



# ChemGenex: New Therapies for Oncology

September 2009

[www.chemgenex.com](http://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



- Oncology focused biopharmaceutical company
  - Small molecule drugs with novel mechanisms of action
  - Development programs addressing
    - serious hematological malignancies
    - significant unmet medical needs



- OMAPRO™ (omacetaxine mepesuccinate)
  - Lead product candidate for TKI-failure CML
  - NDA submitted September 2009, Priority Review requested
  - MAA to be submitted in Q4 2009
  - Orphan Drug designations in CML and MDS
  - Granted U.S. and international patents



# Investment Highlights



- NDA submitted on lead product
- Targeting unmet medical needs
- Growing market opportunity in leukemia
- Global product rights
- Major market exclusivity
- Strong leadership team
- Blue chip investors



# Corporate Strategy



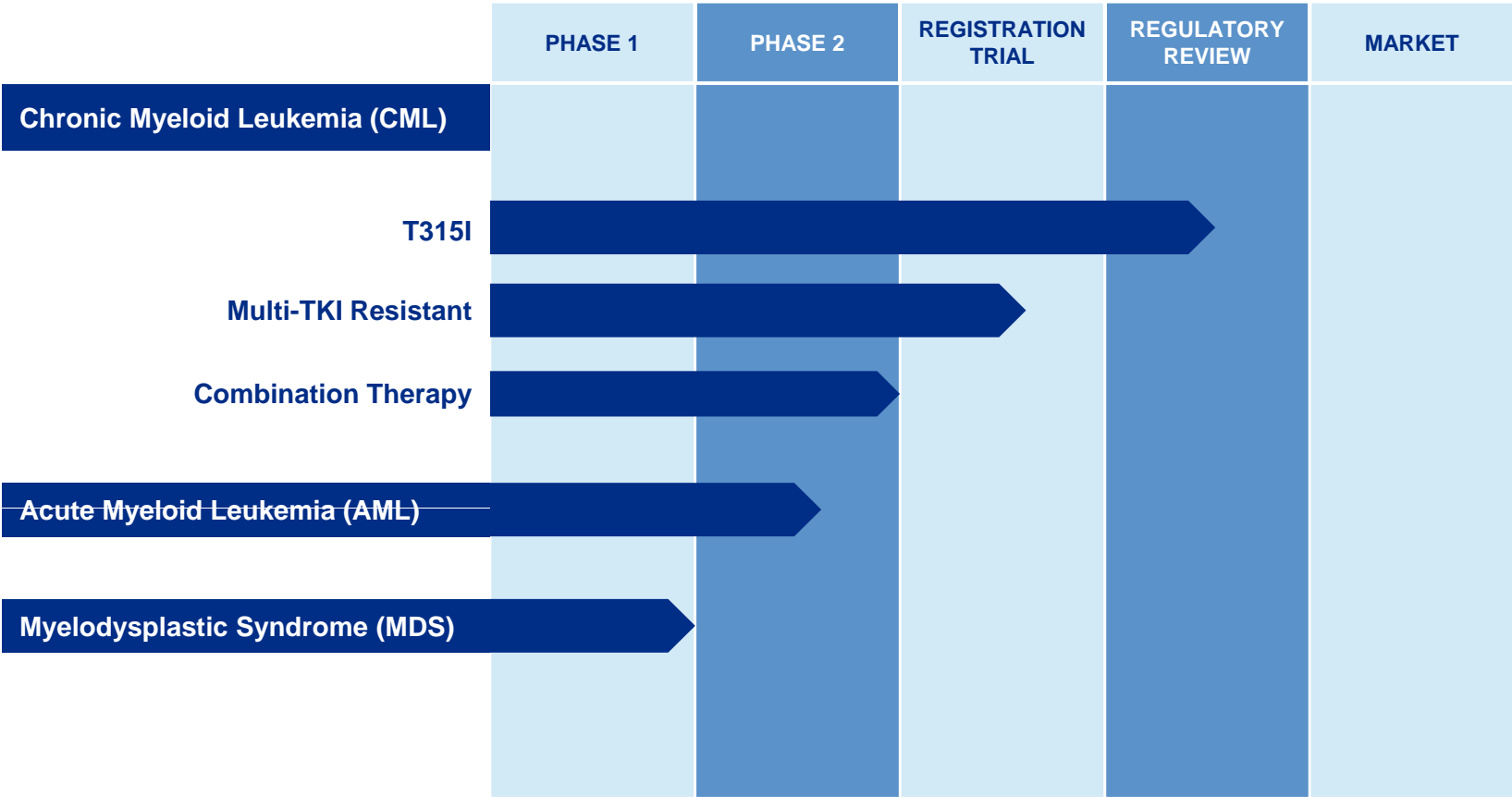
- Strategic goal
  - Retain product rights in North America
  - Out-license in Europe to fund U.S. product launch
  - Gain U.S. approval for OMAPRO™ in H1 2010, followed by launch



