

#### **FOR IMMEDIATE RELEASE**

#### ChemGenex to Present at BIO CEO & Investor Conference

MELBOURNE, Australia, and MENLO PARK, California U.S.A. (9 February 2011) – ChemGenex Pharmaceuticals Limited (ASX: CXS) announced today that Greg Collier, PhD, Chief Executive Officer and Managing Director will present a company update at the BIO CEO & Investor Conference in the Duke of Windsor Room of the Waldorf Astoria Hotel in New York City on Tuesday, 15 February 2011 at 8:00 a.m. Eastern Time in the U.S.

Dr Collier will provide an update on the development of omacetaxine and upcoming milestones. ChemGenex is preparing a New Drug Application (NDA) for omacetaxine in CML patients who have failed therapy with two or more tyrosine kinase inhibitors (TKIs) based on data from its two pivotal trials, and anticipates submission to the U.S. Food and Drug Administration (FDA) in H2 2011. Reflecting the alignment of U.S. and European regulatory strategies for omacetaxine, ChemGenex has withdrawn the prior NDA for omacetaxine.

Dr Collier's presentation, being the company's corporate overview, can be accessed from the company's website; http://www.chemgenex.com/investors/presentations/ and is appended to this announcement.

#### **About ChemGenex Pharmaceuticals Limited**

ChemGenex is an oncology focused biopharmaceutical company developing small molecules with new mechanisms of action to treat malignancies with significant unmet medical needs. A New Drug Application is in preparation for submission to the U.S. Food and Drug Administration in H2 2011 for omacetaxine in CML patients who are resistant to, or have failed two or more currently approved tyrosine kinase inhibitors. A parallel Marketing Authorisation Application is in preparation for submission to the European Medicines Agency in H2 2011. ChemGenex has established a corporate alliance with Hospira Inc. to develop and commercialize omacetaxine in Europe, the Middle East and parts of Africa, and is seeking to establish commercial partnerships in the rest of the world. ChemGenex plans to commercialize omacetaxine itself in North America. ChemGenex trades on the Australian Securities Exchange under the symbol "CXS". For additional information on ChemGenex Pharmaceuticals, please visit the Company's website at http://www.chemgenex.com.

OMAPRO™ is a trademark of ChemGenex Pharmaceuticals Limited.

#### **Contacts**

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#### Safe Harbor Statement

Certain statements made herein (including for this purpose sites to which a hyperlink has been provided) 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.



**Corporate Overview** 

February 2011

www.chemgenex.com

**ASX:CXS** 

# Safe Harbor Statement and Recognition of Trademarks

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#### **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



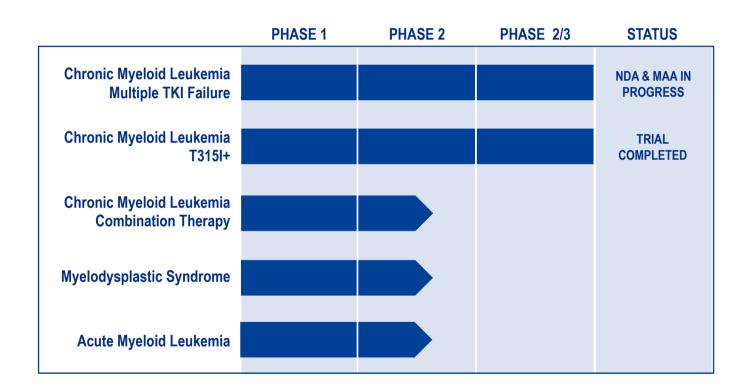
- A first-in-class cetaxine
- Clinical activity as a single agent in CML, AML and MDS
- A unique mechanism of action
  - Specifically binds the ribosomal A-site cleft inhibiting protein translation<sup>1</sup>
  - Selectively reduces the levels of short-lived oncoproteins such as Mcl-1 and c-Myc that 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>



- Recent data suggests that Mcl-1 protein expression may be essential for the survival of AML, CLL and MDS cells
  - Possible that omacetaxine may prove useful in treating these diseases



