

# REPORT OF THE COUNCIL ON MEDICAL SERVICE

CMS Report 6, November 2020

Subject: Value-Based Management of Drug Formularies  
(Resolution 814-I-19)

Presented by: Lynda M. Young, MD, Chair

Referred to: Reference Committee A

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1 At the 2019 Interim Meeting, the House of Delegates referred Resolution 814, “PBM Value-Based  
2 Framework for Formulary Design,” which was sponsored by the American Society of Clinical  
3 Oncology (ASCO). The Board of Trustees assigned this item to the Council on Medical Service for  
4 a report back to the House of Delegates at the 2020 Interim Meeting. Resolution 814-I-19 asked:

5  
6 That our American Medical Association (1) emphasize the importance of physicians’ choice of  
7 the most appropriate pharmaceutical treatment for their patients in its advocacy; and (2)  
8 advocate for pharmacy benefit managers (PBMs) and health plans to use a value-based  
9 decision-making framework that is transparent and includes applicable specialty clinical  
10 oversight when determining which specialty drugs to give preference on their formularies.

11  
12 This report provides background regarding the development, use and transparency of prescription  
13 drug formularies; outlines mechanisms for the value-based management of formularies;  
14 summarizes relevant AMA policy; and presents policy recommendations.

## 15 16 BACKGROUND

17  
18 Formularies are lists of covered drugs used by health plans and PBMs to direct increased and  
19 decreased usage of certain pharmaceuticals. Some formularies attempt to tie the level of coverage  
20 of a pharmaceutical to its “value”—its cost as well as clinical effectiveness. At the most basic level,  
21 formulary drug tiers signal which pharmaceuticals are preferred or discouraged by payers, with  
22 “preferred” drugs requiring lower patient cost-sharing levels than their counterparts. That being  
23 said, “preferred” status on a formulary is not solely influenced by a drug’s price and effectiveness.  
24 For example, drug placement on formularies is also influenced by the number of rebates and  
25 discounts PBMs can secure from pharmaceutical manufacturers.

26  
27 Within formularies, generic drugs are often promoted over their brand counterparts, and therefore  
28 typically require much lower patient cost-sharing amounts. However, the dynamic created by  
29 rebates and discounts sometimes generates exceptions to this rule. Formulary design is not only  
30 tied to patient cost-sharing levels; health plans and PBMs also leverage prior authorization, step  
31 therapy and quantity limits in conjunction with their formulary tiers to influence drug selection. For  
32 those drugs not covered by formularies, patients and their physicians must pursue a formulary  
33 exception to get some level of a drug’s cost covered, or patients have to pay the full retail price for  
34 a drug.

1 An underlying concern of referred Resolution 814-I-19 pertains to the tiering of specialty drugs in  
 2 formularies. Specialty drugs, which have the highest prices, continue to enter the market, raising  
 3 questions of how these drugs will be covered by health plans. Spending on specialty drugs is  
 4 approaching one-half of drug spending.<sup>1</sup> Responding to this financial reality, some private and  
 5 public payers have taken steps to subdivide the specialty tier of formularies into separate preferred  
 6 and non-preferred categories, which can further exacerbate the financial burden posed by specialty  
 7 drugs on patients as well as complicate physician prescribing decisions. For example, a proposed  
 8 rule released in February 2020 included a proposal to allow Medicare Part D sponsors to establish a  
 9 second, “preferred” specialty tier that would have lower cost-sharing than the current specialty tier.  
 10 The proposed rule, if finalized without changes, would also establish a cost-sharing maximum that  
 11 would be applicable to the higher-cost specialty tier. The proposed rule stipulates that if there are  
 12 two specialty tiers, one must be a “preferred” tier that has lower cost sharing than the proposed  
 13 maximum allowable specialty tier cost-sharing, defined as between 25 and 33 percent, which is  
 14 dependent upon whether a Part D plan includes a deductible.<sup>2</sup> The AMA submitted comments in  
 15 response to the proposed rule, noting that the creation of a second specialty tier may lead to  
 16 increased patient copays/cost shares for a chronic medication on which the patient is stabilized. In  
 17 addition, AMA’s comments stressed that in the case of biologic medications, switching to a  
 18 biosimilar on a lower specialty tier may have negative clinical implications for a patient stabilized  
 19 on a reference product. As such, the AMA urged the Centers for Medicare & Medicaid Services to  
 20 consider any Medicare patients currently stabilized on a specialty drug to be exempt from  
 21 unfavorable coverage changes (e.g., increased patient copays/cost shares) resulting from a  
 22 secondary specialty tier.<sup>3</sup>

