

### **Annual General Meeting**

**CEO Presentation** 

29th November 2010

Dr Greg Collier CEO

www.chemgenex.com

**ASX:CXS** 

# 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 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.

OMAPRO™ is a trademark of ChemGenex Pharmaceuticals Limited



#### Overview







- Biopharmaceutical company delivering novel solutions to cancer patients with unmet needs
  - Expertise in hematologic malignancies
  - Small molecule drugs with novel mechanisms of action
- Lead asset OMAPRO™ (omacetaxine mepesuccinate)
   effective in TKI Resistant Chronic Myeloid Leukemia (CML)
- Completed two pivotal trials;
  - T315I+ CML patients
  - Multi-TKI resistant CML patients



## OMAPRO: A Potential New Treatment for Hematologic Cancers



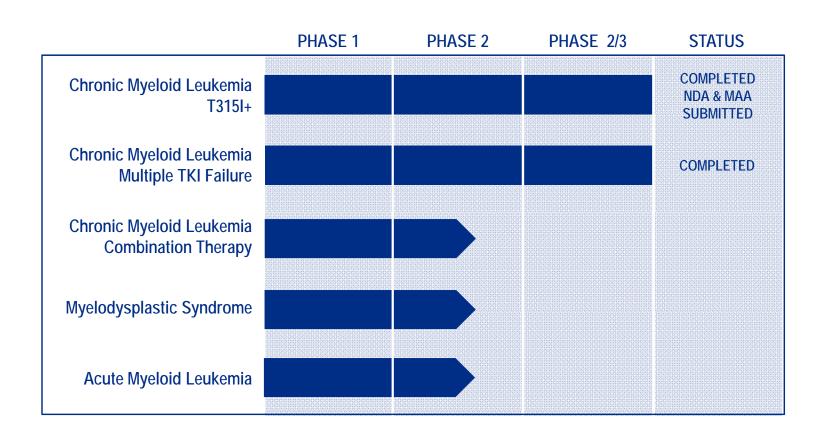
- A first-in-class cetaxine
- Clinical activity as a single agent in CML, AML and MDS
- A unique mechanism of action
  - Specifically binds the ribosomal A-site cleft inhibiting protein translation<sup>1</sup>
  - Selectively reduces the levels of short-lived oncoproteins such as Mcl-1 and c-Myc that are up-regulated in leukemic cells<sup>2</sup>
  - Demonstrated, in vitro, to kill human CML stem cells and peripheral leukemic cells<sup>3</sup>







### **OMAPRO Clinical and Regulatory Status**





# The CML Market & Current Treatment Options



### Paradigm Shift in the Management of CML

- Chronic Myeloid Leukemia (CML)
  - Malignancy of the bone marrow
  - 5,000 new cases per annum in the U.S.
  - Worldwide prevalence > 100,000 patients and growing
- First Line Therapy: Two approved TKIs
  - Imatinib approved in 2001
    - Global sales of US\$2.1 billion in 1H 2010<sup>1</sup>
  - Nilotinib approved in June 2010
- Second Line Therapy: Two approved TKIs
  - Dasatinib approved in June 2006 (US\$265M in 2010 1H sales)<sup>2</sup>
  - Nilotinib approved in October 2007 (US\$164M in 2010 1H sales)<sup>3</sup>

<sup>&</sup>lt;sup>1,3</sup>Novartis June 2010 Finance Report www.novartis.com;

<sup>&</sup>lt;sup>2</sup> Bristol-Myers Squibb June 2010 10Q

## Multiple TKI Resistance represents an increasing unmet medical need in CML



- Patients who have failed imatinib and subsequent therapy with nilotinib or dasatinib due to:
  - Emergence of Bcr-Abl mutations
  - Bcr-Abl over-expression

