MOLMED

Company presentation

Jefferies Global Healthcare Conference

New York, June 6, 2013

Claudio Bordignon

Chairman and CEO

Forward-looking statements

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

MolMed: at a glance

- Listed on the Milan Stock Exchange (MLM)
- Company Focus: oncology and genetic orphan diseases
- 112 employees, 2/3 staff scientists
- Net financial position: € 16.9 million (March 31, 2013)
- Company core competencies:

Recombinant proteins

✓ NGR-hTNF: tumor vascular targeting agent – pivotal Phase III results in 3Q 2013

Cell and gene therapies

✓ TK: cell therapy product – expected filing for conditional approval in EU in 2013

✓ CMO activities for third parties: growing revenues

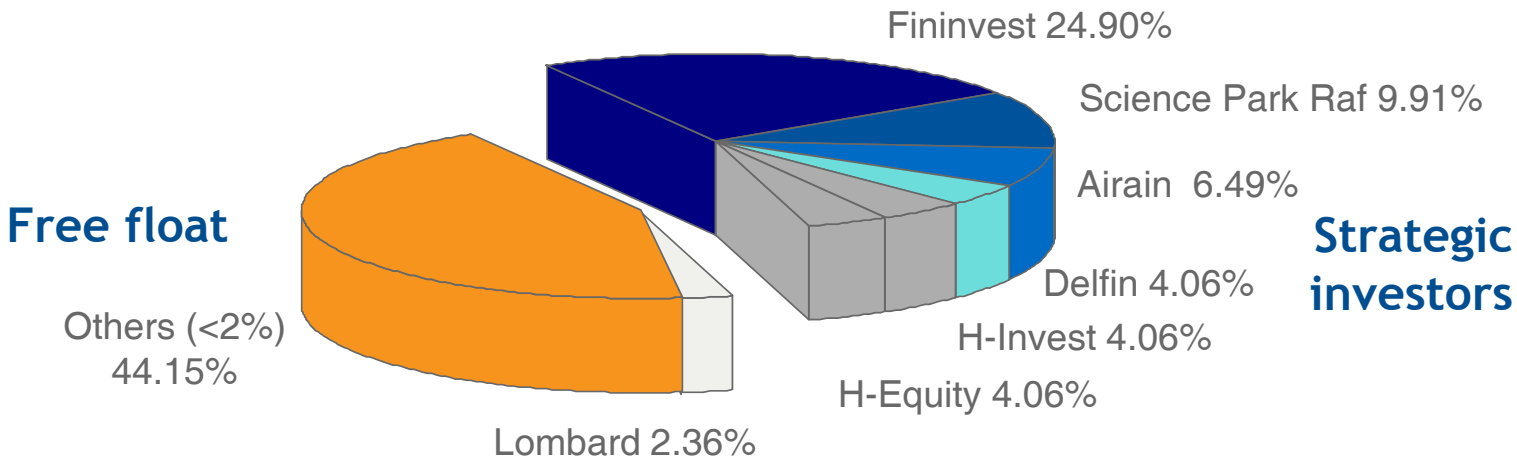
Key financials

(€ thousand)	2012	2011	2010	1Q 2013	1Q 2012
Operating revenues	5,059	3,418	2,676	1,281	1,183
▪ Revenues from activities for third parties	4,593	2,767	2,081	1,164	1,091
▪ Other income	466	651	595	117	92
Operating costs	(27,441)	(26,098)	(20,424)	(6,402)	(6,328)
Result for the period	(22,001)	(21,569)	(17,582)	(5,122)	(4,895)

(€ thousand)	Dec 31st 2012	Dec 31st 2011	Dec 31st 2010	Mar 31st 2013	Mar 31st 2012
Net financial position	17,526	38,667	60,040	16,910	33,045

Shareholders' structure

- Market cap (end of May 2013): ~ €130 million
- Daily traded volume (average 3 months): ~ 1,400,000 shares
- Shareholders as of 22/04/2013:



MolMed business model: innovation and risk mitigation

Two innovative platforms with different business strategies

Recombinant proteins	Cell and gene therapy

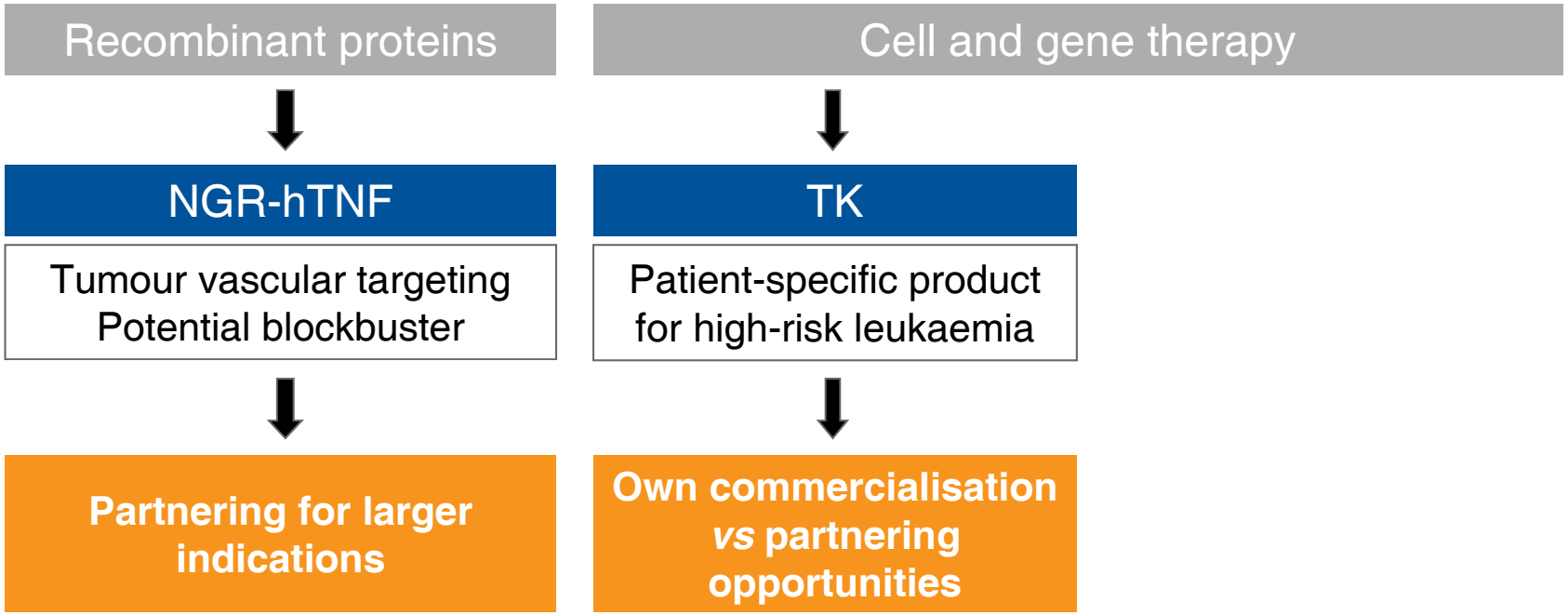
MolMed business model: innovation and risk mitigation

