REPORT OF THE BOARD OF TRUSTEES

B of T Report 14-A-19

Subject:	Reforming the Orphan Drug Act (Resolution 217-A-18) An Optional National Prescription Drug Formulary (Resolution 227-A-18) Reform of Pharmaceutical Pricing: Negotiated Payment Schedules (Resolution 238-A-18)
Presented by:	Jack Resneck, Jr., MD, Chair
Referred to:	Reference Committee B (Charles Rothberg, MD, Chair)

At the 2018 Annual Meeting, the American Medical Association's (AMA) House of Delegates (HOD) referred three resolutions for a combined Board of Trustees (BOT) Report (Report) at the 2019 Annual Meeting. The first resolution, Resolution 217-A-18, "Reforming the Orphan Drug Act," was introduced by the Medical Student Section and asks that:

> Our AMA: (1) support efforts to reform the Orphan Drug Act (ODA) by closing loopholes identified by the Food and Drug Administration [(FDA)]in order to protect the Act's original intent of promoting therapies targeting rare diseases; (2) support increased transparency in development costs, post-approval regulation and overall earnings for pharmaceuticals designated as "Orphan Drugs" and (3)support modifications to the exclusivity period of "Orphan Drugs" to increase access to these pharmaceutical drugs for patients with rare diseases.

The second resolution, Resolution 227-A-18, "An Optional National Prescription Drug Formulary," was introduced by the Florida Delegation and asks that:

> Our AMA: (1) develop a set of principles for a National Prescription Drug Formulary (NPD Formulary) that are designed to lower prescription drug prices to the patient, and be transparent, independent, non-profit, and fee-based, with a report back to the AMA HOD at the 2018 Interim Meeting; (2) produce model legislation for an NPD Formulary with input from appropriate stakeholders based on a set of principles for such a Formulary that the AMA will develop; and (3) that our AMA join with appropriate stakeholders to advocate that Congress authorize the establishment of this NPD Formulary that will be available to all Americans as an option to their healthcare insurance program in an actuarially appropriate manner.

The third resolution, Resolution 238-A-18, "Reform of Pharmaceutical Pricing: Negotiated Payment Schedules," was introduced by the Illinois Delegation and asks that:

> Our AMA: (1) support federal legislation that modifies the Hatch-Waxman Act and the Biologics Price Competition and Innovation Act (Biosimilars Act) to institute the replacement of time-specific patent protections with negotiated payment schedules and indefinite

exclusivity for U.S. Food and Drug Administration-approved drugs in the Medicare Part D Program.

The reference committee heard varying testimony on Resolutions 217, 227, and 238. There was testimony providing strong support for the current strategic focus of AMA advocacy and initiatives to increase market competition as well as increased transparency of cost and price along the pharmaceutical supply chain. There was testimony in response to Resolution 217 noting that incentives are needed to support innovation in drug development for rare diseases and general support for the intent of the ODA, but there was concern that manufacturers are manipulating ODA exclusivities and may be driving higher drug costs to vulnerable patient populations. The reference committee heard testimony on Resolution 227 that a new national not-for-profit pharmaceutical benefit manager (which is referred to in the resolution as a national formulary) would not necessarily promote innovation and competition and could substantially limit patient access to medically necessary options. The reference committee heard testimony on Resolution 238 that it did not accurately identify the federal laws that would have to be amended in order to institute the replacement of time-specific patent protections with negotiated payment schedules and indefinite exclusivity for FDA-approved drugs in the Medicare Part D benefit prescription drug program. Testimony was offered noting it would require marked changes to the U.S. Patent Act, the U.S. Food, Drug, and Cosmetic Act (FDCA), and the Social Security Act (SSA). Furthermore, testimony was offered that such changes could limit patient access to clinically necessary alternative options and depress innovation while interjecting significant confusion and complexity in the patent system and the FDA regulatory regime. The reference committee found that all three resolutions are either a potentially complex solution to address the high cost of prescription drugs, or too narrowly crafted. Given these concerns, the reference committee recommended referral for a consolidated report.

