VIEWPOINT

HEALTH POLICY

Novel Therapies for an Aging Population
Grappling With Price, Value, and Affordability

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The US health care sector is beginning to experience the full effect of the aging "baby boomer" population, 76 million of whom were born between 1946 and 1964, with nearly half eligible for Medicare in the coming year. In 2018, Medicare costs amounted to 14% of total federal spending, and by 2028, are projected to increase to 18%.[1] Growing enrollments, increased use of services, and rising prices for prescription drugs are major drivers of these increasing costs.[2]

In planning for the care of an expanding older population, a priority is being placed on what is most important to patients and families. This means "delivering appropriate care that fits the needs and circumstances of older patients and that actively avoids wasteful care."[3] Although this approach makes perfect sense, it is also clear that for many older patients, what is important will include new treatments and technologies to help them live longer, prevent or cure disease, reduce the likelihood or delay the onset of cognitive and functional impairment, and better manage their symptoms.

The prospect of extraordinarily high-cost therapies that could be used for treatment of common chronic conditions in older adults creates complexity in planning for the care needs of this population. This Viewpoint considers the implications of novel, high-cost drugs applicable to the care of older patients and also suggests recommendations for maintaining affordability and sustainable access to new therapies for chronic conditions in older adults that demonstrate added patient benefit.

A quote from the Director of Health Programs at the Office of Management and Budget, "If we get a cure for Alzheimer's priced at $100 000 a pop, we're toast,"[4] highlights hypothetical concerns about the cost implications of an effective therapy for a highly prevalent geriatric condition, Alzheimer disease. Nevertheless, an actual example of a high-cost, effective, and potentially broadly applicable therapy for a prevalent geriatric condition may be imminent. Tafamidis is now under priority review by the FDA for the treatment of transthyretin amyloid cardiomyopathy. For patients with this disease, clinical trial evidence has shown that treatment with tafamidis vs placebo for 30 months reduces mortality (78 of 264 [29.5%] vs 76 of 177 [42.9%]; hazard ratio, 0.70; 95% CI, 0.51-0.96) and cardiovascular-related hospitalization (0.48 per year vs 0.70 per year; relative risk, 0.68; 95% CI, 0.56-0.81), and slows declines in functional capacity and quality of life, based on the 6-minute walk test and the Kansas City Cardiomyopathy Questionnaire–Overall Summary score.[5] One estimate suggests that transthyretin amyloid cardiomyopathy may have a prevalence of 13% among patients with heart failure with preserved ejection fraction (HFpEF).[6] As HFpEF is the most common type of heart failure in older adults, the number of patients eligible for treatment with tafamidis could reach into the hundreds of thousands.

If tafamidis is approved, with a projected annual price in the range of $150 000-$300 000, and a large potential patient population, it could be among the most costly cardiovascular treatments ever marketed. Foreseeing a future in health care characterized by the development of numerous breakthrough therapies with the potential to affect a range of common chronic conditions in the geriatric population, scenarios as described for tafamidis could become the reality many times over. Having novel, high-cost treatments that provide important benefits to patients, while welcomed, will exacerbate concerns about the value and affordability of drugs for the Medicare population.

Clinical Effectiveness, Cost-effectiveness, Value, and Affordability

Any clinically effective treatment that addresses important health outcomes is likely to be considered valuable by patients, families, and clinicians, as long as that treatment is paid for by insurance. However, even highly effective treatments can be of low value to the health system—and to those who ultimately pay for insurance, including employers, Medicare, Medicaid, patients, and families—if the price is out of proportion to the clinical benefits. Even for some treatments that demonstrate good long-term value through a cost-effectiveness analysis, if the population that can benefit from treatment is very large, cumulative costs in the short-term may create substantial budget pressures. In these situations, even drugs with excellent long-term value may be so costly that budgets for insurers and delivery systems are adversely affected, while patient access is severely restricted.

Achieving High-Value With Novel Therapies for an Aging Population

Most people maintain that care should not be rationed for older patients nor should strategies to maintain the financial viability of Medicare rely on drastic increases in deductibles and cost-sharing requirements pushing even greater costs onto patients and families. Drugs with prices that exceed a reasonable premium for their added benefit are found in all areas of medicine and influence the care of patients of all ages. But as one of the largest, and most politically powerful population cohorts in US history, older patients, together with the clinicians who care for them, have an important role in advocating for a health system that retains incentives for future innovation and that also can adapt to provide sustainable access to high-value care for all patients.

The following recommendations may help advance these principles:

1. Drug makers and insurers should work to align the prices of drugs with their added benefits for patients. Whether this is achieved by drug makers through regulation to enhance competition and negotiating leverage for private insurers or through direct negotiation by Medicare, drug prices should be transparently and explicitly linked to their cost-effectiveness. Pharmaceutical manufacturers need to be rewarded for innovation and should also commit to responsible pricing of novel therapies guided by rigorous, independent, value-based assessments.
2. Clinicians should integrate the best possible evidence, together with their patients' individual clinical characteristics and personal values, in shared decision-making. Evidence to guide treatment decisions must be based on generalizable research findings, with measurements of the outcomes that matter most to older patients, including symptom burden, physical function, and health-related quality of life.[7] This is likely to be an enormous challenge for a number of reasons, including that clinical trials are often not representative of the general population; many trials contain only limited measurement of patient-centered outcomes; presenting complicated issues—for example the benefit of a drug vs potential adverse effects—is challenging; and because benefit of some therapies may not accrue for a number of years, the clinician and patient may need to consider life-expectancy in the discussion.
3. Specialty societies should incorporate information about the cost-effectiveness of novel, high-cost therapies for chronic conditions in older adults into guideline development. Information on cost-effectiveness should also be integrated into online, evidence-based clinical decision support resources that many clinicians use in real-time care of patients.
4. Insurers (including Medicare) and pharmacy benefit managers (PBMs) must provide clinicians, patients, and families with better information on how cost-effectiveness information is used to guide coverage policies and price negotiations. Insurers and PBMs must also meet their social responsibility by improving access to drugs by placing lower out-of-pocket financial burdens on patients and families for drugs that are priced fairly, consistent with their long-term value and affordability.

Conclusions

In providing care for the expanding older population, "What matters most?" cannot mean any therapy at any price. Aligning the price of new therapies with demonstrated added benefits is an important step on the road to a health system that can guarantee sustainable access to effective therapies for older patients. Policies around pricing and coverage must be developed in a transparent fashion with adequate consideration of the views of patients and families. Pricing and coverage decisions must also reflect that "high-value," high-cost treatments applicable to large numbers of older patients will require measures to ensure that the potential budgetary consequences do not impinge on patients' access to treatment and the affordability of their care.

Widely applicable drug therapies with extremely high prices are likely to emerge for the prevention and treatment of chronic conditions that are common among the older population in the United States. The ability of the US health system to deliver innovation, value, affordability, and access will be tested as never before. For the benefit of older patients, it is time to address these important challenges.

ARTICLE INFORMATION

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