MOLMED

Leading the way in cell & gene therapy

March, 2017

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.

The Company assumes no responsibility to update forward-looking statements or adapt them to future events or developments.

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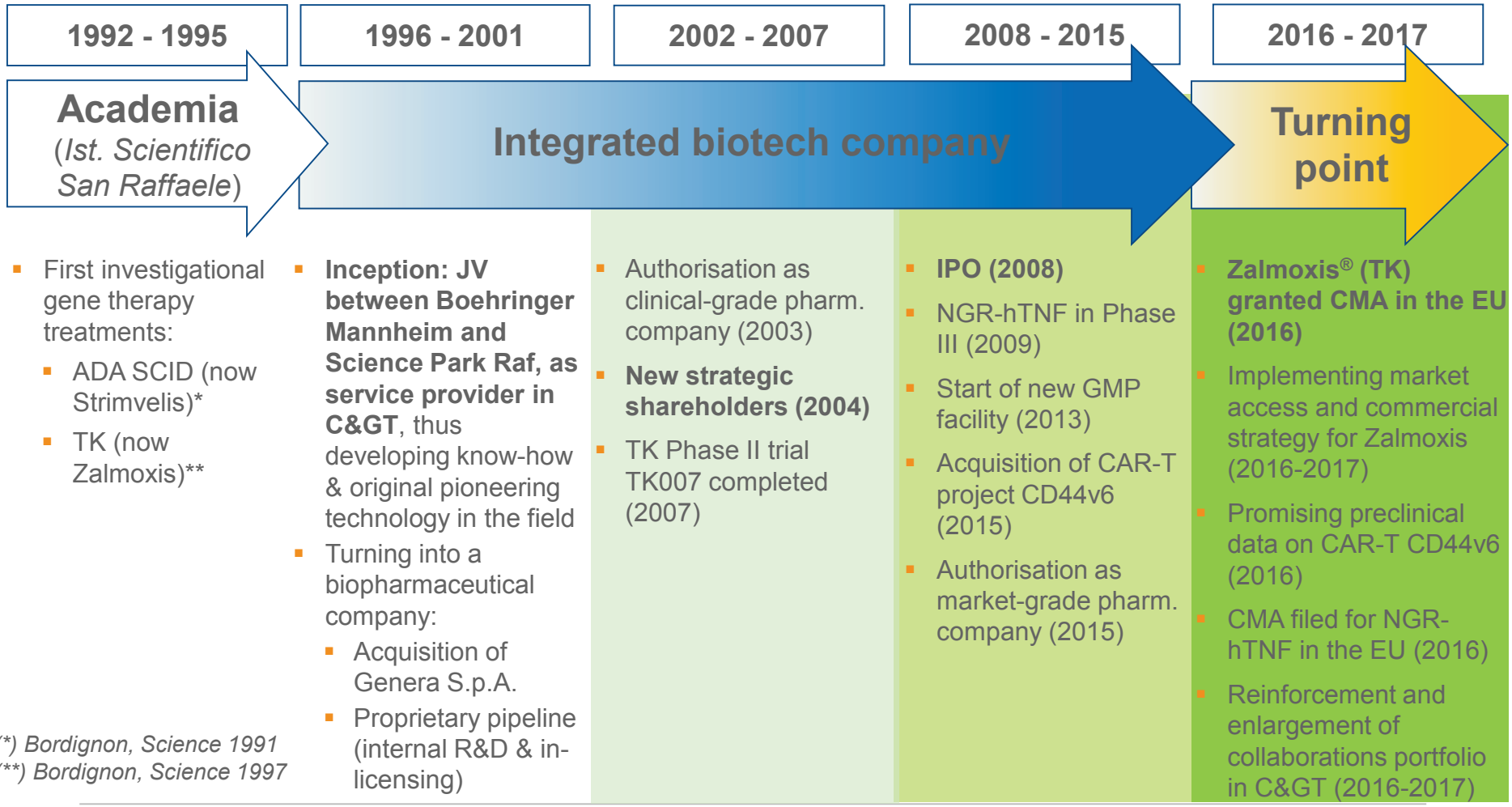
Declaration by the official Corporate Financial Reporting Manager:

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.

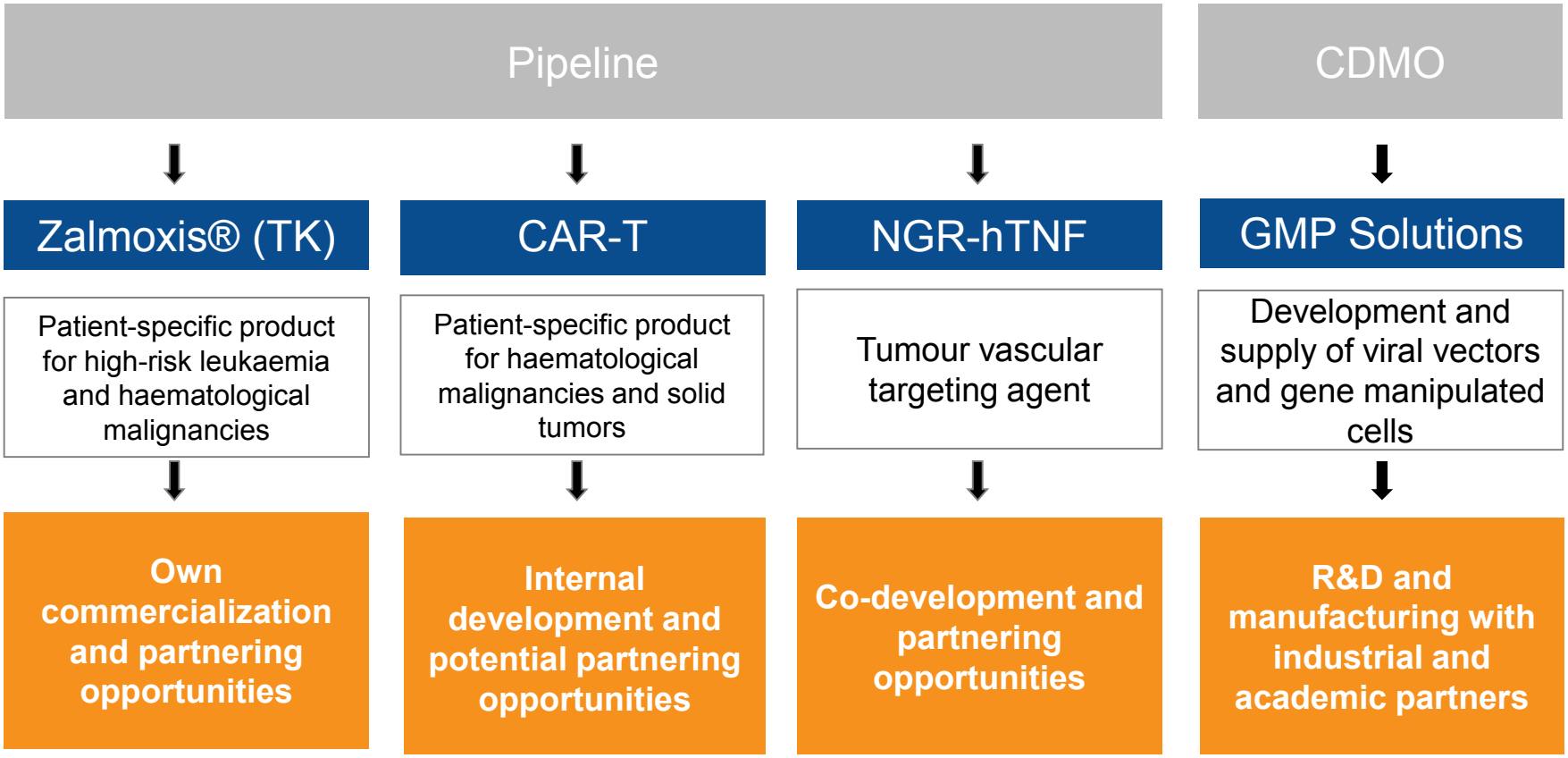
Andrea Quaglino, Chief Financial Officer, official Corporate Financial Reporting Manager