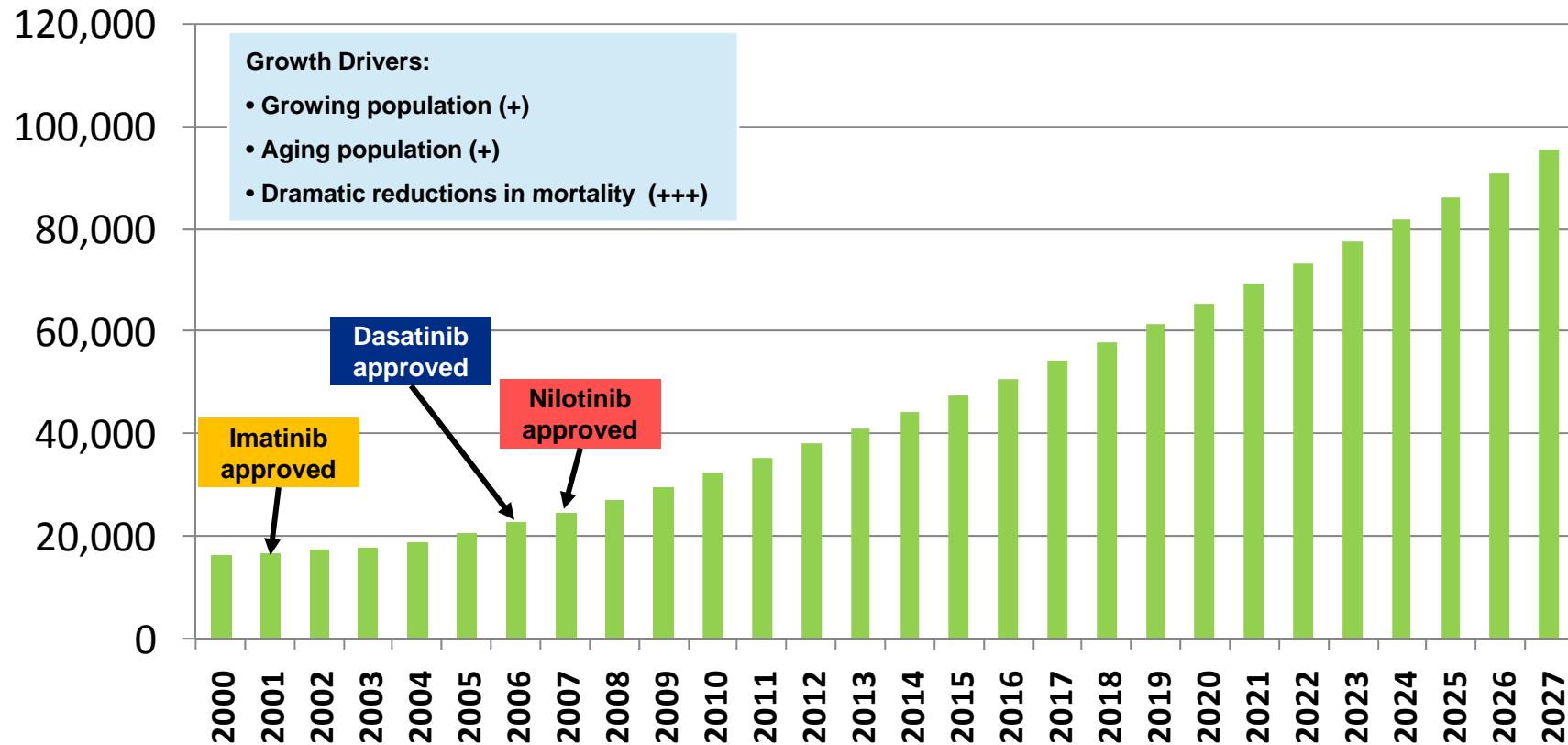
- Ideal European partner profile
  - European focused, commercial infrastructure
  - Hematology/oncology product and sales force presence



