Statement Regarding the Pre and Post Market Assessment of Durable, Implantable Ventricular Assist Devices in the United States

Michael A. Acker, MD, Francis D. Pagani, MD, PhD, Wendy Gattis Stough, PharmD, Douglas L. Mann, MD, Mariell Jessup, MD, Robert Kormos, MD, Mark S. Slaughter, MD, Timothy Baldwin, PhD, Lynne Stevenson, MD, Keith D. Aaronson, MD, Leslie Miller, MD, David Naftel, PhD, Clyde Yancy, MD, Joseph Rogers, MD, Jeffrey Teuteberg, MD, Randall C. Starling, MD, MPH, Bartley Griffith, MD, Steven Boyce, MD, Stephen Westaby, MD, PhD, Elizabeth Blume, MD, Peter Wearden, MD, PhD, Robert Higgins, MD, and Michael Mack, MD

Division of Cardiothoracic Surgery, University of Pennsylvania, Philadelphia, Pennsylvania (MAA); Department of Cardiac Surgery, University of Michigan, Ann Arbor, Michigan (FDP); Campbell University College of Pharmacy and Health Science, Buies Creek, North Carolina (WGS); Cardiovascular Division, Washington University, St. Louis, Missouri (DLM); Division of Cardiovascular Medicine, University of Pennsylvania, Philadelphia, Pennsylvania (MJ); Department of Cardiothoracic Surgery, University of Pittsburgh, Pittsburgh, Pennsylvania (RK); Division of Cardiothoracic Surgery, University of Louisville, Louisville, Kentucky (MSS); National Heart, Lung, and Blood Institute, Bethesda, Maryland (TB); Cardiovascular Division, Brigham and Women's Hospital, Boston, Massachusetts (LS); Division of Cardiovascular Medicine, University of Michigan, Ann Arbor, Michigan (KDA); Department of Cardiovascular Sciences, University of South Florida, Tampa, Florida (LM); Department of Cardiothoracic Surgery, University of Alabama, Birmingham, Birmingham, Alabama (DN); Division of Cardiology, Northwestern University, Chicago, Illinois (CY); Division of Cardiology, Duke University, Durham, North Carolina (JR); Heart and Vascular Institute, University of Pittsburgh Medical Center, Pittsburgh, Pennsylvania (JT); Cardiovascular Medicine, Cleveland Clinic, Cleveland, Ohio (RCS); Division of Cardiac Surgery, University of Maryland, Baltimore, Maryland (BG); Department of Cardiac Surgery, MedStar Heart Institute, Washington Hospital Center, Washington, DC (SB); Oxford Heart Centre, John Radcliffe Hospital, Oxford, United Kingdom (SW); Department of Cardiology, Boston's Childrens Hospital, Boston, Massachusetts (EB); Department of Cardiothoracic Surgery, Children's Hospital of Pittsburgh, Pittsburgh, Pennsylvania (PW); Division of Cardiac Surgery, Ohio State University; Columbus, Ohio (RH); and Cardiothoracic Surgery, Medical City Dallas Hospital, Dallas, Texas (MM)

The incorporation of complex medical device technologies into clinical practice is governed by critical oversight of the US Food and Drug Administration. This regulatory process requires a judicious balance between assuring safety and efficacy, while providing efficient review to facilitate access to innovative therapies. Recent contrasting views of the regulatory process have emphasized the difficulties in obtaining an optimal balance. Mechanical circulatory support has evolved to become an important therapy for patients who have advanced heart failure with the advent of more durable, implantable ventricular assist devices. The regulatory oversight of these new technologies has been difficult owing to the complexities of these devices, associated adverse event profile, and severity of illness of the intended patient population. Maintaining a regulatory environment to foster efficient introduction of safe and effective technologies is critical to the success of ventricular assist device therapy and the health of patients with advanced heart failure. Physicians representing key surgical and cardiology societies, and representatives from the Food and Drug Administration, National Heart Lung, and Blood Institute, Centers for Medicare and Medicaid Services, Interagency Registry of Mechanically Assisted Circulatory Support, and industry partners gathered to discuss relevant issues regarding the current regulatory environment assessing ventricular assist devices. The goal of the meeting was to explore innovative ways to foster the introduction of technologically advanced, safe, and effective ventricular assist devices. The following summary reflects opinions and conclusions endorsed by The Society of Thoracic Surgeons, American Association for Thoracic Surgery, American Heart Association, Heart Failure Society of America, International Society for Heart and Lung Transplantation, and Interagency Registry of Mechanically Assisted Circulatory Support.

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REPORT

The statement is endorsed by The Society of Thoracic Surgeons, American Association for Thoracic Surgery, American Heart Association, Heart Failure Society for America, International Society for Heart and Lung Transplantation, and the Interagency Registry of Mechanically Assisted Circulatory Support.

