MOLMED

Leading the way in cell & gene therapy

October, 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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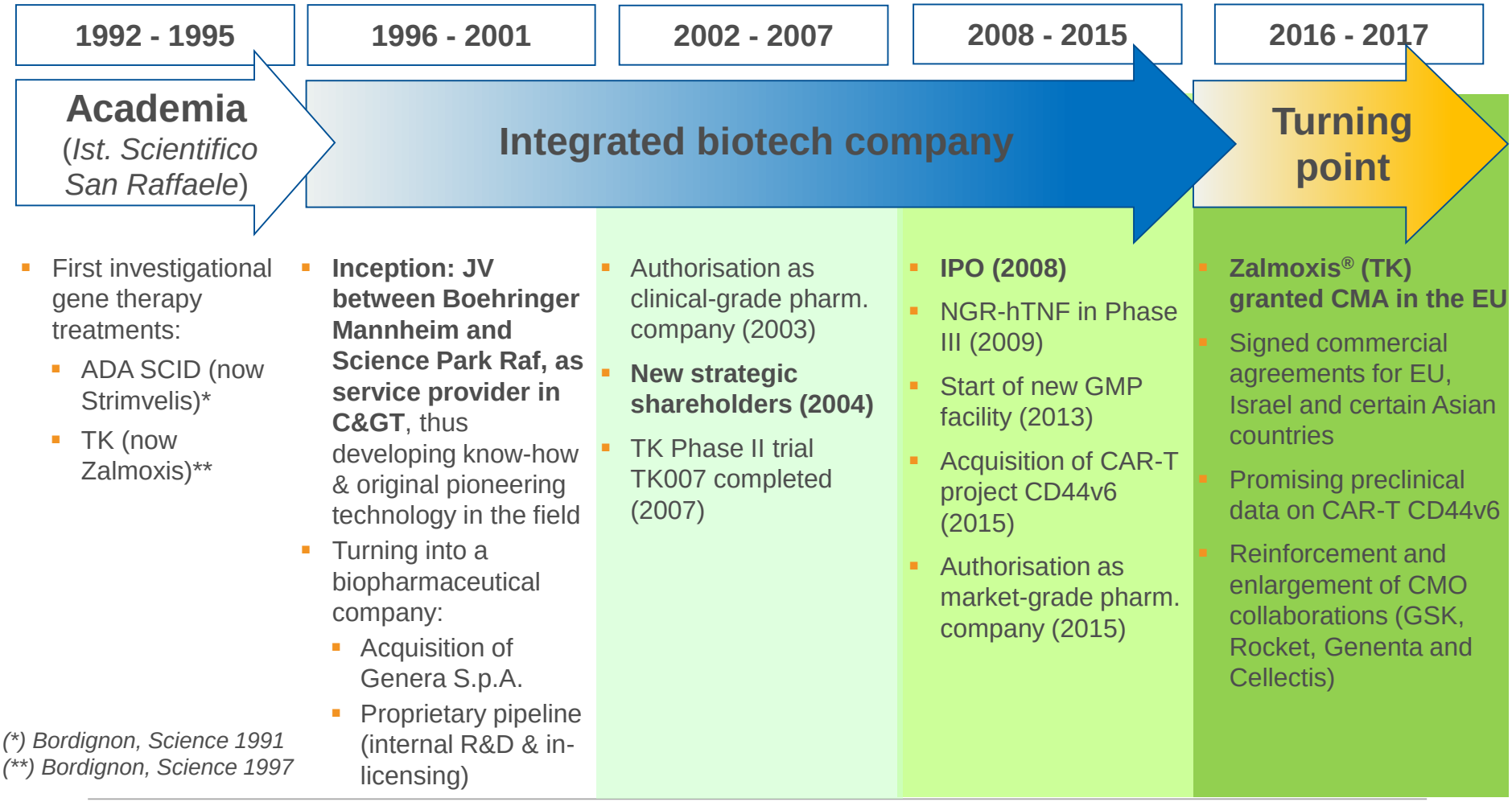
Declaration by the official Corporate Financial Reporting Manager:

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 & gene field leveraging common technological assets

