MOLMED

Leading the way in cell & gene therapy

BIT Small Cap Conference
Milan (Italy), November 29, 2016

From genes to therapy

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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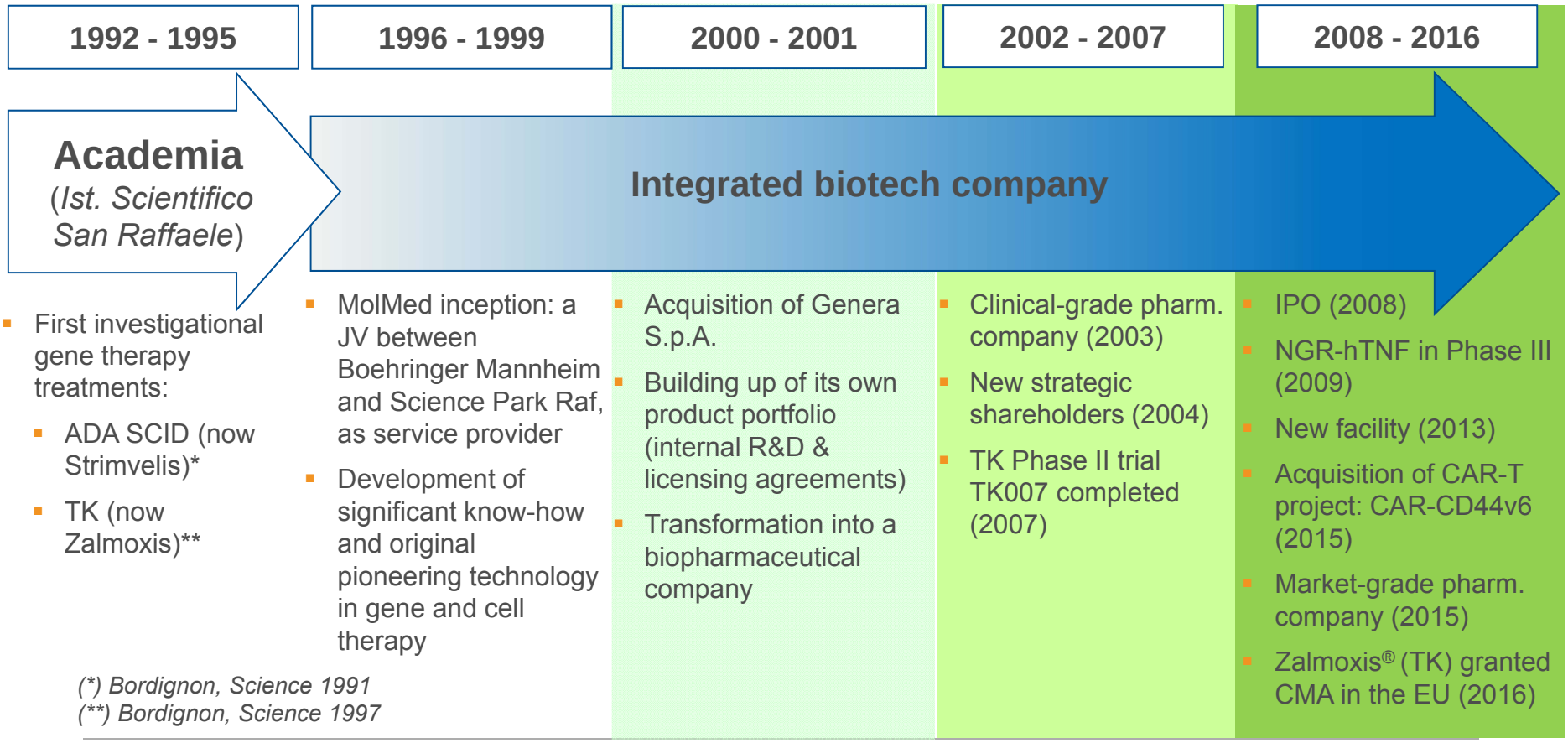
Declaration by the official Corporate Financial Reporting Manager:

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.

