

Statement of Mara Aspinall, Genzyme Genetics

Food and Drug Administration Public Meeting on
In Vitro Diagnostic Multivariate Index Assays

February 8, 2007

I am Mara Aspinall, President of Genzyme Genetics, a worldwide provider of reproductive and oncology diagnostic testing, as well as genetic counseling services. As a business unit of Genzyme Corporation, Genzyme Genetics employs over 1,500 people and has eight laboratories located across the United States and operations in Europe and Japan.

I appreciate the opportunity to talk today, to focus on one aspect of the impact of the draft IVDMIA Guidance.

Genzyme greatly appreciates that FDA, in response to a number of expressions of concern about the Draft Guidance and to glean more information about this important issue, has taken two significant steps. First, the deadline for comments on the Draft Guidance was extended, to allow stakeholders additional opportunity to analyze the matter and formulate their responses. And, second, the Agency has convened this meeting. These two steps are necessary, we believe, to begin the dialogue that is essential to ensuring that any new regulatory approaches are necessary and appropriate and, more importantly, that any changes improve physician and patient access to and confidence in diagnostic testing critical to appropriate health care.

Thank you for taking these two steps; they are both very helpful. However, because of legal, administrative, time, and other constraints, neither a public docket nor a public meeting such as this can provide the optimal opportunity for a real exchange of information, including scientific information, between FDA experts and those outside the Agency. We therefore urge the Agency to convene, before any guidance or new requirements are finalized and with an independent third party if appropriate, a workshop or similar format where interactive dialogue can occur. Such a format would provide an opportunity for those who will be affected by the Agency's proposals – patients, physicians, laboratories, and others -- to engage in an open dialogue with you about the details that are so important in developing such guidance. Importantly, a truly interactive format would allow us to hear your responses and reactions – in real time and on an issue-by-issue, question-by-question basis.

We look forward to such a further opportunity. In this statement, I want to focus my brief comments on one concern among a number that are being mentioned today. That issue is the potential impact of the Draft Guidance on innovation and, thus, on timely physician and patient access to the most up-to-date and newest science and technology.

Health care today is moving from what I like to call “traditional medicine” to Personalized Medicine. Personalized Medicine requires more specific information about the detailed health status of a patient and will require more and more targeted tests in smaller and smaller patient populations. To fulfill the promise of Personalized Medicine, we will need to ensure that innovation is possible and is fostered in the laboratory-developed-test part of the diagnostics industry. If we fail to do that, we will be unable to carry out our unwavering commitment to excellent patient care and patient safety.

Toward the goal of highlighting that point, I want to take a moment to talk about what laboratory-developed tests are, how they come into use, and the benefit they bring to the physicians who order them and the patients being treated by those physicians. These tests often have their beginnings in academic centers -- in research that results in scientific publications about, for example, the usefulness of particular biomarkers or assays. Academic centers then look to independent laboratories to make these tests available to the relevant patients. In some cases, laboratories themselves develop tests based on scientific and medical information -- in the literature or presented at scientific meetings or conferences -- indicating the utility and importance of the tests.

Laboratories validate the tests, ensure the scientific underpinnings are robust, and develop processes that guarantee the tests will be reproduced accurately and that they are appropriate to offer to physicians. Tests are developed and validated under the direction of board-certified pathologists and clinical scientists. In every case, physicians make the final choice about which validated test is appropriate for a particular patient and ensure that each test is medically necessary. Physicians make decisions regarding specific tests based on patient need, their own clinical knowledge, and information from the medical and scientific literature.

Typically, unless and until a new diagnostic test reaches a critical and relatively large volume, no commercial test kit can be developed. Lacking that critical volume, there is no market incentive to develop a kit and spend the resources required to take the kit through a full FDA process.

The bottom line is that for conditions that affect relatively small numbers of patients, or subpopulations of patients, the only access to valuable and necessary testing will be through laboratory-developed tests. And, just as balancing expenditures with potential returns on investment may dissuade a company from developing a test kit for a small market, financial realities will apply to laboratories as they consider developing innovative, cutting edge tests. Extensive and costly regulatory requirements would serve as an extremely strong disincentive to the development of tests such as those for rare genetic disorders, orphan diseases, cancers that affect targeted subpopulations, etc.

Why is this the case? It very simply comes down to this: the current reimbursement system does not compensate laboratories adequately even now. The added costs associated with an FDA clearance or approval would be impossible to recoup. The end result would be that laboratories could not afford to develop new tests. Diagnostic testing – a key piece of moving forward in Personalized Medicine -- would suffer enormously;

physicians would have seriously limited access to important, cutting-edge information that would help them determine the best course of treatment for their patients; and, above all, patients would lose.

As we look to the future, we envision that many of the complex new tests Genzyme Genetics will develop will serve a very specific and relatively small population of patients. Many of these tests are expected to be in the area of oncology patient management and will provide critical diagnostic information essential to selecting the most appropriate available therapies for each patient. We also believe that most of such tests will meet the definition – as currently included in the Draft Guidance – of an IVDMA that potentially would require additional regulation and/or costly FDA pre-market approval. Because these tests are, truly, in the realm of Personalized Medicine, the market for them will be small. Even currently, the reimbursement system presents a challenge to laboratories making decisions about investing in new tests. An additional level of regulation would make such investment virtually impossible. Because we believe that each patient, and each physician, deserves access to the important information provided by these kinds of tests, we also believe that the regulatory system should not be one that promotes the development of only high-volume testing.

Our message is this: if you determine that additional regulation in this area is absolutely essential, please ensure that all the information and facts are thoroughly vetted and fully considered before proceeding. Please, as you are determining the way forward, look at the costs and the facts about reimbursement because this, together with increased regulation, could stifle the innovation needed for the future of Personalized Medicine. And finally and most importantly, we ask you to consider fully the implications that derive from this information for physicians and their patients.

Again, thank you for the opportunity to participate at this meeting and in the comment process for this Draft Guidance.