Common Assets

- **20 year experience** in cell&gene therapy development and manufacturing
- **2 GMP manufacturing facilities** of almost 5.000SQM (55.000SQF)
- **>150 highly qualified scientist** and operators plus ~40 support staff
- Manufacturing of the **only 2** *ex-vivo* gene therapy products **approved in EU**
- 12 proprietary patent families including 256 granted patents and 43 pending applications
- Wide network of collaborators

CDMO

Product/ Therapy	*Partner*	*Dev.*	*PhI/II*	*PhIII*	*Com.*
Strimvelis™	gsk				
MLD WAS					
BTHAL					
MPS GLD CGD	telethon				
IFN	genenta science				
FA	rocket pharma				
Undiscl.	celectis				

Pipeline

Product/ Therapy	*Partner*	*Disc/ Feas*	*Precl*	*PhI/II*	*PhIII*	*Com.*
Zalmoxis®						
CAR-CD44v6 Hae. Mal	MOLMED					
CAR-CD44v6 Solid Tum	MOLMED					

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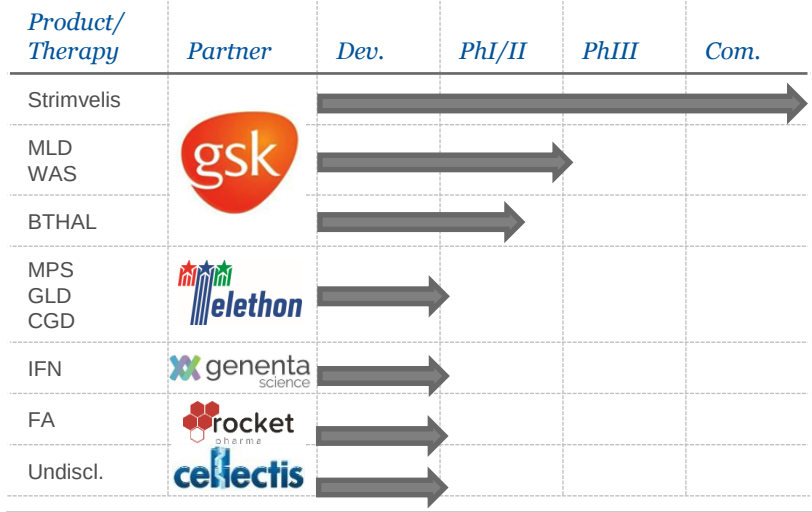


Pipeline

Partnerships in cell & gene field

Year	*Partner*	*Product/ Therapy*	*Collaboration*
2010	gsk	Strimvelis MLD WAS	The partnership was established in 2010 and in 2016 the companies emended the agreement increasing the minimum guaranteed payment to **48M€**. MolMed manufactured RV/LV and transduces HSC for the clinical programs (MLD and WAS) and for the **commercially approved** Strimvelis.
2015	telethon	BTHAL MPS GLD CGD	The long lasting partnership was reinforced in the last years (2015 and 2016) to cover the development and manufacturing of vectors and transduced cells for the clinical programs of **several genetic diseases**
2016	genenta science	IFN	Since 2016 MolMed support the development and manufacturing of **Genenta's leading program** on hematological malignancies part of an agreement with Amgen
2017	rocket pharma	FA	Since Q12017 MolMed support the US based Rocket Pharma for the development and manufacturing of the **Fanconi Anemia** program
2017	cellectis	UCART	Since July 2017 MolMed support Cellectis for the development and manufacturing of Cellectis **UCART programs**