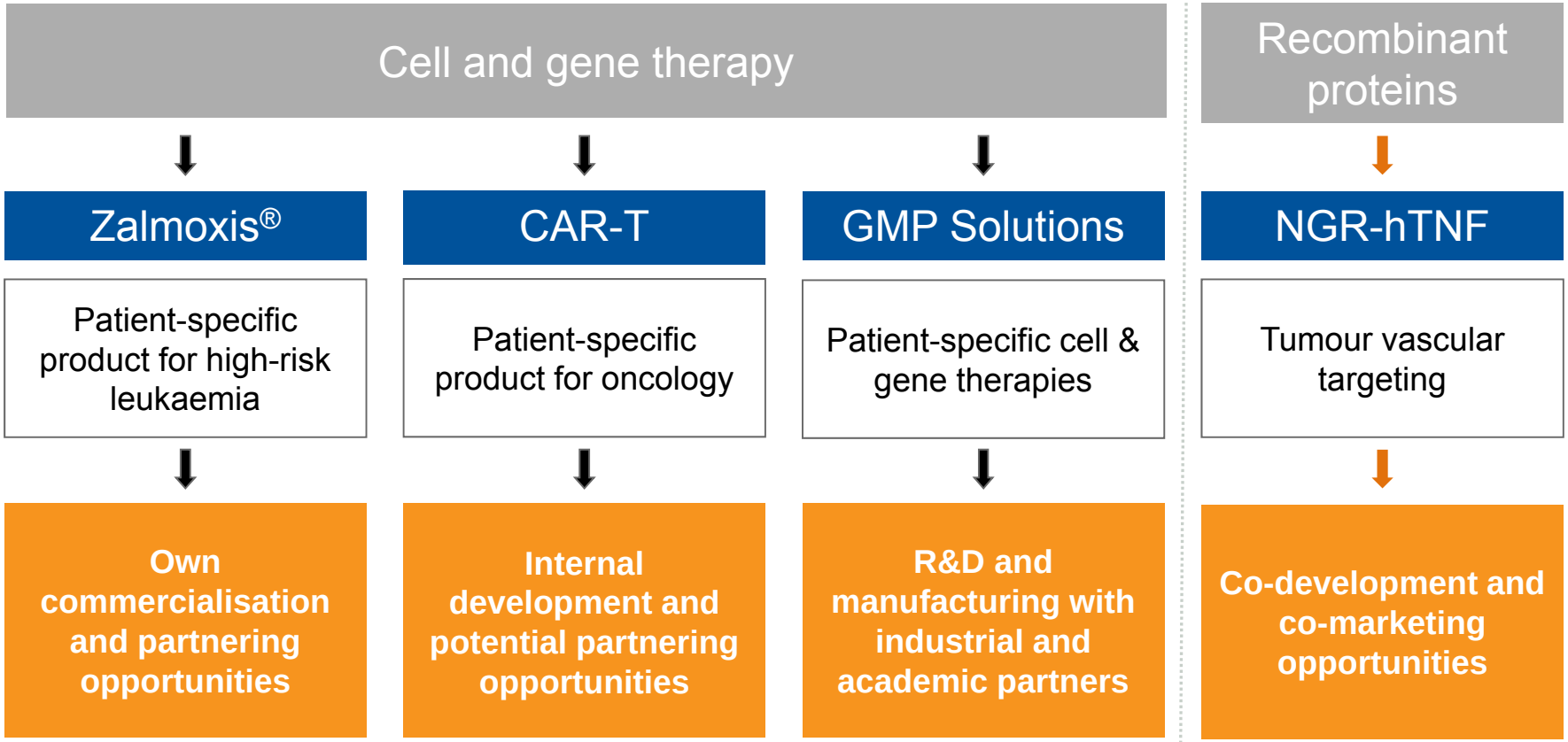
Andrea Quaglino, Chief Financial Officer, official Corporate Financial Reporting Manager

MolMed, from academia to public company



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

MolMed's technology platforms



A leading position in cell & gene therapy

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

Products and services in cell & gene therapy

Vector	*Product/ Therapy*	*Product development*	*Clinical stage manufacturing*	*Commercial development*	*Commercial manufacturing*
RV	Zalmoxis®	EC authorised			MOLMED
RV/LV	CAR-CD44v6	MOLMED			
RV	Strimvelis®	EC authorised			gsk
LV	MLD WAS			gsk	
LV	βThal MPS-I GLD CGD		iget		
---	DMD			telethon	
LV	MM	genenta science			

A new paradigm in immunogene therapy of hematological malignancies

- Authorised by EC for Conditional Marketing Authorisation (August 18, 2016) in haplo-identical haematopoietic stem-cell transplantation (HSCT) for adult patients with high-risk haematological malignancies
- Cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immunosuppression:
  - ✓ Inducing a rapid immune reconstitution associated with prolonged survival, regardless of disease status at transplant
  - ✓ Readily controlling Graft-versus-Host-Disease (GvHD) in almost 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) fully detailed into EPAR (soon available on EMA website) :
  - ✓ Halved non-relapse mortality, particularly due to infections
  - ✓ Increased overall survival
- 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

