NHF's annual meeting.

For info: www.hemophiliafed.org

soundbites



NHF's HemAware magazine has an excellent article on **hemophilia and bipolarism**: hemaware.org/mind-body/fighting-two-battles-bipolar-and-bleeding-disorders

Bayer submitted a Biologics License Application in the US for BAY94-9027, a long-acting, site-specifically **PEGylated recombinant factor VIII** for treating hemophilia A.

patient programs

Hemocraft

Pfizer has developed a new video game to teach hemophilia patients about their condition. Based on the popular sandbox game Minecraft, Hemocraft was developed in collaboration with the Entrepreneurial Game Studio at Drexel University and members of the hemophilia community. Players on a quest interact with a professional healthcare character—the village doctor—to learn how to follow their treatment plan, why treatment is important, and how it works. **Why this matters:** The game offers young patients, ages 8–16, a fun way to learn about integrating treatment into their routine.

To download: hemocraftquest.com



AN EPIC QUEST TO SLAY A DRAGON AND STAY ON TREATMENT!

Disaster Relief for Puerto Rico



A group of national and state organizations, including NHF, HFA, Coalition for Hemophilia B, and Hemophilia Alliance, have agreed to a plan for soliciting and distributing funds to assist

families with bleeding disorders in Puerto Rico. Most of the families registered with the national public hospital in San Juan sustained damage to their homes from Hurricane Maria on September 20. Though FEMA and humanitarian organizations are helping the island, many families still need help. Funds will be channeled through HFA's disaster relief program. **Why this matters:** In times of crisis, donated money can be mismanaged, but with a solid plan, donations will go straight to families with bleeding disorders.

For info: hemophiliafed.org

In Memoriam

Harold R. Roberts, MD, who served as the first executive director of the International Society on Thrombosis and Hemostasis (ISTH), passed away in September 2017 at age 87. Dr. Roberts earned his undergraduate and medical degrees from the University of North Carolina–Chapel Hill, where he was appointed to the faculty in 1961. Four years later, Dr. Roberts co-developed the first high-purity factor VIII concentrate for treating hemophilia A; the product was later commercialized by Baxter Hyland. In 1978 Dr. Roberts founded the UNC Center for Thrombosis and Hemostasis, which he oversaw for the next 20 years.

In 1987, a UNC patient with hemophilia A, George McCoy, who also passed away in 2017, became the first person in the world to be infused with recombinant factor VIII. Dr. Roberts received numerous honors for his achievements in hematology research, including the French International Prize for Research in Hemophilia, Kenneth Brinkhous Award for Excellence in Clinical Research from the National Hemophilia Foundation, and American Society of Hematology's Henry M. Stratton Medal.





A Breakthrough for People with Inhibitors?

In two phase 3 clinical trials, Hemlibra demonstrated impressive results, with a significant reduction in bleeding episodes, and in many cases, with no reported bleeds!²

Two additional phase 3 clinical trials of Hemlibra are also underway: Haven 3 is evaluating prophylactic use of Hemlibra versus no prophylaxis in hemophilia A participants without inhibitors (to be completed in September 2019). And Haven 4 is looking at higher doses of Hemlibra, but monthly dosing as opposed to weekly dosing (to be completed in July 2018).³

A Black-Box Warning

Despite the impressive results, Hemlibra carries an FDA “black-box” warning—the strictest of warnings the FDA can require a manufacturer to put on prescription drug or drug product labels. Hemlibra’s product insert carries extensive warnings of an increased risk of thrombotic events if the product is used with FEIBA. Thrombotic events are clots that cause partial or total obstruction of a blood vessel.

Why the need for warnings? In late 2016 and early 2017, Roche reported that five participants (out of 109) who were being treated for breakthrough bleeds in the first clinical trial had experienced serious thrombotic events. In all of these cases, the affected patients had received repeated doses of aPCC (FEIBA) to control breakthrough bleeding. Roche concluded that simultaneous use of aPCC and ACE910 is associated with an elevated risk of thrombotic events. So the company issued a new protocol in its clinical trials that recommends avoiding aPCC whenever possible and only administering at low doses. After the new protocol was implemented, no new cases of thrombotic events were reported.

Hemlibra’s Potential Impact on the Marketplace

Right now, Roche is targeting only people with hemophilia A and inhibitors. Roche revealed its pricing for Hemlibra in November 2017: “At an average weight of about 127 pounds, the cost is approximately \$482,000 for the first year of treatment and then approximately \$448,000 per year, which is less than half of the WAC [wholesale acquisition cost] of the only other approved prophylactic treatment for these [inhibitor] patients.”

