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Hematological Cancer Therapies: Pipelines, Markets, and Business Considerations

Lucy J. Sannes, PhD, MBA

Recent years, newer and more specific therapies for hematological cancers have been developed, such as targeted small-molecule drugs and biological therapies including monoclonal antibodies. However, there remains significant need from a clinical perspective, as well as challenges and opportunities for pharmaceutical companies. This report discusses:

- The different types of hematological cancers, including information about the specific disease, its epidemiology, subtypes and/or staging if appropriate, current therapies, and patient prognosis.

- Pipelines of emerging drugs for treatment of hematological cancers including leukemia, lymphoma, myeloma, myelodysplastic syndromes, and myeloproliferative diseases.

- Business and strategic considerations relating to pharmacological therapy for hematological cancers, including assessment of the current market as well as potential markets for emerging new therapies.

- Major challenges and hurdles for companies developing hematological cancer therapies.

- Interviews with experts in the field of hematological cancers, which were conducted exclusively for this report.

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Hematological cancers arise from and are found in the blood, bone marrow, and lymphatic systems. The first hematological cancer to be identified was Hodgkin’s disease, or Hodgkin lymphoma, in 1832. Since that time, many different hematological cancers have been identified, including the various types of leukemia, lymphoma, and myeloma, plus the myelodysplastic syndromes and myeloproliferative diseases. Altogether, hematological cancers represent a significant medical problem: The American Cancer Society estimated that there would be 140,310 new cases of leukemia, lymphoma, and myeloma, combined, in the United States in 2011, and that 53,010 people would die of these diseases that year.

In this Insight Pharma Report, *Hematological Cancer Therapies: Pipelines, Markets, and Business Considerations*, we focus on the many different types of leukemia, lymphoma, and myeloma, and myelodysplastic syndromes and myeloproliferative diseases. Current pharmacological therapies that are used to treat these various hematological cancers are discussed. In addition, there is significant activity on the part of pharmaceutical companies to further develop a number of the currently available therapies for additional indications.

A primary focus of this Insight Pharma Report is on pipelines of emerging therapies in development for treatment of hematological cancers. Specifically, the clinical development pipelines for leukemia, lymphoma, myeloma, myelodysplastic syndromes, and myeloproliferative diseases are reviewed. Selected emerging therapies that are in Phase II or later stages of development are discussed in the text, while the extensive accompanying tables include these emerging therapies plus many additional earlier-stage therapies that are in development.

Despite the smaller number of patients who develop hematological cancers (compared to solid tumors and certain other, non-oncology markets), it is possible to develop and commercialize hematological cancer therapies that become blockbuster drugs (i.e., with sales exceeding $1 billion per year). *Hematological Cancer Therapies: Pipelines, Markets, and Business Considerations* reviews the current market for hematological cancer drugs and includes sales data for selected drugs. Also addressed are major challenges and hurdles, in addition to other strategic considerations such as personalized medicine, that companies in this market must address. Full transcripts of interviews with experts in the field of hematological cancers are included in this report.

<table>
<thead>
<tr>
<th>Table 6.1. Selected Companies Developing Drugs for Myeloma</th>
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<tbody>
<tr>
<td><strong>Company</strong></td>
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<tr>
<td>4SC AG (Germany)</td>
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<td>Acetyfon Pharmaceuticals</td>
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<td>Adoptimmune</td>
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<td>Advanced</td>
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**SOURCE:** Sannes & Associates, Inc.
This page contains information on emerging therapies for leukemia, including details on various drugs in development and their applications. The table of contents includes chapters on different types of hematological cancers, such as leukemia, lymphoma, and myelodysplastic syndromes. Specific drugs mentioned include:

- Gilead Sciences' GS 1101
- Genzyme's/Sanofi's Clolar
- EpiCept's Ceplene
- Eisai's and Janssen-Cilag's Dacogen
- CytRx' Bafetinib
- Cyclacel Pharmaceuticals' Sapacitabine
- Clavis Pharma's Elacytarabine
- Chroma Therapeutics' and Cell Therapeutics'
- Celgene's REVLIMID
- Boehringer Ingelheim's Volasertib
- AstraZeneca's AZD1152
- Astellas Pharma's and Ambit Biosciences'
- ARIAD Pharmaceuticals' Ponatinib
- Astellas Pharma's YM155
- BioCryst Pharmaceutical's Forodesine
- Celgene's REVLIMID
- Allos Therapeutics' Folotyn
- Abbott's and Genentech/Roche's Navitoclax
- Amgen's Nplate (Romiplostim)

The text also discusses the current pharmacological treatment options for hematological cancers, with a focus on leukemia. The section on current pharmacological treatment options includes various drugs in development for different types of hematological cancers, such as:

- Chronic Myelogenous (Myeloid) Leukemia (CML)
- Acute Myelogenous (Myeloid) Leukemia (AML)
- Chronic Lymphocytic Leukemia (CLL)
- Acute Lymphocytic Leukemia (ALL)

Furthermore, the document covers emerging therapies for myelodysplastic syndromes, lymphoma, and myeloproliferative diseases, with a comprehensive overview of the current landscape and potential future treatments.