TESTIMONY

BEFORE THE

COMMITTEE ON HEALTH, EDUCATION, LABOR AND PENSIONS
UNITED STATES SENATE

BY
NORMAN R. AUGUSTINE

ON BEHALF OF

THE NATIONAL ACADEMIES OF SCIENCES, ENGINEERING AND MEDICINE
COMMITTEE THAT PREPARED THE REPORT

"MAKING MEDICINES AFFORDABLE—A NATIONAL IMPERATIVE"

WASHINGTON, DC

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Chairman Alexander, Ranking Member Murray and members of the Committee, thank you for this opportunity to share with you the results of the National Academies of Science, Engineering and Medicine study on the affordability and availability of prescription biopharmaceuticals. The National Academies of Sciences, Engineering, and Medicine provide independent, objective analysis and advice to the nation and conduct other activities to solve complex problems and inform public policy decisions.

I appear today in my capacity as chair of the committee that performed the study and I will therefore be presenting materials contained in our report. The report is an evidence-based consensus document in which all of the eight recommendations and twenty-seven implementing actions contained therein enjoy the support of a substantial majority of the committee members, while some enjoy unanimous support. Two of our colleagues, while agreeing with some of the recommendations, have prepared a minority dissenting view which expresses the concern that the recommendations taken in totality would prove excessive and thus damaging to the nation's health care and biopharmaceutical system in particular. Seven other colleagues have expressed full support of all of the recommendations and findings but believe further actions are warranted, particularly in the areas of pricing, transparency and value assessment. The recommendations and implementing actions contained in the report thus represent the views of a strong consensus of the committee's members.

Our committee was composed of individuals with highly diverse professional backgrounds in such fields as federal and state government, pharmaceutical manufacturing, the practice of medicine, health policy, consumer engagement, research and development, economics, law, public health and business management. During our year-long deliberations, the committee received presentations from 39 individuals either representing themselves or specific organizations, received inputs from members of the public, reviewed several thousand pages of documents, and benefitted from written submittals provided by various individuals and organizations. The committee's draft report was subjected to in-depth review by 16 anonymous reviewers and two overseers chosen by the National Academies and the committee provided specific responses as to the disposition of each of the reviewers' comments.

Notwithstanding the broad range of perspectives of our members, we sought to find common ground on which to base recommendations that would serve today's patients by reducing the cost of biopharmaceuticals while enabling a vigorous program to develop new drugs to serve future patients. The result of this effort is contained in our report "Making Medicine Affordable—A National Imperative," a report we collectively hope can assist the nation in resolving what is currently an unacceptable circumstance.

As our presence here today attests, making medicines affordable has emerged as a national priority. The cost of biopharmaceuticals now represents 17 percent of the total cost of healthcare in America. Further, the rate of growth in the cost of biopharmaceuticals significantly exceeds the rate of inflation in the economy, the rate of growth of family income and the cost of healthcare as a whole. A recent survey of adult

Americans' priorities for the U.S. Congress through the end of this year placed reducing prescription drug prices as highest ranked; above raising the minimum wage, lowering the deficit, rebuilding the nation's infrastructure, and reducing taxes.

The amount of money Americans spend on health care as a whole now equals 18 percent of the nation's gross domestic product. This number has increased steadily for the past 60 years, leading to what today is the highest per capita expenditure on health care in the world. Further, the trend of increasing spending, including on biopharmaceuticals, is projected to continue for the foreseeable future as the Baby Boomer generation ages.

The nation with health care spending that most closely approaches that of the United States allocates about 7 percentage points less of its gross domestic product to this purpose. For perspective, that *difference*, 7 percent of the United States gross domestic product, would fund America's primary and secondary education system or two of its defense budgets or three of its public transportation and highway budgets.

While it is clearly in the public interest to devote significant resources to health care, such spending is not without its opportunity costs.

Annual expenditures on biopharmaceuticals in the United States now exceed a half trillion dollars. As the cost of drugs has escalated in recent years, insurance plans have implemented benefit designs that attempted to preserve access to care yet keep health insurance premiums affordable by adjusting formularies and increasing copayments and deductibles—each of which impacts patient cost. Deductibles themselves have, on average, increased by a factor of 2.5 in the past decade.

Yet, while few argue that the current situation is acceptable, virtually each newly proposed potential corrective measure has confronted strong opposition from one or more quarters.

This is in part because an overarching moral issue remains unresolved in the United States: is access to health care—including prescription drugs—a fundamental human right? And if it is not, who is to decide, and based on what criteria, which individuals are to be denied access to the drugs and the care that they need? But if health care *is* a right, who is to pay its costs? And is this cost affordable not only to the individual but also to society as a whole, and does it represent the most appropriate allocation of the nation's resources?

The burden of high-priced drugs often falls disproportionately on vulnerable elements of the population in spite of government, industry and charitable efforts to alleviate its impact. For example, the Kaiser Family Foundation reports that in 2015, about 20 percent of Americans did not fill at least one prescription due to affordability considerations, while others rationed the drugs that they did acquire. Two-thirds of personal bankruptcies in the United States have been attributed in part or entirely to the overall cost of medical care, including drugs.

