
Public Comments

DR. TEUTSCH: Welcome back, everyone. Before we get on with our discussion on patents, we will be turning to public comment, which is one of our critical functions. As you know, we serve as a public forum for deliberations on a broad range of health and societal issues raised by the development and the use of genetic technologies.

So we truly value all the input that we hear from members of the public. This is one of the important ways in which we get that input.

As you know, we have a very full schedule. In the interest of time here, I will ask the commenters to please keep their remarks to five minutes or less. I'm going to adhere to that because we really do have a full slate. We should have copies of your full statements, which will be made part of the meeting record.

So let's begin. Is Ms. Lisa Salberg here from the Hypertrophic Cardiomyopathy Association?

[No response.]

DR. TEUTSCH: No? Well, I see that our next presenter is sitting in the back. He is a frequent attendee of these meetings and someone who we always learn a lot from. Mike Watson is representing the American College of Medical Genetics.

Welcome again, Michael. We appreciate your comments.

DR. WATSON: Thank you. I'm going to keep my comments brief. I think most of what I have to say was pretty clear in the letter that I wrote to the Committee.

I had the luxury, that most here obviously didn't have, of listening to the webcast from my office this morning, so I will try not to repeat things that you have already talked about. Perhaps I will raise a few issues that have risen recently that I didn't hear mentioned this morning. They may have come up while I was driving down here, but who knows.

I'm from the American College of Medical Genetics. We represent board-certified medical geneticists, both clinical and laboratory geneticists, in the United States.

As far as I know, we are the only organization that has an actual policy position that genes are naturally occurring substances and should not have been patentable initially. However, given the inability to adequately address that problem, we have focused a lot of our interest on unfair licensing issues.

Now, I do want to say in preface that I would never want to encourage anyone to infringe on a patent. Anything I say I hope you take as purely educational. I have had people inquire about the value of my home in the past in relation to patent issues, so I clearly don't want anyone to be encouraged to infringe on a patent.

I will say that, at this point in time, there is little evidence that patents have led to products. There are very few products available in genetic testing. Products used to be the way by which most licensing was done. Royalties were accrued through the development of a particular product that made testing better and easier, or cheaper, and that laboratories thought improved on their own laboratory-developed tests.

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Among those 1,500 genes on which we currently do testing, there is very little evidence that patents have led to any products, aside from a very few, at this point in time. There is limited evidence that the patents and their license have improved services, either. A few examples I would agree to, but for the most part there is very little evidence of improvement in the delivery of services.

Now, I think one of the interesting things about gene patents is that they are typically very well developed in the diagnostic sector before anybody imposes patent rights or licensing rights on particular genes. That is because they are primarily for rare diseases and there is no financial incentive to go into enforcement of those genes until the point when the test moves out of diagnostic and family-based medicine and into population-based areas.

This is what happened with Canavan disease when it went to carrier screening. It was very shortly after two organizations, ACOG and us, recommended that carrier screening begin that the enforcement of those patents came into play. That is a very common phenomenon for the patents held in diagnostic genetic testing.

There are studies that have been done about gene patenting. Almost everybody in this room has watched these for 10 years. As far as I can tell, they largely focus on the research issues, not on clinical investigation as we know it in genetics but really on basic research, and have documented not a significant impact on research. I think the situation is very different in the clinical practice arena.

There was a recent paper in Science. Christopher Holman just a few weeks ago made a couple of arguments about gene patenting. He argued that there was very little litigation and that in and of itself was evidence that there was not a problem with patenting of genes as they related to genetic testing.

I think that is a misstatement. Our experience is that the litigation has been extremely limited due to the extreme cost of litigation in patent-related issues. We engaged in a litigation backing Kaiser Permanente in a case involving human chorionic gonadotropin back in the mid to late '90s. At that time it was only about \$1.5- to \$2 million to engage in one of these cases and get all the way to the merits of the case in court.

We actually went through about \$200,000 in that case and never got to the merits of the case. They gave a covenant not to sue to Kaiser, who then allowed them to do all the testing they wanted to do, without ever getting to the merits. Everybody else who had contracts and other relationships was then in the same boat they had been in.

The other argument they make is that there has been no imposition of gene patents on the new multiplex array technologies. I think this is clearly no longer the case, either. There have been a couple of recent examples. A laboratory has been told to take the dystrophin gene for Duchenne muscular dystrophy off of its CGH arrays.