Hemlibra’s price still places it within the top 10 most expensive drugs for rare conditions. And for people without inhibitors, the cost is significantly higher than prophylaxis with factor concentrates. It remains to be seen whether Roche will retain the present pricing

Disruptive Therapies for Hemophilia in Development

- **Gene therapy for hemophilia A and B** is underway in almost a dozen clinical trials. These trials have produced significant and sustained results and, in some cases, have cured patients of their hemophilia.
- Catalyst Biosciences and ISU Abixs are conducting a phase 1/2 clinical trial of their highly potent, **next-generation factor IX product (CB 2679d)** that is administered subcutaneously to treat **hemophilia B**.
- Alnylam Pharmaceuticals and Sanofi are restarting a phase 1/2 clinical trial of a subcutaneously administered RNA interference (RNAi) therapy, **ALN-AT3** (also called fitusiran), that targets antithrombin (AT, a protein that inhibits clotting) to improve thrombin production. **Fitusiran** can be used to treat **hemophilia A or B**, with or without inhibitors.
- Bayer Healthcare Pharmaceuticals and Novo Nordisk each have phase 1/2 clinical trials underway testing **concizumab**, a subcutaneously administered monoclonal antibody that is an anti-tissue factor pathway inhibitor (anti-TFPI). Like AT, TFPI is a protein that inhibits clotting. By blocking the function of TFPI, concizumab improves thrombin production and can be used to treat **hemophilia A or B**, with or without inhibitors.
- Chugai Pharmaceutical Co., Ltd., and Genentech (both subsidiaries of the Swiss healthcare company Roche Group) have underway two phase 3 clinical trials (Haven 3 and Haven 4) of **Hemlibra** (emicizumab-KXWH, formerly ACE910), a subcutaneously administered bispecific antibody that mimics the function of factor VIII, to treat **hemophilia A with or without inhibitors**.

structure in a year or two—after obtaining an additional FDA indication for use of Hemlibra on patients without inhibitors. If the price doesn’t drop significantly, Roche will encounter pushback from insurance companies, slowing its move into the hemophilia marketplace for patients without inhibitors.

You can be sure that current leaders in the hemophilia marketplace won’t “go without a fight.” Assuming Roche can navigate legal challenges, Hemlibra is certain to make waves in the annual \$2 billion BPA marketplace, affecting mainly Novo Nordisk (manufacturer of NovoSeven® RT, the most popular recombinant factor VIIa BPA used in inhibitor patients) and Shire (manufacturer of FEIBA, the most commonly used aPCC BPA for inhibitor patients).

Hemophilia patients are famous for their reluctance to switch products. But Roche has a winning combination with its new product: Hemlibra is significantly less expensive than current inhibitor therapies and significantly more effective at preventing bleeds. So Roche is poised to upend the hemophilia inhibitor market. And, if Roche can lower the cost when offering the product to people with hemophilia and without inhibitors, then the company can potentially upend the entire clotting factor industry—which mainly consists of producing factor VIII and BPAs.

Hemlibra is only the first of several disruptive therapies that will probably enter the market over the next several years. The wheels of change have been set in motion. Will the manufacturers lower their prices to stay competitive? Possibly, but prices did not drop with the introduction of any of the new clotting factors over the past few years. Will some manufacturers go out of business? Also possible, but not likely—all the big players in the hemophilia market have been planning for the arrival of disruptive therapies for some time, and they’re also developing their own disruptive therapies. This is the first rumbling of what will be a seismic shift in the hemophilia industry: within a decade, the hemophilia industry—and how we treat bleeds—will look very different. ☺

2. The Haven 1 clinical trial studied once-weekly prophylaxis with Hemlibra compared to on-demand bypassing agents in adults and adolescents with hemophilia A with inhibitors. Results showed a reduction in bleed rate of 87% with Hemlibra prophylaxis, compared with on-demand treatment with BPA. And after a median observation time of 31 weeks, 62.9% of patients receiving Hemlibra—compared to 5.6% of those receiving on-demand BPAs—experienced zero treated bleeds. 3. For more information on Roche’s clinical trial involving Hemlibra, see www.otcmarkets.com

"I'd still be proud to have done the Seven Summits as a personal accomplishment," Chris acknowledges, "but I'm more excited that there's a bigger purpose to this. I think that's the reward for me, and this will benefit more than just me. I'm hopeful about how the summits will impact patients in developing countries through greater public awareness and increased funding." Like many parents and patients living with hemophilia, Chris has tremendous compassion and empathy for others. When he summited Aconcagua in subfreezing temperatures, he carried with him the picture of young Brian from Zimbabwe, an impoverished orphan in severe pain with many joint contractures and bleeds. "It's so resounding for our community," says Chris. "We've all gone through this pain. And now we want to help others." Chris's climbs have helped raise funds for Save One Life's programs, and helped inspire a movie currently being made about his summit quest, *Bombardier Blood*.⁵

Chris's message to people in the bleeding disorder community: "I hope to inspire others to reach their goals." To do that, he

advises, "Focus on something specific. Have a vision and goal that relates to something you care about deeply and believe in. And make sure you know why you care about it."

