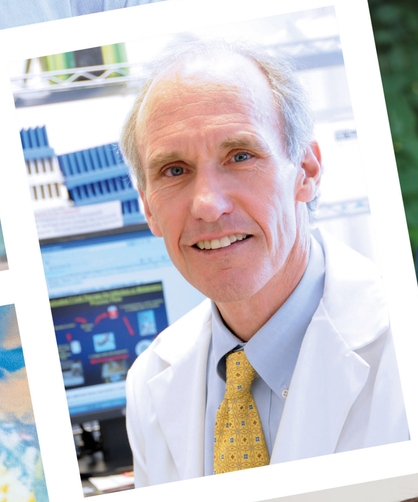
Special Series
Pioneers

From Carl June to Peter Marks to Kiran Mazumdar-Shaw; check out our thought-provoking interviews with some of the biggest names in the pharma field.



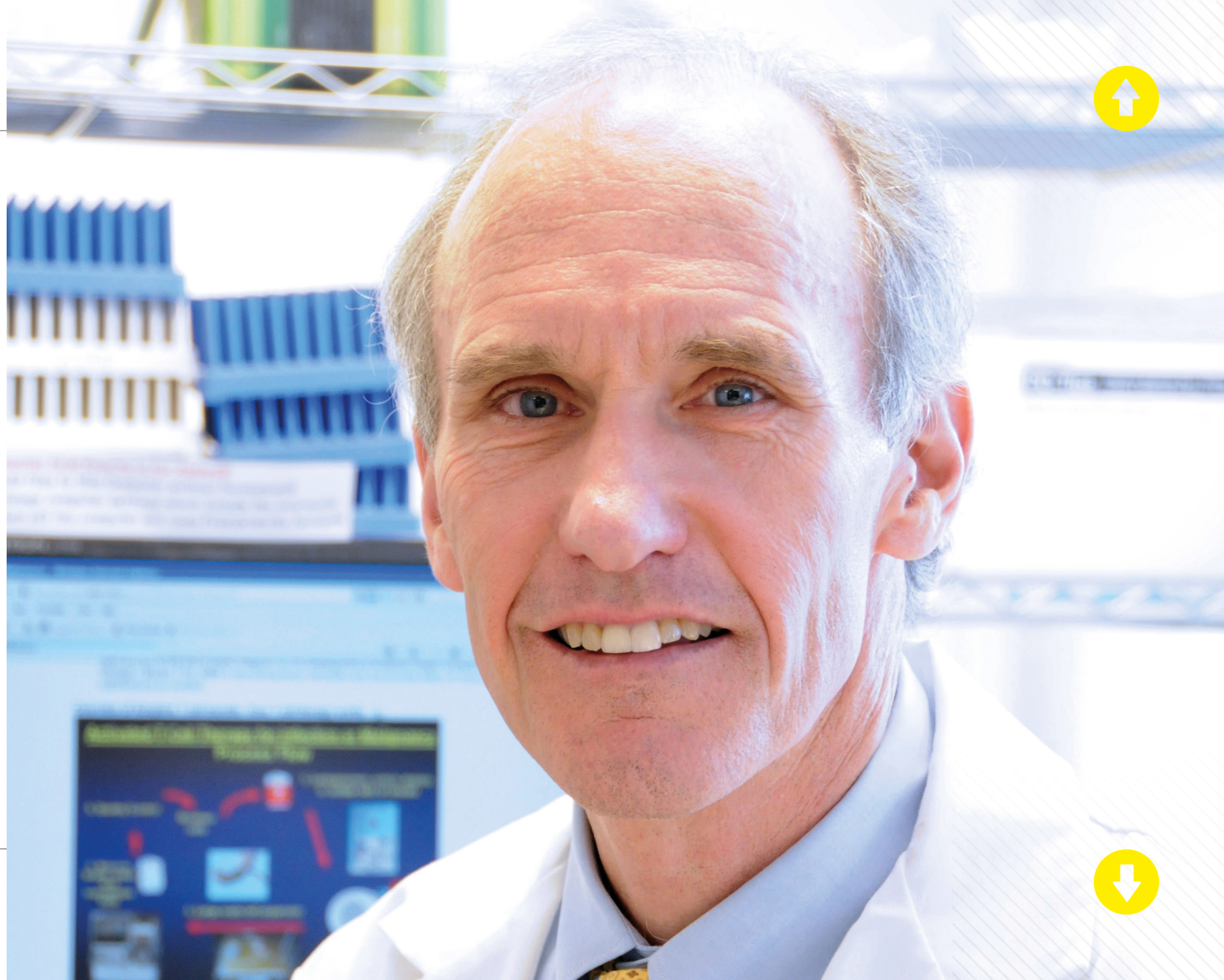
the Medicine Maker

Time for (CAR) T

Carl June, considered the father of CAR-T therapy, has many affiliations. He is Richard W. Vague Professor in Immunotherapy, as well as Director of the Center for Cellular Immunotherapies at the Perelman School of Medicine, Medicine Director of Translational Research Programs and Director of the Parker Institute for Cancer Immunotherapy, all at the University of Pennsylvania. He tells us about the roots and successes of his research, as well as his thoughts on academia and industry, and the emotional impact of patient-doctor relations.

How did you feel when you were recently honored at Advanced Therapies Week with the Lifetime Achievement Award?
It is a huge honor – and really it's down to the team. In some cases, I've been working with people for 25 years who have been involved with this. It's great to have the recognition, particularly at such an exciting time for the field since it's the 10-year anniversary of when we started CAR T therapy in humans.

How far back in time do the roots of CAR T reach?
The first successful cell therapies in humans were bone marrow transplants in the 1980s. In this type of transplant, a donor's T cells are given to a patient with cancer, but the cells are not genetically modified. Around 1989, Zelig Eshhar at the Weizmann Institute of Science made something called "T bodies." He made the first T cell that worked with antibodies binding the target cells instead of a T cell receptor – and this is really at the heart of what a CAR is. With this work in place, it was acknowledged that T cells could be very potent for bone marrow transplants and could work with an



antibody redirection – a chimeric form of a cell between a B and a T cell. However, it took until 2017 to get FDA approval for a CAR T.

How did you get involved with this field?
I'm a medical oncologist and immunologist. After completing medical school, I trained in bone marrow transplantation and became interested in how T cells could activate and kill with "graft versus host disease". In the case of a bone marrow transplant, donor T cells can go out of control and cause severe damage. T cells are highly potent and research in this area has led to breakthroughs in CAR T therapy – but it's taken 25 years to get to this point.

