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Re: *Medicare Drug Price Negotiation Program Draft Guidance*

California Life Sciences (CLS) appreciates the opportunity to comment on the recent guidance by CMS, *Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191-1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price (MFP) in 2026 and 2027.* CLS welcomes the chance to provide feedback on the implementation of the Medicare Drug Price Negotiation Program (MDPNP) and to highlight key considerations when implementing the law. CLS appreciates the steps the agency has taken to establish a dialogue with key stakeholders about the negotiation program and other elements of the Inflation Reduction Act (IRA), but we have significant concerns about the effects the implementation of this law will have on California's life sciences ecosystem and our companies' abilities to bring new, lifesaving medicines to patients.

CLS is proud to represent more than 1,200 companies and organizations across California and to advocate for the whole breadth of our state's life sciences sector, with membership spanning biotechnology, biopharmaceutical, medical device and technology, diagnostic companies, venture capital firms, and research hospitals and universities. California's life sciences industry generates more than 1.1 million direct and indirect jobs[i] and over $472 billion in economic output for our state on an annual basis,[ii] and our members drive innovations in patient care and save lives nationally.

The process of therapeutic development is a high risk and long-term endeavor. Life sciences leaders are inspired to take on this challenge by their desire to improve the lives and health of patients and their communities. CLS strongly supports policies that both uphold the scientific enterprise and ensure that these products are affordable and accessible to all.

The Initial Price Applicability Year for 2027 (IPAY27) Draft Guidance dictates how CMS will implement the MDPNP, which will have significant impacts on the future of Medicare, patients, access to medicines, and the future of the life sciences ecosystem. We are hopeful that CMS will incorporate the meaningful feedback provided by industry, patient groups, providers, and others on the IPAY27 Draft Guidance.

CLS is deeply concerned that the seismic shift, caused by the CMS price setting authority in the IRA, will drive us away from the current market-based systems that underpin both Medicare Part D and Medicare Part B and will erode patient access as well as undermine continued biopharmaceutical innovation. Unfortunately, the IPAY27 Draft Guidance only serves to reinforce and increase our concerns. Please see the below considerations for CMS to consider before finalizing the IPAY27 Draft Guidance.

**Qualifying Single Source Drug (QSSD)**

CLS is disappointed to see that CMS maintains a broad definition of QSSD, inclusive of New Drug Applications (NDAs) and Biological Licensing Agreements (BLAs) with the same active



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moiety or ingredient held by the same NDA/BLA holder. CLS remains concerned about the continued use of an extremely broad approach to identifying selected drugs, stating that any form of a drug from the same manufacturer with the same active moiety or active ingredient will be swept into the definition of a QSSD. This means that a drug approved only a year ago by the Food and Drug Administration (FDA) could be subject to price-setting even if it has a different trade name and if the new drug represents a significant advancement for patients. CMS' overly broad interpretation of the statute will have serious and negative effects on innovation intended to improve patient lives.

However, CLS strongly supports CMS' continued treatment of fixed combination drugs with distinct combinations of active moieties or active ingredients as distinct QSSDs. Specially, as under the IPAY26 guidance, the IPAY27 Draft Guidance proposes that if a selected drug is "a fixed combination drug with two or more active moieties/active ingredients," then "the distinct combination of active moieties/active ingredients will be considered as one active moiety/active ingredient for the purpose of identifying potential qualifying single source drugs" This approach is consistent with the QSSD statutory definition, which limits a QSSD to a drug approved under a NDA or BLA and uses the terms "drug product" or "biological product." Fixed dose combination drugs are not merely changes in the "dosage form" or "dosage strength" of an existing drug. Rather, they include the addition of an entirely different molecular entity and constitute distinct drugs that involve significant alterations from existing products. Not only is treating fixed combination drugs as distinct QSSDs consistent with the IRA, but it is supported by the clinical benefits brought to patients.

**Generic and Biosimilar Competition**

A robust market for generic and biosimilar drugs provides patients, Medicare, and other payers with significant savings, while encouraging ongoing therapeutic innovation. Safeguarding the incentives for generic and biosimilar development is vital for CMS to maintain long-term savings for the Medicare program and the health care system broadly.

