MOLMED

Leading the way in cell & gene therapy

New York, June 2016

From genes to therapy

Forward-looking statements

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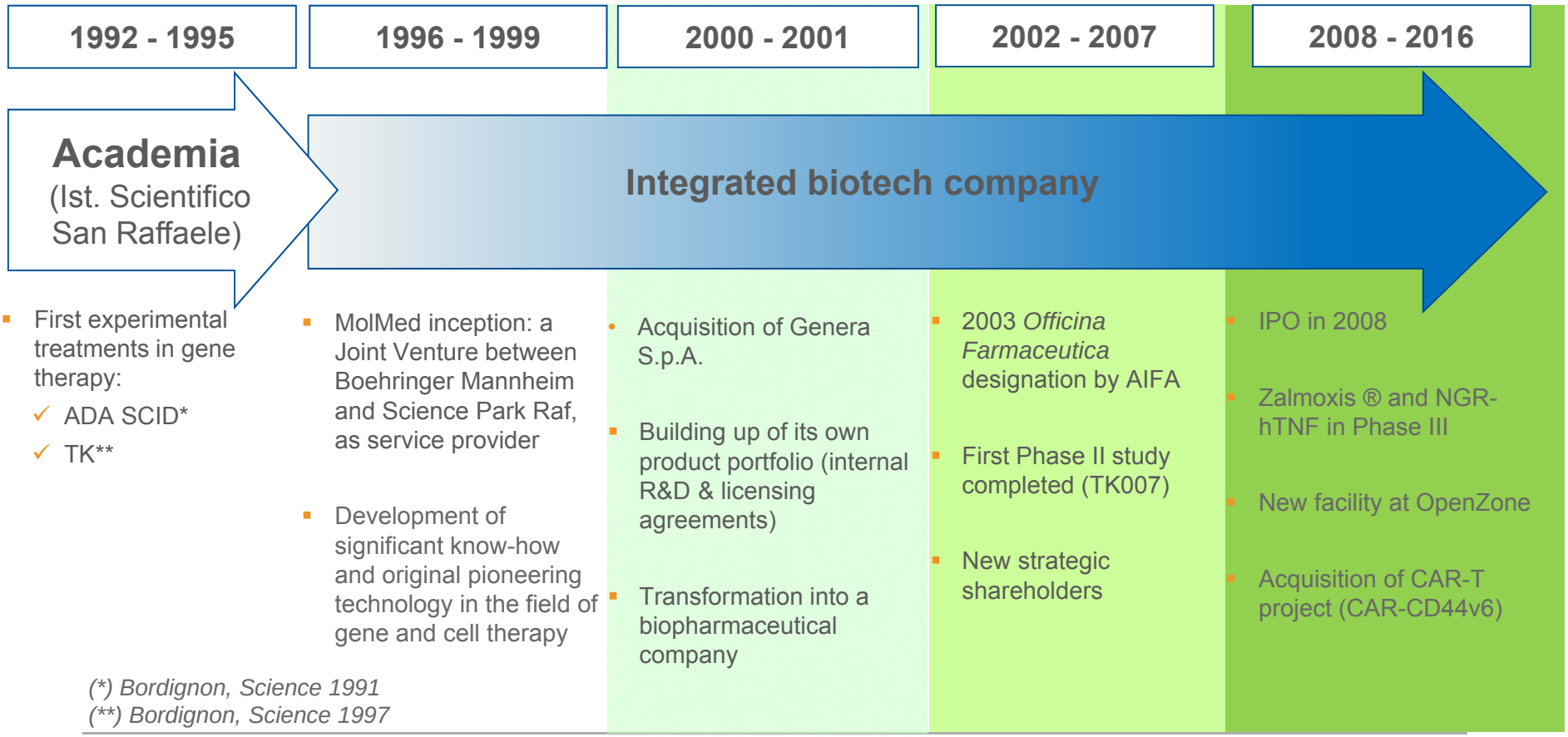
The undersigned herewith attests, pursuant to Article 154-bis, paragraph 2 of the Italian Consolidated Law on Finance (Legislative Decree 58/1998), that the accounting disclosure contained in this presentation matches documentary evidence, corporate books, and accounting records.

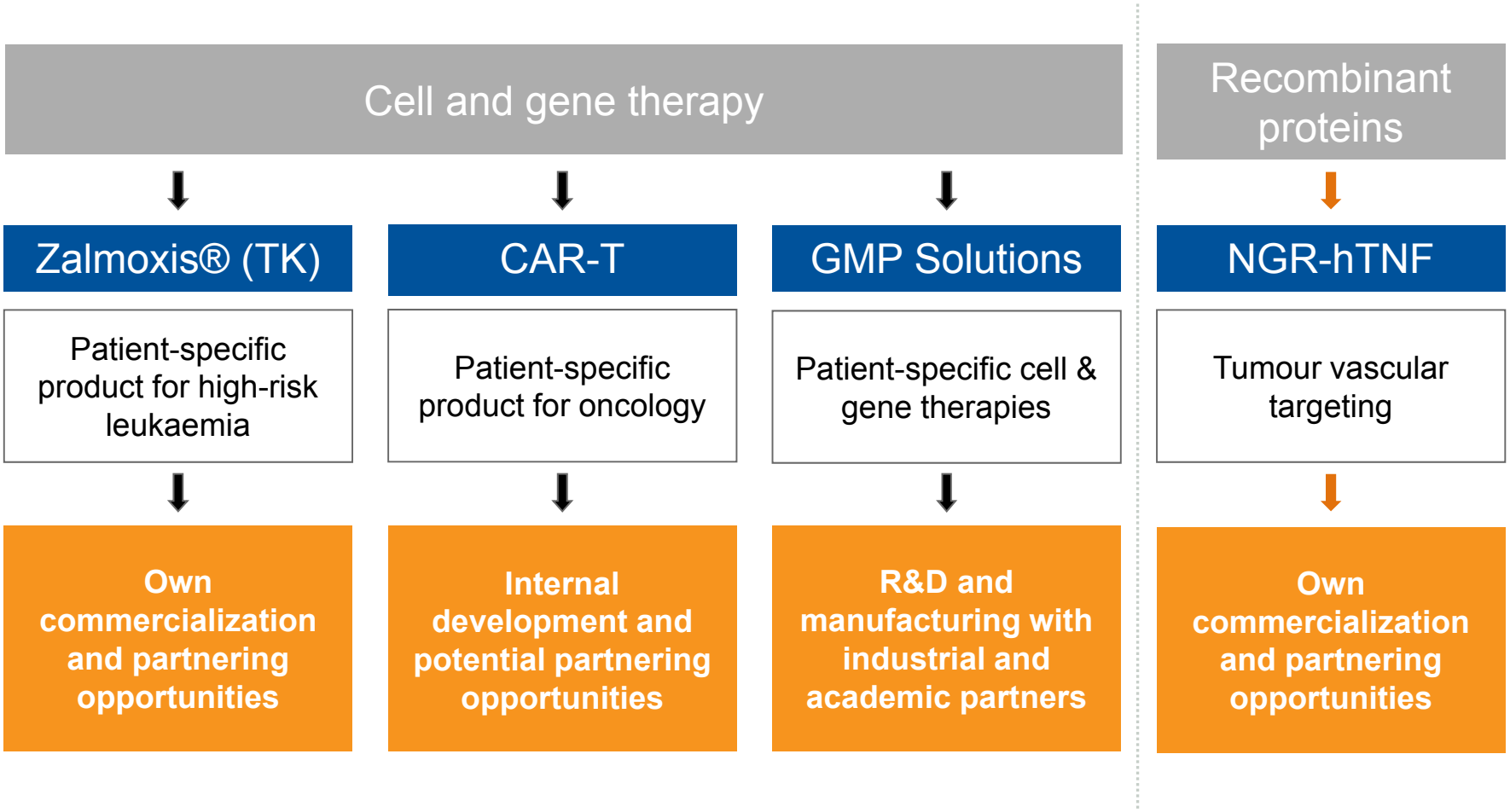
Andrea Quaglino, Chief Financial Officer, official Corporate Financial Reporting Manager

