July 31, 2023

VIA ELECTRONIC FILING - REGINFO.GOV

Office of Management and Budget (OMB)
725 17th St NW
Washington, DC 20503
Attn: OMB Desk Officer

Re: ICR Reference Number: 202306-0938-013. Information Collection Request for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act (CMS-10847).

To The OMB Desk Officer:

The Pharmaceutical Research and Manufacturers of America (PhRMA) is pleased to submit these comments in response to the Centers for Medicare & Medicaid Services' (CMS, the Agency) *Information Collection Request for Negotiation Data Elements under Section 11001 and 11002 of the Inflation Reduction Act* (ICR or the ICR), including the Federal Register Notice, Supporting Statement – Part A, ICR Form (CMS-10847, OMB, 0938-NEW), and the Comment Summary Responses submitted to the Office of Management and Budget.[1] PhRMA represents the country's leading innovative biopharmaceutical research companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. Since 2000, PhRMA member companies have invested more than $1.1 trillion in the search for new treatments and cures, including $102.3 billion in 2021 alone.

PhRMA submitted lengthy comments on CMS' original ICR, proposed March 21st, 2023, for a 60-day comment period and attached with this submission as Appendix B. Unfortunately, CMS fails to address the vast majority of such comments, which focused on key considerations under the Paperwork Reduction Act (PRA): (1) the scope, necessity, and utility of the proposed information request for proper performance of CMS' functions relating to the Drug Price Negotiation Program (the Program); (2) ways to enhance the quality, utility, and clarity of the information to be collected; and (3) burden estimate.

Rather than reiterate prior comments, PhRMA is attaching our comments on CMS' initial Guidance (Appendix A) and our comments to CMS in response to the original ICR (Appendix B), as well as outlining in this letter the main reasons CMS' ICR continues in its failure to comply with the letter and spirit of the PRA. Importantly, CMS deviates from PRA regulations, which require an agency to take "every reasonable step to ensure that the proposed collection of information:

> (i) Is the least burdensome necessary for the proper performance of the agency's functions to comply with legal requirements and achieve program objectives;
>
> (ii) Is not duplicative of information otherwise accessible to the agency; and

[1] 88 Fed. Reg. 42,722 (July 3, 2023). Centers for Medicare and Medicaid Services (CMS), Information Collection Request for Negotiation Data Elements under Sections 11001 and 11002 of the Inflation Reduction Act; Supporting Statement – Part A (June 30, 2023). Available at: https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10847;). Information Collection Request for Negotiation Data Elements under Section 11001 and 11002 of the Inflation Reduction Act, ICR Form (June 30, 2023). Available at: https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10847. CMS, Responses to Public Comments Received for CMS-10847, Final clean 60-Day Data Elements Comment Summary Responses (Uploaded June 29, 2023). Available at: https://www.reginfo.gov/public/do/PRAViewDocument?ref_nbr=202306-0938-013.

> (iii) Has practical utility. The agency shall also seek to minimize the cost to itself of collecting, processing, and using the information, but shall not do so by means of shifting disproportionate costs or burdens onto the public."[2]

Because CMS' revised ICR fails OMB's regulatory test for the PRA, it is incumbent on OMB to now work with CMS to further modify the form.

I. Concerns with Respect to Burden

CMS acknowledges it received comments that the proposed submission requirements (i) are burdensome; (ii) request a large volume of data and/or level of detail Primary Manufacturers may not have access to; (iii) are unreasonable in light of the narrow, 30-day time frame manufacturers have to respond to the ICR; and (iv) would be better effectuated if respondents could employ reasonable assumptions regarding data submission.[3] Nevertheless, CMS makes only de minimis changes to the form. CMS has deleted a few questions (as further discussed below), yet the ICR continues to extend for 47 pages, with each question comprised of multiple subparts. The form continues to require Primary Manufacturers to obtain data from "Secondary Manufacturers," and to submit all data within just 30 days. In a number of cases, CMS also refuses to allow reasonable assumptions, despite stating that doing so "may reduce reporting burden."[4] CMS also argues that reporting to the SEC and under the Medicaid Drug Rebate Program demonstrates that manufacturers have experience providing "similar data."[5] But CMS fails to address the ways these collections differ significantly (in granularity, volume of information requested, and the ability to employ reasonable assumptions) from the revised ICR. For example, in prior comments PhRMA noted:

> PhRMA does not believe that CMS should be capturing [research and development (R&D)] cost data at a granular level and should instead amend the ICR to allow a single global response for R&D costs, similar to a Form 10-K for Securities and Exchange Commission (SEC) filing, and a single attestation (YES/NO) regarding the extent to which these costs have been "recouped."

