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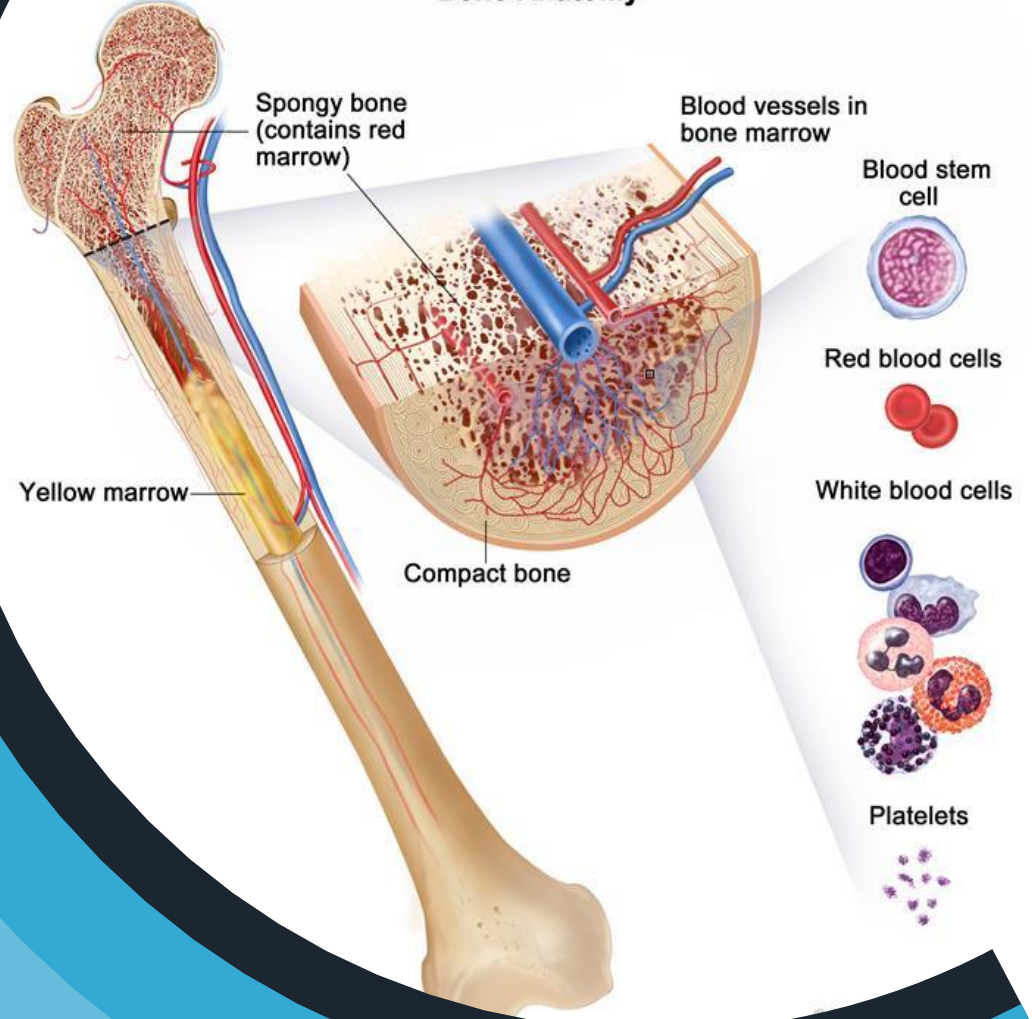