Two innovative platforms with different business strategies

Recombinant proteins	Cell and gene therapy
NGR-hTNF	
Tumour vascular targeting Potential blockbuster	
Partnering for larger indications	

MolMed business model: innovation and risk mitigation

Two innovative platforms with different business strategies



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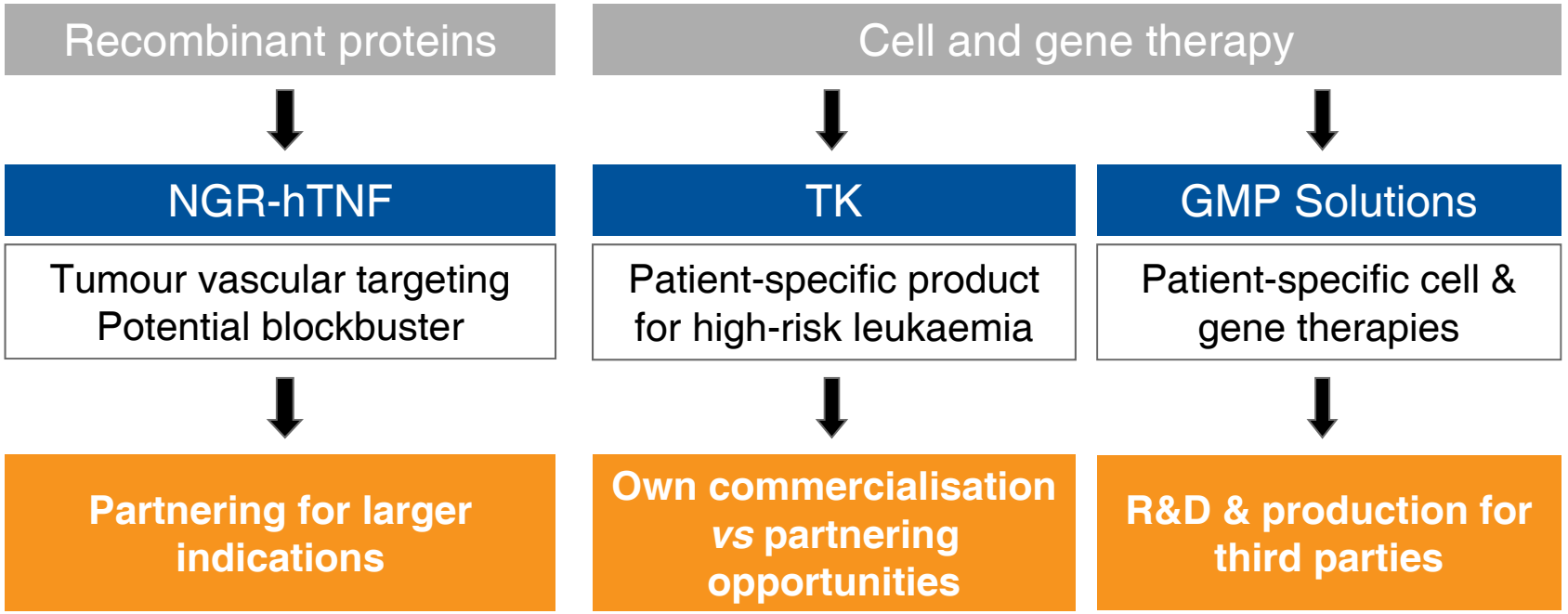
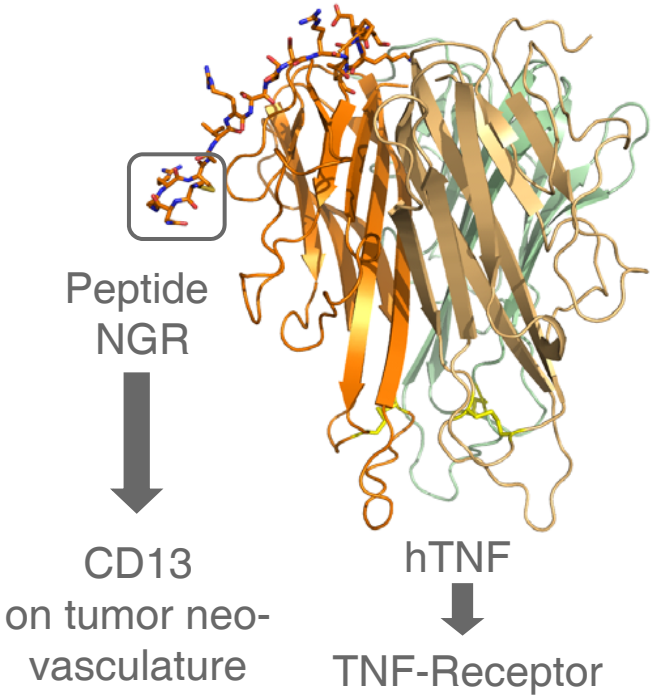
Two innovative platforms with different business strategies

