
# Corporate Update

April 9 2009

CXSP
NASDAQ
LISTED

CXS
ASX
LISTED

# Safe Harbor Statement and Recognition of Trademarks

Certain statements made herein that use the words “estimate”, “project”, “intend”, “expect”, “believe,” and similar expressions are intended to identify forward-looking statements within the meaning of the US Private Securities Litigation Reform Act of 1995. These forward-looking statements involve known and unknown risks and uncertainties which could cause the actual results, performance or achievements of the company to be materially different from those which may be expressed or implied by such statements, including, among others, risks or uncertainties associated with the development of the company’s technology, the ability to successfully market products in the clinical pipeline, the ability to advance promising therapeutics through clinical trials, the ability to establish our fully integrated technologies, the ability to enter into additional collaborations and strategic alliances and expand current collaborations and obtain milestone payments, the suitability of internally discovered genes for drug development, the ability of the company to meet its financial requirements, the ability of the company to protect its proprietary technology, potential limitations on the company’s technology, the market for the company’s products, government regulation in Australia and the United States, changes in tax and other laws, changes in competition and the loss of key personnel. These statements are based on our management’s current expectations and are subject to a number of uncertainties that could change the results described in the forward looking statements. Investors should be aware that there are no assurances that results will not differ from those projected.







CHEMGENEX PHARMACEUTICALS

# Agenda

- Overview
- Market opportunity
- Development update
- Commercialization strategy
- Upcoming Milestones



CHEMGENEX PHARMACEUTICALS

# Company Overview

- Oncology focused biopharmaceuticals company
  - Novel small molecule therapeutic solutions
  - New mechanism of action
  - Addressing unmet medical needs in major hematological disorders
- Late-stage product pipeline
  - NDA and MAA filings 2009
  - Additional indications in late clinical development
- Strong board and senior management team
- Listed on ASX (CXS) and Nasdaq (CXSP)

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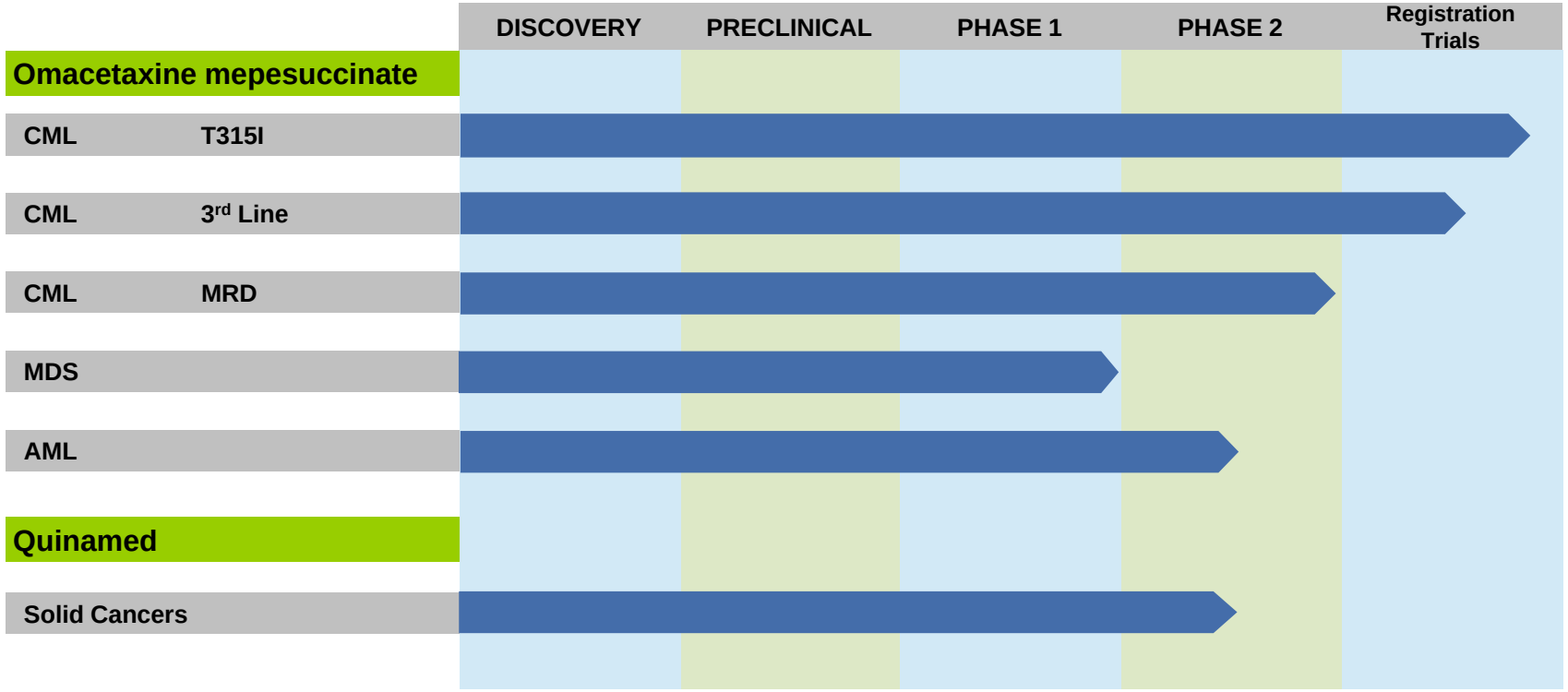
# Pipeline