A breakthrough method to overcome GvHD, the most severe haplo-HSCT limitation

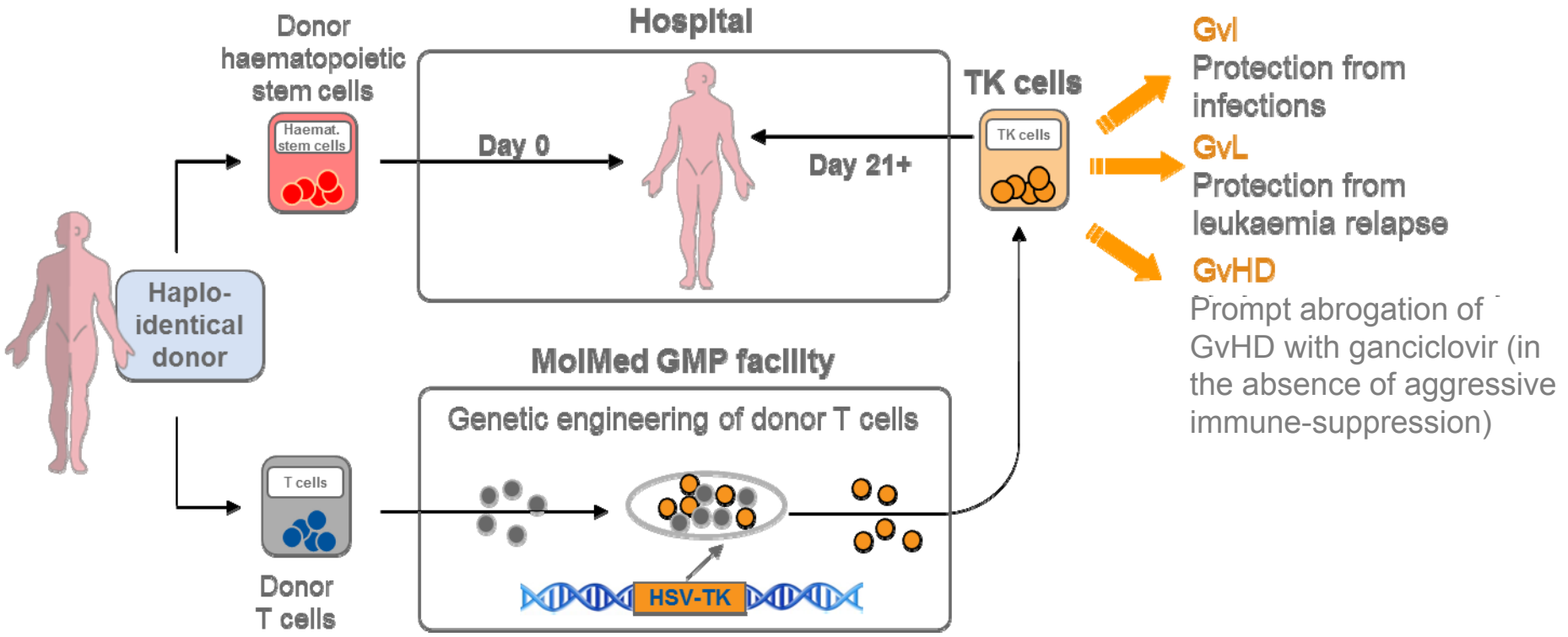
- Since donors and patients are not fully matched, there is a higher risk of graft-versus-host-disease (GvHD), which is the most severe adverse reaction occurring after the transplantation, caused by donor T cells

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

- Zalmoxis® 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

TK cells allow to preserve GvI and GvL effects…

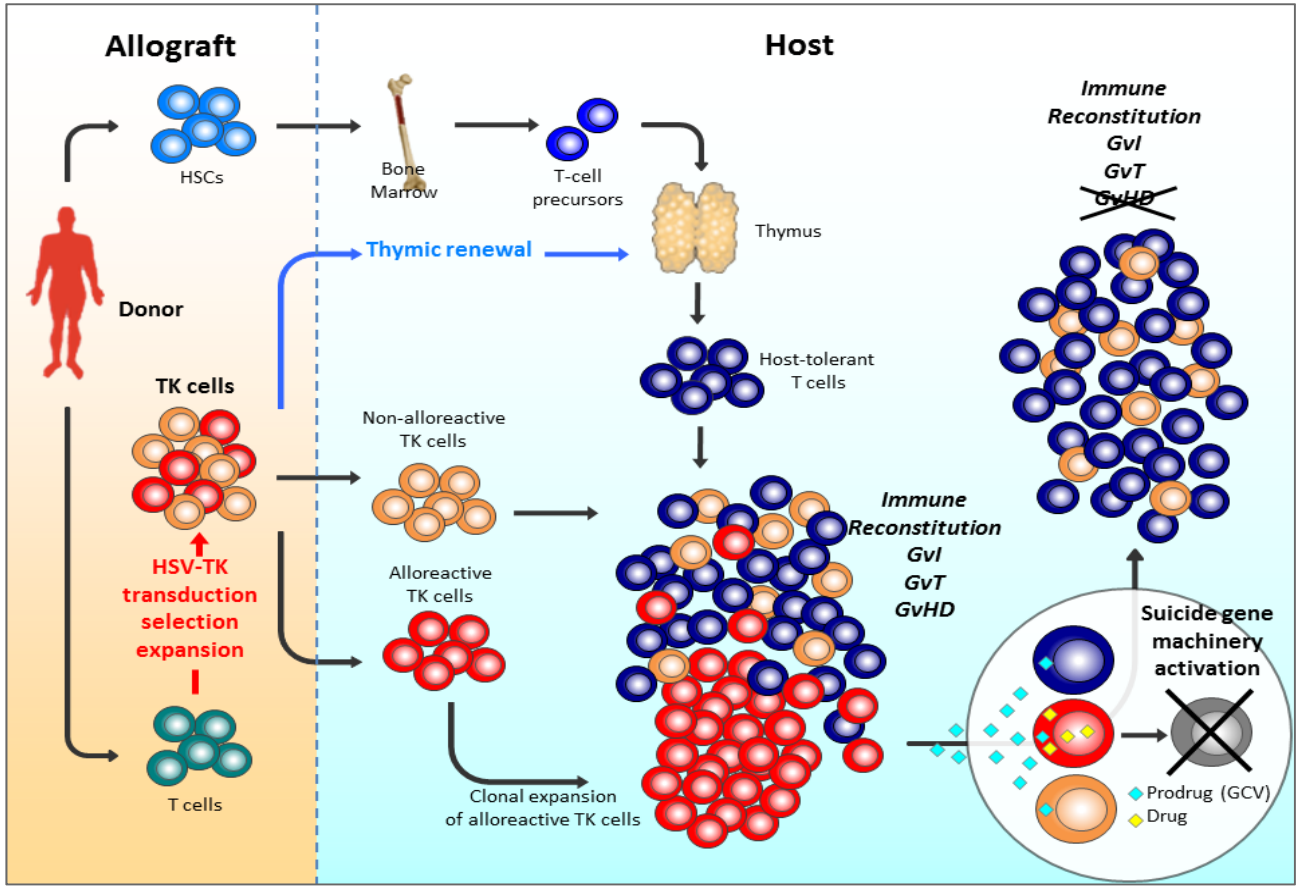
The TK haploidentical HSCT procedure makes a suitable donor available for any patient, without interfering with the timeframe of a normal transplantation