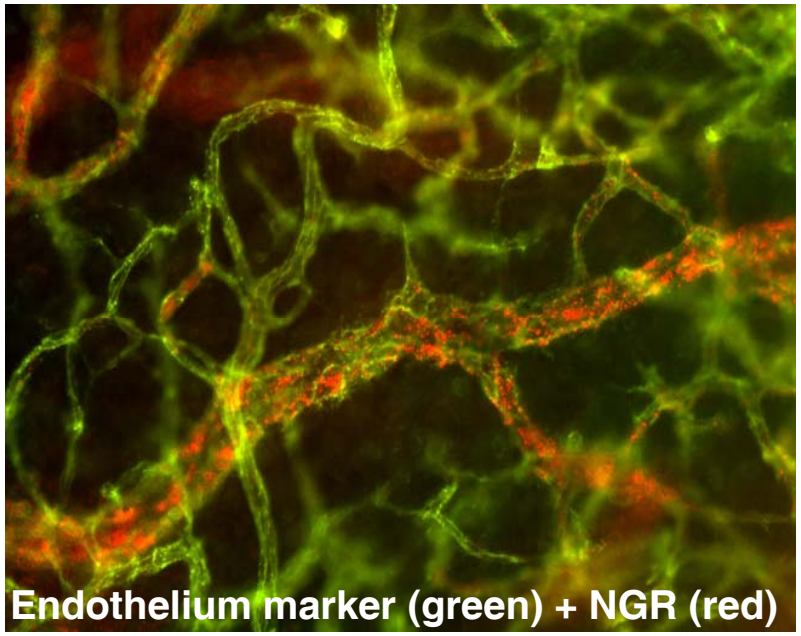


NGR-hTNF: a selective vascular targeting agent

Recombinant fusion protein



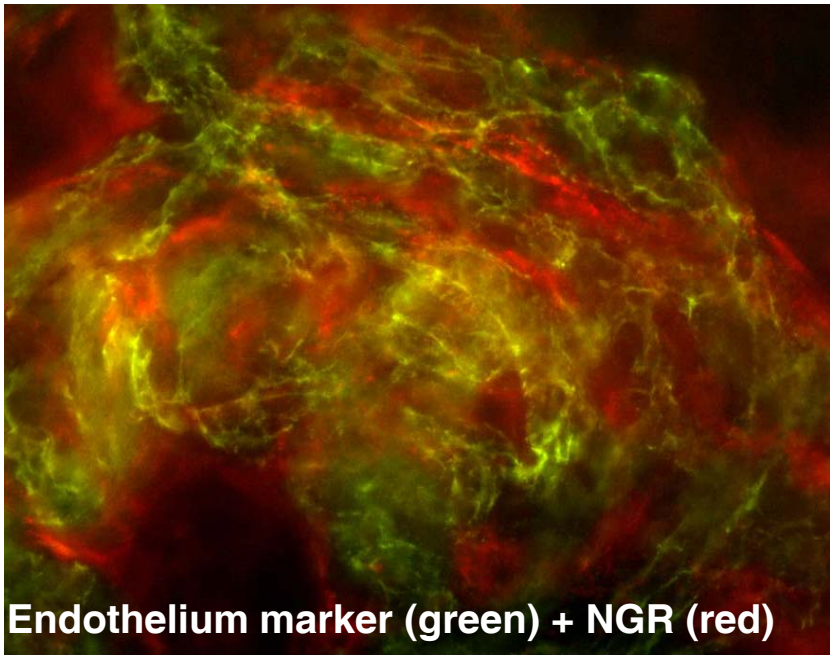
NGR binding
to tumor blood vessels



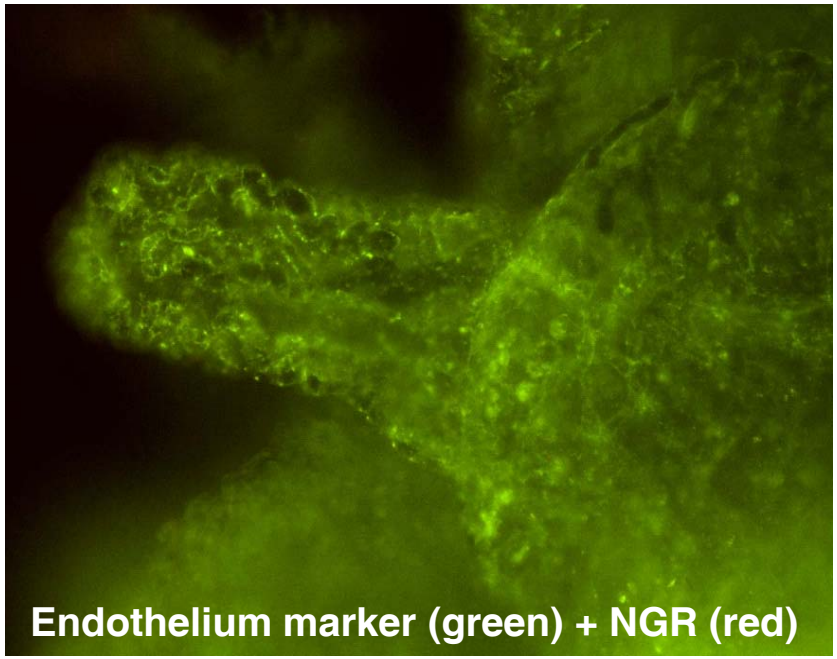
Doses of 0.8 µg/sqm systematically show antitumor activity