From academia to public company

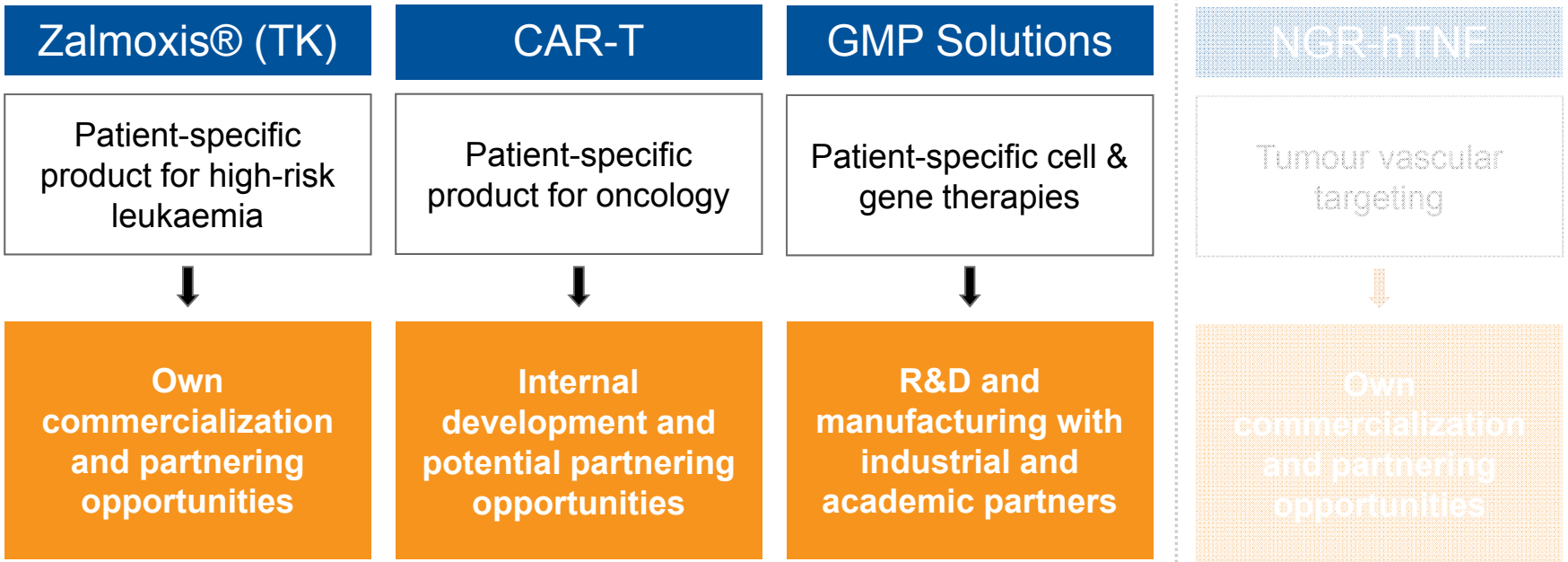


(*) Bordignon, Science 1991
(**) Bordignon, Science 1997

MolMed's offer: two technology platforms



Two technology platforms: cell & gene therapy



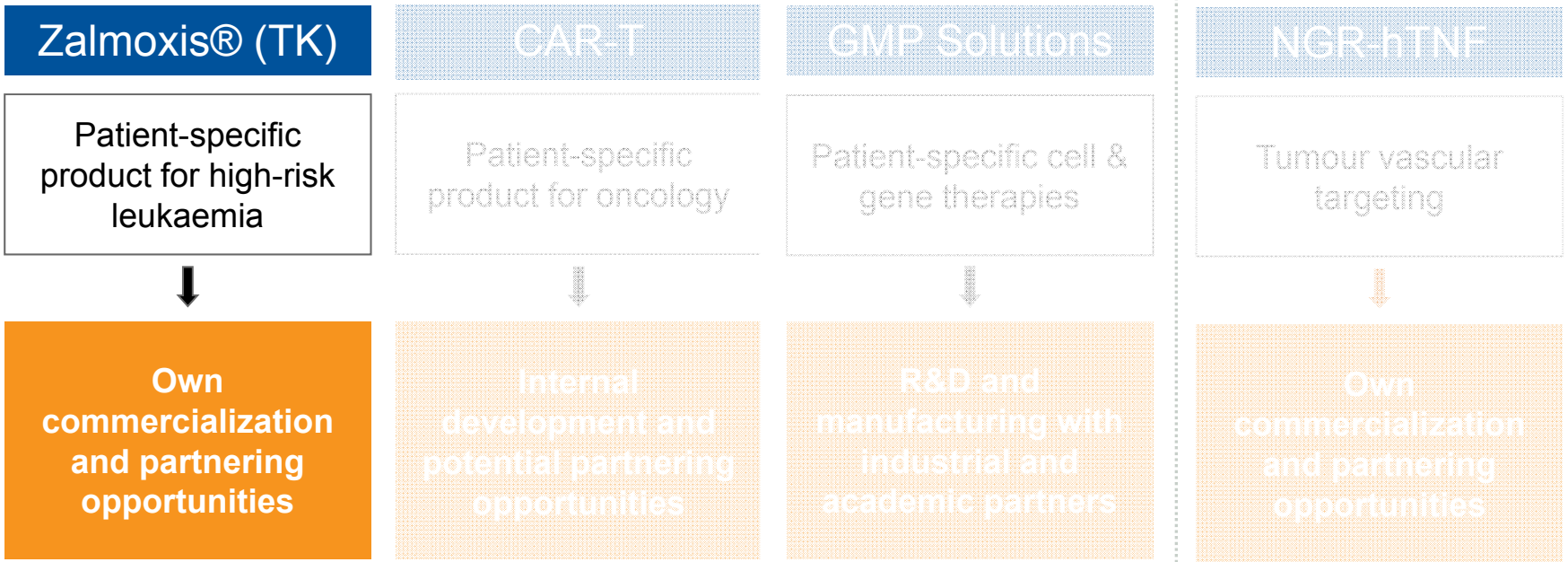
Leading position in Europe in cell & gene therapy

- More than **15 years experience** in RV/LV vector manufacturing and genetically modified T-cells and hematopoietic stem cells
- Two novel investigational treatments: **Zalmoxis®** (TK), a cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immune-suppression, currently in Phase III in high-risk acute leukaemia and **CAR-CD44v6**, an immuno-gene therapy project potentially effective for many haematological malignancies and several epithelial tumours, currently in preclinical development
- **Authorization** for manufacturing products for clinical trials and for market
- **Long lasting collaboration** with pharma, biotech, charities and academia (GSK, Telethon, San Raffaele Hospital)
- One of the **largest and most advanced facility** for cell transduction and vector production in the cell & gene therapy field
- **Opportunity to partner** with US and EU biotech and pharma companies for the clinical implementation of cell & gene therapy programs

Cell & gene therapy activities: for proprietary products and for external partners

Vector	*Therapy*	*Product development*	*Clinical manufacturing*	*Commercial development*	*Commercial manufacturing*
RV	TK	→	→	→	MOLMED
RV/LV	CAR-CD44v6	→	MOLMED		
RV	ADA	→	→	→	gsk
LV	MLD WAS	→	→	gsk	
LV	βThal MPS-I GLD CGD	→	iget		
---	DMD	→	→	Telethon	
LV	Hemophilia	→	**Undisclosed multinational biotech**		
LV	MM	→	genenta science		

Two technology platforms: cell & gene therapy



Zalmoxis®: MolMed's paradigm of immuno-gene therapy of cancer

- Cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immune-suppression:
  - ✓ Inducing a rapid immune reconstitution associated with prolonged survival, regardless of disease status at transplant
  - ✓ Readily controlling Graft-versus-Host-Disease (GvHD) in 100% of patients, without administering immune-suppressive drugs
- Safety and efficacy data of Zalmoxis® trials compared to data from both EU and US registries (EBMT and CIBMTR):
  - ✓ Halved non-relapse mortality, particularly due to infections
  - ✓ Increased overall survival
- Currently in Phase III in high-risk acute leukaemia, it is the largest immuno-gene therapy of cancer program, with more than 170 patients treated worldwide
- Under evaluation by EMA for a Conditional Marketing Authorization
- Patent protection up to 2030 (with SPC) and Orphan Drug designation in Europe and US: proof of unmeet clinical need for patients lacking HLA-matched donor
- 2 GMP facilities for in-house vector production and patient's cell transduction

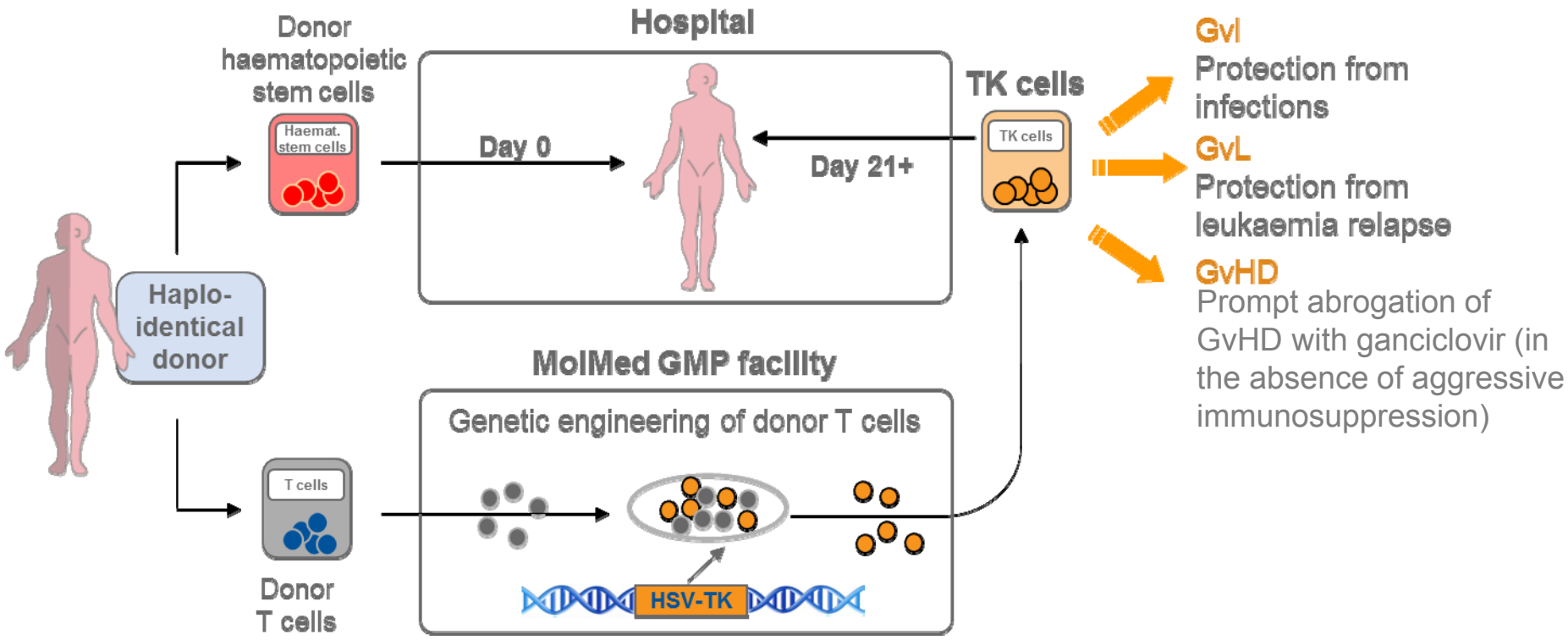
Limits of haploidentical HSCT: GvHD

- Since donors and patients are not fully matched, there is a **higher risk of graft-versus-host-disease** (GvHD), which is the most severe adverse reaction occurring after the transplantation, **caused by donor T cells**

- There are two protocols currently used to prevent GvHD:
  1. T-cell depletion
  2. Post-transplant immunosuppression → mainly through cyclophosphamide administration

- TK is now emerging, in the scientific arena, as a promising method to overcome major limitations of haplo-HSCT, increasing the rate of success and enabling a curative approach to virtually all patients in clinical need *(Michael Pulsipher, Blood, 31 July 2014)*

