MedPAC Backs Bundled Pay for Hospitalizations

BY ALICIA AULT
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WASHINGTON — The Medicare Payment Advisory Commission has given its backing to bundling payment for hospitalization, which would essentially give hospitals and physicians an incentive to control costs and avoid readmissions.

At its April meeting, the commission (MedPAC) unanimously voted to include a bundling recommendation in its June report to Congress. As a first step, physicians and hospitals should be required to report to the Centers for Medicare and Medicaid Services (CMS) on resource use and readmissions during an "episode of care," which is proposed to include the first 30 days post hospitalization. The data would be confidential initially, but by the third year, should be made public, MedPAC commissioners recommended.

Once the resource and readmission data are in hand, CMS should start adjusting payment to hospitals, according to the recommendation. There would be the possibility for gainsharing among hospitals and physicians. The commissioners also voted to direct CMS to study the feasibility of "virtual" bundling. With virtual bundling, the payment would be adjusted based on aggregate use of services over an entire episode of care.

Finally, MedPAC voted to recommend that CMS create a voluntary pilot to test actual bundled payment in selected disease conditions.

The data collection and adjusting payment based on readmission are interim steps aimed at getting providers to collaborate to improve care and cut costs.

The pilot could throw some light on how the hospital or accountable care organization receiving the payment decided to share funds, and how Medicare might share in any savings, according to MedPAC staff.

The pilot represents Medicare's ultimate goal—making bundled payments, said MedPAC chairman Glenn Hackbarth who is a health care consultant in Bend, Ore.

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Commissioner Ronald Castellanos, a urologist in private practice in Fort Myers, Fla., said he thought it would take 5 or 10 years to make collaboration work, but that he agreed that it was the ultimate end point. ■

GENOMIC MEDICINE

Old Drugs, New Tricks

BY GREG FEERO, M.D., PH.D.

As a former gene therapy researcher, I must confess that to me, nearly all attempts at gene therapy for genetic disorders have been disappointing. The sad fact is that our immune system is its own worst enemy as far as gene therapy goes, clearing attempts to use vectors to introduce new genetic material into cells and organs without breaking a sweat.

When I was a graduate student, I was fond of saying (probably not originally) that with gene therapy we were attempting to treat disorders we didn't understand, in systems we didn't understand, using gene vectors we didn't understand. At that time, many expected that, like a medical "Hail Mary," something good would come out of the considerable efforts directed at gene replacement–based therapies.

Moving forward, the prospects for successful primary gene therapy for most disorders remain distant. However, remarkable gains—fueled by discoveries in genomics—have been made in understanding the pathophysiology of many genetic disorders, and they are yielding therapeutic breakthroughs.

A particularly compelling story is the evolution of our understanding of Marfan syndrome (MFS), one of the classic autosomal dominantly inherited disorders that is characterized by tall stature, disproportionately long limbs, dislocated lenses, and other connective tissue abnormalities.

The most devastating consequence of MFS is a predisposition to aortic root dilatation and aneurysm formation that all too frequently leads to death in early adulthood. Unfortunately, the disorder is not that rare, affecting about 1 in 5,000 individuals (as a benchmark, cystic fibrosis affects about 1 in 2,500 whites). It is caused by mutations in the fibrillin-1 gene, which encodes the protein fibrillin-1, a constituent of the extracellular matrix in connective tissues and blood vessel walls.

Until recently, most investigators thought that MFS was a nearly hopeless case for targeted therapeutic interventions, largely because the defect was in a structural protein, rather than in an enzyme.

In general, it is comparatively easy to come up with rational ways for treating disorders with enzyme replacement; however, it is much harder to conceptualize treating a disorder if the cause is a structural element defect. MFS patients were therefore relegated to risky surgical correction of developing vascular abnormalities, or marginally beneficial use of β-blockers to slow blood vessel dilatation.

However, investigators were not satisfied that a classic structural protein defect could explain all of the features of MFS, and a few years ago, they made a vital discovery: Defects in fibrillin-1 cause dysregulation of transforming growth factor β (TGF-β) signaling in affected tissues.

By using mouse models for MFS and TGF-β–neutralizing antibodies, investigators were able to show rescue of the blood vessel abnormalities. This alone would be a remarkable scientific finding, but delivering antibodies over a long period to patients isn't a much more appealing clinical solution than the prospects of gene therapy.

Then something bordering on magical happened. One group of investigators recognized that an already commonly used antihypertensive in the class of drugs known as angiotensin II type 1 receptor blockers (ARBs) also interfered with TGF-β signaling, so they tried the drug in the mouse Marfan model.

The results were nothing short of spectacular: The vascular consequences of MFS could be prevented in the mouse model system (Science 2006:312;117-21).

This success, coupled with the grave prognosis for MFS and the known safety profile of the ARB drugs, has led to a large prospective human clinical trial funded by the National Heart, Lung, and Blood Institute.

The trial, comparing the effectiveness of losartan and atenolol in a pediatric to young adult (aged 6 months–25 years) population, will have as its primary outcome measurement of body surface–adjusted aortic root dilatation, with measurement at 2, 12, 24, and 36 months. The preliminary results are due out soon, and many in the field expect that the trial will show clear, major benefits from the use of ARBs.

It is interesting—and probably prophetic—that MFS treatment might soon be revolutionized through a careful tweaking of a formerly unrecognized but important pathway rather than through brute-force correction of the underlying genetic defect.

Expect that this will be the model for other truculent genetic disorders, not the least of which appears to be cystic fibrosis, for which a drug targeting patients with a particular genetic variant (unfortunately not the most common) has shown promising results in phase II trials in recent months.

Although almost 12 years have passed since I was a grad student, gene therapy remains the genomic medicine equivalent of a "Hail Mary"—a play not to be counted on or out. The difference today is that the ground game is fundamentally sound: Those 4-yard gains might carry the contest for a variety of disorders. ■

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