What their lawyers determined was that they would not have to take them off of the array but they would not be able to report out a deletion or duplication in the dystrophin gene itself, seriously imposing on the practice of medicine and the duty to inform when that laboratory identifies that Duchenne muscular dystrophy-related abnormality in array CGH.

Another situation has arisen recently. It is circuitous because it overlaps a couple of the examples Bob Cook-Deegan gave you this morning. He talked about newborn screening for hearing loss. He also talked about Long QT syndrome.

In the hearing loss world, one of the goals of manufacturers has been to develop an array that can identify kids in newborn screening molecularly. They come out with a functional test found to be hearing loss, and we would like a molecular test that allows us to identify the multitude of abnormalities that can lead to hearing loss.

Unfortunately, one of those is Jervell and Lange-Nielsen syndrome, also associated with Long QT syndrome. When one is doing this for a child that presents with hearing loss, you are now not allowed to test for that particular gene in the arrays because it imposes on the Long QT patents.

I think increasing examples are arising of real patent thickets developing around gene patents that are going to require us to find some way out of the box. We really only see two options. One is to go back to the Ganske-Frist amendment and separate out the exemption for diagnostic use of gene patents from the protection of gene patents for the development of therapeutics. Clearly, that is a high-investment area where one wants to protect that investment to lead to the products we need in therapeutics. The evidence of that benefit arising on the diagnostic testing side is quite thin.

I had better not go on. There is another case. I would encourage you to look at the case of Mayo Labs v. Prometheus Labs because it is bringing us back to the Metabolife Labs v. Lab Corp. case in the very near future. It is currently at the circuit court.

DR. TEUTSCH: Thanks so much, Michael. We appreciate that. Our next speaker is changing her role here. Debra Leonard is representing the Association of Molecular Pathology. So you are going to change hats instantly, I assume.

DR. LEONARD: I am here representing the Association for Molecular Pathology. We have recently rewritten our AMP position statement on gene patents and exclusive licensing of genetic discoveries. I would like to share that with you.

Many disease-associated human genes and human pathogens have been identified in recent years, and more will be discovered in the coming decades. Clinical laboratories in both the public and private sectors translate and develop many of these discoveries into molecular diagnostic tests and seek to make these tests widely available as clinical services for the public good.

Clinical laboratories can only develop these important tests when they have access to the broadest base of genomic discoveries. The U.S. Patent and Trademark Office has historically granted broad patents on genomic discoveries. Frequently, patent holders and their exclusive licensees are choosing to monopolize molecular testing by restricting healthcare providers from developing or performing tests covered by these patents and licenses.

AMP believes that molecular test services are medical procedures. As such, they should be widely available to promote optimal patient care, medical education, and medical research. Research, development, and practice of molecular testing is essential to medical practice, the education of physicians, researchers, and healthcare professionals, and the continued improvement of the quality of medical care.

While attaching intellectual property rights to true acts of invention, such as new therapeutics, diagnostics, or technology platforms is essential to encourage investment and reward innovation, a single gene or a sequence of the genome is a product of nature and should not be patentable.

Gene patents can serve as a disincentive to innovation in molecular testing because they deny access to a vital baseline of genomic information that cannot be invented around. Moreover, the threat of enforcement from a patent holder and the ensuing litigation costs lead to a chilling effect, as clinical laboratories are reluctant to develop new tests which could directly benefit patients.

In addition to the concern about gene patents, exclusive licenses that confine molecular testing to a single provider are detrimental to the public interest by limiting patient access to testing, restricting medical practice and research, and impeding the advancement of medical knowledge and enhancement of the public's health through informed clinical decision-making.

Moreover, no governing standards currently exist that would prohibit the practice of granting exclusive licenses. Most patented discoveries of human genes or human pathogens can be effectively translated into molecular tests provided they are licensed on a non-exclusive basis and licenses are easily obtainable both in financial and practical terms.

Therefore, AMP recommends the following.

The patenting of single genes, sequences of a genome, or correlations between genetic variations and biological states should be discontinued, either as a result of judicial review or through an act of Congress.

Entities, including higher educational and research institutions, that currently hold gene patents should not grant exclusive licenses to these patents.

To ensure that access to innovative molecular tests remains widely available and affordable to patients, financial terms for test licenses should be reasonable and sole-source testing should be prohibited. License agreements should also be free of any terms that limit the number of tests that can be performed by a laboratory or regulate the technical performance or clinical uses of a test.

License agreement should be likewise free of terms that inappropriately limit research related to testing or the public dissemination of the resulting research findings.

