



## Company presentation

Claudio Bordignon,  
Chairman and CEO

BIT Small Cap Conference  
Milan, 21 November 2011

# Forward-looking statements

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The presentation contains certain forward-looking statements. Although the Company believes its expectations are based on reasonable assumptions, these forward-looking statements are subject to numerous risks and uncertainties, including scientific, business, economic and financial factors, which could cause actual results to differ materially from those anticipated in the forward-looking statements.

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## **Declaration by the official Corporate Financial Reporting Manager:**

The undersigned herewith attests, pursuant to Article 154-bis, paragraph 2 of the Italian Consolidated Law on Finance (Legislative Decree 58/1998), that the accounting disclosure contained in this presentation matches documentary evidence, corporate books, and accounting records.

*Enrico Cappelli, Chief Financial Officer, official Corporate Financial Reporting Manager*

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## MolMed at a glance

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- › Focus on oncology
- › Two highly innovative anticancer therapeutics in Phase III
  - TK: cell therapy product
  - NGR-hTNF: selective vascular targeting agent for solid tumours
- › Two technology platforms for a diversified business model
- › Cell & gene therapy: activities for third parties
- › Solid investor base - listed on the Milan Stock Exchange (MLM)

## MolMed top management

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Claudio Bordignon  
*Chairman and CEO*



Marina Del Bue  
*General Manager, Business Development & Administration*



Germano Carganico  
*Direttore Generale, R&D and Operations*

90 years overall experience in the biotech sector

## Significant progress achieved in 2011

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- › NGR-hTNF
  - promising data of Phase II trials
  - progress of randomised Phase II trials and of international pivotal Phase III trial
  
- › TK
  - pursued international pivotal Phase III trial
  
- › Gene therapy
  - new agreements for development and production of new gene therapy treatments

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# Clinical development pipeline



Product	Indication	Trial code	Phase I	Phase II	Phase III
TK	High-risk leukaemia	TK007, TK008 <i>random.</i>	[Ongoing Phase I, Phase II, and Phase III]		
	<i>Leukaemia (Japan/Takara Bio)</i>		[Ongoing Phase I]		
NGR-hTNF	Solid tumours → MTD	EORTC 16041	[Completed Phase I]		
	Solid tumours - low dose	NGR002	[Completed Phase I]		
	Solid tumours - high dose	NGR013	[Ongoing Phase I]		
Monotherapy	Colorectal cancer	NGR006		[Completed Phase II]	
	Liver cancer	NGR008		[Completed Phase II]	
	Mesothelioma	NGR010, NGR015 <i>random.</i>		[Completed Phase II, Ongoing Phase III]	
	Mesothelioma/maintenance	NGR019 <i>random.</i>		[Ongoing Phase II]	
+ doxorubicin	Solid tumours	NGR003	[Completed Phase I]		
	Lung cancer/SCLC	NGR007		[Completed Phase II]	
	Ovarian cancer	NGR012		[Completed Phase II]	
	Ovarian cancer	NGR018 <i>random.</i>		[Ongoing Phase II]	
	Soft tissue sarcomas	NGR016 <i>random.</i>		[Ongoing Phase II]	
+ Xelox	Colorectal cancer	NGR005		[Completed Phase II]	
+ cisplatin	Solid tumours	NGR004	[Completed Phase I]		
	Lung cancer/NSCLC	NGR014 <i>random.</i>		[Ongoing Phase II]	

