Dan Leonard – President & CEO, National Pharmaceutical Council (NPC), USA

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Dan Leonard is president & CEO of the National Pharmaceutical Council, which sponsors and participates in research on the appropriate use of pharmaceuticals and the clinical and economic value of pharmaceutical innovation. Leonard outlines the activities of the Council – which celebrated its 65th anniversary in 2018 – his priorities for 2020, and addresses the changes needed to transform US healthcare into a value-based system that will improve patient outcomes in the long-term.

Dan, with the National Pharmaceutical Council (NPC) celebrating its 65th anniversary in 2018, what would you highlight as some of the council's main achievements?

I'm proud of what the NPC team has achieved. For more than 65 years, NPC has taken a research-based approach, using strong evidence and methods to address the challenges facing our U.S. healthcare system. We regularly collaborate with other organizations, academics and thought leaders to better understand and appreciate how our healthcare system operates as well as the unique role that biopharmaceuticals play in the United States.

During the last decade, our work has focused on several areas in which we've seen real progress. These include real-world evidence, in which we worked with other organizations to develop strong

methods to conduct and review it. We're now at a point where the U.S. Food and Drug Administration (FDA) is considering how to utilize RWE in decisions about treatments. More recently, we've been involved in having broader conversations about value assessments. NPC has been working to ensure that value assessment frameworks are effective tools for achieving better outcomes for patients, rather than well-intentioned but flawed tools that impede such progress. Following patient-centered guiding practices can help put assessment frameworks along a better path.

We're also proud of our efforts to spark a broader, national dialogue on healthcare spending issues. The focus is on digging deeper to understand what is driving health spending and finding solutions, without finger-pointing at one healthcare sector over another.

What is at the top of your agenda for 2020?

We have a lot of ongoing work. For example, in the area of healthcare spending, last year we created the "Going Below The Surface" (GBTS) Forum, which is a coalition of more than 20 stakeholders from across the healthcare sector. They have all come together to evaluate, through honest conversations, the underlying factors driving healthcare spending and how we can address those challenges. Through this venture, we have conducted regional town-hall meetings, speaking with local communities about the healthcare issues that are most important to them. We're also working with GBTS partners in hosting smaller, salon-style discussions and webinars and have launched projects examining how to address low-value care.

Separately, we're supporting the "Considering Health Spending" series of research articles and blog posts being published in *Health Affairs*, a leading healthcare policy journal. We'll be continuing all of these efforts throughout 2020 as a way to spark needed dialogue about how to spend our healthcare dollars more effectively. Research is also important. And having health-spending data that adjusts for increases in prevalence and inflation and accounts for patient outcomes will enable sound, disease-specific policy solutions that address health spending and improve outcomes for patients.

Our research also is examining a variety of creative financing mechanisms that will enable payers and manufacturers to work together to provide improved patient access to curative gene therapies and other treatments. Our current healthcare system isn't built for recognizing the full value of these innovations. Making sure that the value of these innovative treatments is considered properly is top of mind.

Separately, we also are seeing changes in how the FDA will be considering and using real-world data to generate evidence to support the approval of new indications of medicines. High quality, real-world data and evidence can provide meaningful information for patient care and improve the treatments brought to market.

Of course, with the ongoing global pandemic, we're also looking at some of these challenges through a different lens.

How do you assess the country's ability to advance much-needed reforms in these areas?

There is no question that we have a unique healthcare system in the United States. There are multiple aspects of our system that differentiate it from the European or government-run system. There are a lot of different levers that need to be pulled to effect change. Of course, you can follow the path of addressing issues with governmental solutions, but that's not always the preferred outcome. We need to work together with payers and industry while keeping patients at the center of the conversation.

We have seen examples of self-driven change when looking at value-based agreements, where the manufacturer takes on financial risk by proposing a performance guarantee to payers, i.e. payment varies based on whether their product meets certain clinical or utilization specifications in the payers' populations. This is a newer and unique solution, and a more efficient way of approaching cost-based issues than a top-down governmental solution. These agreements are especially beneficial with more expensive, curative types of therapies.

There are large gaps between the data, public discourse and government reactions. Looking at data on drug spending, in 2019 the growth was 2 percent, in 2018 it was 2.5 percent, in 2017 it was less than 1.5 percent. This makes 2020 the fourth year in a row where drug spending is below both healthcare and general inflation.

