Peter L. Saltonstall – President & CEO, National Organization for Rare Disorders (NORD), USA (October 2020)

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Ahead of the 2020 US National Organization for Rare Disorders (NORD)'s Rare Diseases and Orphan Products Breakthrough Summit, NORD president and CEO Peter L. Saltonstall outlines his hopes for the Summit, the impact of COVID-19 on the US rare disease community, and touches on the current reality and potential problems that could be solved by telehealth.

Peter, what are your expectations for this year's Rare Diseases and Orphan Products Breakthrough Summit?

At a time when it is vitally important for the rare disease community to come together, the National Organization for Rare Disorders (NORD®) has announced a program of broad-ranging topics with speakers of unparalleled expertise for the 2020 NORD Rare Diseases and Orphan Products Breakthrough Summit, taking place virtually October 8-9.

With rare disease patients and families deeply affected by COVID-19, important policy issues at a critical point and scientific innovation advancing more rapidly than at any time in history, we feel it is imperative for the community to come together virtually to share perspectives and address time-sensitive topics. At the NORD Summit, we will tackle the issues that matter most to the community at this unique moment in time and hear directly from leading experts on the front lines of public policy, medical research, patient advocacy and regulatory science. Because of the convenience of a virtual event, we have people registered to attend from around the world, which will add new perspectives to discussions of today’s most pressing issues. This is the first time in NORD’s history that we have presented the Summit as a virtual event, and I’m looking forward to the new experience, fitting as our theme for 2020 is “Entering a New Era.”

What has been the impact of COVID-19 on the Rare Disease community?

COVID-19 has affected people living with rare diseases in several ways, presenting new challenges and obstacles. NORD has found through two recent surveys and from conversations with the community that rare disease patients and their families have been impacted by cancelled medical appointments, diminished access to both personal protective equipment and medications, and financial instability, including job loss. In addition, the community has struggled with anxiety stemming from these issues as well as being at greater risk for the virus and the threat of potential drug shortages. Months of social distancing and lockdown measures have compounded the feelings of isolation ordinarily experienced by many in the rare disease community. On the research front, many clinical trials have been cancelled or postponed indefinitely, creating new, unexpected challenges in the progress towards new treatment options for the more than 7,000 rare diseases, over 90 percent of which are without an FDA-approved treatment.

To what extent can those affected by rare disease be part of future vaccination programs? What considerations would the community hold in terms of safety? Would they have the same considerations as other parts of the population?

We firmly believe that everyone should be part of future vaccination program whether rare or not. NORD also supports the FDA approval process and feels strongly that any approved therapy should meet current FDA safety and efficacy standards prior to wide-scale distribution.

What is the current status and particular relevance of telehealth in this context?

Prior to March 2020, few people had seen a health care provider outside of a medical facility. Typically, rare disease patients receive care in hospitals, health centres, and doctors’ offices. Due to the specialized nature of the treatment that many rare disease patients require, this often necessitates travelling significant distances, even across state lines, to obtain care. In the face of the COVID-19 pandemic, these patients are at higher risk. Fortunately, both federal and state governments temporarily but rapidly expanded the types of patients and providers eligible to utilize and be reimbursed for telehealth services. Though many challenges remain, the expansion of telehealth services has the potential to create long-lasting improvements for rare disease patients.

This change in telehealth has been well received by the rare disease community. For the last several months, NORD has been hosting virtual discussion groups in all 50 states, which have provided valuable feedback on the patient and provider experience with telehealth. In our recent survey of the rare disease community we found that out of over 800 respondents, more than 83 percent had been offered a telehealth visit during the COVID-19 pandemic by their health provider and of those who were offered a visit, almost 88 percent accepted. Out of all patients who reported having had a telehealth visit, 92 percent described it as a positive experience. This is encouraging news for a patient population that depends on being able to access specialized health care services in order to thrive.

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