Transcript Details

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Cost-Effectiveness of Universal vs. Selective Screening for ATTR-CM in HFpEF Patients

Announcer:
You're listening to *Heart Matters* on ReachMD. On this episode, we'll hear from Dr. Kibum Kim, who's an Assistant Professor of Pharmacy Systems, Outcomes, and Policy at the University of Illinois Chicago. He'll be discussing his recent research on the cost-effectiveness of universal systematic screening of transthyretin amyloid cardiomyopathy, or ATTR-CM in patients with heart failure with preserved ejection fraction. Let's hear from Dr. Kim now.

Dr. Kim:
The objective of our research titled "Cost Effectiveness of Systematic Screening of ATTR-CM in Patients with HFpEF and Wall Thickening in the United States" is to assess the cost, measured in the United States dollar, and effectiveness measured in quality-adjusted life years between two screening strategies: universal technetium-99 pyrophosphate scintigraphy, also called PYP scanning, combined with free light chain test and immunofixation electrophoresis test versus selective screening for provider-acknowledged high-ATTR-CM-risk patients. The selective screening is provided for around 12 percent of the patients in our research, while we assume that the universal screening can be given for the HFpEF patient older than 60 years old.

We formulated the cost-effectiveness questions based on the two key factors or assumptions. One, the costs of medication specifically indicated for ATTR-CM exceed the generally accepted threshold. We wanted to demonstrate the value of these costly treatment options by quantifying the anticipated long-term benefits measured in quality-adjusted life years relative to the overall cost, particularly when the treatment is fully directed by either universal or selectively provided screening. And second, universal screening will allow more ATTR-CM patients to correctly utilize the innovative medication as indicated for those who would not be correctly diagnosed through the selective screening strategy.

We performed the analysis using an economic model that synthesizes various inputs from clinical trials, real world studies, health research utilization, and the cost following diagnosis using a simplified model framework. Typically, the cost-effectiveness model is designed to reflect the decrease in healthcare resource use and clinical benefits for those who received treatment or were precisely treated upon an accurate diagnosis compared to patients who were not able to receive treatment due to misclassification. Therefore, patient-level clinical benefits, reduced premature mortality, or healthcare resource savings per unit period are compared with the treatment cost escalation or increased economic burden associated with the larger volume of advanced treatment use.

The key findings from our research would be the cost increase when you replace the standard screening given only to selectively preferred patients with universal screening for all aged HFpEF patients exceeding the acceptable level of cost increase to gain additional clinical benefit. Specifically, incremental cost per effectiveness gain of around $900,000 per quality-adjusted life years gained exceeds the generally acceptable threshold.

We need to understand the source of the cost increase. As I mentioned before, the cost escalation is not due to the screening, but to the cost of the medication to meet the threshold willingness to pay $200,000 per quality-adjusted life years gained. The suggested

daily cost of the pharmaceutical intervention would need to match around 15 percent of the current cost, which almost exactly matches the benchmark price from another study. Does this mean that we have to push manufacturers to lower the price? I would definitely say no. Instead, we need to consider what motivations we can provide to expand access to medications, including sharing the cost escalation across the relevant parties and stakeholders or value-based coverage.

Another reason for the high incremental cost-effectiveness ratio is the target population. Since the target population suffers from already

high mortality, the potential maximum benefits in life years would not offset the cost of the screening strategies or the treatment strategy. Therefore, I would argue that the clinical approach should be tailored to pinpoint patients at high risk of ATTR-CM prior to the clinical manifestation, such as through the TTR gene mutation testing, which would benefit patients longer and then increase the value of the screening as well as advanced treatment option.

I also wanted to highlight that the cost escalation is not simply because patients could receive the advanced option after universal screening but because more patients were correctly classified with the proper diagnosis. When screening is selectively given, detection is limited to around one out of five true ATTR-CM patients. On the other hand, 93 percent of ATTR-CM patients immediately benefit from the universal screening. So we must consider that the scope of benefit is not limited to known ATTR-CM patients, but extend to both currently known and unknown patients despite the significant increase in the cost in reference to the clinical benefit.

Announcer:
That was Dr. Kibum Kim discussing his recent study on the cost-effectiveness of systematic screening of transthyretin amyloid cardiomyopathy in patients with heart failure with preserved ejection. To access this and other episodes in our series, visit *Heart Matters* at ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!