Looking back to what first got you interested in the field, do you think the success of CAR T could have been predicted?
No one could have predicted what has happened in CAR T – for many reasons! For one thing, it actually worked a lot better in our initial trials in humans than it had worked in mice. That's a very unusual situation; over the years, many mice have been cured of cancer but there are still only very few new therapies for humans. Also, back in the 1990s, there were only about five labs working on CAR T cells. There was no pharmaceutical industry involvement back then, and for the academics (including my own lab) that were working on the topic, it was more of an academic thought experiment: Could you redirect a T cell and use it to treat cancer? We weren't necessarily thinking it would or could ever be commercialized, but it worked. Back then, there was no cell therapy industry – but now there is. And the statistics are amazing.

You were in the movie Of Medicines and Miracles; how did that come about?
Ross Kauffman is an accomplished documentary filmmaker who has won Academy Awards. When he saw the first report in The New York Times about our CAR T cell therapies, he thought it would be an interesting story.

He got permission to make a three-minute documentary called Fire With Fire about Emily Whitehead's treatment, severe cytokine storm, and then recovery. That three-minute video went viral with about 25 million views, and it also served a really important purpose because it allowed people to see that cell and gene therapy had promise. It also helped increase research funding – which at the time had been difficult to obtain.

Ross Kaufman then decided to make a full-length documentary, which was released in 2022 at the Tribeca Film Festival. It's been an exciting time and I never thought in my career that I would end up in a film! I'm really glad that he has made Of Medicines and Miracles because it highlights the true benefits of these new therapies, and can help educate the public about the long-term need for funding basic science research.

How has CAR T success affected the University of Pennsylvania?
Usually, new findings in academia at the bench get licensed and go into industry so there is a clear handover. Since there is a handoff, the academics don't really benefit from the growth or participate in new directions of the research. In the case of the CAR T cells, we worked with those first patients, and that caught the attention of Novartis, who then licensed the CAR molecule; we had a very vibrant research partnership with them.

The effect of that first CAR T trial has led us to become a center with broad experience and expertise. And that has led to new faculty, attracted very talented postdocs and graduate students, and led to huge growth and innovation at the university. But the real reason it happened is strategic planning. In the early 1990s, Penn made a strategic plan to bring in cell and gene therapy, which is how I got recruited to the university in 1999 to establish human immune therapy. Today, there is a large and diverse research portfolio at the university.

Are you emotionally affected by your work?
Personalized therapy is a unique experience because the therapy is made from the patient's own cells. When a pharmaceutical company usually makes a batch of drugs, the people who make that product never actually see the patients. But with cell therapy, it is hard baked into what we do. We get the blood from the patient and then a few weeks later the patient gets their treatment. The people in our group get to know our patients and it is hugely rewarding. We've seen cases where people are deathly ill but then they come back to our center and they are healthy: true Lazarus cases. When you are so involved with the patient, this experience is hard to put into words.

You are well known in these circles for being named one of the 100 most influential people in the world in 2018. But could you share a little known fact about yourself?
I've had a lifelong interest in athletics. During the COVID-19 pandemic, I suffered a little from cabin fever because I could not go outside and do the races that I usually do, so I took part in something called the Everesting Challenge, which is where you go up the equivalent height of Mount Everest (29,000 feet) in one bike ride. About 12,000 people – and only seven of my age – have done the challenge. I did it in Haleakala with my wife and daughter. And it took 19 hours!

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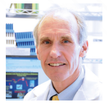
Online Article



Watching the Future

We spoke with Peter Marks when he was still the Director of the Center for Biologics Evaluation and Research at the FDA. He resigned from the agency in 2025, but has been described as a visionary leader and champion of cell and gene therapy who has helped bring many new breakthroughs to patients.

How did you start your career?
I went into college thinking I was probably going to become a PhD biochemist. At the time, cell and molecular biology were becoming more popular and they caught my interest. After I got a part-time job at a hospital taking blood, I started to think about going to medical school and working in medical research. Ultimately, I chose to become a physician-scientist but, over the years I've occupied several different roles in academia and industry.

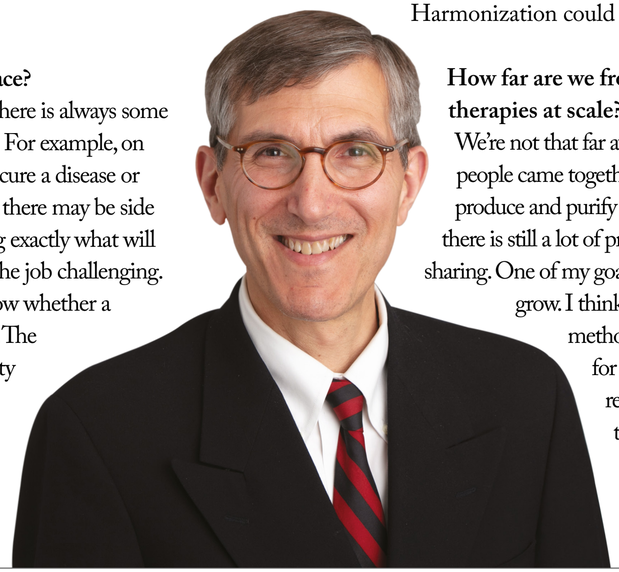
How did you get involved with the FDA?
My first industry role was with Genzyme and involved interacting with the Center for Biologics. The Center had both an applied scientific research component and a regulatory component working with a nifty set of products. It was so interesting to me that, in 2011, I applied for a job there. At the time, gene and cell therapies were becoming very exciting – and, as a hematologist-oncologist, blood products were, of course, of interest to me. The opportunity to have an impact on the development and availability of important medical products was attractive. And it meant I could make use of different skills in one job. What I do now is a combination of science, medicine, administration, and even a little teaching from time to time.

What skills are important for a regulator?
Looking at people who have been mentors in this space, like Janet Woodcock, it's clear that you need to understand science and medicine really well to do a good job as a regulator. This includes the science at a fundamental level, as well as the manufacturing of products and the technologies involved. Without that knowledge, you can't make necessary decisions about cutting-edge products. You also have to know how to manage people. The Center for Biologics has around 1,300 full-time equivalents. They are mostly knowledge workers… and managing knowledge workers can be challenging. You need to know when to zoom in to get into the weeds of the data and when to zoom out and let others deal with the data while you make the high-level decisions. That, to me, is an important balance to have.