Selective binding to angiogenic tumour vessels

Human colon carcinoma



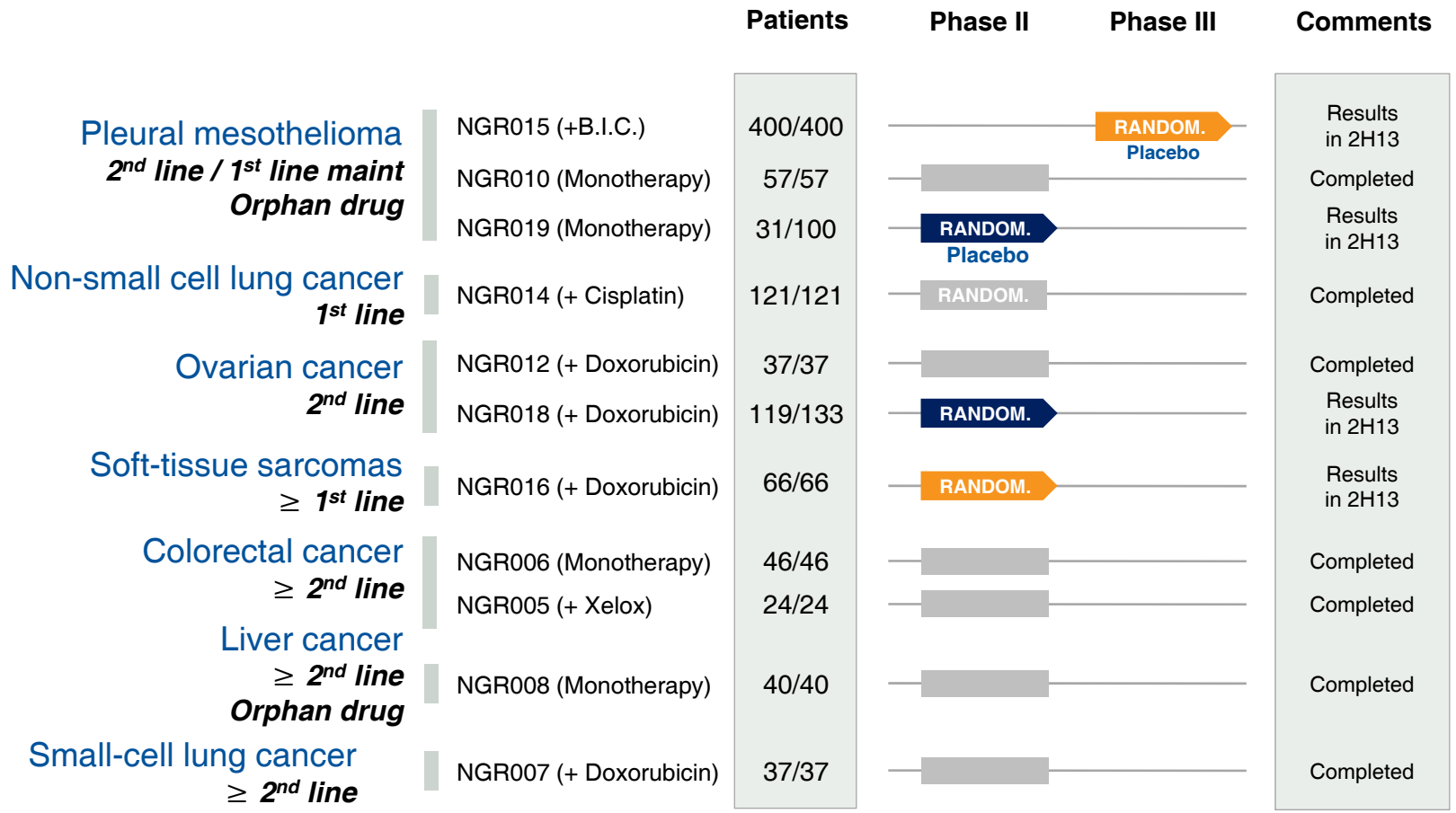
Normal human colon



Whole mount analysis of tissues obtained from the same patient (N=3)

NGR binds to tumour vessels of CRC and not to those of normal intestine

NGR-hTNF clinical development plan

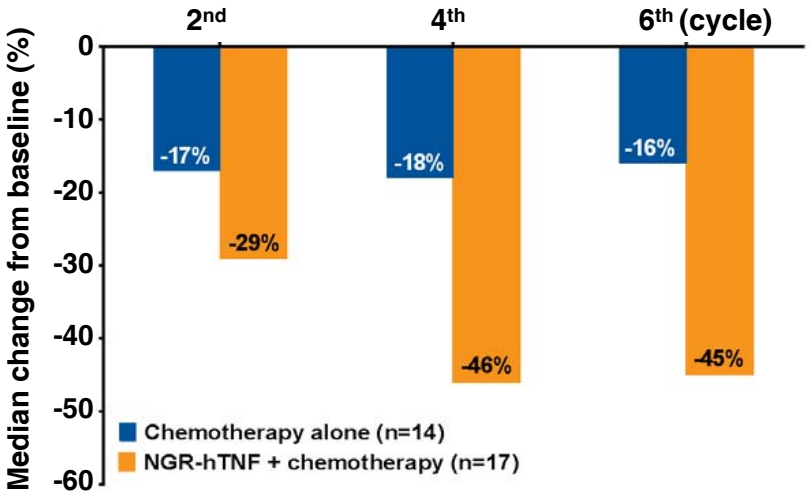


NSCLC: final data show statistically significant survival improvement in squamous subset[1]