ongoing      completed

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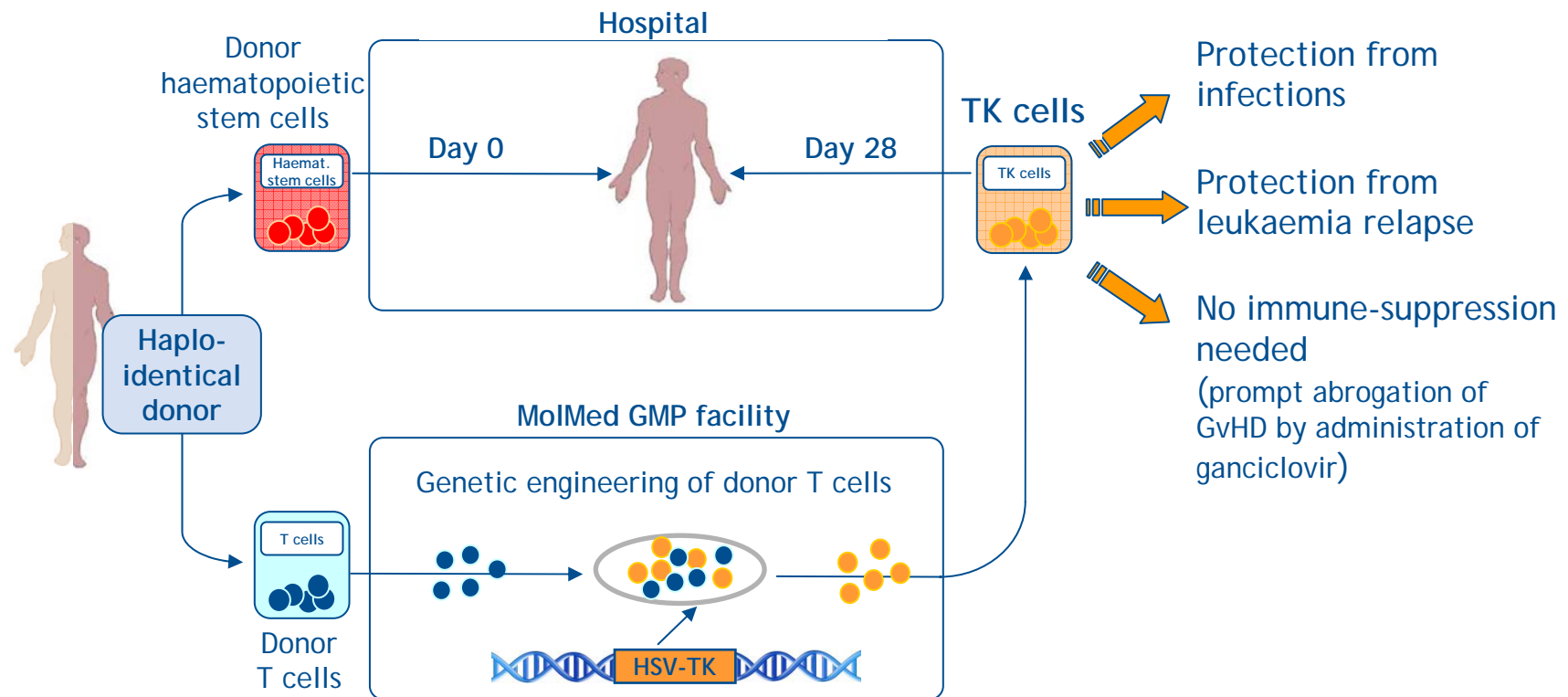
## TK: a new technology for haematopoietic stem cell transplant

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- › Indication: haematopoietic stem cell transplants (HSCT) for high-risk leukaemias
- › Unmet need: ~50% of patients candidate to HSCT miss a fully matched donor
- › TK technology enables HSCT derived from the bone marrow of a partially matched donor without need of post-transplant immune-suppression

Technological innovation within bone marrow transplant, oldest & well consolidated cell therapy (>50 years of medical practice)



Sources: adapted from Bonini et al., Science 1997; Bonini et al., Nat. Med. 2003; Recchia et al., PNAS 2006; Ciceri et al, Blood 2007; Ciceri, Bonini et al., The Lancet Oncology 2009

## Key results of completed Phase II trial TK007

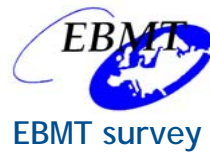
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### TK superiority vs historical data

- › 75% (22/28) of patients receiving TK achieved immune-reconstitution
  - no immune-suppression
  - longer disease-free survival
  - reduced leukaemia relapse
  - reduced transplant-related mortality

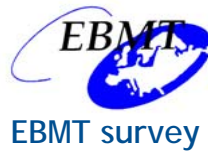
# Partially compatible transplant (haplo-HSCT): TK increases disease-free survival



Haplo-HSCT data (EBMT survey, 266 patients) <sup>1</sup>	
Median age	35 years
Transplant-related mortality (at 50 months from HSCT)	50%
Leukaemia relapse	20-30%
4-year disease-free survival	20-30%
GvHD - occurrence - control	n.d.

