



Alone we are rare. Together we are strong.®

June 20, 2024

Dear NORD Patient Organization Members,

I am reaching out because I have come to learn that some of NORD's member patient organizations and their Board of Directors may have recently received a letter from Senator Mike Braun, the lead sponsor of the Promising Pathway Act (PPA) and Promising Pathway Act 2.0 (PPA 2.0), which contains false and misleading allegations concerning NORD. I believe it important to clarify the facts and reassure you of NORD's unwavering commitment to accelerating the development of safe and effective therapies to treat all rare diseases, which is why NORD has voiced strong concerns about both PPA and PPA 2.0.

I am particularly alarmed by Senator Braun's mischaracterization of NORD's advocacy regarding PPA. NORD has been public with our concerns, which are substantive and long-standing. They have been shared directly with Senator Braun, and his staff multiple times. For example, NORD publicly released on June 26, 2020 a [statement of opposition](#) to PPA, and on November 2, 2023 submitted a [statement for the record](#) to the Senate Special Committee on Aging, for which Senator Braun is the Ranking Member. As detailed in this statement, NORD explained our multiple concerns about the consequences of PPA to the rare disease community.

NORD is not alone in our assessment and opposition; many other trusted patient organizations, other stakeholders, and the FDA have expressed concerns about the various versions of PPA that have been introduced since 2020. Without fundamental revisions, NORD will continue to urge Senators to oppose PPA 2.0 and instead support policies that would bring better benefit to all rare disease patients.

Additionally, I am aghast by Senator Braun's claims that NORD does not represent patient interests. Specific to PPA 2.0, NORD has hosted two webinars for our patient organization members to raise awareness, obtain community feedback, outline our concerns, and answer questions from our patient organization members. While Senator Braun's letter claims that 30 of our member organizations have expressed support for PPA 2.0, if this figure is accurate, it represents fewer than 10% of NORD's patient organization members. This is not a comprehensive representation of our membership or the greater rare disease community.

All patient advocacy organizations, like NORD, have a constitutional right to express support or opposition to pending legislation and be free from attempts by Members of Congress to intimidate any patient organization on the basis of those positions.

NORD outright rejects Senator Braun's unsubstantiated allegations regarding our compliance with federal tax laws. Annual financial audits continue to confirm our full compliance with all federal and state reporting and tax laws. These unfounded accusations could intimidate other patient

advocacy organizations that oppose this bill and create a chilling effect on the voices of patient advocacy groups nationwide.

In light of Senator Braun's misleading comments about NORD's financial practices, I want to assure you that NORD operates with the highest level of integrity. Over the last five years alone, 93% of every dollar NORD receives goes directly to patient programs and services, which has included more than \$175 million in direct patient assistance support. Furthermore, our revenue structure, which relies primarily on external contributions rather than patient organization member dues, is intentionally designed to keep patient organization member dues nominal. This approach ensures that as many rare disease patient organizations as possible can be part of NORD and benefit from our over forty years of advocacy, services, and expertise.

NORD stands firm in our commitment to advocating for the rare disease community with integrity and transparency. Since our inception, NORD's Board of Directors has not included any representatives from drug manufacturers, ensuring that our policy positions are genuinely representative of patient voices and their needs.

I pledge that NORD will continue our collaboration with any individual or organization in pursuit of effective public policy that will increase patient access to safe and effective rare disease treatment options. NORD staff are always available to discuss PPA or any other issue of importance to our community. A copy of this letter will be sent to Senator Braun's office.

Thank you for your trust and support.

Sincerely,

A handwritten signature in cursive script that reads "Pamela Gavin".

Pamela Gavin, CEO

CC:

Heidi Ross, Vice President, Policy and Regulatory Affairs

Patrick Collins, Vice President, Corporate and Community Relations