Public Comments

DR. WILLARD: We now have our public comment session. As Reed Tuckson noted yesterday, one of our critical functions at each meeting is to serve as a public forum for deliberations on the whole range of health and societal issues that are raised by the development and use of genetic and genomic technologies. We set aside time each meeting and each day to hear from the public, and that's what we'll do now.

We have two speakers, and in the interest of our full schedule and the fact that we're tight on that schedule, I'd ask the commentators to keep their comments to five minutes, and if you have written comments, to please give us a copy of those so they can be entered into the permanent record.

Our first speaker is JoAnne Glisson from the American Clinical Laboratory Association.

If you would just come to the front, there's an open seat there. Welcome. Thank you for joining us.

MS. GLISSON: Thank you for having me.

ACLA is an association of independent clinical laboratories, national, regional and local laboratories. Our members include large reference labs and small focused, esoteric labs. Independent laboratories and the laboratory-developed tests they develop and perform represent a key constituency in the development of this exciting new technology. We look forward to working with the committee as you continue your consideration of the issues associated with pharmacogenomics and its promise.

Thank you.

DR. WILLARD: Thank you. I appreciate your brevity.

Any questions or comments from the members of the committee?

DR. WINN-DEEN: I just want to make a comment on behalf of the group that tried to put the program together today. We didn't in any way mean to slight the reference laboratories that are doing lab-developed tests, and we recognize the valuable role that you're playing in this field. There just simply wasn't enough time on today's program to hear from all constituencies. We certainly would like to reserve the right to call on you for a future meeting.

MS. GLISSON: Thank you.

DR. WILLARD: Other comments from the committee?

(No response.)

DR. WILLARD: If not, thank you very much.

Our second speaker is Robert Yocher, who is vice president of regulatory affairs at Genzyme. Welcome and thank you for joining us.

MR. YOCHER: Thank you. Thank you for the opportunity to comment on the exciting topic of pharmacogenomics.

We at Genzyme believe we are uniquely positioned to discuss this as a biotechnology company and who develops unique therapeutic products for unmet medical needs; and also as a laboratory service provider of genetic tests and clinical pathology.

The age of pharmacogenomics has started, but it's at its earliest stages, and like all science in its early formative years, the process is truly iterative. While there has been a handful of notable successes, for the drug companies in the pipeline now, it's really only the earliest few drops out of the pipeline. Most of the fruits of our efforts will not be realized for seven to ten years from now.

However, the agreement on the systems and the understanding of what the requirements are for the realization of targeted therapeutics which are now defined by pharmacogenomic testing, need to be in place now. Therefore, Genzyme believes the following are necessary strategies to understand the realization of the full potential of pharmacogenomics.

First, we believe there needs to be a broad coordinated effort necessary integrating pharmacogenomics as this is a paradigm shift. All of key constituencies within the health care system need to understand the role of pharmacogenomics. There should be education of physicians and other providers to get them on board and thinking about it. There needs to be education of payers. Education is necessary on a number of levels for the foundation of pharmacogenomics as a concept, as a benefit to patients, and benefits to payers.

More importantly to this committee, there needs to be education and coordination of agencies throughout the HHS, FDA for the drug and test development, CDC and CMS for laboratory services, CMS for adequate payment, CDC for education, and NIH for the design of experiments and the new statistical approaches that will be necessary to lead these development technologies.

It's critical that the efforts between the agencies are coordinated, especially as new rules and recommendations are created. We cannot have new rules in one agency which are not consistent with the other agencies. For example, for biomarkers deemed valid by FDA, it should also be accepted by CMS as valid. There should not be two levels of evidence required.

Some other examples. There needs to be a shift in thinking about population means evidence-based medicine to targeted populations and cohort outcomes. The whole classic drug approach has been on centrist, large populations, and now we're looking at truly just the outliers. So there needs to be new statistical methodologies developed.

For instance, a prospective analysis of retrospectively collected samples in biobanks, and validation of these biomarkers. At the recent DIA/FDA meeting, NIH and FDA had a quite interesting discussion and came to no agreement on the process of how to do that. Terminology must also be agreed upon in organizations. Dr. Janet Woodcock stated in her presentation to the DIA and FDA workshop on April 11th of this year that further exploration of the concept of the framework is needed, and reassessment of the ideas of validation, and perhaps even adopting new nomenclature for validation.

We also believe that the government needs to pay to encourage innovation. Innovation is critical to moving the health care system forward. With the fast pace of medicine today, laboratory-developed tests are considered the state of the art diagnostic tests and are often the way that innovation occurs in the laboratory. In many cases, manufacturers will not seek FDA approval

through 510(k)s or PMAs for these products or devices because the routes are either not economically viable because the populations are too small, or especially since the technology is changing so rapidly and the pipeline is so long that by the time you get your test approved, the technology has passed you by, as was mentioned this morning.

For drug manufacturers, it's important to provide incentives such as label extensions or exclusivity for drugs associated with new pharmacogenomic tests to justify the additional development of cost and timelines. But in doing so, the regulatory pathways must be clear, predictable, and easy to implement. For pharmacogenomics to work, we believe that drug manufacturers must understand and recognize the benefit of creation of drugs that will be more targeted to the right patient for the populations, and therefore show better efficacy and safety.

We need to bolster the support of the current multiple approaches to diagnostic access, especially inclusion of laboratory development tests which right at this moment are not discussed in the early FDA models.

We have submitted more details in writing to this committee, but we've covered many of those topics this morning, and we stand here ready to help assist you and volunteer in your efforts going forward.

DR. WILLARD: Thank you very much.

Questions from the committee, or comments?

DR. WINN-DEEN: Are you going to make your written comments available to us?

MR. YOCHER: They have been provided already.

DR. WINN-DEEN: Okay.

DR. WILLARD: Thank you very much. Appreciate that.

We are now at our lunch break. An announcement first for those who will be headed to the airport at the end of the afternoon. You should sign up for airport transportation at the registration desk to facilitate getting out in a timely manner.

For the lunch break, committee members and ex officios, the lunches that we ordered will be just outside, as they were yesterday. For members of the public, lunch is available in the hotel restaurant, as well as other restaurants in the area.

We will reconvene promptly at 1:30 p.m. and continue the session on pharmacogenetics. Thank you very much.

(Whereupon, at 12:27 p.m., the meeting was recessed for lunch, to reconvene at 1:30 p.m.)