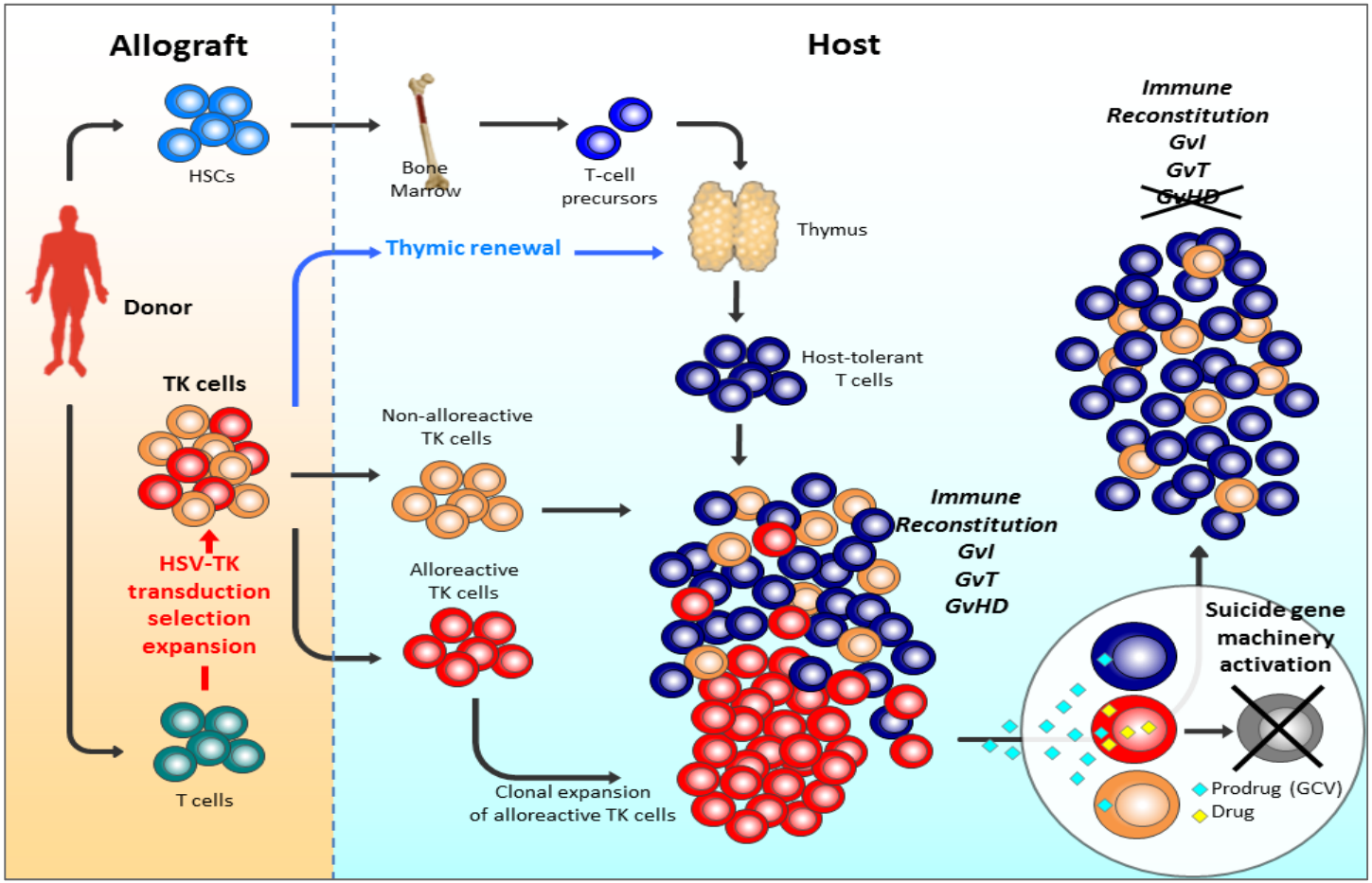
Zalmoxis® allows to preserve the GvI and GvL effects while controlling GvHD



The TK haploidentical HSCT procedure makes a suitable donor available for any patient, without interfering with the timeframe of a normal transplantation

Bordignon, *Hum Gene Ther 1995;* Bonini, *Science 1997;* Bonini, *Nat Med 2003;* Traversari, *Blood 2007;* Ciceri, *Blood 2007;* Ciceri, *Lancet Oncol 2009*

Selective control of GvHD: ganciclovir is active only on proliferating TK cells



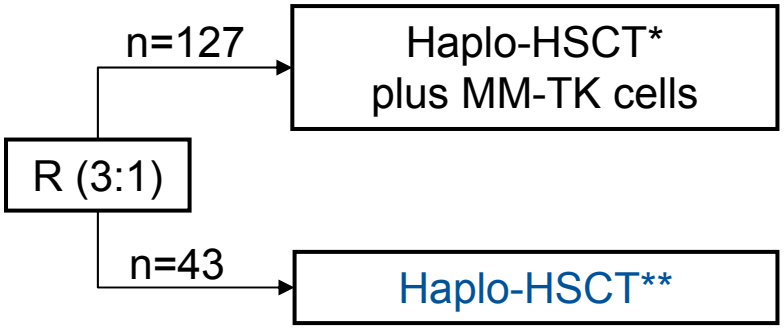
TK008 (Phase III trial): study design

- **Key inclusion criteria**
  - ✓ **AML-ALL at high-risk in first CR**
  - ✓ **AML-ALL in ≥ second CR**
  - ✓ **secondary AML in CR**
  - ✓ **advanced-stage AML/ALL**
  - ✓ **lack of HLA-matched relat/unrel donor**
- **Stratification**
  - ✓ **disease status (1st vs > 1st vs relapse)**
  - ✓ **performance status (0 vs 1)**
  - ✓ **country**
- **Endpoints**
  - ✓ **Primary: DFS/PFS**
  - ✓ **Key secondary endpoints: OS, NRM, CIR, IR, GvHD**
- **Statistics**
  - ✓ **n=170 patients**
  - ✓ **HR=0.55;**
  - ✓ **1-β=80%; α=0.05 1-year DFS, 30% vs 52%**
- **Dose of MM-TK cells: 1x107/Kg**

  Up to 4 monthly doses, in absence of IR and/or GvHD

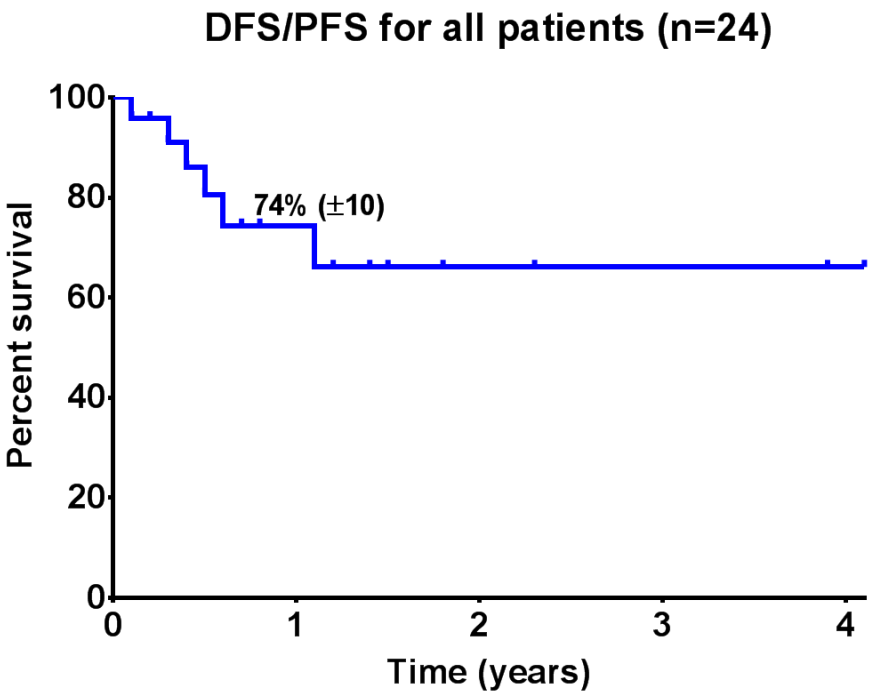
  Starting 21 to 49 days after HSCT

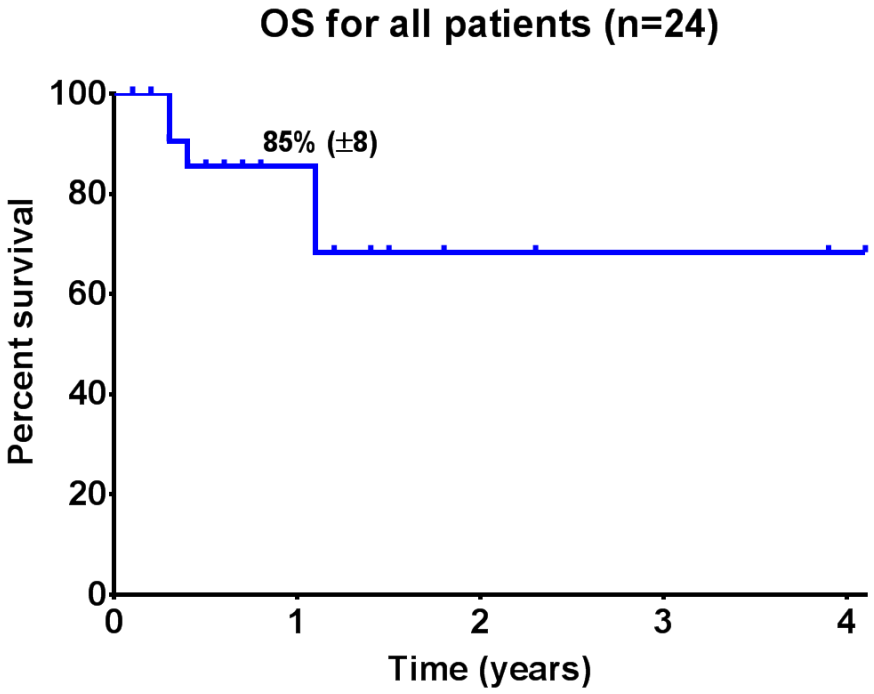
  IR: CD3+ cell count ≥ 100/µL



* T-depleted (T cells, 1x10^4/Kg)
**T-depleted (T cells, 1x10^4/Kg)
or
**Unmanipulated BM/PB + HD CTX

TK008: disease-free survival (DFS) and overall survival (OS)





1-year DFS/PFS:

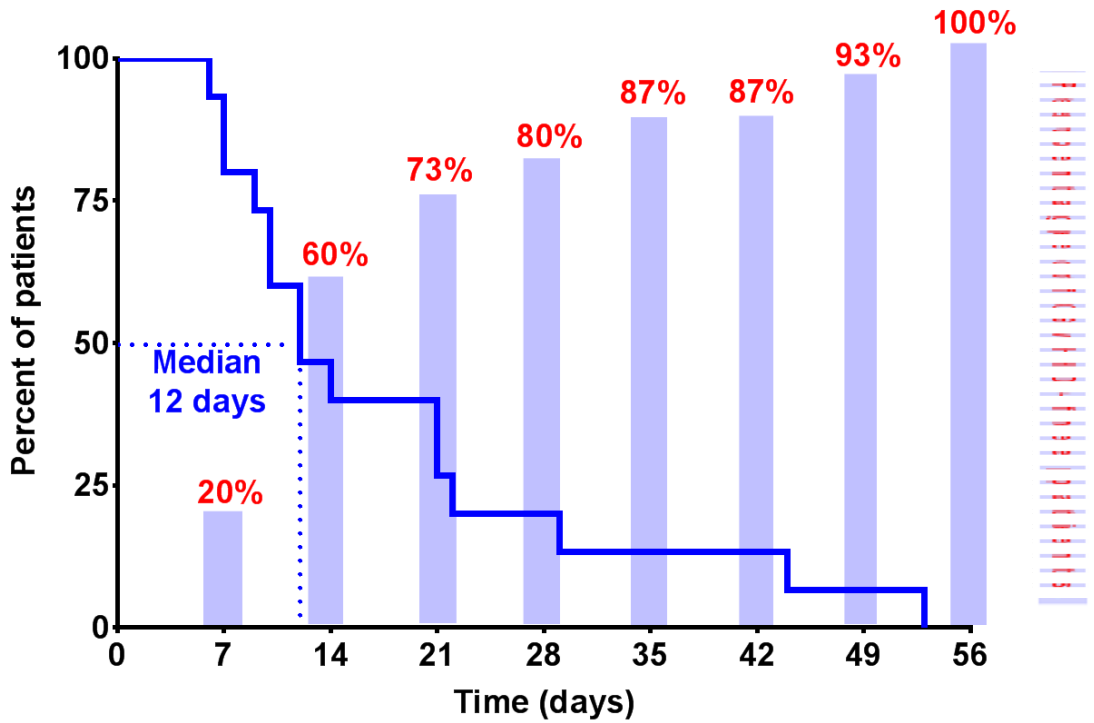
Expected in the experimental arm: 52%
Expected in the control arm: 30%

1-year OS:

Expected in the experimental arm: 60%

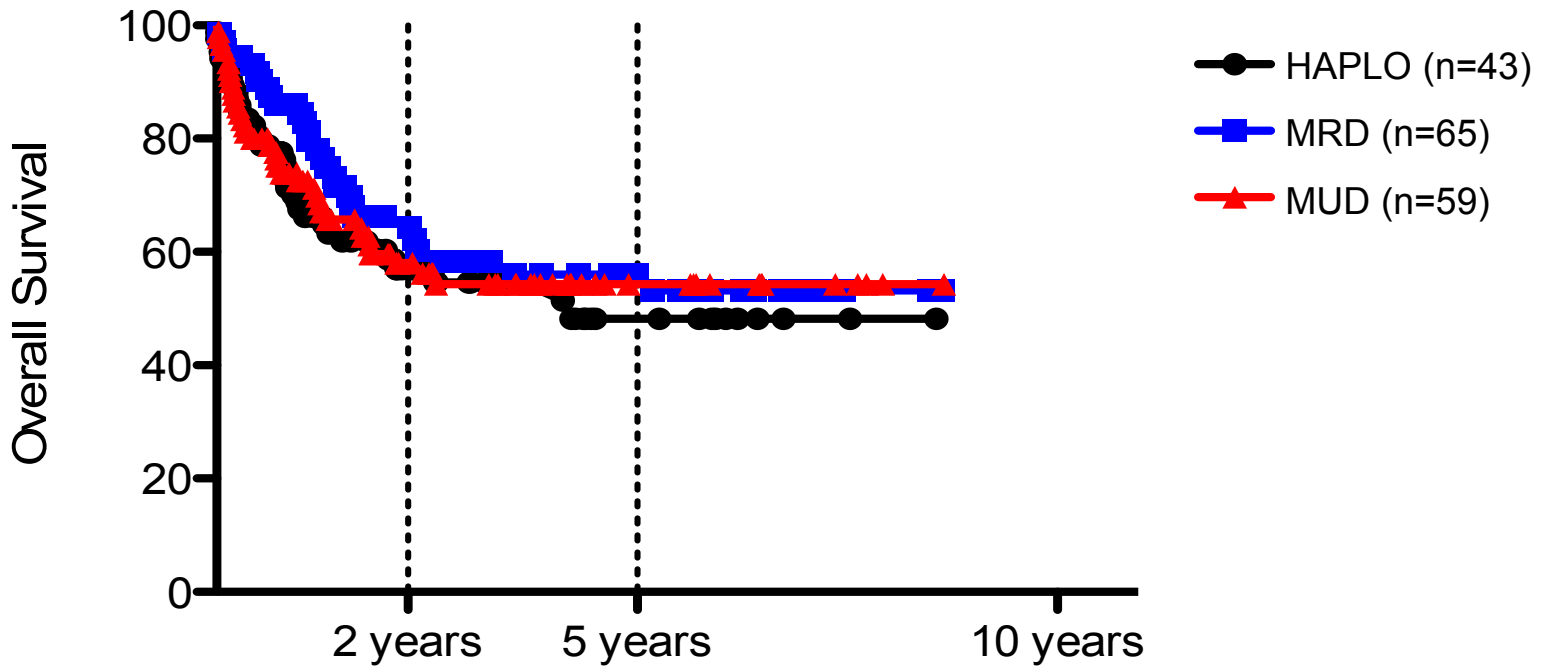
TK008 & TK007 (pooled): Rapid and complete resolution of GvHD

TIME TO RESOLUTION AND % OF PATIENTS GVHD FREE FROM GVHD ONSET (DAYS; N=16)



Note: Pulled data from TK007 and TK008 (experimental arm)
Source: ASH Meeting 2014, Abs. 2535

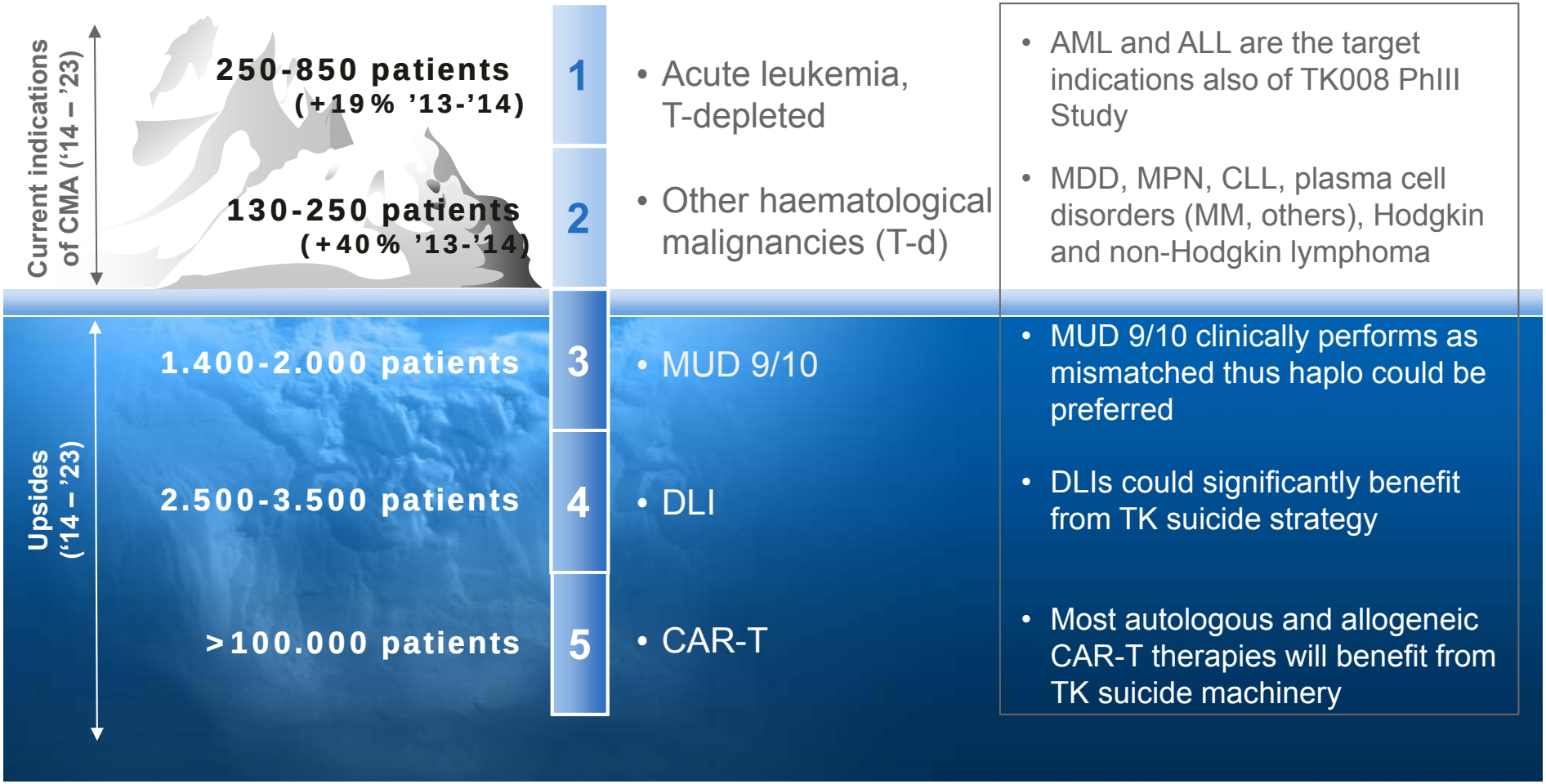
The TK technology in haploidentical transplants



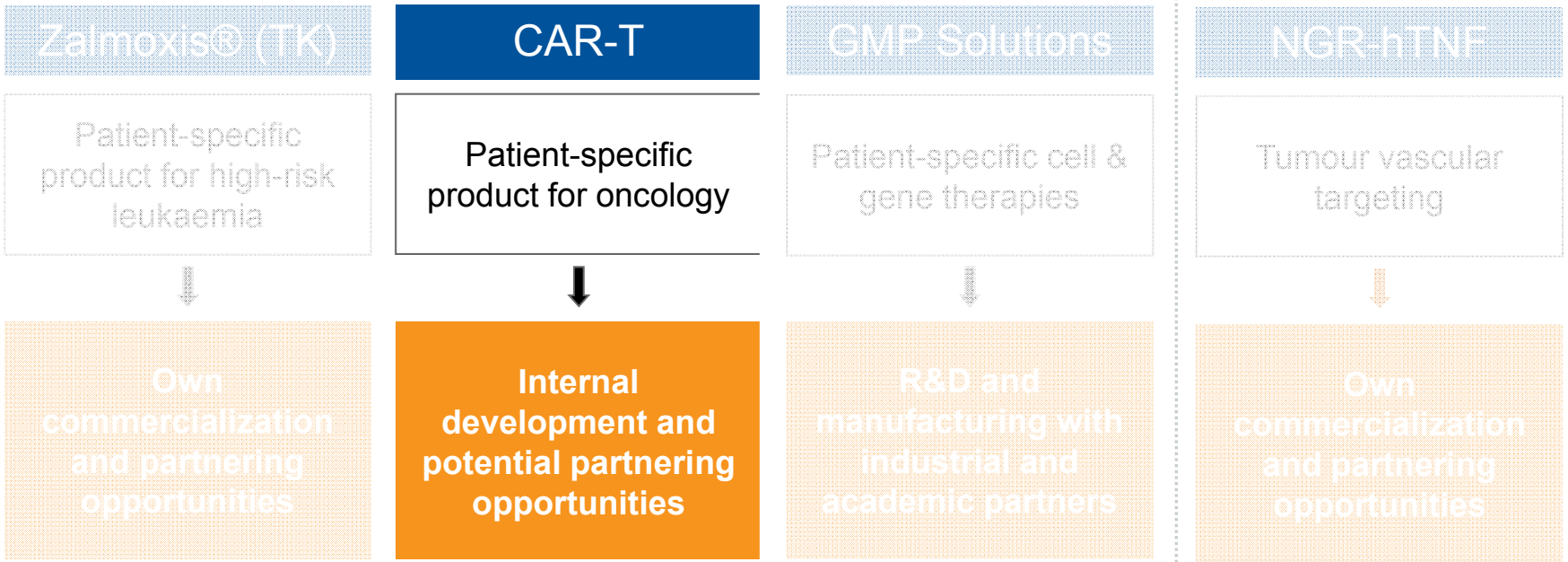
The use of TK has enabled:

- the execution of haploidentical donor transplants, with an overall survival similar to transplants from fully compatible donors
- the extension of eligible transplant patients from 50% to 85%

Zalmoxis market potential (EU): strong growth and relevant upsides



Two technology platforms: cell & gene Therapy



CAR-T: the new frontier of immune-gene therapy for cancer

- On April 13, 2015, MolMed significantly expanded its pipeline, entering one of the most promising fields of new anticancer strategies, tumour “immune-gene therapy”, by purchasing the project CAR-CD44v6 from the San Raffaele Hospital
- A CAR (Chimeric Antigen Receptor) is an engineered receptor, usually derived from an antibody, that grafts an arbitrary specificity (usually of a monoclonal antibody) onto an immune effector cell (usually a T cell), thus directing patient's immune system against cancer via the recognition of a specific antigen on the surface of tumour cells
- The CAR-CD44v6 is specific for the CD44v6 antigen, which is expressed by haematological tumours (e.g. leukaemia and multiple myeloma) and by several solid tumours of different histotypes, including breast, lung and colon carcinomas

CD44v6 is expressed by several blood and solid cancers

CD44 is over-expressed in haematological and epithelial tumours

- 60% of AML and 90% of MM express CD44v6
- Historically knowns as **«metastatic factor»** in multiple epithelial cancers, including:
  - ✓ **breast cancer** (triple negative)
  - ✓ **pancreatic adenocarcinoma**
  - ✓ **head & neck cancer**
- Crucial role in growth of **brain tumour stem cells**
- Specifically expressed on **colon cancer stem cells**

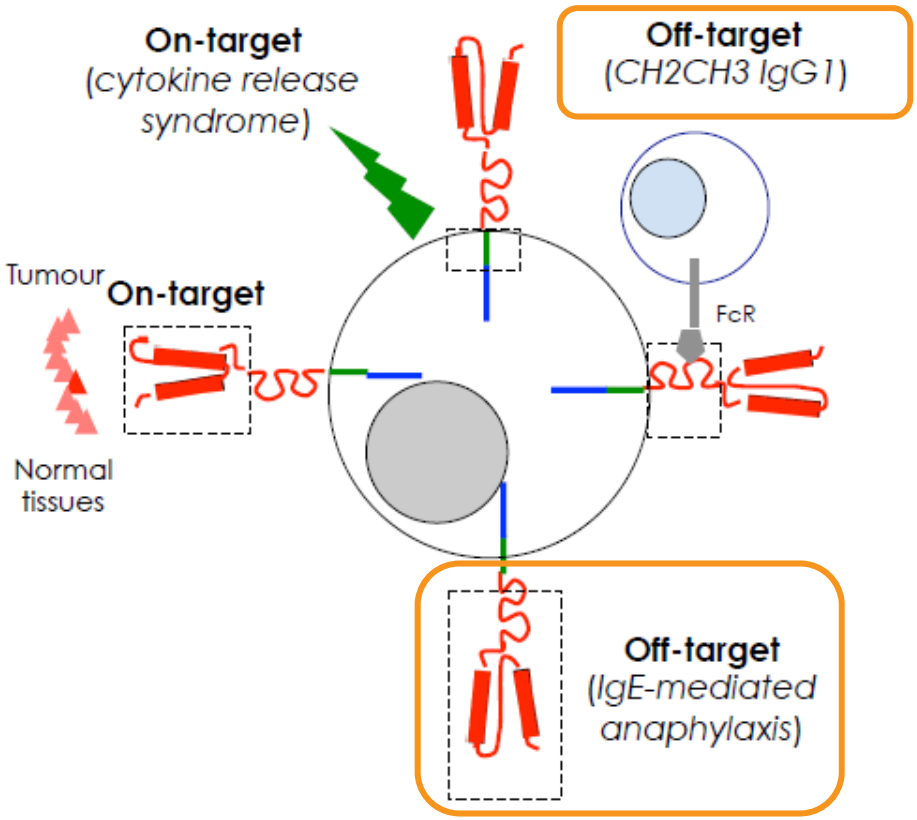
The target is clinically validated

Clin Cancer Res. 2006 Oct 15;12(20 Pt 1):6064-72.

A phase I dose escalation study with anti-CD44v6 bivatuzumab mertansine in patients with incurable squamous cell carcinoma of the head and neck or esophagus.

Tijink BM[1], Buter J, de Bree R, Giaccone G, Lang MS, Staab A, Leemans CR, van Dongen GA.

Off-target toxicities might be managed by exploiting the combination of a suicide gene



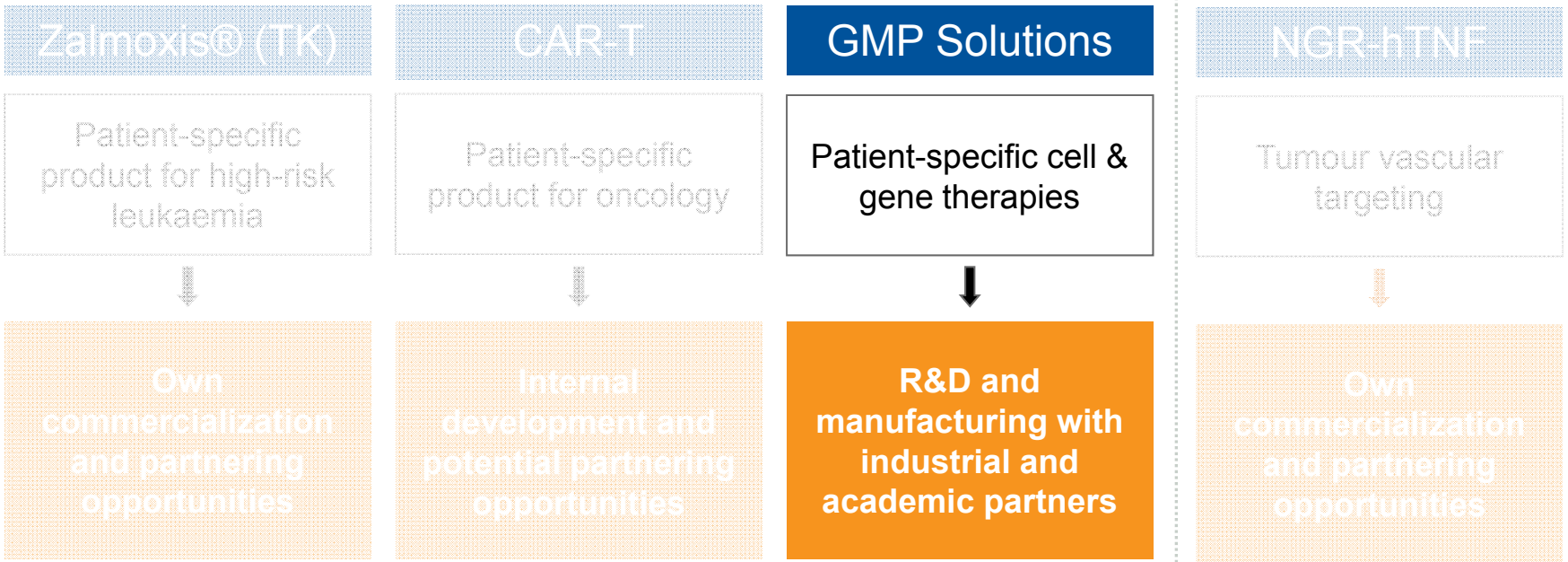
OFF-TARGET TOXICITIES

- CD44v6 expression by normal cells
- Cytokine release syndrome
- Non-specific spacer-mediated activation
- CAR-CD44v6 expression on effector cells

Use in combination with TK suicide gene

Source: Casucci et al, Cancer Immunol Immunother

Two technology platforms: cell & gene therapy



Challenges in cell & gene therapy

Scientific idea, Discovery & Vision

Preclinical and Clinical Development

Process, Manufacturing and Control

La persistencia de la memoria **(Salvador Dalì, 1931)**

Bench

Translation (>15-20 years)

Bedside

*Challenges in Process, Manufacturing and Control of cell & gene therapy…**fully managed by MolMed***

Bench

- Scale-up, robustness and reproducibility of the process
- QC robustness & strategy to assess vector and DP (identity, potency, purity and safety):
- Process and analytical method validation strategy and economic viability of CMC
- Quality Assurance
- Continuous interactions with Regulatory Authorities
- Manufacturing demands and quality-control standards for more prevalent diseases

Bedside

Almost 15 years of GMP manufacturing for MolMed's GMP facility at DIBIT (Milan)

UAO/PC/IM

AIFA/UAO/P/ 11652

Roma, 17 NOV. 2015

n. aM - 170/2015 del 13/11/2015

Agenzia Italiana del Farmaco

AIFA

Ufficio Autorizzazioni Officine

- Authorized GMP manufacturing facility since 2003 for **clinical programs**
  - ✓ Patient-specific manufacturing and production of critical reagents for cell&gene therapy
- Authorized GMP manufacturing facility for **commercial products in 2015***
  - ✓ Zalmoxis (TK)
  - ✓ Strimvelis (ADA-SCID): EMA authorization on Apr. 1st, 2016

Note: (*) Authorization of manufacturing for commercial products is subordinated to product specific-marketing authorization by EMA