Sources: <sup>1</sup>Ciceri et al. *Blood*. 2008. <sup>2</sup>Ciceri. Bonini et al.. *Lancet Oncol*. 2009. ASCO 2011. abstract 6526

# Partially compatible transplant (haplo-HSCT): TK increases disease-free survival



	Haplo-HSCT data (EBMT survey, 266 patients) <sup>1</sup>	Phase II TK: immune-reconstituted patients (22 patients) <sup>2</sup>
Median age	35 years	56 years
Transplant-related mortality (at 50 months from HSCT)	50%	14%
Leukaemia relapse	20-30%	10%
4-year disease-free survival	20-30%	45%
GvHD - occurrence	n.d.	50%
- control		100%

Sources: <sup>1</sup>Ciceri et al. *Blood*. 2008. <sup>2</sup>Ciceri. Bonini et al.. *Lancet Oncol*. 2009. ASCO 2011. abstract 6526

## Ongoing pivotal Phase III trial (TK008)

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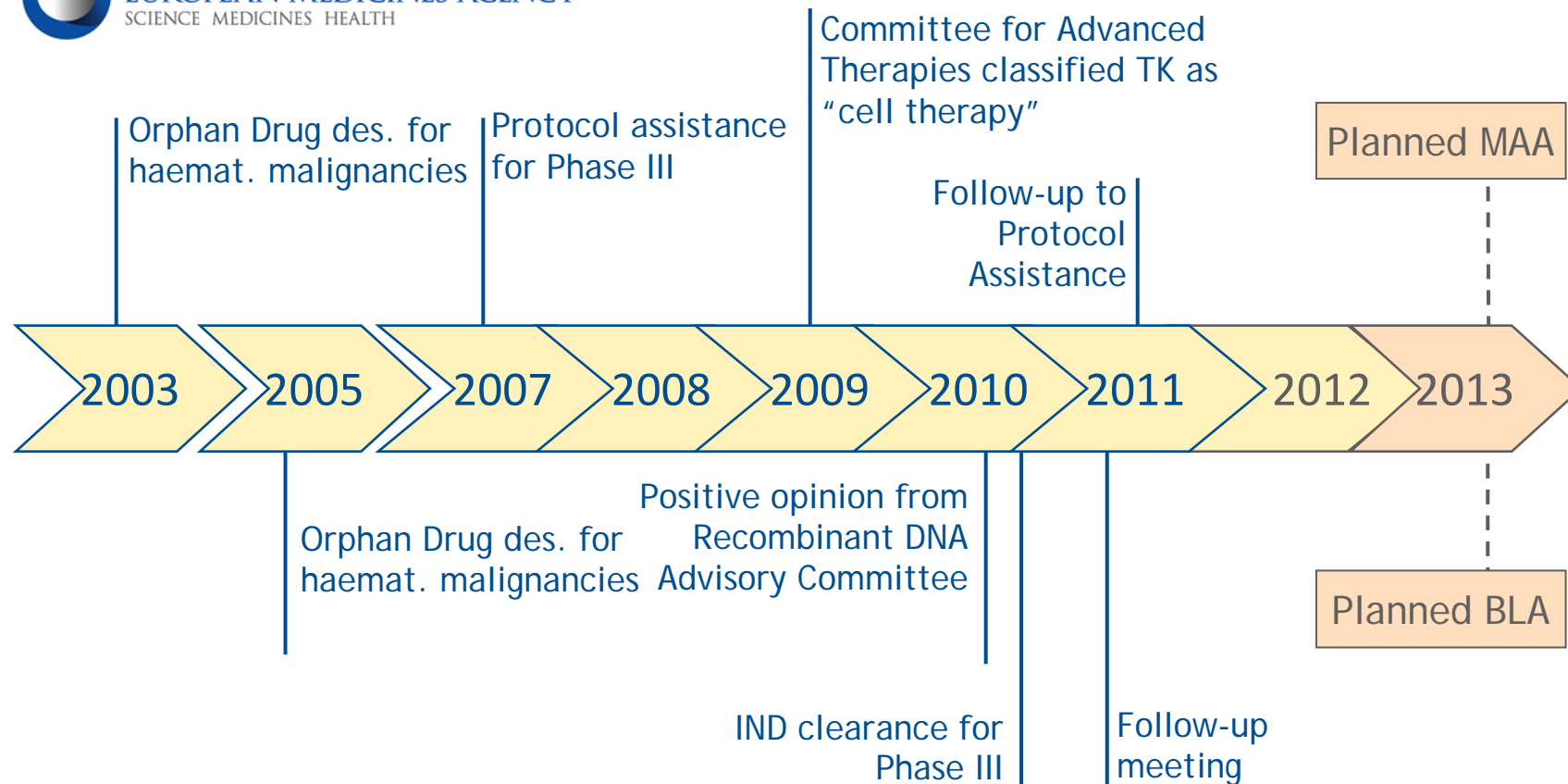


- › International multicentre controlled trial
- › One of the first and most extensive Phase III trials in the area of bone marrow transplant
- › Enrolment: 170 patients – randomisation 3:1 in favour of TK
- › Primary endpoint: disease-free survival
- › Results expected in 2013

# Continuous interaction with regulatory authorities



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH



## Potential market

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- › Target market: MolMed estimates a potential market for TK in Europe, US and Japan of ~12.500 patients/year
- › Key patent:
  - granted in Europe(29 National patents in EPC countries), USA and Japan
  - Market exclusivity until 2025 + 5 years of extension
- › Orphan Drug designation in EU andUS:
  - Upon market launch, exclusivity for 10 years in EU and 7 years in US
- › *Partnership*: development and marketing in Asia by Takara Bio Inc.