While most brand medicines with an approved competitor are exempt from price setting, the timing for selection in the law predates the typical timeline for generic and biosimilar competition. CLS is concerned that in the Draft Guidance, CMS states it will look at specified data in Medicare and Medicaid to evaluate if a competitor is engaged in "bona fide marketing" – a concept nowhere in the statute and that ignores the reality that insurers and PBMs decide what medicines are covered. This standard for determining the date of marketing of a generic or biosimilar is incompatible with the statute and contrary to sound public policy. CLS disagrees with CMS's plan to use this concept to determine if a marketed generic or biosimilar "counts" as a competitor and encourages CMS to abandon its bona fide marketing standard. As a result, marketed generics or biosimilars will be forced to compete against medicines with government-set prices, significantly reducing the incentive to bring them to market. It is imperative that CMS abandon this standard and instead adopt as its standard the "market date" reported under the Medicare Drug Rebate Program (MDRP). The MDRP "market date" standard should be used for identifying both the date on which a generic or biosimilar is marketed and the date on which CMS determines that a generic or biosimilar has been marketed.

Further, to enhance the process for a biosimilar manufacturer to request a delay in the selection of a reference product for negotiation, CLS recommends including meeting the "high likelihood" determination. CLS encourages CMS to make a determination of "high likelihood" based on the most up to date and complete information and believes CMS has

the statutory authority for broad discretion in specifying that the manufacturer can submit all relevant information. To ensure that CMS decides a delay request based on the most mature information possible, CMS should set the delay request submission deadline as close as reasonably possible to the selected drug publication date and permit broad supplementation of timely request with late-breaking information or otherwise good cause. Information on the expected timing of licensure and marketing often rapidly changes and may fluctuate based on a range of factors. For CMS to make an informed determination regarding eligibility for delayed selection, it is vital that the Agency rely on all of the most recent available information that bears on the likelihood of market entry within the requisite time period.

Furthermore, CLS recommends that CMS provide notice of its delay request determination in advance of the selected drug publication date and establish a dispute resolution process. As it is currently laid out in the Draft Guidance, CMS will not inform a biosimilar manufacturer of an unsuccessful delay request until after the selected drug publication date.[iii] This eliminates the ability for a manufacturer to dispute the determination. CLS encourages CMS to provide a preliminary notice of an unsuccessful delay request in advance of the selected drug publication date and establish a process by which the biosimilar manufacturers can dispute an erroneous determination.

**Small Biotech Exemption (SBE)**

CLS continues to urge CMS to establish a dispute resolution process for the implementation of the small biotech exception. In recognition of the potential hardships to small and emerging companies who likely do not have significant reserves or multiple products on the market or in the pipeline, the IRA exempted small biotech drugs from negotiation until 2029. We urge CMS to continue to engage stakeholders regarding the SBE so that the exemption is workable for the small companies it was created to support.

First, CLS believes it is imperative that CMS implement a predictable and transparent process for small biotech manufacturers applying for this exemption. This includes a clear process for how to apply for an exemption, appropriate timelines to submit information, consistent criteria for evaluating submissions, and timely and clear notification if a drug meets or does not meet the SBE requirements.

Second, we ask that CMS provide flexibility in its discussions with the companies and maintain a dialogue with companies throughout the process to ensure complete and accurate data submissions. Additionally, if a drug has received an SBE, and the manufacturer's circumstances have not changed in a material way, the manufacturer should not have to re-apply in subsequent years. We also believe that CMS must protect the confidentiality of the proprietary information that is submitted by a manufacturer.
As stated above, of utmost importance to CLS members is for the Agency to establish a dispute resolution process where a manufacturer can respond to and appeal a negative determination by CMS—similar to the process that has been instituted for the specified small manufacturers phase-in under the Medicare Part D benefit redesign. Specifically, the small biotech manufacturer should have the opportunity to provide additional data or other information to the Agency to support its application for the small biotech exemption.
We also encourage CMS to initiate the SBE Information Collection Request (ICR) process earlier in the year, to allow sufficient time for a dispute resolution process to conclude prior to the February 1 deadline for CMS to select drugs for negotiation. Per CMS's IPAY 2027 Negotiation Guidance, SBE eligibility determinations are rendered after publication of the

selected drug list. Initiating the SBE process earlier would allow sufficient time for a robust dispute resolution process.