AMP encourages all stakeholders to work cooperatively to develop alternative models to gene patents and exclusive licenses. Innovative, alternative models should be developed that increase patient access to health care and achieve greater benefit from our current knowledge of the human genome. Thank you.

DR. TEUTSCH: Great. Thanks so much, Debra. We appreciate all of that. I'm going to move us along because we are just pressed for time.

The next speaker is Guido Brink, who is from Agendia. Thanks for coming.

MR. BRINK: Thank you so much. I have a quick question or comment for the Committee. My name is Guido Brink. I am director of regulatory affairs and reimbursement for Agendia. I think Dr. Gutman can agree with me that, when we talk about genetic tests, the devil is in the details of

the definition. What we have seen with the whole discussion around IVDMIAs is that industry has taken a lot of time and effort to try to define IVDMIAs and to try to exclude certain deaths from the IVDMIA definition.

When I look at the definition currently stated by the Committee, it says genetic tests are, for purposes of this study, any test performed using molecular biology methods to test DNA or RNA. In our case, we have a gene expression profile. We do not assess any mutations. We do not want to assess any mutations. We assess the expression of a gene or multiple genes and put that into an algorithm to come to a conclusion on disease state.

My recommendation to the Committee, or my question, would be within this definition gene expression profiling tests would be genetic tests, although when I look at the case studies and at the investigations performed, no genomic profiles or expression profiles are investigated. It is purely mutation assays. So my question would be, or my recommendation, is looking back at what has been investigated to clearly define what has been investigated and to maybe redefine "genetic test" in this study.

DR. TEUTSCH: Great. Thank you. That is very helpful information that we can look at as we revise the draft.

The last one I have on my list is Carol Reed from Clinical Data, Incorporated. Welcome.

DR. REED: Hello. My name is Carol Reed. I'm chief medical officer of Clinical Data. Just to clarify for everyone, we are the parent company of which PGx Health is a subsidiary. I think it was reversed on the slides earlier today. We offer the FAMILION test for Long QT testing, a high-quality test of which we are very proud.

This test is actually a great example of a product that has arisen out of an exclusive patent license, and I think that has been extensively discussed already.

I would just like to make three points for the Committee. First of all, as a public, for-profit company, yes, we do license intellectual property. Our intent is to commercialize that, not to sit on it or hide it. That is too expensive a proposition. I think we have shown our intent to do that by launching our FAMILION test in 2004.

In the time since that test was launched, other genes for Long QT syndrome have in fact been identified. We feel that one of the reasons for this is the success of our commercial test because the burden of testing for those five genes has in fact relieved research laboratories of having to sequence those more common causes of Long QT syndrome and freed their resources to identify more rare causative genes.

Secondly, I would like to address the issue of patient access. Although patents are certainly a major topic of discussion in this area, we should not ignore the issue of reimbursement and payer policy in covering these tests. In fact, I believe that patients are more directly affected in terms of their access to testing by payer reimbursement policies.

Again, to use Long QT testing as an example, we have made a significant investment in our customer service group as well as our prior authorization group, and in fact many times acquiring authorization to pay for a test takes more time than it does to actually perform the test and return the results to patients.

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We have invested significantly in people who work directly with managed care. We have succeeded in getting Medicaid coverage in 38 states and have coverage pending in the remaining 12. We are also an approved Medicare provider and now, by combining with private and government insurance, we have succeeded in gaining coverage for over 160 million lives in the United States. This is a significant advantage that we would not have invested in without patent protection for our test.

Thirdly, I think we should not underemphasize the importance of expertise in interpretation of these mutational analysis tests. It is very important to be able to draw a direct relationship between a discovered mutation and the structural relationship to the protein and to have a normal database against which to compare frequencies of mutations and other variants identified during testing. Without the investment that we made to build the normal mutational and SNP database, we would not be able to provide interpretation of these tests.

Moving towards sequencing these tests in whole-genome scans may in fact prove to be dangerous for our patients because low-risk patients are going to have variants identified without the appropriate background against which to interpret and analyze these results. Patients may in fact be put in danger of inappropriate interventions, including the implantation of defibrillators.

Finally, I would suggest to Brian that perhaps he might include the cost of interpretation of these sorts of tests and the resources that are put into that in his cost modeling, as we begin to understand the impact of price and cost of genetic testing.

Thank you to the Committee for hearing my comments.

DR. TEUTSCH: Great. Thank you very much. These are very helpful comments for us as we deliberate.

Let me just check again. Is Ms. Salberg here?

[No response.]