Also, this is significantly less and growing at a slower rate than what we spend in other health sectors, like hospital care and providers. Yet, when you listen to political arguments, some view skyrocketing drug prices as the sole reason for all of our healthcare cost concerns. There is a significant disconnect here.

Do you think the debate surrounding pricing will impact the ability or will of manufacturers to innovate in the future?

NPC's member companies are committed to innovation and the development of treatments that improve patients' lives. That commitment has been especially clear during these last few months, when we've seen biopharmaceutical companies, researchers and other healthcare professionals come together to explore the development of new therapies and redeployment of existing medicines in the fight against COVID-19.

Each year, there are 7,000 to 10,000 new drugs in development for a wide range of conditions; some of the proposed regulations on drug pricing would ultimately result in a significant number of new treatments not being developed. We must maintain an innovative system to continue delivering breakthrough treatments like CAR-T and other cell and gene therapies.

When we spoke with Janet Woodcock at the FDA, she suggested that the US healthcare system lags far behind Europe in terms of investment in RWE. What are you and your members' views on this?

Certainly, the FDA has made progress significant progress on RWE in the last few years. They are moving deliberately but cautiously, which is something we should expect from the agency, and considering all the aspects of RWE. Understanding how treatments work in the real world is an incredibly important component of the clinical practice environment. When done correctly, RWE can provide meaningful information. However, when done incorrectly, RWE can often lead to misleading interpretations. At NPC, we have a long body of work in this area. We have done research into developing evidence that is fit for purpose, organizing patient roundtables with the National Health Council, working with Tufts University to understand why payers are or are not using RWE, among other studies. Moreover, our efforts over a decade ago have led to the development of best practices and standards for the design, conduct, reporting, and evaluation of RWE studies, which have been recognized by leading scientific and professional societies, health plans, and even the FDA.

NPC members and biopharmaceutical companies are heavily investing in RWE by building data assets and capabilities, hiring and training the data science workforce, and participating in collaborative partnerships with patient registries, health plans, and health systems. Most health plans and systems in the US use RWE in some way, but how and when it is used varies. For example, many health systems use RWE routinely to measure medication adherence. Fewer health

systems use it for more advanced uses such as for predictive analytics or as a centerpiece for value-based contracting. Over the past few years, clinical practice guideline groups and payers are beginning to use best available evidence to inform clinical practice guidelines and coverage decisions, however the use application of RWE for these decisions is more anecdotal rather than consistent. Within the public sector, we are encouraged to see the consideration of RWE and recommend that the FDA prioritize guidance for use of RWE to measure product effectiveness not just safety. Beyond the FDA, the use of RWE should expand to guide other federal policies, healthcare programs such as Medicare and Medicaid and federal agencies. Without more consistent application of high-quality evidence by every sector of the US healthcare system, further investments in the RWE infrastructure and workforce may be stymied rather than stimulated.

In January, you wrote a "wish list for the healthcare sector in 2020": to make healthcare more efficient, reduce wasteful spending, ensure greater value for healthcare dollars, and break down barriers that block patient access to medicines. What do you hope for the remainder of 2020?

Of course, the wish list was drafted before COVID-19 reached our shores, but even before the pandemic we have seen significant progress in many national conversations on these topics.

There is a lot of momentum on identifying and minimizing low-value care, which are treatments or services that don't provide value to patients and could even harm them. With our *Going Below The Surface* partners, we are working on a roadmap to help healthcare decision-makers develop programs to reduce low-value care within their organizations.

The wish list mentions value-based agreements, which are gaining traction. I hope we will see some regulatory reforms or changes in this area. In our research, we have seen a lot of regulatory hurdles and barriers preventing these agreements from becoming more widespread in the country, and we hope that reforms will speed up the move toward more value-based agreements.

We also have been working with MIT and other organizations to examine innovative payment mechanisms and how we can improve our current system.

We have seen positive movements in the first quarter of 2020 and hope we'll be able to pick up where we left off later this year.

To conclude, how do you think your nation will reflect on its own public health during the COVID-19 pandemic?

First, we should salute all our healthcare heroes and our industry around the world for rallying to fight COVID-19. It is unbelievable work that we are seeing in real-time, with new therapies and vaccines being tested and delivered, and there is no doubt that our industry will help to pull us out of the global pandemic.

The crisis has also exposed further cracks in our healthcare system and challenges in delivering care to our most vulnerable populations. We're also seeing an increase in the use of telemedicine, which is likely to increase, and we've seen changes to health benefit designs as well. What we learn from the current crisis and how we apply those learnings remains to be seen, but I'm hopeful.

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