23  
 24 In addition, physicians and patients continue to raise concerns pertaining to the complexity as well  
 25 as the transparency in the development and administration of formularies, prescription drug cost-  
 26 sharing requirements, and utilization management requirements. This lack of transparency makes it  
 27 exceedingly difficult for physicians to determine what treatments are preferred by a particular  
 28 payer at the point-of-care, what level of cost-sharing their patients will face, and whether  
 29 medications are subject to any prior authorization, step therapy or other utilization management  
 30 requirements. For patients, lack of formulary transparency can lead to confusion regarding their  
 31 plan’s utilization management requirements and/or their cost-sharing responsibilities, which could  
 32 result in delays in accessing necessary prescription medications, impact their ability to afford their  
 33 prescription medications, and ultimately result in treatment adherence issues. These transparency  
 34 issues are further exacerbated when formularies are changed mid-year, which can have negative  
 35 effects on patients and can have a major impact on health care costs. When PBMs choose to  
 36 remove a medication from a patient’s formulary, change its tier within the formulary, or add new  
 37 restrictions on continued prescription of that medication, sub-optimal outcomes may occur as  
 38 patients are encouraged to try new medications that may or not be as efficacious for them, or that  
 39 they have previously failed. These may result in expensive trips to the emergency room and/or  
 40 hospitalizations, increased out-of-pocket drug costs for the patient, and potentially wasted  
 41 physician and patient resources used on appeals and attempts to determine an alternative treatment  
 42 solution.

43  
 44 **VALUE-BASED MANAGEMENT OF PRESCRIPTION DRUG FORMULARIES**

45  
 46 Various public and private payers have moved forward in implementing initiatives to further  
 47 incorporate “value” in formulary development and management. However, the term “value” has  
 48 different meanings to different stakeholders. Policy H-460.909 defines value as “the best balance  
 49 between benefits and costs, and better value as improved clinical outcomes, quality, and/or patient  
 50 satisfaction per dollar spent. Improving value in the US health care system will require both  
 51 clinical and cost information.”

### *Indication-Based Formularies*

Under indication-based formulary design, health plans and PBMs can tailor on-formulary drug coverage based on specific indications. The use of indication-based formulary design constitutes a significant transition away from what has been the status quo—a drug’s coverage being the same on a formulary, regardless of the indication it is treating. While indication-based formulary design has been promoted as a way to better target drug coverage to individual patient characteristics as well as more closely tie a drug’s price to its value, indication-based formularies can make patient selection of a health plan (in Medicare Part D, for example) much more difficult. In addition, it presents new complications for physicians in making the best prescribing decisions for their patients, as drugs could be removed from formularies for indications where they are not deemed as effective. Moreover, the prescription drug formulary and benefit data currently available to physicians in their electronic health records (EHRs) is not sufficiently granular to report differential coverage based on indication, and EHRs typically do not provide sufficient information about the coverage or cost-sharing of a particular drug for a patient, including whether the patient has met his or her deductible. Physicians cannot access basic levels of information, let alone indication-based formulary data in their EHRs at the point of prescribing, which further exacerbates the existing transparency issues surrounding health plan and PBM formulary design. Of note, as of calendar year 2020, indication-based formulary design is allowed in Medicare Part D. Significantly, indication-based formulary design and utilization management are now allowed for new starts in five of the six protected classes in Medicare Part D (excluding antiretroviral medications), which permits Part D plans to exclude a protected class Part D drug for non-protected class indications.