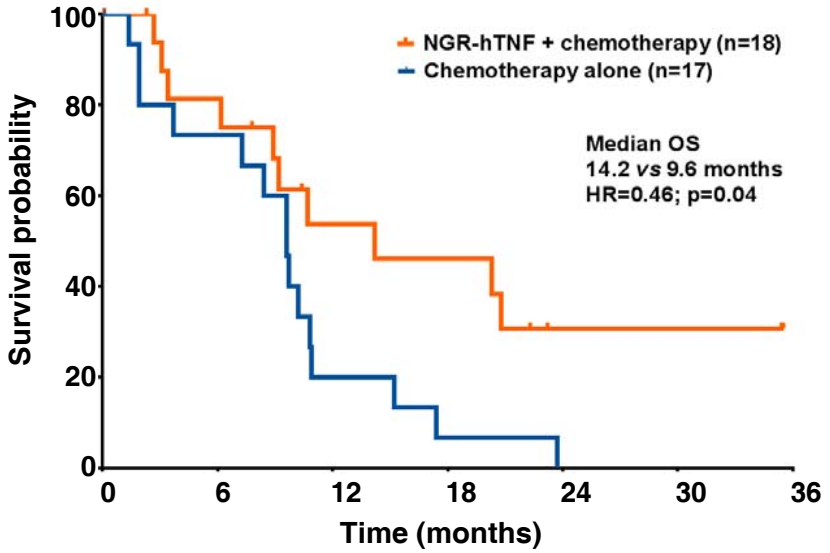
ASCO Annual '13 Meeting

Phase II	Randomized	+ Cisplatin and Gemcitabine	1st line

Decrease in tumour size over treatment



Overall survival (n=35)



After a 2.5-year follow-up time, there is a more than 50% relative reduction in the risk of death

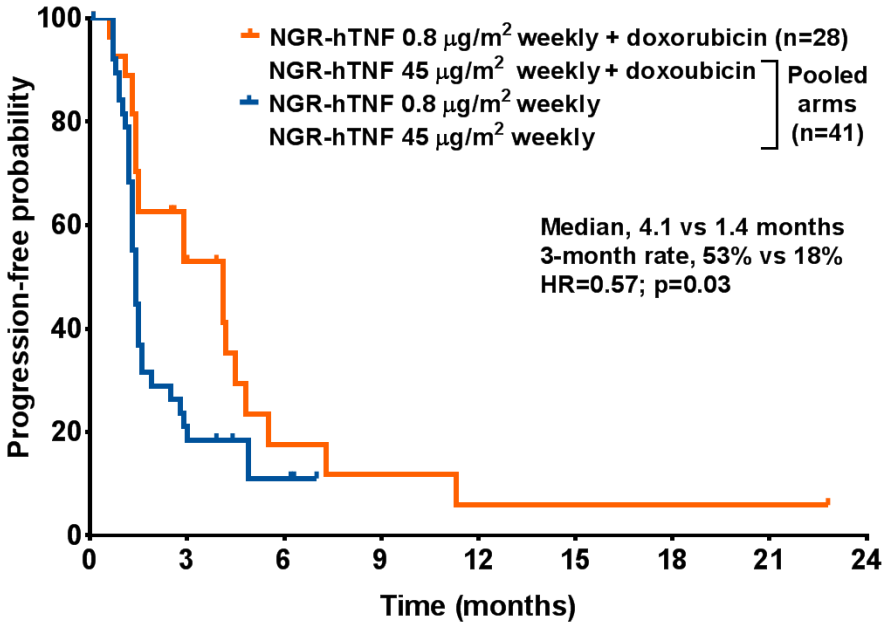
Note: [1] Predefined per protocol

Soft tissue sarcomas: preliminary data show statistically significant clinical benefit

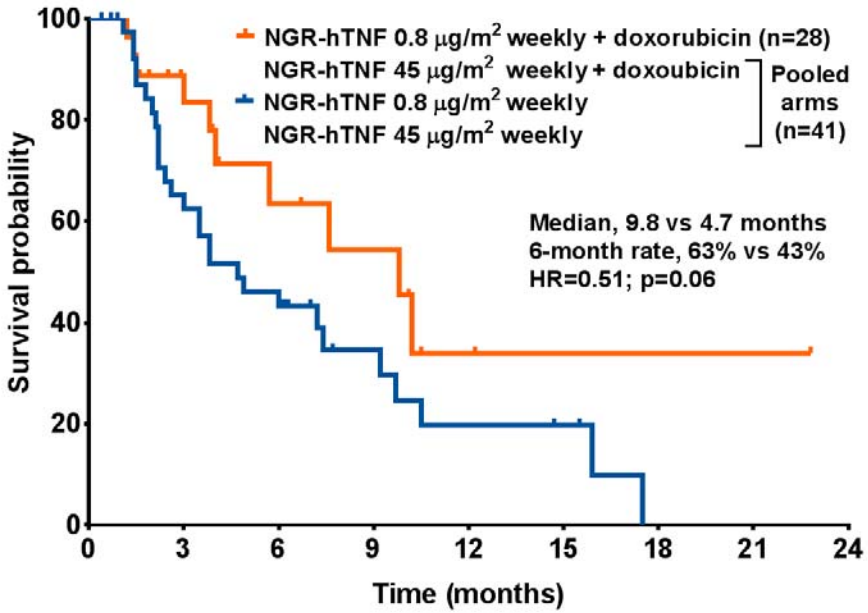
ASCO Annual '13 Meeting

Phase II	Randomized	+ Doxorubicin (60mg/sqm)	≥ 2nd line

Progression free survival (n=69)