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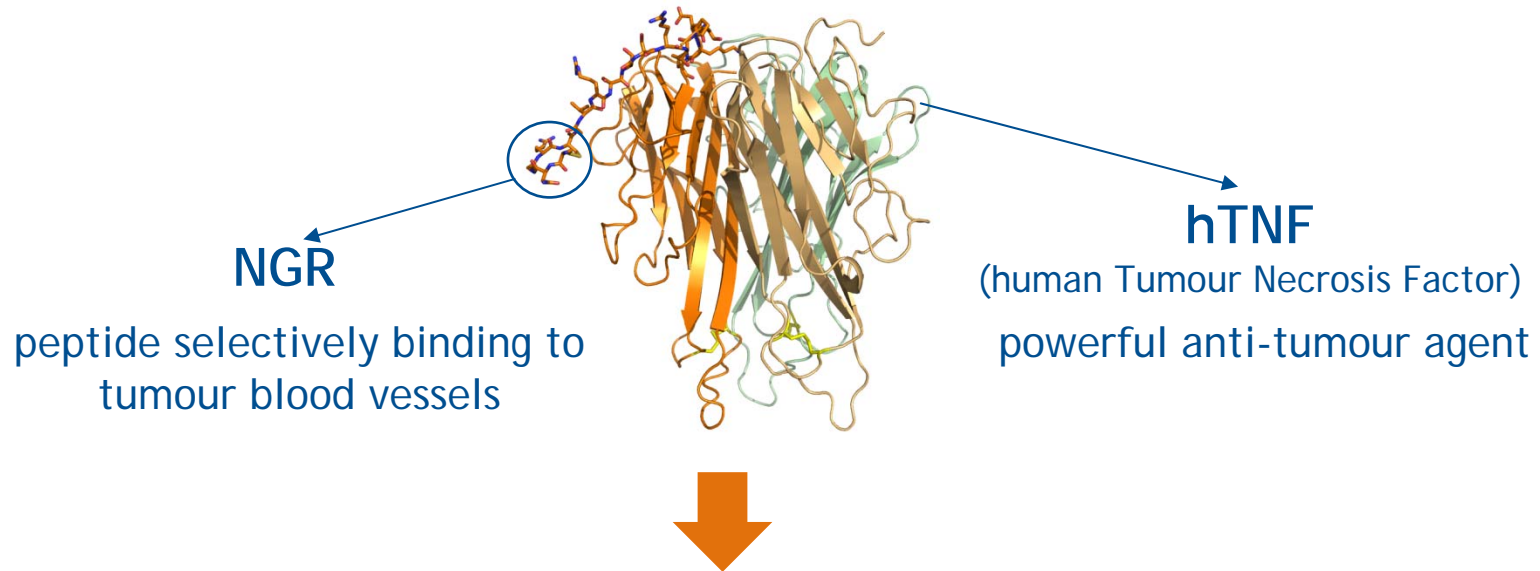
# NGR-hTNF: a selective vascular targeting agent

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- › Indications in clinical development: 7 different solid tumours
  - Mesothelioma, ovarian cancer, lung cancers (SCLC and NSCLC), colorectal cancer, liver cancer, soft tissue sarcomas
- › Unmet need: low-toxic drugs allowing long-time treatment
- › Anti-tumour activity through inhibition of new tumour blood vessels

Fusion protein consisting of 2 moieties



**Molecule with unique biological properties**

- › Direct anti-tumour activity at very low doses with no toxic side effects
- › Inducing enhanced permeability of tumour blood vessels → boosting activity of chemotherapy administered in combination

## Clinical activity observed in six different tumours

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- › Results obtained in ongoing or completed Phase II trials in 6 indications:
  - Mesothelioma, ovarian cancer, lung cancers (SCLC and NSCLC), liver cancer, colorectal cancer
  
- › Antitumour activity observed:
  - Tumour shrinkage
  - Improved survival
  
- › Favourable toxicity profile , better than competitors (>500 pts treated)

