RECORDATI RARE DISEASES
Focused on the Few

May 2, 2025

Hon. Howard Lutnick
Secretary of Commerce
U.S. Department of Commerce
1401 Constitution Avenue NW
Washington, DC 20230

Re: Docket No. 250414-0065: Comments of Recordati Rare Diseases Inc. on the Section 232 National Security Investigation of Imports of Pharmaceuticals and Pharmaceutical Ingredients. XRIN 0694-XC120

Dear Mr. Lutnick:

Recordati Rare Diseases Inc. (Recordati) welcomes the opportunity to provide comments on the Department of Commerce's investigation into the national security impact of imports of pharmaceuticals and pharmaceutical ingredients. Recordati is a global company focused on providing innovative and urgently needed therapies to patients suffering from very rare conditions. In the United States, we make therapies available and support patients with rare diseases and genetic disorders in the areas of endocrinology, oncology, hematology and metabolic. For the reasons outlined below, Recordati urges the Department to avoid the imposition of tariffs, quotas, or other import restrictions on rare disease treatments and orphan drugs. Such drugs, which are imported to serve small patient populations that often have no other alternative, are not being "imported into the United States in such quantities or under such circumstances as to threaten or impair the national security."[1] Accordingly, Recordati urges the Department to exclude rare disease drugs from any tariffs or other import restrictions that it might recommend as part of this proceeding.

Background

Recordati is "Focused on the Few." Our company's mission is to reduce the impact of extremely rare and devastating diseases by providing urgently needed therapies. We work side-by-side with rare disease communities to increase awareness, improve diagnosis, and expand availability of treatments for people with rare diseases. We are committed to U.S. patients and the U.S. market, and we have steadily expanded our presence and activity in the United States.

[1] 19 U.S.C. § 1862(b)(3)(A).

Our U.S. corporate headquarters are located in Bridgewater, NJ, with global headquarter offices located in Milan, Italy. We employ more than 260 people in the United States to conduct research, serve our patients, and engage with healthcare providers. Our U.S. entity has an annual operating budget of over $500 million. Across the United States we do business with 150 partners and vendors. We serve patients in all 50 states.

Since 2013, we have been commercializing products and have grown our footprint in the United States. Recordati has increased employment from 20 to 260 people over the past 12 years and invested significant resources in growing our team as well as building out our U.S. infrastructure. Our employees are engaged in innovation, healthcare provider education and patient outreach. In addition, we have invested in partnerships worth well over $2 billion with the goal of providing and approving orphan therapies to benefit patients in the United States. Recordati has also invested money and resources in partnering with U.S. patient advocacy groups, research institutions, U.S. based specialty distributors and pharmacies, and contract manufacturing partners to serve adults and children living with very rare diseases.

Imported Rare Disease Medicines Do Not Threaten or Impair National Security

The purpose of a Section 232 investigation is to determine the effects of imports on national security, and specifically to determine whether the goods in question are being imported in ways that "threaten to impair the national security."[2] A key factor that the Department must consider in this analysis is U.S. national defense requirements and whether the domestic industry can meet those needs. Drugs for rare diseases, while very important for the patients we serve, *do not impact defense preparedness or national security.*

In past Section 232 actions, the Department has focused on industries for which domestic manufacturing capacity is arguably important for national security and defense readiness. The law has been used to impose tariffs on steel and aluminum imports and auto imports. In imposing those imports, the President considered that declining domestic production in those industries jeopardizes the "domestic industrial base and national security."[3] While some part of the pharmaceutical supply chain might merit similar consideration, drugs for rare diseases do not. Imports of rare disease medicines, which do not have domestic competitors, do not (and cannot) jeopardize the domestic industrial base or national security.

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[2] 19 U.S.C. § 1862(b)(3)(A).

[3] Adjusting Imports of Automobiles and Automobile Parts into the United States, Presidential Proclamation 10908 (March 26, 2025), 90 Fed. Reg. 14705 (April 3, 2025)

The markets for rare disease drugs are unique. A rare disease is defined as a condition that affects fewer than 200,000 people in the United States.[4] In many cases, patient populations are much smaller than the 200,000 statutory threshold. The Food Drug and Cosmetic Act also defines a rare disease or condition as any disease or condition "for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug."[5] Thus, by their very definition, imports of rare disease drugs are not jeopardizing domestic capacity and would not be replaced by domestic production if imports were "adjusted" via tariffs. Instead, such import adjustments would almost certainly mean drugs would be unavailable to patients or would become significantly more expensive.

The patients we serve exclusively have rare or ultra-rare diseases. In all cases, Recordati's medicines serve populations that are significantly smaller than the threshold for qualification as an orphan drug. Indeed, in some cases, the populations served by our drugs are as small as a few hundred people in the United States. By way of example, a few of our products represent the only approved medication available in the United States to treat the diseases for which they are approved. In many other cases, our products represent critical options to reduce disease burden and/or extend survival for patients. Tariffs or other import restrictions would jeopardize our ability to supply our patients, and for most rare disease treatments, no other alternative exists. In addition, the small population of customers in the United States for any given drug makes the likelihood of innovation, alternative treatments, or a newly emerging domestically produced alternative extremely unlikely. Without imports, U.S. rare disease patients would be left with no treatments.

By their very nature, rare disease drugs are often manufactured in a single global location to supply the global population of patients. Access to global markets, including the U.S. market, helps ensure that rare disease drug makers, like Recordati, are able to profitably produce these important medicines. Access to the U.S. market is essential for innovation and investment in rare disease treatments as well. Recordati is actively investing in opportunities to bring additional products to U.S. patients. We are funding clinical development projects in the United States to secure U.S. approval and are also working via partnerships with third parties to invest and bring their products to the U.S. market. Tariff barriers to rare disease drugs would undermine current patient access and would make investments in bringing new drugs to the U.S. market unsustainable.

