We invite you to join a coalition to Protect Innovation in Rare Cancers (PIRC), whose mission is to REBALANCE THE IRA so that it ensures both affordability of today's treatments AND continuation of rare cancer research, development, and innovation.

Congress and CMS must curb unfettered drug price increases, reduce barriers to generic competition, and encourage manufacturers to offer discounts to all patients—***without*** **discouraging investment in desperately needed rare cancer research and development.**

Affordability of Today's Treatments. Having to forego medications due to cost is a choice no cancer patient should ever have to face. And yet we do. Every single day. The cancer community celebrates a long overdue win with Congress enacting:

a. An annual $2,000 out-of-pocket (OOP) cap
b. 'Smoothing'

Protecting Future Innovation. We fear CMS' IRA implementation decisions could change incentives for future research and development in rare cancers, and sacrifice rare canner innovation while attempting to keep treatment costs low and reduce 'monopolistic' behavior:

a. CMS' decision to lump together all of a manufacturer's drugs that share the same moiety/ingredient

b. CMS' introduction of new and complicated concepts of "Primary/Secondary Manufacturers"

c. CMS' limitations on both patients and patient advocacy organizations to have a meaningful opportunity to help shape the drug selection and price negotiation processes.

d. A rigid Medicare price 'ceiling' lacking flexibility to consider R&D risks for rare cancer treatments, or the possibility that timeline for recouping investment is different for small population treatments. The ***maximum is the maximum without exception*** and is not subject to any negotiation.

e. An orphan exemption that forces companies to consider withdrawing a designation, ceasing any research they anticipate doing later, or delaying research on a rarer cancer while focusing on a larger orphan use.

a. A generic/biosimilar exemption that fails to consider lack of generic manufacturer interest in small population treatments, and related steeper discounts for products on the market without such competition.

Impact on Rare Cancers.

1. Lack of generic interest in rare diseases will mean rare cancer treatments will face the crosshairs of IRA negotiations much sooner than intended.

2. Congress' orphan exemption threatens the current paradigm of rare cancer drug development, while CMS' reliance on a single designation to mitigate impact is not helpful for rare cancer treatments where possible indications are more difficult to bring together under one designation.

3. The decision to treat all drug and biologic applications as a single product for negotiation purposes combined with the narrow orphan drug exemption will curb interest in research toward adding new rare cancer indications to existing approved treatments.

4. New drugs will inevitably have higher launch prices across all payers, and will be reluctant to offer discounts to *an*y patients or payers that could eventually result in even steeper discounts to Medicare, especially in rare cancers, where an evolving treatment landscape can unpredictably shorten a treatment's lifecycle.

5. CMS' evolving policy on patient assistance programs affecting calculations of maximum price will likely impact continuation of non-Medicare patient assistance programs.

6. No meaningful opportunity to acknowledge/address lack of actual therapeutic alternatives for a rare cancer or other lower-population indication from among CMS' selected alternatives. The active ingredient decision also makes choosing alternative therapies untenable.

7. Congress' concept of 'value' as a negotiation factor ignores rare uses of treatments indicated for more common conditions. Even if uses in small population conditions represent the only treatment to address an unmet need, the 'value' will always reflect the most common uses and minimize the experience of rare patients.

8. The reimbursement landscape has long influenced where research dollars go. Oncology has seen this before when, prior to the Part D benefit, manufacturers poured significant investment into physician administered drugs, even as technological advances made patient-friendly oral/self-administered options feasible. IRA-related investment decisions are being made now, are likely far more negative, and will be felt for generations, including:

a. Investing in biologics rather than small molecule drugs, which could mean longer lead time in bringing the drug to market, a formulation less favorable for patients, or higher healthcare costs to administer the drug;

b. Investing in diseases that impact younger patients (under 65) to avoid Medicare's price negotiation.

c. Making decisions on which rare cancer indication to pursue based on population, and delaying or simply not pursuing new uses of existing cancer treatments that have historically improved survival and quality-of-life for rare cancer patients.

The Protect Innovations in Rare Cancer (PIRC) Coalition seeks to *rebalance the IRA* so it recognizes and addresses circumstances common to rare cancers.

Join, Support, and Participate in PIRC on behalf of your patients.

Let's raise our collective voices on behalf of *solutions developed together* … by and with all rare cancer stakeholders recognizing both the positive and concerning aspects of the IRA.