Public concern regarding the cost of biopharmaceuticals has been accentuated in recent years by sudden unexplained increases in the price of various existing drugs. For example, media reports cited the unanticipated increase in the price of a two-pack of EpiPens (used to administer epinephrine, a treatment for potentially fatal allergic reactions) from $160 to more than $600. Perhaps the most egregious case involved rights to the existing, non-patent-protected drug Daraprim (used in the treatment of severe infections) with a relatively small market that makes it unattractive to potential competitors. The rights to Daraprim were purchased from its developer by Turing Pharmaceuticals, which raised the drug's price from $13.50 to $750 per tablet.

An effective biopharmaceutical enterprise, the source of a long history of life-enhancing and life-saving accomplishments, is critically important to the nation's well-being. Without past contributions of this sector, supported by research funded by various agencies of the federal government, universities, private philanthropy, venture capital, and biopharmaceutical firms themselves, there would have been no vaccines for many deadly diseases, no statins, and no cure for conditions such as hepatitis C. Almost certainly, some of us in this room would not be here today were it not for the past accomplishments of America's biopharmaceutical enterprise.

Yet, rising prices today threaten to make the products of that enterprise unaffordable to patients, and potentially even to society as a whole.

In the case of most business sectors in the United States, the pressure of competition is the dominant force controlling prices and, to the extent that competition is present, the biopharmaceutical industry is no exception. Nonetheless, if firms that have invested heavily to introduce new products were to be immediately confronted with competitors not having made such investments, there would be little motivation or justification for conducting research and innovating.

In recognition of the importance of encouraging innovation, the U.S. Constitution provided Congress with the authority "to promote the progress of science and useful arts, by securing for limited times to authors and inventors the exclusive right to their respective writings and discoveries." That is, in exchange for undertaking research and development to introduce new products, the government can, and does, grant patents to firms and individuals, thereby conferring on them for a specified period of time what are in effect sole-source positions in the market.

When the period of patent exclusivity for a drug expires, companies other than the developer are free to introduce "copies"—known as generics or biosimilars—into the market. These latter products represent 89 percent of all prescriptions written and 24 percent of the total cost of all prescription drugs. When generics enter the market, experience shows that the price of the original patented product frequently drops precipitously as the developer seeks to compete with the new, lower-cost entrants or else forfeits some or all of the market. As but one example, the price of Lipitor, the widely used anti-cholesterol drug, dropped from $3.29 per unit to 11 cents per unit when its patent protection expired.

Market forces that promote innovation, while also providing price controlling pressures, have worked quite effectively in most United States industrial settings, raising the question why they appear to be far less effective in the prescription biopharmaceutical arena. The answer resides in the fact that this particular market has important features that distinguish it from most other markets.

Perhaps most significant among these features is that the products of the biopharmaceutical industry can be indispensable, even to life itself—thereby leaving the most important element of the biopharmaceutical chain, the patient, with little or no negotiating strength. Further, the biopharmaceutical sector of the United States has a market structure that is more complex than any other sector in health care—and perhaps more complex than any other sector in the entire economy. It is fraught with discordant viewpoints, divergent priorities and potential conflicts of interest that impede the provision of affordable biopharmaceuticals, especially to socioeconomically disadvantaged populations. The party often possessing the least power in this complex, rather opaque structure is, ironically, its *raison d'être*: the patient.

The committee concludes that the current approach to the provision of biopharmaceuticals in the United States is not sustainable. If that is indeed the case, only two broad options remain: repair the current system or replace it with a new system. Having dismissed the option of doing nothing, the report offers recommendations based on the preponderance of the available evidence and seeks to substantially improve the existing system. Should such steps, or others like those proposed, prove insufficient, the remaining choice is a system involving substantially increased government sponsorship and control, a single payer (i.e., government insurance), accompanied by governmentally imposed explicit or *de facto* price regulation.

Some of the package of actions proposed by the committee are as follows:

The federal government should consolidate and apply its purchasing power to directly negotiate prices with the producers and suppliers of medicines and strengthen formulary design. The government should also improve methods for assessing the value that drugs provide and ensure that incentives to develop drugs for rare diseases are not extended to widely sold drugs. In addition, increased disclosure of the financial flows and profitability among the participants in the biopharmaceutical sector should be required.

Action should be taken to continually foster greater access to off-patent generic drugs, which are usually much less expensive than branded products. One way this can be accomplished is to prevent practices that delay entry of generics into the market and thereby extend market exclusivity of branded products. Another critical step is to accelerate the review processes that are required of manufacturers before they can produce generic drugs.

Actions should be taken to eliminate existing incentives that encourage patients and clinicians to seek or prescribe more expensive drugs rather than less expensive alternatives of comparable efficacy. One such action would be to discourage direct-to-consumer advertisements for prescription drugs and to provide substantially more balanced information to patients about the potential benefits and costs of alternative treatments, thereby reducing unjustified demand for higher priced drugs.

Insurance plans should be modified to reduce the financial burden that patients and their families currently experience when they need costly prescription drugs, and individual cost-sharing arrangements that are based on drug prices should be calculated as a fraction of the net purchase price of drugs rather than the list price set by manufacturers. The government should also tighten qualifications for discount programs that have drifted from their original intent which was to help vulnerable populations. Finally, cost-sharing by patients enrolled in Medicare Part D should be terminated when the annual catastrophic coverage threshold has been reached.

Other implementing actions are discussed in detail in the report.

In the end, drugs that are not affordable are of little value; and drugs that do not exist, are of no value.

Thank you for this opportunity to appear before you on behalf of my colleagues on the National Academies committee and myself.