Epigenetics is a rapidly growing field with promising implications for treatment of cancer and potentially other diseases. Research interest in epigenetics has exploded in recent years, and considerable progress has been made in the commercialization of discoveries in this field.

**Discussed in this Report:**
- Diagnostic applications of epigenetics
- Diseases and conditions targeted by current and emerging epigenetic-based therapies
- Companies marketing and/or developing therapies that target epigenetic changes within the disease process
- Current and projected markets for epigenetic diagnostic and therapeutic products
- Strategic issues and major challenges for developers of epigenetic-based products
- Thought-leader interviews
OVERVIEW

Many companies have recognized the potential benefits of epigenetic-based diagnostic and therapeutic products. Two epigenetic tests for colorectal cancer screening are CE-marked and available in Europe, and a number of epigenetic tests are available through clinical laboratories in the United States as laboratory-developed tests (LDTs). Four epigenetic drugs have now been approved by the US FDA.

The first mechanism of epigenetics to be widely studied was DNA methylation. Two of the approved epigenetic drugs are demethylating agents, and most of the epigenetic diagnostic activity to date has focused on methylation markers. A second major epigenetic mechanism is modification of histones by various enzymes, including histone deacetylases (HDACs). The other two approved epigenetic drugs are HDAC inhibitors. In Chapter 2, *Epigenetic Drug & Diagnostic Pipelines: DNA Methylation, HDAC Inhibitors, and Emerging New Targets* provides an overview of epigenetics and its mechanisms, diagnostic methods to detect changes in DNA methylation, and epigenetic therapeutic targets.

Companies developing methods to detect methylation changes are being joined by reference laboratories which have licensed their technology to develop LDTs, and by certain diagnostic companies that are entering the field through agreements with epigenetic diagnostic companies. In addition, one early-stage company is focusing on histone modification patterns. The activities of these diagnostic companies are discussed in Chapter 3.

Considerable activity is focused on epigenetic-based therapeutics. It is expected that epigenetic drugs may be useful for treatment of a wide range of diseases, including hematological cancers, solid tumors, and other non-cancer indications. Major disease indications that are being targeted by companies developing epigenetic-based drugs are discussed in Chapter 4.

The potential therapeutic applications of epigenetics have attracted a large and growing number of participants. Chapter 5 of *Epigenetic Drug & Diagnostic Pipelines: DNA Methylation, HDAC Inhibitors, and Emerging New Targets* discusses over 40 companies that are active in this area. Much of the current activity in epigenetic therapy is focused on HDAC inhibitors, which has become a very crowded field. More than 30 companies developing nearly 40 HDAC inhibitor compounds or programs are presented in this chapter. Many companies interested in epigenetics are now developing potential new therapies that are directed against novel epigenetic targets.

Chapter 6 discusses business considerations and strategic issues for companies developing epigenetic-based products. We examine specific concerns faced by the diagnostic and therapeutic segments. Many of the corporate agreements that exist within the therapeutic epigenetic segment are also discussed. Chapter 7 presents interviews with experts who discuss the overall field of epigenetics; challenges and issues facing companies in this field; and their companies’ activities.
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