# OMAPRO™ Pipeline



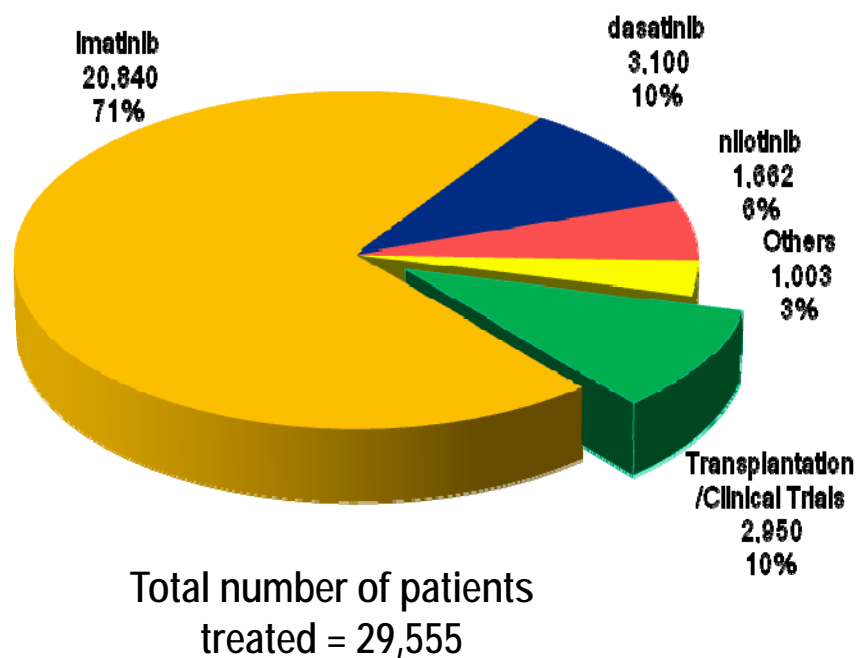
# Prevalence of CML in USA 2000 - 2027



- ChemGenex estimates of growth rates in CML prevalence are more conservative than others c.f. 200,000 - 300,000 patients surviving in the USA by 2027 (Jabbour, E, Cortes, J, Kantarjian, H. Optimal First-Line Treatment of Chronic Myeloid Leukemia, Oncology. Vol. 21, No. 6, May 1, 2007)
- 2006 Prevalence: Leukemia & Lymphoma Society "Facts 2009-2010" P.7 Table 2 (referenced to SEER) 2000-2005, 2007-2027 – ChemGenex Estimates
- Incidence and Deaths due to CML: Novartis publication (referenced to SEER)
- ASCO 2008 abstract 7088 "Kinetic of chronic myeloid leukemia (CML) prevalence in Northern France since the introduction of imatinib".

# The USA CML Market

## Overall Patient Share\*



## Pricing of approved drugs\*\*

Drug	Daily Dose	Annual – AWP*** (US\$)
Imatinib	400mg	52,800
	600mg	82,118
	800mg	105,601
Dasatinib	100mg	87,936
	140mg	87,936
Nilotinib	400mg	49,304
	600mg	73,956

\*Source: IntrinsicQ (April 2009)

\*\*Source: Red Book Q2 2009

\*\*\*AWP = Average Warehouse Price



# Paradigm Shift in the Management of CML



- Chronic Myeloid Leukemia (CML)
  - Chronic malignancy of the bone marrow
  - 5,000 new cases per annum in the US
  - Worldwide prevalence >200,000 patients and growing
- Gleevec<sup>®</sup> (imatinib) approved in 2001
  - Targeted tyrosine kinase inhibitor (TKI)
  - Global sales of US\$3.7 billion in 2008 (60-70% in CML)
- Two recently approved second generation TKIs
  - dasatinib approved in June 2006
  - nilotinib approved in October 2007
- Failure to TKIs in CML is an emerging problem

