DRAFT

American Heart Association

Testimony to the Center for Medicare and Medicaid Services Regarding Ventricular Assist Devices as Destination Therapy

February 20, 2003

Background:
The American Heart Association is a voluntary health organization whose mission is to reduce death and disability from cardiovascular disease and stroke. With 3000 staff, 30,000 volunteer members of our Scientific Councils, and more than 22 million supporters nationwide, our impact goal is to reduce events of and risk for coronary heart disease and stroke by 25% by 2010, in partnership with the Healthy People 2010 programs.

The American Heart Association believes more can be done to prevent cardiovascular diseases in the first place, but is also acutely aware of the nearly 5 million individuals in this country who currently suffer from heart failure, and of the disability that this produces. While we welcome the opportunity to work with the Congress and the Administration to offer more preventive screenings for Medicare beneficiaries and to develop more aggressive programs and policies to lower obesity rates and other risk factors in the nation, we are also aware of and have contributed to the important advances in the treatment of heart failure; including much more effective approaches using pharmacological agents, reparative surgical procedures, devices, and cardiac transplantation. With effective medical therapy for heart failure, the risk of death due to heart failure can be reduced by nearly 50%, even for those patients with advanced left ventricular dysfunction and moderately severe to severe heart failure. We support the widespread use of these medical treatment options, as we believe this will truly impact the risk of death due to heart failure. Although prevention is key to reaching our joint 2010 goals, we commend you for your work to provide those already afflicted by cardiovascular diseases with optimum care. As always, the Association is pleased to help in any way possible.

The advances cited above, and others on the horizon utilizing new approaches such as novel surgical procedures, newer diagnostic/prognostic aids and cell- and gene-based therapies, have greatly improved the outlook for patients with heart failure. It is the purpose of the American Heart Association to vigorously support the genesis of new information to address the alarming problem of heart failure. However, the prevalence of hypertension and obesity, the aging population and our ability to rescue the patient from the acute risk of a myocardial infarction are combining to produce a greater prevalence of heart failure, as reflected in the number of hospital discharges with this diagnosis—currently over one million annual hospitalizations. Heart failure is the one cardiovascular illness which is increasing in

incidence while all others are declining commensurate with the striking advances in cardiovascular therapeutics.

For the patient with far advanced heart failure, the therapeutic options beyond comprehensive medical therapy have traditionally been limited to cardiac transplantation. More recently, device therapy with implantable defibrillators and multi-lead pacemaker strategies appear to be holding some promise but there are important issues related to patient selection, device implantation and incremental costs that will need to be addressed before the use of these devices become commonplace. These are the same dilemmas now emerging with the introduction of left ventricular support devices as “destination therapy” for advanced heart failure.

The wait for cardiac transplantation can be precarious but that risk has been ameliorated with the use of left ventricular assist devices for patients in whom pharmacological management is insufficient for survival to transplant. These mechanical circulatory support devices (ventricular assist devices, or VADs) have provided an important additional support option, and are accepted as a “bridge to transplantation”. Patients who are treated with a mechanical support system prior to transplant have a >60% chance of ultimately undergoing transplantation.

The success of mechanical support has now been extrapolated to an equally ill patient population but one in which cardiac transplantation is not an option. This is usually due to older age and/or important co-morbidities that would clearly limit success after transplantation. Application of chronic left ventricular support could be hypothesized to prolong life and to improve its quality, and its removal would not be anticipated. It is this use of the left ventricular assist device as “destination therapy” to which this testimony is directed.

The support for the use of VADs as destination therapy is based on the REMATCH (Randomized Evaluation of Mechanical Assistance for the Treatment of Congestive Heart failure) trial, in which 129 patients with severe heart failure who were judged not to be appropriate candidates for transplantation were randomized to a VAD or optimized medical therapy. VAD use was associated with improved survival over a 1-year follow-up, as compared with patients treated with oral and intravenous medications. However, the morbidity of the procedure was considerable. Complications included bleeding, infections, strokes and device failure—a phenomenon that was seen to occur late after device implantation. At two years, 75% of VAD patients were dead but 92% of patients treated with optimized medical therapy were dead. Importantly, the REMATCH data identified the striking mortality risk of far advanced heart failure, especially when therapy is limited to currently available pharmacological choices. The quantity, and the quality of the prolonged life, albeit not ideal, did show improvement over the medical treatment option. Clearly, additional study is needed: how can device implantation be simplified? Can the postoperative morbidity be limited? And can truly long-term outcomes be expected? Newer iterations of the devices are addressing these concerns but more data will be needed and a strict ongoing review process is definitely required. It is important to note that the complication rate associated with VAD implantation was not trivial, even in the carefully selected and highly skilled REMATCH centers. There is concern that the morbidity and perioperative mortality risk might be much higher in centers that are less specialized and/or have limited infrastructure for long-term follow-up and support.

Nevertheless, there is a demonstrated benefit for an increasingly needy patient population of patients with advanced heart failure. For highly selected patients cared for in experienced medical centers with appropriate multidisciplinary teams in place, the use of VADs as "destination therapy" appears worthwhile. The patients in whom benefit appears to exist are those with objective evidence of poor long term outcomes based on persistent symptoms, an ongoing requirement for inotropic support, demonstrated hemodynamic decompensation and terribly impaired aerobic capacity. There are no data points regarding the benefit of "destination therapy" in patients who are less ill and aggressive medical treatment strategies should remain the standard of care.

It is our position that several caveats be considered:

1. These devices should only be implanted at centers with an established record of proficiency with VAD technology and implantation.
2. Candidate selection should be rigorously monitored and guidelines should be established with close adherence to the same; patients receiving destination therapy should be "pre-certified"
3. Multidisciplinary teams are required to support the patient long-term and these teams need to include expert nursing support and where needed family support services
4. A registry should be established and followed carefully for the discovery of new issues. Unlike the usual pharmacological trial in heart failure, which may include thousands of patients, the pivotal trial for this technology included fewer than 150 patients. It is plausible that unforeseen complications may arise
5. An ongoing cost analysis is likewise desirable and data regarding cost per year of life saved should be reviewed as a part of this overall review process
6. New centers will need to undergo careful scrutiny before being approved for implantation. Features that might be expected of all VAD implantation centers include:

    a. Previous experience with VADs
    b. An acceptable complication rate
    c. Fully-staffed, multi-disciplinary team
    d. Ongoing review and evaluation process
    e. Willingness and ability to engage in further research to improve the process
    f. Appropriate informed consent procedures to include the substantial risks of morbidity and the limitation of the mortality benefit
    g. ? Alignment with an established heart transplantation program

It is suggested that CMS consider convening a panel of experts from a cross-section of stakeholders (ISHLT, AHA, ACC,Thoracic Surgical Societies, etc) to draft specific recommendations pursuant to the above principles.

Future clinical trials using VAD technology are imperative but will necessarily be small due to the limited patient population in whom such a strategy is acceptable. Yet, the event rate in this population is alarmingly high, so that a smaller number of patients may be all that is required to determine benefit. Mechanistic studies designed to address the issues of VAD related morbidity need to be pursued. As newer medical therapies are developed, the ongoing use of VADs will need to be reassessed. The funding for future research in this area will need to represent cooperation between industry, the NIH, AHA and perhaps even CMS. The public health implications are substantial and adequate support for well-designed clinical investigations is an inviolate requirement if this technology is to become entrenched in the cardiovascular armamentarium for the treatment of advanced heart failure.

The very guarded approach outlined in this document would allow for the provision of the benefit to those patients who meet REMATCH criteria, while providing a platform that will move this technology forward and bring relief to more patients with advanced, end-stage heart failure.