

Rare Disease Company Coalition

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Media Contact: Courtney Corrado, courtney.corrado@curastrategies.com

Rare Disease Company Coalition Urges Further Action to Protect Rare Disease Patients and Innovation from Pharmaceutical Tariffs

RDCC appreciates the administration's leadership to protect and advance innovation for the over 95% of rare diseases with no FDA approved treatment. In particular, we are grateful for the administration's inclusion of an exemption from Section 232 pharmaceutical tariffs for orphan products from jurisdictions that have a current or forthcoming trade and security framework agreement.

However, as currently enacted, this policy does not protect all 30 million Americans living with a rare disease. The Department of Commerce's recently-released Federal Register Notice for implementation of the Section 232 pharmaceutical tariffs requires a case-by-case assessment to determine which rare disease therapies meet the ambiguous definition of an "urgent U.S. health need" for products from jurisdictions without a trade agreement. The lack of a clear and categorical exemption for orphan drugs threatens the progress this administration and Congress have made to reinforce the United States as the world leader in rare disease innovation and jeopardizes patient access.

RDCC urges the administration to issue further clarification that orphan drugs for which all approved indications are orphan categorically meet an "urgent U.S. health need" and as such will receive a zero-percent tariff. Such clarification would reinforce the original intent of the administration demonstrated by the orphan exemption for products from jurisdictions with trade agreements.

Rare disease medicines serve extremely small patient populations, rely on highly specialized supply chains and may represent one of few, or the only, available treatment options for patients. These unique circumstances make predictability particularly important to companies developing these medicines, investors, and most importantly, the patients who rely on consistent access. Without an unequivocal exemption of orphan therapies where all approved indications are orphan, we are concerned that the process outlined in the FRN may lead to inconsistent application of the exemption, decreased investment in rare disease focused biotech companies, and reduced patient access.

The administration has demonstrated a strong commitment to addressing the unique needs of the rare disease community. We look forward to continuing our constructive dialogue to ensure implementation advances patient access while fostering innovation.