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Introduction[1]

The number of new drug approvals has remained reasonably steady for the past 50 years at around 20 to 30 per year (Munos, 2009), while at the same time the total spending on health-related research and development has tripled since 1990 (Scannell, 2012). There are many suspected causes for this trend, including increased regulatory barriers, the rising costs of scientific inquiry, a decrease in research and development efficiency, the downstream effects of patent expirations on investment, and the lack of production models that have successfully incorporated new technology (Paul, 2010; Scannell, 2012). Regardless, this trajectory is not economically sustainable for the businesses involved, and, in response, many companies are turning toward collaborative models of drug development, whether with other industrial firms, academia, or government (IOM, 2011). Introducing greater efficiency and knowledge into these new models and aligning incentives among participants may help to reverse the trends highlighted above, while producing more effective drugs in the process.

New technologies have the potential to open up avenues of development and to identify new drug targets to pursue. Specifically, improved validation of gene–disease associations through genomics research has the potential to revolutionize drug production and lower development costs. Genetic information has helped developers by increasing their understanding of the mechanisms of disease as well as individual patients' reactions to their medications. Warfarin, Gleevec®, XALKORI®, Kalydeco™, and Zelboraf® are all examples of pharmaceuticals that utilize genetic information to inform dosing or whose activity and effectiveness is determined by inherent genetic properties of the patient or their tumor (i.e., a targeted therapeutic). However, even with these successes there remains skepticism over how useful genomic information will be to the larger drug development process (Pollack, 2010; Wade, 2010). There is a need to identify the success factors for the various models that are being developed, whether they are industry-led, academia-led, or collaborations between the two.

The Roundtable on Translating Genomic-Based Research for Health held a workshop on March 21, 2012, titled New Paradigms in Drug Discovery: How Genomic

[1] The planning committee's role was limited to planning the workshop, and the workshop summary has been prepared by the workshop rapporteurs as a factual summary of what occurred at the workshop. Statements and opinions are those of individual presenters and participants and should not be construed as reflecting any group consensus.

Data Are Being Used to Revolutionize the Drug Discovery and Development Process. The purpose of the workshop was to examine the general approaches being used to apply genomic-based research results to the discovery and development of new drugs, the successes achieved so far, and the challenges ahead.[2]

BOX 1-1
Workshop Objectives

The workshop New Paradigms in Drug Discovery: How Genomic Data Are Being Used to Revolutionize the Drug Discovery and Development Process had three broad objectives:

1. To examine the impact of and investment in genetic and genomic technologies and data in drug discovery and development.
2. To examine and discuss the challenges for incorporating genomic technologies into drug development and to explore solutions to remedy those challenges.
3. To discuss and explore how innovative, novel, and global partnerships between academia, industry, foundations, and government can enable the use of genomic information for more efficient and effective drug discovery and development programs.

STRUCTURE OF REPORT

Box 1-1 provides the overall objectives of the workshop. Chapter 2 discusses the current environment for personalized medicine approaches to drug discovery and development. Chapter 3 examines three case studies as an illustration of both the promise and the challenges of genomic-based drug discovery and development. Chapter 4 looks at the application of emerging technologies, such as next-generation sequencing, to this field. Chapter 5 examines several specific evolving paradigms in genomic-based drug development, approval, and prescribing. Finally, Chapter 6 features comments and discussions drawn from throughout the workshop on overcoming the challenges and achieving the promise of genomic-based drug discovery and development.

[2] The full statement of task can be found in Appendix C.