CMS does not address how much more burdensome its revised ICR is as compared to SEC filings, or Medicaid reporting. OMB should therefore perform due diligence and require CMS to support or correct its assertions on the SEC and Medicaid Drug Rebate Program.

Finally, despite numerous comments to the contrary, CMS adheres to its initial reporting estimate that manufacturers (across all team members working on a submission) will spend 500 hours each to gather and submit the information CMS requires (at a cost of about $51,600 per respondent). Such an estimate is unreasonable on its face, but especially given CMS' estimate that the Agency will spend more than 60 times this amount for its own work (at approximately $3,450,000 -- $3,131,538 for CMS staff plus $316,116 for staff and contractors to build HPMS) (Supporting Statement, Tables 2, 6 and 7).

II. Concerns with Respect to Duplicated Information

The PRA requires agencies to ensure they do not demand data already available, so as to avoid duplication. CMS admits it is requesting available data but states it will require submission by the Primary Manufacturer to ensure data is up to date.[6] CMS also asserts: "the Primary Manufacturer is best positioned to provide the requested data and the statute provides the manufacturers participating. . .will submit the requested data."[7] CMS fails to explain why it cannot be sure data are complete or up to date if

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[2] 5 C.F.R. § 1320.5(d)(1)(i)-(iii).
[3] CMS, Response to Public Comments Received for CMS-10847 at 1-2.
[4] *Id.* at 2.
[5] *Id.*
[6] *Id*. at 3.
[7] *Id.*

the Agency uses already available information, such as "publicly available" "Federal Supply Schedule" and "Big Four Prices." Nor does CMS explain why the PRA would permit an agency to impose heavy burdens on respondents for the possibility that data could be marginally more up to date. CMS further fails to respond to industry comments that it may comfortably interpret its statutory authority to allow a manufacturer to agree that CMS' use of a specific source of data would constitute the manufacturer's "submission" of such data and be tantamount to an affirmative submission.[8] OMB should review PhRMA's already submitted comments on the ICR, and work with CMS to identify any data that duplicates already available information. To the extent information is already available, OMB should require CMS to use such sources, as required under the PRA.

III. General Comments and Other Concerns

Please refer as well to our prior comments on other aspects of CMS' proposed ICR, including but not limited to:

Unnecessary Word Limits:

Eliminating character and word limits gives manufacturers the ability to better explain their data elements and therefore provides CMS a better understanding of what data have been submitted. At the very least, given that it is in the first year of the program, CMS should eliminate word limits, and if responses provide extraneous information on certain questions, CMS could then implement revised word limits on those questions in subsequent years of the program. In the alternative, CMS could *recommend* certain word limits, explain Agency limits on staffing, and state that it is best able to review responses if the responses comply with certain word limits. In particular, by imposing word limits on the questions on therapeutic alternatives (particularly for questions 28-31), CMS is depriving respondents of the ability to provide important contextual and narrative information in a way that is user-friendly and less burdensome. Further, CMS announcing in advance that it will cut patients and caregivers off partway through a potential written explanation of their experiences potentially sends a message to these patients and caregivers that discourages them from providing input.

Unnecessary Citation Limits:

Similar to our concerns over word limits, we feel that unnecessary citation limits may prevent CMS from fully understanding the full benefits that a selected drug may bring to patients. CMS should seek out all relevant information, especially during the first year of the program. That said, we acknowledge CMS' clarification that tables, charts, and graphs are permitted to be submitted as additional materials for certain questions.

