BIO
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

TESTIMONY OF HANS SAUER
DEPUTY GENERAL COUNSEL FOR INTELLECTUAL PROPERTY,
BIOTECHNOLOGY INDUSTRY ORGANIZATION
UNITED STATES PATENT AND TRADEMARK OFFICE
PUBLIC HEARING ON GENETIC DIAGNOSTIC TESTING
FEBRUARY 16, 2012

Good morning,

My name is Hans Sauer; I am Deputy General Counsel for IP at the Biotechnology Industry Organization, on whose behalf I testify today. We thank you for giving us this opportunity.

BIO is the nation's largest biotechnology trade association, representing over 1100 companies, academic institutions, and biotechnology centers in all 50 States and in countries around the world. BIO members undertake research and development of biotechnological healthcare, agricultural, environmental, and industrial products. BIO members range from start-up businesses and university spin-offs to Fortune 500 corporations. The vast majority of BIO's members are small companies that have yet to bring products to market or attain profitability, and thus depend on venture capital and other private investment for their growth.

Biotechnology products typically require close to a decade of development work and a fully-capitalized investment in the range of $1.2 billion. Biotechnology companies rely heavily on patents to protect such substantial investments of time, resources, and capital. As a general proposition, devaluation of patent assets leads to a reduced incentive for companies to conduct research, development, and commercialization of new biotechnology products. This is true for new therapeutic products, agricultural products, diagnostic kits, devices, and instruments. It is, we believe, also true for advanced molecular diagnostic services, which require significant investments to procure the clinical data packages necessary for coverage determinations and payor and physician adoption, even though the investments required for their development and commercialization are not as high as those for new medicines or crops.

Only few of BIO's member companies are in the market for advanced diagnostic services. Yet, the PTO's FR notice has generated considerable interest among our membership, many of whom hold so-called gene patents on isolated or purified DNA molecules, on which they rely to protect, e.g. the production technology for recombinant biologic drugs, or recombinant traits for genetically modified crops or microorganisms. Many BIO members have expressed concern that the current public discourse about gene patents takes place exclusively on a

platform of genetic diagnostic testing services and is focused, implicitly, on a single genetic diagnostic testing company. That company is not a BIO member, but we do have member companies that do research on sugarcane or oil palm genes who are incredulous that the validity or propriety of their patents would be drawn into question based on the purported marketplace behavior of a single diagnostic test provider. We trust that the PTO will not lose sight of possible unintended consequences outside the genetic testing area as it develops its policy recommendations from this study. The same is true for any positions the PTO might advocate to the U.S. Government in the context of certain ongoing litigation.

The role of patents in the development, patient utilization and other aspects of advanced genetic tests generally has been studied before without firm conclusions. Most recently, the SACGHS committee issued a report, including policy recommendations, relating to gene patents and genetic testing two years ago. While the report itself and its policy recommendations generated serious controversy (including an openly dissenting opinion), we think of the underlying commissioned research studies as valuable and high-quality contributions to a discussion that had up to that point been dominated mainly by anecdotal reports, opinion surveys, and other "soft" information. The SACGHS-commissioned publications were published as a special supplement to Genetics in Medicine in April 2010 and are made available on the web without subscription. BIO believes these publications will prove valuable to the PTO. But they, too, contain grist for every mill – so these studies will not conclude the never-ending debate about patents and patient access.

Fortunately for the PTO, its mandate is narrower than that. Section 27 of the America Invents Act directs the PTO to conduct a study on ways to effectively provide independent confirmatory genetic tests where gene patents and exclusive licensing exists. Elsewhere in Section 27, the AIA uses the term "second opinion" testing. It is important to avoid the impression that we are all embarking on this study already having agreed that there is in fact a lack of confirmatory testing opportunities, and that an entitlement to such testing would automatically translate into a patient benefit. Indeed, BIO does not believe that these problems have been demonstrated.

The clinical practitioners with whom we spoke told us that it is rare for a patient to ask for a repeat of an advanced molecular diagnostic test, just like it is rare that patients would ask for a repeat of an MRI scan or x-ray. What patients ordinarily mean when they ask for a second opinion is a second medical opinion, a confirmation of the physician's treatment recommendation, and the like. Absent a reason to believe that the original test was defective or unreliable, neither patients nor their physicians would seem to have a reason to ask for a repeat test. And absent a reason to believe that the original test provider will again produce an unreliable test result, there is no reason to request a repeat test by an independent third party laboratory. Again, we are told that such instances are exceedingly rare, whether or not the test is available from multiple providers or not. In fact, the PTO

should, in the course of this study, evaluate how often patients actually procure confirmatory tests where independent alternative providers are available. Or, in other words, figuring out the availability of "confirmatory tests where gene patents and exclusive licensing exist" almost presupposes figuring out the utilization of such tests where gene patents or exclusive licensing don't exist.

