March 1, 2013

Margaret A. Hamburg, M.D.
Commissioner
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD  20993-0002

Re:  Creating an Alternative Approval Pathway for Certain Drugs Intended to
Address Unmet Medical Need

Dear Dr. Hamburg:

On behalf of the Ovarian Cancer National Alliance (Alliance), we thank you for the opportunity
to comment on the Food and Drug Administration’s (FDA’s) consideration of the development
of a potential new pathway to expedite the development of prescription drugs for serious or life
threatening conditions to address an unmet medical need. The Alliance advocates for policies,
programs, and investments to support the development of an early detection test; improved
health care practices; access to life-saving therapies; and improved awareness among health
professionals and the public of the risks and symptoms of ovarian cancer. Too many women are
unaware of their risk of developing ovarian cancer; tens of thousands each year are diagnosed
too late and, therefore, lose their lives unnecessarily. It is our mission to improve these
outcomes.

Ovarian cancer is the deadliest gynecological cancer. According to estimates from the American
Cancer Society, in 2013 approximately 22,240 new cases of ovarian cancer will be diagnosed and
15,500 women in the U.S. will die from ovarian cancer. Ovarian cancer mortality rates have
remained virtually unchanged for nearly 40 years, while we have seen improvements in many
other cancer types.

The Alliance supports and encourages research and development that will treat and/or prolong
the life of women diagnosed with ovarian cancer. To that end, we are pleased to submit
comments regarding the FDA’s efforts to create a new alternative approval pathway for
treatments to address a serious or unmet medical need – such as unchanged mortality rates for
ovarian cancer. Additionally, we recognize that U.S. drug approval remains the world’s gold
standard, and we support the FDA’s commitment to its primary mission of ensuring that
prescription drugs are safe and effective for their intended use.

Need for Alternate Pathway

The Alliance agrees with many of our colleagues in the oncology advocacy community, that the
FDA should provide additional guidance regarding existing regulatory mechanisms and their
relationship to each other. We suggest that FDA develop a document that describes and
differentiates: 1) fast track designation, 2) accelerated approval pathway, 3) priority review, and
4) breakthrough therapy designation. A guidance of this sort would educate sponsors about the
pathways that may be available to them and might also serve to answer patient and provider questions about the need for the alternative approval pathway. We believe that this agency effort would answer questions about the need for the alternative pathway.

**Safety**

As the FDA considers the development of the alternative pathway, the Alliance believes its focus should remain in safety and efficacy of the products submitted under the new pathway. At the FDA public hearing held on February 4, 2013, some presenters raised the possibility of providing manufacturers with additional periods of exclusivity if they seek product approval under this new pathway. The Alliance believes that additional periods of exclusivity are unwarranted and may require legislative action. Moreover, additional periods of exclusivity likely could increase the overall cost of the treatments for patients, thus threatening individuals’ access to these treatments.

The new pathway could involve smaller and more rapid clinical trials. While the Alliance sees the value in such approach, we would strongly encourage the FDA to include additional post-marketing approval for new indications and/or populations and monitoring requirements on drugs approved under this new pathway. Because these products will not have undergone the same safety and efficacy tests as products approved under the traditional pathway, we strongly urge the FDA to provide additional monitoring to ensure the long-term safety and efficacy of these products.

**Outcomes**

As the FDA defines the parameters for the new pathway, the Alliance encourages the agency to consider different end-points and effectiveness criteria/standards for ovarian cancer therapies, generally. For example, a therapy that may not extend life longer than existing therapies but which helps preserve fertility or provide additional quality-of-life may be an important addition to the available cancer therapeutic regimens.

**Education**

Finally, because products approved under this pathway will have undergone a new and different approval process, it is imperative that health professionals and patients are properly informed of the risks and benefits associated with the approved therapy. This education should include general information regarding any possible limitations of the approval process as well as any specific known information about the efficacy of the drug. Health professionals need to be made aware themselves of the particulars of drugs approved under the new pathway so they can first make the best decisions for their patients and, in turn, communicate information about the drug to their patients.

Education should come in a variety of forms. While prescription drug labels are one source of information, most patients do not read (or understand) prescription drug labels. We urge the FDA to develop materials to help educate patients about these new treatments and work with health professionals to disseminate and communicate this information. The FDA should convene all stakeholders to solicit input regarding how best to convey information about drugs approved under the new pathway. The Alliance stands ready to work with the FDA to ensure that health professionals and patients have the information they need – in modalities and language appropriate for each audience – so they can make informed treatment recommendations and decisions.
Utilization

We join the Cancer Leadership Council in raising concern regarding the recommendation that drugs approved according to the alternative pathway could not be prescribed for off-label use. As an evidence-based organization, we support off-label uses that are medically appropriate, for example, contained in compendia listings or on the basis of the scientific literature.

We join our colleagues in our concern about the use of a special logo or labeling that might be interpreted as representing a different review standard under the alternative pathway. Third-party payers use aggressive tools, including formulary restrictions and utilization limits, to control prescription drug expenditures. We are concerned that third-party payers would embrace the suggestion of a different standard of review (or labeling that hints at a different standard) to limit payment for drugs that have been reviewed according to the alternative pathway.

Thank you again for the opportunity to submit comments on the FDA’s consideration of the development of a potential new pathway to expedite the development of prescription drugs for serious or life threatening conditions to address an unmet medical need. If you have any questions, please do not hesitate to contact us.

Sincerely,

Calaneet Balas
Chief Executive Officer