Data Submission Deadlines:

We appreciate CMS' inclusion of planned meetings by CMS (with manufacturers and public, patient-focused "listening sessions") in the fall of 2023 that will provide an opportunity for interested stakeholders to "share new information on the Section 1194(e)(2) factors" and provide context on the 1194(e)(1) submission.[9] However, PhRMA believes that CMS has broader discretion to accept additional written data from manufacturers and members of the public related to 1194(e)(1) and (e)(2) factors. Although the Guidance now allows for up to 50 pages of material to be shared with the Agency to aid the pre-initial offer meeting between CMS and manufacturers held after October 2nd, the Agency has not created explicit mechanisms to enable additional data from manufacturers and the public under its guidance or ICR form on data elements. Furthermore, the patient and provider groups which serve

[8] We believe CMS' erroneous conclusion is based upon statutory language stating that the Secretary should consider certain data with respect to the selected drug "as submitted by the manufacturer." SSA § 1194(e)(1).
[9] Negotiation Program Final Guidance at 5.

underserved or underprivileged communities – who will likely be most impacted by the unnecessarily short data submission window – still do not have additional opportunities to submit data. *Note:* If CMS and OMB embrace PhRMA's statutory interpretation that the Agency may accept such data after October 2nd, CMS would no longer be collecting large amounts of proprietary data, solely so the Agency has the data it *might* need to determine the initial offer. CMS has requested large amounts of data (sometimes five years of data) – without fully explaining why or how it needs such data. And instead of finding flexibility where it can, the Agency instead imposes additional burden on respondents. By collecting such large volumes of proprietary data, the Agency increases the severity of any potential breach. OMB should work with CMS to re-examine the ability to gather just the information the Agency knows it will use, and then to supplement such information, only when necessary.

Identify and Protect Proprietary Information:

Under the MFP process, it will be critically important for CMS to protect proprietary data. Although CMS has identified the HPMS portal for data submission, CMS has failed to identify a secure, alternative data submission portal if HPMS is not ready by the end of the data submission window. In the supporting statement to the revised ICR, CMS notes that "[i]f CMS HPMS is not used for submission because the CMS HPMS tool is delayed, responses to this ICR should be sent by email to IRARebateandNegotiation@cms.hhs.gov." PhRMA believes that the alternative submission process via email is not an acceptable way to secure confidential information. CMS should guarantee an encrypted alternative submission mechanism if HPMS is unavailable.

In addition, accurately identifying data that are proprietary represents a critical first step, and CMS should create room on the ICR form so manufacturers may easily identify information as proprietary. While CMS states it will consider certain information proprietary, it does not offer manufacturers the opportunity to identify data as proprietary, as PhRMA previously requested in comments to the Agency.

Access to Secondary Manufacturer Information:

Primary Manufacturers may not necessarily have access to Secondary Manufacturers' information, particularly within the deadlines required under the Program. While CMS notes that Primary Manufacturers may have "agreements" with Secondary Manufacturers, it fails to address or even acknowledge the narrow window for revising such agreements (i.e., revised guidance not issued by CMS until June 30th, just about three months before data submission will be due to CMS, and only approximately one month between drug selection and data submission). OMB should prevail on CMS to further explain its reasoning on this issue and create exceptions for Primary Manufacturers unable to revise their agreements.

IV. Manufacturer Data

Please refer as well to our prior comments on other aspects of CMS' proposed ICR, including but not limited to:

Non-FAMP Data:

CMS has statutory authority to align with Veterans' Affairs Non-FAMP data so as to avoid duplicating information, in accordance with the PRA. OMB should explore with CMS why it failed to read the statute in a more reasonable manner that would reduce reporting burden and encourage CMS to adopt such a reading.

R&D Costs and Recoupment:

While CMS makes minor revisions to the reporting of R&D costs, it will still break R&D costs into five categories, along with a sixth and seventh category for R&D recoupment (global total lifetime net revenue, along with U.S. lifetime net revenue). As PhRMA previously explained in comments, this

seven-part subdivision is well beyond how manufacturers report such data in other contexts, how they organize data, and potentially contravenes manufacturers' document retention policies. OMB should consider the fact that some companies do not readily have access to this information. In addition, CMS failed to adequately define and clarify components of R&D cost data, causing ambiguity that further hinders companies' ability to respond. For example, neither the ICR nor the June 30th guidance adequately define "same therapeutic class", which could be interpreted as FDA Established Pharmacologic class, USP Drug Class, or something else. OMB should require CMS to avoid ambiguity of such terms or fully define them.