**Orphan Drug Exclusion**

Recognizing the unique challenges in orphan drug research and development, and the significant unmet medical need for rare disease patients, Congress created an exemption for orphan drugs from the MDPNP. The law says that CMS must exclude from negotiation a drug "for only one rare disease or condition and for which the only approved indication (or indications) is for such disease or condition" (Section 1191(e)(3)(A)). However, CLS remains concerned that the exemption is insufficient and, while well intentioned, undermines the long-standing incentives for orphan drug development as laid out in the Orphan Drug Act. The current Draft Guidance exempts only orphan drugs with one disease or condition and therefore could limit opportunities for additional research and development for indications to other rare diseases. Most of the research and development in additional therapeutic areas happens years after a drug is approved. But if the drug receives an additional orphan designation, it is no longer exempt and therefore we are concerned that companies will no longer have the incentive or the ability to invest further in these products. Another area we believe needs clarification is when the timeline for negotiation eligibility would begin for a product that no longer qualifies for an orphan exemption. The Draft Guidance indicates that the eligibility timeline would be based on the date of approval for the first approved indication, not approval for the additional indication.

CLS urges CMS to clarify the scope of the orphan drug exclusion in a manner that maximizes protections for orphan drugs that patients desperately rely on. We believe that CMS should clarify that the eligibility for the selection clock only begins for an orphan drug upon approval for another non-orphan indication.

Furthermore, CLS requests additional clarification around how "disease or condition" will be defined for the exemption and criteria that CMS will use to determine "conditions" from separate "indications." In addition, CMS should create a process that enables manufacturers to provide evidence that an indication falls within an orphan drug designation, where such fact is not ascertainable from FDA databases alone.

**Maximum Fair Price (MFP) Considerations and Price Setting Methodology Factors**

The IRA statute directs the Secretary to develop and use "a consistent methodology and process that aims to achieve the lowest [MFP] for each selected drug," which must include consideration of certain specified manufacturer-specific factors, factors related to therapeutic alternatives, and the statutory ceiling price. CLS remains concerned that the agency has not articulated a "consistent methodology," as required by the IRA. Furthermore, we feel that the agency should better explain how it will weigh the factors it intends to use to set prices, including how the agency will incorporate patient and caregiver experiences.

The process for setting the ceiling price and MFP can have significant impact on the investment in future therapeutic research and development. CLS continues to encourage CMS to consider the importance of driving value for patients in limiting the negotiation program's impact on the sector. A drug should be valued for its elements over the lifetime of its use, rather than at the moment in time that CMS offers the MFP. CLS encourages CMS to establish MFPs at the ceiling price for selected products that address unmet needs or significantly advance patient care. Setting higher MFPs for these products will help maintain

investment in assets and clinical programs that show scientific promise and address needs not served by current therapies.

CLS strongly encourages CMS to emphasize negotiation factors that are most important to patients—those that are related to clinical value and unmet need—and to de-emphasize manufacturer specific data elements such as cost of production and research and development costs. CLS is supportive of CMS considering additional steps to further standardize submitted information, facilitate a better understanding of the solicited information and reduce reporting burden. One way CMS can improve consistency of information submitted is to provide more detail on the definition of the manufacturer specific conditions, including to utilize a more robust definition of unmet medical need. If CMS must consider manufacturer-specific data, CLS wants to ensure that a robust, comprehensive set of information submitted by manufacturers— with necessary supplemental material—will be accepted and considered.

