BIO
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

BY ELECTRONIC SUBMISSION

AHRQ Effective Health Care Program
c/o Center for Outcomes and Evidence
Agency for Healthcare Research and Quality
540 Gaither Road
Rockville, MD 20850

Re: Effectiveness and Off-Label Use of Recombinant Factor VIIa

The Biotechnology Industry Organization ("BIO") appreciates this opportunity to comment on the Agency for Healthcare Research and Quality's ("AHRQ") proposed research regarding the effectiveness and off-label use of recombinant Factor VIIa.[1] AHRQ seeks stakeholder input on five key questions relating to the use of recombinant Factor VIIa under specific circumstances.

BIO is the largest trade organization to serve and represent the biotechnology industry in the United States and around the globe. BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers and related organizations. BIO's members are strongly committed to increasing the body of quality evidence available to further the clinical decision making process. Our members invest millions of dollars each year on clinical studies, both before and after Food and Drug Administration ("FDA") approval, to produce high-quality clinical evidence to support appropriate medical decision making.

BIO supports a rigorous research process that encompasses all aspects of a disease, from examining how a disease affects the body to studying the relative effectiveness and benefits of therapies. Our members' research initiatives go well beyond clinical safety and efficacy to advance the understanding of disease pathology and therapeutic mechanisms of action, clinical effectiveness in naturalistic settings, health-related quality of life issues, and health economic

[1] AHRQ's proposal is available at: http://effectivehealthcare.ahrq.gov/getInvolved/commentFormKQ.cfm?topic=54.

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impacts of therapies. As such, BIO supports the goals of AHRQ's Effective Health Care Program, which aims to provide high quality research to inform the clinical decision making process. It is with these goals in mind that BIO offers the following general comments to AHRQ's key questions.

I. AHRQ Should Provide Stakeholders with Greater Transparency Around Topic Selection for Effective Health Care Reviews.

Section 1013 of the Medicare Modernization Act ("MMA") requires AHRQ to "establish a process to develop priorities that will guide the research, demonstrations, and evaluation activities undertaken."[2] As a result, AHRQ hosted listening sessions and issued *Federal Register* notices to seek stakeholder input on possible research topics. Taking into account this public input, AHRQ developed a list of ten priority conditions to guide its research.[3] BIO firmly believes that all stages of comparative effectiveness research should be conducted through an open and transparent process involving all stakeholders, starting from the research priority setting and planning stages. As such, BIO appreciates AHRQ's efforts to seek public input on priority conditions, key questions, and draft reports under the Section 1013 Effective Health Care program.

However, BIO has specific concerns regarding the key questions related to the effectiveness of certain off-label uses of recombinant Factor VIIa. BIO has reviewed the list of priority conditions and believes that certain aspects of AHRQ's five research questions reach beyond the scope of the priority conditions to include diseases that stakeholders have not identified as a priority. For example, AHRQ is seeking input on patients with trauma-related hemorrhagic shock and those undergoing liver transplants, cardiac surgery or prostatectomies, none of which are listed as a priority condition by AHRQ. BIO believes that AHRQ's research should remain focused on the priority conditions and only examine other diseases after receiving stakeholder input through a separate public and transparent process. If AHRQ does decide to deviate from the priority conditions in selecting research topics, which appears to be the case for the recombinant Factor VIIa review, BIO strongly urges AHRQ to provide the public with its rationale for focusing on non-priority conditions. Providing such justification would increase the transparency and openness of the topic selection process, which is critical to enhancing the credibility of any comparative effectiveness research program.

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[2] MMA §1013(a)(2)(A).

[3] The list of ten conditions is available at: http://effectivehealthcare.ahrq.gov/aboutUs/index.cfm

II. BIO Urges AHRQ to Focus on Broad Healthcare Delivery Issues and Multiple, Defined Interventions When Conducting Effective Health Care Research Reviews.

Although AHRQ is requesting comments on the draft key questions for a research review on recombinant Factor VIIa, BIO notes that the MMA's mandate requires AHRQ to support research on "strategies for improving the efficiency and effectiveness" of healthcare programs, "including the ways in which such [health care] items and services are organized, managed and delivered."[4] BIO believes that this sort of research often is neglected and that AHRQ could play an important leadership role in improving the overall effectiveness of the U.S. healthcare system. Although BIO members and others have incentives to perform research related to specific diseases, often including comparative effectiveness research, the benefits of more research on the efficiency and effectiveness of health care in general are often are neglected. BIO believes that AHRQ should focus more on this general research.

In cases such as this where AHRQ focuses on specific diseases or conditions, the scope of the clinical interventions should be both clearly defined and broad. Here, the first draft key question compares recombinant Factor VIIa with "usual care" in terms of mortality, disability, and treatment-related outcomes. Likewise, draft key question 2 compares potential adverse events associated with recombinant Factor VIIa with adverse events associated with "usual care." BIO believes that this comparison is too vague to lead to any substantive or useful conclusions. In keeping with the approach used in AHRQ's other research reviews, BIO requests that AHRQ clearly define comparators which cover a full range of possible clinical interventions.

III. Conclusion

Although BIO fully supports the goals of AHRQ's Effective Health Care Program, we are concerned by the lack of transparency in research topic selection, particularly given that this proposed research review substantially veers from the priority conditions developed by stakeholders. In addition, BIO urges AHRQ to take a broader approach to research to include the healthcare delivery system as a whole but when focusing on a specific disease or condition to provide clear comparators across a range of interventions. Again, we appreciate the opportunity

[4] MMA §1013(a)(1)(A)(ii).

to submit these comments. If you have any further questions, please feel free to contact me at 202-312-9281.

Respectfully submitted,

/s/

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