What is the biggest challenge you face?
The biggest challenge is uncertainty. There is always some uncertainty with cutting-edge science. For example, on one hand, a gene therapy may help to cure a disease or treat it long-term. On the other hand, there may be side effects associated with it. Not knowing exactly what will happen ahead of time is what makes the job challenging. Sometimes, it takes a long time to know whether a decision was a good idea or a bad one. The challenge is to negotiate the uncertainty in as skilful a manner as possible.

What work are you doing in terms of harmonizing gene therapies?
This is one of my favorite topics and an area we are actively working on. We're developing a white paper on global harmonization of cell and gene therapy regulatory approaches. If we have different regulatory frameworks in different countries, then patients in different countries likely will be deprived of these therapies simply because of the cost of market entry. If studies are performed in one location and are then required in a different location, that will present a barrier. We're going to need a lot of work to move toward harmonization and we'll need to start small. Right now, if someone in the US develops a therapy for mucopolysaccharidosis type I and someone in the EU develops a therapy for mucopolysaccharidosis type III, the regulatory requirements may be different and the therapies may never cross the Atlantic. This means patients would have to travel to get access.

Harmonization could help therapies enter other countries.



How far are we from being able to manufacture gene therapies at scale?
We're not that far away, but there are challenges. With mAbs, people came together to help develop technologies that could produce and purify large protein quantities. With gene therapies, there is still a lot of proprietary work that can limit information-sharing. One of my goals is to help the field share information and grow. I think we can make better cell lines and purification methods and develop continuous methodologies for producing these gene therapies. But that will require a type of collaboration and cooperation that we haven't yet fully achieved.

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Rebel With a Cause

Kiran Mazumdar-Shaw is the Chairperson and Managing Director of Biocon, India. She is seen as a pioneering biotech entrepreneur, a healthcare visionary, a global influencer, and a philanthropist. She is the recipient of two of India's highest civilian honours: the Padma Shri and Padma Bhushan. She has also received many other accolades from overseas.

From Master Brewer to The Medicine Maker Power List. How?
In India in the 1970s, it was really tough to get acceptance as a female brewmaster, and I quickly realized that although I was very keen to pursue this profession, it was going to be tough to earn credibility within the brewing fraternity. People believed that a woman would find it difficult to deal with male employees and trade unions – a lame excuse, especially as even then I was being consulted for advice on operational issues by leading breweries! Disappointed with the system, I took up a job offer with a leading brewery in Scotland and was preparing to fly out of India when I had a chance meeting with an Irish entrepreneur who offered me an opportunity to set up a biotechnology company in India. I thought, why not start my own business and show them what a woman can do? It was rebellion and the desire to prove myself that made me take up the challenge. In those days, biotechnology was an unknown area in India and enzyme technology, which I started with, was unheard of.

Who inspires you?
I'm inspired by people who are change-makers – people who go off the beaten track. While building my own company, I was inspired by Anita Roddick (founder of The Body Shop) because she is someone who has changed the rules of her business. I was also inspired by one of the leading bankers in India, who acted as my mentor. He was one of the first Indian bankers to go into venture funding with Biocon. When I first discussed my business idea with him, he got very excited. In those days the normal way of funding a business was to take a debt-based loan, but he told me that instead of giving me a loan he would like to fund me in exchange for a small stake in the company – that was music to my ears! Through my entrepreneurial and business journey I have always challenged the status quo and tried to create a new business model. In fact, I've always tried to do things in a different way. I think the difference lies in my DNA.

What do you consider your greatest achievement?
For 20 years, I developed innovative enzyme technologies for a large number of industries. Then, in the 1990s, I decided to transform my business model and leverage our strengths in technology for cutting edge innovation towards one goal – to produce biopharmaceuticals. That was a great inflection point in my entrepreneurial journey. I'm glad I made that decision because I'm really passionate about delivering affordable biotech-based drugs around the world. One of the biggest challenges in the developing world is affordable access to these very expensive complex biological drugs. As an Indian, my whole ethos is to address that problem. I often say a blockbuster drug should not be measured by the billion dollars it earns, but by the impact it makes on a billion patients.

What's next for Biocon?
The drugs we have in the pipeline are very exciting. For example, we have an oral insulin under development, which could be a huge

game changer in diabetes management. Early insulin therapy can be hugely beneficial to diabetic patients but compliance is poor for injected insulin so it is usually only prescribed in severe cases. A tablet form will be easier to administer, so has a lot of potential for diabetes management.

We also have a range of monoclonal antibodies. I'm very excited by the whole area of immunology. The immune system has a major role to play in a large number of diseases, especially those treated with biopharmaceuticals – whether it is diabetes, cancer or autoimmune diseases. The importance of antibodies is gaining a lot of traction and there are so many inspiring discoveries being made. This makes it very exciting for me as a scientist and as an entrepreneur.

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Online Article

The Speed of Science

From the GSK boardroom to the White House, and now the board of Abzena, the career path of Moncef Slaoui has resulted in billions of doses of lifesaving vaccines. He shares his story.

What inspired your interest in science, and how did you come to focus on biologics and vaccines?
My interest in science began early in life. I have always been fascinated by the complexity of the human body and living organisms, and I was eager to understand their mechanisms beyond the religious explanations provided in my early education. By the age of ten, I had developed a strong passion for biology, which ultimately drove me to pursue a career in science. While my initial ambition was to become a physician, I eventually pursued a PhD in immunology, which led me to the study of the immune system.

My transition into vaccines was somewhat serendipitous. During my postdoctoral research in the US, my future wife, an HIV expert, was recruited by a vaccine company, which was a division of GlaxoSmithKline (GSK) in Belgium. As an immunologist, I began advising the company, and through that engagement I developed a deep appreciation for vaccines.

A personal factor also played a role in my commitment to vaccines; my parents lost a child to whooping cough before I was born. This tragedy instilled in my family a strong awareness of the importance of immunization. Over time, this awareness evolved into a lifelong professional passion.

You have been involved in numerous vaccine projects. What do you consider the biggest challenges in the field?
Vaccine development has evolved significantly over the years. Historically, vaccines were produced by growing entire pathogens — bacteria or viruses — and then either killing or attenuating them for use in immunization. However, advances in immunology and molecular biology in the 1960s revolutionized the field. These breakthroughs enabled us to identify and utilize specific proteins from pathogens, allowing for more targeted and rational vaccine design.

