August 17, 2018

Steven D. Pearson, MD, MSc
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

Dear Dr. Pearson:

In connection with the Institute for Clinical and Economic Review’s (ICER) examination of new therapies for the treatment of hereditary transthyretin-related (hATTR) amyloidosis, the Association of Black Cardiologists (ABC) wishes to express the critical need to expand the types of treatments for this rare, progressive, and deadly disease that disproportionately afflicts black Americans.

The most frequent variant of transthyretin in the United States is the V122I mutation that is predominantly isolated to the heart. Other variants, like V30M, may be more common worldwide. Carriers of this mutation are at increased risk of developing congestive heart failure. In fact, studies show that carriers of this mutation have increased prevalence of heart failure and an earlier onset age than noncarriers. As noted in ICER’s Draft Evidence Report on Inotersen and Patisiran for hATTR, this variant is most common among African Americans, with a prevalence of 3.4 percent of the general population.

Transthyretin-related cardiac amyloidosis mimics hypertensive and hypertrophic heart disease and may, consequently, go undiagnosed. Beyond improving awareness of amyloid heart disease and improving diagnosis, there is an unmet need for better therapies. There is no Food and Drug Administration (FDA)-approved drug for this indication and traditional medications for heart failure have had no proven role in the treatment of amyloid heart disease. In fact, most medications have potential to cause harm.

The ABC is encouraged by new treatment strategies under investigation and is particularly optimistic about the recent FDA approval of patisiran for treatment of hATTR and its potential to reverse or mitigate its debilitating manifestations, including a decline in cardiac functioning.
We applaud ICER’s thorough scientific evidentiary review of new therapies for hATTR amyloidosis and encourage widespread availability to patients of FDA-approved treatments. For additional information or questions, please contact Cassandra McCullough, ABC CEO and Executive Director, at (212) 661-1438 or cmccullough@abcardio.org.


Sincerely,

Cheryl Pegus, M.D., M.P.H.
Board Chair
Association of Black Cardiologists