PERSONALIZED MEDICINE COALITION

April 3, 2025

The Honorable John Joyce
U.S. House of Representatives
2102 Rayburn House Office Building
Washington, DC 20515

The Honorable Donald Davis
U.S. House of Representatives
1123 Longworth House Office Building
Washington, DC 20515

Re: Support for H.R. 946, the *Optimizing Research Progress Hope and New (ORPHAN) Cures Act*

Dear Representative Joyce and Representative Davis:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 200 institutions from across the health care spectrum, thanks you for reintroducing H.R. 946, the *Optimizing Research Progress Hope and New (ORPHAN) Cures Act.* The *Inflation Reduction Act (IRA)* included policies intended to lower prescription drug costs for Medicare beneficiaries and reduce drug spending by the federal government. However, multiple studies demonstrate the potential for the *IRA* to result in fewer new therapies being developed to address current and future unmet needs. Due to smaller patient subpopulations, personalized medicines that address the root causes of disease can sometimes be expensive and risky to develop. Medicare's drug price negotiation program established by the *IRA* could have an outsized effect in discouraging the pharmaceutical industry from seeking expanded rare disease indications for orphan drugs. Bipartisan policy solutions like the *ORPHAN Cures Act* would forestall disruption to the innovation ecosystem that has allowed patients and providers to benefit from personalized medicine. PMC supports this bill and urges Congress to swiftly advance it during the 119th Congress.

Personalized medicine is an evolving field in which physicians use diagnostic tests and individual details about a person's health to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine is playing an important role in transforming care and patient outcomes for a range of serious and life-threatening diseases and conditions, helping to shift patient and provider experiences away from trial-and-error medicine and toward a more streamlined process for making clinical decisions.

Statement of Neutrality

PMC's members may present their own views on H.R. 946, the *Optimizing Research Progress Hope and New (ORPHAN) Cures Act*. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance

and are not intended to adversely impact the ability of individual PMC members, alone or in combination, to pursue separate positions with respect to the *ORPHAN Cures Act*.

Improving the Prospects for Orphan Drugs to Treat Rare Diseases under the *IRA*

Personalized medicines now account for more than a quarter of the new therapies approved since 2015. They have comprised more than a third of new drug approvals for six of the last seven years.[i] Recent approvals have brought a record number of new treatments for rare genetic diseases and new ways to address certain cancers and other diseases, including Alzheimer's disease. Multiple analyses, including those from the Congressional Budget Office (CBO), have called attention to the potential consequences of the Medicare drug price negotiation program established by the *IRA*, such as canceled research and development and disincentives to invest in small-molecule medicines and therapeutic areas that require incremental innovation.[ii, iii, iv, v]

Only about one quarter of all orphan drugs approved in the last two decades have a single indication.[vi] Researching additional orphan indications for existing rare disease treatments plays an important role in identifying new treatments for patients with rare diseases who do not have treatments available to them. Orphan drugs are excluded from the Medicare drug price negotiation under the *IRA* if they treat only one rare disease or condition. If an orphan product has designations for multiple diseases, even if these are also orphan designations, then it loses its exclusion from negotiation. Furthermore, as soon as a drug is designated for a second disease, even without any associated approvals, it becomes negotiation eligible — and the clock for when a product could be selected for negotiation starts at the date of the drug's first approval, even if the second designation or additional approval occurs many years later.

The *IRA*'s narrow exclusion for orphan drugs contradicts the goals of the *Orphan Drug Act* to foster the development of new treatments for rare diseases and could stifle post-approval research into additional orphan indications for rare diseases. Even when making investment decisions among multiple potential orphan indications, manufacturers may be incentivized to prioritize indications for rare diseases with larger patient populations over indications for very rare diseases. To preserve hope for the 95 percent of rare disease communities without disease-specific FDA approved treatments, PMC supports the *ORPHAN Cures Act*, which would ensure orphan drugs treating one or more rare diseases or conditions are excluded from Medicare drug price negotiation under the *IRA* and clarify that the countdown to price negotiation eligibility would begin only when an orphan drug loses its exclusion.

Conclusion

To sustain progress in the development of groundbreaking personalized medicine treatments for the benefit of patients and health systems, Congress must support policies that encourage the advancement of the field. PMC would be pleased to serve as a resource for you and your staff to ensure that the *ORPHAN Cures Act* is signed into law this year. If you have any questions about the content of this letter, you can contact me at cbens@personalizedmedicinecoalition.org or David Davenport, PMC's Director of Public and Science Policy, at ddavenport@personalizedmedicinecoalition.org.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

[i] Personalized Medicine Coalition. *Personalized Medicine at FDA: The Scope & Significance of Progress in 2023*. February 29, 2024. https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf (Accessed March 30, 2025).
[ii] Congressional Budget Office. *CBO's Simulation Model of New Drug Development: Working Paper 2021-09*. August 26, 2021. https://www.cbo.gov/publication/57010 (Accessed March 30,2025).
[iii] Vital Transformation. *Build Back Better Act: Total Market Impact of Price Controls in Medicare Parts D and B*. July 28, 2022. https://vitaltransformation.com/2022/07/build-back-better-act-total-market-impact-of-price-controls-in-medicare-parts-d-and-b/ (Accessed March 30, 2025).
[iv] Avalere. "Drug Pricing Bill Could Reduce Manufacturer Revenue by Over $450B." July 22, 2022. https://avalere.com/insights/drug-pricing-bill-could-reduce-manufacturer-revenue (Accessed March 30, 2025).
[v] O'Brien, John. "Branded Drug Report 2023: John O'Brien, NPC." *Chain Drug Review*. January 9, 2023. https://www.chaindrugreview.com/branded-drug-report-2023-john-obrien-npc/ (Accessed March 30, 2025).
[vi] Chambers, James D. "Follow-On Indications for Orphan Drugs Related to the Inflation Reduction Act." *JAMA Network Open*. August 15, 2023. https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2808362 (Accessed March 30, 2025).