MolMed, from academia to public company and beyond



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

Dual business model in the cell and gene therapy area



A breakthrough method to overcome open limitations in HSCT

…"*still some unmet medical need when it comes to solve this limitations…*"

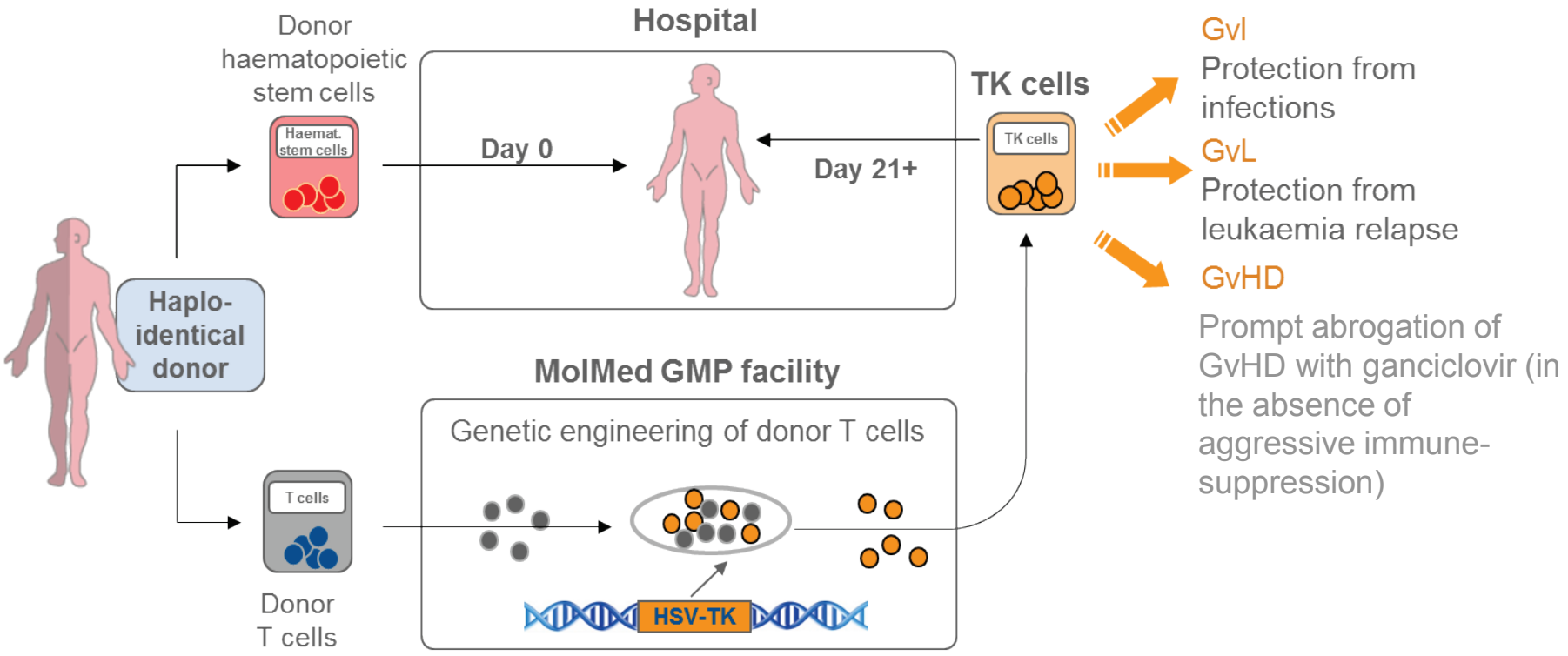
1. Delayed immune-reconstitution
2. Higher incidence of opportunistic infections
3. Higher incidence of GvHD

Prof. Mohamad Mohty (EBMT President) at the 58th Annual Meeting of the American Society of Hematology (ASH): "*HSV-TK cells as adjunctive treatment for haplo-HSCT*" (https://www.youtube.com/watch?v=E2x3AUN4zLs)