AMA STRATEGIC FOCUS: INCREASING TRANSPARENCY AND COMPETITION

The varied contributing causes fueling the rise in prescription medication prices and the proliferation in barriers faced by patients who need medically necessary medication have resulted in the HOD adopting a wide-range of policies concerning prescription medication affordability and access. In order to prioritize impactful and viable policies that would enable the AMA to effectively advocate at the federal and state levels, Policy H-110.987, “Pharmaceutical Costs,” adopted in 2015 directed the AMA to convene a task force of appropriate AMA Councils, state medical societies, and national medical specialty societies to develop principles to guide advocacy and grassroots efforts aimed at addressing pharmaceutical costs and improving patient access and adherence to medically necessary prescription drug regimens. Accordingly, the AMA convened a Task Force on Pharmaceutical Costs, which met four times in the first six months of 2016 to develop principles to guide advocacy and grassroots efforts aimed at addressing pharmaceutical costs. The Task Force agreed that increasing transparency among pharmaceutical companies, health plans, and pharmacy benefit managers (PBMs) should be the initial focus of the campaign, which led to the launch of a grassroots campaign in the third quarter of 2016, and the launch of the TruthinRx website, TruthinRx.org, on November 1, 2016. The foregoing was done in concert with the AMA’s long-standing advocacy to increase competition. Based on the foregoing the AMA has vigorously supported the focus of policymakers at the federal and state levels to address pharmaceutical supply chain transparency and accelerated and expanded legislative and regulatory action to increase pharmaceutical market competition by, among other things, combating anti-competitive practices.

Increase Pharmaceutical Market Competition and Combat Anticompetitive Practices

Policymakers have increased scrutiny of laws enacted to ensure drug safety and efficacy and to promote innovation that have been manipulated by pharmaceutical manufacturers to delay or block competition. Building off policy raising concerns with anti-competitive practices, the AMA has focused on increasing the authorities and resources of the Federal Trade Commission (FTC) to combat anti-competitive actions of manufacturers as well as changes to the FDA's oversight of the FDCA provisions that have been misused by manufacturers to delay the entry of more affordable generics as outlined below. In addition, the AMA has urged changes to the U.S. Patent Act that are inviting misuse for anti-competitive reasons by manufacturers.

Consistent with long-standing advocacy, the AMA continues to support the FTC's actions to stop pay-for-delay settlements, whereby a brand-name pharmaceutical manufacturer pays a potential generic competitor to abandon its challenge and delay offering a generic drug product for a number of years, for anti-competitive purposes. The AMA is also urging the FTC and Congress to evaluate certain uses of U.S. Patent Act and market exclusivities conferred under the FDCA by pharmaceutical companies that appear primarily designed to increase litigation costs for generic manufacturers and delay market competition. The AMA is also urging more rigorous FTC evaluation of mergers and consolidations among pharmaceutical companies and their impact on competition as well as consumer access by, among other things, expanding clinical expertise within the FTC and consulting with the relevant national medical specialty societies. The AMA is also expressing strong support of enforcement action referrals by the FTC against manufacturers that engage in anticompetitive actions to the U.S. Department of Justice.

In addition, the AMA continues to support measures to address the misuse of FDCA provisions for anti-competitive purposes. The AMA continues to urge Congress and federal agencies to take action to: (1) end the ability of generic manufacturers to indefinitely "park" the 180-day exclusivity period authorized by the FDCA by delaying final approval of their application by the FDA as part of a settlement agreement with a brand manufacturer; (2) further expand the ability of the FDA to address anticompetitive abuse of risk evaluation and mitigation strategies by brand manufacturers—particularly voluntary elements to assure safe use that involve proprietary measures that pose barriers to use by generic competitors; (3) make necessary amendments to the U.S. Patent Act and the FDCA to prevent the inappropriate extension of the exclusivity and patent life of pharmaceuticals. The AMA also strongly supports passage of legislation to increase competition and thus access to some of the most-costly prescription medications: biologicals. The AMA supported the original legislation establishing the follow-on biological pathway and it is now evident that there is a need to shorten the exclusivity period for biological products in order to spur competition which will not decrease the impetus to innovate.

