Neil F. McFarlane – President & CEO, Zevra Therapeutics

Our mission is about redefining what is possible for people living with rare diseases and bringing truly life-changing therapies to patients

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Neil F. McFarlane, President and CEO of Zevra Therapeutics, reflects on how his experiences in clinical practice, military service, and leadership roles across biotech and mid-sized pharma come together and led him to this moment. Under McFarlane, Zevra is driven by the urgency of patient needs and engaging with patient communities, while maintaining a sharp focus on sustainable growth. Neil goes on to share his perspective on scaling impact in ultra-rare conditions, navigating global expansion, and fostering a mission-driven culture as Zevra enters its next phase of growth.

Having had such a diverse career journey, how did your experiences ultimately lead you to Zevra Therapeutics?

My career journey has certainly not been linear, but there has always been an underlying common theme. From my early days in the clinic caring for patients, to my time in the military, and then across roles in biotech, that thread has always been about patient care.

When I look back over the past 30-plus years, across these different sectors and roles, what has kept me engaged is a focus on high-impact science and areas where innovation can truly change outcomes. That naturally led me to rare diseases.

When you joined as CEO, how did you assess the company's level of ambition and strategic direction at that point in its lifecycle?

I joined Zevra in October of 2023, and for me it was an opportunity to step back into a CEO role and help bring together an organization that had a lot of potential and needed focus to really unlock value. What attracted me was that Zevra had multiple elements in place including commercial opportunity, late-stage development assets, and a management team and board that were clearly aligned around driving meaningful impact for patients. That combination, along with the culture of the organization, made it feel like the right place at the right time.

At that point in its lifecycle, Zevra had many of the bones of what could become a very special organization. The company had brought together several assets and businesses at different stages of development. The opportunity was to pull those pieces together, focus on doing a few things really well, and drive real value for patients.

That became the strategy. We acquired companies and assets, aligned around the highest-value priorities, and focused execution where it mattered most. By doing that, we were able to start unlocking value.

Could you provide an overview of Zevra's current commercial portfolio and the therapies the company has on the market today?

Today, we have two commercial products focused on ultra-rare diseases. The first is MIPLYFFA, which was approved in 2024 by the US FDA for Niemann-Pick disease type C (NPC). This was our first major commercial milestone for our flagship product. We have also submitted our marketing authorization application in Europe, and it is now in the evaluation process as we look to expand global access to MIPLYFFA.

Our second US commercial product is OLPRUVA, which is indicated for urea cycle disorders, another rare and serious condition. We also have a third product, AZSTARYS, that we developed and subsequently licensed. For that asset, we receive royalties and milestone payments, but we are not actively commercializing it ourselves.

Beyond our commercial portfolio, we also have an active Phase III development program in the US for vascular Ehlers-Danlos syndrome (VEDS). The study is currently enrolling patients and is being conducted as a pivotal, event-driven trial under a Special Protocol Assessment (SPA) with the FDA.

What unmet needs do these products address, and what kind of impact are they having for patients within their respective disease communities?

Starting with MIPLYFFA, this therapy was approved in combination with miglustat for NPC. In the US, there are an estimated 900 patients from a prevalence standpoint, but only about 300-350 that have been formally diagnosed. It is a rare, progressive disorder that is ultimately fatal for many patients. At a biological level, the disease is caused by the buildup of cholesterol in the lysosome, which leads to both neurological and visceral symptoms.

One important context point is that in Europe, there are approximately 1,100 NPC patients and miglustat has been approved for more than a decade. As a result, there is greater diagnostic maturity in Europe compared to the US. That said, in our first year post-launch, we have reached approximately 40 percent of the US diagnosed patient population through prescription enrollment forms. For an ultra-rare disease, that level of uptake in year one is quite meaningful for patients and families and reflects a strong execution by Zevra.

Turning to OLPRUVA, this product is a nitrogen scavenger indicated for urea cycle disorders. These are conditions in which a defect in an enzyme or transporter leads to the accumulation of nitrogen in the body, resulting in significant central nervous system effects. OLPRUVA came to us through an acquisition, and we have now been commercializing it for several years, providing a treatment option for patients managing these disorders.

Finally, celiprolol, which we are developing for VEDS, addresses a rare connective tissue disorder characterized by fragility and stiffening of arteries and hollow organs. Because the tissues lack flexibility, patients are at risk for arterial ruptures, dissections, and potentially fatal bleeding events.

Celiprolol is an adrenergic receptor blocker that is already considered standard of care in Europe for this condition, even though it has never been formally registered for the indication. In the US, there are an estimated 7,500 patients based on prevalence, and there are currently no approved therapies available to them. We are actively enrolling patients in a pivotal US trial, with the goal of bringing a much-needed treatment option to this community.

How does Zevra’s portfolio align with its agile commercial approach to maximize patient impact?

When the company rebranded, there was a very intentional decision to focus squarely on becoming a rare disease company with a commitment to the patients we serve. But rare disease is not a catch-all term. For us, it means concentrating our efforts on roughly 40 centers of excellence across the US. These are the institutions that diagnose and help to manage rare disease patients, typically through highly multidisciplinary care teams, and they play a critical role in shortening the diagnostic odyssey that so many rare disease patients experience.

Rare diseases are often difficult to diagnose. Symptoms can overlap with other conditions or appear progressively over time, making it challenging for patients to receive a definitive diagnosis. Our goal has been to ask a very simple question: how can we best support these centers of excellence and provide a fully integrated platform that truly meets their needs?

