Invasive treatment strategy may increase survival for patients with certain neuromuscular disorder

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Patients with a cardiac irregularity and myotonic dystrophy type 1 (a severe neuromuscular disorder with a high risk of sudden death) who received an invasive treatment strategy that included testing of their heart's electrical conduction system and if needed, implantation of a device such as a pacemaker, had an associated higher rate of 9-year survival compared to patients treated noninvasively, according to a study in the March 28 issue of *JAMA*.

Myotonic dystrophy type 1 (DM 1) is the most common inherited neuromuscular disease in adults, with an incidence of 1 in 8,000. "The manifestations of the disease include muscle weakness, myotonia [abnormally long muscular contractions], multiple endocrine disorders, respiratory insufficiency, and cardiac abnormalities. The prevention of sudden death is central to patient management," according to background information in the article. Progression of conduction system (special muscle fibers that conduct electrical impulses throughout the muscle of the heart) disease to complete atrioventricular block (impairment of the conduction between the atria and ventricles of the heart) is the presumed cause of sudden death in a high proportion of patients. Permanent cardiac pacing has been recommended by the American College of Cardiology and the American Heart Association for patients who meet certain criteria. "Up to one-third of patients with myotonic dystrophy type 1 die suddenly. Thus far, no intervention has effectively prevented sudden death," the authors write.

Karim Wahbi, M.D., of the Department of Cardiology, Cochin Hospital, Paris, and colleagues conducted a study to determine the overall survival rates of patients with conduction abnormalities on the electrocardiogram who were managed with a noninvasive strategy and regular surveillance compared with patients managed with an invasive strategy with electrophysiological study (testing of the heart's electrical conduction system) and prophylactic cardiac pacing for patients when the measurement of the conduction time of a specific area of the heart exceeded a certain level. The study, the DM1 Heart Registry, included 914 patients older than 18 years with genetically confirmed myotonic dystrophy type 1 who were admitted to a teaching medical center in Paris, between January 2000 and December 2009. This analysis included 486 patients who presented with minor conduction defects. Among these patients, 341 were assigned to the invasive strategy group and 145 were assigned to the noninvasive strategy group. The median (midpoint) patient follow-up was 7.4 years.

Among the 486 patients, 80 died during follow-up, corresponding to a 9-year survival of 74.4 percent. Of these, 50 patients died in the invasive strategy group and 30 died in the noninvasive strategy group. Analysis indicated that survival in the invasive strategy group was consistently higher than in the noninvasive strategy group, and their respective 9-year survival rates were 76.7 percent and 69.2 percent. After adjustment for various factors, the hazard of dying was nearly 40 percent lower in the invasive strategy group than in the noninvasive strategy group.

The researchers suggest that the survival difference was largely attributable to a lower incidence of sudden death; the 9-year cumulative incidence of sudden death was 4.5 percent in the invasive strategy group and 18.0 percent in the noninvasive strategy group, with the hazard of dying suddenly 75 percent lower in the invasive strategy group than in the noninvasive strategy group.

At 9 years, the cumulative incidence of death from respiratory failure was 11.1 percent in the invasive strategy group and 9.1 percent in the noninvasive strategy group. No significant difference in the rate of respiratory death was observed between the 2 groups.

The authors note that the markedly lower incidence of sudden death in the invasive strategy group suggests that conduction system disease is a major cause of sudden death that appears to be preventable by implementation of an invasive strategy; and that electrophysiological study might have contributed to the identification of malignant ventricular arrhythmias, and might account for some of the observed differences between the invasive strategy group and the noninvasive strategy group.

"In summary, among patients with DM1 with major infranodal conduction delays, management with an invasive strategy based on systematic electrophysiological studies and prophylactic permanent pacing is associated with longer survival. While other studies are needed to confirm these findings, consideration of this strategy may be prudent in this population at higher than average risk for sudden death."

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