CMS also states that the statute does not permit the Agency to allow for an attestation, as PhRMA recommended. CMS, however, does not specify why or how the statute, which requires "consideration" of "research and development costs of the manufacturer for the drug and the extent to which the manufacturer has recouped research and development costs,"[10] would prohibit an attestation. CMS also fails to justify the utility of its proposal. Why is the seven-part test essential to arriving at an MFP offer? CMS provides no clarity for how recoupment data will be evaluated or weighed. As discussed in our comments, we would appreciate justification for why global lifetime net revenues are necessary, and how CMS will account for global sales of non-Food and Drug Administration (FDA)-approved indications.

PhRMA urges OMB to require robust, detailed explanations from CMS as to why it requires the excruciating level of detail it has proposed. If CMS cannot justify its requests or cannot adequately explain why the IRA provides no flexibility to request information in a more reasonable and less burdensome manner, OMB should work with the Agency to significantly streamline the form.

Current Unit Costs of Production and Distribution:

PhRMA notes that CMS' definition of "marketing costs" in the ICR conflicts with CMS' final guidance policy on bona fide marketing. In the ICR, CMS defines "marketing costs" as "expenditures incurred in the introduction or delivery for introduction into interstate commerce of a drug product, specifically including media advertisements, direct-to-consumer promotional incentives including patient assistance programs, promotion of the drug to health professionals, and other paid promotion."

Prior Federal Financial Support:

We appreciate CMS accepting the comment that if CMS is to limit R&D manufacturer costs to only FDA-approved indications for the selected drug, CMS similarly should be consistent and consider only the Federal financial support directly relevant to such labeled indications. However, PhRMA still has significant concerns with CMS' overall approach to the calculation of R&D costs. Manufacturer systems may not have the necessary data to identify, calculate, or allocate R&D of Federal funding in the manner in which CMS prescribes. In particular, these costs have never been required to be "assigned" or "allocated" to an FDA-approved indication, and it is likely many manufacturers will not have the information or ability to comply with CMS' proposal, particularly for historical costs, some of which could be decades old. In order to attempt to calculate their responses, manufacturers may necessarily need to develop allocation assumptions, based on the information that is available to them. Alternatively, we urge OMB to call on CMS to adopt one of the three proposals in PhRMA's original comments.

Beyond the concerns with R&D costs and Federal funding outlined above, CMS fails to articulate directions for allocating tax credits or other support to research and development with the selected drug specifically. OMB should prevail upon CMS to acknowledge that respondents may take a variety of methodological approaches to the allocation of broadly applicable support to their selected drugs specifically. We appreciate CMS accepting the comment that if CMS is to limit R&D manufacturer costs

[10] SSA § 1194(e)(1)(A).

to only FDA-approved indications for the selected drug, CMS similarly should be consistent and consider only the Federal financial support directly relevant to such labeled indications. However, CMS fails to articulate directions for allocating tax credits or other support to research and development with the selected drug specifically. OMB should prevail upon CMS to acknowledge that respondents may take a variety of methodological approaches to the allocation of broadly applicable support to their selected drugs specifically.

We also appreciate CMS clarifying that prior Federal financial support is reported only for the period starting from when the manufacturer acquired the drug. However, CMS is still requiring an inordinate amount of detail for Federal financial support (including, for example, a listing of "*each* licensing agreement, pricing agreement, purchasing agreement, and other agreement in place between your company and any federal government agency related to the discovery, research and/or development of the selected drug"), without explaining precisely why a disaggregated amount is necessary to support an MFP initial or final offer. CMS makes a tautological statement that a disaggregated amount will allow for a more "complete understanding,"[11] but does not explain why this "complete" understanding (as compared to an understanding based on an aggregated amount) will or should affect CMS' initial offer or final offer. If a company has received Federal financial support, will the type of support (tax credit versus grant) affect how CMS calculates the initial or final offer? The Agency does not say. We urge OMB to require more of CMS. If CMS cannot justify why its multi-layered request is essential, OMB should insist upon one rolled-up figure for prior Federal financial support, as doing so complies with IRA statutory requirements and is consistent with the PRA's "least burdensome" requirement or should adopt one of PhRMA's proposals.

Patents, Exclusivities, Applications, and Approvals:

Please see our prior comments on how CMS can, in accordance with the PRA, use already-available information, along with our discussion of CMS' statutory authority to do so.

