

## Introduction

ChemGenex is an emerging oncology focused biopharmaceutical company that develops small molecules with new mechanisms of action to address unmet medical needs in major hematological disorders.

ChemGenex is managed by a strong board and senior management team. The board consists of executives with significant commercial experience in the pharmaceutical industry and the senior management team has a proven track record in clinical development, regulatory approval and commercialization of drug products.

ChemGenex aims to develop personalized medicines and clinical development activities are focused on specific populations, as defined by disease or patient genotype. By focusing on more specific targets and leveraging the genetic differences between patients ChemGenex seeks to increase efficacy, reduce side-effects and increase the likelihood of clinical trial success.

ChemGenex is listed on the Australian Stock Exchange (CXS).

## Product Pipeline

### *Omacetaxine mepesuccinate*

The lead product candidate, omacetaxine is a first-in-class cetaxine with demonstrated clinical activity in major hematological disorders including Chronic Myeloid Leukemia (CML), Acute Myeloid Leukemia (AML) and Myelodysplastic Syndrome (MDS).

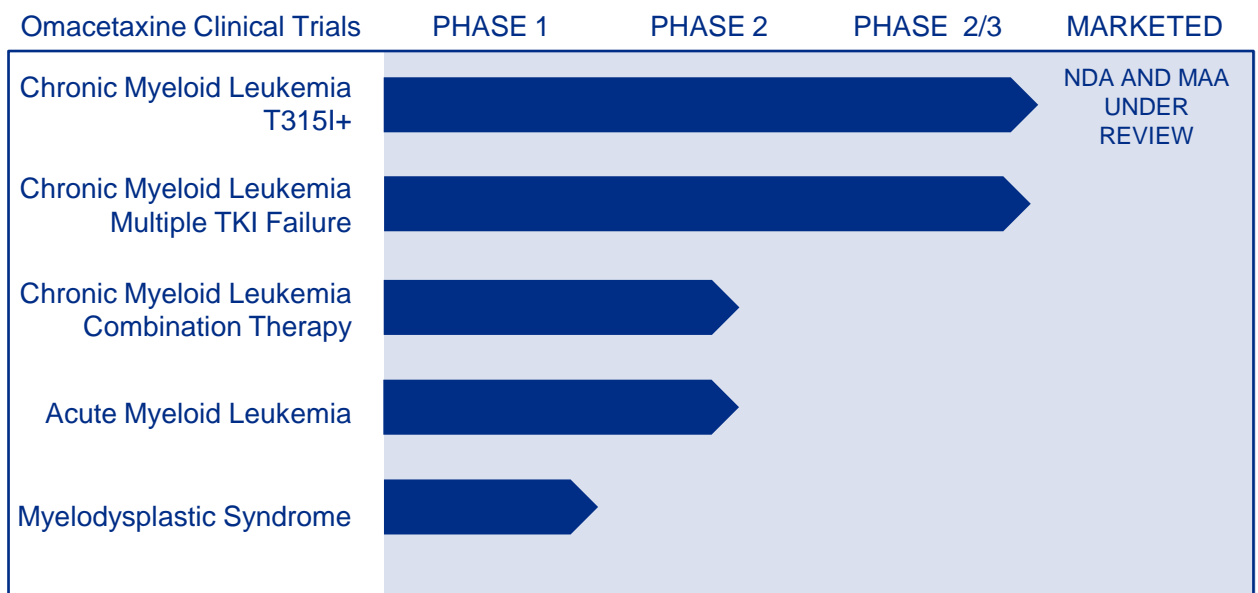
Omacetaxine induces apoptosis by inhibiting key oncoproteins such as Mcl-1 and C-myc. Omacetaxine's novel mechanism of action is independent of tyrosine kinase inhibitors (TKIs), making it effective against major TKI mutations, including the T315I mutation.

Unlike TKIs, omacetaxine has been shown to be effective in killing CML stem cells as well as peripheral leukemic cells.

Omacetaxine has been granted orphan drug status in the USA (CML & MDS) and EU (CML & AML) and has also received Fast Track designation from the US FDA.

Omacetaxine is currently being investigated in two international phase 2/3 clinical trials in CML. The first study is a pivotal trial in patients who have developed resistance to the TKI imatinib mesylate and who have the T315I Bcr-Abl kinase domain mutation. This mutation is resistant to treatment with all approved TKIs and therefore represents an important and growing clinical problem.

Clinical data from this study has been presented in several fora, the most recent being at the American Society of Hematology (ASH) annual meeting in December 2009, where data from 81 patients were presented (49 in chronic phase, 17 in accelerated phase and 15 in blast phase).



Highlights of the data presented at ASH include:

- Complete hematologic responses (CHR) in 86% of chronic phase patients, median response duration 9 months
- Total cytogenetic response rate of 41% in chronic phase patients, with major cytogenetic response (MCyR) rate of 27%
- Overall hematologic responses in 35% of accelerated phase patients (median duration 7 months)
- Overall hematologic responses in 47% of blast phase patients (median duration 2 months)
- Investigators reported that omacetaxine is safe for self-administration, is well tolerated, and that reversible and manageable myelosuppression is the most common side effect

A complimentary phase 2 CML study in patients who are resistant or intolerant to more than one TKI, irrespective of their Bcr-Abl mutation status in being completed. A positive outcome from this study may help position omacetaxine as the standard of care for CML patients whose disease is not controlled by TKIs for any reason.

ChemGenex has completed a New Drug Application (NDA) which was accepted by the U.S. Food and Drug Administration (FDA) and a Marketing Authorization Application (MAA) that has been validated by the European Medicines Agency for CML patients with the Bcr-Abl T315I mutation. The first approval (in CML patients with T315I mutation) is anticipated in 2010.

While ChemGenex will be seeking initial approval of omacetaxine for the treatment of CML, it has ongoing clinical development for MDS and AML. In addition to these indications, the novel mechanism of action for omacetaxine may also be relevant in the treatment of additional types of tumors.

With the possibility of Omapro™ (omacetaxine mepesuccinate) receiving marketing approval by the FDA in 2010 ChemGenex is preparing to independently commercialize the drug in the USA. In Europe, the Middle East and parts of Africa ChemGenex and Hospira, Inc. have entered into an exclusive agreement to license, develop and commercialize omacetaxine. For the remaining rest of world territories ChemGenex continues to hold the rights to omacetaxine.

#### **Quinamed®** (Amonafide dihydrochloride)

Is a novel topoisomerase II inhibitor and has completed a phase 1/2 clinical study as a potential treatment for solid tumor cancers. A maximum tolerated dose (MTD) was established according to patients' N-acetyl transferase (NAT) genotype. In the phase 2 part of this study, patients were prospectively treated at the dose appropriate to their genotype, resulting in an absence of dose limiting toxicity.

In addition, evaluable clinical and/or biochemical responses were observed in patients suffering from prostate, ovarian or GIST cancer. All patients had failed multiple prior regimens of chemotherapy.

The final data from the phase 1/2a study were presented at the ASCO annual conference in Chicago in 2007. Based on the results of the phase 1/2 study ChemGenex is preparing to initiate a phase 2 study in a defined patient population, most probably in hormone refractory prostate cancer.

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