Require Pharmaceutical Supply Chain Transparency

The second component of AMA advocacy has been to encourage transparency throughout the pharmaceutical supply chain. The ability of patients and physicians to have the information they need to make key decisions regarding medication, and of policymakers to craft viable solutions to high and escalating pharmaceutical costs, has been hampered by the often byzantine and confidential arrangements that are driving increased medication prices without a clear and justifiable reason. The practices and policies of pharmaceutical manufacturers, pharmacy benefit managers (PBMs), and health insurers warrant steps by Congress to interject much needed transparency. To that end the AMA strongly supports: (1) requiring pharmaceutical manufacturers to provide public notice before increasing the price of any drug by 10 percent or more each year or per course of treatment and provide justification for the price increase; (2) requiring pharmaceutical

manufacturers to publicly disclose a variety of information, which could include research and development costs, expenditures on clinical trials, total costs incurred in production, and marketing and advertising costs; (3) requiring PBMs to apply manufacturer rebates and pharmacy price concessions to drug prices at the point-of-sale to ensure that patients benefit from discounts as well as eliminate some incentives for higher drug list prices; (4) requiring insurers to provide increased transparency in formularies, prescription drug cost-sharing, and utilization management requirements for patients and physicians at the point-of-prescribing as well as when beneficiaries make annual enrollment elections; and (5) prohibiting removal of drugs from a formulary or moving to a higher cost tier during the duration of the patient's plan year unless a change is made for safety reasons.

AMA POLICY

The AMA has extensive policy relevant to the issues raised in all three resolutions. In general, the AMA opposes the use of price controls in any segment of the health care industry, and continues to promote market-based strategies to achieve access to and affordability of health care goods and services (Policy H-155.962, "Maximum Allowable Cost of Prescription Medications").[1] The AMA has adopted comprehensive policy to address anti-competitive measures by manufacturers and to promote increased cost and price transparency (Policy H-110-987, "Pharmaceutical Costs"). AMA policy provides support for action by federal agencies to address manufacturer price gouging. AMA policy also outlines support for the FTC in its efforts to stop "pay for delay" arrangements by pharmaceutical companies and federal legislation to expand the FTC's existing authorities to stop such arrangements (Policy H-110.989, "Pay for Delay Arrangement by Pharmaceutical Companies"). The AMA also supports FDA implementation of the biosimilar pathway established under the Biologics Price Competition and Innovation Act of 2009 in order to ensure patient access, protect patient safety, and preserve market competition and innovation (Policy H-125.980, "Abbreviated Pathway for Biosimilar Approval").

In support of driving increased competition, AMA policy provides for ongoing evaluation of strategies by manufacturers to extend the patent life of pharmaceuticals, and to work with Congress and the Administration where such actions are pursued for anti-competitive purposes (Policy D-110.994, "Inappropriate Extension of Patent Life of Pharmaceuticals"). The AMA also continues to advocate that the FDA and Congress ascertain the pervasiveness of brand manufacturers forcing switching from an established drug formulation about to lose market exclusivity and patent protection to another formulation that retains such protections. This practice is called evergreening and AMA policy provides that a balance must be struck between incentivizing innovation (superior formulations) versus anti-competitive practices designed to slow generic competition (Policy H-125.978, "Patient Protection from Forced Switching of Patent-Protected Drugs"). AMA policy also provides that physicians who develop medical innovations may ethically patent their discoveries or products but should uphold the following guidelines: (a) Not use patents (or other means, such as trade secrets or confidentiality agreements) to limit the availability of medical innovations and patent protection should not hinder the goal of achieving better medical treatments and technologies; and (b) Not allow patents to languish and physicians who hold patents should negotiate and structure licensing agreements in such a way as to encourage the development of better medical technology (Policy H-110.988, "7.2.3 Patents & Dissemination of Research Products").

