Executive Summary

According to a 2001 definition from the Biomarkers Definitions Working Group, a biological marker (or biomarker) is a "characteristic that is objectively measured and evaluated as an indicator of normal biological processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention."[4] Biomarkers can be used to predict response to therapy or risk of side effects for personalized medicine applications. Other types of biomarkers include predisposition biomarkers, screening biomarkers, diagnostic biomarkers, prognostic biomarkers, toxicity biomarkers, pharmacodynamic biomarkers, and others. Biomarkers can be valuable tools in clinical diagnostics, and also in drug discovery and development.

Interest in biomarkers has exploded, and thousands of articles are published each year that mention biomarkers. Yet despite the high level of interest and research in this field, the number of new therapies approved by the FDA each year remains low, while the cost of developing new drugs has climbed dramatically. In diagnostics, the large number of research publications on biomarkers has resulted in a relatively much more limited number of tests that are widely accepted and routinely used. The question is, why?

This biomarker report focuses on issues and questions that must be addressed to successfully take advantage of the potential of biomarkers. We present strategies to address these issues and successfully commercialize products utilizing the growing knowledge about biomarkers.

Chapter 2 provides an overview of biomarkers. The chapter starts with a discussion of definitions of biomarker and related terms, followed by an overview of the different types and applications of biomarkers. This is followed by a brief overview of the range of different technologies that may be used to detect and measure biomarkers. The final section of Chapter 2 discusses a very critical issue for biomarkers: validation. This includes discussion of recommendations and guidelines that have been made by several different groups for the development and validation of biomarkers, including the FDA's concept of fit-for-purpose method validation.

Chapter 3 provides examples of current and potential emerging applications of biomarkers in different fields such as oncology, cardiology, neurology, safety, and other applications. These selected examples demonstrate the wide range of potential applications of biomarkers. Chapter 4 discusses the market for research products (tools) and services in the field of biomarkers. This includes a discussion of selected companies and their activities, as well as selected examples of agreements and acquisitions in this area.

Chapter 5 focuses on biomarker strategies for diagnostic companies, including the questions surrounding what is required to successfully commercialize biomarkers in the diagnostics market. This includes identification of novel biomarkers, analytical validation of diagnostic tests for new biomarkers, clinical validation and clinical utility of biomarkers, research and other information needs regarding biomarkers,

models and options for diagnostic companies in the field of personalized medicine, strategic options for the delivery of novel biomarker assays, health economics and reimbursement, and other issues.

Similarly, Chapter 6 focuses on biomarker strategies for pharmaceutical and biopharmaceutical companies. This includes a discussion of the potential roles of biomarkers in various aspects of drug development, validation of biomarkers, regulatory issues, options for companies developing drug/diagnostic combinations, health economics and reimbursement in personalized medicine, and other issues. The chapter concludes with a discussion of biomarker agreements between pharmaceutical and diagnostic companies.

Chapter 7 discusses 40 key companies to watch and includes a table of 175 companies that are commercializing biomarkers and/or systems for their detection and measurement. The focus of discussion for each company is what they are doing in the biomarker field that makes them interesting. The final chapter (Chapter 8) includes interviews with experts in the field of biomarkers.