December 18, 2019

Mr. David Seltz
Executive Director
Health Policy Commission
50 Milk Street, 8th Floor
Boston, MA 02143

**Re: Proposed Regulations for 958 CMR 12.00: Drug Pricing Review**

Dear Mr. Seltz:

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to comment on the proposed regulations for 958 CMR 12.00: Drug Pricing Review, and the draft form. We are deeply concerned about the impact these regulations will have on patient access to innovative medicines, especially those that treat rare diseases.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than thirty other nations. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics yield not only improved health outcomes, but also reduced health care expenditures due to fewer physician office visits, hospitalizations, and surgical interventions.

We are deeply concerned that the Health Policy Commission (HPC) developed the proposed drug price review form without adequate input from manufacturers as the statute requires, and indeed, the proposed regulation itself would require in 958 CMR 12.04(2).[1] The statute requires that a draft form be developed "with input from manufacturers." We do not believe that there has been any meaningful attempt to gain input from manufacturers consistent with the requirements of the statute. Offering manufacturers the same opportunity as every other member of the public during a comment period does not constitute a separate and distinct solicitation from manufacturers as the statute requires. We urge the HPC to withdraw the proposed form and solicit manufacturer input in developing a new form.

In addition to the following comments on the proposed regulations and form, BIO agrees with the comments submitted by MassBIO.

[1] 958 CMR 12.04(2), Proposed Regulations, November 20, 2019.

## Transparency Disclosures

Since the Medicaid Drug Rebate Program already guarantees a state the lowest price available to all purchasers,[2] we believe the HPC's request for information on a variety of elements of a manufacturer's expenditures would be irrelevant to the determination of a supplemental rebate, and could be taken out of context. For example, not only does the regulation ask for research and development costs, but also the sources of that funding. Biopharmaceutical manufacturers, particularly small, emerging companies, must seek funding from a variety of sources to keep their business operating while research continues. Ninety-two percent of publicly traded biopharmaceutical companies operate on a net negative profit.[3] The source of that funding is irrelevant to the determination of price, and more specifically, a determination of a supplemental rebate or proposed value. Furthermore, for a determination of a "proposed value," the HPC would not be able to quantify the number of failures that a company may experience in drug trials, which also factors into future pricing. On average, it takes 10-years and $2.6 billion to bring a bio-pharmaceutical product from research and development to market.[4] This figure includes product failures. Out of thousands of compounds only one will receive approval. The overall probability that a drug or compound that enters clinical testing will be approved is estimated to be less than 12%.[5]

These risks are undertaken by the biopharmaceutical industry, not the government. The overwhelming majority of drugs have been discovered, researched and developed by innovative biopharmaceutical manufacturers. While some drugs are developed on the basis of "basic research" supported by government grants and the National Institutes of Health (NIH), the vast majority of innovative research comes from the private sector. The percent of drugs approved from 1990 to 2007 that benefited from a small amount of government research was estimated to be as low as 9.3%.[6] In 2016, the NIH research budget was approximately $30.5 billion, while the entire biopharmaceutical industry invested approximately $90 billion in research and development.[7]

The proposed rule also requires the disclosure of marketing costs for the medicine in question, as well as the aggregate marketing budgets for the manufacturer's entire portfolio. In addition, the HPC would have a manufacturer disclose production and distribution costs. Much of this information is protected proprietary information and would be protected under state and federal law. Yet, there is no area on the HPC's draft submission form that would allow manufacturers

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[2] Section 1927 of the Social Security Act

[3] *BIO FactSet*, Industry Analysis, BIO. January 2016.

[4] DiMasi, JA, et al., Innovation in the pharmaceutical industry: New estimates of R&D costs. Journal of Health Economics. February 12, 2016.

[5] Biopharmaceutical Research and Development, The Process Behind New Medicines. PhRMA, 2015. http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf

[6] Stephens, Ashley J., et al., "The Role of Public-Sector Research in the Discovery of Drugs and Vaccines," New England Journal of Medicine, February 10, 2011.

[7] Research!America, U.S. Investments in Medical and Health Research and Development, 2013-2016, Arlington, VA, Fall 2017. https://www.researchamerica.org/sites/default/files/ RA-2017_InvestmentReport.pdf

to identify proprietary or otherwise confidential information. As with other items, we believe this information is irrelevant to Medicaid pricing, as well as the negotiation of a supplemental rebate and should be deleted from the form and proposed regulations.