## Phase II results in liver cancer: Complete response

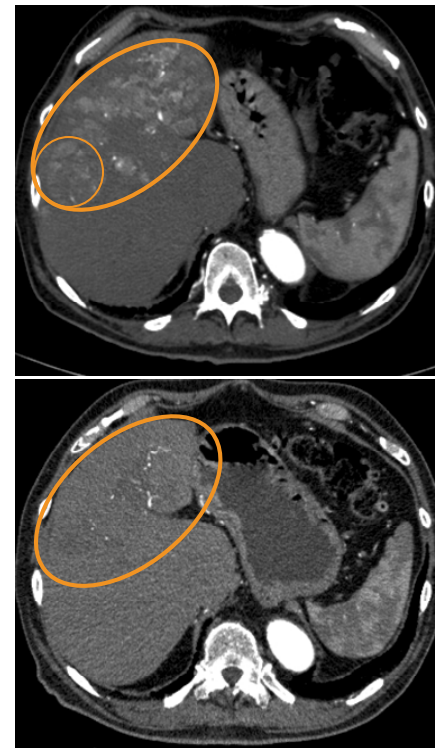


Among products available or in development,  
only NGR-hTNF achieved a complete response

Antitumour activity (40 pre-treated patients):

- › One complete response achieved, ongoing since May 2008 (patient was treated with NGR-hTNF as maintenance therapy for 24 months)
- › One important partial response observed
- › No approved drugs and no widely-accepted chemotherapies available in second line
- › Only one candidate product in Phase III trial in second line (brivanib)

CT-scan of complete response  
ongoing 3+ years



February  
2008:  
baseline

May 2008:  
after 4  
cycles of  
NGR-hTNF

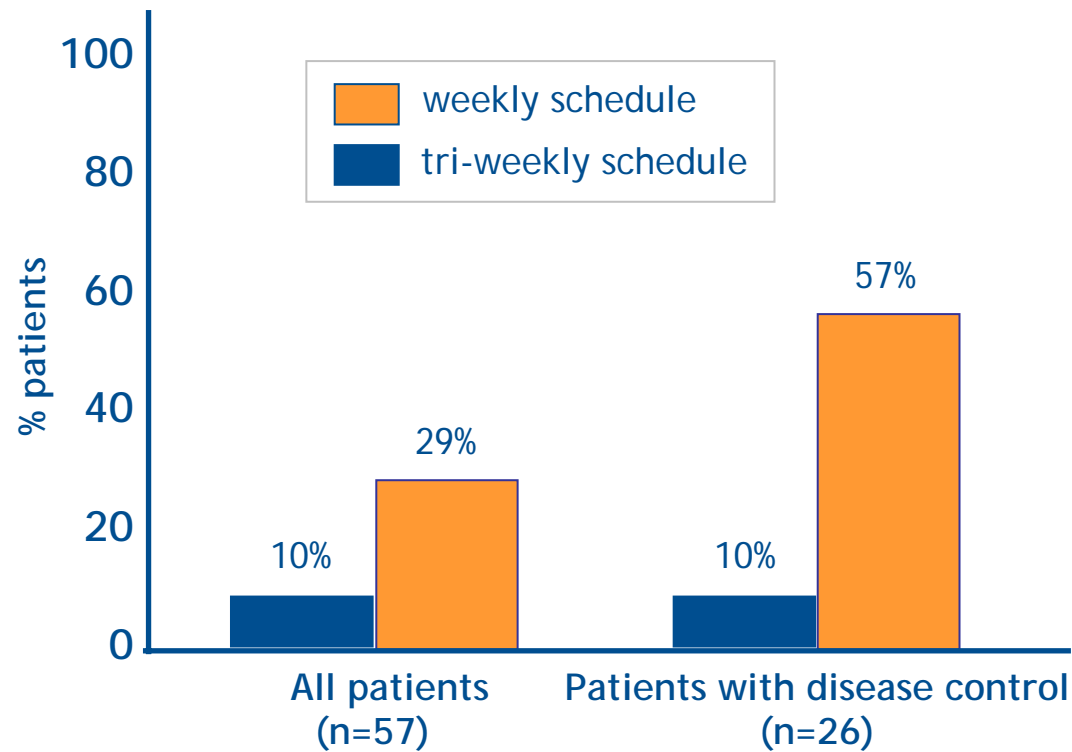
*Source: Santoro et al, Br J Cancer (2010) 103, 837-844*

# Phase II results in mesothelioma: long-term efficacy



Weekly schedule induces prolonged clinical benefit  
by avoid long treatment gaps