Despite these advances, several challenges remain. The first major challenge is overcoming pathogens that have evolved sophisticated mechanisms to evade the immune system. Viruses such as HIV and herpes, as well as bacteria such as chlamydia, have developed ways to subvert immune responses, making vaccine development for these diseases extraordinarily difficult.

The second challenge lies in the complexities of large-scale vaccine manufacturing. Producing vaccines requires a high level of precision to ensure consistency across billions of doses. This demands advanced infrastructure, significant investment, and highly skilled personnel.

A third and growing challenge is public trust. Vaccines are only effective if people accept and receive them. Unfortunately, misinformation has contributed to a decline in vaccine confidence, which is deeply concerning. Recent policy changes in the US may further exacerbate this issue, making it even more critical to engage in transparent and effective communication about the safety and necessity of vaccines.

How did it feel to be involved in major vaccine projects like Shingrix and the malaria vaccine?
I feel incredibly fortunate to have played a role in these projects.

My early involvement in vaccines happened largely by chance, but it became a deeply fulfilling career. Some vaccines, such as the malaria vaccine, took over 25 years to develop from initial concept to regulatory approval. The moment we receive key data demonstrating efficacy is profoundly emotional. I have often found myself overwhelmed with joy, sometimes even in tears, knowing that our work will save millions of lives.

This impact is particularly meaningful in low-income countries, where access to life-saving vaccines can determine survival for large populations. When I was an executive at GSK, I ensured the company remained committed to developing vaccines and medicines that were not necessarily lucrative but were crucial for global health. The malaria vaccine, for example, was never designed to generate profit – it was designed to save hundreds of thousands of children's lives each year in sub-Saharan Africa.

How did you become involved in Operation Warp Speed, and what was the experience like?
My involvement stemmed from prior collaborations with the US government during previous health crises, including the H1N1 flu pandemic in 2008, the Ebola outbreak, and the Zika virus outbreak. During those events, I strongly advocated for better pandemic preparedness. Unfortunately, little action was taken at the time.

When COVID-19 emerged, former congressman Jim Greenwood reached out to me, and after a 45-minute discussion on how to accelerate vaccine development, he informed me that the White House would likely call. Within days, I was in discussions with senior government officials, including Jared Kushner and Alex Azar. I agreed to lead the scientific efforts of what was initially called the "Manhattan Project 2," later known as "Operation Warp Speed".

The first major decision was selecting which vaccines to support. Over 100 candidates were submitted, but I narrowed them down to six based on my experience and intuition. Fortunately, all six turned out to be highly effective. The program involved thousands of professionals across pharmaceutical companies, the US Army, and various agencies. It was an intense, high-pressure experience, but ultimately, we achieved our goal of delivering vaccines in record time.

Given the current political climate, do you feel your efforts were in vain?
Not at all. Our work was never just for the US, it was for the world. Approximately six billion people have been vaccinated, saving tens of millions of lives. While political controversies and misinformation have been frustrating, I remain confident in the vaccines' effectiveness and safety. The rapid development process was rigorous, and after billions of doses, we know these vaccines are both safe and critical in preventing disease. Science enabled us to reclaim normalcy, and that is something to be proud of.

How did you become involved with Abzena, and what excites you about the company?
Since retiring from GSK, I have been active in venture capital, supporting biotech companies. Abzena caught my attention because it operates at the intersection of discovery and manufacturing, offering expertise in designing complex biologics and bioconjugates, with the capability to produce them at scale. Their ability to bridge these two critical areas is invaluable, particularly in the era of advanced biopharmaceuticals such as bispecific and trispecific antibodies, antibody-drug conjugates (ADCs), antibody-oligonucleotide conjugates (AOCs), and radiopharmaceuticals.

What trends in biopharma particularly interest you?
I am especially excited about engineered antibodies, which are revolutionizing oncology and immunoinflammatory diseases. Additionally, cell therapies hold immense potential, though manufacturing remains a significant hurdle. Vaccines remain a core passion, and I continue to monitor new technologies in that space.

With Donald Trump back in office, do you anticipate significant policy changes?
Every administration brings changes, and while I have had many interactions with President Trump, my focus is on science, not politics. My priority is to contribute where I can to advancing healthcare and making scientific progress. Scientists should engage with policymakers constructively while maintaining scientific integrity.

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Online Article

From Start-Up Biotech to the Boardroom

"The drive for me is enabling more patients to access medicines by doing things smarter and more efficiently, so that we can offer the same products at more affordable prices." Anne Marie de Jonge-Schuermans, Board Member of the Swiss Biotech Association and Global Head of Biologics & Injectables Operations at Sandoz, is passionate about patients and access to medicine.

What is it about your current role that you love?
My career has progressed in unpredictable ways. I've worked in prescription pharma, OTC medicines, innovator biotech, and now I'm focused on biosimilars and generics, which I find very rewarding. You may know that Sandoz pioneered the launch of the world's first biosimilar in 2006. And the drive for me is enabling more patients to access medicines by doing things smarter and more efficiently, so that we can offer the same products at more affordable prices.

I manage a couple of factories and the company has made substantial investments to ensure the future of our biosimilar business. We have 10 biosimilars on the market, and 24 biosimilars in our pipeline. We continuously scout the reference medicines market to select suitable candidates for biosimilars that we can develop and offer to patients at affordable prices. It's not easy to make a biosimilar, especially when you want to be the first to market, and there is always a lot of pressure to do it better, and faster – but that's also fun and rewarding too.

How did you come to work with the Swiss Biotech Association?
I previously worked for Sobi (Swedish Orphan Biovitrum), which acquired various small biotech companies. I became very interested in start-ups, followed some courses, and began doing some start-up coaching and advisory work. Through networking, I met Michael Altdorfer, CEO of the Swiss Biotech Association. The association is all about networking, sharing knowledge, and helping biotech companies be stronger together. I really liked the spirit of the association, so I was super happy when they asked me to join their board last year. Both small companies and big companies are members, and it has a very nice culture of connection and community. As an example of some of the work the association does, in January there was a startup CEO day, which enabled CEOs to get together and share notes. How did you get funding? How do you manage recruitment? Which CRO did you work with? What kind of labs do you have? These types of exchanges are incredibly valuable.

Why is Switzerland's biopharma climate so favorable?

Switzerland has a very long biopharma tradition and many cantons in the country have some kind of Bio Innovation or Start-Up park. Due to the favourable legal and economic framework (including tax conditions), it is well suited to biotechs. It's also very easy and quick to launch a new start up. Moreover, there are good incubators and networks – and, of course, the Swiss Biotech Association aims to help too!