The AMA supports collaboration with federal and state agencies, policymakers and key stakeholders (e.g., the FTC, FDA, and the Generic Pharmaceutical Association) to identify and promote adoption of policies to address the already high and escalating costs of generic prescription drugs (Policy H-110.988, "Controlling the Skyrocketing Costs of Generic Prescription

Drugs"). The same policy provides that the AMA will also seek to advance with interested parties legislation to ensure fair and appropriate pricing of generic medications. The policy also provides that the AMA encourages the development of methods that increase choice and competition in the development and pricing of generic prescription drugs and the AMA supports measures that increase price transparency for generic prescription drugs.

The AMA has policy to support programs that are designed to contain the rising costs of prescription drugs, provided that physicians have significant input into the development and maintenance of such programs and such programs must encourage optimum prescribing practices and quality of care (Policy H-110.997, "Cost of Prescription Drugs"). Furthermore, under this AMA policy all patients must have access to all prescription drugs necessary to treat their illnesses and physicians must have the freedom to prescribe the most appropriate drug(s) and method of delivery for the individual patient; and the freedom to use either generic or brand name pharmaceuticals in prescribing drugs for their patients. In addition, AMA policy provides support for consumer choice of at least two options for their pharmaceutical benefits program. This must include a fee-for-service option where restrictions on patient access and physician autonomy to prescribe any FDA-approved medication are prohibited and reaffirms support for physicians to use either generic or brand name pharmaceuticals in prescribing drugs for their patients. Finally, the AMA policy provides support for a managed pharmaceutical benefit option with market-driven mechanisms to control costs, provided cost control strategies satisfy AMA policies and standards defined in AMA Policy H-125.991 (Policy H-100.964, "Drug Issues in Health System Reform").

The AMA also has a growing body of policy concerning PBMs given growing concerns with their role on patient costs. Policy adopted last year provides that the AMA will gather more data on the erosion of physician-led medication therapy management in order to assess the impact PBM tactics may have on patients' timely access to medications, patient outcomes, and the physician-patient relationship (Policy D-120.933, "Pharmacy Benefit Managers Impact on Patients"). In addition, the same AMA policy provides for an examination of PBM-related clawbacks and direct and indirect remuneration (DIR) fees to better inform existing advocacy efforts. AMA policy further provides that physicians should report to the FDA MedWatch reporting program any instances of adverse consequences (including therapeutic failures and adverse drug reactions) that have resulted from the switching of therapeutic alternates precipitated by PBM actions (Policy H-125.986, "Pharmaceutical Benefits Management Companies"). The policy provides support for increased oversight by the FTC to assess the relationships between pharmaceutical manufacturers and PBMs, especially with regard to manufacturers' influences on PBM drug formularies and drug product switching programs, and to take enforcement actions as appropriate where there are indicia of anti-trust and anti-competitive practices. Further, AMA policy provides that certain actions/activities by PBMs and others constitute the practice of medicine without a license and interfere with appropriate medical care to patients. The policy also outlines support for effort to ensure that reimbursement policies established by PBMs are based on medical need; these policies include, but are not limited to, prior authorization, formularies, and tiers for compounded medication.

DISCUSSION

The AMA is engaged in a comprehensive advocacy campaign at the state and federal level to advance legislation and agency action to increase patient access to affordable prescription medication by increasing market competition and increasing price and cost transparency along the pharmaceutical supply chain. Two of the resolutions and associated resolves would materially depart from this strategy and existing policy. The two resolutions are Resolution 227-A-18, which would involve a major initiative to advance the creation of a not-for-profit PBM fashioned as a national formulary, and Resolution 238-A-18, which would require substantial changes to the

U.S. Patent Act, the FDCA (to alter FDA conferred market exclusivities) and the Social Security Act (to alter relevant Medicare Part D drug benefit provisions). In the case of Resolution 227-A-18, the lack of transparency among the existing commercial PBMs hampers any effort to assess the true value of PBMs in driving affordable pricing and there are widespread concerns, as demonstrated by AMA policies summarized above, that PBM practices have negatively impacted medical practice and patient access to the most appropriate treatment options.