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Address correspondence to Dr Acker, Hospital of the University of Pennsylvania, 3400 Spruce St, 6 Silverstein Pavilion, Philadelphia, PA 19104; e-mail: michael.acker@uphs.upenn.edu.

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Background and Purpose

The incorporation of complex medical device technologies into clinical practice is governed by critical oversight of the US Food and Drug Administration (FDA). This regulatory process requires a judicious balance between assuring safety and efficacy, while providing efficient review to facilitate patient access to innovative therapies. The regulatory approval process in the United States is complex owing to the increasing technical sophistication of devices and the dynamic nature of the medical device industry. Increasing scrutiny has been placed on the regulatory process because of concerns about introduction of unsafe technologies into the medical community, while the medical device industry and some clinicians have perceived the process of obtaining regulatory approval of new devices as inefficient and lengthy [1–5]. The US House Energy and Commerce Subcommittee on Oversight and Investigations held a hearing on July 20, 2011, entitled "Medical Device Regulation: Impact on American Patients, Innovation, and Jobs," and examined, in part, the migration of US device companies abroad to develop and evaluate innovative medical devices [6]. On July 29, 2011, the Institute of Medicine released an FDA-commissioned report on the 510(k) clearance process and concluded that it was impossible for the 510(k) regulatory pathway to assure safety and effectiveness of medical devices [7]. These contrasting views of the regulatory process emphasize the critical balance between ensuring safety and efficacy and permitting timely access of innovative devices to patients.

Mechanical circulatory support (MCS) has evolved to become an important therapy for patients who have advanced heart failure, with the advent of more durable, implantable ventricular assist devices (VAD) [8]. Innovative technological advances in durability and reductions in device size have fostered growing adoption of this therapy by physicians and patients [9]. The regulatory oversight of these new technologies has been difficult owing to the complexities of MCS devices, associated adverse event profile, severity of illness of the intended patient population, heterogeneity of device designs, and varied implantation indications. Maintaining a regulatory environment to foster efficient introduction of safe and effective technologies is critical to the success of VAD therapy and important to improving the health of patients with advanced heart failure.

On September 16, 2011, physicians representing The Society of Thoracic Surgeons, American Heart Association, American Association for Thoracic Surgery, Heart Failure Society of America, American College of Cardiology Foundation, and International Society for Heart and Lung Transplantation, and representatives from the FDA, National Institutes of Health (NIH), National Heart Lung, and Blood Institute (NHLBI), Centers for Medicare and Medicaid Services (CMS), Interagency Registry of Mechanically Assisted Circulatory Support (INTERMACS), medical insurance providers, and industry partners gathered in Washington, DC, to discuss relevant issues regarding the current regulatory environment assessing MCS therapy. The goal of the meeting was to explore innovative ways to foster the introduction of technologically advanced, safe, and effective MCS devices.

Five important areas of the regulatory process were examined for ways to improve predictability and efficiency of evaluation, and assessments and standards of safety and efficacy. These areas focused on the following: (1) innovative clinical trial designs; (2) the need for a new encompassing indication for therapy to facilitate device evaluation and reduce disparities in patient access; (3) assessing appropriateness of international standards for regulatory evaluation of MCS devices; (4) exploring methods for preclinical MCS device evaluation; and (5) development and regulatory oversight of MCS devices in the pediatric population. In each area, critical questions were raised, and perspective was provided by panel members.

This paper summarizes the discussions that took place at this pivotal meeting. Consensus among the experts was reached for some issues, whereas other areas were identified where more data are needed to advance the field toward agreement. The overarching goal of both the meeting and this document is to serve as a platform to launch next steps toward achieving a collaborative effort focused on bringing safe and effective MCS therapies to appropriate patients in an efficient, consistent, and economically responsible manner.

Abbreviations and Acronyms

BTC	= bridge to decision or candidacy
BTT	= bridge to transplant
CMS	= Centers for Medicare and Medicaid Services
DT	= destination therapy
FDA	= Food and Drug Administration
HDE	= Humanitarian Device Exemption
INTERMACS	= Interagency Registry of Mechanically Assisted Circulatory Support
MCS	= mechanical circulatory support
NHLBI	= National Heart, Lung, and Blood Institute
PMA	= premarket approval
VAD	= ventricular assist device

See Appendix for financial relationship disclosures of authors.

Innovative Clinical Trial Designs

Are Active-Controlled Large Randomized Clinical Trials Necessary to Establish Long-Term Safety and Efficacy for Destination Therapy?

Randomized controlled trials are the gold standard for evaluating the safety and efficacy of new therapeutics.

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