2-year overall survival rate by treatment frequency



Sources: Gregorc et al. JCO 2010; ASCO 2011. Abstract 7089

## Pivotal Phase III trial in mesothelioma as 2<sup>nd</sup> line therapy (NGR015)

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- › Double-blind, placebo-controlled trial ongoing in Europe and US
- › Enrolment: 390 patients (101 enrolled) - randomisation 1:1
- › Primary endpoint: overall survival
- › Results expected in 2013
- › No treatments available or in Phase III trials in 2<sup>nd</sup> line

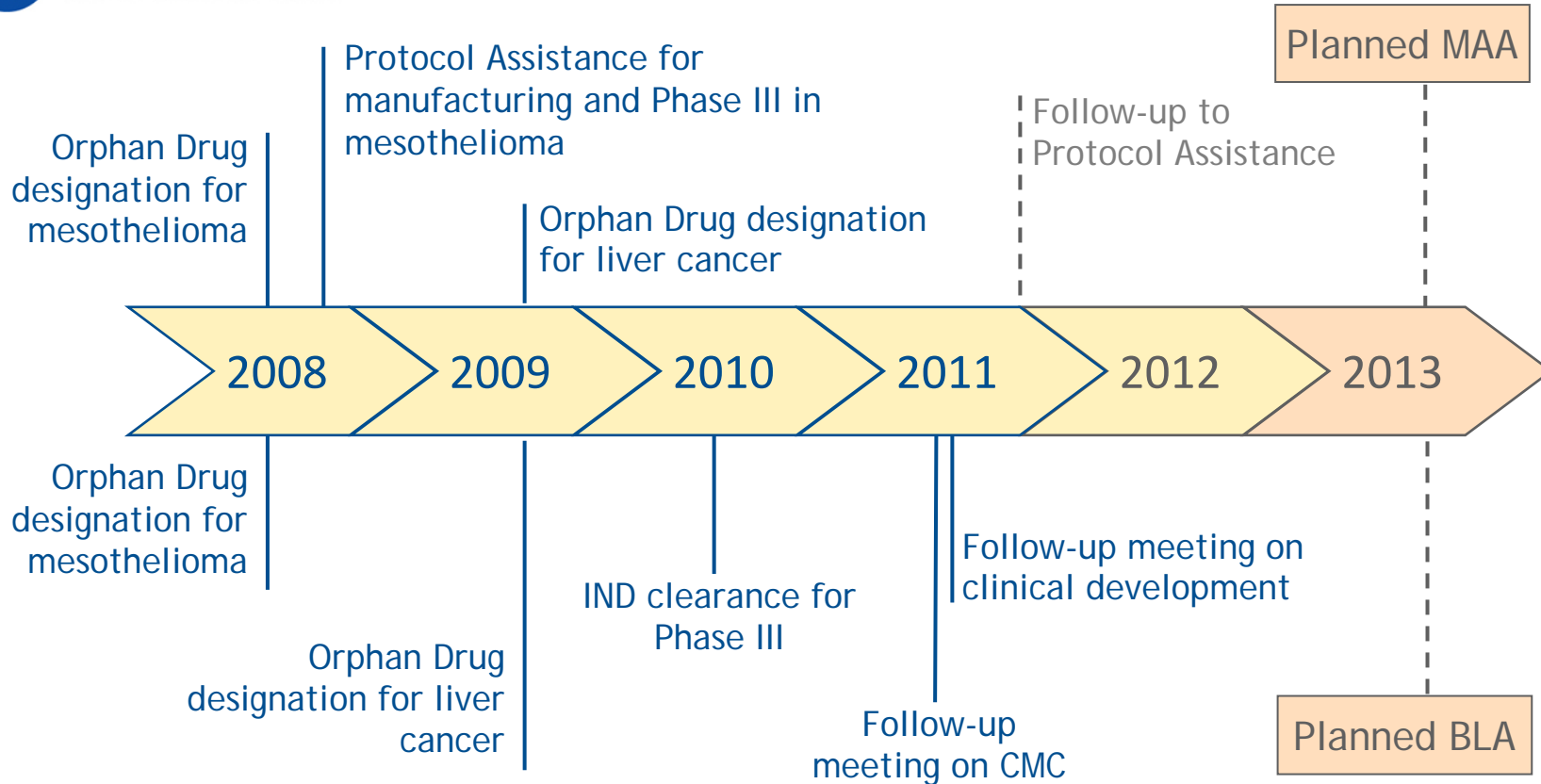
## Ongoing randomised Phase II trials

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- › 4 trials in 4 indications:
  - non-small cell lung cancer - 1<sup>st</sup> line
  - soft tissue sarcomas - 1<sup>st</sup> and 2<sup>nd</sup> line
  - mesothelioma - 1<sup>st</sup> line as maintenance therapy
  - Ovarian cancer- 2<sup>nd</sup> line
  