Global CML Market Report: 2022

August 2018

Table of Contents

List of Figures

List of Tables

Glossary

- **BCR-ABL:** The ABL gene from chromosome 9 joins to the BCR gene on chromosome 22, to form the BCR-ABL fusion gene. The changed chromosome 22 with the fusion gene on it is called the Philadelphia chromosome. The BCR-ABL fusion gene is found in most patients with chronic myelogenous leukemia, and in some patients with acute lymphoblastic leukemia or acute myelogenous leukemia.
- **CML:** Chronic myeloid leukemia, also known as chronic myelogenous leukemia, is a type of cancer that starts in certain blood-forming cells of the bone marrow.
- **ALL:** Acute lymphoblastic leukemia, is a cancer that starts from the early version of white blood cells called lymphocytes in the bone marrow (the soft inner part of the bones, where new blood cells are made).
- **AML**: Acute myeloid leukemia, is a cancer of the myeloid line of blood cells, described by the fast growth of abnormal cells that build up in the bone marrow and blood and affect normal blood cells.
- **Ph +:** Philadelphia chromosome positive
- **TKIs:** Tyrosine kinase inhibitors, a substance that blocks the action of enzymes called tyrosine kinases. Tyrosine kinases are a part of many cell functions, including cell signaling, growth, and division. These enzymes may be too active or found at high levels in some types of cancer cells, and blocking them may help keep cancer cells from growing. Some tyrosine kinase inhibitors are used to treat cancer. They are a type of targeted therapy.
- **c-Kit (CD117):** A protein found on the surface of many different types of cells. It binds to a substance called stem cell factor (SCF), which causes certain types of blood cells to grow. C-Kit may also be found in higher than normal amounts, or in a changed form, on some types of cancer cells, including gastrointestinal stromal tumors and melanoma. Measuring the amount of c-Kit in tumor tissue may help diagnose cancer and plan treatment. C-Kit is a type of receptor tyrosine kinase and a type of tumor marker. Also called CD117 and stem cell factor receptor.
- **SCF:** Stem cell factor, is a cytokine that binds to the c-KIT receptor. SCF can exist both as a transmembrane protein and a soluble protein.
- **MDS/ MPD:** Myelodysplastic syndromes, are a group of cancers in which immature blood cells in the bone marrow do not mature and therefore do not become healthy blood cells**.**

- **PDGFR:** Platelet-derived growth factor receptors, are cell surface tyrosine kinase receptors for members of the platelet-derived growth factor (PDGF) family.
- **CTG:** Loss of cytogenetic
- **CHR:** Complete hematological response
- **NCI:** National Cancer Institute
- **ASM:** Aggressive systemic mastocytosis, is a severe and rare form of systemic mastocytosis (SM) characterized by considerable infiltration of mast cells in different tissues.
- **HES:** Hypereosinophilic syndrome, constitutes a rare and heterogeneous group of disorders, defined as persistent and marked blood eosinophilia and/or tissue eosinophilia associated with a wide range of clinical manifestations reflecting eosinophil-induced tissue/organ damage.
- **CEL:** Chronic eosinophilic leukemia, has not yet been clearly defined, mainly due to the fact that it has not been conclusively shown as a monoclonal disease which should be separated from chronic myelogenous leukemia, acute myelogenous leukemia with eosinophilia (AML, FAB M4Eo), and the idiopathic hypereosinophilic syndrome.
- **FIP1L1:** Factor interacting with PAPOLA and CPSF1 (i.e., FIP1L1; also termed Pre-mRNA 3'-end-processing factor FIP1) is a protein that in humans is encoded by the FIP1L1 gene (also known as Rhe, FIP1, and hFip1).
- **GIST:** Gastrointestinal stromal tumor, is the most common mesenchymal neoplasm of the gastrointestinal (GI) tract, typically presenting in adults over the age of 40 (mean age 63), and only rarely in children, in various regions of the GI tract, most commonly the stomach or small intestine but also less commonly in the esophagus, appendix, rectum and colon. GISTs can be asymptomatic or present with various non-specific signs, depending on the location and size of tumor, such as loss of appetite, anemia, weight loss, fatigue, abdominal discomfort or fullness, nausea, vomiting, as well as an abdominal mass, blood in stool, and intestinal obstruction. GISTs can also be seen in familial syndromes such as Carney triad and neurofibromatosis type 1.
- **DFSP:** Dermatofibrosarcoma protuberans, is a rare tumor. It is a rare neoplasm of the dermis layer of the skin, and is classified as a sarcoma. There is only about one case per million per year. DFSP is a fibro sarcoma, more precisely a cutaneous soft tissue sarcoma.

