August 11, 2014

The Honorable Fred Upton (R-MI)
Chairman
Committee on Energy and Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette (D-CO)
Member
Committee on Energy and Commerce
U.S. House of Representatives
2368 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Rep. DeGette:

On behalf of the National Venture Capital Association (NVCA) and our nearly 400 members, I want to thank you for your leadership on the issue of medical innovation and the launch of the 21st Century Cures initiative. We appreciate the judicious manner in which you are approaching this important reform effort. There is no simple solution to close the gap between the rapid advancement in the field of medical research and the many factors that hold back innovation in the development of innovative new medicines and medical devices. Your approach to listen first and then act will ensure this initiative is a success.

As you have acknowledged repeatedly throughout this process, it's not enough to simply look at one stage of the medical innovation arc; you need to examine the full arc—from discovery to development to delivery—to really get to the root of the issue. We believe that by taking a holistic look at each individual stage of the medical innovation process, you will ensure the greatest chance for success.

In many ways, the fate of the U.S. medical innovation ecosystem rests in your hands. If more is not done to improve the drug and device development process and modernize our regulatory and reimbursement systems to keep pace with the rapid advancement in medical research, the U.S. is at risk of losing its leadership in medical innovation.

NVCA believes it is critical to advance public policies that will encourage investment in medical innovation so new treatments and cures will be available to patients. Central to doing so is to continue to reform our regulatory system to make it agile and flexible to the changing pace of innovation as well as develop a predictable and transparent reimbursement system that pays for innovative medical products that provide value to patients and the overall healthcare system.

We appreciate your acknowledgement of the venture capital industry as an important stakeholder in this discussion and your understanding of the critical role venture capital plays in the advancement of medical innovation in the U.S. Over the last three decades, venture capital has been the primary force in translating scientific discoveries into medical advances for patients and remains one of the few sources of capital to fund and nurture small, emerging companies focused on medical innovation.

However, venture capital investment in early-stage life sciences companies has been facing significant pressure in recent years. In fact, in 2012 the number of first-time financings of new life sciences companies hit a 15 year low. A primary reason for this long-term decline in financing for medical innovation has been the increased time, cost and uncertainty involved in developing new drugs and medical devices. In 2013, early-stage venture financing of life sciences companies rebounded somewhat, although it remains well below the levels needed to keep pace with advances in science and medical research. An important reason for this recent rebound is the improved regulatory environment for innovative product development, which is related to the Food and Drug Administration Safety and Innovation Act of 2012. This highlights the critical role that this committee can play in advancing public policy initiatives that help encourage investment in medical innovation.

Regulatory and reimbursement policies have a major impact on the flow of private investment capital. Against this backdrop, the following are NVCA's high level recommendations on how to encourage investment in the innovative drugs and medical devices of the future. We look forward to working with you to develop specific and more granular concepts based on these recommendations.

Make medical innovation a national priority

- Create a national advocacy strategy focused on preserving U.S. leadership in medical innovation.

Continue to fund basic science and applied R&D

- Support continued government funding for basic research and development which drives the discovery of breakthrough innovations with the potential to cure disease and treat unmet patient needs.

Provide appropriate incentives for collaborative public/private partnerships that can help address key barriers to innovation

- Encourage and support continued FDA efforts to implement a patient-centered benefit/risk framework for drug and medical device development.

- Encourage the work of the Medical Device Innovation Consortium (MDIC) and other public-private collaborations to improve regulatory science and enhance drug and medical device development.

Develop novel, interactive and flexible regulatory models for disruptive innovation

- Ensure that the FDA has the resources and the mandate it needs to fulfill its dual missions of protecting patient safety and encouraging medical innovation.

- Continue to develop flexible and innovative regulatory pathways for cutting edge drugs and medical devices.

- Create new approval pathways, such as the proposed Special Medical Use pathway, that enable the development of drugs and medical devices for subpopulations of patients in areas of high unmet need.

- Review and ensure the effectiveness of FDA's Special Protocol Assessment (SPA) process.

- Support the use of innovative clinical trial designs, including the use of adaptive trials, biomarkers, and single-arm clinical trials with historical control groups under appropriate circumstances.

Provide greater clarity, transparency and flexibility in the regulatory process for laboratory developed tests (LDTs) to encourage investment and development of personalized medicine that will provide value to patients and the healthcare system

- Develop clarity, transparency and flexibility in the regulatory process for LDTs that will keep pace with scientific advances and genomic science.

- Organize a partnership between government and the private sector to align the common interest in advancing the improvement of patient care using precision or personalized medicine.

Provide greater transparency and use of clinical trial data

- Explore the opportunity to unleash the power of information by publicly releasing FDA submissions and correspondence between companies and FDA, with a mechanism to redact legitimate trade secret information. This will provide a heightened level of transparency that will allow better drug and device development decisions and more efficient clinical trials, and will help ensure that investment flows to the most promising areas of drug and device development.

- Facilitate innovators' access to post-market clinical data in patients' electronic health records.

Work to integrate real world data into the drug and medical device review process

- Achieve the appropriate balance of pre- and post-market data requirements for regulatory approval that encourage the development of innovative products.

Develop coverage and payment policies that reward investment in medical innovations that provide value both to patients and the healthcare system

- Develop a set of principles on how to evaluate the value of innovative medical technologies.

- Promote coding, coverage and payment policies that support innovations that can improve the cost, quality and outcomes of medical care.

Provide greater patent and intellectual property incentives for medical breakthroughs

- Extend data exclusivity period in areas of unmet need.

- Assure that efforts to reform the patent system to deal with "patent trolls" do not undermine the need of legitimate patent holders for protection of their intellectual property.

Summary

The success in the initiatives we have outlined above will help ensure that patients get access to innovative new treatments and cures, and will help cement U.S. leadership in medical innovation and generate economic growth and high-quality jobs across the U.S. for decades to come. We look forward to being part of the solution.

Sincerely,

Bobby Franklin
President & CEO

Cc: House Energy & Commerce Committee Members