FOR IMMEDIATE RELEASE
Kathy Moran, MBA
Kmoran@acmg.net

Insuring Patient Access and Affordability for Treatments for Rare and Ultra-Rare Diseases: Points to Consider - A Statement of the American College of Medical Genetics and Genomics

BETHESDA, MD – September 5, 2018 | The last 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 and others) and most recently, Duchenne Muscular Dystrophy and Spinal Muscular Atrophy (SMA). The American College of Medical Genetics and Genomics (ACMG) is concerned with the staggering projected cost of these new treatments. 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. Cost-containment efforts must 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. There is no one single answer to how to best manage this category of drugs, but ACMG has just released, "Insuring Patient Access and Affordability for Treatments for Rare and Ultra-Rare Diseases: Points to Consider."

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.

ACMG Executive Director Michael S. Watson, MS, PhD, FACMG, said, "It will be important to get the costs of rare disease drugs under control as more and more increasingly narrowly targeted molecular treatments in subsets of patients with a disease are becoming available or are in late stage clinical trials. While the target group is often small, the financial impact of the cost to healthcare may be low. In aggregate, the treatments for many rare genetic diseases will be substantial to society and to patients which can limit their development and accessibility if the problem isn't addressed."

Background

Tremendous emphasis has been placed on controlling the overall cost of health care in the U.S., 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 co-pays, 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 passing of the Orphan Drug Act (ODA) in 1983 was a major step in encouraging development of treatments for rare diseases, defined as those which affect fewer than 200,000 in the U.S. It provides tax incentives, 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.

ACMG's Points to Consider

1. **Prevent abuses of The Orphan Drug Act.** The designation of orphan drug status should be limited to one seven-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.

2. **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.

3. **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.

4. **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 ultra-rare diseases, the extraordinary costs of these treatments may make them unavailable or unaffordable to patients. A national policy on controlling drug costs and insuring their affordability is urgently needed.

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 new ACMG Points to Consider statement is available at:
http://www.acmg.net/PDFLibrary/ACMG-Policy-Statement-Insuring-Patient-Access-And-Affordability.pdf

About the American College of Medical Genetics and Genomics (ACMG) and ACMG Foundation

Founded in 1991, ACMG is the only nationally recognized medical society dedicated to improving health through the clinical practice of medical genetics and genomics. The American College of Medical Genetics and Genomics (www.acmg.net) provides education, resources and a voice for more than 2,200 biochemical, clinical, cytogenetic, medical and molecular geneticists, genetic counselors and other healthcare professionals, nearly 80% of whom are board certified in the medical genetics specialties. The College's mission is to develop and sustain genetic initiatives in clinical and laboratory practice, education and advocacy. Three guiding pillars underpin ACMG's work: 1) Clinical and Laboratory Practice 2) Education and 3) Advocacy. *Genetics in Medicine*, published monthly, is the official ACMG peer-reviewed journal. ACMG's website (www.acmg.net) offers a variety of resources including Policy Statements, Practice Guidelines, Educational Resources, and a Find Genetic Services tool. The educational and public health programs of the American College of Medical Genetics and Genomics are dependent upon charitable gifts from corporations, foundations, and individuals through the ACMG Foundation for Genetic and Genomic Medicine.

-end-