We also recommend that OMB require CMS to explain why it needs each piece of requested patent, exclusivity, application, and approval data. For example, CMS demands information on patents and patent applications related to the selected drug that "include, but are not limited to" "any patents that are, have been, or may be listed for the selected drug in the FDA Orange Book or Purple Book; utility patents that claim the drug product (formulation or composition), drug substance (active ingredient), metabolites or intermediaries of a selected drug, method(s) of using the drug, or method(s) of manufacturing the drug; and design patents that, for example, claim a design on the packaging of the selected drug." CMS does not explain why it must request such a burdensome list of data: a list that is more expansive and more burdensome to comply with than the prior version. OMB's PRA oversight responsibility – as well as its commitment to reducing burden wherever possible – makes it incumbent on your Office to require specific justifications for each element of data and if these elements cannot be justified, to work with CMS to reformulate this request to reduce the associated burden.

Market Data, Revenue, and Sales Volume Data:

We support CMS's decision to remove metrics related to "manufacturer average net unit price to Part D Plan sponsors," 340B ceiling price, and 340B Prime Vendor Program price. Even so, CMS continues to engage in serious overreach, collecting an extreme amount of data, when the IRA simply requires the Secretary consider the factor of: "Market data and revenue and sales volume data for the drug in the United States." The IRA does not require the metrics CMS has proposed, with the exception of "non-FAMP." CMS has also not responded to PhRMA's questions as to why data must be reported for each quarterly period in the most recent five years, presenting a substantial burden without any basis in statute.

[11] CMS, Response to Public Comments Received for CMS-10847 at 11.

Nor does CMS address PhRMA's concerns related to "Primary Manufacturers" reporting these data on behalf of "Secondary Manufacturers," when such reporting could be inconsistent with contractual agreements, without sufficient time to revise such agreements. CMS also fails to explain why it continues to rely on FSS and "Big Four" pricing when the Senate overwhelmingly rejected (by 99-1) amendments that would have incorporated FSS and "Big Four" pricing into the IRA.[12] In regards to CMS' three newly minted metrics of: (1) U.S. commercial average net unit price; (2) U.S. commercial average net unit price – without patient assistance program; and (3) U.S. commercial average net unit price – best; CMS fails to address comments that the new metrics are not defined with specificity and the lack of clear definitions will likely result in inconsistencies,[13] and the requirement for manufacturers to provide data on these new metrics covering quarterly periods for five years creates a particularly excessive burden. The excessive burden is attributed to the requirement of manufacturers having to develop and launch a new transaction-level data collection and analysis infrastructure for the new U.S. commercial average net unit price measures, on an extremely expedited timeline, due especially to the added complexity of having to account for supported provided to purchasers and potentially including patients – such as "coupons" and "goods in kind"—as "concessions" in the average net unit price calculation. However, CMS' interpretation is at odds with industry's view and treatment of such support services. If Congress had wanted CMS to base the MFP on commercial "best" price or similar metric, it would have required disclosure of such a proprietary metric. If it does maintain these ultra vires metrics, CMS should explicitly exclude all prices that are not prices to commercial customers.

OMB should ensure that CMS complies with the law, and direct CMS to eliminate from the ICR all pricing metric requests that go beyond non-FAMP (as opposed to market, revenue, or sales data).

Certification for Sections A through G:

PhRMA notes that CMS has created two certification statements – one for Sections A through G of the ICR, and one for all respondents for Section I of the ICR. For the first certification, CMS responds to comments only by comparing such certification statement to "other information collection requests related to the Negotiation Program."[14] As noted in PhRMA's prior comments, CMS will require respondents to acknowledge that responses may "give rise to liability, including under the False Claims Act." Other than False Claims Act liability, CMS does not specify what kind of liability the form "may" give rise to. CMS also cites no other program – other than IRA price-setting – that requires an analogous certification threatening False Claims Act liability for an information collection. Despite comments on this issue, CMS also did not delete, or even further specify, the unclear and open-ended requirement that respondents "timely notify" the Agency if "I become aware that any of the information submitted in this form has changed." Notably, CMS responds to comments on the certification statement only by comparing the statement to "other information collection requests related to the Negotiation Program." As noted in PhRMA's prior comments, CMS will require respondents to acknowledge that responses may "give rise to liability, including under the False Claims Act." Other than False Claims Act liability, CMS does not specify what kind of liability the form "may" give rise to. [15] also cites no other program – other than IRA price-setting – that requires an analogous certification threatening False Claims Act liability for an information collection. Despite comments on this issue, CMS also did not delete, or even further specify, the unclear and open-ended requirement that respondents "timely notify" the Agency if "I become aware that any of the information submitted in this form has changed."

