Insuring patient access and affordability for treatments for rare and ultrarare diseases: a policy statement of the American College of Medical Genetics and Genomics

ACMG Board of Directors[1]

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The past decade has seen tremendous progress in the development of new drugs for patients with genetic disorders, including cystic fibrosis, many lysosomal storage disorders (Gaucher disease, Fabry disease, mucopolysaccharidoses, and others) and most recently, Duchenne muscular dystrophy and spinal muscular atrophy (SMA). These new drugs are specialty drugs, and the projected cost of these new treatments is staggering. For example, the projected cost for the first year of treatment with nusinersen (Spiranza™) for SMA is $750,000, and $350,000 per year after that.[1] Spending on prescription drugs increased by 20% between 2013 and 2015 in the United States, and spending on specialty medications has increased by $54 billion since 2011, accounting for more than 70% of all prescription spending growth.[2] The problem is likely to worsen unless action is taken.

The high cost of prescription drugs has become a national concern, being discussed by patients, prescribers, payers, and policymakers. It affects not only drugs for treating genetic diseases, but also new treatments for cancer and other more common conditions. However, with few exceptions, genetic diseases are rare, which presents particular challenges for drug development, pricing, and reimbursement.

At the same time, tremendous emphasis has been placed on controlling the overall cost of health care in the United States, and a staggering array of reimbursement schemes have been developed in an effort to achieve this. A common component of these is "cost sharing," through deductibles and copays, forcing the patient to bear some of the cost of care, including prescription drugs. The confluence of breakthrough drugs and the enormous cost of these new drugs has become a "perfect storm," in which patients and their families face harsh economic choices or forgo appropriate care because of them.

The American College of Medical Genetics and Genomics and its members are committed to providing the most effective and cost-efficient treatments to their patients with genetic disorders. Access to affordable treatment with these orphan drugs for these conditions is not only medically necessary, it is life-altering for patients and their families. There is no one single answer to how to best manage this category of drugs, but we wish to propose points to consider in dealing with this. Potential solutions should be affordable, simple, and transparent. Cost-containment efforts must also address the burden on the entire health care system as high prescription drug prices may be shifted and absorbed in ways that negatively impact patient and prescriber access to important medications.

The passing of the Orphan Drug Act (ODA) in 1983 was a major step to encourage development of treatments for rare diseases, defined as those that affect fewer than 200,000 people in the United States. It provides tax incentives,

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enhanced patent protection and marketing rights, and subsidies for clinical research. However, the cost of developing a new drug and successfully bringing it to market are enormous, and include the cost of research, development, and clinical trials for drugs that are never approved, as well as those that are. Given the limited market for these drugs, it seems inevitable that they will be priced high to recover these costs, and generate a profit for the manufacturers and shareholders. We believe the ODA should continue to provide these incentives, but that changes may be warranted in the way these (and other) drugs are priced, marketed, and made available to patients, so that all patients have access to the most appropriate therapies.

A number of bodies have examined these issues, including the National Academies of Sciences, Engineering and Medicine;[3] the American College of Physicians;[4] the American Society of Clinical Oncology;[5] the American Academy of Dermatology;[6] and the Council of Medical Specialty Societies.[7] These points to consider were developed with particular reference to treatment for rare and ultrarare disorders.

PREVENT ABUSES OF THE ORPHAN DRUG ACT

The designation of orphan drug status should be limited to one 7-year period, and should not be granted to drugs already available in the market. "Salami slicing," the practice of selecting a subset of eligible patients to qualify for orphan drug status, and then expanding the scope of the drug's use to extend the market to more than 200,000 patients, should be prevented.

Create transparency in drug pricing

The basis for pricing a drug at a particular point should be publicly available, to make it more difficult to price a drug based on what the market is felt to be able to bear. Recently published discussions regarding value-based pricing may be evaluated as a potential solution.

Make prior authorization timely and evidence based

Prior authorization policies for specialty drugs should be evidence-based, transparent, and publicly available. The process through which prior authorization determinations are made should be standardized and speedy. Delay in beginning treatment for progressive genetic disorders can lead to suboptimal outcome or otherwise preventable deaths. Prior authorization and appeals policies should not unduly burden physicians or patients in accessing optimal drug therapy.

Place caps on patients' out-of-pocket costs for drugs

While copays for drugs may be appropriate, out-of-pocket costs for specialty drugs should not be solely based on a fixed percentage of the total cost of the drug. Setting caps on patients' out-of-pocket costs would prevent copays that are unaffordable for all but the wealthiest of patients.

Align drug packaging with dosing recommendations

Drugs (particularly injectables) should be packaged in quantities that prevent excessive waste, i.e., the need to purchase more drug than is necessary or appropriate because of the way it is provided.

Permit the federal government to negotiate prices with producers and suppliers of drugs

Current law prevents the government from negotiating drug prices for Medicare recipients. This is absurd.

Prevent the practice of "pay-to-delay," whereby a producer offers financial incentives to prevent the marketing of generic equivalents of drugs.

CONCLUSIONS

While research by privately and publicly funded entities has led to the development of life-saving and life-changing therapies for rare and ultrarare diseases, the extraordinary costs of these treatments may make them unavailable or unaffordable to patients. A national policy on controlling drug costs and ensuring their affordability is urgently needed.

DISCLOSURE

The authors declare no conflict of interest.

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