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 medical need

TK cells allow to preserve GvI and GvL effects…

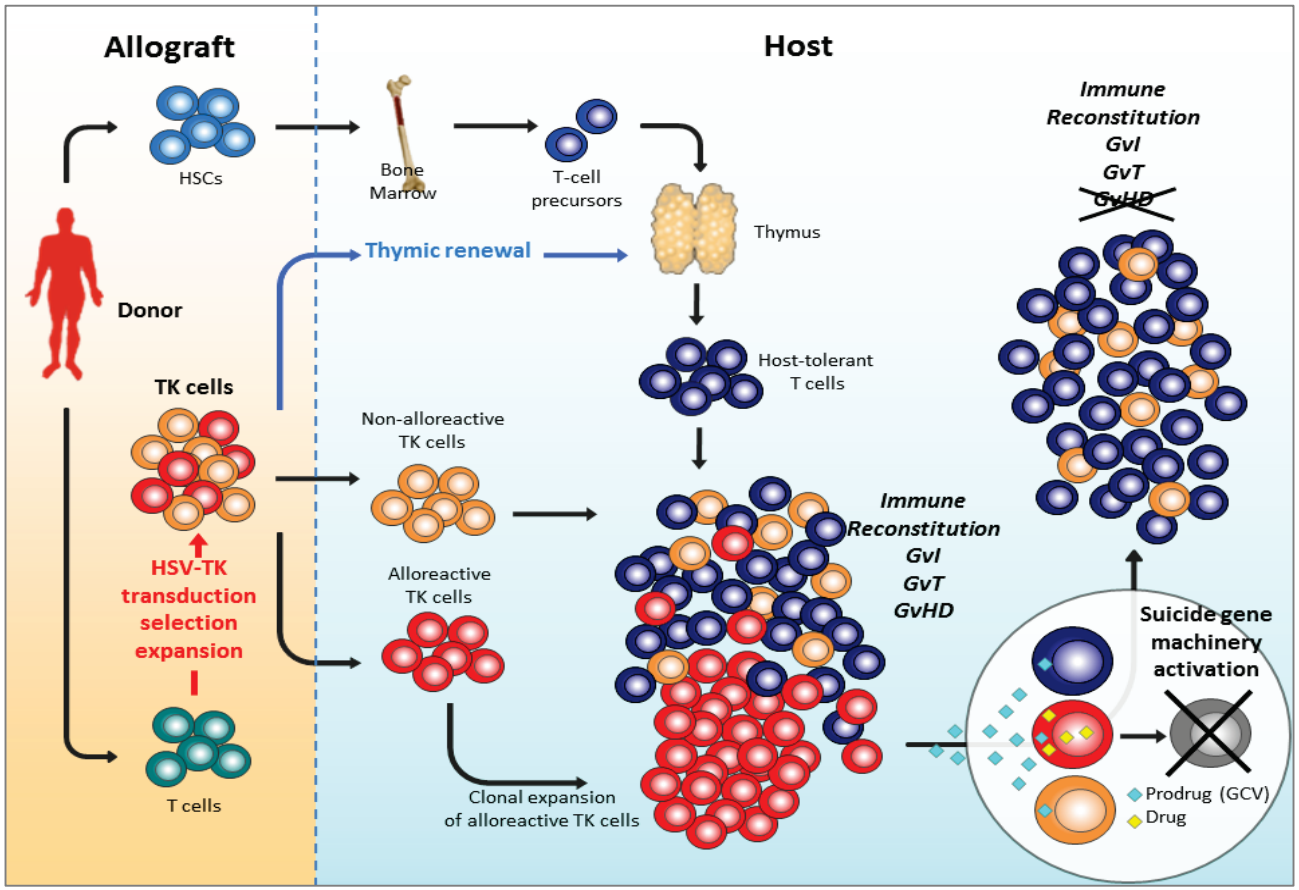
The TK haploidentical HSCT procedure makes a suitable donor available for any patient, without interfering with the timeframe of a normal transplantation



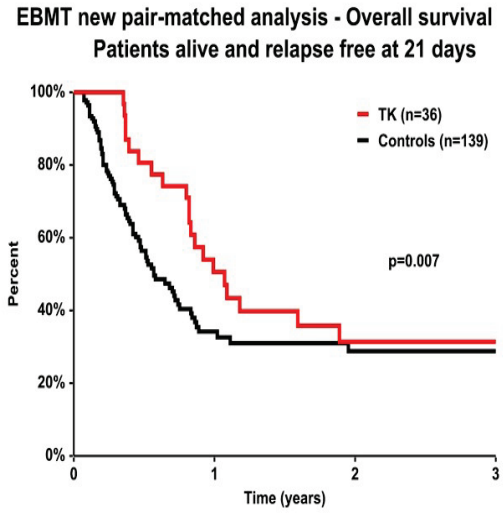
Bordignon, *Hum Gene Ther 1995;* Bonini, *Science 1997;* Bonini, *Nat Med 2003;* Traversari, *Blood 2007;* Ciceri, *Blood 2007;* Ciceri, *Lancet Oncol 2009*

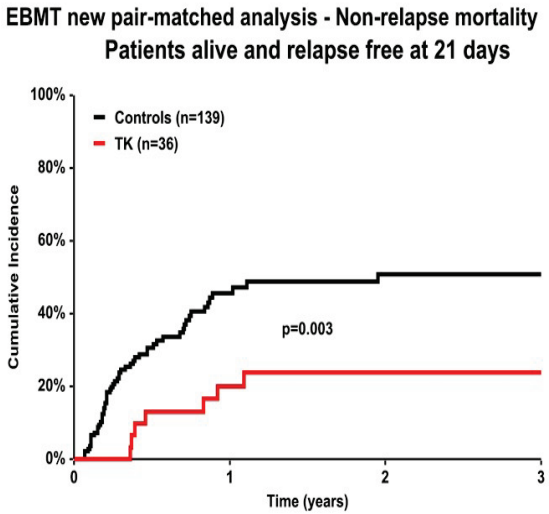
...while selectively controlling GvHD

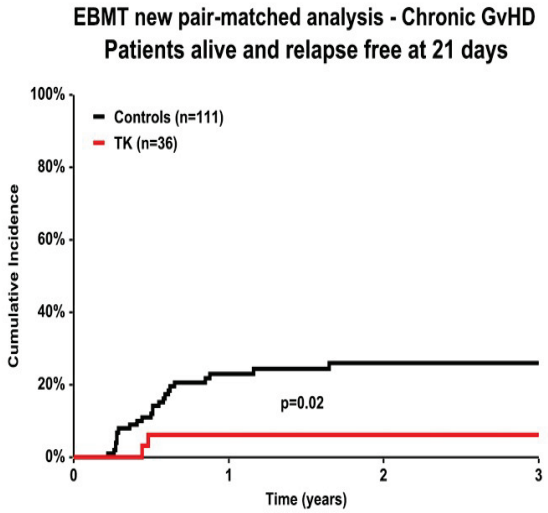
Ganciclovir is active only on proliferating TK cells



