Executive Summary

The immune system is a complex biological process that protects the body from infectious pathogens such as bacteria, viruses, parasites, and fungi, and also from cells that have transformed into cancer cells, but have not yet escaped the immune system. However, sometimes this process goes awry and the immune system behaves as if the individual's own body if "foreign". This condition can result in an autoimmune disease or to an overreaction or exaggerated response to a foreign substance that is normally harmless to most people leading to an allergic response. In these situations, therapies are needed to suppress the unwanted immune response. Also, in transplantation medicine when an organ or tissue from one individual is transferred to another individual, the recipient's immune system will recognize that organ or tissue as foreign, and will activate the immune process leading to subsequent rejection. In each of these settings, therapies are needed to suppress the unwanted and harmful immune response.

A number of drugs are available today to treat these disorders, but they are not always effective. In addition, early immunosuppressive therapies to reach the market suppress much of the immune response, and can lead to serious adverse events such as increased risk of infection and possibly increased risk of cancer development. This has led to an interest in identifying approaches to balance the immune system, to suppress the part of the immune response that is causing the disease, while maintaining or enhancing other parts of the immune system.

This report focuses on the immunotherapies that suppress or balance the immune response for selected autoimmune diseases, asthma, allergies, and prevention of organ transplant rejection. The autoimmune diseases discussed in this report include rheumatoid arthritis, type 1 diabetes, Crohn's disease, ulcerative colitis, psoriasis, psoriatic arthritis, and systemic lupus erythematosus.

Chapter 2 discusses each of these autoimmune diseases, asthma, allergies, and prevention of organ transplant. This discussion includes information on the pathology and epidemiology of each disorder. Chapter 3 discusses the current pharmacological treatment options for these diseases.

The major focus of this report is on emerging candidate therapies for these autoimmune and inflammatory disorders, and for prevention of organ transplant rejection. Approximately 400 product candidates and development programs are identified in this report. A wide range of potential targets have been identified for potential new therapies being developed to treat autoimmune or inflammatory disorders, or to prevent rejection of organ transplants. In Chapter 4, Table 4.1 summaries current and emerging therapies that are directed against many of these molecular targets. Even though a large number of potential therapeutic targets have been identified, many of the emerging therapies are directed against the same or related molecular targets. This issue is highlighted in Table 4.1, and the need for companies to differentiate their emerging therapies is discussed in this chapter as well as in Chapter 9.

Chapters 5 through 8 discuss many of the emerging therapies that are in development for the eight autoimmune diseases included in this report, asthma, allergies, and prevention of organ transplant rejection respectively. These are all crowded and highly competitive fields, but this is especially true for the field of autoimmune disease. There are approximately 400 product candidates and development programs identified in this report as well as more than 250 emerging autoimmune disease therapies. These include:

- More than 100 emerging therapies for rheumatoid arthritis
- 15 emerging therapies for type 1 diabetes
- More than 35 emerging therapies for inflammatory bowel disease (Crohn's disease; ulcerative colitis)
- More than 50 emerging therapies for multiple sclerosis
- More than 40 emerging therapies for psoriasis
- 3 emerging therapies for psoriatic arthritis
- More than 30 emerging therapies for lupus (systemic lupus erythematosus; lupus nephritis)
- More than 30 early stage autoimmune disease emerging therapies and programs (disease target not specified).

The totals of each disease add up to more than 250 emerging autoimmune therapies because many emerging therapies are being developed for treatment of more than one autoimmune disease. In addition, this report includes more than 80 emerging therapies in development for treatment of asthma, more than 50 examples of emerging therapies for treatment of allergies, and more than 30 emerging new therapies in development for prevention of organ transplant rejection.

This intense competition creates a number of additional issues and hurdles for companies developing the therapies for these diseases. These are discussed in Chapter 9. The report also includes interviews of five experts in the field of autoimmune disease and inflammatory disease, who discuss the progress, and the challenges and hurdles, faced by researchers and companies working in this field.