### *Outcomes-Based Contracts*

Payers have also moved forward with initiatives that tie how much they pay for drugs to the health outcomes of patients. Under outcomes-based contracts, a PBM negotiates not only a drug’s price, but also measurable outcomes, with a pharmaceutical manufacturer on behalf of a health plan. If the drug delivers its intended outcomes for patients, the original negotiated price remains in place. However, if the drug does not meet the agreed-to outcomes in patients, the drug manufacturer would issue a rebate for part, or all, of the cost. Payers thus far have entered outcomes-based contracts with pharmaceutical companies covering medications for conditions including high cholesterol, diabetes, hepatitis C, multiple sclerosis and chronic heart failure. Outcomes-based contracts have also emerged as a mechanism to address the high costs of new gene therapies. For example, Harvard Pilgrim Health Care entered an outcome-based contract with Spark Therapeutics, the manufacturer of Luxturna, a gene therapy to treat a form of retinal dystrophy. Under the contract, the level of payment for Luxturna is tied to measured improvements in patients after a 30- to 90-day period, and then again at 30 months. If the therapy does not meet the measured outcomes agreed to, Harvard Pilgrim will receive a rebate from Spark Therapeutics.<sup>4</sup>

### *Leveraging Value-Based Frameworks in Guiding Formulary Placement*

Payers are also increasingly using analyses of entities such as the Institute for Clinical and Economic Review (ICER), not only in their drug price negotiations with pharmaceutical companies, but also in their decisions pertaining to formulary inclusions of newly launched drugs. For example, in 2018, CVS Caremark launched a program that would allow its clients to exclude any drug launched at a price of greater than \$100,000 per quality adjusted life year (QALY) from their plan. The QALY ratio used by CVS Caremark in this program originated from ICER analyses. CVS Caremark stipulated that breakthrough therapies would be excluded from this program, instead focusing on drugs for which similar effective drug therapies already exist—“me

1 too” drugs.<sup>5</sup> As of the end of 2019, this plan offering had gained little traction with CVS Caremark  
 2 clients, with patient advocacy groups raising significant concerns.<sup>6</sup>

3  
 4 The Value Assessment Framework developed by ICER includes two components: a drug’s long-  
 5 term care value and the potential short-term budget impact following a drug’s introduction to the  
 6 marketplace. ICER determines a drug’s long-term value by evaluating a drug’s comparative  
 7 clinical effectiveness, incremental cost-effectiveness, other benefits or disadvantages (e.g., methods  
 8 of administration, public health benefit) and contextual considerations (e.g., future competition in  
 9 the marketplace). ICER also develops a “health-benefit price benchmark” as part of all of its  
 10 assessments, which puts forward a price range that is in line with the added benefits of a treatment  
 11 for patients over their lifetime. Such prices align with long-term cost-effectiveness thresholds,  
 12 ranging from \$100,000 to \$150,000 per QALY gained and from \$100,000 to \$150,000 per Equal  
 13 Value of a Life Year Gained (evLYG).<sup>7</sup>

14  
 15 *American Society of Clinical Oncology*

16  
 17 ASCO, the sponsor of referred Resolution 814-I-19, released a conceptual framework in June 2015  
 18 to assess the value of cancer treatment options to be used in shared decision-making. Two versions  
 19 of the framework were developed: one for advanced cancer and one for potentially curative  
 20 treatment. ASCO then opened up the conceptual value framework to a 60-day public comment  
 21 period; more than 400 comments were received. Based on the input and feedback received, ASCO  
 22 released revised versions of the framework for advanced disease and adjuvant settings in May  
 23 2016. In both frameworks, points are awarded based on clinical benefit and toxicity, and bonus  
 24 points can also be applied. Overall, both versions of the framework use points to determine the net  
 25 health benefit, and have the net health benefit and the cost of the regimen side by side in order to  
 26 assist physicians and patients to assess value at the point-of-care.<sup>8</sup>

27  
 28 RELEVANT AMA POLICY

29  
 30 Addressing the first resolve of Resolution 814-I-19, Policy H-120.988 strongly supports the  
 31 autonomous clinical decision-making authority of a physician and that a physician may lawfully  
 32 use an US Food and Drug Administration approved drug product or medical device for an off-label  
 33 indication when such use is based upon sound scientific evidence or sound medical opinion; and  
 34 affirms the position that, when the prescription of a drug or use of a device represents safe and  
 35 effective therapy, third-party payers, including Medicare, should consider the intervention as  
 36 clinically appropriate medical care, irrespective of labeling, should fulfill their obligation to their  
 37 beneficiaries by covering such therapy, and be required to cover appropriate “off-label” uses of  
 38 drugs on their formulary.