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# Omacetaxine Profile

- Broad clinical efficacy
  - Demonstrated clinical activity major diseases; CML, MDS and AML
- Novel mechanism of action
  - Induces apoptosis by inhibition of key oncoproteins, particularly Mcl-1 and XIAP
  - Acts independently of tyrosine kinase inhibitors
  - Effective against major TKI resistance mutations
  - Complementary mechanism of action to current therapies
  - Effective at killing CML stem cells as well as peripheral leukemic cells, unlike the tyrosine kinase inhibitors
- Patient convenient administration
- US patents covering manufacturing, uses and formulations
- Orphan drug designation in US and EU in CML

# Commercialization of Omacetaxine

- T315I+ CML: Lead indication
  - Speed to market strategy
  - Niche market: will require a niche strategy to be profitable
- The full potential of omacetaxine driven by:
  - Approval in Myelodysplastic Syndrome (MDS) and/or
  - Development in CML Minimal Residual Disease (MRD)
- Focused capital requirements
  - Complete development of T315I+ CML indication
  - Establish corporate partnership for Omacetaxine EU/ex US
  - Launch of Omacetaxine in US market for T315I+ CML

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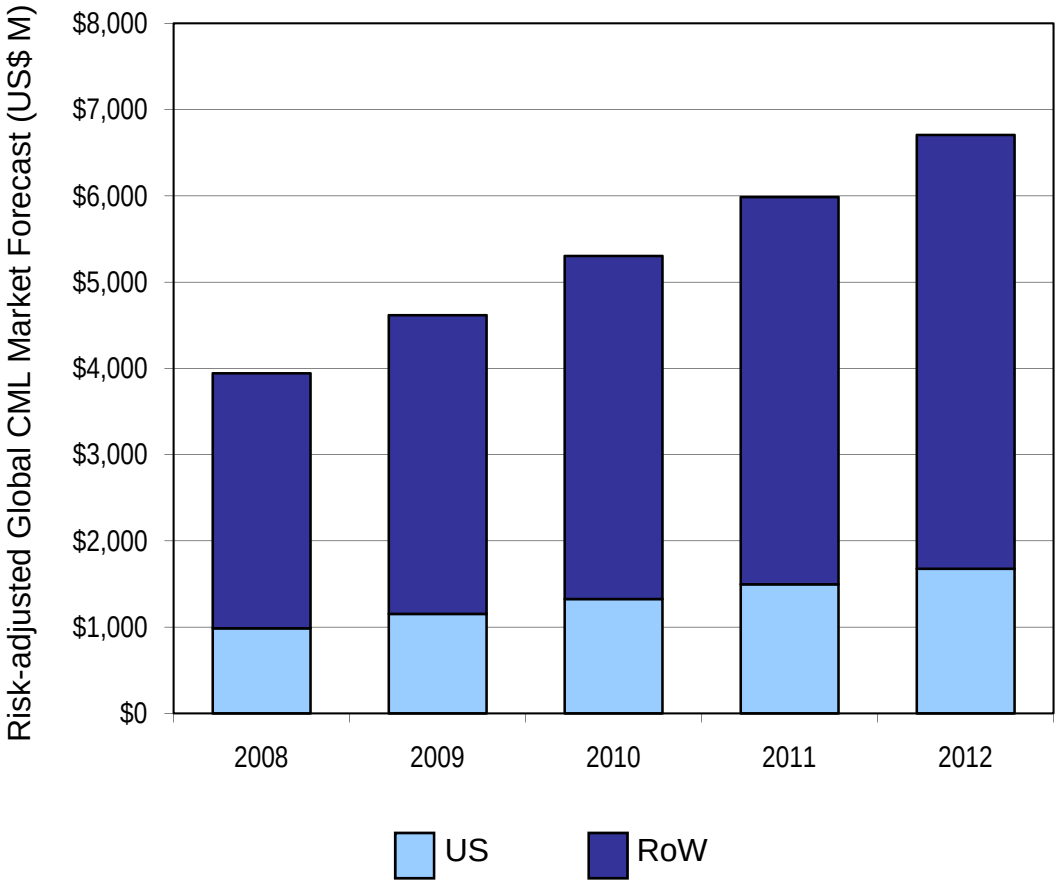
# Present and Emerging Opportunities in CML