# OMAPRO™: Novel Mechanism of Action

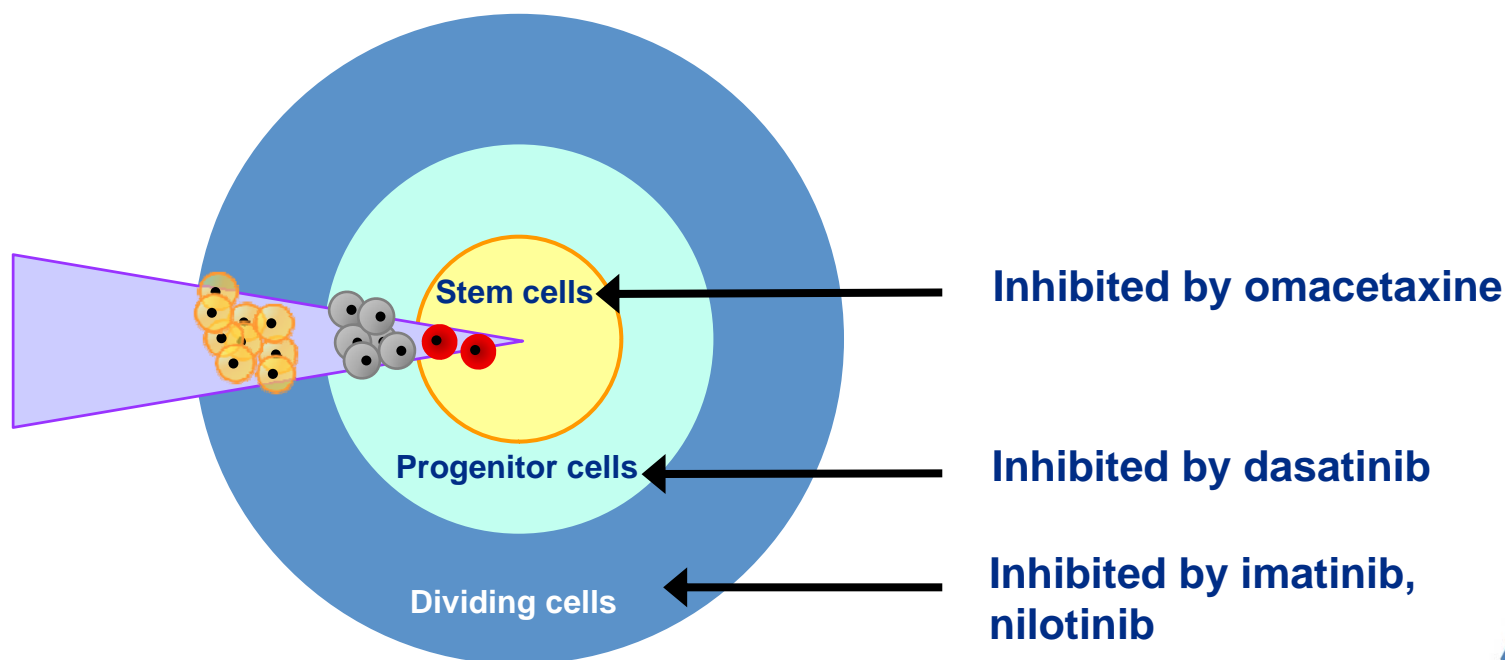
- Omacetaxine is a first in class cetaxine that inhibits protein translation resulting in selective inhibition of short-lived oncoproteins that are up-regulated in leukemic cells<sup>1,2</sup>
  - Cyclin-D1 - proliferation
  - Mcl-1 - apoptosis
  - c-Myc – differentiation

<sup>1</sup> Gurel, G. *et al.* J. Mol Biol. (2009), doi:10.1016/j.jmb.2009.04.005