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







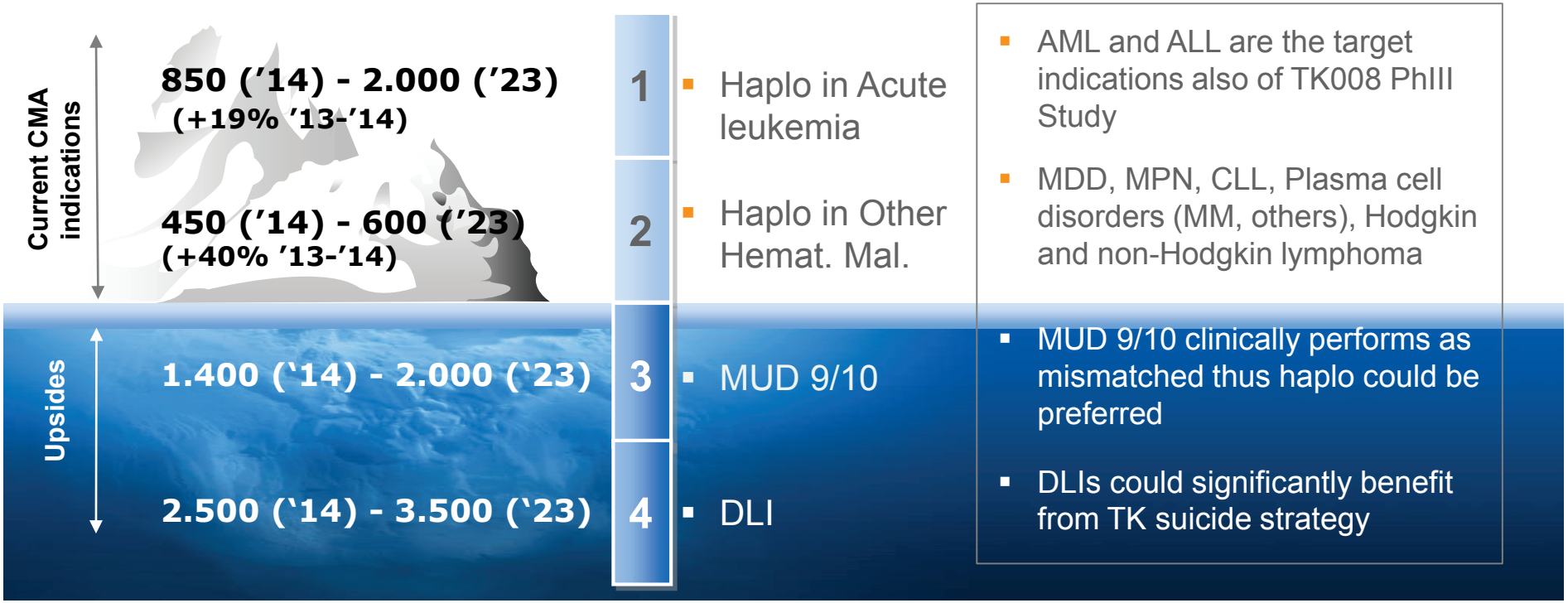
New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	**46%**	**34%**	**23%**
Zalmoxis (n=36)	**20%**	**51%**	**6%**
p-value^	***0.003***	***0.007***	***0.02***

*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)*

A new paradigm in immunogene therapy of haematological malignancies

- Authorised for Conditional Marketing Authorisation in the EU in 2016, for use in combination with haplo-identical haematopoietic stem-cell transplants (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 (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

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*

Market access process following CMA authorisation

Implemented activities:

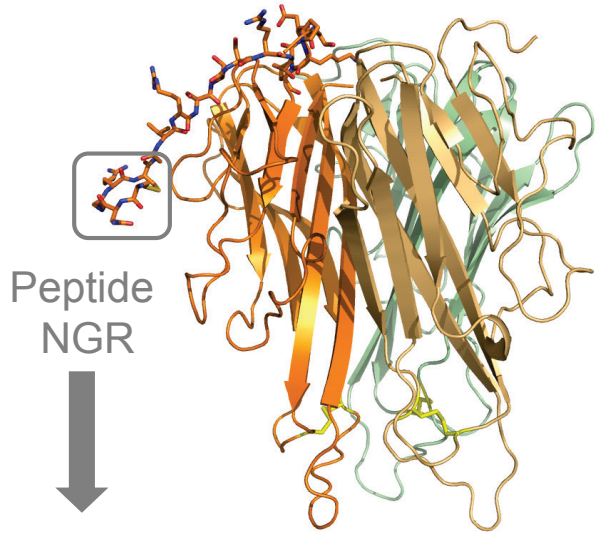
- Preparation of Core Value Dossier
- Definition of European and National P&R strategy
- Identification of target pricing corridor
- Submission of early access program in Italy
- Preliminary discussion with G-BA (DE)
- Submission of P&R dossier in Italy
- Entered into a term sheet to commercialise Zalmoxis® in Israel (Megapharm) and in some Asian territories (TTY Biopharm)

Ongoing activities:

- Preparation of PE Model (UK)
- AMNOG application in Germany
- Preparation of Transparency Committee dossier, pricing declaration and T2A exclusion list in France

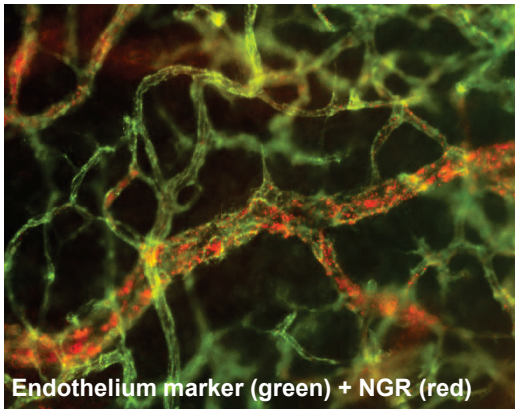
NGR-hTNF: a safe and selective vascular targeting agent in clinical phase II and III in EU and US