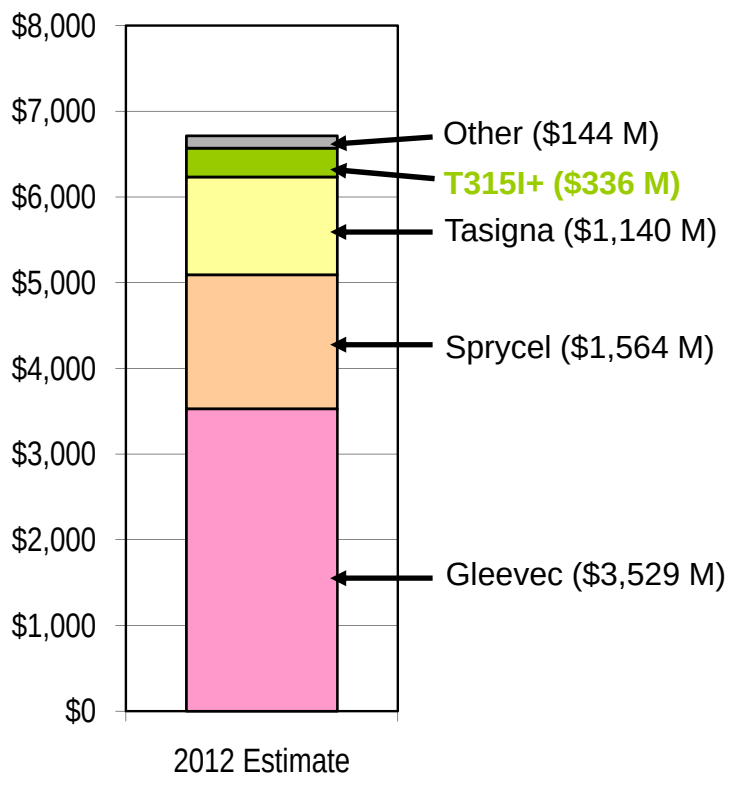
- World CML prevalence >150,000 patients and growing
  - Market growth 18% (CAGR)
- Gleevec® (imatinib) approved in 2001 – first effective therapy
  - Targeted tyrosine kinase inhibitor (TKI)
  - Global sales of US$3.7 billion in 2008 (60-70% in CML) (~ US$46-74k pa)
- Current challenges with Gleevec
  - Resistance is an emerging issue in CML
  - Gleevec is a suppressant not an eradication therapy - Minimal Residual Disease
- Resistance is linked to Bcr-Abl point mutations
  - 44% of TKI failures have mutations - T315I most frequent (15-20%)
- Two approved second line therapies
  - Sprycel (dasatinib) by BMS approved in June 2006 (~ US$68k pa)
  - Tasigna (nilotinib) by Novartis approved in October 2007 (~ US$79k pa)
  - Second generation and TKIs in development are ineffective against T315I mutation
- No therapeutic options for 3rd line interventions