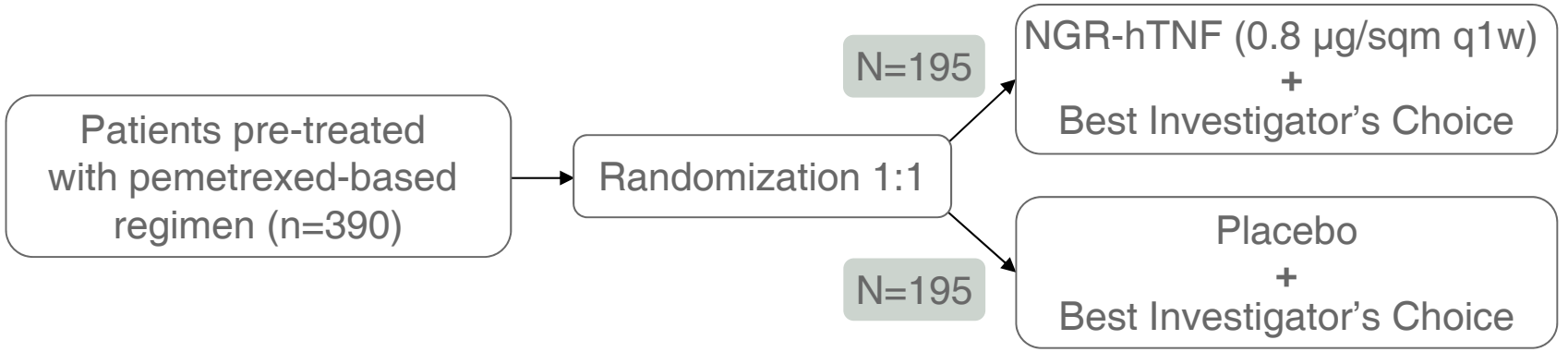


Overall survival (n=69)



Low-dose NGR-hTNF plus doxorubicin significantly improve the clinical benefit in sarcoma patients

Mesothelioma: pivotal Phase III trial as 2nd line therapy (NGR015)



- **Design:** double-blind, placebo-controlled, with a 1:1 randomization ratio
- **Primary endpoint**: survival (N=390; 80% power; 0.05 alpha level; HR=0.72)
- **Status**: enrolment completed (400 pts in EU, US, Canada and Egypt)
- **Primary efficacy analysis**: 3Q 2013

Mesothelioma: doubled median survival duration versus historical controls

Phase II	Single arm	Monotherapy	≥ 2nd line

Comparison with historical controls in 2nd line treatment

	NGR-hTNF Ph II (n=57)	Placebo vs Monochemoth. Ph III (n=660)*
Median PFS (months)	2.8	1.4 vs 1.4
Median OS (months)	12.1	6.2 vs 7.1

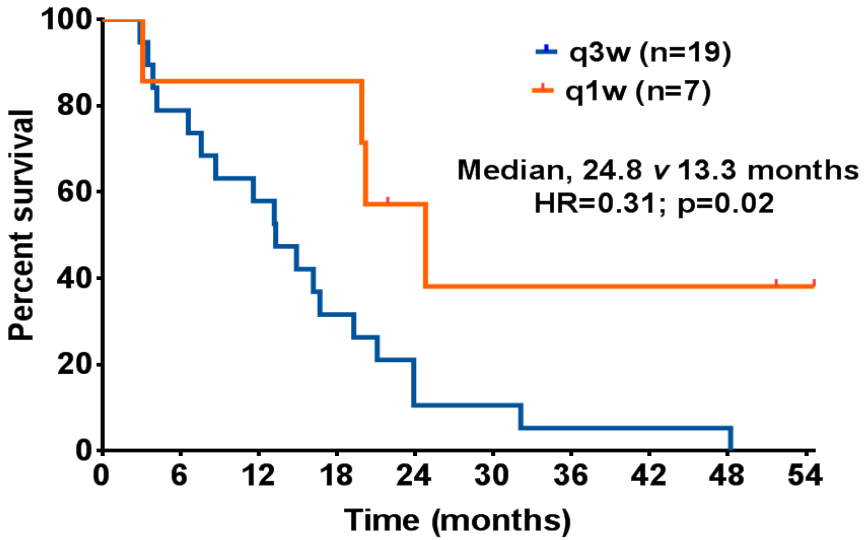
**Krug LM et al. EMCC 2011*

Comparison with Phase III data recently reported in the same setting suggests superior clinical benefit of NGR-hTNF

Mesothelioma: treatment intensification leads to longer patient benefit

Phase II	Single arm	Monotherapy	≥ 2nd line

OS by schedule in patients with disease control



3-year follow-up results strongly confirm the benefit of dose intensification and convincingly support the statistical hypothesis testing of Phase III trial

NGR-hTNF: gearing up for marketing authorization in mesothelioma as first indication

- Enrolment completed in pivotal Phase III (400 patients), results expected by 2H 2013, and registration planned as first indication
- High unmet medical need and low competition scenario: no drugs registered for second-line treatment or in Phase III development
- Orphan Drug designation + patent protection up to 2029
- Development of commercial-scale manufacturing ongoing for liquid and lyophilised formulations:
  - ✓ Low COGS: obtained by fermentation in *E.coli* (one single gene construct)
  - ✓ Strong margin, also in case of price pressure or intense competition

Very low toxicity profile

More than 700 patients treated so far:

- No grade 3-4 drug-related toxicity
- No cumulative toxicity
- No worsening of chemo-associated toxicities
- No pulmonary hemorrhage or bleeding events
- No treatment discontinuations due to toxicity

