PIRC

PROTECTING INNOVATION IN RARE CANCER

REBALANCE THE IRA

PIRC Meeting

May 13, 2024

Saira Sultan, Policy and Government Affairs

PIRC

PROTECTING INNOVATION IN RARE CANCER

REBALANCE THE IRA

PIRC Purpose

- Speak loudly with one voice
- Speak from a rare cancer perspective
  - ***Educate*** *ourselves (& our communities)*
  - **Applaud** what we can
  - **Prepare** for what is coming
  - **Fight** against what must change
    - NOW AND NEXT YEAR
    - PREPARATION

PIRC

PROTECTING INNOVATION IN RARE CANCER

REBALANCE THE IRA

Agenda

Medicare Drug Price Negotiation Program

IPAY 2027 Draft Guidance

a. May 3 release
b. 60-day comment period
c. July 2, 2024 deadline

Resources

- Draft Guidance
- Fact Sheet with an updated timeline for the second cycle of negotiations
- Information Collection Request for Small Biotech Exception and Biosimilar Delay for comment

Broadly Addresses…

The Draft Guidance Covers a Range of Manufacturer-Specific Issues

- Ensuring pharmacies/dispensers are reimbursed for acquisition cost minus Maximum Fair Price (MFP)
  - Entities eligible for MFP
  - 14-day clock for reimbursements
  - Data for dispensing entities
- Role of Medicare Transaction Facilitator (MTF)
  - Verify selected drug was dispensed
  - Payment mechanism
  - Manufacturer enrollment with MTF
  - Data requirements
  - Disputes
- 340B implications – verification, duplicates
- Bona fide marketing of biosimilars/generics
- QSSD Definition – active moiety/ingredient remains; clarification that CMS will investigate products with different trade names marketed under different NDAs/BLAs

Context: Setting the Stage for Round II…

Round II

- 15 Part D Drugs Selected
- Single MFP, subject to an MFP ceiling, across all dosage forms and strengths, based on 30-day supply

- Timeline
  - Feb 1‘25: Deadline for CMS to publish the list of 15 Part D drugs for negotiation
  - Feb 28’25: Deadline for manufacturers of selected drugs to sign negotiation agreements.
  - March 1 ’25” Deadline for manufacturers to submit required data
  - Post-March 1, ‘25: Patient-focused listening sessions & other opportunities to engage with CMS
  - June 1’25: CMS to send initial offers to manufacturers.
  - July 1’25: Manufacturers to accept/reject initial offer/propose a counteroffer
  - Summer ‘25: CMS to respond to counteroffers
  - October 31 ‘25: Manufacturers to accept/reject CMS’s final offer
  - November 30 ‘25: CMS to publish final prices for 2027
  - March 1’26: CMS to publish an explanation of final prices

Formularies

Draft Guidance appears to indicate changes may be possible once details on Part D formularies for 2025 become available.

2026 Final guidance: Part D plans must include all 10 negotiated drugs on formularies (all dosage forms and strengths to which the final price applies) and CMS will review to ensure Part D plans do not:

- place negotiated drugs on non-preferred tiers
- place a negotiated drugs on higher tier than another drug in the same class
- require "stepping" through an "alternative" prior the negotiated drug
- require more UM for a negotiated drug than an "alternative" drug in the same class

Consider: Commenting on the new draft guidance may help agency reconsider what else should be included in review of formularies even though they've finalized this since they signal in this draft that CMS may make changes to this in future. [There are, in most cancers, no 'alternatives' in the traditional sense. This is why Part D formularies today must cover "all or substantially all" cancer drugs.]

Factors CMS Will Continue to Consider …

CMS reiterates it considers "alternative treatments" in setting initial and final price:

1. Extent to which selected drug represents a therapeutic advance compared to existing therapeutic alternatives for the selected drug and the costs of such existing therapeutic alternatives;

2. FDA-approved prescribing information for the selected drug and its therapeutic alternatives;

3. Comparative effectiveness of the selected drug and its therapeutic alternatives, including the effects of the selected drug and its therapeutic alternatives on specific populations (incl/ individuals with disabilities, elderly, terminally ill, children, and other patient populations); and

4. Extent to which the selected drug and the therapeutic alternatives to the drug address unmet medical needs for a condition for which treatment or diagnosis is not addressed adequately by available therapy

Patients/Patient Orgs Can Submit:

- ✓ information on the drugs, "alternatives," impact on specific sub-pops, patients experience, and unmet need
- ✓ Outcomes – e/g., changes to productivity, independence, quality of life considered when these correspond w/ direct impact on the individuals taking the drug/alternative if appropriately measurable and quantifiable.

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

“Listening Sessions” Redefined

CMS will also host patient-focused *events* to seek verbal input from patients and other interested parties. “Events” intended to bring together patients, beneficiaries, caregivers, and consumer and patient organizations, other interested parties to share patient-focused feedback with CMS on patient experiences with the conditions or diseases treated by the drug/alternatives as CMS reviews data submissions and develops an initial offer for each selected drug.

“CMS intends to improve upon the design of the patient-focused listening sessions …and is soliciting comments… on event format, scope, and logistics. ….CMS is considering events where there is discussion among speakers and in which CMS may ask clarifying questions.