# Estimates Put Global CML Market Growth Above 18%

**Estimated Market Size**




**Estimated Market Structure**




Source: Bernstein Research January 2007





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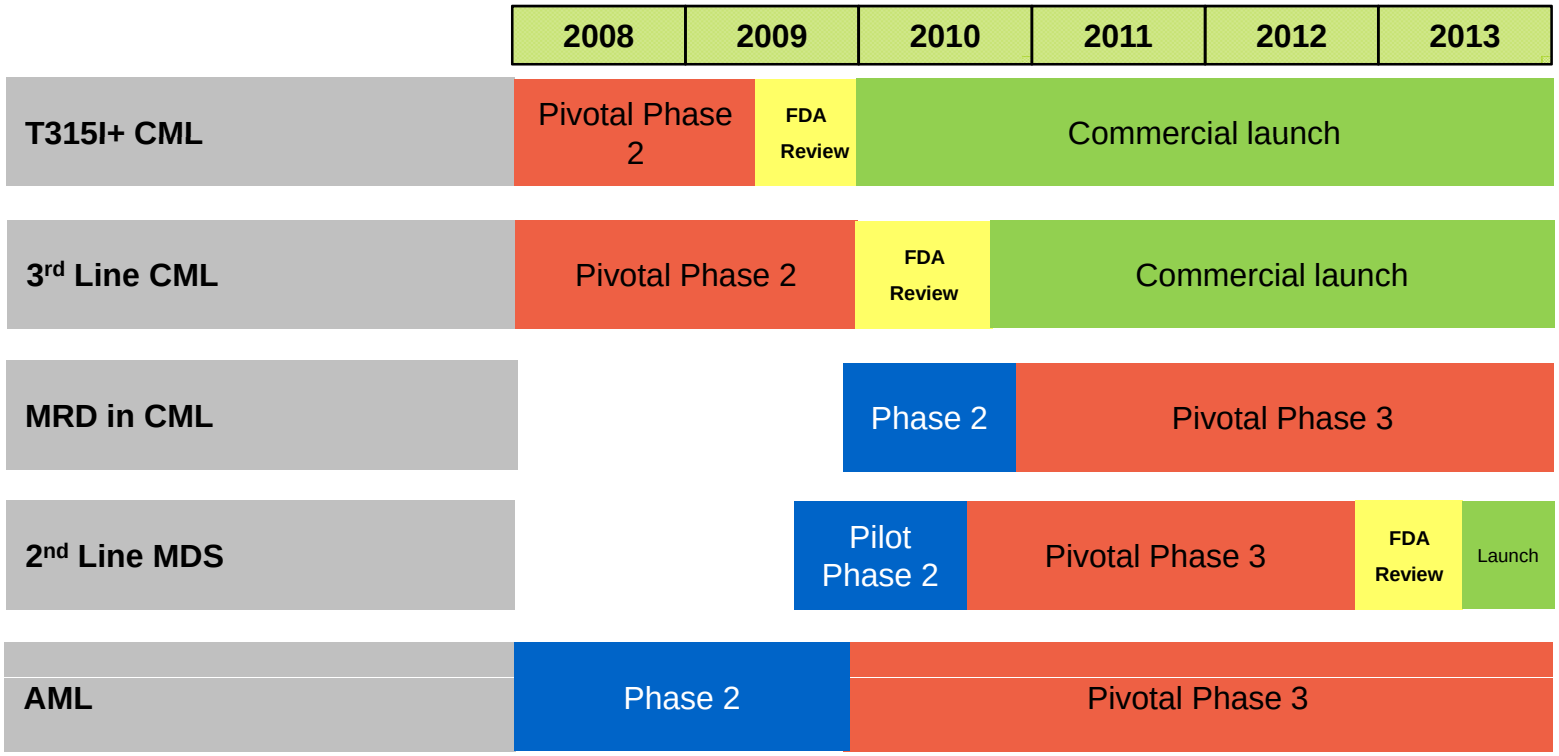
# Omacetaxine Clinical and Regulatory Timeline