GMP Solutions – Activities with third parties

- Impressive track record of successful completion of development programs in collaboration with industrial and academic partners
- Tailored programs spanning from early development phase up to market-compliant manufacturing processes
- Flexibility in agreement structuring according to partner's needs:
  - ✓ feasibility studies
  - ✓ initial fee-for-service contracts
  - ✓ milestone-based strategic agreements
  - ✓ long lasting collaborations including IP exclusivity
  - ✓ long term GMP suite reservation
- Support for clinical development and regulatory activities, based on long lasting experience of interaction with EU and US authorities

EMA marketing authorization on Strimvelis™

PRESS RELEASE

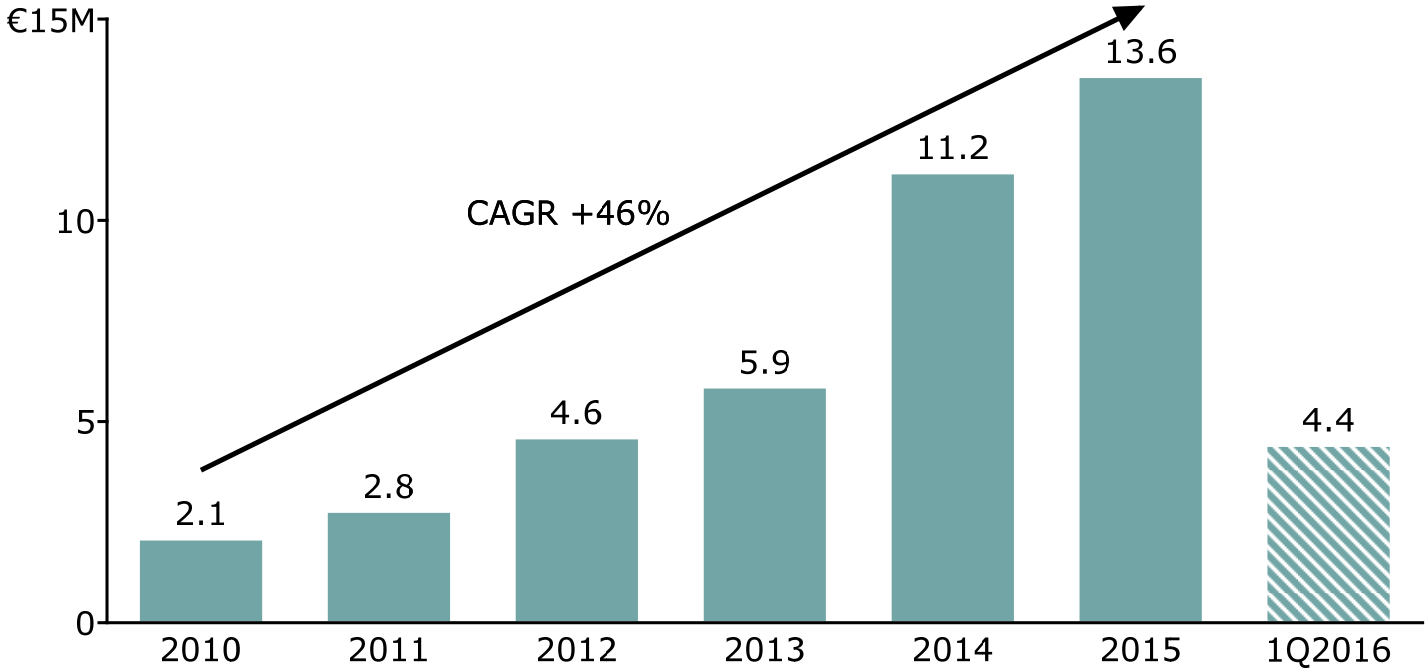
The European Commission grants the European marketing authorisation to Strimvelis™, GSK stem cell therapy for ADA-SCID patients

Milan (Italy), May 30, 2016 – The European Medicines Agency (EMA) has approved Strimvelis, the first *ex- vivo* stem cell gene therapy to treat patients with a very rare disease called ADA-SCID (Severe Combined Immunodeficiency due to Adenosine Deaminase deficiency). Strimvelis (autologous CD34+ cells transduced to express ADA) is the first corrective gene therapy for children to be awarded regulatory approval anywhere in the world. It is indicated for the treatment of patients with ADA-SCID for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

[…]

Significant growth of revenues from CMO services and partnering with third parties

REVENUES FROM CMO SERVICES (M€)



YoY (%)		33%	66%	27%	91%	21%	76%

New MolMed facility at OpenZone Campus, Bresso (Milan)

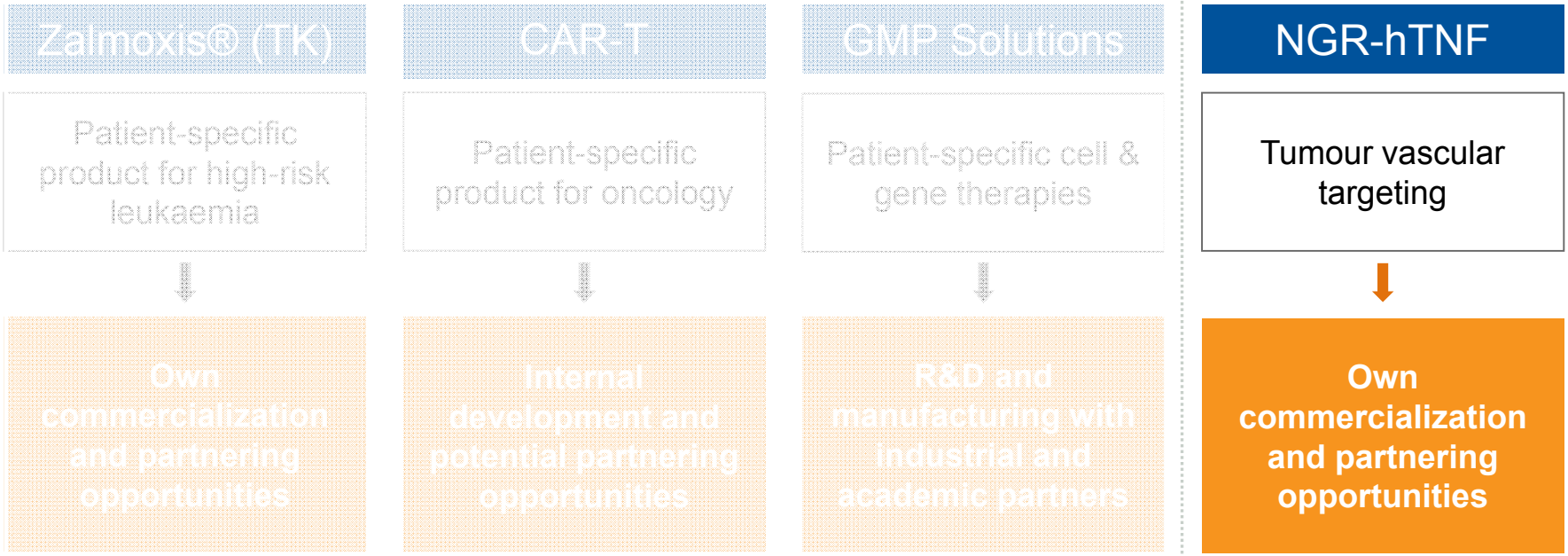
New GMP facility to face increasing internal and external demand

- Manufacturing capacity enhanced by 3,300 square meters, three times the current one
- Quality Control and Development laboratories completely equipped
- State of the art design and technical solutions allowing modular usage of the facility
- Possibility of customized suite layout in accordance with partner's needs
- Additional 21 clean rooms will be gradually made operational and authorized starting from second half 2016
- People hiring and training program ongoing

Grade C Corridors – walls, windows and false ceiling in sealing step

Grade B/C/D rooms' variable air volume regulators

Two technology platforms: recombinant proteins

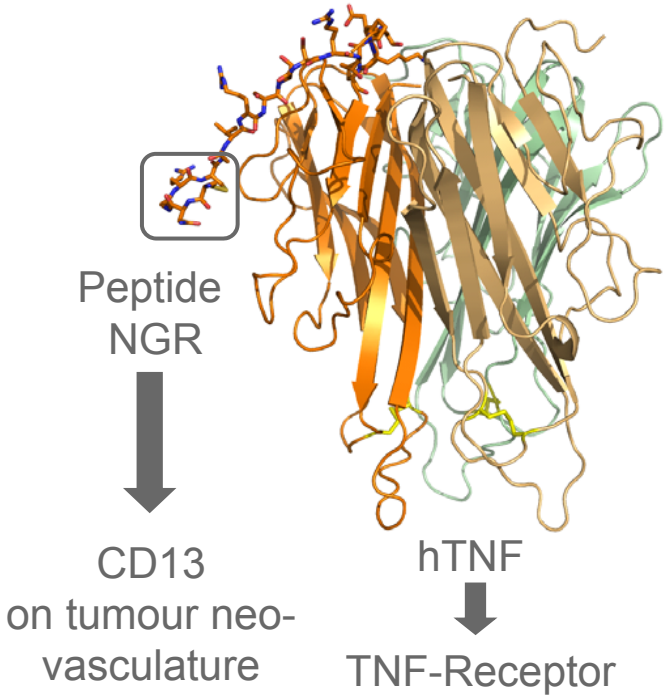


NGR-hTNF : a high potential vascular targeting agent in late stage development

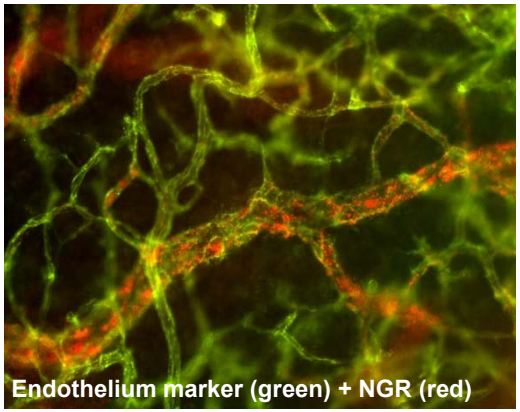
- Statistically significant efficacy data from randomized studies in mesothelioma, NSCLC, soft tissue sarcomas and ovarian cancer
- Phase III data in mesothelioma data indicate a statistically significant increase of survival in patients with a very poor prognosis (~50% of population)
- Grounds for conditional/accelerated approval
  - ✓ rarity/seriousness of disease with high and rapid mortality
  - ✓ significant safety profile (no therapy discontinuation because of toxicity)
  - ✓ benefit/risk balance highly positive
  - ✓ lack of either approved drug or valid therapeutic option
- Patent protection up to 2029 and orphan drug designation in EU and US
- Filing for conditional/accelerated approval in EU and US for high-risk mesothelioma patients as second-line treatment foreseen in Q4 2016
- Business strategy: out-licensing / partnership