[4] Rare Diseases Act of 2002 Pub. Law 107-280.
[5] Section 526(a)(2)(B).

Where it can, Recordati also produces in the United States. Its U.S. production of Enjaymo® and Neoprofen services not only American patients but patients in Canada, Europe, South America and Japan.

Tariffs on Rare Disease Drugs Will Not Increase U.S. Investment of Manufacturing

Currently, the vast majority of the drugs we sell are manufactured outside the United States. The reasons for foreign manufacturing vary across our products. In some cases, the drug was originally developed and marketed in the market where it is manufactured. In other cases, the manufacturing location was driven by other supply chain considerations, or home country or affiliation of the original innovator. Moving production to the United States in response to tariffs is simply not a viable near-term strategy for Recordati -- and likely is not a viable strategy for any producer of rare disease medicines. The result for these companies would be to raise prices and, in some instances, withdraw the rare disease drug from the U.S. market altogether.

The process of moving pharmaceutical production is significantly more complicated than it is for other industries. The complexity of manufacturing and the unique regulatory environment for pharmaceuticals means such a move would be very time consuming and prohibitively expensive. For any pharmaceutical product, moving manufacturing to the United States would likely cost tens of millions of dollars, and would almost certainly take several years before a facility was producing for commercial sale. In addition, there will be an increase in the amount of government resources that will be needed to inspect, approve, and audit manufacturing sites.

Recordati and other rare disease drug companies face even greater obstacles than those faced by pharmaceutical companies making large lot production of medicines. In most cases, we cannot justify the expense of transferring manufacturing to the United States given the small size of the populations we serve and the revenue of the individual product. Even in cases where transferring production might be viable, U.S. contract manufacturers are already at capacity, and are capacity limited in comparison to providers outside the United States. Thus, in many cases it simply is not feasible to move manufacturing to the U.S.

Tariffs on Rare Disease Drugs would be Dangerous and Harmful to U.S. patients.

While imports of rare disease drugs do not implicate U.S. national security, tariffs on such drugs would cause significant physical and financial harm to Recordati's U.S. customers. The number of people suffering from any individual disease may be very small, however, many people in the U.S. are impacted by uncommon disorders. There are over 10,000 rare diseases affecting more than 30 million Americans. Recordati exclusively serves this population of

Americans, who will face shortages of lifesaving drugs and/or significant price increases or withdrawals for those drugs if tariffs are imposed.

If tariffs are imposed across the board on pharmaceuticals, Recordati will have few options to mitigate the burden on our business. We estimate that tariffs could reduce our current profit by tens of millions of dollars or more, depending on the tariff rates and other details. This could represent a very significant part of our U.S. profit and could even reduce profit to below zero.

We would likely have no other option than to consider significant price increases in response to the tariffs. Changes to our pricing strategy will result in increased burden to U.S. payers (including the Federal Government) and patients. In addition, we would be forced to cut costs, likely by reducing our patient engagement efforts. Patient engagement is essential for rare disease diagnosis, drug development, and adoption. In addition, Recordati would likely be faced with cutting parts of its U.S. workforce and reducing its investment activity that brings new drugs to U.S. patients. The loss of investment potentially deprives U.S. patients of new life sustaining treatments. Well over 90% of rare diseases have no available therapy, and companies like Recordati invest in the development of new drugs for these diseases. Tariffs would undermine our ability to invest.

If cost cutting, price increases, and other strategies to mitigate the burden of tariffs are not feasible, Recordati will be faced with withdrawing from the U.S. market. Withdrawal from the market means our patients, who rely on our products for life saving and life enhancing treatment, will be left without their preferred treatment. And, in many cases, it means they will be left without *any* treatment.

Conclusion

We submit that imports of orphan products that serve patients with rare diseases do not implicate U.S. national security in any way. Thus, the Department has no reason to include such products in the scope of any import adjustment strategy recommended to the President.

We specifically request that any drug product, or API for a drug product that qualifies as a rare disease drug, should not be subject to tariffs, quotas, or any other measure to limit imports.

Appendix 1
Recordati Rare Diseases Products

Drug name	Treatment for	Country of Origin (API)
Carbaglu®	Used in adults and children to treat genetic conditions resulting in high blood ammonia levels due to certain enzyme deficiencies	Italy
Chemet®	Used in children to treat lead poisoning	India
Cystadane®	Used is adults and children to treat a genetic disorder in which there is abnormal accumulation of amino acid homocysteine in the blood	Germany
Cystadrops®	Used in adults and children to treat corneal crystal deposits in the eye	Italy
Enjaymo®	Used in adults with Cold Agglutin Disease to treat the breakdown of red blood cells.	U.S.
Isturisa®	Used in adults with endogenous Cushing's Syndrome to treat elevated levels of cortisol in the blood when surgery has failed or is not an option	Slovenia
Neoprofen®	Used in premature infants who are no more than 32 weeks gestational to close a significant patent ductus arteriosus	Canada
Panhematin® (hemin for injection)	Used in adult women to treat acute intermittent porphyria	Finland or U.S.
Signifor®	Used to treat adults with Cushing's Disease for whom surgery has failed or is not an option	Ireland
Signifor LAR®	Used to treat adults with acromegaly for whom surgery has failed or is not an option	Switzerland
Sylvant®	Used in adults for the treatment of idiopathic multicentric Castleman's Disease who are HIV and HHV-8 negative	Ireland