For talent in the biopharma industry, there are many different opportunities in Switzerland because there are so many companies here. There is a lot of cross-fertilization as talent moves between different companies too.

What is your advice for others in industry who want to move into leadership?
It's very difficult to plan a career. There are many things that you can do to influence things, but there are also special moments that are not entirely in your hands, where you depend on the trust of others and your network.

I have three daughters – two of them are already in university – and I always tell them to find something you do with passion and something that is difficult; show the world that you challenge yourself, and that you can do difficult things. People who challenge themselves over and over again will be able to make their dreams come true.

And do you have additional career aspirations or even dreams?
In recent years, I've been involved in Biotech board work. I enjoy what I do, and I especially like the combination of working with the association and with industry.

I'm also interested in what comes next for biosimilars. We have found a way to bring microbial and mammalian products to larger patient populations – perhaps next there will be more complex biosimilars, such as ADCs. And then further in the future, what about cell therapies?

You've worked for start ups and big pharma – what are the main differences?
I started out in big pharma, which gave me a lot of opportunities. In big pharma, it's quite typical for people to move to different departments and there will usually be specialists in each area. The smaller the company becomes, the more you need to know about other aspects. A start up may only have five people, so you need to be able to cover a lot of ground – which can be really interesting. And because there are so many biopharma companies in Switzerland there is a lot of opportunity to move around. Companies will come and go, and other companies will spin off into new companies. Even if you just want to stay with one company, you'll still find that things change so you'll have a dynamic environment.

What is the most important lesson you've learned over your career?
I think it's important to be close to your purpose and what you believe in – and also to realize that you are not just somebody who's working; you're also a private person with a family and friends. Being in biopharma allows you to bring that all nicely together. When you have a career where you can connect your purpose and be yourself, you will be able to give so much more.

When you were younger, did you ever imagine working in the (bio)pharma industry?
The motivation to make the world a better place certainly always resonated strongly with me; I studied environment and environmental sciences as a master and PhD, specializing in the life sciences industry for the latter. When I got started in biopharma, it was for an environmental role, but then I moved to health, safety, and environment (HSE), before taking up roles in quality, manufacturing, and the supply chain. By now, I know a lot about different functions in the industry!

I may not have predicted it – but it's great to work in an industry that makes medicines. My mother died young from multiple sclerosis, and, for a time, I worked for Biogen, which is very focused on MS. Now, I'm at Sandoz, we bring a more affordable biosimilar version of an originator MS therapy to the market… Sometimes things in your life come together in an unexpected way.

Link
Online Article

No Better Time to Be Humble

When we interviewed Bruno Sepodes, he was the Professor of Pharmacology and Pharmacotherapy, University of Lisbon, Portugal, and Chair of the EMA Committee of Orphan Medicinal Products (COMP). In 2024, he became the new Chair of the EMA's Committee for Human Medicinal Products. This interview explores his focus on rare diseases, shared lessons from his work with the EMA, and his interest in regulatory science.

How did it all begin?

As a child, you don't really know what you want to do for a career, but you know what you like at school; for me, it was biology and everything related to the human body. I studied pharmacy at university and I became really interested in chemistry and medicine. After moving into toxicology, it wasn't a big leap to enter the regulatory world – after all, knowledge in toxicology and pharmacology is very important from the non-clinical side when it comes to understanding new drugs. I became an assessor for the Portuguese medicine regulator INFARMED and then the magic happened: I ended up connecting myself with EMA. I feel very privileged to not only be doing something that I love, but to be doing it within an international arena.

How did you become active in "regulatory science?"

My interests have naturally shifted from the research setting to regulatory science as I've spent more time in regulatory roles. I am interested in understanding how we better develop and organize

"I fell in love with the orphan regulation because of the way it was creating incentives for development – and so genuinely changing public health and patient lives."

egulatory tools for understanding new drugs. I am now quite proactive in this area and I have students pursuing masters and PhDs in regulatory sciences, which I believe is incredibly useful for society because it helps bring better and safer medicines to patients.

What led to your focus on rare diseases?
I became a member of COMP in 2008. From a scientific perspective, the world of rare diseases is very much unknown, with so many questions and so few answers. The quest for knowledge is very rewarding, but the big problem is funding; we need to be able to develop proper products. I fell in love with the orphan regulation because of the way it was creating incentives for development – and so genuinely changing public health and patient lives. Between 2008 and 2012, when I became Chair of COMP, my passion really blossomed. We are seeing products based on science that are coming through the doors of the agency for the first time – they are completely innovative and have the potential to create wonderful drugs that represent the first of a new class. We see them first and give manufacturers the incentives to carry on, which is exciting!

What lessons have you learned working for EMA?
The EMA is a unique place and so eye opening in many aspects! Collaborating with EMA gives you a completely different view to working within a national setting. When you are in an academic setting, you are very focused on the lab. Even when you connect to the clinical side and get involved with translational work, the truth is that patients, patient organizations and even the industry are very far-away players. Moving to the regulatory arena – especially the EMA (the brain of the regulatory world in Europe) – allowed me to really understand patients as stakeholders.

One of the wonderful things about the EMA is the diversity; not only are you able to meet people from all over Europe, but also from all over the world, such as regulators from the US, Canada, Australia, Taiwan, and elsewhere. The diversity has an incredibly positive impact in terms of what you learn and bring home.

You recently received the EURORDIS Rare Disease Leadership Award. How did it feel?
It was very unexpected! I have never felt as if I am doing something that is outside of my job. When I was elected Chair of the COMP, I felt it was my role to not only conduct meetings but also to really get involved with the field of rare diseases and engage patients more in discussions. I can't believe that I been given this award (or that I have been asked to give this interview). I feel very humbled because I have learned so much through the informal interactions that I have had with patients, parents of patients and caregivers. They are unbelievable people – they are fighters and their strength is such an inspiration. I feel very privileged to work in this area and to receive this award – I will treasure it all my life! In today's world, I think it is becoming increasingly rare to receive any sort of recognition and it is very inspiring. My work is just a small part of the overall efforts that have gone into positively changing the landscape for orphan drugs.