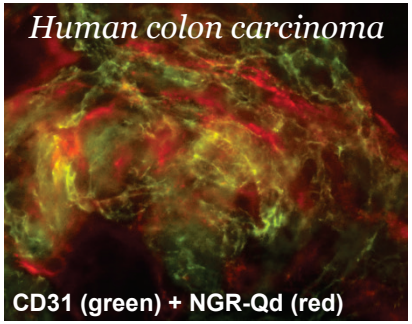
Recombinant fusion protein

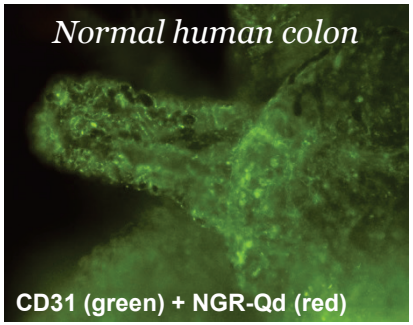


Doses of 0.8 µg/sqm systematically show antitumour activity

Specific NGR binding to tumour blood vessels



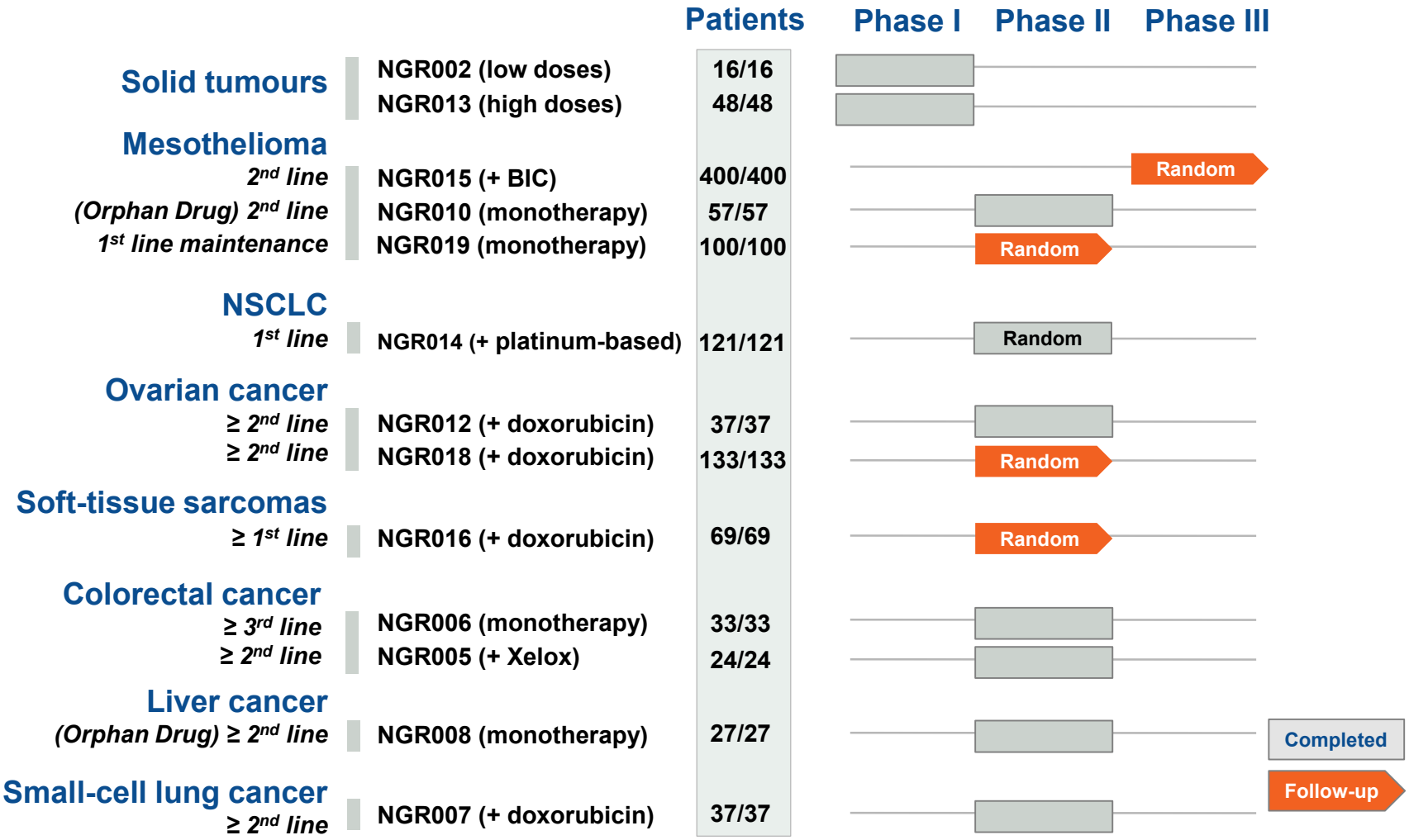




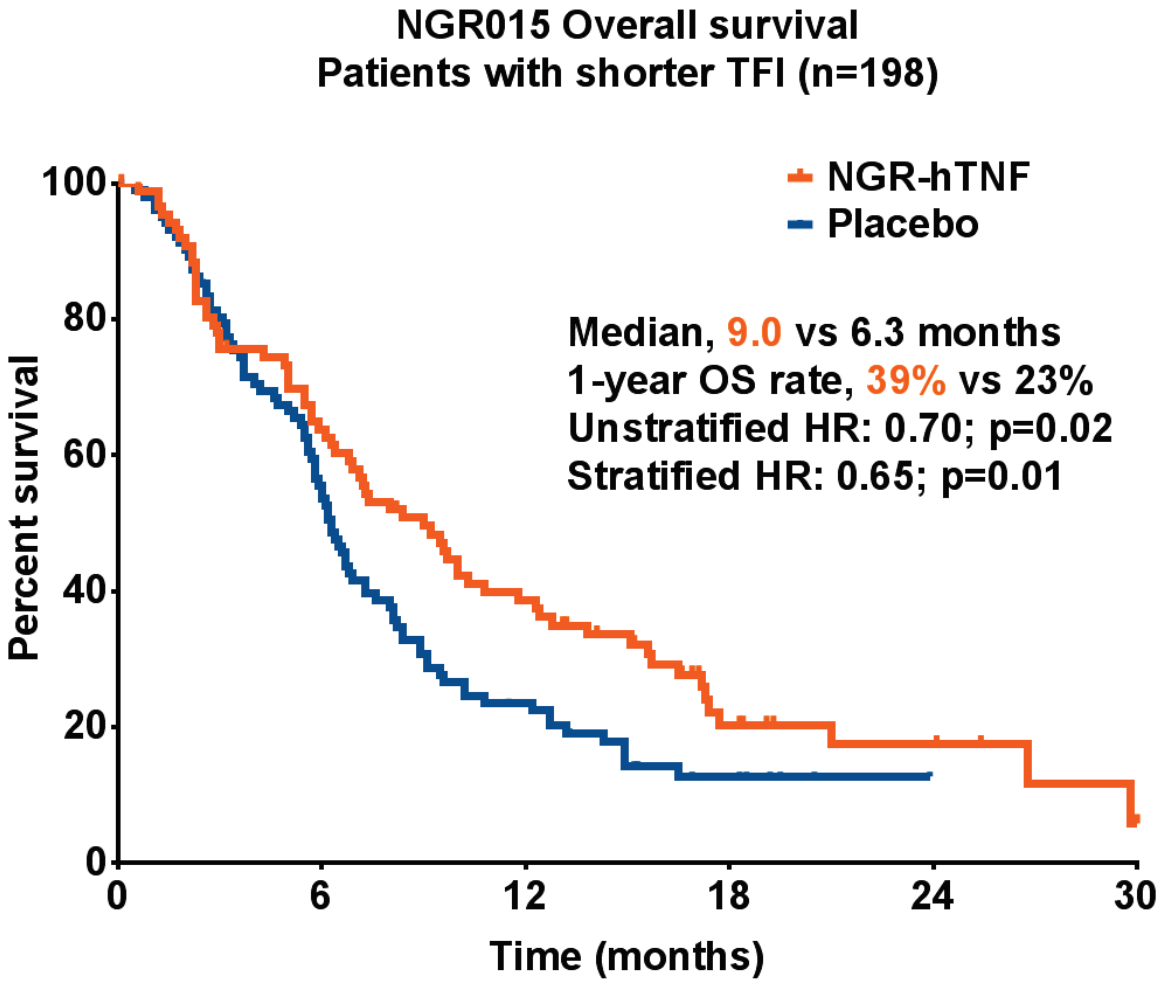
Whole mount analysis of tissues obtained from the same patient (N=3)

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

Comprehensive clinical development program in several oncology indication



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



A high potential vascular targeting agent in late stage development

- Statistically significant efficacy data from randomised 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)