Suitable for long-term maintenance treatment

NGR-hTNF: data from 5 randomised studies (on 800 patients) available over the next 7 months

	4Q12	1H13	2H13
MPM Ph III (2nd line)	**Accrual completion**		**Primary results**
MPM Ph II (1st line maint)			**Accrual completion**
NSCLS Ph II (1st line)	**Accrual completion**	**Complete results**	
OC Ph II (2nd line)	**Accrual completion**	**24 pts added (weekly dose)**	**Primary results**
STS Ph II (1st and 2nd line)		**Accrual completion**	**Primary results**

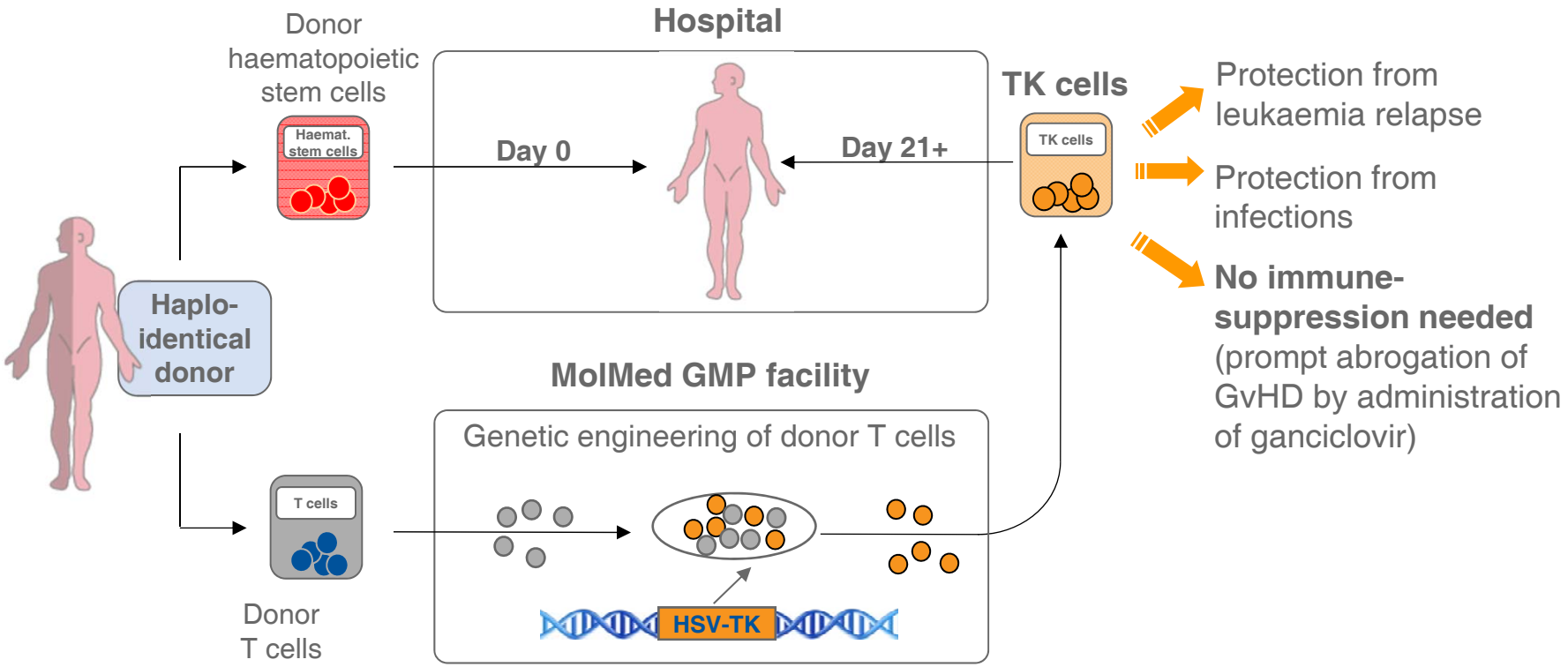
NGR-hTNF: well positioned to become a potential blockbuster

- Proposed mechanism of action confirmed by clinical observations
- Optimal dosing regimen robustly defined
- Compelling results presented at ASCO from randomised studies confirm the antitumor activity of NGR-hTNF observed in key indications
- Favourable tolerability profile confirmed in more than 700 patients
- Easy-to-use predictors of drug efficacy identified

TK: addressing high unmet need to treat high-risk leukemia

- Indication: haematopoietic stem cell transplants (HSCT) for high-risk leukaemia
- Unmet need:
  - ✓ ~50% of patients candidate to HSCT miss a fully matched donor
  - ✓ Without a transplant, high-risk leukaemia patients have extremely low survival rate
- TK technology enables HSCT from partially matched donor **without post-transplant immune-suppression**

TK therapy



Technological innovation within HSCT, the oldest & most consolidated cell therapy (>50 years of clinical practice)