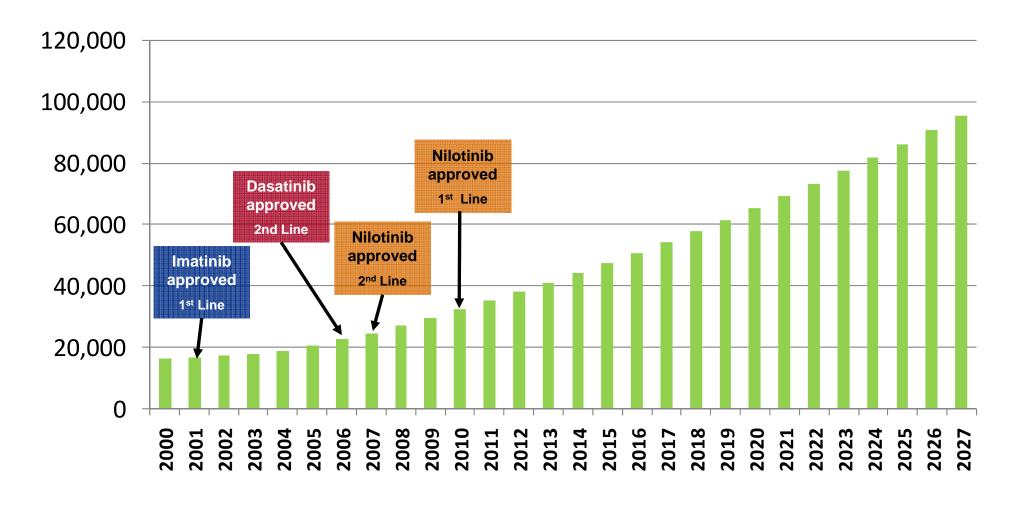


- 45% of patients fail second generation TKIs within the first year<sup>1</sup>
- No approved treatments available for patients who have multiple TKI resistance



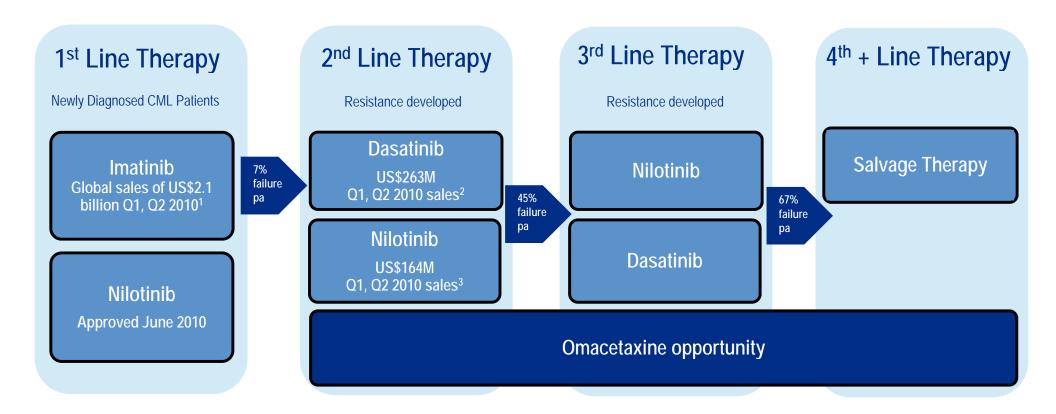


### Reductions in Mortality Increase CML Prevalence





### Multiple TKI Resistance Represents an Increasing Unmet Medical Need in CML





# $\mathsf{OMAPRO}^\mathsf{TM}$ for the treatment of CML



#### **OMAPRO Addresses Unmet Medical Needs**

- OMAPRO™ (omacetaxine mepesuccinate) for subcutaneous injection
- Convenient and safe BID self-administration
  - Induction up to 14 days per month
  - Maintenance up to 7 days per month
- Strong safety profile
  - Myelosuppression is the most common side effect and is normally manageable and reversible
  - Infrequent grade 3/4 non-hematologic events experienced
  - Adverse events easily manageable with dose adjustments
  - Minimal injection site reactions
- Initial indications in multi-TKI resistant CML

