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Creating Tomorrow's Vaccines

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**U.S. Senate Committee on Appropriations**
**Hearing on U.S. Government Response to Ebola Outbreak**
**Testimony: Novavax, Inc.**
**Wednesday, November 12, 2014**

Chairwoman Mikulski, Ranking Member Shelby, Members of the Committee, thank you for the opportunity to provide written testimony on behalf of Novavax, Inc. regarding our efforts to respond to the current Ebola crisis by rapidly advancing the development and production of a promising vaccine candidate.

Novavax is a clinical-stage biopharmaceutical company based in Maryland, focused on the discovery, development and commercialization of recombinant nanoparticle vaccines and adjuvants. Our principal vaccine candidates currently in clinical development include the world's most advanced vaccine for respiratory syncytial virus ("RSV") and vaccines against seasonal influenza and pandemic influenza, all of which are in Phase 2 clinical trials.

At Novavax, we use our technology to produce vaccine candidates that can rapidly respond to emerging diseases. For example, under our $179 million HHS/BARDA contract, we have developed and delivered compelling safety and immunogenicity data in humans for vaccines against two pandemic influenza strains: H5N1 and H7N9. We also have been monitoring other emerging diseases, such as Middle East Respiratory Syndrome (known as "MERS"), a novel coronavirus first identified in September 2012, as well as the recent outbreak of Ebola virus disease in West Africa.

It is worth noting that five (5) strains of Ebola have been identified, and that the strain currently afflicting West Africa is known as the "Guinea strain", which was identified in August 2014. With the speed and flexibility of our platform, we have been able to customize our vaccine to the currently circulating strain. While current publicly known vaccine approaches target earlier strains of the virus, Novavax' Ebola vaccine candidate is the first, and currently only, reported Ebola vaccine produced using the genetic sequence of the Guinea strain. Our Ebola vaccine has recently been successfully tested in both rodent and rabbit pre-clinical models. We have also tested the vaccine with our Matrix-M™ adjuvant in these same pre-clinical models, with results showing that Matrix-M appears to significantly contribute to enhanced immunogenicity and induction of neutralizing antibodies.

Due to the urgent global public health need for an Ebola vaccine, Novavax feels that it important to further develop our Ebola vaccine candidate. Therefore, we recently publicly announced our initiation of a non-human primate study and our expectation to initiate a Phase 1 clinical trial in December 2014 that will evaluate the safety and immunogenicity of our vaccine candidate in ascending doses, with and without our Matrix-M adjuvant. Subsequent clinical studies will be designed following the data from the non-human primate study and the Phase 1 clinical trial.

Despite heroic efforts by numerous global health agencies and product developers, development of vaccination strategies for emerging pathogens like Ebola are particularly challenging because of the sudden emergence and rapid dissemination of such diseases, as well as the long process of traditional vaccine development. Although the U.S. government is working with health agencies and industry players to stream-line and fast-track regulatory requirement, the U.S. government should also prioritize appropriations for vaccine platform technologies that are rapidly adaptable to novel and re-emerging pathogens. Novavax offers the following recommendations to the U.S. government:

1. **Invest in Flexible, Rapid and Proven Vaccine Technologies**. An effective response requires willingness to adopt vaccine technologies that demonstrate flexibility to make the first doses available on a rapid timeline from the identification of pathogen to first doses available.

   This approach would also minimize time and investment dollars in future emergencies. For example, Novavax' technology allows us to monitor reports concerning emerging diseases and, as soon as the genetic sequence of the pathogen is published, proceed from gene to human clinical data in comparatively short periods of time.

   Novavax demonstrated this capability last year in response to the H7N9 pandemic influenza strain, which was first recognized by Chinese health authorities as a potential pandemic influenza threat in late March 2013. In a three-month period, we developed a vaccine antigen, conducted multiple animal studies and initiated the world's first Phase 1 clinical trial of a vaccine against an H7N9 influenza strain. Our clinical results were published in November 2013 in *The New England Journal of Medicine*, where we showed that we achieved protective levels from vaccinations within just 116 days of the announcement of the H7N9 outbreak.

   Similarly, we have cloned the current Guinea strain of Ebola now circulating in West Africa, and we expect to be in a clinical trial next month (December 2014). Our platform affords the U.S. government the opportunity to address not only Ebola, but other emerging infectious diseases, with a timely response that we believe can minimize future risks, as well as expenditures, for the benefit of citizens of the United States and the world.

2. **Invest in Scalable Technologies**. Technology that can rapidly produce the first doses of vaccine is critical, but effective vaccine platforms must also be rapidly scalable to produce sufficient vaccine to move through the development process and be deployed for effective use. As was seen with the antibody therapeutic developed by ZMapp, promising solutions can be handicapped by the lack of expandable production processes. The Novavax platform uses insect cell culture and disposable manufacturing equipment, both of which have proven to be rapidly scalable, to produce thousands up to potentially millions of vaccine doses within a matter of months.

3. **Continue to Invest in Adjuvant Technologies to Expand Supply and Broaden Protection**. Vaccines for highly pathogenic viruses, such as Ebola, should be made rapidly and in conjunction with the use of adjuvants. Adjuvants are immunostimulating molecules, designed to do one or more of the following: (1) improve the immunogenicity (i.e., to increase protection), (2) provide antigen dose-sparing (i.e., lower antigen doses increase number of doses available for distribution), and (3) broaden the scope of protection (e.g., cross-strain protection) as pathogens change and mutate. Several NIH and BARDA funded programs for pandemic influenza vaccines have demonstrated the significant dose-sparing potential of adjuvants when combined with vaccines. Novavax' own pandemic influenza program has demonstrated that certain adjuvants provide both immunogenicity and dose-sparing benefits compared to unadjuvanted vaccines. Moreover, Novavax pandemic influenza vaccines have demonstrated that certain adjuvants contribute to the broader cross-reactivity to different strains of pandemic influenza.

   Given the concern about manufacturing sufficient numbers of doses in a timely manner, Novavax recommends that the U.S. government support the development of Ebola vaccines with dose-sparing adjuvants so more vaccine doses can be available in less time.

Novavax has demonstrated the ability to rapidly and successfully advance vaccine candidates from gene sequence to clinical trials. We are currently committing our own human and technological capital in order to demonstrate our Ebola vaccine is worthy of consideration as a possible solution for this immediate health crisis. In addition, our platform technology can provide cost-effective and timely solutions to address future emerging disease threats.

Such efforts necessitate that the U.S. government ensures that emergency programs for Ebola products continue to be funded, but at the same time the U.S. government should evaluate the available technologies to address its longer-term needs to develop solutions to future emerging threats. Novavax agrees with BIO that to prepare for the full range of potential threats, we must prioritize funding for Project BioShield, BARDA, pandemic influenza, the SNS, and other programs that are essential to public health preparedness this year and in coming years. These programs simply cannot be funded only after a disaster hits; such reactionary thinking would put lives at risk. The U.S. biotechnology industry is uniquely capable of advancing early scientific innovations to products that can be safely and effectively used, and it is time to make the necessary investments to address Ebola and other newly emerging, novel infectious diseases with investments in companies capable of making new vaccines, such as Novavax.