



# Insight Pharma Reports

## Expert Intelligence for Better Decisions

Interview Transcript from Insight Pharma Reports' *Molecular Diagnostics: A Rapidly Dynamic Market*

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**Jeffrey E. Miller, PhD**  
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Board of Directors, InVivoScribe Technologies, San Diego, CA

*CHI: To start, please give an overview of your company's technology (or technologies) in the field of molecular diagnostics, and the key or most significant current applications that your company is marketing and/or developing using this molecular technology.*

**Jeffrey E. Miller:** InVivoScribe (IVS) is a research reagent manufacturer. We are working with leading research institutions to provide them with tools to aid in their discoveries. We are also transitioning into a diagnostics company, as some of our research products find use in the IVD world. In addition, we have a CLIA and CAP-accredited national reference laboratory, Laboratory for Personalized Molecular Medicine (LabPMM). This reference laboratory provides information to the clinical community on the status of the important biomarkers. At least one of these is clearly an internationally recognized standard of care for patients with AML [acute myelogenous leukemia]. FLT3 is a tyrosine kinase expressed at high levels in AML. Activating mutations of FLT3 have significant prognostic value, indicating that conventional treatment for AML is less effective in this patient population. Our LabPMM provides testing to determine the mutation status of FLT3, as well as other related biomarkers (e.g., NPM1), so physicians and patients can make the most informed and coherent treatment decisions.

Working with hospitals and patient care facilities, as well as pharmaceutical companies, we believe we will play an important role in helping physicians provide a higher quality of customized care to their patients, while also assisting in pharmaceutical development. So, our company is dedicated to improving the quality of healthcare by providing reliable cutting-edge tools for molecular diagnostics.

*CHI: What are the key or most significant future applications that your company may develop using this molecular technology?*

**Dr. Miller:** There are two major areas: in vivo cDNA synthesis technologies and personalized molecular diagnostics. For the short term, the latter is where we are seeing the most rapid growth toward routine use. It appears certain during the next several years, as the potential of this revolutionary field becomes clear, that personalized molecular diagnostics will be embraced and gain wide acceptance. There will be a better commercial atmosphere for the use of defined biomarker testing to stratify and monitor disease, aid in treatment decisions, and determine the efficacy of therapy. Minimal residual disease tests, which are highly sensitive patient-specific tests that target the antigen receptor genes, are an example of tests we design to monitor patient therapy. These tests quantify cancer cells in patients who have achieved remission. They are sentinel tests, as they provide an "early warning" on cancer recurrence so the physician has time to intervene most effectively. In many ways, these oncology tests are analogous to the infectious disease tests, such as HIV viral load assays, used to identify when the virus has evolved or mutated so that the current cocktail of antiviral drugs are no longer effective. Tests such as these are already fulfilling the promise of personalized molecular medicine. We think that personalized molecular medicine, even in the short term,

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is likely to help clinicians and accelerate drug development. In the further term, new biomarkers will be used in conjunction with new, as yet undeveloped, testing algorithms to tailor patient therapy. It is clear that molecular diagnostics, in the relatively short term, has the very real potential to achieve many of the promises of personalized medicine, perhaps even getting some control of the escalating healthcare costs. Downstream, as the field of molecular diagnostics matures and undergoes a few permutations, refinements, and evolution, we are confident that molecular techniques will help predict disease, so that, combined with patient education, there will be earlier intervention and more use of preventive medicine. This is where we might expect to see real cost savings.

Our in vivo cDNA synthesis technologies need further development in order to be commercially viable, but standardized, efficient, point-of-care specimen stabilization will have a huge impact on RNA-based technologies, so this technology promises to open avenues for assessment of more typical heterogeneous specimens that are not currently being examined. The market for individual cell expression pattern stabilization is enormous and we see a huge upside in bringing this exciting technology to market.

**CHI:** *The diagnostics market is highly competitive. What are the advantages or benefits of using your molecular technology for diagnostic testing?*

**Dr. Miller:** We purposely stayed out of solid tumors because it is a large market that is well represented. We focus on hematopathology, which is an exciting field that has plenty of opportunities. It is a relatively niche market, and therefore we can leverage our abilities and technologies to achieve something substantial. We have a good team. We use a combination of intramural and collaborative people (scientists and other resources) to identify promising areas early. We try to cipher through and figure out what are the most promising leads, and then pursue those in a targeted way to develop and commercialize them on time so that they are available when they will be most useful to the community.

**CHI:** *The diagnostics market is also rapidly changing. What have been the important advances or changes for your company in the past year?*

**Dr. Miller:** We are transitioning. We are a research company that provides research-use reagents. As customers determine that there is a potential for clinical-use versus research markers, we try to meet the need by providing either research products or ASR product materials for the US market and CE-marked IVD products for our customers in Europe. We are putting a lot of emphasis now on QSR; we are listening very carefully to the regulatory bodies and embracing what they feel is important for us as a growth company. It is clear that we are transitioning from a research organization to a diagnostic company.

**CHI:** *What do you consider to be the most important trends in the molecular diagnostics market?*

**Dr. Miller:** I think that one of the things that is most helpful is cooperation. I am seeing a little more seamless cooperative spirit, an interaction between countries and groups that is facilitating development. Consensus, or at least respectful understanding of differences based on the science, is important for standardization. There are guidelines from a number of different regulatory bodies to include both those in the US and abroad. Each group has some very thoughtful, important contributions and we are starting to take advantage of that. This cooperation will be very helpful because we all have a common goal, and that is to provide high-quality reagents and products. It helps to work together.