39  
 40 Policy H-125.991 outlines standards for drug formulary systems as well as pharmacy and  
 41 therapeutics (P&T) committees. Policy H-285.965 states that P&T committee members should  
 42 include independent physician representatives, and that mechanisms should be established for  
 43 ongoing peer review of formulary policy as well as for appealing formulary exclusions. Policy  
 44 D-110.987, established by [CMS Report 5-A-19](#), supports improved transparency of PBM  
 45 operations, including disclosing P&T committee information, including records describing why a  
 46 medication is chosen for or removed in the P&T committee’s formulary, whether P&T committee  
 47 members have a financial or other conflict of interest, and decisions related to tiering, prior  
 48 authorization and step therapy; and formulary information, specifically information as to whether  
 49 certain drugs are preferred over others and patient cost-sharing responsibilities.

1 [CMS Report 5-I-16](#) established Policy H-110.986, which supports value-based pricing programs,  
2 initiatives and mechanisms for pharmaceuticals that are guided by the following principles:  
3 (a) value-based prices of pharmaceuticals should be determined by objective, independent entities;  
4 (b) value-based prices of pharmaceuticals should be evidence-based and be the result of valid and  
5 reliable inputs and data that incorporate rigorous scientific methods, including clinical trials,  
6 clinical data registries, comparative effectiveness research, and robust outcome measures that  
7 capture short- and long-term clinical outcomes; (c) processes to determine value-based prices of  
8 pharmaceuticals must be transparent, easily accessible to physicians and patients, and provide  
9 practicing physicians and researchers a central and significant role; (d) processes to determine  
10 value-based prices of pharmaceuticals should limit administrative burdens on physicians and  
11 patients; (e) processes to determine value-based prices of pharmaceuticals should incorporate  
12 affordability criteria to help assure patient affordability as well as limit system-wide budgetary  
13 impact; and (f) value-based pricing of pharmaceuticals should allow for patient variation and  
14 physician discretion.

15

## 16 DISCUSSION

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18 Long-standing AMA Policy H-120.988 strongly supports the autonomous clinical decision-making  
19 authority of a physician to determine the most appropriate pharmaceutical treatment for their  
20 patients. The policy outlines a key AMA position: When the prescription of a drug represents safe  
21 and effective therapy, third-party payers, including Medicare, should consider the intervention as  
22 clinically appropriate medical care, irrespective of labeling, and should fulfill their obligation to  
23 their beneficiaries by covering such therapy. The Council believes that the AMA has historically  
24 advocated strongly for its members and the nation's patients in this regard and calls for the  
25 reaffirmation of Policy H-120.988 to highlight both the policy and ongoing advocacy of the AMA.

26

27 Overall, PBMs and health plans must use a transparent process in formulary development and  
28 administration and include practicing network physicians from the appropriate medical specialty  
29 when making determinations regarding formulary inclusion or placement for a particular drug  
30 class. This builds upon the intent of Policy H-285.965, a policy that also stresses the importance of  
31 there being a mechanism to appeal formulary exclusions, providing another avenue for patients to  
32 receive the pharmaceutical treatments they need. Overall, physicians and patients need to have  
33 access to information relating to how pharmaceuticals are included and/or tiered in formularies, as  
34 called for in Policy D-110.987.