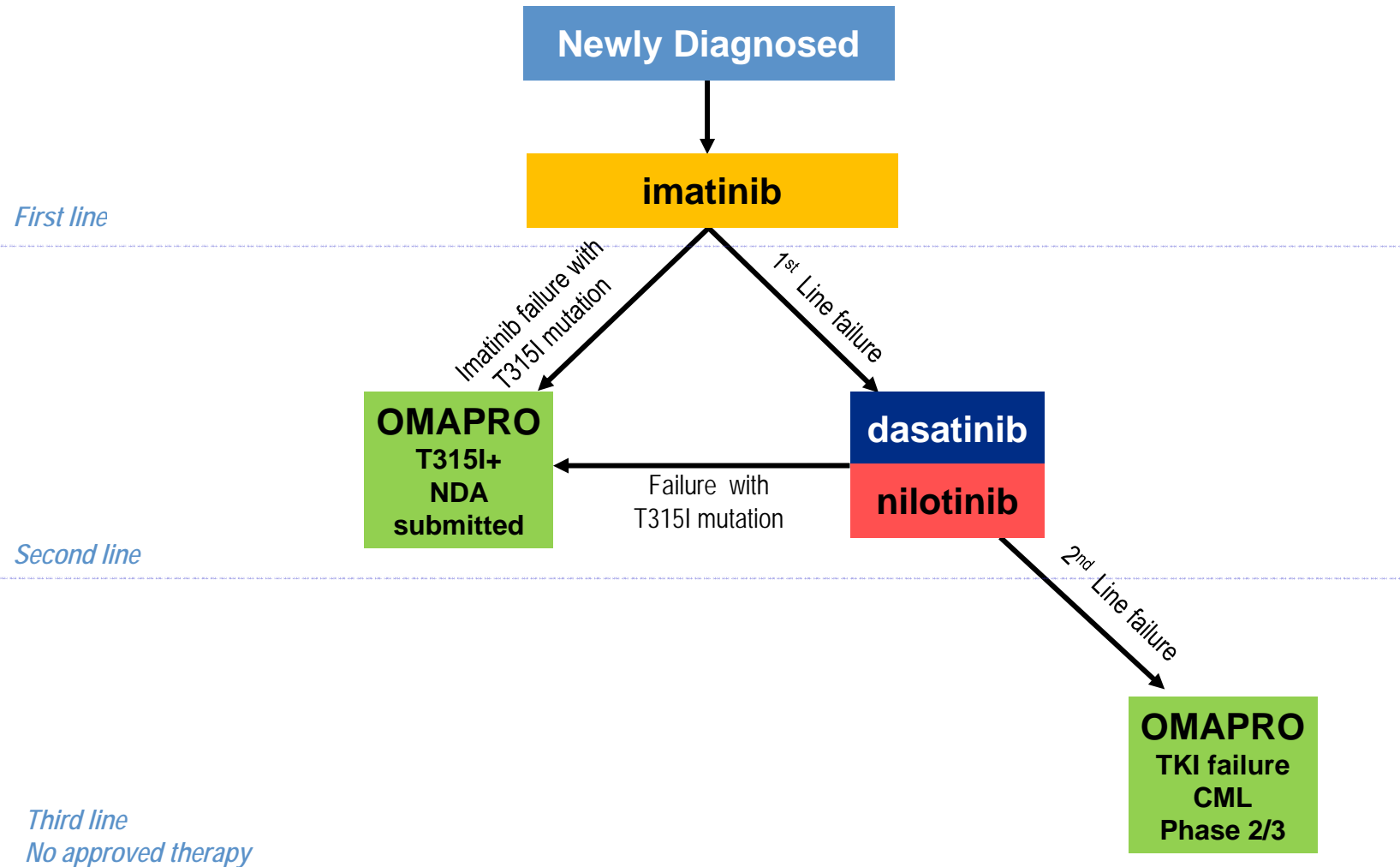
<sup>2</sup> Robert F *et al.* (2009), PLoS ONE 4(5): e5428 doi:10.1371/journal.pone.0005428

# OMAPRO™: Novel Mechanism of Action

- Omacetaxine has been demonstrated, *in vitro*, to be effective at killing human CML stem cells as well as peripheral leukemic cells<sup>1</sup>



# OMAPRO™ Addresses Unmet Medical Needs in CML





OMAPRO™

for the treatment of CML

# OMAPRO™: A Potential New Treatment for CML patients

- OMAPRO™
  - Omacetaxine for subcutaneous injection
- Presentation
  - lyophilized powder for reconstitution
  - convenient and safe administration
- Dosage – Twice a day
  - Induction – up to 14 days per month
  - Maintenance – up to 7 days per month
- Safety and tolerability
  - Myelosuppression is the most common side effect and is generally manageable