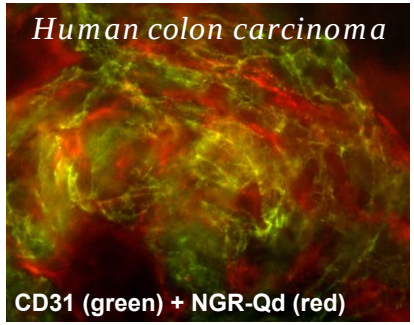
NGR-hTNF: a safe and selective recombinant protein

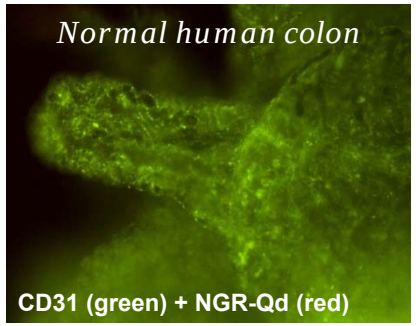
Recombinant fusion protein



Specific NGR binding to tumour blood vessels





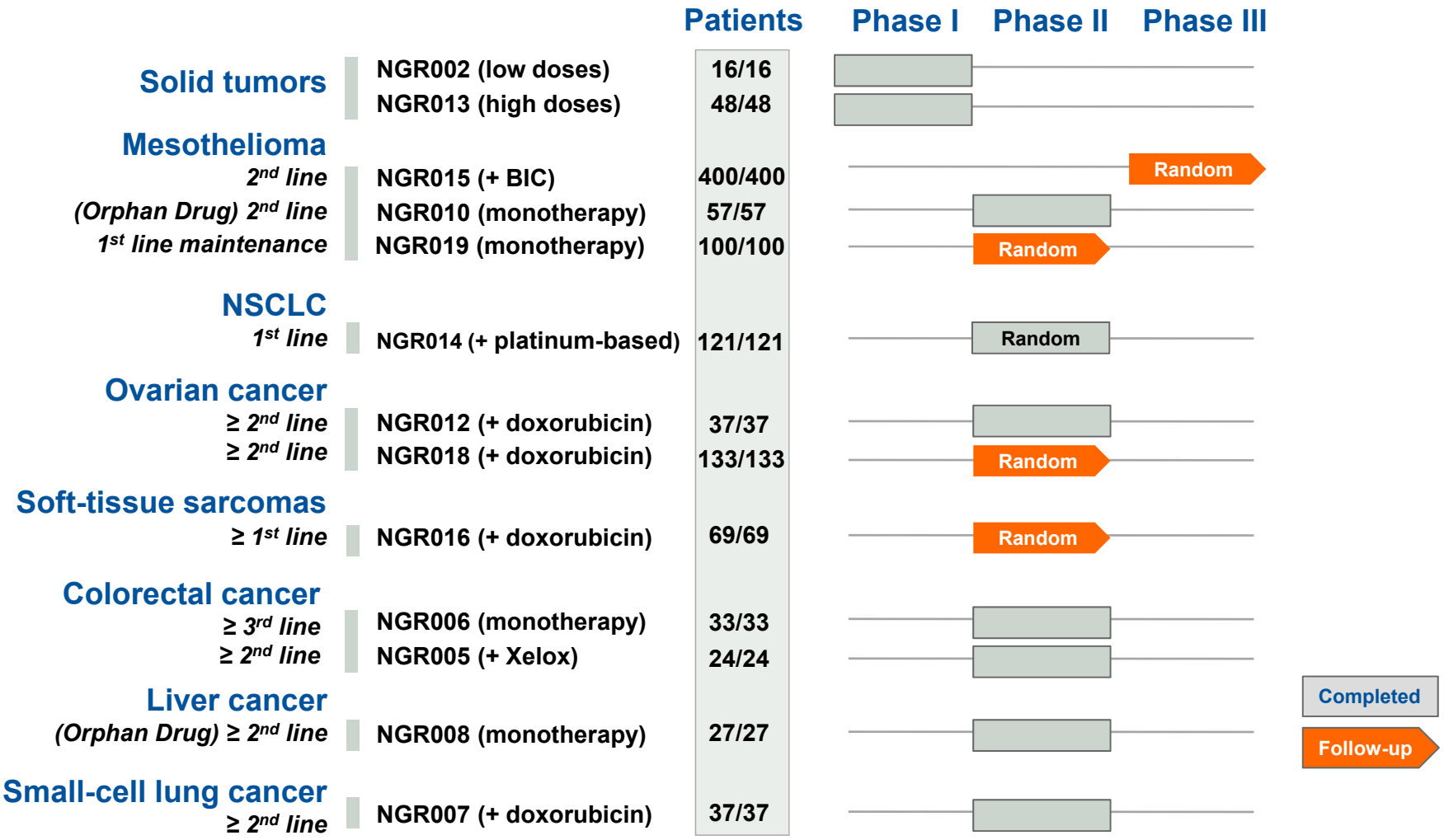


Whole mount analysis of tissues obtained from the same patient (N=3)

Doses of 0.8 µg/sqm systematically show antitumour activity

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

NGR-hTNF: clinical development with more than 1.000 enrolled patients

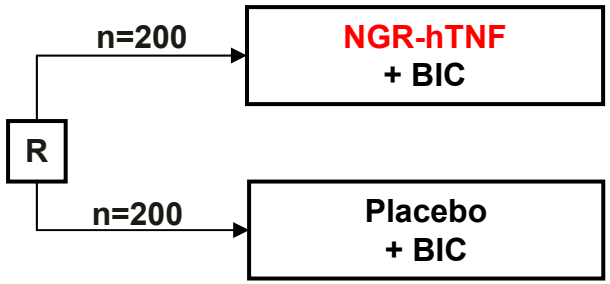


NGR015 (Phase III in mesothelioma): study design

- **Primary endpoint**
  - ✓ overall survival (OS)
- **Key secondary endpoint**
  - ✓ progression-free survival (PFS)
- **Stratification factors**
  - ✓ performance status (0 or 1-2)
  - ✓ chemotherapy (yes or no)
  - ✓ chemotherapy agent
- **Statistical considerations**
  - ✓ α=0.05; 1-β=0.80; HR=0.72; n=390 (306 events)
  - ✓ accrual time: 33 months
  - ✓ median follow-up time: 18.9 months
  - ✓ subgroup analysis by 8 baseline risk factors: age, sex, PS, histology, EORTC score, best response to prior therapy, neutrophil-to-lymphocyte ratio (NLR) and treatment-free interval (TFI)
- **Investigational sites:** 41 in EU (Italy, UK, Poland, Belgium, France, Spain, Sweden, Ireland and Netherlands), USA, Canada and Egypt

Source: ASCO 2015 Abs 7501 Oral presentation

- **Indication:** Patients with advanced malignant pleural mesothelioma who had previously failed a pemetrexed-based regimen



- **NGR-hTNF/placebo**

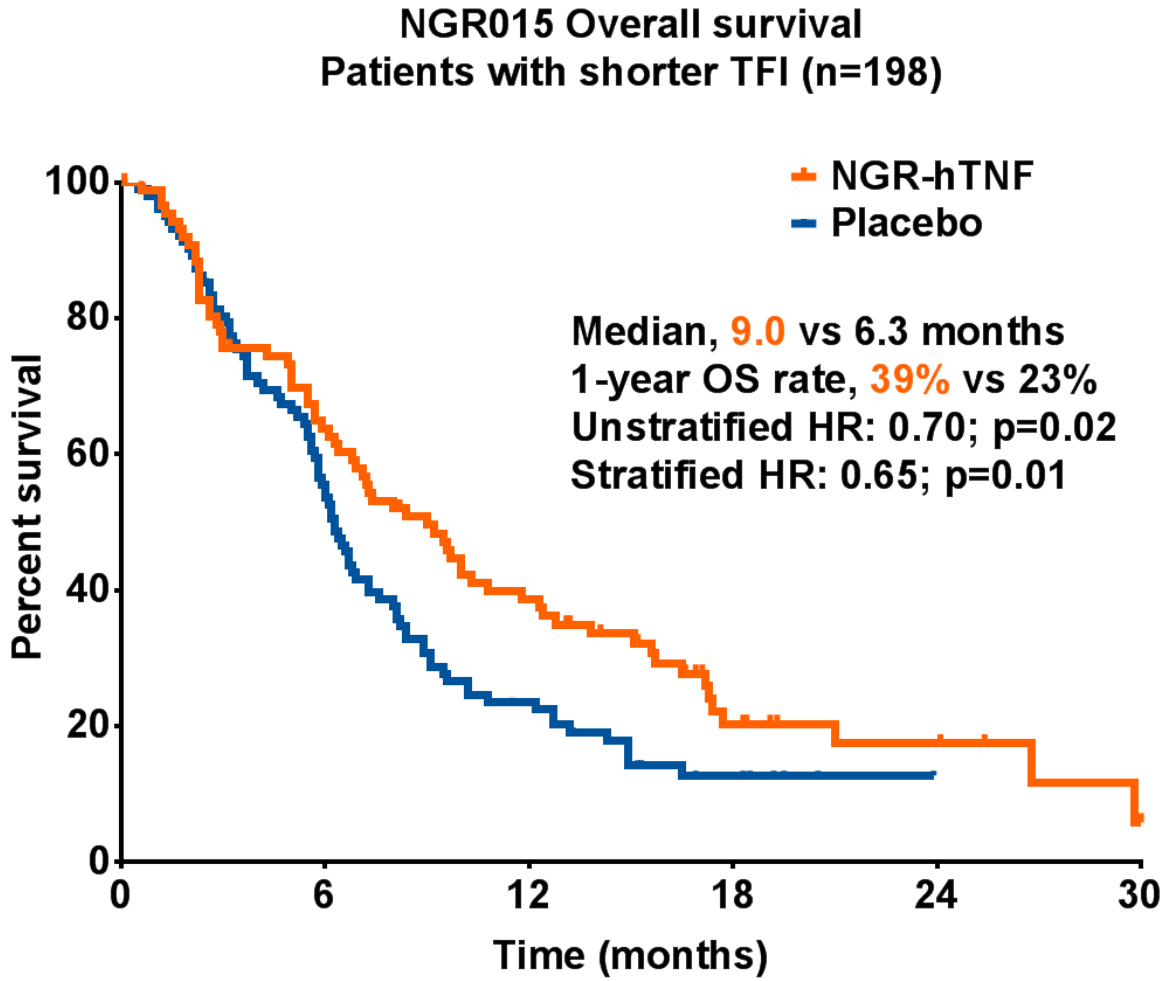
  0.8 μg/m² weekly until progressive disease (PD)