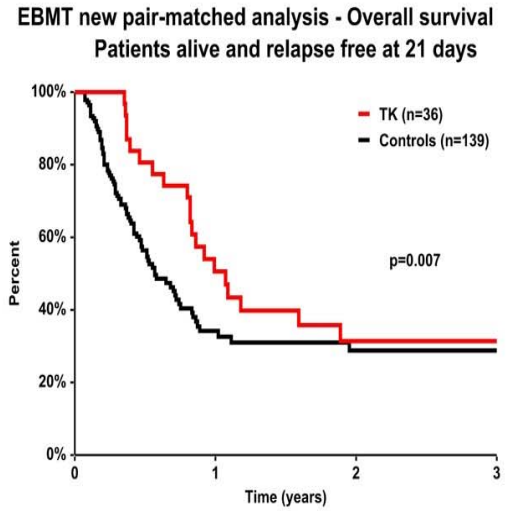
Bordignon, *Hum Gene Ther 1995;* Bonini, *Science 1997;* Bonini, *Nat Med 2003;* Traversari, *Blood 2007;* Ciceri, *Blood 2007;* Ciceri, *Lancet Oncol 2009*

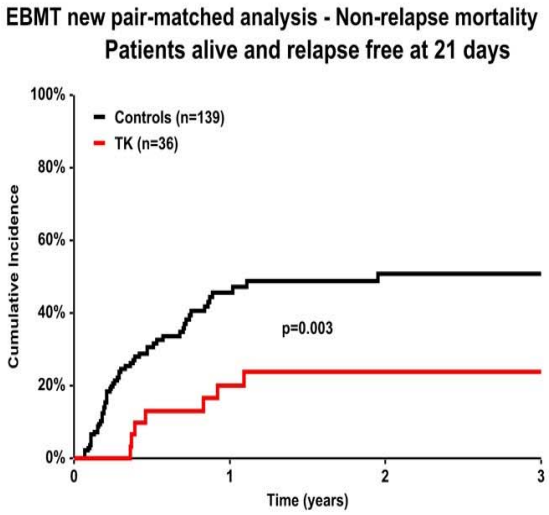
...while selectively controlling GvHD

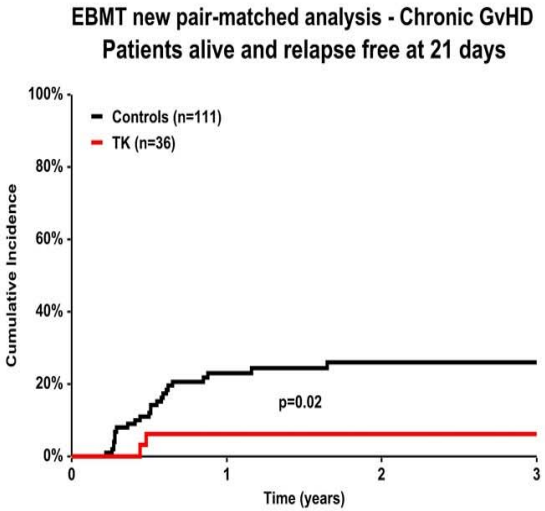
Ganciclovir is active only on proliferating TK cells



Clinical efficacy (EBMT pair-matched analysis): benefit in OS, NRM and chronic GvHD







New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	**46%**	**34%**	**23%**
Zalmoxis (n=36)	**20%**	**51%**	**6%**
p-value^	***0.003***	***0.007***	***0.02***