## OMAPRO Clinical and Regulatory Status





# The CML Market & Current Treatment Options



### Paradigm Shift in the Management of CML

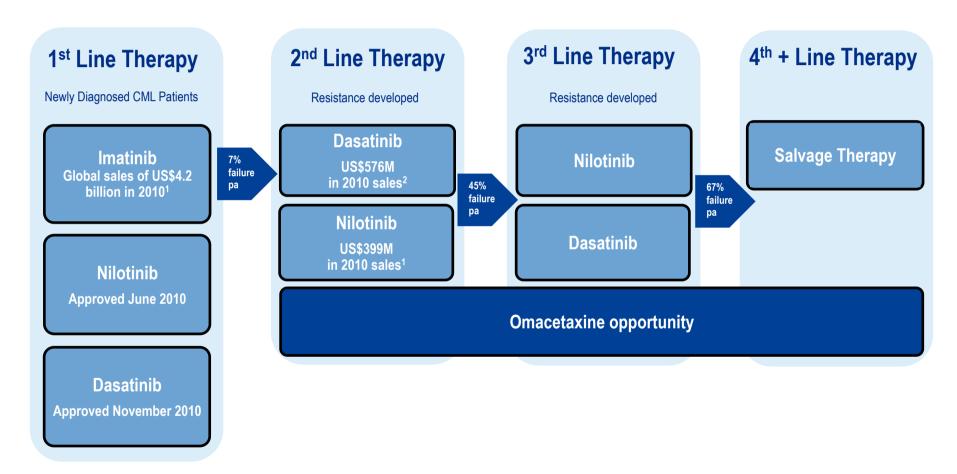
- Chronic Myeloid Leukemia (CML)
  - Malignancy of the bone marrow
  - 5,000 new cases per annum in the U.S.
  - Worldwide prevalence > 100,000 patients and growing
- First Line Therapy: Three approved TKIs
  - Imatinib approved in 2001 (US\$4.26 billion in 2010 sales)<sup>1</sup>
  - Nilotinib approved in June 2010 (US\$399M in 2010 sales)<sup>1</sup>
  - Dasatinib approved in November 2010 (US\$576M in 2010 sales)<sup>2</sup>
- Second Line Therapy: Two approved TKIs
  - Dasatinib approved in June 2006
  - Nilotinib approved in October 2007



<sup>&</sup>lt;sup>1</sup> Novartis Finance Reports www.novartis.com

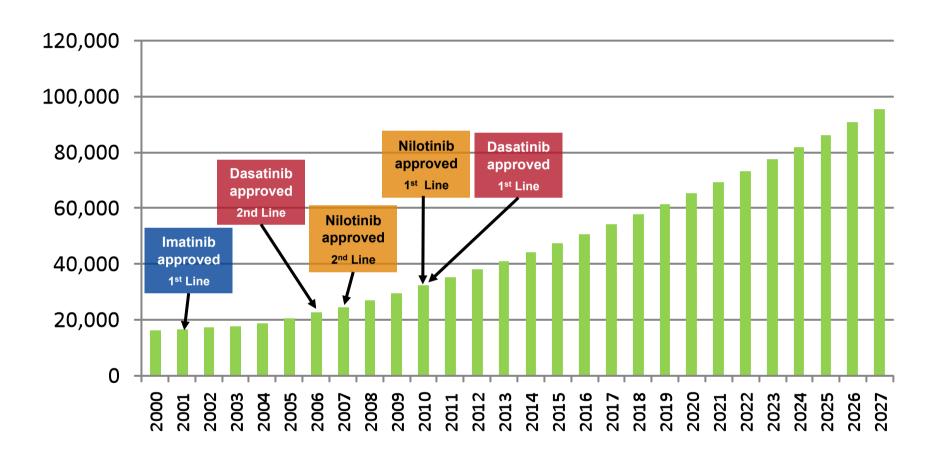
<sup>&</sup>lt;sup>2</sup> Bristol-Myers Squibb quarterly earnings updates

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





### Reductions in Mortality Increase CML Prevalence



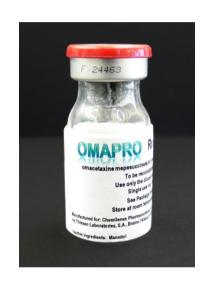


# OMAPRO™ for the treatment of CML



#### **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 indication 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	Fully enrolled	Fully enrolled

### ChemGenex's Approval Strategy For Omacetaxine

- ChemGenex is seeking approval for the treatment of CML-CP and CML-AP patients who are resistant or intolerant to at least 2 approved TKI
- Supplemental analysis CML-300: a combined dataset of patients from Studies CML-202 and CML-203 who meet the above criteria
- Data presented is from patients who had received 2 or more approved TKIs (imatinib, dasatinib and nilotinib)



## Response Rates in All Patients (ITT)

	CML-CP N=85	CML-AP N=37
Hematologic response, n%		
CHR	62 (73)	13 (35)
Cytogenetic response, n%		
Major	17 (20)	0
Complete	9 (11)	0