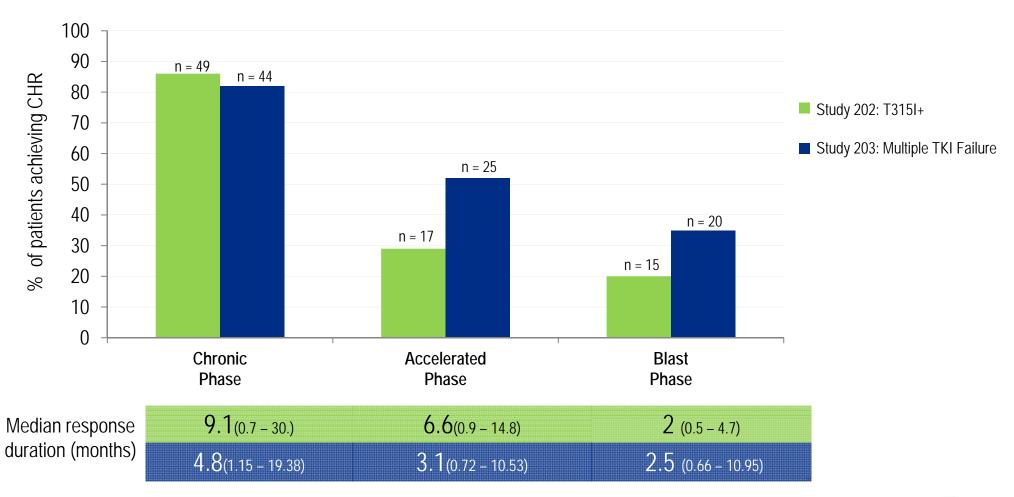




#### Enrollment Completed in Two Phase 2/3 Clinical Trials

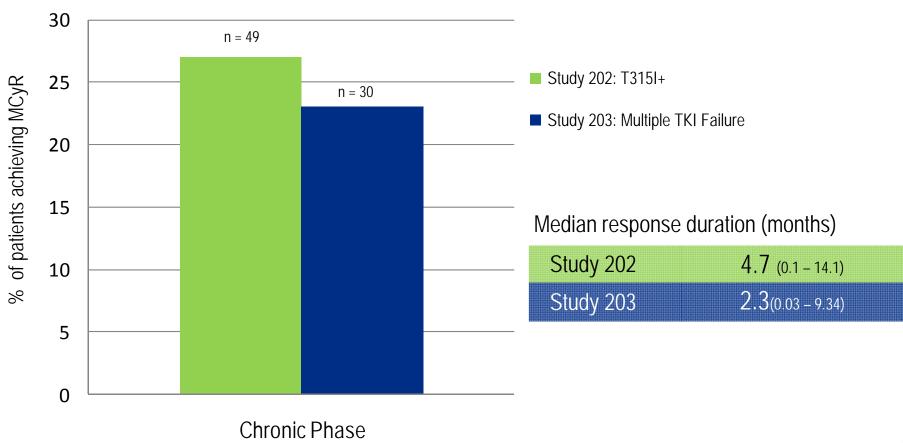
	STUDY 202 CML T315I+ Patients	STUDY 203 Multiple TKI Failure CML
Design	Open label, Single arm	Open label, Single arm
Patients	Enrollment complete 103 patients	Enrollment complete 100 patients
Sites	35 in US, EU, Asia Pacific	35 in US, EU, Asia Pacific
Inclusion criteria	Patients who have failed imatinib and have T315I+ Bcr-Abl mutation	Patients who have failed two or more tyrosine kinase inhibitors
Dose (subcutaneous injection)	<ul> <li>Induction: 1.25 mg/m² two times a day for 14 days, every 28 days; up to 6 cycles</li> <li>Maintenance: as per induction phase, but 7 days treatment every 28 days</li> </ul>	<ul> <li>Induction: 1.25 mg/m² two times a day for 14 days, every 28 days; up to 6 cycles</li> <li>Maintenance: as per induction phase, but 7 days treatment every 28 days</li> </ul>
Primary endpoints	<ul> <li>Cytogenetic response</li> <li>Hematologic response (chronic, accelerated, blast phase)</li> </ul>	<ul> <li>Cytogenetic response</li> <li>Hematologic response (chronic, accelerated, blast phase)</li> </ul>
Status	Completed	Completed