*Contemporaneous haploidentical transplants (period 2000-2013), including 36 Zalmoxis and 139 controls (70 T-cell replete and 69 T-cell depleted) were matched (1 to 4 ratio). 28 controls without information on cGvHD. *RI and NRM are competing risk events (when one competing event occurs, patients are no longer at risk for the other event, with those with shorter survival being less likely to develop relapse) and NRM events occur earlier than relapse events. ^Cox test stratified on match group (LFS and OS) and Gray test (RI, NRM and chronic GvHD)*

Market access process following CMA authorisation

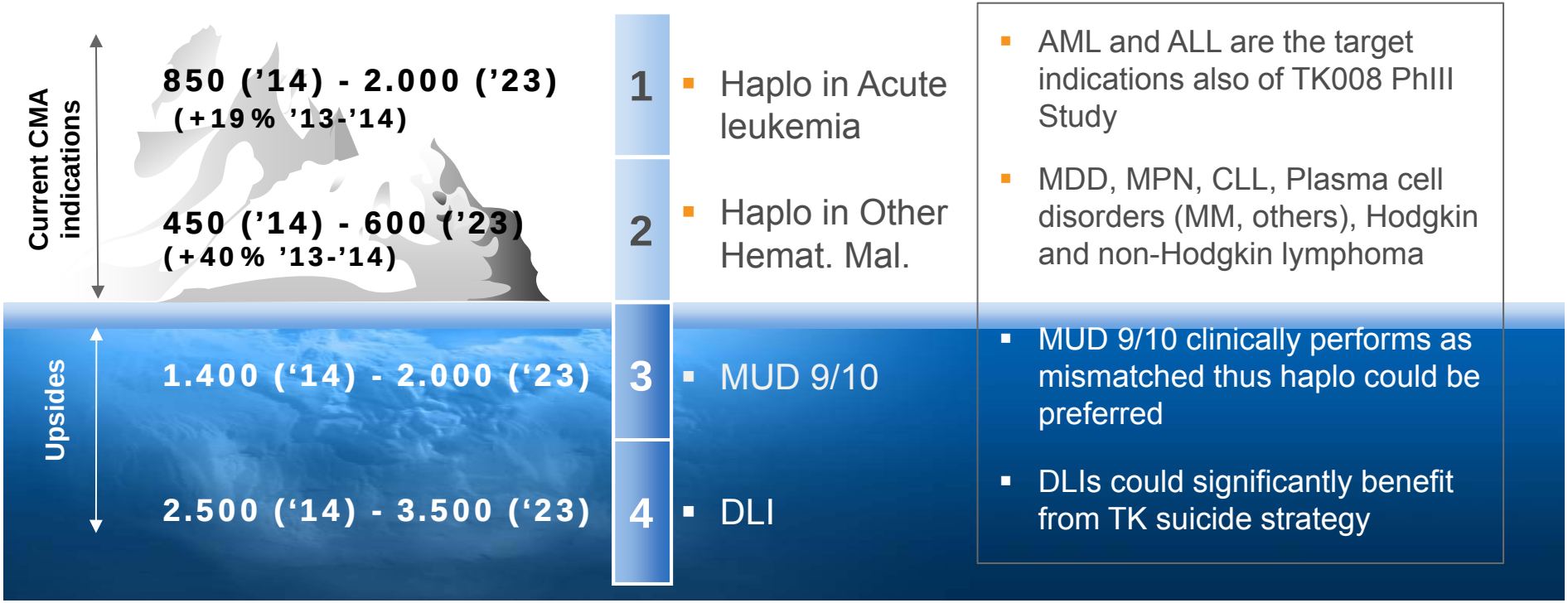
Implemented activities:

- Definition of European and National P&R strategy
- Identification of target pricing corridor
- Preparation of Core Value Dossier
- Submission of early access program in Italy
- Preliminary discussion with G-BA (DE)

Ongoing activities:

- Preparation of PE Model (UK)
- Preparation of P&R dossier in Italy
- AMNOG/NUB application in Germany
- Screening of French partners for preparation of French Dossier

European market potential analysis: strong growth and relevant upsides*



and

Most autologous and allogeneic CAR-T therapies may benefit from TK suicide gene machinery