- CMA request filed with and validated by the EMA in December 2016, as second-line treatment of malignant pleural mesothelioma
  - 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
- Business strategy: co-development and co-marketing solutions to fully explore and exploit its broad therapeutic potential in many solid tumours

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

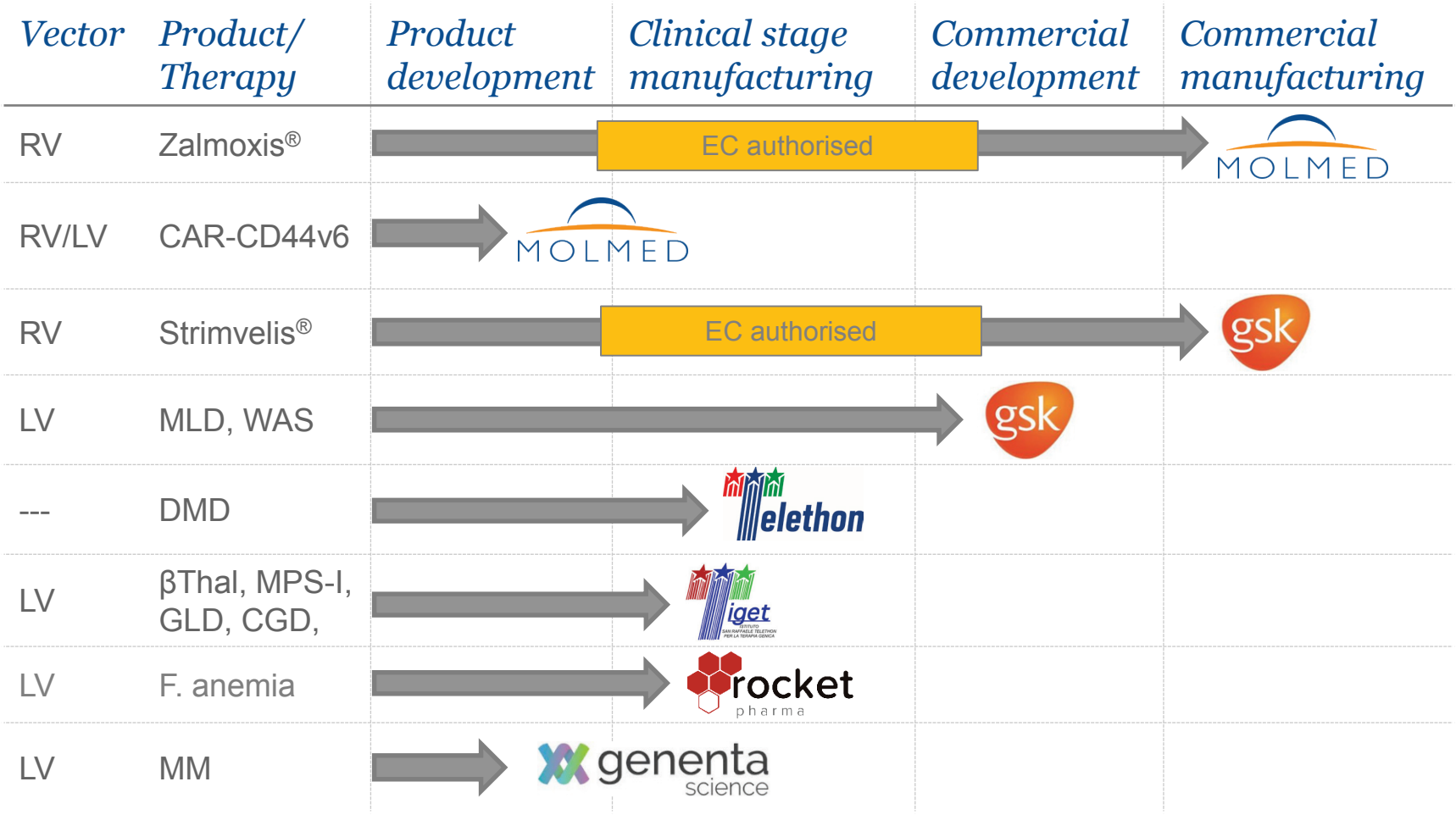
Indications	*Clinical phase*	*Incidence* (EU27, USA, CA)*	*Incidence* (CN, JP, KR)*
Pleural Mesothelioma First line - Maintenance	II	8'300	3'000
Pleural Mesothelioma Second line	III	5'800	2'100
Sarcomas	II	a blockbuster potential	
Ovarian carcinoma Platinum-resistant	II		
Liver carcinoma Sorafenib-resistant	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's project in the most promising field of immune-gene therapy of cancer