For Chris, the mountain is the metaphor for his life. And Everest can be the metaphor for your own life. Does your "Everest" seem impossible? To Chris, climbing the Seven Summits seemed impossible at first. But almost nothing is impossible when you are clear and committed, have a strategy, and persevere step by step.

On the trek to Everest base camp, Chris and his guide Ryan passed by the daunting mountain Ama Dablam. Chris told Ryan that he didn't understand how anyone could climb it. Ryan replied, "Well, it's the funny thing about mountains. The more steps you take toward them, the less challenging they look. A long ways away, everything looks so daunting. But with every step you take, you can see the next step."

And with each planned step, you will get closer to *your* dream, *your* Everest. Make it happen this year! ☺

5. *Bombardier Blood* was conceived and is being directed by Patrick James Lynch, person with hemophilia and founder and president of Believe Ltd. A trailer is available at www.bombardierblood.com.

Richard's Review... from page 5

resistance workers in a secret attic room. In February 1944, the Gestapo arrested Corrie, along with her sister and father, and then imprisoned all three in concentration camps in Holland and Germany. Only Corrie survived.

One irony is that the ten Boom family had always taken in those in need, including undernourished children from Germany after World War I, followed by foster children. Coincidentally, Corrie knew of hemophilia in Holland. In her book *In My Father's House*, she mentions that during the 1930s, a Dutch woman died after childbirth because of her hemophilia.

Both family stories of survival during the Nazi occupation of Holland stress the hardships from shortages of all basic necessities and the lack of freedom. Add to that the constant threat of arrest,

imprisonment, and even death, due to resistance activities.

What these stories omit is the danger to children with mental handicaps, physical disabilities, and hereditary diseases, including those with hemophilia. These children risked being killed by the Nazis according to the regime's "euthanasia" ideology.³

We don't know exactly how many people with hemophilia were killed. And because carrier status could not be determined, any sister, without confirmation or diagnosis, would be considered a potential carrier and could be forcibly sterilized. It's hard for us to realize today that, under the Nazis, having hemophilia was a crime punishable by extermination. Let's hope that with continual vigilance, those heinous practices will never happen again. ☺

3. Also called "mercy killing," this policy targeted children and adults with disabilities whom the Nazis wanted to eliminate, to improve "Aryan racial purity." Those who were not killed were forcibly sterilized. Many people with disabilities were housed in large institutions or asylums, so the Nazis could easily locate and kill them without public attention. The best estimates for the total number of people with disabilities who were murdered by the Nazis is at least 750,000, plus 500,000 forcibly sterilized.

YOU... from page 6

treatments based on their illness, and those with complex medical devices like trachs and heart-pumps."

Never Too Early to Start!

It's never too early to start teaching our children the importance of setting goals and achieving them.

Christy VanBibber's eight-year-old daughter Elise was disappointed by her neighborhood's lack of knowledge and awareness of hemophilia. When Elise began sharing the news about her baby brother Timothy, who has hemophilia, she realized that many people didn't know what hemophilia was. "She came home with a real concern," Christy notes, "and asked, 'Mom, why does nobody know what hemophilia is?'"

Elise made it her goal to generate awareness in her community

about hemophilia—by making and selling beaded bracelets and then donating all proceeds to the Southwestern Ohio Hemophilia Foundation.

Christy recounts the initial success: "She made some bracelets and asked me to put them on Facebook. We decided to sell them for \$3 each. Immediately she sold ten!" Elise went on to sell her bracelets at two local farmers' markets and local football games.

Christy was thrilled. "She raised \$880! Also, people recognize Elise and Timothy at school and the grocery store, asking questions and praising her for such a great job. We are really proud of her!"

All these women—and one young girl—achieved their goals fueled by close-to-home, real-life concerns, and you can too! If you want to learn how you can reach your goals this year, check out our cover story and find out how to make your goals SMART. ☺



Project SHARE

WE ARE GRATEFUL to Project SHARE and Octapharma AG for choosing the Philippines as one of the recipients of 6 million IU of factor. This is the first time ever that the Philippines has received such a huge donation. It means that for the next few years, Filipinos with hemophilia A will have immediate access to treatment.