** Source: Company and EBMT*

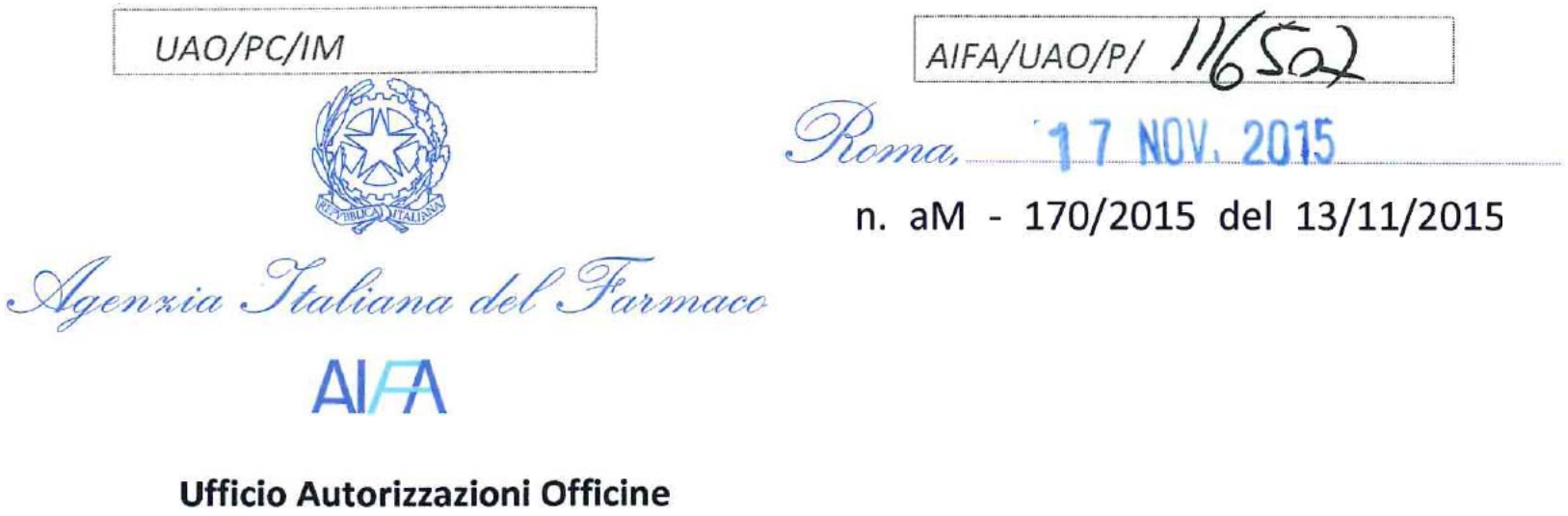
A new frontier of immunogene therapy for both haematological and solid tumors

- On April 13, 2015, MolMed significantly expanded its pipeline, entering one of the most promising fields of new anticancer strategies, tumour "immunogene 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
- MolMed's 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
- Potential toxicities might be managed by exploiting the combination of a suicide gene

A fully comprehensive and high level range of activities offered to 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

Excellence supported by a solid track record of GMP authorizations



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Roma, 17 NOV. 2015

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Agenzia Italiana del Farmaco

