



February 27, 2013

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

Re: Docket No. FDA-2012-N-1248: Creating an Alternative Approval Pathway for Certain Drugs Intended to Address Unmet Medical Need; Public Hearing; Request for Comments

Dear Sir/Madam:

The National Venture Capital Association's (NVCA) Medical Innovation and Competitiveness (MedIC) Coalition, a partnership between NVCA member venture capital firms, entrepreneurs, and early-stage portfolio companies, appreciates the opportunity to provide comments on the Food and Drug Administration's (FDA) draft proposal that would create an alternative approval pathway for certain drugs intended to address high-unmet medical need populations, also referred to as a Special Medical Use (SMU) designation.

The NVCA is a trade association representing the approximately 400 venture capital firms in the United States, with a mission to foster greater understanding of the importance of venture capital to the U.S. economy and support entrepreneurial activity and innovation. NVCA's MedIC is comprised of NVCA member firms and their life sciences portfolio companies, and advocates for policies and regulations that advance and support U.S. medical innovation. MedIC seeks to 1) educate policymakers on the critical role America's medical innovation plays in the U.S. healthcare system, 2) establish incentives for investors and entrepreneurs to drive medical innovation in the U.S., and 3) develop policies that will reduce the time, cost and risk of drug and medical device development that will help increase investment and drive innovation.

Background

Over the last three decades, the U.S. has been the global leader in the advancement of medical innovation. Many scientific discoveries have created novel insight into mechanisms of certain diseases and have provided important cures and treatments for patients. New scientific breakthroughs in human genetics, molecular biology, nanotechnology and other areas show even greater promise for patients in the generations to come.

Venture capital has been a primary driver for advancing scientific discoveries into medical products for patients and remains one of few sources of capital that fund and nurture early-stage companies focused on developing new cures and treatments. The majority of new drugs and medical devices that serve significant unmet medical needs have had roots in venture-backed start-up companies.

Unfortunately, venture capital investment in early-stage life sciences companies has been facing significant pressure in recent years. In fact, in 2012, the number of start-up life sciences companies receiving venture capital investment for the first time dropped to the lowest level in over 16 years. A primary reason for this decline is the increased time and cost of developing new drugs and medical devices. Long timelines, rising costs and risks have caused the number of innovative drugs being developed to decrease significantly and will adversely impact the availability of important new therapies for patients. This points to the urgent need for new approaches to drug development that can speed beneficial therapies to the patients who need them most.

NVCA/MedIC supports the concept of a Special Medical Use (SMU) Pathway

NVCA/MedIC supports the concept of creating the SMU pathway. As described in the presentation by Dr. Jim Healy, Managing General Partner, Sofinnova Ventures, at FDA's public hearing on February 4, 2013, . [As described in the presentation by Dr. Jim Healy, Managing General Partner, Sofinnova Ventures, at FDA's public hearing on February 4, 2013](#), we see the potential for this pathway to accelerate the development and availability of important new treatments and cures for many diseases across a broad range of therapeutic areas. Some have suggested that the pathway could be limited to the area of antibiotics. While we recognize the important role this pathway could play in the development of badly needed new antibiotics to treat resistant infections, we strongly believe that the SMU pathway should be available for a broad range of therapeutic areas and that limiting it to antibiotics would result in a missed opportunity to advance innovative medicines to patients suffering from many other serious diseases. Examples of potential disease areas that could be positively impacted by the SMU pathway include anti-infectives, central nervous system diseases such as Alzheimers, autoimmune diseases such as lupus, and metabolic disorders such as obesity and diabetes.

NVCA/MedIC believes the SMU pathway should be voluntary and at the sponsor's discretion. It is also important that the SMU designation can be obtained early in the pre-clinical development process to ensure the sponsor can design appropriate clinical studies for use under the new pathway. Finally, it is important that SMU be designed as a tool for better communicating the benefit-risk of a given therapy in the targeted population for which it is intended, to ensure that healthcare providers have the best information about appropriate use at their disposal, but that neither FDA nor sponsors be put in a position where they are expected to police the practice of medicine or constrain the judgment of medical professionals in treating individual patients.

NVCA/MedIC greatly appreciates the efforts by FDA to continue to seek ways to safely and responsibly speed innovative therapies to patients in need and reduce the time and cost of development. NVCA/MedIC believes the SMU pathway, like Accelerated Approval and the Breakthrough Therapies Designation, is an example of how FDA is working collaboratively and proactively with all constituencies to design appropriate regulatory tools to help meet the needs of patients and modern drug development.

Conclusion

NVCA/MedIC believes that modern drug development requires FDA and sponsors to have a range of tools at their disposal to customize drug development programs to specific situations and patient populations. We thank and congratulate FDA for continuing to think creatively and

expansively about how to evolve the regulatory process for new drugs as science advances. The SMU pathway, which could be used to encourage the most efficient and responsible development programs in targeted high-unmet need subpopulations while helping to protect broader populations from inappropriate use, is an example of a modern regulatory tool that could serve patients and the innovation community well.

We look forward to being part of an open dialogue with all stakeholders to develop a balanced framework that incorporates the appropriate elements to ensure the SMU pathway benefits patients by accelerating innovative new treatments and cures to those who need them most.

Sincerely,

Jonathan S. Leff
Partner, Deerfield Management
Chairman, Deerfield Institute
NVCA Board Member and
MedIC Chairperson