- **BC:** Blast crisis, is the final phase in the evolution of CML, and behaves like an acute leukemia, with rapid progression and short survival. In the blast phase, there are 20% or more blasts in the blood or bone marrow, and it is difficult to control the number of white blood cells.
- **AP:** Accelerated phase, here is no single definition of accelerated phase. However, most patients with this phase of CML have 10 to 19 percent blasts in both the blood and bone marrow or more than 20 percent basophils in the peripheral blood.
- **CP:** Chronic phase, the blood and bone marrow contain less than 10 percent blasts. Blasts are immature white blood cells. This phase can last for several years. However, without effective treatment, the disease can progress to the accelerated or blast phases. About 90 percent of people have chronic phase CML when they are diagnosed.
- **DMF**: drug master files
- **ANDA**: Abbreviated new drug approval
- **EMA:** European Medicines Agency
- **PAH:** Pulmonary arterial hypertension, is a group of diseases characterized by elevated pulmonary arterial resistance leading to right heart failure. PAH is progressive and potentially fatal. PAH may be idiopathic and/ or familial, or induced by drug or toxin (drug-or toxin-induced PAH, see these terms) or associated with other diseases like congenital heart disease, connective tissue disease, HIV, schistosomiasis, portal hypertension (PAH associated with other disease, see this term).
- **PFS:** Progression-free survival
- **FGFR:** Fibroblast growth factor receptor
- **PCgR:** Partial cytogenetic response
- **CHR:** Complete hematologic response
- **MCyR:** Major cytogenetic response
- **MMR:** Major molecular response
- **CCyR:** Complete cytogenetic response
- **B-CLL:** B-cell chronic lymphocytic leukemia
- **INF:** Interferon

- **rINF α :** Recombinant Interferon alpha
- **COMP:** Committee for Orphan Medicinal Products
- **SRC:** also known as proto-oncogene C-SRC or simply SRC, is a non-receptor tyrosine kinase protein that in humans is encoded by the SRC gene
- **EGFR:** Epidermal growth factor receptor
- **HER2:** It is a member of the human epidermal growth factor receptor (HER/EGFR/ERBB) family
- **erbB:** It is a family of proteins contains four receptor tyrosine kinases, structurally related to the epidermal growth factor receptor (EGFR)
- **CMPH**: Committee for Medicinal Products for Human Use
- **PBSCT:** Peripheral Blood Stem Cell Transplant
- **BMT:** Bone Marrow Transplant
- **SCT:** Stem Cell Transplant
- **qPCR:** Quantitative Polymerase Chain Reaction
- **FISH:** Fluorescence in Situ Hybridization
- **FBC:** Full Blood Count
- **CBC:** Complete Blood Count

Executive Summary

Chronic myeloid leukemia (CML) is a clonal myeloproliferative disorder resulting from the neoplastic transformation of the primitive hematopoietic stem cell. It is one of the rarest forms of leukemia in terms of annual incidence and accounts for 10 percent to 15 percent of all incident leukemia cases. However, it is anticipated to become the most widespread hematologic disorders in the globe by 2022. CML is an increasingly attractive market opportunity given the transformational impact of targeted therapies on disease progression and survival; the prevalence of CML is now increasing as patients now have a normal lifespan and no longer die shortly after diagnosis, and due to the aging population. The main unmet needs in CML are overcoming TKI resistance and addressing patients with accelerated or blast phase disease. At Present, existing BCR-ABL inhibitors form the stronghold of CML treatment comprises first-generation imatinib and more effective second-generation BCR-ABL inhibitors nilotinib and dasatinib, with ponatinib and bosutinib having been lately approved for market inclusion.

The global CML market is estimated to register 5 percent CAGR during the forecast period 2017 to 2022. The market expected to reach $13,840 million in revenue by the end of 2022. Some of the factors such as a rise in research and development in oncology sector, innovation in drug development, new products in the pipeline, and the establishment of novel therapies are resulting in the growth of the market. However, the high cost of treatment and lack of Medicare coverage is one of the biggest factor hampering the growth of the global CML market. The US was observed as the leading geographical region in CML treatment due to factors such as rising diagnosis rate in patients suffering from cancer, rising awareness in CML patients related to novel drug treatments and highest yearly treatment price compared to others regions.