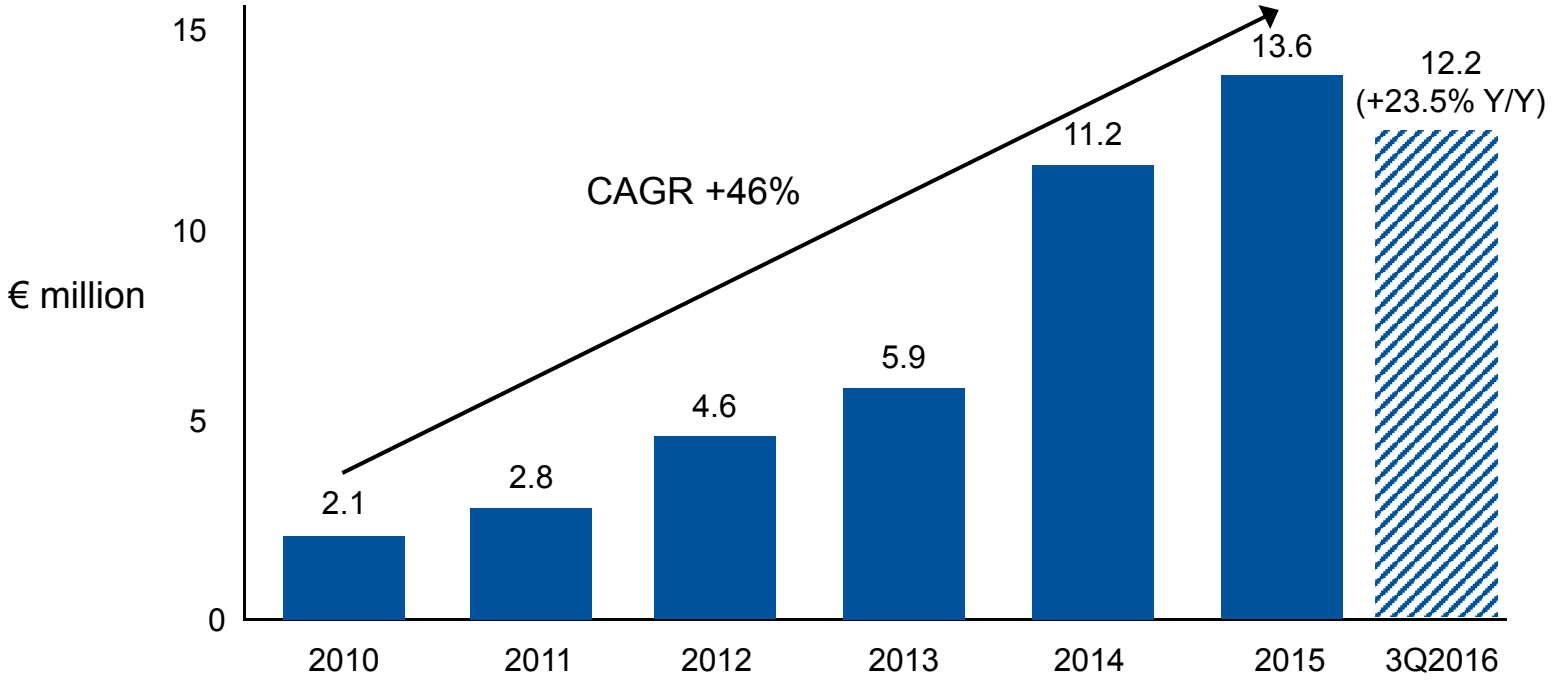
AIFA

Ufficio Autorizzazioni Officine

- Authorised GMP manufacturing facility since 2003 for **clinical programs**
  - Patient-specific manufacturing and production of critical reagents for cell & gene therapy
- Authorised GMP manufacturing facility since 2015 for the **market**
  - Zalmoxis®
  - Strimvelis®

The new MolMed facility at OpenZone in Bresso (Milan)

Significant revenues growth from development & manufacturing services and partnerships

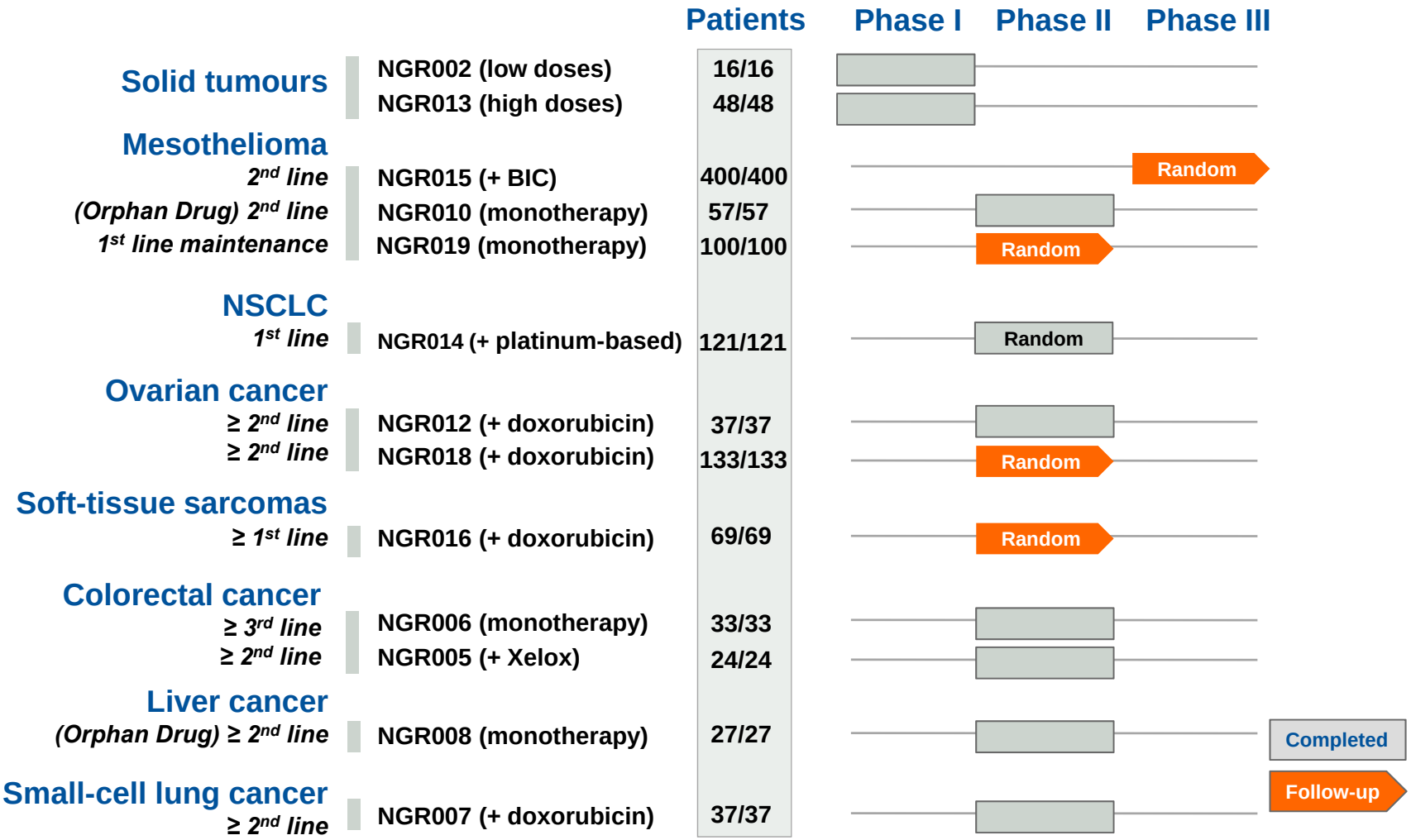


Year over Year	33%	66%	27%	91%	21%	23.5%

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)
- Recent non-binding discussions held with the European authorities confirm ground 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/US for high-risk mesothelioma patients as second-line treatment foreseen in Q4 2016
- Business strategy: co-development and co-marketing solutions