Since we received the donation of Nuwiq® in May, we have already responded to about 1,000 cases, many of them life-threatening bleeds.

The Philippines is an archipelago of 7,107 islands, so we developed a distribution system to send factor to our chapters or contact people, especially in distant areas. This has been lifesaving, because patients no longer have to wait for factor to be sent from Manila, which usually takes two to four days.

Hemophilia Advocates-Philippines has about 500 registered members, but we also regularly serve three other hemophilia organizations outside of Manila with a combined membership of about 200. According to World Federation of Hemophilia (WFH), about 8,000 Filipinos have hemophilia A, although the Philippine registry has identified over 1,500 so far.

The donation has also helped us in our lobby and advocacy work. Experiencing ease for the first time in getting treatment, many members of our community became inspired to join us in lobbying for a law on hemophilia care. We now have a growing number of active volunteers in our youth group, women's group, and men's group. People could hardly participate before, because of bleeds; but now that they get immediate assistance, they can volunteer to help in the office, do home visits, provide support, or share information from our monthly patients' education program.

Andrea Trinidad Echavez
President, Hemophilia Advocates-Philippines

IN 2016, WE got 1.6 million IU of factor VIII from WFH. In 2017, we got 1.4 million IU of factor VIII from WFH and 1.2 million IU of factor VIII from Project SHARE. Your donation was significant, and we'd never dreamed of such generosity.

Of your donation, 612,000 IU was used for prophylactic treatment for 15 children

with hemophilia. We could not practice prophylactic treatment without your donation. Another 45,000 IU went to upper Myanmar, where a colleague runs a hemophilia center. The remaining factor was used in our center, Yangon General Hospital, and for emergency surgeries.

Haemophilia Society of Myanmar, the caring physicians, and our hemophilia patients wish to thank Project SHARE and Octapharma AG for the generous donation for Myanmar. Without proper prophylaxis and on-demand treatment, hemophilia patients' lives could become miserable due to chronic hemarthropathies, crippling arthropathies, and severe disabilities. Without emergency factor support, savable lives can sadly be lost.

Dr. Sein Win
Haemophilia Society of Myanmar

I DIDN'T KNOW about Project SHARE for many years, and only learned about it when I met Father Don Kill in 2007. Fellow patients with hemophilia are lucky now to receive free factor, though in my time, we sold everything we had to buy factor. I wish all with hemophilia could be made aware that Project SHARE exists and provides free factor. Thank you for saving my life during my major surgery in 2007. I'm looking at my deformities now, wishing I could turn back time, and that I'd found Project SHARE earlier. But I am thankful to have a second life now.

Joe Angelo Cuevas
PHILIPPINES

THANK YOU HEMOPHILIA Advocates-Philippines, Andrea Trinidad Echavez, Laurie Kelley, and Project SHARE for the factor VIII donation. I'm very thankful because I was able to attend my afternoon class after my infusion. Without you, we can't continue to pursue our dreams and fulfill our daily tasks. We hope that you always support the HAP Youth Group. God bless!

Peter Paul Colinares Carreon
PHILIPPINES



THANK YOU FOR the donation of factor and for the wonderful work that you and your team are doing.

Sanjeev Kumar
Fiji

YOUR FACTOR IX donation arrived safely. This shipment has been well received and we thank you so much for your love and consideration toward our son Hirwa! He is improving.

Sylvestre Mulinda
RWANDA

THANK YOU FOR your efforts to facilitate and obtain this expensive medicine for our patients. I received the concentrate from FedEx, and it's now in the pharmacy for storage.

Dr. Jackson Kasiime
UGANDA

WE HAVE RECEIVED the donated FEIBA and factor IX. We want to express our sincere gratitude for your support. The medicines will be of great help to our members.

Suraksha Thapa
Nepal Haemophilia Society

**Our Deepest
Thanks to
PEN'S CORPORATE
SPONSORS**

Shire

844-229-2582
bleedingdisorders.com


novonordisk®

800-727-6500
novonordisk-us.com

Hemophilia History Made! See page 11

**Talk to your doctor to see if
ADYNOVATE may be right for you.**

For more information, please visit
www.ADYNOVATE.com


ADYNOVATE
[Antihemophilic Factor
(Recombinant), PEGylated]

©2016 Shire US Inc., Lexington, MA 02421. All rights reserved. 1-800-828-2088.
SHIRE and the Shire Logo are registered trademarks of Shire Pharmaceutical Holdings Ireland Limited or its affiliates.
ADYNOVATE is a registered trademark of Baxalta Incorporated, a wholly owned, indirect subsidiary of Shire plc.
S32930 06/17