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[12] 24 S. Amdt. 5210 to S. Amdt. 5194 to H.R 5376. Available at: https://www.senate.gov/legislative/LIS/roll_call_votes/vote1172/vote_117_2_00288.htm.

[13] CMS should be well aware that other mandatory pricing metrics (such as Average Manufacturer Price, Best Price, and Average Sales Price) have involved nuances in definition that have taken many years to fully address. Creating completely new mandatory pricing metrics under such short timelines for consideration risks an ill-defined and ill-targeted metric.

[14] CMS, Response to Public Comments Received for CMS-10847 at 15.

V. Evidence about Alternative Treatments

Primary Manufacturers and interested third parties may submit information on the factors described under Section 1194(e)(2) of the SSA on the selected drug and available therapeutic alternative(s) under the "Evidence About Alternative Therapies" section of the ICR. While we appreciate that CMS did revise some areas of this section, including expanding the number of respondent types and adding a new question on the Patient and Caregiver experience, we still have some concerns with this section. Please refer to Section V of our prior comments for our concerns over this section of the ICR, including but not limited to:

Transparency for Manufacturers of Selected Drugs:

CMS should provide transparency and visibility as to how it will conduct its review of the evidence and provide further guidance on whether this information obtained will be disclosed to manufacturers and other data submitters. Further, the Agency should publicly describe the process it will use to obtain information for clinical and subject matter experts through mechanisms other than the ICR, and how this information will be made available to the public and/or manufacturers participating in the MFP process. Upon review, CMS should make publicly available the non-proprietary data it gathers under Section 1194(e)(2) on alternative treatments and should share information with the manufacturers of selected drugs and therapeutic alternatives as quickly as possible.

Clarification of Terms:

While PhRMA appreciates that the Agency clarified some key themes and terminology included in the ICR, CMS still leaves many definitions unclear. As stated in Section V of our comments to CMS on the Data Elements ICR, since Section I of the ICR is open to the public with various levels of pre-existing knowledge regarding CMS' price-setting process, it is critically important that CMS provides clear definitions for all stakeholders at the beginning of each question and in the instructions to help stakeholders understand what information CMS is seeking. In addition to the key terms outlined in our comments to CMS, additional examples of areas of concern are set forth below:

- Therapeutic Alternative: While the additional details regarding the definition of "Therapeutic Alternative" provided in Questions 27 and 28 are helpful, they fall short on several points. The first deficiency is using terms such as "drug class," "chemical class," and "therapeutic class," without formal definitions or relationships to established definitions. Consequently, the definition of therapeutic alternative remains ambiguous and subjective. Additionally, when many potential therapeutic alternatives exist (highly probable for most potential drugs under review), CMS may focus on alternatives that are "most clinically comparable." How will clinical comparability be defined? By whom? This again adds a degree of subjectivity to the definition that makes a reliable and predictable response highly unlikely. CMS apparently recognized this issue and noted in its crosswalk that it had "added a definition of therapeutic class," but then did not add such a definition to the form itself.[16]

- Therapeutic Impact: CMS declined to include a definition of therapeutic impact in the ICR. CMS should provide additional detail on what this entails or use and clearly define an alternative term.

- Unmet Medical Need: While PhRMA appreciates that CMS expanded their definition of "unmet medical need" to align more closely with the definition currently in use by the FDA, this does not capture the full range of unmet needs and may inadvertently undervalue the needs and values of certain communities. As such, OMB should explore with CMS the option of expanding this

[16] Negotiation Data Elements ICR Crosswalk of Changes Between 60-Day Notice and 30-Day Notice. https://www.cms.gov/regulations-and-guidance/legislation/paperworkreductionactof1995/pra-listing/cms-10847

definition to explicitly recognize other types of unmet needs including, but not limited to: 1) personalized medicines for certain subpopulations; 2) progress against rare and hard-to-treat illnesses; 3) treatments that improve patient adherence and quality of life; 4) need for additional treatments in a therapeutic area, such as a curative treatment; 5) treatments that improve the health of underserved and vulnerable communities who face health disparities; 6) treatments that benefit multiple common comorbidities at once; 7) populations and individuals failing to meet established treatment guideline goals from available therapies and; 8) the stepwise nature of progress in which significant gains for patients are achieved via advances that build on one another.