CLS also believes that CMS should ensure an inclusive definition of costs – for example, research and development costs should include research costs of failures where a drug did not come to market, the cost of ongoing studies, acquisition costs for both marketed and failed drug candidates, and partnering and licensing agreements. Implementing an MFP that is reflective of the complete costs of bringing a product to market will be critical to ensure companies have the ability to continue to invest in new innovation.

As the top ranked state in National Institutes of Health funding, CLS is also concerned about the requirement for CMS to consider the use of prior federal funding in the calculation of MFP. If this could further lower the price ceiling, it may discourage the use of federal funds for drug research moving forward. The inclusion of prior federal funding in the calculation may also cause hesitation to invest in companies that have used such funds, particularly as there is a lack of clarity in what constitutes prior financial support. We urge CMS to ensure a balanced approach to including the use of federal funds that will not undermine the future of public-private partnerships. Additionally, we believe CMS's suggestion that tax credits should be included into the calculation, goes against their intended purpose of advancing medical innovation and seems punitive, particularly for small and emerging companies.

**Therapeutic Alternatives**

CLS encourages CMS to clarify how it will evaluate the evidence about alternative treatments by different stakeholders and how different evidence will be considered in setting the MFP. CLS looks forward to the forthcoming data elements Information Collection Request (ICR) to ensure the collection process, question format, and content received is clear and accessible for all stakeholders to provide feedback on.

**Manufacturer Engagement**

CLS has serious concerns with CMS' process for interfacing with manufacturers of selected drugs. In the IPAY27 Draft Guidance, CMS has also proposed an abbreviated and restrictive negotiation process by setting a maximum of meetings, and only one at the request of the manufacturer. As set forth in the Guidance, manufacturers may only have up to three meetings with CMS – all occurring after the initial MFP is set by the Agency. While we agree with CMS that meetings occurring after CMS rejects a manufacturer's counteroffer is necessary and will allow for a more efficient and effective process, starting meetings only *after* rejection of the manufacturer counteroffer is too late. In the vast experience that CLS members have in negotiating with states and payers' the process CMS has implemented and

proposed in the IPAY27 guidance is unusual and arbitrary. Given the importance of these negotiations and the complexity of the data, we believe it is important to have a more flexible and meaningful process. We encourage CMS to allow for more meaningful dialogue with manufacturers throughout the process such as appropriate flexibility to start the dialogue with a manufacturer sooner, have as many meetings as necessary and not place arbitrary limitations on meetings and engagements.

CLS strongly disagrees with CMS' assertion that the selection of 15 drugs, or more in the future, "may present challenges" that would warrant the Agency to allow for *less* meetings with manufacturers than the previous years, especially in light of the fact that the current meeting structure is already counter to standard negotiations. CMS incorrectly proposes an "either-or" approach in the Guidance – three meetings OR "an additional written offer…" – when, at a minimum, CMS should be suggesting an "and" approach – three meetings *and* additional written offers, as appropriate.

CLS encourages CMS to make changes to achieve a more meaningful process, not just with the number of meetings, but with the frequency. CLS strongly believes that CMS should meet with the manufacturer of a selected medicine at multiple points during the negotiation process to allow manufacturers to address questions and provide additional commentary on the value of these medicines. Further, the manufacturer should generally be permitted to supplement its timely submission where post-development submission development arises, or there is otherwise good cause.

Another change CMS can make is to align with the standard rules of negotiation that manufacturers are bound by with other payers and in other markets. That way it is less difficult for manufacturers to adequately prepare for ongoing negotiations with CMS and to come to a shared understanding of the mutual value that these drugs bring to the Medicare program. This could, for example, be through an updated offer or counteroffer from CMS directly after a negotiation meeting so that both the manufacturer and the Agency are aligned on their shared understanding of value and are well prepared for the next steps in the negotiation process.

