Executive Summary

The focus of this report is on drug/diagnostic combinations – drugs used to treat patients, and on diagnostic tests that can provide information about how a patient may respond to treatment with that drug.

Personalized medicine has emerged as an important field for companies in both the pharmaceutical and diagnostic industries. While many drugs in development and on the market today still follow the traditional approach where drugs are developed to treat all patients with a certain disease or condition, the "one-size fits all" approach, it is now widely recognized that not all drugs work equally well in all patients. Also, some percentage of patients experience adverse reactions, sometimes severe, to drugs while other patients experience few or no side effects.

Many different factors can affect how a patient responds to a particular drug. These factors can include the genetic makeup of the patient, genetic changes that occur with diseases such as cancer, as well as diet, age, environmental factors. Considerable interest exists in the identification of methods to determine which patients will respond well to a particular therapy and which patients are likely not to benefit from therapy as well as which patients are likely to experience adverse side effects caused the therapy. This interest is leading the advancement of personalized medicine.

"Personalized medicine" is a broad term that is defined somewhat differently by different groups. Chapter 2 discusses various definitions of "personalized medicine" as well as different definitions for terms such as pharmacogenomics, pharmacogenetics, and other terms used in personalized medicine. Chapter 2 also briefly discusses the wide range of disease applications in which personalized medicine can be used, and technologies used in personalized medicine tests.

Chapter 3 discusses many examples of drug/diagnostic combinations currently available today for use in personalized medicine. Some combinations, such as testing for HER2 over expression in breast cancer before treating the patient with Herceptin, have been available for a number of years and are now widely used. Other examples of available drug/diagnostic combinations discussed in Chapter 3, but are not necessarily widely used at this time. The largest number of personalized medicine tests are available for oncology drugs, although personalized medicine is already being applied to many different disease indications. Extensive tables in Chapter 3 provide information on diagnostic companies that market personalized medicine in vitro diagnostic (IVD) tests and/or test services through their CLIA laboratories.

Chapter 4 highlights some aspects of the development of diagnostic and therapeutic products that apply to the filed of personalized medicine, but not necessarily to all diagnostic tests and drugs in development. This includes identification of biomarkers and validation of personalized medicine tests. Validation includes analytical validation, clinical validation, and validation of clinical utility or benefit. In addition, development and regulatory issues facing both therapeutic and diagnostic companies are discussed. Chapter 4 concludes with a section that discusses certain specific personalized medicine tests and why they either are, or are not, widely used at this time.

As with all emerging and rapidly changing fields, companies participating in this field face a number of different strategic issues. This is especially true for personalized medicine, where companies from two different market segments (diagnostics and pharmaceuticals) are both participating and must interact with each other. Selected strategic issues discussed in Chapter 5 include the need for more research and basic knowledge, models and options for pharmaceutical companies developing the drugs in drug/diagnostic combinations, models and options for diagnostic companies developing the tests in drug/diagnostic combinations, options for diagnostic companies for delivery of these tests, a number of aspects of health economics and reimbursement, intellectual property issues, and certain critical issues facing companies as they try to penetrate the market with newly developed personalized medicine products. Company perspectives on this market are discussed in Chapter 6.

Today, personalized medicine is a rapidly growing field with many pharmaceutical and diagnostic companies actively participating. As with personalized medicine drug/diagnostic combinations available today, the greatest focus of activity is in the field of oncology. Many different products in development programs for oncology are discussed in Chapter 7. In addition, Chapter 7 discusses personalized medicine products in R&D directed to a wide range of other disease indications. These indications include autism, autoimmune disease, cardiovascular disease, CNS disorders, HIV infection, osteoarthritis, osteoporosis, pain, type-2 diabetes, and others.

A number of pharmaceutical companies are now interested in personalized medicine. Since many of these companies do not have internal diagnostic divisions (or internal capabilities to develop and market diagnostic tests), this has led to a number of agreements between pharmaceutical and diagnostic companies. These agreements are discussed in the final section of Chapter 7, and 35 agreements are listed in Table 7-3. Of these, 14 agreements were made or announced in 2009, and another 4 agreements have been announced to date in 2010. It is likely that even more pharmaceutical/diagnostic company agreements have occurred recently. This activity demonstrates that companion diagnostics is a topic of interest to at least some pharmaceutical and biopharmaceutical companies.

The final chapter of this report, Chapter 8, includes interviews with executives at both pharmaceutical and diagnostic companies that are actively participating in personalized medicine. These experts in personalized medicine discuss the activities of their companies, the overall field of personalized medicine, and many of the issues facing companies in this field.