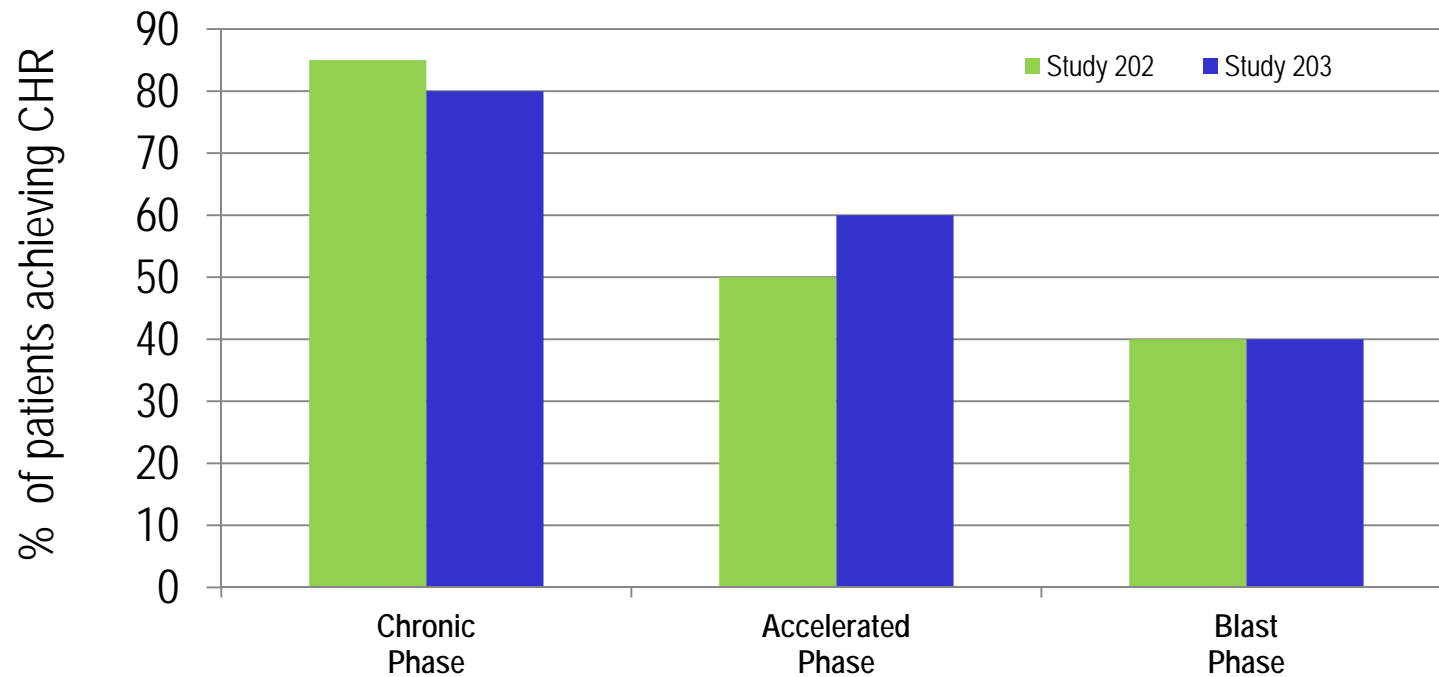


# OMAPRO™: Phase 2/3 Clinical Trials

	STUDY 202 T315I TKI failure CML	STUDY 203 Multiple TKI failure CML
Design	Open label, single arm	
Patients	Up to 100 patients	Up to 100 patients
Sites	35 in US, EU and 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
	Patients categorized according to chronic, accelerated and blast-phase CML	
Dose	<ul style="list-style-type: none"> <li>Induction phase: 1.25 mg/m<sup>2</sup> by subcutaneous injection two times a day for 14 days, every 28 days; up to 6 cycles</li> <li>Maintenance phase: As per induction phase, but 7 days treatment every 28 days</li> </ul>	
Primary endpoints	Hematologic (CP, AP, BP) and cytogenetic response (CP)	
Status	NDA submitted September 2009 Initial indication	Ongoing Phase 2/3 Possible label expansion