Finally, CLS remains concerned with the opaque nature of CMS' price-setting process. In the Draft Guidance, CMS reaffirmed that it will not disclose information about how it will set medicine prices until months after these decisions are made. CLS is concerned with the premature nature of the IPAY27 initial guidance, and the timelines set forth within related to the explanation publication and drug selection. The IPAY27 drug selection process begins prior to the required date of publication of the IPAY26 MFP explanations. Those explanations are to include, at a minimum: 1) therapeutic alternative(s) for each indication and how they were selected; 2) how each factor was weighed; 3) data and analysis CMS developed and considered supporting each factor, including evidence provided by third parties engaged formally or informally by CMS; 4) benefits and impacts considered; and 5) stakeholders, and other government agencies and organizations CMS engaged, formally or informally, in the process and how their input factored into the Agency's decision-making. The explanations of the MFP provide all stakeholders with the necessary insights into program implementation and the potential impact on patients. For this reason, we ask that CMS adhere to good governance standards and delay the IPAY27 selection process until after the explanations are made public for the previous IPAY selected drugs. The current sequence of events leaves manufacturers in the dark as they head into future negotiation cycles, hindering meaningful manufacturer engagement.

**Stakeholder Engagement**

CLS is pleased to see CMS acknowledge that there is an opportunity to improve patient engagement throughout the process for determining the MFP. CLS urges CMS to take steps to ensure this process is predictable, transparent and allows for meaningful engagement with key stakeholders, particularly the patient community. CLS strongly supports CMS's efforts to improve upon the patent-focused listening sessions that were held for IPAY2026.

We agree that an approach that allows for bidirectional engagement, allows discussion among a range of stakeholders and that allows CMS to ask clarifying questions, would be much more effective in leveraging the expertise from the patient community. CMS should also clarify the questions that they want answered, rather than leaving it vague and unclear, and allow patients and stakeholders more time to share their perspectives. CMS should also consider alternative ways to enhance dialogue between patients and the Agency, such as smaller group sessions, and find ways to engage with speakers from diverse backgrounds and perspectives. It may also be impactful to hold patient engagement events outside of those that require public speaking and use formats such as roundtables and focus groups. CLS also encourages CMS to not place arbitrary restrictions around stakeholder engagement, rather, continuously engage with relevant patients, patient representatives, or clinicians throughout its decision-making process. Finally, CMS should share—at a high level—how information from patients and stakeholders was used in determining the MFP.

**Part D Access**

The IRA made the most significant changes to Medicare Part D since its inception. CLS strongly believes that CMS should take appropriate action to proactively protect beneficiaries from anticipated harm, including worse access to medicines and more restrictive formularies. In the Draft Guidance CMS has recognized the importance of these issues but declined to take important steps to strengthen formulary standards and oversight. CLS is concerned that because of CMS' continued inaction, many seniors will likely face disruptions and barriers to accessing the medicines they need. CLS encourages CMS to clarify how it will ensure robust beneficiary access to needed therapies, including selected drugs, and institute safeguards that ensure diversity of formularies to meet patient needs.[iv] CMS should act in ways that mitigate narrower formularies and fewer choices as a result of the MFP process. CLS also encourages CMS to monitor plan coverage and tiering design, clinical appropriateness of utilization management policies, cost-sharing levels, and patient out-of-pocket exposure.

**Conclusion**

CLS remains concerned about the significant and potentially negative impacts the MDPNP will have on companies' investments in research and development, which in turn will harm beneficiary access to future treatments and cures, particularly for rare, hard-to-treat diseases and those areas with high unmet need. We continue to urge CMS to consider these impacts as the agency works to finalize this Draft Guidance based on stakeholder feedback.

CMS can mitigate harm to patients through a thoughtful and stakeholder-informed approach to implementation. We hope that CMS will consider the risks of the drug price negotiation program to patient access and future innovation. CLS welcomes any questions and further discussion on the topics above, and you can contact me at bfisk@califesciences.org.

Sincerely,

Brent Fisk

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California Life Sciences

[i] https://www.califesciences.org/california-life-sciences-sector-report/
[ii] https://www.califesciences.org/california-life-sciences-sector-report/
[iii] *Id.* at 24.
[iv] Patient Impact of the Inflation Reduction Act. https://www.manatt.com/insights/white-papers/2024/patient-impact-of-the-inflation-reduction-act June 26, 2024.