Rare Disease Company Coalition

May 5, 2025

Mr. Eric Longnecker
Deputy Assistant Secretary for Technology Security
Bureau of Industry and Security
Office of Strategic Industries and Economic Security
U.S. Department of Commerce
1401 Constitution Ave., NW
Washington, DC 20230
Submitted Electronically

BIS-2025-0022
XRIN 0694–XC120
Re: Response to Request for Public Comments on Section 232 National Security Investigation of Imports of Pharmaceuticals and Pharmaceutical Ingredients – Considerations for Rare Disease Products

Dear Mr. Longnecker:

On behalf of the Rare Disease Company Coalition (RDCC), a group of life sciences companies committed to developing therapies for individuals affected by rare diseases, we commend the Trump Administration for its commitment to maintaining the United States' leadership in biopharmaceutical innovation and for recognizing the urgent medical needs of Americans living with rare diseases. U.S.-based innovators are at the forefront of advancing therapies for rare diseases, with development programs predominantly structured to meet the rigorous regulatory standards of the Food and Drug Administration (FDA) and to prioritize access for American patients. Building on this legacy, we respectfully request that orphan-designated drugs and their components be excluded from any tariffs recommended as part of the ongoing investigation under Section 232 of the Trade Expansion Act of 1962.[1]

RDCC has an acute appreciation of the challenges of rare disease drug development, as well as the tremendous potential of scientific advances to transform the lives of people living with rare diseases. The companies represented in the Coalition have spent a collective 530 years dedicated to rare disease innovation. Our companies invest, on average, nearly 60 percent of annual expenditures on research & development (R&D) – over $17 billion annually – to ensure more rare disease patients have access to new and innovative treatments.[2] RDCC members have brought more than 50 rare disease

[1] *See* 90 Fed. Reg. 15951(April 16, 2025).
[2] *See* RARE DISEASE COMPANY COALITION, 2024 OUTLOOK (May 2024), https://www.rarecoalition.com/2024/05/22/rdcc-presents-2024-outlook/.

treatments to market, and have over 200 programs in our development pipelines, many of which would be the first ever FDA-approved therapy for their respective diseases.[3]

Rare or "orphan" drugs address conditions that affect fewer than 200,000 Americans[4] and are often the only treatment option for individuals living with severe, progressive, and life-threatening illnesses. These therapies are the product of a highly specialized and innovative segment of the biopharmaceutical industry. The broader policy framework and deliberate incentives carefully created by the Orphan Drug Act (ODA) and subsequent legislative and administrative efforts indicate a recognition of the need to protect these treatments from trade-related disruptions. Congress has historically recognized the importance of protecting these longstanding commitments to rare disease patients when considering new trade measures. Consistent with this intent, it is important to ensure that any new trade measures, including tariffs, are carefully evaluated to avoid unintended impacts on the vulnerable populations these policies aim to protect.

While RDCC members support efforts to bolster domestic manufacturing and enhance supply chain resilience, we urge the Department to exclude orphan drugs and their critical components – including finished drug products, critical inputs such as active pharmaceutical ingredients (APIs), key starting materials, research materials, and derivative products of those items – from new tariff actions. Working in coordination with the U.S. Customs and Border Protection and the FDA, we respectfully request that the Department establish a specific Harmonized Tariff Schedule of the United States classification for orphan designated drugs to ensure clarity and consistency in the application of the exclusion.

Orphan drug imports pose no national security risk due to limited volume and dependable supply chains.

RDCC recognizes and appreciates the importance of the Department of Commerce's responsibility to assess national security risks under a Section 232 investigation, including evaluating defense needs and the domestic industry's capacity to meet them. In this context, RDCC respectfully highlights that orphan drug imports support the needs of people with rare diseases without posing any risk to national security and are not directly tied to national defense requirements. Orphan drugs represent a fraction of global pharmaceutical trade, typically serving fewer than 1,000 patients per product annually in the U.S. and are sourced primarily through trusted trade partners such as the European Union, Japan, Canada, and Mexico. Due to their low volume, highly specialized use, and minimal risk of stockpiling or misuse, these imports do not present supply chain vulnerabilities that threaten military or economic security. Further, the current orphan

[3] *Id.*
[4] *See* 21 U.S.C.S. § 360bb(a)(2) (LexisNexis 2025).

drug supply chain is effectively meeting U.S. demand and is essential for maintaining access to life-saving treatments.

Reshoring orphan drugs is not commercially viable because of their low volume.

Efforts to rapidly reshore the entire supply chain for orphan drugs, including those in development and those already approved, would be economically prohibitive. Orphan drug manufacturers lack economies of scale and often depend on highly specialized overseas partners for producing and packaging the final dosage form. Reproducing this complex infrastructure domestically would require massive capital investment and duplicative regulatory approvals, diverting resources from ongoing research and clinical development. Technology transfers of biologicals would be particularly challenging. These structural barriers challenge the feasibility of reshoring orphan drug manufacturing at scale.

We respectfully urge the Department to protect access to life-saving rare disease therapies by exempting orphan designated drugs and their components from any section 232-related trade actions. We welcome the opportunity to provide additional information or discuss this matter further.

Sincerely,

Stacey Frisk

Executive Director

Rare Disease Company Coalition