



Randel E. Richner
Vice President
Government Affairs and
Reimbursement & Outcomes Planning

1331 Pennsylvania Avenue, NW
Suite 550 South
Washington, DC 20004

BY ELECTRONIC SUBMISSION

August 23, 2004

The Honorable Tommy Thompson
Secretary
Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Re: Solicitation of Comments on Stimulating Innovation in Medical Technologies [Docket No. 2004S-0233]

Attn: Jeffrey Shuren, M.D., Assistant Commissioner for Policy, FDA

Dear Secretary Thompson:

Boston Scientific Corporation (Boston Scientific) appreciates the opportunity to comment on how the Department of Health and Human Services (HHS) and its agencies can work together to facilitate the development and approval of new medical technologies.

As the world's largest company dedicated to the development, manufacturing, and marketing of less-invasive therapies, Boston Scientific supplies medical devices and technologies used by the following medical specialty areas:

- Electrophysiology;
- Endoscopy;
- Gynecology;
- Interventional Cardiology;
- Neurovascular;
- Oncology;
- Peripheral Interventions;
- Urology; and
- Vascular Surgery.

One important policy area where HHS can stimulate innovation in medical technologies involves Humanitarian Use Devices (HUDs, or "orphan devices") – innovative medical devices that are designed to treat rare or orphan diseases or conditions. Boston Scientific is concerned about ensuring patient access to **technologies that treat orphan diseases**. Whereas, Congress and the FDA have taken **explicit actions to promote the development of these technologies through the Humanitarian Device Exemption (HDE) process, no corresponding mechanism has been established to promote Medicare and Medicaid coverage and payment for these technologies**. Boston Scientific recommends that CMS

establish a streamlined coverage and payment process for HDEs in a similar fashion to the special approval process that Congress and FDA established to improve medical device innovation for diseases affecting small populations.

In 1990, as part of the Safe Medical Devices Act, Congress created a special subcategory of medical devices – the “humanitarian use device” (HUD) -- to ensure patient access to devices intended to treat or diagnose diseases or conditions that affect fewer than 4,000 individuals annually in the United States. In doing so, Congress recognized that for diseases and conditions affecting small populations, a device manufacturer’s considerable research and development costs would likely exceed its expected market returns, thus creating a significant obstacle to the development and marketing of such devices.

To ensure that patients with rare diseases or conditions would have access to HUDs, Congress exempted them from the requirement under the traditional pre-market approval (PMA) process that a device be clinically proven “effective” for the target population. To be legally marketed in the U.S., an HUD must be approved through the Humanitarian Device Exemption (HDE) process, which is similar to the PMA process but without the effectiveness requirement. The devices must be proven safe, and demonstrate a probable benefit that outweighs the possible risks of injury or illness to patients.

The HDE approval process initiates with a rigorous application process to the FDA’s Office of Orphan Products by which the manufacturer must demonstrate that there are no comparable devices available to treat the target population, and the device would likely not be made available to patients unless the exemption from the “effectiveness” requirement was granted. (For manufacturers of devices designed to treat 4,000 or fewer patients a year, it was recognized that it would be difficult, if not impossible, to amass a statistically valid study population large enough to demonstrate effectiveness.)

A device with an approved HDE is authorized to be marketed in the U.S.; it is not an investigational device for which clinical trials are still ongoing. Treatments that involve approved HDEs remain under strict controls and may only be used in facilities where an Institutional Review Board (IRB) has approved and oversees use of the device.

To further foster innovation in the treatment of orphan diseases, CMS should allow a streamlined Medicare coverage and payment process for Humanitarian Device Exemption technologies.

For patients suffering from rare conditions, or whose diseases or conditions are not treatable with existing technologies, having access to HDEs is critical and, for some, represents the last chance therapy. Intracranial atherosclerosis is an example of disease for which a subset of the patient population is not responsive to medical therapy, and with no other treatment options, they are at significant risk for ischemic stroke. This orphan patient group could benefit greatly from promising medical innovation in development. FDA approval of an HDE is only the first step. For patients to truly have *access* to these innovative technologies, the devices must be included in the patient’s health care coverage. For Medicare beneficiaries, access is only achievable if a reasonable CMS coverage process accompanies the FDA HDE approval pathway currently in place. Medicare coverage also sends a positive signal to private payers, making Medicare coverage of an HDE essential to securing access to the device by all patients suffering from the rare condition treated by the device.

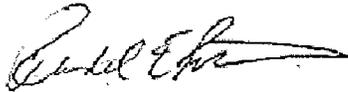
Medicare coverage is important to help facilitate investment in the development of these life-saving technologies. Since the HDE process was finalized in 1996, 34 HDEs have been approved. The small number of HDEs that have been approved in the last seven years demonstrates how rare the development

of these devices is. In comparison, the Center for Devices and Radiological Health of FDA approved 551 PMA applications, and 3,711 510(k) clearance applications, in calendar year 2002 alone.

Because Medicare coverage is such an important component of ensuring access to Humanitarian Use Devices, CMS needs to ensure that more streamlined processes and procedures are implemented to ensure that beneficiaries have access to devices that have been approved as HDEs by the FDA. Through the HDE process, FDA has taken steps to facilitate getting these devices to market. CMS needs to do its part to ensure that beneficiaries with rare diseases or conditions have access to these important innovative technologies. We would be happy to work with you in the coming months to develop more specific strategies for eliminating obstacles to access to FDA-approved HDEs.

Please do not hesitate to contact me (508-652-7410; randel.richner@bsci.com) or Thomas Grissom (202-637-8025; thomas.grissom@bsci.com) in our Washington office with any questions or if we can be of further assistance.

Sincerely,



Randel E. Richner, BSN, MPH
Vice President, Government Affairs and Reimbursement & Outcomes Planning

cc: Mark McClellan, Administrator, CMS
Herb Kuhn, Director, Center for Medicare Management, CMS
Sean Tunis, Director, Office of Clinical Standards and Quality, CMS