Our understanding is that for the vast majority of genetically transmitted diseases, multiple providers are available, and therefore, independent confirmatory tests are available. It should therefore be possible to gage, at least anecdotally, the actual patient and physician demand for such re-tests, to the extent such demand exists at all. Predictions are that such demand is probably very low. That is, in fact, a conclusion even the SACGHS committee drew in its 2010 report.

Irrespective of whether there is demand for second opinion testing, BIO hasn't identified a single genetic test for which an independent confirmation would be truly unavailable. Even in instances where the U.S. market is served by a single source provider, samples could for example be sent to ex-US laboratories or referred to research laboratories. For most tests, noninfringing alternative tests are available too. In fact, for certain genetic conditions, some decision trees in Europe use a sequence of different tests, involving, for example, protein-based tests (like protein truncation testing) before proceeding to full genetic sequence analysis. In any event, the barriers to access for a plain confirmatory re-test will always be higher than for a first test. Payors are unlikely to pay for confirmatory testing, irrespective of how many providers are available.

But most importantly, the notion of independent re-testing as an entitlement doesn't necessarily translate into a patient benefit at all. To the contrary, it could lead patients to incur significant out-of-pocket costs (because payors won't pay for a repeat test), prolong uncertainty, give rise to unrealistic hopes, and cause delayed treatment decisions. These negatives, too, must be weighed by the PTO as it develops its recommendations.

To the extent the PTO might find that systematic confirmatory testing is unavailable but needed to ensure the quality and reliability of genetic diagnostic testing services, it has been suggested that non-patent-based policies could provide a more directly applicable tool. For example, it has been proposed that as a condition of CLIA approval, test providers would have to provide for independent validation of testing procedures, for example exchange samples with outside labs for quality assurance, and the like. BIO takes no position on such proposals, but we believe they are at least directed at the question raised, and would present a much more logical avenue for exploration than going down the patent route.

In fact, in BIO's view, one has to strain hard to trace existing problems with patient access and utilization of genetic tests back to patents. First of all, coverage of genetic diagnostic tests appears to be quite good for the majority of patients who have private health insurance. Where private payor coverage is adequate, it is

adequate independently of patent status. Actual access concerns have been raised mainly for patients who must rely on public payors, so the problem seems confined to a certain class of patient and payor. [SHOW MAPS] Such differences between public and private payors don't directly point at a patent problem.

So what are some of these differences? The screening exclusion under Medicare limits coverage for genetic counseling services and access to predictive or predisposition tests, and that limitation exists without regard to patent status. Also, because states are responsible for making coverage decisions under Medicaid programs, for at least some genetic tests there exist significant geographic disparities that result in disparate access to genetic tests and counseling services for poor patients. Non-uniform access across the country would seem to indicate that there are other forces at play than patents. Revenue shortfalls and public health priorities may cause some state Medicaid programs to cover some genetic tests not at all, or only at a fraction of the reimbursement rate that other states are paying. SACGS reported instances where public payor reimbursement rates for some genetic tests were at or below the participating provider's cost, as well as frequent instances where claims are not reimbursed at all. So, irrespective of patent status, state funding shortfalls and pressure for non-utilization mean that participation in state Medicaid programs can be a difficult proposition from any provider's perspective. Importantly, monopoly pricing is unlikely to have anything to do with non-uniform access to genetic tests and services. As Robert Cook-Deegan and colleagues have demonstrated, there is no price premium on patented single provider genetic diagnostic tests compared to equally-complicated tests that are available from multiple competitors. In other words, such tests are equally expensive regardless of whether they are only available from only one provider or more. We should at least move on from the oft-repeated assertion that patents drive up prices for these kinds of tests.

My time is up; there is a lot of food for thought in the PTO's questions, and I hope this testimony shed light on some of them. As an ending, I believe a final look at these coverage maps is appropriate [show Medicaid map]. If a poor patient in Nevada doesn't have access to an advanced molecular diagnostic test, and right across the border in California a similar patient has access to the same test, there's obviously something wrong with that, but how can that be a patent problem? How can it be a patent problem in Nevada and not a patent problem in California? That's not how we understand patents to operate - inescapably, there's something else at work here. Thank you for your kind attention.