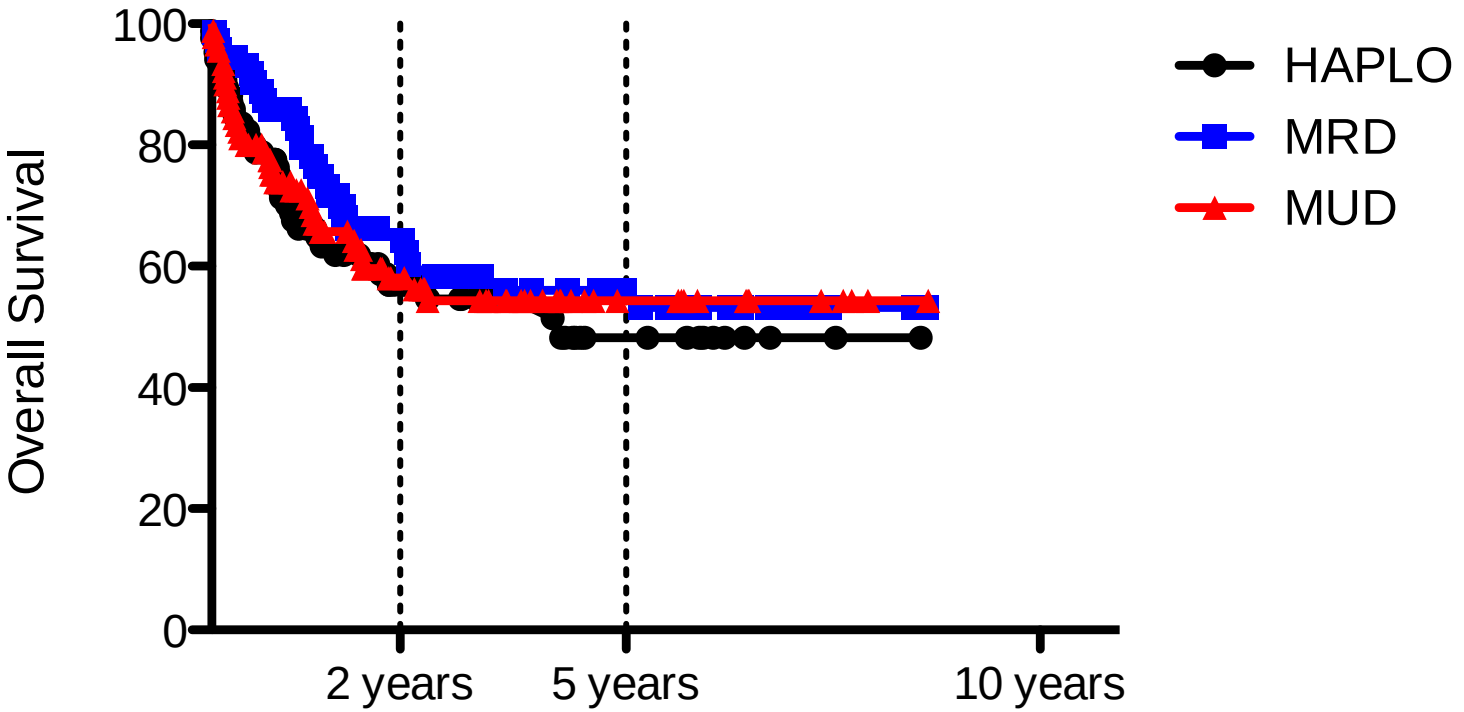
Key results of completed Phase II trial (TK007)

- 73% (22/30) of patients receiving TK achieved immune-reconstitution (IR)

	EBMT survey Mismatched HSCT data (EBMT survey, n=266)	Phase II trial TK007 Phase II TK: patients with IR (n=22)
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 - control	n.d.	50% 100%

TK superiority *vs* historical data

The TK technology in haploidentical transplants



The use of TK has enabled the execution of haploidentical donor transplants, with an overall survival similar to transplants from fully compatible donors

Ongoing pivotal Phase III trial (TK008)

- Enrolment planned: 170 patients, randomisation 3:1 in favour of TK
- Primary endpoint: disease-free survival
- Ongoing in Europe and US
- Recent improvements to the TK Phase III trial:
  - ✓ Enlargement of study population to relapsed patients
  - ✓ New treatment option in the control arm to perform an unmanipulated HSCT followed by cyclophosphamide, as GvHD prophylaxis
  - ✓ Modifications already implemented in the majority of clinical centers

TK: getting ready for the market

- Orphan Drug designation + patent protection (with SPC) up to 2030
- Phase II long-term data available and pivotal Phase III trial under way
- Planned application for Conditional Approval in EU in 2013 based on:
  - ✓ Proof of efficacy
  - ✓ Established long-term safety data
  - ✓ High unmet medical need for patients lacking HLA-matched donor
- Automation of cell manufacturing process ongoing
- Small dedicated sales force required

MolMed, the pioneer in gene-modified cell therapy

- MolMed's pioneering TK technology: *ex vivo* genetically-engineered T cells
- Telethon's ADA-SCID (bubble boy) project: developed by MolMed on the same technology platform
  - ✓ 12 children suffering from ADA-SCID were treated
  - ✓ 8 of 10 first treated patients no longer require enzyme-replacement therapy*
  - ✓ All 10 lead a normal life with significantly improved physical conditions*
- ADA-SCID gene therapy was in-licensed by GlaxoSmithKline from Telethon in 2010

*Source: *N Engl J Med 2009; 360:447-458*

MolMed, the leader in cell and gene therapy

- ADA-SCID success has placed MolMed among the top players in the field of cell and gene therapy

- GlaxoSmithKline signed an agreement for the development of the ADA-SCID commercial production process (€ 5.5 million in two years)
- Telethon Foundation signed an agreement for the development of six gene therapies for rare genetic diseases (€ 8.3 million in four years)

Opportunity for further industrial partnerships

Summary

- NGR-hTNF
  - ✓ Statistically significant clinical benefit demonstrated in randomised studies
  - ✓ Phase III enrolment completed, results expected in 3Q 2013
  - ✓ Manufacturing process on track, IP protection granted
- TK
  - ✓ Planned application for Conditional Approval to EMA in 2013
  - ✓ Proof of efficacy achieved and long-term safety data established
  - ✓ High unmet medical need for patients lacking HLA-matched donor
- GMP solutions
  - ✓ Successful development of ADA-SCID therapy
  - ✓ Important contract signed with big pharma
  - ✓ Opportunity for further industrial partnership

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Thanks for your attention