- On April 13, 2015, MolMed significantly expanded its pipeline by purchasing the project CAR-CD44v6 from the San Raffaele Hospital
- MolMed's CAR-CD44v6 is specific for variant v6 of antigen CD44, 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
- Possible combination with suicide gene system
- Preclinical outcomes presented at ASH (December 5, 2016) highlighted very promising features in leukaemia and solid tumors
- MolMed is coordinator of EURE-CART, a R&D project on CAR-CD44v6, awarded a € 5.9 million grant by the European Commission within Horizon 2020

Products and services in cell & gene therapy



Strimvelis™, the first ex vivo gene therapy approved by EMA

MOLMED

PRESS RELEASE

The European Commission grants the European marketing authorization 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.

…

GSK's treatment for ADA-SCID patients is the tangible and encouraging result of the strategic collaborations existing between GSK, the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET) and MolMed. Actually, MolMed previously produced on behalf of Fondazione Telethon the investigational gene therapy where the correct form of the ADA gene is inserted into the patients' own bone marrow derived stem cells. Since 2010, GSK took the responsibility of the clinical development of the ADA-SCID gene therapy, in collaboration with HSR-TIGET, from which they in-licensed the rights to develop and commercialize the therapy, and with MolMed for the manufacturing process optimization, standardization and characterization, as well as for the drug product supply intended to be used for compassionate treatment of patients, accordingly with agreements signed in 2011 and 2013. Then, as a capping stone of this successful collaboration, by means of the agreement signed in March 2015 and of the authorization gained in December of last year, MolMed will produce Strimvelis for commercialization.

Reviewed strategic agreement with GSK: increased minimum anticipate revenues

MOLMED

PRESS RELEASE

MolMed future revenues will benefit from a review of the strategic agreement with GlaxoSmithKline, increasing minimum anticipated revenues from € 34 to € 48 million

Milan, September 1, 2016 – MolMed S.p.A. (MLM.MI) announces an amendment of the strategic agreement signed with GlaxoSmithKline (GSK) on March 19th, 2015, concerning MolMed's supply of development, manufacturing and technology transfer services for the clinical application of gene therapies based on viral vector cellular transduction until March 31, 2020.
Based on the successful, long lasting collaboration between MolMed and GSK, resulting in an increased demand of MolMed's resources necessary for GMP manufacturing of cell and gene therapies for the GSK programs, on the 1st of September MolMed and GSK amended and restated the original strategic agreement, to reflect the additional resources to be applied to the GSK programs and costs related thereto.
Under the terms of this amendment, MolMed will be eligible, through the 5 years-period covered by the contract, for a minimum anticipated of € 48 million (respect to prior € 34 million total of upfront payments, milestones, services and supply), of which around € 14 million have already been received to date

Excellence supported by a solid track record of GMP authorisations for its historical facility