CMS is also **weighing different event formats**, such as **round table sessions** on broader topics with a **mix of speaker types** (e.g., patients, providers, and health data experts) or focus groups on targeted topics with one speaker type (e.g., patients or caregivers).

CMS is particularly interested in comments on events that **promote discussion versus listen-only events**. CMS is also considering **combining events for selected drugs that treat like condition(s) /disease(s)**, instead of having drug-specific events, or organizing events based on another factor.

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

“Listening Sessions” Redefined cont’d

Instead of livestreaming, CMS is considering **publishing an event summary** or, as CMS provided following the initial price applicability year 2026 patient-focused listening sessions, sharing a **redacted transcript** afterwards **omitting names and/other identifying data** for patients, patient advocacy organization representatives, and family members/caregivers (per HIPAA, etc.)

Furthermore, CMS understands that patient-focused listening sessions conducted by FDA are not livestreamed. However, CMS is soliciting feedback on the **tradeoff between maximizing participation in events and promoting access and transparency for these events by enabling livestreaming** functionality, including the option of audio-only livestreaming. CMS would appreciate comments on methods to mitigate any barriers to participation for patients and other interested parties.

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

Data Collection

CMS intends to improve upon the collection process, question format, and content received for initial price…For example, CMS may group Qs as: manufacturer input, patient or caregiver experience, clinical experience, and health research (e.g., economic and health equity data)

CMS believes this would improve the data collection process with information more closely aligned to a respondent's areas of expertise, although any interested party would be invited to respond to all questions regardless of area of expertise or question grouping.

CMS also considering revising questions within these categories; for example, pertaining to patients' conditions, CMS is considering requesting a description about what it is like to live with a medical condition treated by the selected drug or its therapeutic alternative(s) and the factors a patient cares about most when assessing the value of a drug.

CMS is considering requesting 'section 1194(e)(2) evidence' specific to the FDA-approved indications and off-label uses for a selected drug and its therapeutic alternative(s)

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

Data Collection Cont'd.

Literature from public & manufacturers and its own review: *CMS will consider the source, rigor of the study methodology, current relevance to the selected drug and its therapeutic alternative(s), whether the study has been through peer review, study limitations, degree of certainty of conclusions, risk of bias, study time horizons, generalizability, study population, and relevance to the negotiation factors listed in section 1194(e)(2) of the Act*

CMS will prioritize research, incl. (i) observational research and (ii) research based on randomized samples, that is methodologically rigorous, appropriately powered (i.e., has sufficient sample size) to answer the primary question of the research, and structured to avoid potential false positive findings due to multiple subgroup analyses.

CMS will consider research and real-world evidence relating to Medicare populations, including on individuals with disabilities, [ESRD] patients, and Medicare-aged populations, as particularly important.

*An indication **will** incl. FDA-approved indication and **may include** off-label use(s) in nationally recognized, evidence-based guidelines and listed in **CMS-recognized Part D compendia***

Consider: CMS doesn't really have expertise for such analysis. What if studies in drug and/or alternative aren't in Medicare pop.? CMS understanding of different evidentiary standards in rare?

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

Methodology: Developing Initial Offer/Adjusting Starting Point…

CMS distinguishes b/w "**fda approved indication**" and "Indication" – latter = condition for which the drug is used; states CMS will use latter to develop initial offer. And take stakeholder info, and "prioritize clinical appropriateness in the selection of therapeutic alternatives."

In considering treatment effect and price differentials, CMS will identify a **therapeutic alternative** ("alternative") for each indication of selected drug, The term "therapeutic alternative" may refer to one or more therapeutic alternative(s) or a subset of therapeutic alternatives *that are clinically comparable*.

CMS may also consult with clinicians, patients or patient organizations, and/or academic experts, to ensure that appropriate therapeutic alternatives are identified.

Consider: Will CMS disclose its list of alternatives?

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

Adjusting the Starting Point…. And Analysis for Selected Drugs with Therapeutic Alternative(s)

CMS will consider/defines **patient centered outcomes** as an outcome important to patients' survival, functioning, or feelings as identified or affirmed by patients themselves, or judged to be in patients' best interest by providers and/or caregivers when patients cannot report for themselves.

CMS notes throughout it will consider drug/alternatives' impact on **unmet medical need**, which it defines as "no other treatment options exist, or existing treatments do not adequately address the disease or condition" at the indication level.

Finally, CMS states it will use a qualitative approach to preserve flexibility in negotiation, including the ability to consider nuanced differences between different drugs, for example interactions with other treatments commonly prescribed simultaneously for a condition or disease, and other factors that might not be captured in a more thoroughly pre-specified quantitative approach.

PIRC
PROTECTING INNOVATION IN RARE CANCER
REBALANCE THE IRA

PIRC

PROTECTING INNOVATION IN RARE CANCER

REBALANCE THE IRA

Remember

Sign On Letter Due July 2
PIRC Letter Stronger with Diverse Examples

PIRC Standing Calls:

1. Next call cancelled for Mem. Day

We will continue cancel calls if:

1. No CMS actions
2. No requests for deep dives on particular topics (good time for learning)