# OMAPRO™: Patient Outcomes in Key Trials

## Hematologic Responses in TKI failure Patients



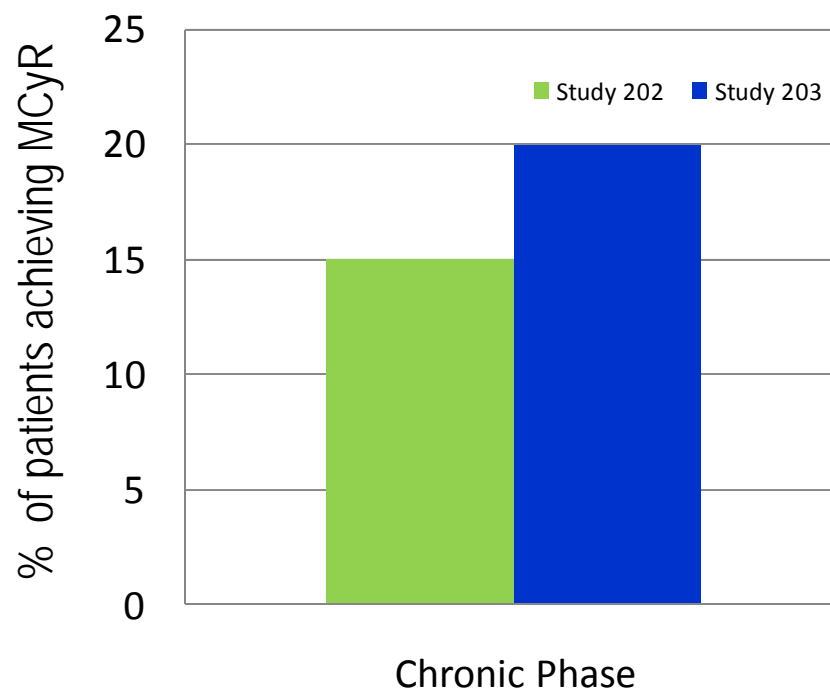
## Median response duration (months)

Study 202	8.9 (1.7 – 23.9)	4.1 (0.9 – 15.0)	3.3 (2.2 – 4.4)
Study 203	7.5 (1.9 – 22.2)	8.9 (1.8 – 23.2)	5.7 (2.4 – 19.3)



# OMAPRO™: Patient Outcomes in Key Trials

## Cytogenetic Responses in TKI failure Patients



## Median response duration (months)

Study 202	6.1 (0.7 – 16.2)
Study 203	2.7 (0-10.8)

# OMAPRO™: A Potential New Treatment for CML



- OMAPRO™ is a first in class cetaxine
- Mechanism of OMAPRO™ is different than TKIs
- Growing market with unmet medical needs
- Subcutaneously administered and myelosuppression is the most common side effect and generally manageable
- Clinical data in TKI resistant CML patients
- First potential indication in T315I mutation CML patients and the NDA has been submitted

# OMAPRO™: Development Timeline - USA

	2008	2009	2010
<b>CML: T315I</b>		Pivotal Phase 2/3	FDA Review Commercial launch
<b>CML: Multi-TKI Resistant</b>		Pivotal Phase 2/3	FDA Review
<b>CML: Combination Therapy</b>		Prior published Phase 2 studies	Phase 2
<b>AML</b>		Phase 2a trial	Phase 2
<b>MDS: 2<sup>nd</sup> Line</b>		Prior published Phase 2 studies	Phase 2

# Manufacturing and Supply Chain



- Semi-synthetic API
- Stable lyophilized powder
- GMP validated manufacturing
- Commercial manufacturing partnership with attractive Cost of Goods Sold
- Specialty pharmacy distribution



# OMAPRO™: Commercial Strategies

- Initial sales and marketing promotional efforts directed at U.S. Hematology Centers of Excellence
- Key customer targets include:
  - Key opinion leaders in hematology/oncology
  - Regional thought leaders
  - Patient advocacy and social media outlets
  - Payors
- Targeted specialty pharmacy distribution approach

# Strong Board and Senior Management Team

## Management

Greg Collier, PhD\*

Adam Craig, MD, PhD, MBA

James Campbell, PhD, MBA

Tom DeZao, BA

Luana Staiger, BS

Don Joseph, JD

Chief Executive Officer and Managing Director

Senior Vice President and Chief Medical Officer

Chief Financial Officer and Chief Operating Officer

Senior Vice President and Chief Commercial Officer

Vice President of Regulatory Affairs

Head of Corporate Development

## Board of Directors

Brett Heading, LLB (Chairman)

Dan Janney, BA, MBA

Geoff Brooke, MBBS, MBA

Elmar Schnee, BCom Mkting

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\$ 192 million
Cash held**	A\$ 17.6 million
Enterprise value	A\$174 million
Significant Shareholders	Alta Partners (15%), Stragen Pharma (13%), Orbis Investments (10%), Merck Serono (9%), GBS (8%)

\*Effective 7 September 2009

\*\*As of 30 June 2009

USD/AUD approximately 0.84

# Key Upcoming Events

Event	Timing
✓ Complete submission for drug approval in USA	Q3 2009
Establish corporate partnership for EU	H2 2009
Complete submission for drug approval in Europe	Q4 2009
Targeted approval of omacetaxine in USA	H1 2010
Targeted approval of omacetaxine in Europe	Q3 2010



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# Contacts

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