## **Duration of Response in All Patients (ITT)**

Median Duration of Best Response (months)	CML-CP N = 85	CML-AP N=37
MCyR		
Median	7.4	NA
Range	0.9 - 26+	NA
CHR		
Median	8.24	3.7
Range	0.7 - 42+	0.7 - 14.8+



#### Response Rates in Evaluable Patients\*

	CML-CP N=61	CML-AP N=31
Hematologic response, n %		
CHR	54 (89)	12 (39)
Cytogenetic response, n %		
Major	17 (28)	0
Complete	9 (15)	0

<sup>\*</sup>Evaluable patients are defined as those patients who 1) had been adjudicated by an independent Data Monitoring Committee (DMC) and 2) had a bone marrow report available for cytogenetic assessment.



# Response Rates in Evaluable\* CML-CP Patients who have Failed 2 or 3 TKIs

	IM + DA or NI N=36	IM + DA + NI N=25
Hematologic response, n %		
CHR	32 (89)	22 (88)
Cytogenetic response, n %		
Major	12 (33)	5 (20)
Complete	6 (17)	3 (12)

<sup>\*</sup>Evaluable patients are defined as those patients who 1) had been adjudicated by an independent Data Monitoring Committee (DMC) and 2) had a bone marrow report available for cytogenetic assessment.



#### Most Commonly Reported Hematologic Treatment - Emergent AEs (>5%)

Adverse Event		ic Phase =85
	Overall, n(%)	<b>Grade 3/4,n (%)</b>
Thrombocytopenia	60 (71)	54 (64)
Anemia	47 (55)	29 (34)
Neutropenia	42 (49)	40 (47)
Febrile neutropenia	12 (14)	12 (14)
Leukopenia	19 (22)	18 (21)
Lymphopenia	17 (20)	15 (18)
Pancytopenia	11 (13)	8 (9)
Bone marrow failure	9 (11)	9 (11)

#### Most Commonly Reported Non-Hematologic Treatment-Emergent AE's (>10%)

Adverse Event	Chronic	PhaseN=85
	Overalln, (%)	Grade 3/4n, (%)
Diarrhea	36 (42)	1 (1)
Nausea	29 (34)	1 (1)
Fatigue	24 (28)	4 (5)
Headache	14 (17)	0 (0)
Pain in extremity	14 (17)	1 (1)
Arthralgia	15 (18)	1 (1)
Constipation	15 (18)	0 (0)
Injection site erythema	16 (19)	0 (0)
Asthenia	17 (20)	0 (0)
Pyrexia	19 (22)	1 (1)
Abdominal pain	11 (13)	0 (0)
Vomiting	11 (13)	0 (0)
Abdominal pain upper	12 (14)	0 (0)
Oedema peripheral	12 (14)	0 (0)
Hypertension	9 (11)	1 (1)



## **OMAPRO Regulatory Status**



- Robust efficacy and safety database
- Regulatory filings in process
  - U.S. NDA and European MAA (multi-TKI resistance)
    - Data collection and analysis ongoing



 Individual patients treated globally under compassionate use scheme





# Corporate Overview



#### **Corporate Strategy**

- U.S. commercialization planned
- Partnered with Hospira in Europe, the Middle East, parts of Africa
- Convertible note with Cephalon Inc.
  - A\$15 million in two tranches already received
  - Convertible at A\$0.50 per share (13% premium to VWAP)
  - Convertible upon 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 exercisable 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\*

Chief Executive Officer and Managing Director

Adam Craig, MD, PhD, MBA Senior Vice President and Chief Medical Officer

James Campbell, PhD, MBA Chief Financial Officer and Chief Operating Officer

Tom O'Neil, BA, MBA

Vice President of Finance and Administration

Katie Cairati, MS Senior Director of Regulatory Affairs

#### **Board of Directors**

Brett Heading, BCom, LLB (Chairman)

Dan Janney, BA, MBA

Geoff Brooke, MBBS, MBA

Elmar Schnee, 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, Stragen Pharma



## **Financial Snapshot**

Financial Parameter	
Shares (ASX: CXS)	283 million
Market capitalization*	A\$ 145 million
Cash held	A\$ 12.8 million (as of 30 June 2010) A\$ 15 million – Cephalon convertible note drawn down
Significant Shareholders	Alta Partners (15%), Stragen Pharma (13%), Orbis Investments (13%), Merck Serono (9%), GBS (8%)

\*Effective 4 Feb 2011 USD/AUD approximately 1.00



#### **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
  - U.S. commercialization planned by ChemGenex
- Strong leadership team and blue chip investors





#### **Contacts**

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