

Most-Favored Nation Policies Stifle Rare Disease Innovation and Inhibit Patient Access

The Rare Disease Landscape

A staggering **95 percent** of rare diseases lack an FDA-approved treatment – and rare disease companies face unique risks in the race to discover, develop, and deliver treatments to patients. On average, rare disease drug development takes **15 years** and costs **hundreds of millions or billions of dollars** per program. However, the cost of inaction is far greater. Without treatments, rare diseases cost patients, caregivers, and the health care system invaluable time and resources. Societal costs may reach up to **\$8.6 trillion** per year.



1 in 10 Americans live with a rare disease



10,000 known rare diseases. 95% have no approved treatment



70% of rare diseases begin in childhood.



30% of children with a rare disease don't see their 5th birthday

The Investment Ecosystem

With high research and development (R&D) costs stretching into the millions and billions, and a long, challenging journey from development to market, rare disease companies operate on a razor's edge, with funding runways often measured in days and months rather than years. Rare disease companies are heavily reliant on capital investments to launch and sustain development programs. This reliance makes these companies uniquely vulnerable to market shifts. Policy changes can increase the risks associated with rare disease R&D investments – and the consequences of these changes are clear.



The trading index for rare disease companies has declined by nearly 7% per year over the last 5 years, compared to a 1.3% decline for non-rare disease companies.



Rare disease companies have seen billions of dollars less in investment in the past decade stemming from decreases in venture capital, the IPO market, and partnership revenues.



Only 1/3 of commercial-stage rare disease companies were profitable in 2022, compared to more than half of comparable non-rare commercial stage companies.



More than 1/3 of biotechnology companies have less than 12 months of cash runway remaining, and the capital drought is acute for rare disease companies.

Policy uncertainty destabilizes the precarious investment landscape and threatens critical capital investments. These decisions ultimately make or break the ability for rare disease companies to deliver hope for over 30 million Americans living with a rare disease.

What is Most-Favored Nation Pricing?

Most-favored nation (MFN) pricing is a policy through which prescription drug prices in the United States are tied to the lowest prices paid for those drugs in select nations. MFN policies will not achieve the goal of reducing U.S. spending on prescription drugs and increasing affordability for Americans. Rather, in many countries, price setting and external reference pricing have delayed patient access to innovative treatments. One in four FDA-approved orphan drugs are not approved in Europe, the U.K., or Japan, and orphan drugs face greater hurdles than non-orphans in securing reimbursement and launch abroad. A U.S. MFN policy risks replicating those challenges, undermining access, eroding incentives, and discouraging future investments.

**PROTECT RARE DISEASE INNOVATION AND PATIENT ACCESS:
SUPPORT AN ORPHAN DRUG EXEMPTION FROM MFN POLICIES**

Impact of MFN on Rare Disease Innovation and Patient Access

Rare disease financing depends on **stable, predictable revenue expectations** because patient populations are small and development and manufacturing costs are largely fixed. Added **price volatility** and retroactive rebate **risk** can quickly make a viable program **financially infeasible**.

Out-licensing agreements play a critical role in allowing small and mid-size companies to commercialize. But these companies often have **no authority or visibility** into foreign list prices or reimbursement negotiations. MFN policies would **undermine** the utility of these agreements and **eliminate** a critical tool used to spur innovation and increase access.

Increased uncertainty and compressed expected returns **weaken** the assumptions needed to raise capital, secure partners, and sustain post-approval evidence generation. This impact is especially **devastating** for **small and mid-size** rare disease companies.

Programs with the **smallest populations** or the **highest development complexity** are most likely to be **paused, delayed, or abandoned**, shrinking pipelines over time. The net result is **fewer launches** and **fewer treatment options** for patients with limited or no alternatives, leaving more patients reliant on supportive care or off-label treatments.

Europe's leadership in biopharmaceutical innovation **eroded** following policy decisions to implement price controls and **weaken** incentive infrastructures. These policies have led to **longer delays** to patient access, fewer available therapies, reduced R&D investment, and loss of life sciences jobs.

87%

Of investors say federal price setting policies such as MFN would reduce U.S. biotech investment.

63%

Of RDCC members foresee a moderate to significant negative impact to patient access under MFN.

75%

Of RDCC members are reliant on out-licensing agreements for rare disease products.

66%

Of RDCC members have no control over ex-U.S. pricing under out-licensing agreements.



Looking Back

Exempting orphan products from MFN policies is consistent with longstanding U.S. policy to incentivize rare disease drug development. Since enactment of the Orphan Drug Act in 1983, the U.S. has recognized the need for a fit-for-purpose approach to rare disease innovation. More recently, the passage of the ORPHAN Cures Act via the Working Families Tax Cuts Act expanded the orphan exclusion under the Inflation Reduction Act. Since the passage of this critical bill, investors have committed over \$1 billion into rare disease programs. 73% of investors say that the ORPHAN Cures Act has had a positive impact on their interest in investing in rare disease.

Looking Forward

Policymakers should preserve the legislative intent of the ORPHAN Cures Act and build on its momentum by ensuring rare disease therapies are not subject to MFN policies. Without an appropriate exclusion, MFN risks undermining the gains achieved since enactment of the ORPHAN Cures Act and could delay or limit access to future rare disease treatments.



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