Generic imatinib will dominate first-line treatment as Sprycel and Tasigna compete for market share in the second line. The most lucrative CML segment is chronic phase-CML patients currently taking frontline therapy. This segment contains the greatest number of patients, and these patients stay on their treatment for longer than patients who have become refractory to or intolerant of prior therapy. Competition for patient share in this segment is fierce, and Novartis and BMS are trying to prove that their second-generation TKIs are superior to Gleevec. ARAID is also aiming to penetrate this segment. Improving CML patients' compliance with their prescription regimens is of great commercial interest to companies in this space. Consequently, the major players have instituted programs and tools to encourage patients to adhere to their prescribed frequency of dosing.

The CML market is witnessed as the most diversified and competitive market comprising a huge number of players. The market is dominated by numerous players. The main players in this market are Novartis, Bristol-Myers Squibb, Teva., ARIAD Pharmaceuticals, Pfizer, Prism Pharmaceuticals, Incyte, Otsuka Pharmaceutical. and Others. Companies planning to enter into this market are look at different targets and indications in order to capture share in the market.

Novartis and Roche are the most active assignees in the year 2017. The patents/published applications from Novartis majorly focus on Inhibitors to be used for the treatment of Chronic Myeloid Leukemia wherein the patents/published applications from Roche majorly focus on Novel compounds for the treatment of Chronic Myeloid Leukemia. Novartis has majority of patenting activity in the year 2018 as well. Many patents are being filed on treatment method and formulations. Thus, the industry may need the API to conduct research and produce specific formulas using the API. There are patents assigned to Cadila (estimated expiry of 2033) & Teva Pharmaceuticals which claim a highly stable amorphous form of Imatinib mesylate. None of them shows an EP counterpart. Interestingly, US patent for Teva expired in the year 2015 and thus, the possibility of freedom to operate in EP for the stable amorphous imatinib mesylate is high.

The report provides strategic insights into the overall CML treatment market accompanied by the market size and forecast for the period 2012 to 2022 for major regions along with approved product sales. The report covers in-depth product profiles of imatinib (Gleevec), nilotinib (Tasigna), dasatinib (Sprycel), bosutinib (Bosulif), ponatinib (Iclusig) and omacetaxine (Synribo) with market sales data for 2014 to 2022. It also provides detailed epidemiology of the CML of by major regions (US, the Americas, Europe and Asia Pacific). Some key players profiled in this report are Novartis, Roche, Teva and Pfizer.

Key Question Answered

- Global CML Market by Value; 2008-2022
- Global CML Market by Drugs; 2017-2022
- Global Tyrosine Kinase Inhibitors Market by Value; 2008-2022
- Annual Price Change in Tyrosine Kinase Inhibitors; 2013-2016
- US CML Market by Value; 2008-2022
- US CML Market by Drugs; 2017-2022

- Gleevec Worldwide Sales; 2012-2022
- Gleevec Worldwide Sales by Indication; 2014-2022
- Tasigna Worldwide Sales; 2012-2022
- Tasigna US Sales; 2012-2022
- Sprycel Worldwide Sales; 2012-2022
- Sprycel US Sales; 2012-2022
- Iclusig Worldwide Sales; 2013-2022
- Iclusig Sales by Regions; 2013-2022
- Iclusig Sales by Players; 2013-2022
- Iclusig Sales by Indications; 2017
- Bosulif Worldwide Sales; 2012-2022
- Bosulif US Sales; 2012-2022
- Global Prevalence of CML; 2014-2022
- Americas Prevalence of CML; 2014-2022
- Europe Prevalence of CML; 2014-2022
- Asia Pacific Prevalence of CML; 2014-2022
- Overall IP Trends in CML Market
- Patent landscape of CML Market by Focus Area, Composition, TKIs, Route of Administration, Key Players and Top Inventors.
- Growth Drivers, Challenges and Trends
- Detailed Company Profiling of Key Market Players

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