# Hematologic Responses in Patients Treated with OMAPRO





# Cytogenetic Responses in Patients Treated with OMAPRO





### **OMAPRO Regulatory Status**



- Robust efficacy and safety database
- Regulatory filings in process and/or under review
  - U.S. NDA (multi-TKI resistance)
    - Data collection and analysis underway
  - European MAA (T315I+ mutation)









### Corporate Overview



### Corporate Strategy







- U.S. commercialization planned
- Partnered with Hospira in Europe, the Middle East, parts of Africa
  - Upfront payment of A\$17.5 million
  - Potential for an additional €74.1 million based on development and sales milestones plus royalties (CML only)
  - Further milestones and royalties possible with future indications
  - Strong alignment of strategic intent
- ChemGenex retains ROW product rights including North America



### **Recently Secured Capital Position**

- Convertible note with Cephalon Inc.
  - A\$15 million in two tranches
  - Convertible at A\$0.50 per share (13% premium to VWAP)
  - Two conditions
    - Shareholder approval in accordance with Corporations Act item 7, section 611 by 31 December 2010
    - Completion of clinical data collection by 31 March 2011
- Cephalon Inc. option agreements
  - Options to buy 19.9% of common stock for A\$0.70 per share prior to 31 March 2011 or completion of data collection
- Adequate capital to fund operations into Q4 2011

### Strong Board and Senior Management Team

#### Management

Greg Collier, PhD\*

Adam Craig, MD, PhD, MBA

James Campbell, PhD, MBA

Tom O'Neil, BA, MBA

Katie Cairati, MS

Chief Executive Officer and Managing Director

Senior Vice President and Chief Medical Officer

Chief Financial Officer and Chief Operating Officer

Vice President of Finance and Administration

Senior Director of Regulatory Affairs

#### **Board of Directors**

Brett Heading, LLB (Chairman)

Dan Janney, BA, MBA

Geoff Brooke, MBBS, MBA

ElmarSchnee, BComMkting

George Morstyn, MBBS, PhD

Jean-Luc Tétard

McCullough Robertson Lawyers

Alta Partners

**GBS Venture Partners** 

CEO, Merck Serono

Former SVP and CMO, Amgen

President, StragenPharma

CHEMGENEX PHARMACEUTICALS

<sup>\*</sup>Also Board Member

### Financial Snapshot

Financial Parameter	
Shares (ASX: CXS)	283 million
Market capitalization*	A\$ 124 million
	A\$ 12.8 million(as of 30 June 2010)
Cash held	A\$ 10 million – Cephalon convertible note drawn down
	A\$ 5 million – Cephalon convertible note pending
Significant Shareholders	Alta Partners (15%), Stragen Pharma (13%), Orbis Investments (13%), Merck Serono (9%), GBS (8%)

\*Effective 26 Nov 2010 USD/AUD approximately 1.01



### Summary



- OMAPRO is an active drug with a different mechanism of action than current TKIs
- Multiple TKI Resistance represents an increasing unmet medical need



- Completed two pivotal trials offering a potential new treatment option for CML patients
- Commercial strategies in place
  - Omacetaxine partnered in Europe, the Middle East and parts of Africa with Hospira





Strong leadership team and blue chip investors



#### Contacts

<u>Australia</u> <u>USA</u>

Level 4 Suite 200

199 Moorabool St, 4040 Campbell Avenue,

Geelong, Victoria 3220 Menlo Park, CA 94025

Tel: +61 3 5223 9900 Tel: +1 650 804 7660

www.chemgenex.com

