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Peter L Saltonstall, president and CEO of the US National Organization for Rare Disorders (NORD) shares the organization's key priorities, what makes NORD a unique patient advocacy group in the US, prevailing access and affordability issues for American rare disease patients, and how the country should continue to incentivize a positive R&D environment for orphan drugs and rare diseases.

Peter, you have been at the helm of the National Organization for Rare Disorders (NORD) for 12 years now. Could you share what makes NORD a unique patient advocacy organization?

As a patient advocacy organization in the US, we are unique in being the only major umbrella organization that has no industry presence on our board. We are governed, managed and owned by patient organizations. This means there is no industry involvement in our governance or our work around public policy; everything is completely patient-focused. Over the 37 years of our existence and progress, NORD has truly become a trusted voice of patients in the US We go to Capitol Hill and we are listened to because we are seen as the genuine voice of patients.

Of course, we also work closely with industry associations like PhRMA and BIO, as it is important for us to speak to the organizations and entities that represent the companies developing therapies for

rare diseases. We want to know their perspectives, issues and priorities, and we maintain very good relationships with both. We also have a Corporate Council comprised of 125 companies, with whom we meet monthly to stay current on industry topics. It is important to maintain good working relationships with industry and other stakeholders but we are seen very clearly as the essential voice of patients with rare diseases in the US.

In the current situation, what's at the top of the agenda for NORD?

Over NORD's history, we have had many successful initiatives and advocacy efforts we are proud of but currently, the COVID-19 situation has completely overtaken our operations. Through our Patient Assistance Network, we work with manufacturers and patients to provide assistance to patients so they can access medications they otherwise would not be able to afford. Since the pandemic started, we have been inundated with calls – our call volume is up over 200 percent! – and people are calling because they are worried about keeping their jobs and medical insurance, and being able to afford their medications.

Nevertheless, we are also trying to refocus on some of our normal priorities. We anticipate that we will need to return to tackling issues like drug pricing, criticisms of the Orphan Drug Act (ODA) and so on, and therefore we are starting to reshape our priorities so that we can be prepared to advocate in front of the appropriate audiences when the timing is right. Senators and Members are slowly returning to Congress and we have already had initial conversations with them, but the message for the moment is that the focus is still on COVID.

For NORD in general, over the past couple of years, we have really advocated strongly for the use of empirical data surrounding different topics, especially efficacy-related issues. For instance, if we are using a patient story to highlight a specific problem with the existing system, we want to be able to back up that story with empirical data. Over the past three years, we have published reports in conjunction with IQVIA and their data, and these reports have been very effectively used in our conversations with Senators and Members. I have three people at NORD whose job is to meet with our elected officials and Congressional staff and advocate and educate on how the ODA works, what orphan drug spending looks like, and so on. We really believe that data is a powerful tool that allows us to tell the stories of patients with rare diseases better. It is about more than the emotional conversation; empirical data really helps change the conversation and makes you more relevant. This is why everything that we do nowadays is based around data. We recently released a COVID-19 report with data from our survey of over 700 patients to help guide our COVID-19 related

advocacy work. This data-driven approach is going to continue to guide our return to the normal advocacy work we do around orphan drugs.

In addition, this year we have started to look at more international linkages. We already have a great relationship with EURORDIS (Rare Diseases Europe), our counterpart in Europe, and we are reaching out to entities in South America and other regions to see how we can be more connected globally and join forces on common issues. We have already grown to the point where we do have a significant international reach. Every month, our website is accessed by 1.5 million people from 241 countries! We want to do more to partner with our counterparts in other countries.

From NORD's perspective, how should the US continue to incentivize a positive R&D environment for orphan diseases?

NORD worked on the ODA which first passed in 1983 – and we believe that it has worked successfully over the past nearly four decades. Before ODA, there were only 34 orphan drugs approved in the US and today, we have over 700. Today, we hear comments about the ODA needing to be modified or changed, but we do not necessarily agree on that. To our minds, the ODA has been effective in encouraging R&D in orphan diseases.