35

36 As payers continue to move forward in implementing initiatives to further incorporate "value" in  
37 formulary development and management, the Council strongly believes there is a need to closely  
38 examine these initiatives, to ensure they are in the best interests of patients. Existing Policy  
39 H-110.986 took key steps in that direction, but more needs to be done. First, in the event that  
40 payers/PBMs enter into an outcomes-based contract with a pharmaceutical manufacturer, and the  
41 terms of the contract yield savings to the payer, such savings should be shared with impacted  
42 patients. If payers benefit from outcomes-based contracts, so should the patients for whom the  
43 pharmaceutical is meant to help. To facilitate the sharing of savings from such refunds and rebates,  
44 it is essential that rebate and discount information be made transparent, as called for in Policy  
45 D-110.987.

46

47 The Council has significant concerns with the increasing use of indication-based formularies. On  
48 the patient side of the equation, indication-based formularies can make patient selection of a health  
49 plan (in Medicare Part D, for example) much more difficult, as patients would not only have to  
50 search for a particular drug, but also confirm that the drug is covered for their particular indication.

1 And, for newly diagnosed patients already enrolled in a health plan, the drug that may be best to  
2 treat their condition may not be covered for their specific indication.

3  
4 For physicians, indication-based formularies introduce new complications along the chain from a  
5 patient's office visit, to a pharmaceutical being dispensed at a pharmacy. Patients' drug coverage is  
6 already dependent on and varies according to each individual health plan. Indication-based  
7 formularies have the potential to build upon the existing complexity and exacerbate the existing  
8 transparency issues surrounding PBM formulary design, as physicians cannot access indication-  
9 based formulary data in their EHRs at the point of prescribing. Ultimately, there will be even more  
10 variations within and between health plans regarding whether a drug is covered. In addition, drugs  
11 could potentially be removed from formularies for indications where they are not deemed as  
12 effective. Indication-based formularies could also introduce new administrative burdens for  
13 physicians. For example, coverage restrictions will likely not be discovered until after the  
14 prescription claim is submitted by the pharmacy and denied by the PBM, which will request the  
15 applicable diagnosis code. The pharmacy will need to contact the physician practice for this  
16 additional information, and under the best-case scenario, the claim will be resubmitted and paid by  
17 the PBM. However, if the PBM does not cover the drug for the reported indication, the pharmacy  
18 will contact the physician again and request that an alternate therapy be prescribed. This  
19 "prescription rework" and multiple workflow disruptions will further increase physicians' already  
20 significant challenges in navigating patients' prescription drug benefits. As such, the Council  
21 recommends that indication-based formularies be opposed, in order to protect the ability of patients  
22 to access and afford the prescription drugs they need, and physicians to make the best prescribing  
23 decisions for their patients.

#### 24 25 RECOMMENDATIONS

26  
27 The Council on Medical Service recommends that the following be adopted in lieu of Resolution  
28 814-I-19, and that the remainder of the report be filed.

- 29  
30 1. That our American Medical Association (AMA) reaffirm Policy H-120.988, upholding the  
31 ability of patients to access treatments prescribed by their physicians. (Reaffirm HOD Policy)
- 32  
33 2. That our AMA reaffirm Policy H-285.965, which states that pharmacy and therapeutics (P&T)  
34 committee members should include independent physician representatives, and that  
35 mechanisms should be established for ongoing peer review of formulary policy as well as for  
36 appealing formulary exclusions. (Reaffirm HOD Policy)
- 37  
38 3. That our AMA advocate that pharmacy benefit managers (PBMs) and health plans use a  
39 transparent process in formulary development and administration, and include practicing  
40 network physicians from the appropriate medical specialty when making determinations  
41 regarding formulary inclusion or placement for a particular drug class. (New HOD Policy)
- 42  
43 4. That our AMA reaffirm Policy D-110.987, which supports improved transparency of PBM  
44 operations, including disclosing rebate and discount information as well as P&T committee  
45 information, including records describing why a medication is chosen for or removed in the  
46 P&T committee's formulary, whether P&T committee members have a financial or other  
47 conflict of interest, and decisions related to tiering, prior authorization and step therapy; and  
48 formulary information, specifically information as to whether certain drugs are preferred over  
49 others and patient cost-sharing responsibilities. (Reaffirm HOD Policy)