Rare Disease Day took place at the end of February. What comes next?
We must not stop the fight! In Europe, regulation around orphan drugs started in 2000. Before then, we had very few treatments for rare diseases and companies were not incentivized to pursue this research. Eighteen years on, and we have made significant progress; there are life-saving drugs available and we have also changed attitudes around rare diseases. I am so happy with the outcomes and I think that all European citizens should feel proud of the accomplishments. We cannot lose this momentum! So many rare diseases still need to be addressed, and there are many other concerns too, such as access to treatment and medicine costs. We need more conversations and we need the participation of every stakeholder. We must not give up because I believe the best outcomes are yet to come.

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Online Article

Irish Horizons

Following a spell at Bristol Myers Squibb's New Jersey facility, General Manager for the Irish commercial affiliate, Karen Costello, returned to her native country of Ireland with a goal of getting medicines to Irish patients faster.

How have previous geographic roles helped you prepare for your new role in Ireland?
I'm approaching my 24th anniversary with Bristol Myers Squibb (BMS). The majority of my career has been based in the UK, but I've also worked in various global markets and roles.

I was thrilled when I had the opportunity to work in the US. The scale of operations in the US, coupled with the diverse healthcare system, presented a unique dynamic. The pace of innovation in the US, both in technology and data availability, was remarkable. The emphasis on faster patient access and partnerships with renowned institutions was also incredible. This broader perspective will be invaluable as I now take on a role in Ireland.

As General Manager, I'm focused on developing a robust strategy, leveraging the skilled and passionate team here, and ensuring Ireland continues to grow as a critical hub for BMS. My US experience has underscored the importance of developing achievable plans - whilst still aiming high - and fostering external collaboration to help enhance medicine access in Ireland. Key for me will be taking a people-first approach; ensuring the right skills are in the right positions and creating a supportive culture. Leading with a clear vision, focusing on projects that will have the greatest impact, and embracing opportunities to innovate and grow, are equally essential.

What will be your focus in Ireland?
My time in the US gave me a closer view of BMS' enterprise-wide operations, which I'm keen to integrate into our work in Ireland. Leveraging the connections and insights gained in New Jersey, I aim to unlock new opportunities while emphasizing the vital role our Irish team plays. Ultimately, my focus will remain consistent: helping to ensure Irish patients gain timely access to the medicines they need. This is a deeply personal mission for me and drives my commitment to excellence in our work.

How important is Ireland to BMS?
Ireland has a stellar reputation as a global biopharma hub, backed by world-class facilities, a robust regulatory environment, and strong industry-academia collaboration. These factors create a solid foundation for innovation. At BMS, our vision is to make medicines more accessible to patients worldwide, and Ireland plays a pivotal role in that mission.

Our operations in Ireland include a biologics facility in Cruiserath, Dublin and external manufacturing divisions in Blanchardstown, Dublin and Shannon, County Clare. These sites exemplify our commitment to innovation and patient care. Last year, we announced a $400 million investment in a sterile drug product facility at our Cruiserath campus, slated to launch in 2026. This will not only enhance our manufacturing capabilities, but will also create around 350 jobs, reinforcing Ireland's standing as a biopharma leader.

What do you enjoy doing in your spare time?
I'm very sociable and love spending time with family and friends. Since returning to Ireland, it's been wonderful to reconnect and be present with loved ones. I enjoy the theatre, exploring cuisines, and planning European trips. Italy is high on my list, but I'm also eager to explore more of Ireland. Rediscovering the evolving beauty of my home country feels like a meaningful way to connect with my roots and appreciate the country more. Dublin's vibrant restaurant scene is especially exciting. Staying active is also important, though I've yet to reestablish my gym routine after months of travel. I'm also considering taking up golf, which will hopefully provide a new outlet for relaxation.

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Be a Little Different

Luigi Naldini has always been fascinated by research. After initially focusing on signal transduction, he became intrigued by the gene therapy field – where the drive to try something new led to the development of lentiviral vectors for use in commercial gene therapy. Today, he is the Director, San Raffaele Telethon Institute for Gene Therapy in Milan, Italy.

How did it feel to receive the Lifetime Achievement Award at Phacilitate 2024?
It was very rewarding – as with any award! Gene therapy has been neglected for so long, but now there is appreciation from all over the scientific industry. Early on, there were very few of us working and believing in what could be done with gene therapy. Now, there is much better recognition. Although an award goes to a single person, that person doesn't deserve all the credit. This award really goes to a whole team of people who have been involved in different stages.

Have you always wanted to be a scientist?
I always loved science, but early on it was more about nature and wildlife. In high school, I became more familiar with the emerging concept of molecular biology. At that time, there was no real understanding of DNA and RNA, so it was like an entirely new world was opening up – I found that very attractive. I ended up going to medical school, which, at the time in Europe, was a common path if you were interested in a research career in the biomedical area. Although I am an MD, I rarely practice or conduct clinical work. I am more interested in basic science and translational research.

How did you get into gene therapy?
After my MD and PhD, I started work on signal transduction. Back then, we were uncovering the basics of growth factor receptor tyrosine kinase, but I wanted to take a new route. I came across a review about the emerging area of gene therapies by Richard Mulligan (Harvard). After the early hype of gene therapies and the lack of results, he explained that we needed to go back to the hard science.

I was attracted by this idea and I wanted to join the field. I went to the US and I applied to Richard Mulligan's lab, but I didn't get the role! Over the years, I became very close to him and he always said, "Too bad you couldn't come to my lab."

And I would reply, "I could have come to your lab, but my application was rejected!" Fortunately, I was also interviewed at the Salk Institute and ended up in the lab of Inder Verma.

Why focus on lentiviral vectors?
At the time, there was discussion around current vectors, such as the gamma retroviral vector, not being very efficient. On the floor above me was the lab of Didier Trono working on HIV. It was early days for HIV and there was a lot of work focused on understanding this deadly retrovirus, which was very efficient at infecting human cells. We thought, why not try creating a vector from HIV? I was interested in starting something from scratch in gene therapy rather than joining something that was already going on, so building a new vector was very appealing. Though we never dreamed it would become so useful!

I worked for two years on this project – and it was very difficult at the beginning, particularly as it was a new area for me. I spent at least a month in the library, browsing literature (which is amazing to think about today, given that you can do that in a matter of days using the internet!).

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Innovation Driver

"Contributing to health is just about the most noble thing you can do in a career path." Claus Zieler, Chief Commercial Officer at Astellas and a member of the board of EFPIA, discusses his career, the company's investment in Ireland, and his lobbying work with EFPIA to improve investment in Europe.

