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July 29, 2004

Dockets Management Branch (HFA-305)  
Food and Drug Administration  
5630 Fishers Lane, Room 1061  
Rockville, Maryland 20852

Re: [Docket No. 2004-N-0181]  
**Critical Path Initiative**

Dear Sir/Madam:

Baxter, International Inc., through its subsidiaries, assists health-care professionals and their patients with the treatment of complex medical conditions, including cancer, hemophilia, immune disorders, kidney disease and trauma. The company applies its expertise in medical devices, pharmaceuticals and biotechnology to make a meaningful difference in patients' lives. Baxter applauds the initiative taken by the Food and Drug Administration (FDA) in publishing its report<sup>1</sup> addressing national concerns regarding the Medical Product Development process, and is supportive of potential collaborative efforts between industry, academia, and FDA aimed at modernizing the development process, or "critical path", in order to make product development more predictable and less costly, while increasing the number of safe and effective medical products that are available to the public. Baxter welcomes the opportunity to participate in the national dialogue regarding the FDA's Critical Path Initiative, and wishes to offer the following comments for consideration:

**General Comments**

Baxter concurs with the FDA's identification of Safety Assessment, Demonstration of Medical Utility, and Industrialization (manufacturing) as the three main scientific and technical dimensions of the critical path in the delivery of new and innovative medical products to the marketplace. The agency's report appropriately focuses on a variety of critical path research issues and the role that FDA can play in working with medical product developers to speed up the development process, citing both general and specific examples of successful collaborations between FDA and scientists in industry and academia.

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<sup>1</sup> "Innovation/Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products."

In addition to research tools, the report comments on the very important role that FDA guidance documents play in decreasing the uncertainties associated with the development of devices, drugs and biologicals. We support the FDA's continued commitment to develop guidances concerning scientific and regulatory issues associated with crucial public health issues. We feel strongly that FDA's process for interacting with industry on regulatory issues represents a significant component of any effort aimed at improving the product development toolkit.

**Specific Comments – Autologous Adult Stem Cells**

As outlined in the report, FDA has identified development of characterization procedures and standards for expanded stem cells as a critical path initiative. While research into the cultivation and maintenance of stem cell lines will undoubtedly play an important role in the future development of therapeutic applications, refinement of the technical and regulatory framework for autologous adult stem cells represents a significant near term critical path opportunity.

**Critical Path Initiative Opportunity**

As discussed at the March 18-19, 2004 Biological Response Modifiers Committee Meeting, ischemic heart disease and congestive heart failure remain the major causes of morbidity and mortality in the United States, with cellular therapy treatment options derived from myoblasts, mesenchymal stem cells, and adult autologous stem cells being considered for potential clinical application in the treatment of cardiac disease.<sup>2</sup>

Given the significance of this public health issue for all stakeholders in the United States, we respectfully request that FDA consider the following actions as a means of furthering efforts to develop stem cell derived cellular therapies for cardiac disease:

- Evaluate currently marketed stem cell selection systems and establish a basis for regulatory approval of these systems based on their capability to process a stem cell product with defined characteristics as a general indication for use.
- Develop guidance defining the regulatory pathway and identifying product specific consensus standards for adult autologous stem cells vs. stem cells derived from other sources

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<sup>2</sup> Briefing Document-Biological Response Modifiers Committee Meeting #37: Cellular Products for the Treatment of Cardia Disease, March 18-19, 2004

- Re-evaluate the current definition of homologous vs. non-homologous function of hematopoietic stem cells, based on available scientific data, and incorporate any changes into current and future regulatory approaches.
- Work with industry and academia to implement a regulatory pathway for adoption of stem cell therapies for various diseases that appropriately addresses safety and efficacy requirements while minimizing the regulatory burden placed on device systems already approved for use in stem cell processing.

Systems that are already approved for the processing of adult autologous stem cells may represent an opportunity to speed the development of cellular therapies for cardiac disease and other indications while the research tools needed for other stem cell sources are still in development.

In conclusion, Baxter wishes to reiterate our support for FDA's Critical Path Initiative. We look forward to an ongoing and productive dialogue regarding this very important effort to improve the medical product development process. Efforts to strengthen the collaboration between FDA and industry in support of high-priority opportunities will ultimately benefit all national public health stakeholders.

Sincerely,



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Regulatory Policy, External Affairs and Risk Management  
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Round Lake, IL 60073



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