Symposium & Workshop Prospectus:

The Molecular Vaccines Interagency Working Group is hosting a three day symposium and workshop entitled **"Molecular Vaccine Development: Perceived Barriers / Potential Solutions."** The meeting will be held 29-31 January, 2008. Attendance will be limited to 120 individuals. This exciting symposium includes a unique workshop that will be the first-ever of its kind.

The goal is to bring together representatives from the pharmaceutical industry, biotechnology companies, academic institutions and the federal government to examine the developmental pathway for molecular vaccines, from preclinical studies through licensure, to identify solutions to challenges that slow expeditious and timely development of safe and effective novel vaccine candidates. The symposium will explore actual case histories of vaccines that failed to succeed along the Critical Pathway due to inability to meet "go" criteria, such as failed safety tests, insufficient potency, difficulty qualifying a cell substrate, or inability to scale up manufacturing processes, as well as 'no go" decisions based on barriers perceived to lie ahead even if they have not actually occurred -- e.g. the possibility of rare adverse events and associated liability arising in the post-market phase -- that discourage the initiation of a vaccine development project. Although the meeting will focus on scientific challenges, other categories will also be considered, such as regulatory challenges associated with novel technologies, intellectual property concerns associated with business agreements to develop prime-boost regimens, difficulties faced by academia in partnering with industry, and any additional challenges brought to the table by participants. The symposium will also include technologies being developed for veterinary vaccines that may have applications for human vaccines and diseases.

Because the mission of the Center for Biologics Evaluation and Research (CBER) is to promote public health by facilitating the development of safe and effective vaccines, the agency will be attending the meeting in observer status and is vitally interested in the dialogue and insight this symposium will provide. For example, are there molecular technologies that can be facilitated with development of an assay, a qualified standard, or a biomarker? To promote this dialogue, the first two days will be devoted to presentations (see below), and then on the third day, participants will interact in small workshops designed to identify innovative solutions to the challenges facing molecular vaccine development, especially those of cross-cutting concern, and to list concrete steps that could overcome these challenges.

The outcome of the symposium and workshop will be a report for publication. Its core will be the individual reports from the breakout sessions held on the third day, as authored by breakout session leaders, identifying challenges and innovative solutions. The organizing committee, comprised of representatives of the federal government, will use this material to identify overarching issues and generate a list of key innovations. These will be presented to the Molecular Vaccine Interagency Working Groups' parent committee, the White House Subcommittee on Biotechnology, and to the FDA Vaccines and Related Biologic Products Advisory Board as a Critical Path initiative.

Definition of Molecular Vaccines: The term "molecular vaccines" is used broadly to include novel vaccine technologies, including genetic vaccines (in which the genetic material encoding an antigen is delivered, rather than the antigen itself, such as plasmid- or virus-vectored vaccines) and other novel vaccines and vaccine-related technologies developed using molecular

techniques, such as gene-deleted vaccines, reverse genetics, cDNA clones, or expression systems for recombinant proteins.

Instructions for Participants: This meeting is modeled on a highly successful symposium and workshop conducted in Ames, Iowa, in December 2006, also co-sponsored by the Molecular Vaccines Interagency Working Group[*]. The keys to the success of this earlier meeting were the small size, the juxtaposition of individuals working in diverse fields, and the dialogue generated by the breakout sessions as participants reviewed the information provided by the speakers and attempted to clarify the nature of scientific challenges facing vaccine discovery research (the theme of that meeting) and to devise concrete solutions. *To achieve this creative atmosphere, all speakers and participants are requested to stay for the entire meeting, and to attend every session.* At the end of the second day, in preparation for the workshop, each participant will be given time to formulate ideas to share during the breakout sessions.

Instructions for Speakers: Each speaker will have thirty-five minutes of time, and should plan twenty-five minutes for the presentation and ten minutes for questions and discussion. Either of two approaches is recommended. The first is to tell the story of one or more molecular vaccines for which development encountered significant, or even fatal, challenges. Please explain exactly what problem motivated the "no go" decision. Had the problem been foreseen? Were steps devised to circumvent the problem for subsequent vaccine candidates? Were there ripple effects from this event that affected priorities or frameworks for decision making? The second approach is to describe general policies or scientific assessments reached by a vaccine development enterprise regarding molecular technologies and their suitability as vaccine platforms. Is there a concept of red light, amber light and green light technologies? Which technologies fall into which category? What are the factors leading to this classification? What problems need to be overcome to shift the balance in the green light direction?

Speakers will be asked to identify the approach they are taking several months prior to the meeting, and, if possible, the nature of the barriers that they plan to illustrate. This will facilitate organizing the speakers into coherent topics during the initial two day symposium. As a straw dog agenda (see below), sessions have been organized into scientific challenges, safety challenges, partnership challenges, regulatory challenges, manufacturing and process development challenges, and intellectual property and legal challenges. However, these categories will be amended as appropriate, once input from speakers is received.

Speakers will be drawn from the pharmaceutical industry, biotechnology companies, academic institutions and the federal government, as all these institutions play a critical role in developing novel vaccines and vaccine technologies.

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[*] Report of the United States European Commission Workshop "Advances in Immunology and Vaccine Discovery," Ames, Iowa, 12-14 December, 2006: http://ec.europa.eu/research/biotechnology/ec-us/docs/eu-us_annual_meeting_immunology_vaccines_report_04-22-2007_en.pdf