What made you choose a career in the pharma industry?
I'm a molecular biologist by training and I've always been interested in cutting-edge science. When I was studying at Princeton, I was part of the team that produced the first monoclonal antibody in the 1980s. I then went on to do an MBA. What do you do if you have a science degree and an MBA? You join the pharma industry, of course!

Jokes aside, the industry is fascinating to work in and it has an incredibly strong purpose. Contributing to health is just about the most noble thing you can do in a career path. Pharma is also interesting because it is a complex industry full of highly skilled professionals with very different backgrounds and perspectives. As a leader, I have to work with people from across the business, from sales people to pharmacovigilance experts. It is my job to develop a common vision and direction, and to link all the parts together so that the enterprise as a whole can develop innovations. It takes a lot of energy to deliver value in such a complex environment and it's something that I very much enjoy.

How did you join Astellas?

I joined Astellas in 2019. Before that, a lot of my career was spent in emerging markets. I spent 8 years in Latin America and 10 years in Asia. Astellas approached me to ask if I was interested in building a new commercial region for the company from scratch. I'd never done this before and it sounded fun – so I said yes!

I was employee number one in that region. Today, there is a team of 50 people or so in the Singapore office, and there is a strategy and clear direction. I really enjoyed the role but I've now moved on to a different part of the company.

What is it that you enjoy about working in emerging markets?
They are very volatile. When you wake up in the morning you don't know what hurdle you will face, but the people are very hungry in a knowledge sense. They want to progress, they want to learn, and they want to improve. The workforce in emerging markets is often extremely committed and energetic. The people are often younger and less experienced, but they really want to apply the things they learn. It was a lot of fun to work with people who were so driven.

How did you get involved with EFPIA?
Astellas has a seat on the board of EFPIA and I took that seat for the company, which has given me the opportunity to discuss issues with people in different EU member states, parliamentarians and members of the European Commission. I usually make a point to have a productive exchange of ideas on what it takes to bring new products to patients and to deliver value.

I truly believe that the pharma industry is one of the top innovation drivers in the world. If you look at the percentage of sales that the pharma industry spends on R&D, it is higher than the car or software industries. Pharma's contribution to innovation needs to be recognized and should be facilitated on the side of

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policy makers. It needs to be easier for pharma companies to bring innovations forward. There is no use developing something if it sits in the corner and no one has access to it.

In Europe, I believe we have significant access issues when it comes to getting innovations into the hands of doctors so that they can treat patients and improve lives. These issues will only be overcome with intense dialogue between policymakers and the industry. I would like to see incentives for drug development in Europe increased and the regulatory burden lowered.

Tell us about Astellas's latest investment in Ireland…
Astellas has had manufacturing plants in Ireland for around 30 years. We broke ground on a new aseptic facility in March 2024, which will represent an approximate investment of around 330 million euros. Operations are expected to commence in 2028.

Our experience in Ireland, especially with Tralee in Kerry County, is that the local authority has been very keen to understand what it takes to run a manufacturing plant and what our needs are. The relationship has grown over time and there's now a lot of trust between the local government and us as a company. We employ highly skilled people in the community, including people from the local university, and the career opportunities we provide are facilitated in a constructive way. The local government tells us what requirements we need to comply with and what they expect of us. It's been a positive experience.

The new facility is located at Kerry Technology Park and adjacent to a campus of Munster Technology University. It's just down the road from one of our existing manufacturing plants, which makes many things a lot easier because both plants are subject to the same local regulations in the area, such as requirements for sustainability. The new facility will have a significant focus on reducing energy use and achieving zero waste to landfill.

What areas of drug development excite you right now?
I truly think that Europe is one of the major innovation engines for humankind – there are a lot of exciting developments in drug development right now. One is gene therapy, which is allowing us to potentially cure diseases rather than treating the symptoms as we do today. When I was a student, we used to brainstorm the potential of replacing defective genes in the body – and now those medicines actually exist on the market!

I also see a lot of future in cell therapy. Some diseases are so multifactorial that we're not able to actually identify all the different genes that play a role, but we might be able to replace the cell that's defective. Think about type 1 diabetes where the cells in the islets of Langerhans are eliminated through an autoimmune reaction and can no longer produce insulin. Now imagine if we could insert those cells again and have them produce insulin – and the impact this would have on patients.

The same is true in the eye, which is an area that Astellas is working on. If you lose your photoreceptor cells, you essentially lose your eyesight in either one or both eyes. There is no treatment that will restore dead cells, but if you could replace those cells you could potentially restore eyesight again. For me, this has tremendous potential in helping patients to return to a more normal life.

But exciting drug development is not just about replacing genes or cells – we can also still use chemical approaches. Here, Astellas is working on targeted protein degradation. When you've identified a disease mechanism, you can sometimes see where you need to intervene to alter the course of the disease, or stop the disease reaction from continuing, such as by inhibiting a molecule binding to a receptor, hormone, or signal messenger. In some undruggable targets though we can't find a binding site. Targeted protein degradation though means you can degrade your target – essentially chopping it to pieces – which can also stop the disease mechanism from continuing.

I'm very proud to say that Astellas is at the forefront of this innovative area and it could potentially help patients in many different disease settings.

Preservation and Perseverance

Stella Vnook grew up in the former USSR, within the blast range of the Chernobyl nuclear disaster. She fled to the US, spending the remainder of her youth in New York. Through hard work and inspiration, Vnook was determined to become something more than a refugee. Now, she is the CEO of Likarda, and looking to make a mark on cell and gene therapies, biologics, and traditional modalities.

How did your journey to CEO begin?
I completed my pharmacy school residency/internship in oncology and quickly became aware of the lack of options when assigning patients brutal regimens. As a healthcare provider, whether you prescribe the medicine or the regimen, you're working with what you have. I chose to follow the path of drug discovery and commercialization by joining the pharmaceutical industry because I wanted to expand the portfolio of available drugs. It was a very long road to learning everything from drug development and healthcare education, to marketing, strategy, and managing markets, but every step got me closer to having enough ammunition and knowledge to be a good CEO.

What makes Likarda unique?
To optimize CAR-T, you can spend five to 10 years perfecting a specific construct to assure maximum efficacy or increasing the number of cells to reach the target tumor. Every time you change a construct, however, you have to go back to in vitro research, which costs a lot of time and money. Likarda's technology can safely encapsulate the cell therapy without the need to increase the number of cells. In fact, the technology can decrease the number of cells because we're delivering the treatment directly to the targeted organ. Furthermore, Likarda technology enables products to be shipped on dry ice rather than expensive cryopreservation and liquid nitrogen methods.