**Reliance on Foreign Prices**

BIO is concerned that relying on foreign prices to negotiate a supplemental rebate creates a false narrative and jeopardizes patient access to innovative medicines here in the US. Foreign prices are governed by price controls that are frequently based on the use of the discriminatory quality-adjusted life year (QALYs). The federal government has recognized that QALYs are inherently discriminatory to patients with chronic disease and disability. In its November 2019 report on QALYs, the National Council on Disability (NCD) "found sufficient evidence of QALYs being discriminatory (or potentially discriminatory) to warrant concern."[8] It called on Congress to pass legislation prohibiting the use of QALYs in Medicare and Medicaid. In addition, it encouraged CMS to use alternative measurements of value when "the exact cost and benefits of a drug or treatment are not known."[9]

The NCD report also notes that basing prices in the US on foreign prices imports a discriminatory system and jeopardizes patient care.[10] Studies have shown that countries that use QALYs have severe restrictions on patient access to innovative medicines in other countries. For example, one study has shown that between 2002 and 2014, 40% of medicines that treat rare diseases were rejected for coverage in the United Kingdom.[11]

In addition to coverage and access restrictions, price controls have been proven to result in fewer medicines being developed. The economists Joseph Golec and John Vernon estimate that, if the U.S. had adopted European-style price controls on pharmaceutical drugs from 1986 to 2004, the U.S. would have produced 117 fewer new medicine compounds for the world.[12] Similarly, economists Michael Maloney and Abdulkadir Civan estimate that a 50 percent drop in drug prices in the U.S. could see the number of drugs in the development pipeline reduced by 14-24 percent, meaning fewer cures for fewer patients.[13] The impact would be felt far greater by patients with one of the more than 7,000 rare diseases only 5% of which have FDA-approved treatment

[88] "Quality-Adjusted Life Years and the Devaluation of Life with Disability," National Council on Disability, November 6, 2019.
[9] Ibid.
[10] NCD, November 2019.
[11] Mardiguian, S., Stefanidou, M., et al. "Trends and key decision drivers for rejecting an orphan drug submission across five different HTA agencies." Value in Health Journal. 2014.
https://www.valueinhealthjournal.com/article/S1098-3015(14)03070-8/fulltext
[12] "Pharmaceutical Price Regulation: Public Perceptions, Economic Realities, and Empirical Evidence," AEI Press, January 2009.
[13] "The Effect of Price on Pharmaceutical R&D," *The B.E. Journal of Economic Analysis and Policy,* 2009.

options.[14]

**Value and Value Frameworks (Third-Party Analysis)**

BIO is concerned that the proposed rule indicates the HPC will not take into consideration patient outcomes and the extent a medicine may reduce the need for future medical care, or the inherent value a drug may have to individual patients. We believe these factors are an essential determinant of value. Other key determinants should be required factors of consideration, such as the extent the disease addresses an unmet patient need, the severity of the disease being studied, and the impact on caregivers. Thus, input from patients and caretakers must be taken into consideration when determining value.

Furthermore, BIO is deeply concerned that the HPC may use additional analyses from third parties[15] that fail to adequately assess the value a particular therapy may have to patients. Because of their increasing prominence in efforts to deliver value-based care, value frameworks can send important signals to innovators about how new medicines will be evaluated. However, reliance on flawed frameworks that do not consider value from a holistic approach could impede the development of medicines that provide meaningful benefit to patients and reduce costs across the health care system.

The Institute for Clinical and Economic Review (ICER) is one such organization that has gained prominence for its value framework, but its methodology is deeply flawed. Since its inception in 2006, many stakeholders – including many patient groups – have raised serious concerns regarding ICER's approach to their value assessment because it is overly narrow and not representative of the full set of considerations that go into determining the value of a particular medicine or course of treatment. Specifically, ICER's value framework:

- Inappropriately conflates the impact of a therapy on patient health outcomes, including quality of life, with the potential budget impact to any individual payer or group of payers;
- Fails to uniformly rely on robust and validated methodological standards, and apply them consistently and transparently; and,
- Relies heavily on the QALY – a flawed metric which cannot capture the comprehensive value an innovative therapy offers to individual patients, the healthcare system, and society.

Furthermore, ICER's lack of transparency into the creation of its value framework and the inability for external observers to independently verify model

[14] Kaufman, Petra, et al., From scientific discovery to treatments for rare diseases – the view from the National Center for Advancing Translational Sciences – Office of Rare Diseases Research, Orphanet Journal of Rare Diseases, 2018.
[15] Section 801.03:(3)(a)(7) and Section 801:03:(3)(b) of the proposed rule.

outputs have raised serious questions about exactly how ICER's model measures value. It is critical that the State fully understand the deficiencies in ICER's approach to their value assessment before utilizing their data and making determinations that could ultimately impact patient's access to needed medicines.

**Publication of "Proposed Value"**

BIO is seriously concerned that the HPC intends to publicly post the "target value" for a supplemental rebate agreement when the enabling statute does not authorize it. In fact, previous versions of the authorizing statute had language that would make public the targeted proposed value and it was stricken from the bill. If the Legislature had intended for the proposed value to be disclosed to the public, it would have explicitly stated so in the statute. Publication of the proposed value would place the manufacturer at a competitive disadvantage with its competitors knowing what discounts might be necessary to undercut the original drug in question. This would make it much more difficult for the manufacturer to negotiate with its commercial partners and there could be serious nationwide ramifications for competitive, commercial markets should a "proposed value" be disclosed to the public.

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Thank you for the opportunity to comment on the proposed regulations. Should you have any questions regarding our comments, please do not hesitate to contact me at jgeisser@bio.org or at 202-962-9200.

Sincerely,

/s/

Jack Geisser
Senior Director,
Healthcare Policy,
Medicaid, and
State Initiatives