**CHI:** *What are your opinions on the role of molecular diagnostics and personalized medicine?*

**Dr. Miller:** I do not see how you can have personalized medicine without molecular diagnostics; molecular diagnostics is the avenue to achieve personalized medicine. You can only glean so much from clinical information other than molecular testing, and to achieve the promises of personalized medicine, you really have to stratify and define at the molecular level. More information is better. Targeted identification of specific biomarkers is going to be critical to patient management, to controlling costs, and to providing the right drug to the right patient. Immunohistochemistry and flow cytometry are very powerful techniques,

but, given the complexity and the many pathways involved in what are, in fact, a heterogeneous group of diseases, it is critical to further stratify and identify the molecular mechanisms and targets that are not just surface or expressed targets that you can get at with antibodies. The pathways are often very complicated, and there are backup pathways. For instance, if you hit one kinase with a drug, another one often comes to the rescue.

**CHI:** *What do you perceive to be the greatest opportunities in the field of molecular diagnostics in the near future?*

**Dr. Miller:** In the near future, I believe the greatest opportunities for molecular diagnostics are in personalizing patient care and the development of therapeutic products. I know that a few years ago—and I think it is still true—for a lot of the diagnostics, compensation was generally based on labor costs, with little emphasis based on the clinical value of a test. I am hopeful that we will see a change in the future, as there is a real synergy there. As we start to realize the significant potential and the real value, I think that we are beginning to see a paradigm shift: We are going to start seeing more of a hand-in-hand approach, with the drugs and molecular diagnostic tools used together to guide treatment.

**CHI:** *What do you perceive to be the greatest challenges or hurdles for companies developing and marketing molecular diagnostic tests?*

**Dr. Miller:** In the short term, I think that it is going to be limited access to resources for keeping up with therapeutic development through the regulatory process. I believe that the disparity in perceived value between the pharma and the diagnostics is inhibiting the regulatory approval of some tests that will be very helpful in the overall scheme of things. I think that this will change, but there is a lag there. The other hurdles, I think, are ones that we can manage. The intellectual property situation sometimes needs to be presented better, as in some circles there is a question as to the value of IP, yet patients provide the incentive to take risks to move ahead with both drugs and diagnostic test development. Currently, we have a pretty effective and coherent situation and patient costs are manageable; some companies just need to be flexible in working out licensing so everyone is happy. At a conference at Harvard last fall, I proposed that genomics companies involved in whole genome sequencing perhaps should embrace an “iTunes” approach, including graduated royalties depending on the way they interrogate their data. This would provide patent holders protection and revenue from IP, without creating a huge cross-licensing or stacked royalty problem for licensees. I noted that this approach was getting attention during the conference, so I am hopeful that we can be flexible and open to considering new licensing models while retaining the current IP structure.

I do not see real barriers to molecular diagnostics. I think that one of the problems with the field is that it is difficult to identify specific biomarkers that really show clinical utility and value. A number of biomarkers show promise, but if there is no intellectual property to provide an incentive to get them through the regulatory processes, it is difficult for those markers to succeed. The groups that have patents for markers can move ahead with some degree of assurance that it is worth pursuing the necessary costly regulatory steps with the FDA.

**CHI:** *Do you have any additional comments regarding molecular diagnostics—technology, applications, market, or other issues?*

**Dr. Miller:** I will build on what I mentioned earlier. The international cooperation and standardization of regulatory agencies is helpful—some steps have been put in place by the FDA, CAP, and CLIA here, and by regulatory bodies abroad, and I think that their guidelines are excellent. They have very bright people who are extremely dedicated and want to provide the best science-based standards with the least burdensome approach. Being able to come to some harmonized and standardized series of processes will be very helpful.

On the same front, this also goes with the scientific side. One of the reasons that I started this company was to provide standardized tools to the research community for testing because there was such disparity and heterogeneity. I think that having standardized testing is always helpful. That includes specimen preparation, sample handling, and everything else. I have found that standardization leads to established

guidelines for use and interpretation, which then benefits all participants, as assessment of test utility and clinical efficacy can be more readily and confidently evaluated.

Let me mention how working together may stretch even further to everyone's benefit, most notably the patient. Currently, a focus for each pharma, academic, or other research center is to minimize research costs while generating useful data. This is the rational approach. However, as clinical research occurs using a variety of heterogeneous platforms and a variety of methods for sample collection and processing, we are currently participating in a systematic loss in our collective ability to generate future data; lack of standardization is jeopardizing the value of archived clinical specimens so that information and bioinformatics may never be captured from these samples. If we harmonize the upfront capture of critical clinical information in a way that protects both patient privacy and comports, and is consistent with rules and regulations of the international community, and we standardize specimen processing and storage—everyone in the international community will be better served, and patients will be more likely to see pharmaceuticals become available more quickly.

I am confident that, by working together, we can achieve this level of standardization while protecting patients' rights and privacy. We just had a symposium at AMP on clonality testing. One of the reasons that we did that was to bring together the experts from Europe and the United States. The symposium looked at ways that some of these gene rearrangement tests are being done by a consortium of laboratories in Europe, and by leading laboratories in the US. We expected 30 to 50 people at the symposium, and more than 300 showed up. It was a wonderful opportunity to get feedback from people who are using the technology and the group of academics from leading institutions worldwide who are actually developing tests.

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The above interview transcript is included in *Insight Pharma Reports'*

### ***Molecular Diagnostics: A Dynamic and Rapidly Broadening Market***

Discussed in this publication:

- Established and up-and-coming technologies
- The activities of more than 35 companies
- More than 300 current and emerging molecular diagnostic tests for disease applications and genetic testing
- Business considerations associated with product development, commercialization, and marketing
- Regulatory issues
- Challenges facing companies developing molecular diagnostics and/or companion diagnostics and targeted therapies
- Major trends in the molecular diagnostics market, such as the increasing number of tests that are commercially available

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