MolMed enrolled more than 1,000 patients in a comprehensive clinical development program



MolMed's analysis of NGR-hTNF market opportunity: a potential blockbuster

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)*

MolMed: key financials

Income Statement

(amounts in Euro thousand)	First 9 months 2016	First 9 months 2015	Δ %	FY 2015	FY 2014	Δ %
Operating revenues	13,901	10,321	34.7	16,764	12,422	35.0
Revenues from activites for third parties	*12,207*	*9,887*	*23.5*	*13,576*	*11,181*	*21.4*
Operating costs	28,010	26,564	5.4	37,302	25,050	48.90
Operating result	(14,109)	(16,243)	13.1	(20,538)	(12,628)	(62.6)
Net result	(14,266)	(16,475)	13.4	(20,784)	(13,003)	(59.8)

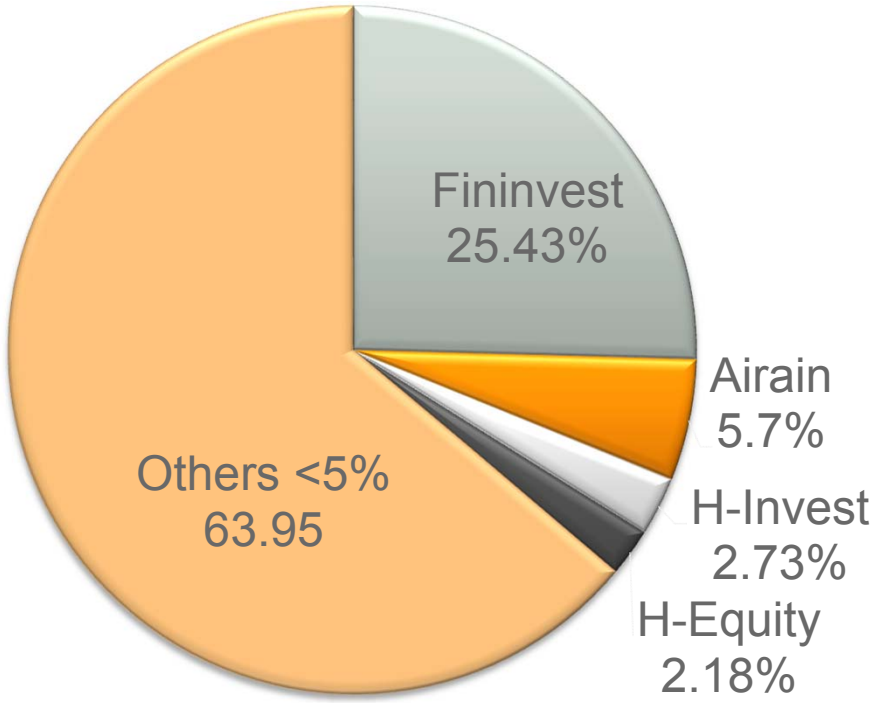
Net Financial Position

(amounts in Euro thousand)	Sep 30, 2016	Dec 31, 2015	Δ €	Δ %
Net Financial Position*	14,569	29,938	(15,369)	(51.34)

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

MolMed: shareholders' structure (November 7, 2016)

Market cap: ~139 M € (at November 22, 2016)



Unique position to capitalise on the momentum of the cell & gene therapy field

Leading technology platforms	▪ Two distinct innovative technology platforms: cell & gene therapy and recombinant protein
Entering the market with Zalmoxis	▪ Zalmoxis authorised for CMA in Europe
Late clinical stage compounds	▪ NGR-hTNF: extensive package of clinical data, proving efficacy and safety, from randomized controlled trials ▪ Significant opportunity from a partnering stand point
High potential program in immune-gene therapy field (CAR-T)	▪ CAR-CD44v6 in preclinical stage
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) ▪ Authorised 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

2016 priorities and achievements √

Zalmoxis®

- Obtain Conditional Marketing Authorisation in EU √
- Intensify activities preparatory to market access (both directly and through distributors/dealers) √

CAR-T

- Advance research and pre-clinical development, in order to enhance its distinctive specificity √
- Preliminary outcomes in preclinical studies (at ASH 2016) √

GMP Solutions

- Complete the new OpenZone facility √
- AIFA authorisation process started √
- First AIFA authorisations gradually expected by end 2016
- Expand collaborations and activities for third parties, taking advantage of the increasing market demand √

NGR-hTNF

- Complete optimisation of market-compliant manufacturing process √
- Submit a CMA application for high-risk mesothelioma indication in late 2016 *(non-binding consultations with the European regulatory authorities, to assess NGR-hTNF eligibility for a conditional marketing authorisation request √)*
- Find a co-development partner to exploit the huge clinical potential

Contacts

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