BIC (Best Investigator Choice)

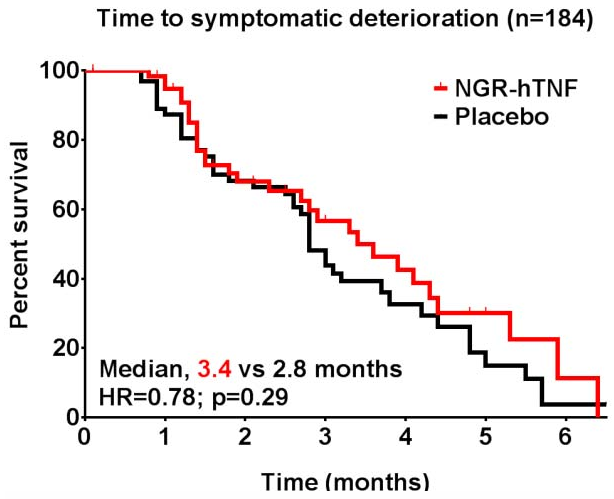
- ✓ Supportive care only
- ✓ Single-agent chemotherapy (up to max 6 cycles)
  - gemcitabine (1,000-1,250 mg/m² iv d1+8 q3w)
  - vinorelbine (25 iv or 60 os mg/m² d1+8 q3w)
  - doxorubicin (60-75 mg/m² iv d1 q3w)

(95% of patients treated with chemotherapy)

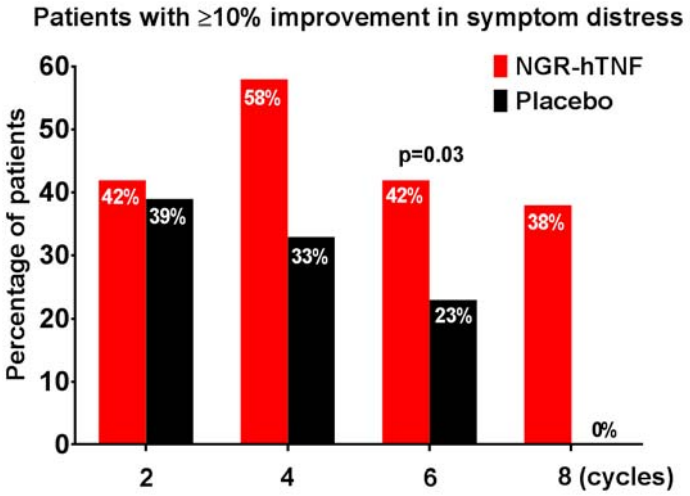
NGR015: significantly improved survival in the patient subset with short TFI

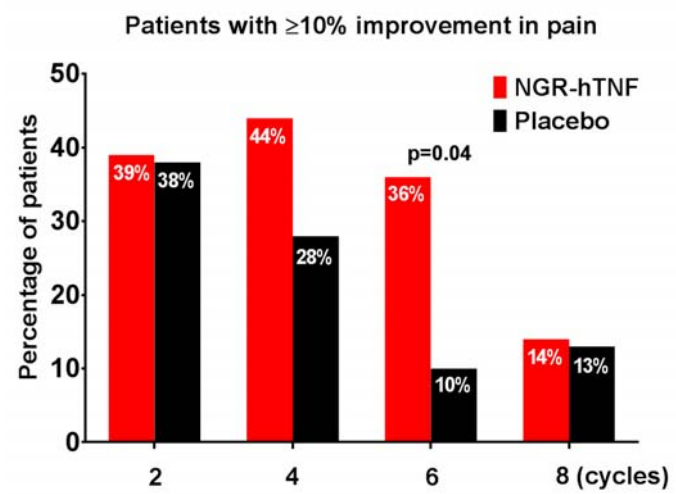


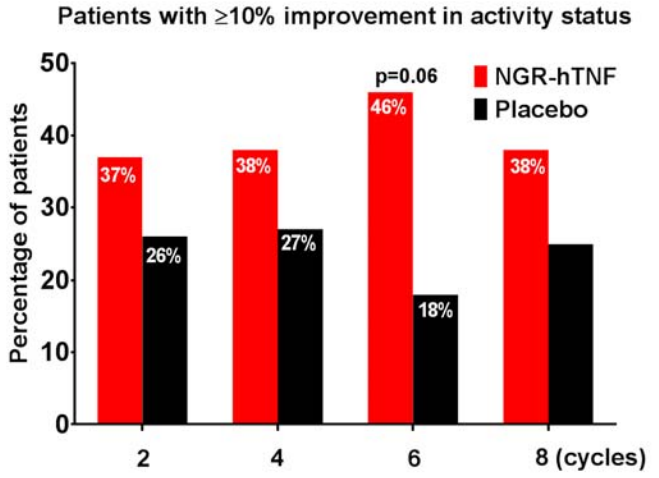
NGR015: subset with short TFI
Quality of Life (Patient-Reported Outcomes)



5 mayor symptoms (appetite loss, fatigue, cough, dyspnea, pain)







Market potential of NGR-hTNF

Indications	*Clinical phase*	*Incidence* (EU27, USA, CA)*	*Incidence* (CN, JP, KR)*
Pleural Mesothelioma First line - Maintenance	II	8'300	3'000
Pleural Mesothelioma Second line	III	5'800	2'100
Sarcomas	II	a blockbuster potential	
Ovarian carcinoma Platinum-resistant	II		
Liver carcinoma Sorafenib-resistant	II		
SCLC	II		
NSCLC Squamous histology	II		
Colorectal carcinoma	II		
Total		> 1'000'000	> 1'500'000

** source: Globocan 2012 (http://globocan.iarc.fr/Default.asp)*

Key financials

Income Statement

(amounts in Euro thousand)	First Quarter 2016	First Quarter 2015	Δ %	FY 2015	FY 2014	Δ %
Operating revenues	5,343	2,658	101.0%	16,764	12,422	35.0%
Revenues from activites for third parties	*4,408*	*2,499*	*76.4%*	*13,576*	*11,181*	*21.4%*
Operating costs	9,383	6,799	38.0%	37,302	25,050	48.90%
Operating result	(4,040)	(4,141)	2.4%	(20,538)	(12,628)	(62.6%)
Net result	(4,126)	(4,232)	2.5%	(20,784)	(13,003)	(59.8%)

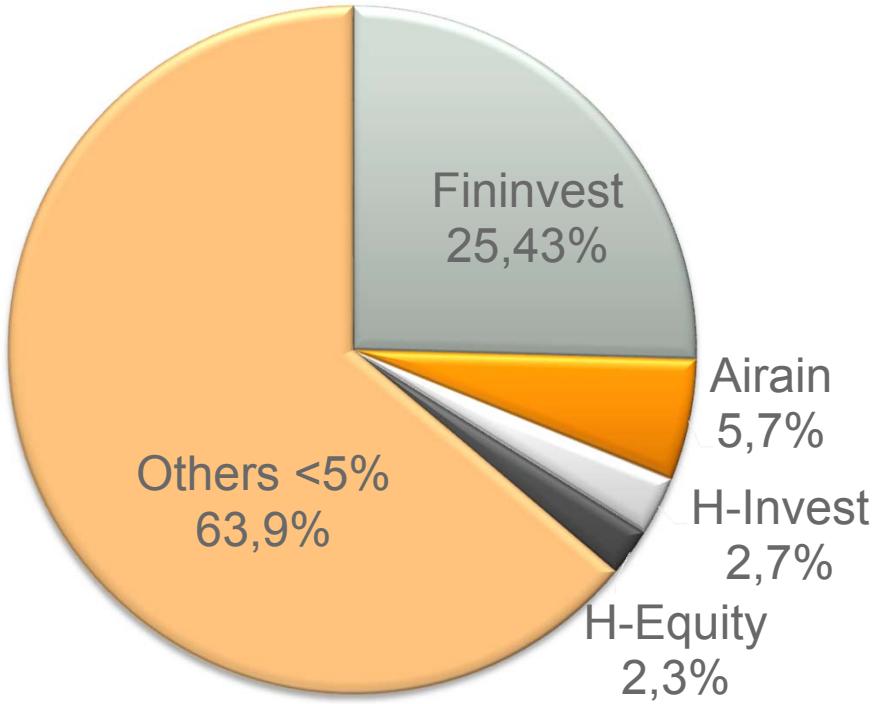
Net Financial Position

(amounts in Euro thousand)	Mar. 31, 2016	Dec. 31, 2015	Δ €	Δ %
Net Financial Position*	23,832	29,938	(6,107)	(20.4%)

** Including solely cash and cash equivalents as the Company has no indebtedness*

Shareholders' structure (at April 7, 2016)

Market cap: ~152 M € (at June 2, 2016)



MolMed is uniquely positioned to capitalize on the positive dynamics of the cell & gene therapy field

Leading technology platforms	▪ Two distinct innovative technology platforms, including cell & gene therapy and recombinant protein
Late clinical stage compounds	▪ TK and NGR-hTNF, two proprietary compounds in phase III clinical development in EU ▪ Extensive package of clinical data, proving efficacy and safety, from randomized controlled trials ▪ Significant opportunity from a partnering stand point
Approaching the market with TK	▪ Conditional Marketing Authorization filed in EU for Zalmoxis®, the largest gene therapy application
Validated GMP solutions	▪ Worldwide renowned leading role for development, translation and market-compliant manufacturing of innovative cures in cell & gene therapy (for GSK and Telethon) ▪ Authorized GMP manufacturing facility for clinical programs and commercial products
Strategic collaborations	▪ Network of partnerships with pharma companies and research institutes
Stable shareholder base	▪ Core group of shareholders with long term commitment

Our priorities (and 2016 achievements √)

TK

- Complete the clinical (Phase III) and manufacturing (automation) development
- Pursue Marketing Authorization in EU and US
- Intensify activities preparatory to market access (both directly and through distributors/dealers) √

NGR-hTNF

- Submit a CMA application for high-risk mesothelioma indication in late 2016
- Expand the use of NGR-hTNF in other clinical indications, following the promising phase II results
- Complete the development and validation of a market-compliant manufacturing process √

CD44v6 CAR

- Continue research and pre-clinical development of the CAR project, in order to enhance its distinctive specificity √

GMP Solutions

- Complete the new OpenZone facility √
- Expand collaborations and activities for third parties, taking advantage of the increasing market demand √
- Continue implementing the automation process for cost containment and application to large patient populations

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