What do advanced therapy stakeholders need to do to maintain momentum in the field?
We have focussed so much on creating new treatments that sometimes we don't think enough about the role drug delivery plays in the evolution of healthcare. We can improve therapeutics and cell therapies so much more if we can figure out how to deliver the therapy directly to a tumor. We need to start thinking across the lines to improve delivery and outcomes for the patient.

We also need common goals and purposes towards improving patients' lives. Some products have marginal benefits for the patient, but the cost is driven up to suit the system, which is not in the best interest of health economics. I would rather see efforts focussed on untapped innovation and truly meeting unmet needs.

You grew up in Belarus. How did your early experiences contribute to your career?
In a communist country, your choices are dictated by the government and by policy. I think this made me very driven when I arrived in the US and it helped me to be structured. Following the

"I want to leave the same legacy my family has left for my children – it has to be inspiring and empowering, because when a person is powerless, their bar is low. I hope to live and lead with passion and purpose."

Chernobyl nuclear disaster, I activated my survival mode and found the resilience required. I understood the benefits of passion in doing what you love, as well as enabling people to do their best.
When I left Belarus, my mother and I were refugees. It was a traumatic experience and I haven't looked back. Until there's some type of political change, I can't imagine returning. Maybe decades will pass before people understand the freedom of following their dreams.

How might your mother feel about having such an impressive legacy and her daughter being the architect of that legacy?
My mother is my hero. She gave up everything – including family and friends – to take me across many countries, with no money, to reach the US. When Chernobyl occurred, people weren't allowed to know about or diagnose conditions that could transpire from the explosion. My health started to deteriorate and people said, quietly, that unless I received treatment, I probably wouldn't have a very long lifespan. My mother sold what she could so she could get us out. She worked many odd jobs through near starvation and poverty until she earned her dietary degree again here in the US. It was just the two of us, so now I feel pressure to not let people down – especially those who have given up so much.

Other members of my family were also unique. My grandfather, a World War II veteran, encouraged both my aunt and my mom to follow the educational path. He was a firm, direct person, but he was also a respected, inspiring, and compassionate individual. He was kind and philanthropic, so I saw from them that you can be firm in your beliefs as well as a strong and compassionate leader. No matter how hard things get, I always remember where I came from.

The journey must have taken you to many places. Do you have a favorite?
I have a deep love for Italy. When we left Belarus, we weren't allowed to take much. It was one suitcase per person and very little money. We went through Poland and Austria into Italy, where we ran out of money. I was barely a teenager, and my mom was very young. She was trying to be brave but I was very angry because I didn't understand why I had to leave everything and everybody. I hadn't eaten for a few days, but some local women came and gestured to us to come and eat. They gave us pasta and helped with a place to sleep. I'll never forget that meal and how they embraced us. It was so heartwarming coming from a harsh "survival mode" environment. That type of kindness to a stranger really shaped my life and my journey.

What do you hope to achieve in the future?
I want to ensure that the technology that Likarda has developed is in good hands with a logistics partner and a cryopreservation partner, and that a manufacturing partner will embed it into what they do. The goal is to take it to where it could be available to every therapeutic that could potentially benefit. I'm going to stay focussed on the goal and help the Likarda team achieve it.

Ultimately, at Likarda and beyond, I want to leave the same legacy my family has left for my children – it has to be inspiring and empowering, because when a person is powerless, their bar is low. I hope to live and lead with passion and purpose. I aim to demonstrate that as long as you believe in yourself, you can champion that change. If people are inspired, and they understand how to channel that passion into the purpose, we can enable new generations of leaders to continue to advance healthcare.

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Maintaining Momentum

Being part of the cell and gene therapy field is an incredible privilege, according to Frank Mathias, CEO, Oxford Biomedica.

What drew you to the pharma industry?
Like most children, I had many dreams, but I was influenced by my father, a pharmacist. I was fascinated by his ability to help sick people through the medicines he prepared. Watching him mix formulas in his pharmacy left a lasting impression on me.

Ultimately, I chose to study pharmacy. To be completely honest, the decision was partly practical – my university was just a few kilometers from home – but it's a choice I've never regretted. That early exposure to pharmacy shaped my path and sparked my passion for healthcare.

I've always been driven by the desire to do something meaningful for others. During the early 1980s, when I began studying in Paris, the field was witnessing groundbreaking advancements, such as the emergence of monoclonal antibodies and the rise of biopharma. I became fascinated by the biotech industry and how it was improving our understanding of diseases.

Pharmacy was, and still is, a beautiful profession. It allows you to directly help people who are very sick by providing the right therapies. But biotech, with its potential to not only improve lives but also cure diseases, captured my imagination. I'm deeply convinced of the value of this industry. It offers meaningful, rewarding work for highly qualified individuals, while driving life-changing innovations.

Are there any career moments that stand out as particularly memorable?
I've been fortunate to work with incredible leaders who shaped my understanding of leadership and business. I've experienced diverse cultures, and worked in companies of different sizes. One of the defining moments came early at the age of 32. I was entrusted with my first general management role at Servier, overseeing more than 250 people. At the time, I had no real idea what it meant to run a company – I was too young! But the trust placed in me, combined with the support I received, allowed me to grow into the role. The experience taught me the importance of leadership and the value of helping others to succeed.

Later, my time at Amgen in the US was equally formative. I gained valuable insights into leadership, storytelling, and the ability to inspire teams to tackle big challenges. Another pivotal experience was serving as Chairman of the German Biotech Association. Leading a group of general managers and CEOs from different companies required strong persuasion skills and collaborative leadership. These roles underscored for me that success ultimately comes down to people, passion, commitment, and willingness to work toward a common goal.

How did you come to join OXB?
I was approached by a headhunter. As I researched OXB, I became intrigued by its unique focus and expertise in cell and gene therapy.

At the time, I was with another CDMO called Renschler Biopharma, where things were going exceptionally well. I hadn't planned to leave. However, the opportunity at OXB was so compelling that I decided to make the leap. I'm grateful to my previous employer, who graciously allowed me to exit my contract early to pursue this new challenge.

What would you say are the greatest challenges of being a CEO?
There are several challenges, but for me, the most significant is creating an environment where employees can thrive. I believe a CEO's primary role is to build a culture in which people are inspired to perform at their best, grow professionally, and have fun while doing so. The key is to bring together teams that share a vision and mission, enabling them to collaborate effectively.

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