From a commercial standpoint, that focus has allowed us to be very efficient. The infrastructure we have built supports both of our products, and there is a high degree of overlap among the stakeholders involved. Geneticists, neurologists, hematologists, gastroenterologists, pharmacists all tend to sit within the same hospital systems. That allows us to take a high-touch, high-support, non-traditional approach that is really required in rare disease, where patient populations are small and every case is complex.

We have been very deliberate about not expanding into larger or less focused rare disease markets. With a relatively small team of about half a dozen people each in sales, patient services and medical affairs, we are able to deliver what these centers and patients need.

Zevra is now embarking on a global journey, starting with a recent submission to the European Medicines Agency. What does this milestone represent for the company’s international strategy?

We are still early in the process. We have submitted our application to the EMA, and it has been validated so we are now progressing through the regulatory review. Over the past nine months, we have been doubling down on what our European go-to-market strategy should look like, and we are evaluating whether we build that capability ourselves or whether we partner.

Right now, everything is still under assessment. We are speaking with a range of potential partners, from distributors to larger multinational companies, and looking closely at what it would take to expand access to MIPLYFFA across Europe. The first thing we look for in potential partners is cultural fit, and that starts with the patient. Are they truly patient-centric and do they make

decisions with patients' best interests at the forefront? From there, we can focus on capabilities and execution. We need to be confident that whoever is involved can help us maximize the launch and get this therapy to patients as quickly and effectively as possible.

In the meantime, we already have an expanded access program in Europe, which is treating around 90 patients across a small number of markets. That experience has reinforced that the rare disease community is truly global. Clinicians, patients, families, and advocacy groups are in constant communication across borders and cultures.

From your perspective, what are the most important success factors for effectively reaching rare disease patients and creating meaningful impact in their communities?

Across rare diseases globally, there is one consistent theme for success, and that is a high-touch, highly personalized approach. That goes for both how patients are treated and how therapies are brought to market. Strong engagement with advocacy organizations is foundational, because they are the anchor for patients, families, physicians, and, ultimately, for industry to be successful as well.

There are many ways an organization can support advocacy, but what really matters in rare disease is bringing all of the pieces together. That means clinicians, regulators, patient advocates, and industry sitting at the same table and working through challenges together. In the US where we do not have a single-payer system but a multi-payer system, that collaboration becomes even more crucial. You need all of those stakeholders aligned to overcome access and reimbursement hurdles.

Another key element is data. Having strong, credible data makes a real difference. When you can clearly demonstrate impact both scientifically and from a health technology assessment perspective, it becomes much easier to engage with payers. In many cases, they recognize the value because of the benefit to patients, families, physicians, and society.

Can you highlight a few examples of how Zevra engages with patient organizations and other stakeholders to support rare disease communities?

We partner very closely with both international and US-based patient organizations. For NPC, that includes close collaboration with groups like the International Niemann Pick Disease

Alliance (INPDA) and the National Niemann-Pick disease Foundation (NNPDF) as well as other NPC-focused organizations.

We also work closely with the National Urea Cycle Disorders Foundation for the urea cycle disorder community. On the development side, particularly around VEDS, we partner with the VEDS Movement and the Marfan Foundation.

These partnerships are essential. Working together with patient organizations allows us to drive disease awareness, support access to treatment, and help patients and families stay engaged over the long term.

The US has a well-established rare disease development ecosystem which is in large part thanks to the Orphan Drug Act. How do you assess the current regulatory environment and which policies do you believe are most important to driving innovation in rare diseases?

From my experience, the US regulatory framework has many of the right tools in place to support rare disease development. For the products we have taken through approval, the agency has provided clear guidance and a workable path to get therapies to patients.

We have lived through the full process, including receiving a complete response letter and then working through that with additional data and an advisory committee to ultimately secure approval. One thing I would like to see is greater consistency in how the existing framework is applied. The policies are there, and when they are applied consistently, they work very well. Overall, our engagement with the FDA has been very positive. We have not experienced delays, and in some cases, timelines have moved faster than expected. The agency has been constructive and collaborative, which is especially important in rare disease.

From a policy standpoint, priority review vouchers (PRVs) are a critical part of the rare disease ecosystem. They create a meaningful, non-dilutive source of capital that allows companies to attract investment and continue advancing therapies that otherwise might not be financially viable. While the PRV program expired, it is currently under reauthorization review and all of us in the rare disease space are hoping for a positive outcome.

We have seen PRVs do exactly what they were intended to do by helping bring rare disease products to market faster. Having received and monetized one ourselves, that non-dilutive capital is now enabling us to accelerate our commercial efforts, advance our development programs, and

explore bringing additional assets into the company.

Looking ahead, what principles are you putting in place to guide Zevra's next phase of development?

Looking ahead, our focus is on continuing to execute within the lanes we have been very deliberate about defining. We are building Zevra as a commercial rare disease company, with a clear emphasis on addressing unmet needs.

Our mission is about redefining what is possible for people living with rare diseases and bringing truly life-changing therapies to patients. Everything we do is grounded in putting patients first.

From a leadership perspective, how would you describe the culture you are building at Zevra as evolves into a global organization?

A lot of what shapes the culture at Zevra comes back to the same underpinnings that have guided my own journey. Everything I have done has been driven by a desire to make an impact, not only on patients' lives, but also on the people who make that impact possible.

There is something very powerful about bringing people together around a mission that many might initially think cannot be achieved. When you have the right people who are aligned around the same purpose, rowing in the same direction, and willing to run through walls for patients, you can accomplish things that once seemed out of reach. Watching that happen, and seeing people grow along the way, is incredibly rewarding for me.

What we do in this industry is not easy. We face more failures than successes, and that is the reality of developing therapies for rare diseases. But when you invest in people who are deeply mission-driven and passionate about the work, and you give them the support to be their best, it is remarkable what we can achieve together.

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