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# T315I+ CML Regulatory Strategy - USA

- Clinical pre-NDA meeting with FDA completed in March
  - Structural format of NDA was accepted by the Oncology Division
- Rolling NDA submission to FDA per Fast Track designation
  - Non-clinical section – Submitted June 2008
  - CMC section – Q2 2009
  - Clinical Section – Mid-2009
  - Approval and launch anticipated Q1 2010

# T315I+ CML Regulatory Strategy - Europe

- EMEA – centralized filing
  - Letter of intent filed in March 2009
  - Pre-MAA meeting scheduled for Q2 2009
  - MAA submission Q4 2009
  - Approval and launch anticipated end of Q3 2010

# Clinical Enrollments and Results Communication

- Recently completed enrollments for chronic phase T315I+ CML patients
- Study 203 enrolments tracking well
- Data from 203 and 203 studies to be presented at the ASCO Annual Meeting in Orlando – May 29 – June 2
- Additional presentations on 202, 203 and human stem cell activity at EHA Annual Meeting in Berlin – June 4-7

# Study 202 – Hematologic and Cytogenetic Responses

| Response<br>Number (%) | Chronic Phase<br>N=25 | Accelerated Phase<br>N=11 | Blast Phase<br>N=8 |
|---|---|---|---|
| Hematologic Response | | | |
| Overall | 20 (80) | 5 (45) | 1 (13) |
| Complete Hematologic Response (CHR) | 20 (80) | 2 (18) | - |
| Hematologic Improvement (HI) | NA | 1 (9) | - |
| Return to Chronic Phase (RCP) | NA | 2 (18) | 1 (13) |
| Cytogenetic Response | | | |
| Overall | 7 (28) | 1 (9) | - |
| Major* | 5 (20) | - | - |
| Complete | 4 (16) | - | - |
| Partial | 1 (4) | - | - |
| Minimal | 2 (8) | 1 (9) | - |
| Molecular Response | | | |
| Major | 2 (8) | - | - |

*One complete and one partial cytogenetic response are unconfirmed

# Study 202 – Duration of Responses

| Median Duration of Response Months (range) | Chronic Phase N = 20 | Accelerated Phase N = 5 | Blast Phase N = 1 |
|---|---|---|---|
| Hematologic Response | | | |
| CHR | 11.5 (3.5 - 25.4+) | 9.6 (8.3 – 10.9+) | - |
| HI | NA | 2.8 | - |
| RCP | NA | 2.0 (2.0 - 2.0+) | 3.4 |
| Cytogenetic Response | | | |
| Complete | 4.8 (0.3 – 9.7+) | - | - |
| Partial | 4.2 | - | - |
| Minimal | 4.0 (3.9 - 20.8+) | 2.3 | - |

# Overall Safety Profile

- The primary toxicity is myelosuppression
  - Easily managed and controlled by reducing the number of dosing days per cycle
- Typical cytotoxic side effects are uncommon
  - Alopecia, nausea, vomiting, diarrhea, mucositis, edema and hepatic toxicity are absent/infrequent (<10%) and mild
- No treatment-related deaths

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# Commercialization Strategy

- Strategic goal
  - Retain product rights in the USA
  - Out-license other territories to fund product development
- Ideal European partner profile
  - European focused, commercial infrastructure
  - Hem/oncology product and sales force presence
  - 5 major markets plus distributor relationships
- Pursue similar partners in Rest of World
- Phased US commercial launch from Q1 2010





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# Upcoming Milestones

| Event | Timing |
|---|---|
| CMC section of rolling NDA submission | Q2 2009 |
| Clinical data presentations at key international conferences | Q2 2009 |
| Complete filing of rolling NDA submission to FDA | Mid 2009 |
| Establish corporate partnership(s) for EU/ex US | H2 2009 |
| Complete filing of MAA to EMEA | Q4 2009 |
| Anticipated approval and launch of omacetaxine in USA | Q1 2010 |

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