Patient-Focused Listening Sessions:

PhRMA appreciates CMS' final guidance that it will host patient-focused listening sessions that will be open to the public, including patients, beneficiaries, caregivers, patient/public advocacy organizations, and other interested parties to share patient-focused input on therapeutic alternatives and other Section 1194(e)(2) information regarding selected drugs. CMS should ensure that these listening sessions are meaningful in line with engagement standards from key stakeholders such as the Patient-Centered Outcomes Research Institute[17] or the International Society for Pharmacoeconomics and Outcomes Research.[18] At least one, if not more than one, listening session per selected drug should occur, and the listening sessions should last long enough for patients, caregivers, and other stakeholders to present their real-word perspectives on the benefits of a selected drug, along with the comparator therapeutic alternative(s). In addition, as stated in our previous comments, the breadth and complexity of the information that CMS is evaluating, and its importance to patients, caregivers and public health all reinforce the importance of the Agency establishing supplementary mechanisms for gaining ongoing stakeholder input (for example, from patients, caregivers and clinicians) cannot be understated. CMS will not be able to ensure a patient-centered process nor gain a complete and accurate picture of factors such as relative clinical benefit and unmet need without a) properly and clearly defining these terms and b) engaging patients, clinicians, and other stakeholders on an ongoing basis.

PhRMA previously commented that CMS should announce at least the potential therapeutic comparators the Agency is considering prior to the data submission deadline of October 2nd. CMS could announce the comparators when it releases the list of selected drugs, and doing so would focus responses from stakeholders. Now that CMS has announced listening sessions, advance notice is even more essential. Commentators will need to understand exactly which therapeutic comparators CMS is considering so that they can adequately prepare to discuss their experiences.

Quality-Adjusted Life Years:

PhRMA appreciates CMS' statement that it will require respondents to identify whether cost-effectiveness measures are used in submitted evidence, and if so whether the measure used treats extending the life an individual who is elderly, disabled, or terminally ill as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill. OMB should explore with CMS whether it believes this clarification will ensure against the use of quality-adjusted life-years (QALYs) or similar measures, as prohibited by both Sections 1182(e) and 1194(e)(2) of the Social Security Act, or whether having a respondent certify or attest that their submitted research does not use QALYs or similar measures would be a more effective option. It also is unclear how CMS will ensure

[17] Patient-Centered Outcomes Research Institute's Advisory Panel on Patient Engagement. (2021). Equity and Inclusion Guiding Engagement Principles. PCORI. Available at: https://www.pcori.org/about/pcoris-advisory-panels/advisory-panel-patient-engagement/equity-and-inclusion-guiding-engagement-principles.

[18] Harrington R. L., Hanna M. L., Oehrlein E. M., et al. Defining patient engagement in research: Results of a systematic review and analysis: Report of the ISPOR Patient-Centered Special Interest Group. *Value Health*. 2020;23(6):677-688.

that the evidence it reviews does not rely on QALYs or similar measures. If in response to the question of whether the submitted evidence "treat[s] extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, nondisabled, or not terminally ill," a respondent checks no, or fails to check boxes at all, what will CMS do? Will CMS commit to reviewing the underlying literature submitted and ensuring QALYs or similar measures are not the basis for the conclusions in the study regarding comparative effectiveness? Will CMS share such underlying literature with manufacturers of selected drugs as a check on CMS consideration of QALYs or similar measures?

Certification of Submission of Section I for All Respondents:

PhRMA appreciates CMS significantly revising the certification by removing, among other things, the clause that stated that "any misrepresentations may also give rise to liability, including under the False Claims Act." PhRMA continues to believe that patients and caregivers, responding in their individual capacity, may be chilled by a requirement to sign any certification whatsoever.

VI. **Conclusion**

PhRMA appreciates the opportunity to submit comments on the revised ICR. This letter includes our key priorities but does not represent the totality of our concerns with CMS' voluminous ICR. We urge OMB to review PhRMA's prior comments, and ensure CMS complies with the PRA in implementing the price-setting provisions of the IRA.

Please contact Judith Haron at jharon@phrma.org and/or Randy Burkholder at rburkholder@phrma.org if there is additional information we can provide or if you have any questions about our comments.

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