- 1 5. That our AMA reaffirm Policy H-110.986, which outlines principles guiding AMA’s support  
2 for value-based pricing programs, initiatives and mechanisms for pharmaceuticals. (Reaffirm  
3 HOD Policy)  
4
- 5 6. That our AMA advocate that any refunds or rebates received by a health plan or PBM from a  
6 pharmaceutical manufacturer under an outcomes-based contract be shared with impacted  
7 patients. (New HOD Policy)  
8
- 9 7. That our AMA oppose indication-based formularies in order to protect the ability of patients to  
10 access and afford the prescription drugs they need, and physicians to make the best prescribing  
11 decisions for their patients. (New HOD Policy)

Fiscal Note: Less than \$500.

## REFERENCES

<sup>1</sup> IQVIA Institute for Human Data Science. Medicine Use and Spending in the U.S.: A Review of 2018 and Outlook to 2023. May 2019. Available at: <https://www.iqvia.com/insights/the-iqvia-institute/reports/medicine-use-and-spending-in-the-us-a-review-of-2018-and-outlook-to-2023>.

<sup>2</sup> Centers for Medicare & Medicaid Services. Proposed Rule: Medicare and Medicaid Programs; Contract Year 2021 and 2022 Policy and Technical Changes to the Medicare Advantage Program, Medicare Prescription Drug Benefit Program, Medicaid Program, Medicare Cost Plan Program, and Programs of All-Inclusive Care for the Elderly (CMS-4190-P). February 18, 2020. Available at: <https://www.federalregister.gov/documents/2020/02/18/2020-02085/medicare-and-medicaid-programs-contract-year-2021-and-2022-policy-and-technical-changes-to-the>.

<sup>3</sup> American Medical Association. Comments to the Centers for Medicare & Medicaid Services (CMS) on the Medicare and Medicaid Programs; Contract Year 2021 and 2022 Policy and Technical Changes to the Medicare Advantage Program, Medicare Prescription Drug Benefit Program, Medicaid Program, Medicare Cost Plan Program, and Programs of All-Inclusive Care for the Elderly Proposed Rule (Proposed Rule). April 6, 2020. Available at: <https://searchf.ama-assn.org/undefined/documentDownload?uri=%2Funstructured%2Fbinary%2Fletter%2FLETTERS%2F2020-4-6-Letter-to-Verma-re-MA-and-Part-D-NPRM.pdf>.

<sup>4</sup> Harvard Pilgrim Health Care. Harvard Pilgrim Is First Plan to Directly Negotiate Outcomes-Based Agreement for Groundbreaking Gene Therapy. January 3, 2018. Available at: [https://www.harvardpilgrim.org/public/news-detail?nt=HPH\\_News\\_C&nid=1471914707173](https://www.harvardpilgrim.org/public/news-detail?nt=HPH_News_C&nid=1471914707173).

<sup>5</sup> CVS Health. Current and New Approaches to Making Drugs More Affordable. August 2018. Available at: <https://cvshealth.com/sites/default/files/cvs-health-current-and-new-approaches-to-making-drugs-more-affordable.pdf>.

<sup>6</sup> Humer, C. CVS Drug Coverage Plan Based on Outside Pricing Review Is off to a Slow Start. Reuters. October 3, 2019. Available at: <https://www.reuters.com/article/us-cvs-health-drugpricing-focus/cvs-drug-coverage-plan-based-on-outside-pricing-review-is-off-to-a-slow-start-idUSKBN1W12IO>.

<sup>7</sup> Institute for Clinical and Economic Review. 2020-2023 Value Assessment Framework. January 31, 2020. Available at: [https://icer-review.org/wp-content/uploads/2019/05/ICER\\_2020\\_2023\\_VAF\\_013120-4.pdf](https://icer-review.org/wp-content/uploads/2019/05/ICER_2020_2023_VAF_013120-4.pdf).

<sup>8</sup> American Society of Clinical Oncology. Value in Cancer Care. Available at: <https://www.asco.org/practice-policy/cancer-care-initiatives/value-cancer-care>.