- › Primary endpoint: progression-free survival
  
- › Comparison *versus* standard chemotherapy treatment
  
- › Results expected in 2012

# Continuous interaction with regulatory authorities



## Potential market

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- › Broad therapeutic potential in different tumour types: 1.4 million\* patients/year (new cases) in the 7 indication investigated by MolMed
  
- › Key patent:
  - granted in Europe (29 National patents in EPC countries), US, Japan, Canada, China, India and Brasil
  - market exclusivity until 2021 + 5 years of extension with Supplemental Certificate
  
- › Orphan Drug Designation in EU and US:
  - For mesothelioma and for liver cancer
  - Upon market launch, exclusivity for 10 years in EU and 7 years in US

\* In EU, US, Canada and Japan

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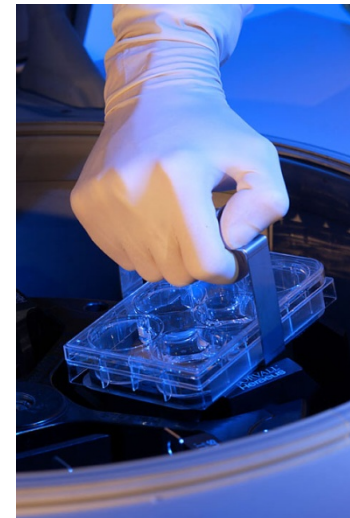
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# GMP solutions for cell & gene therapy



Development and GMP manufacturing  
for in-house product & for third parties

- › GMP facility authorised for production of clinical-grade cell & gene therapeutics
- › Manufacturing of TK for Phase III
- › Important agreements:
  - GSK: development of production process for gene therapy of ADA-SCID (€ 5.5 million over 2 years)
  - Telethon Foundation: development and GMP production of new investigational gene therapy treatments for 6 rare genetic diseases (€ 8.3 million over 4 years)



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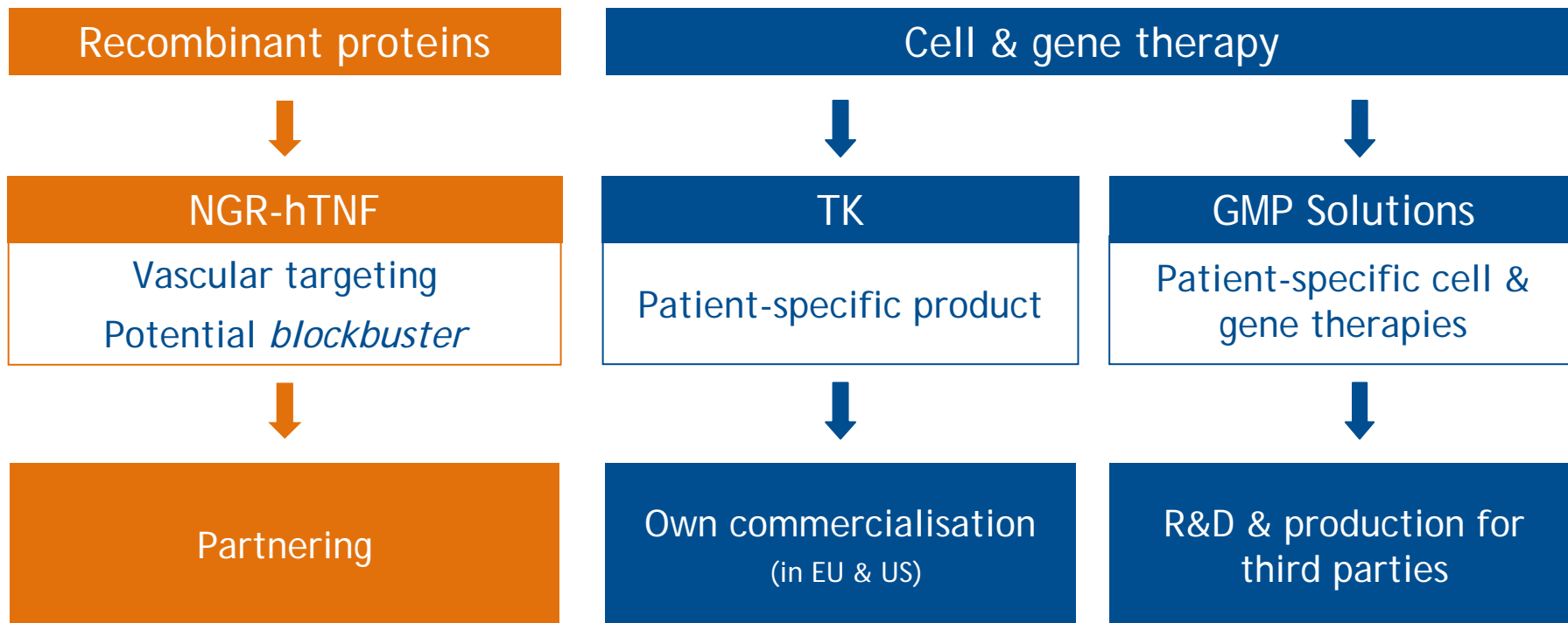