UAO/PC/IM

AIFA/UAO/P/ 11652

Roma, 17 NOV. 2015

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

Agenzia Italiana del Farmaco

AIFA

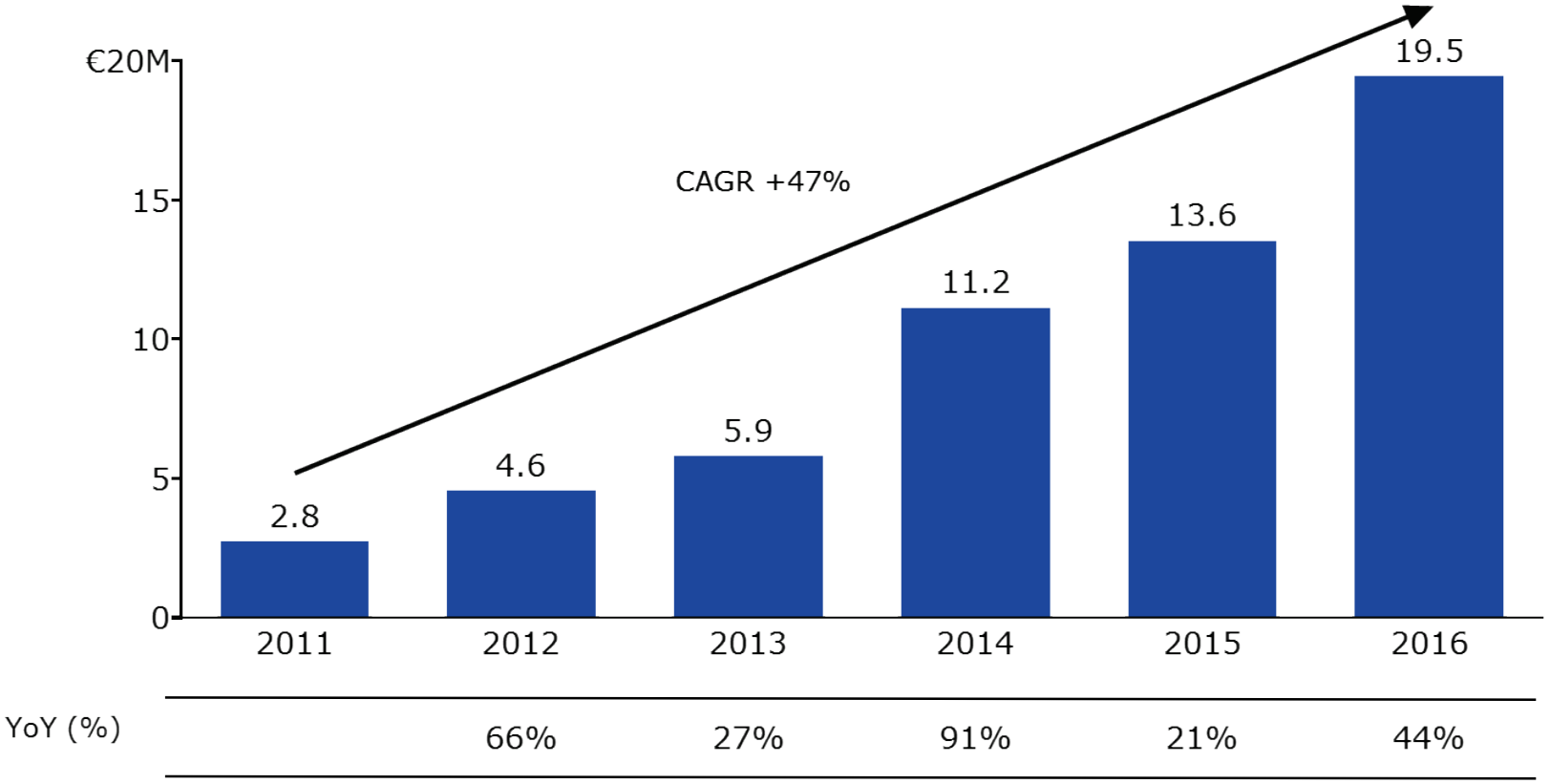
Ufficio Autorizzazioni Officine

- 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)

- Manufacturing capacity enhanced by 3,300 square meters, tripling the current one
- Additional 21 clean rooms will be gradually made operational and authorized starting from second half 2016
- Quality Control and Development laboratories completely equipped
- Quality Control, Materials and Products Storage areas authorized by AIFA

Significant revenues growth from development & manufacturing services and partnerships



Key financials – FY 2016

Key income Statements

(amounts in € thousand)	FY 2016	FY 2015	Variation	
			€	%
Operating revenues	22,825	16,764	6,061	36.2
▪ From activities for third parties	19,484	13,576	5,908	43.5
Operating costs	36,411	37,302	(891)	(2.4)
Operating result	(13,586)	(20,538)	6,952	33.8
Net financial income & charges	(290)	(246)	(44)	(17.9)
Result for the period	(13,876)	(20,784)	6,908	33.2

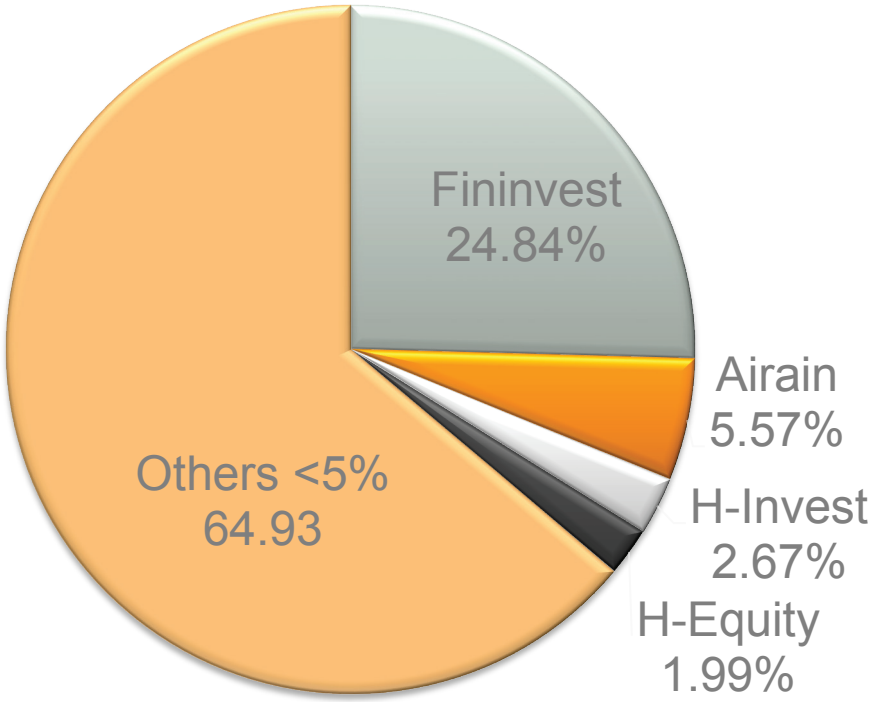
Net Financial Position

(amounts in € thousand)	Dec 31, 2016	Dec 31, 2015	Variation €
Net Financial Position∗	19,702	29,938	(10,236)

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

MolMed's shareholders' structure

Market cap: ~185.0 M € (at March 6, 2017)



2016 achievements

Zalmoxis®

- Conditional Marketing Authorisation granted in the EU
- Term sheet with Megapharm to commercialise Zalmoxis® in Israel

NGR-hTNF

- Optimisation of market-compliant manufacturing process completed
- CMA application filed in the EU for the treatment of adult patients with rapidly progressing advanced malignant pleural mesothelioma

CAR-T CD44v6

- Promising preclinical data in both leukaemia and lung adenocarcinoma
- Grant of € 5.9 million EU funding to the EURE-CART project

GMP Solutions

- New collaboration agreement with Genenta on gene therapy for multiple myeloma
- Expansion of strategic agreement with GSK, with total minimum expected revenues increased from 34 to 48 million €
- New GMP facility at OpenZone completed

Priorities for 2017

Zalmoxis®

- Enter the first EU market
- Final licensing agreements with Megapharm (Israel) and TTY (some Asian countries)
- Partners for Zalmoxis® commercialisation in other EU and extra-EU areas

NGR-hTNF

- Pursue CMA application filed in the EU for the treatment of adult patients with rapidly progressing advanced malignant pleural mesothelioma
- Find a co-development partner to fully explore and exploit the huge clinical potential

CAR-T CD44v6

- Advance EURE-CART project, focused on leukaemia and multiple myeloma
- Advance in preclinical studies to confirm potential in solid tumours

GMP Solutions

- New collaboration agreements in cell&gene therapy:
  - ✓ Rocket Pharma, for Fanconi's anemia ☑
  - ✓ …
- Gradual entry into operation of the new GMP facility

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