Of course, we want to ensure that the ODA is not abused in any way, but my real concern is that the people who often make these soundbites and narratives about the ODA being exploited do not really understand how the ODA works. We have to remember that even if a company seeks Orphan Drug Designation (ODD) for a common drug, they still have to go through the relevant regulatory processes and that the orphan exclusivity only protects the orphan disease indication, not all uses of the drug. There have been comments about the 200,000 figure – denoting the maximum number of patients that could exist in the US for the disease to be considered a 'rare disease' – but we do not see a need to change that figure right now either since it has worked well.

Looking at the empirical data, in 2019, only 9.2 percent of the overall US drug spend was on orphan drugs so we should not obsess on 'fixing' that 10 percent. We believe rare diseases R&D should be encouraged, we want to retain the incentives for doing so under ODA, and we believe that it is important that companies have the ability and incentives to develop therapies for these small patient populations. 90 percent of patients with rare diseases still do not have any approved therapies for their conditions, so the unmet medical needs are urgent and critical.

However, I want to be clear that we do watch very closely for potential abuses of the ODA. If there are abuses, we want to make sure they are properly addressed. For instance, recently we released a joint statement with EURORDIS (Rare Diseases Europe) that COVID-19 treatments should not be expedited through the use of orphan drug incentives (the ODA in the US and the Regulation on Orphan Medicinal Products in the EU) after a company in the US attempted to and was granted Orphan Drug Designation for its potential COVID-19 treatment. The company has since requested for the ODD to be rescinded. We believe this was an exception rather than the rule when it comes to appropriate use of the ODA in the US.

Access and affordability are complex issues in the US – and for rare diseases, the situation seems more challenging still. How have the Affordable Care Act (ACA) and the Medicaid and Medicare programs improved the situation?

Certainly, we are very concerned about access, and it is not about price alone, there are many other aspects complicating the situation. For instance, a few years ago, we did not require prior authorization from insurers for many rare diseases therapies. Today, of the nearly 50 therapies we support through our Patient Assistance Program, 100 percent of them require prior authorization. This brings insurance companies into the fray and it means they are influencing the decision of whether patients receive access to these therapies. We believe that once a therapy has been approved by the US FDA and entered into the formularies, patients should have access to it! We are concerned about these factors that are passing on additional costs to patients and/or limiting access in various ways.

I want to highlight that the general problem of drug prices is not just about rare diseases. As mentioned, orphan drugs are less than 10 percent of the overall drug spend in the US But this issue is affecting our patients. We believe drug pricing will continue to be one of the main driving issues in the US healthcare landscape and we will continue to pay a lot of attention to working on solutions.

With the ACA, the Supreme Court ruling making it an option to expand Medicaid eligibility has meant that access can really vary from state to state. While there are rules and regulations promulgated by the ACA applicable at the federal level, much of our healthcare system is still regulated by states, and we have 50 independent states all making their own decisions. Before COVID-19, we were working on over 200 bills in various State Legislatures to ensure that people can access quality healthcare coverage and timely, affordable access to medications. This is

something we are continuing to work on.

As you mentioned, 2020 is an election year. What are your hopes for the next US administration?

In my personal opinion, we currently have an administration that talks a lot about caring about patients but really has not put their money where their mouth is. Hopefully, the US will wake up and I am hoping for a positive change. We really have to become much more patient-focused and pay more attention to the burden of healthcare expenses on patients. The only way to address issues like drug prices and insurance is through a meaningful dialogue that brings all stakeholders within the ecosystem together. I do not think the current administration has any genuine interest in accomplishing this.

At NORD, we would like to see the ACA maintained and strengthened. Many of the measures and aspects we have worked so hard for over the years were included in the ACA, so we are closely watching the pending Supreme Court case. Instead of trying to end the ACA, I want so see the next Administration expand coverage and affordability components of the ACA so that patients can have better access to healthcare coverage, providers and medications they need to thrive.

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