Continued efforts to increase transparency are gaining support from the Trump Administration and Congress. Diverting current AMA efforts to shine a light on PBM practices in order to instead advocate for the creation of a not-for-profit version would be hindered by a lack of information on the measures and mechanisms used by PBMs. Similarly, adoption of Resolution 238-A-18 would represent support for government-imposed price controls in the Medicare program and involve massive disruptions to established patent law and alterations to FDCA conferred exclusivities without addressing drug prices in the commercial market as the resolve calls for government negotiated prices for Medicare Part D drugs, but makes no mention of the commercial market. It would be expected many brand manufacturers would increase prices in the commercial market to offset lower payments in the Medicare program. This would be successful as under this proposed policy, brand manufacturers would not have generic competition as they would receive "indefinite" FDCA exclusivities per the resolve. Perversely, if adopted as policy Resolution 238-A-18 would drive rapid escalation of drug prices in all commercial markets.

Finally, for the most part, AMA policy already addresses Resolution 217-A-18. There are legitimate concerns that the ODA exclusivities[2] have been misused by manufacturers.[3] In November 2018, the Government Accountability Office (GAO) issued a report, Orphan Drugs: FDA Could Improve Designation Review Consistency; Rare Disease Drug Development Challenges Continue. The GAO found that FDA reviewers evaluating a manufacturer's application seeking orphan drug status were not consistently recording or evaluating the required background information needed to assess the appropriateness of the designation. For example, 48 of 148 cases reviewed by the GAO were missing information on the drug's U.S. marketing history. The GAO concluded that the FDA could not be sure that reviewers are conducting complete evaluations that include all critical information needed for assessing its criteria. The FDA has indicated that steps will be taken to ensure such information is included and evaluated. While such steps are meaningful, reportedly, by 2024, orphan drugs are projected to capture a fifth of worldwide prescription drug sales ($262 billion) and the compound annual growth rate is forecasted to grow by 11.3 percent, which is double the rate forecast for the non-orphan drug market. Thus, continued scrutiny is warranted of how ODA exclusivities are conferred and careful consideration to the impact on market competition will remain essential.

RECOMMENDATIONS

In light of these considerations, your Board of Trustees recommends that the following be adopted in lieu of Resolutions 217-A-18, 227-A-18, and 238-A-18, and the remainder of this report be filed.

1. That our AMA reaffirm Policy H-110.987, "Pharmaceutical Costs," which outlines a series of measures to address anti-competitive actions by pharmaceutical manufacturers as well as policies to promote increased transparency along the pharmaceutical supply chain including among PBMs. (Reaffirm HOD Policy)

2. That our AMA support legislation to shorten the exclusivity period for FDA pharmaceutical products where manufacturers engage in anti-competitive behaviors or unwarranted price escalations. (New HOD Policy)

Fiscal Note: Less than $500

NOTES

[1] While the AMA has policy that provides support for federal legislation which would confer the Secretary of the Department of Health and Human Services (HHS) with the authority to negotiate contracts with manufacturers for covered Medicare Part D prescription drugs, and provides that the AMA will work toward eliminating Medicare prohibition on drug price negotiation (Policy D-330.954), the taskforce prioritized strategies to increase transparency and to combat the pervasive anti-competitive practices by pharmaceutical manufacturers that are blocking or delaying lower cost, affordable alternative options.

[2] An orphan drug is a prescription medication that treats a rare condition or disease affecting fewer than 200,000 nationwide. The development of orphan drugs has been financially incentivized by the market exclusivities provided under FDCA as amended by the ODA as well as tax credits on research and development, grants for phase I and II clinical trials, and, in some cases, waiver of FDA user fees.
[3] In 2017, it was reported that 70, out of 450, prescription medications with orphan drug status were first approved by the FDA for mass-market use. Early in 2017, Senators Orrin Hatch (R-UT), Charles Grassley (R-IA) and Tom Cotton (R-AR) requested that the U.S. Government Accountability Office (GAO) evaluate the performance of the FDA's Office of Orphan Products Development (OOPD) and to identify "any regulatory or legislative changes may be needed in order to preserve the intent of this vital law." Later in 2017, the new FDA Commissioner urged Congress to implement two new ODA requirements in order to curb abuses of the ODA. Tribble S.J., Lupkin S., Drugmakers Manipulate Orphan Drug Rule to Create Prized Monopolies, Kaiser Health New, January 17, 2017.