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# MolMed business model: innovation and risk mitigation



Two innovative technologies with different business strategies



## 1. Orphan indication for first Phase III and first filing for regulatory approval:

- greater potential for reimbursement
- reduction of development costs
- market exclusivity: 10 years in Europe, 7 years in US

## 2. Extension to non-orphan indications

## Cash to support clinical and industrial development of strategic projects



Strong positive financial position to support product development

### MolMed financials 3Q 2011 (€ thousand)

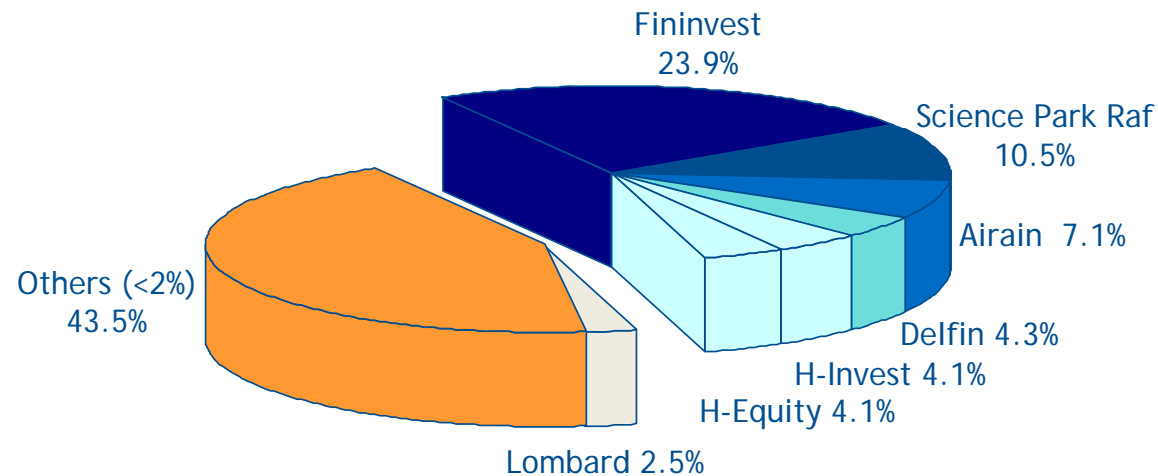
	FY 2010	First 9 months 2011	First 9 months 2010
Operating revenues	2,676	2,091	1,598
• Revenues	2,081	1,490	1,120
• Other income	595	598	475
Operating costs	(20,424)	(18,689)	(14,722)
<u>Result for the period</u>	<u>(17,582)</u>	<u>(15,679)</u>	<u>(12,997)</u>
<u>Net financial position</u>	<u>60,040</u>	<u>45,288</u>	<u>63,800</u>

# Shareholders' structure



High liquidity: average daily traded shares  
nearly 1% of total outstanding shares

- › Listed on the Milan Stock Exchange (Milan:MLM)
- › Financials:
  - Market cap (Nov 17, 2011): € 104 million
  - Issued shares: 210,541,926
  - Daily traded volume (average 3 months): 1.4 million shares



## Getting closer to the turning point



## Getting closer to the turning point



- 
- A photograph of a rowing team in a long, narrow boat on a body of water, viewed from behind. The water is rippled, and the rowers are in a synchronized rowing motion. The image has a warm, orange-brown color cast.
- › Two investigational therapeutics in Phase III
  - › Broad clinical development programme
  - › Filings for regulatory approvals in EU and US planned in 2013
  - › Continuous interaction with regulatory authorities
  - › Broad and long-lasting patent coverage



Thank you very much  
for your attention

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