Excellence supported by a solid GMP manufacturing capacity

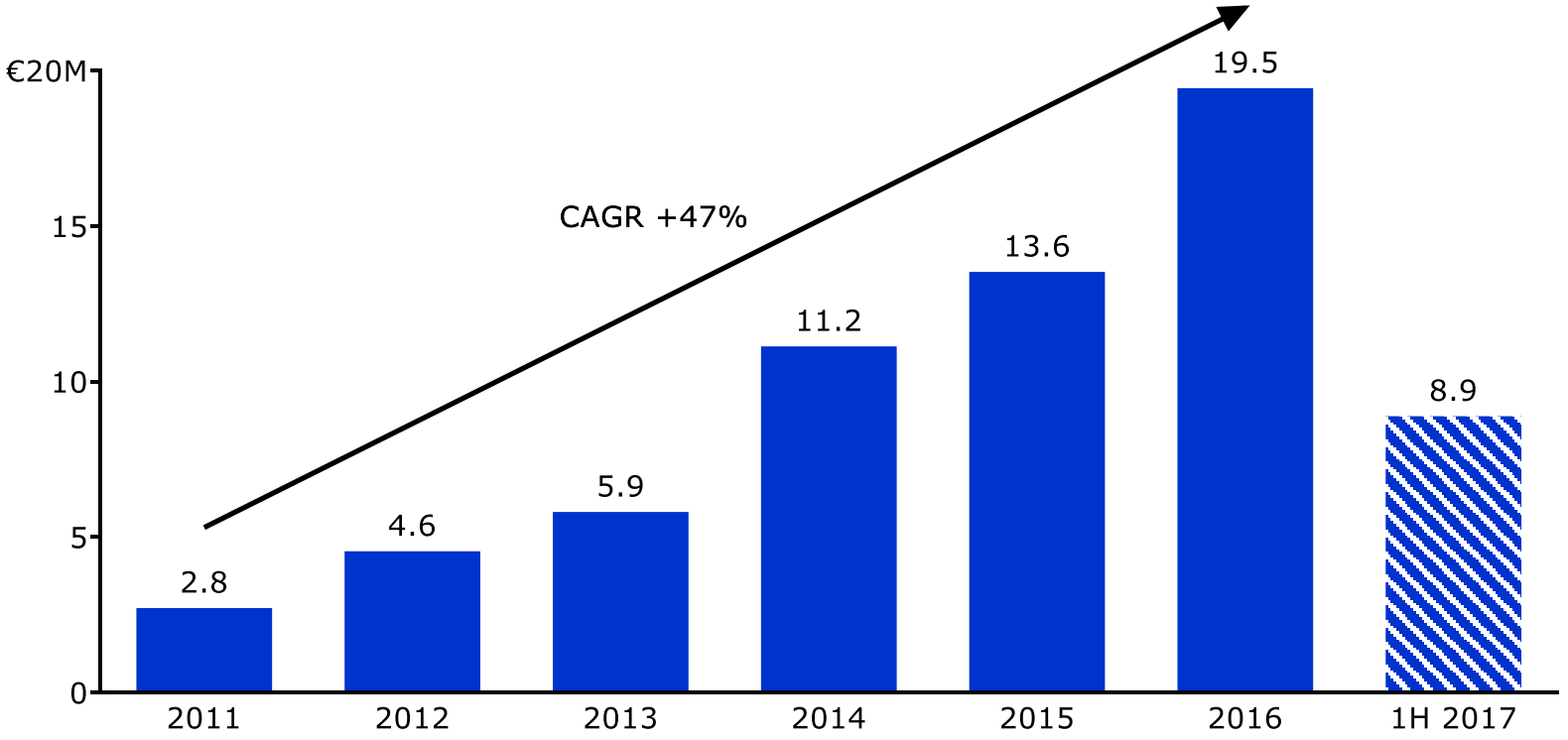
DIBIT Facility (Milan)

- **1,500 SQM** and **9 grade B/C suites**
- Authorized GMP manufacturing facility since 2003 for **clinical programs**
- Authorized GMP manufacturing facility since 2015 for the **Commercial products**

Open Zone Facility (Bresso)

- **3,300 SQM** and **>20 Grade B/C suites**
- Qualified *Officina Farmaceutica*, authorized for **GMP manufacturing** and quality control activities for the production of TK cells used in clinical trials

Significant revenues growth from development & manufacturing services and partnerships



YoY (%)		66%	27%	91%	21%	44%	3%

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Haploidentical transplant (haplo-HSCT) today

- Haploidentical transplant is a lifesaving procedure for many patients with haematological malignancies: number of haploidentical transplants doubled since 2010 for all indications*

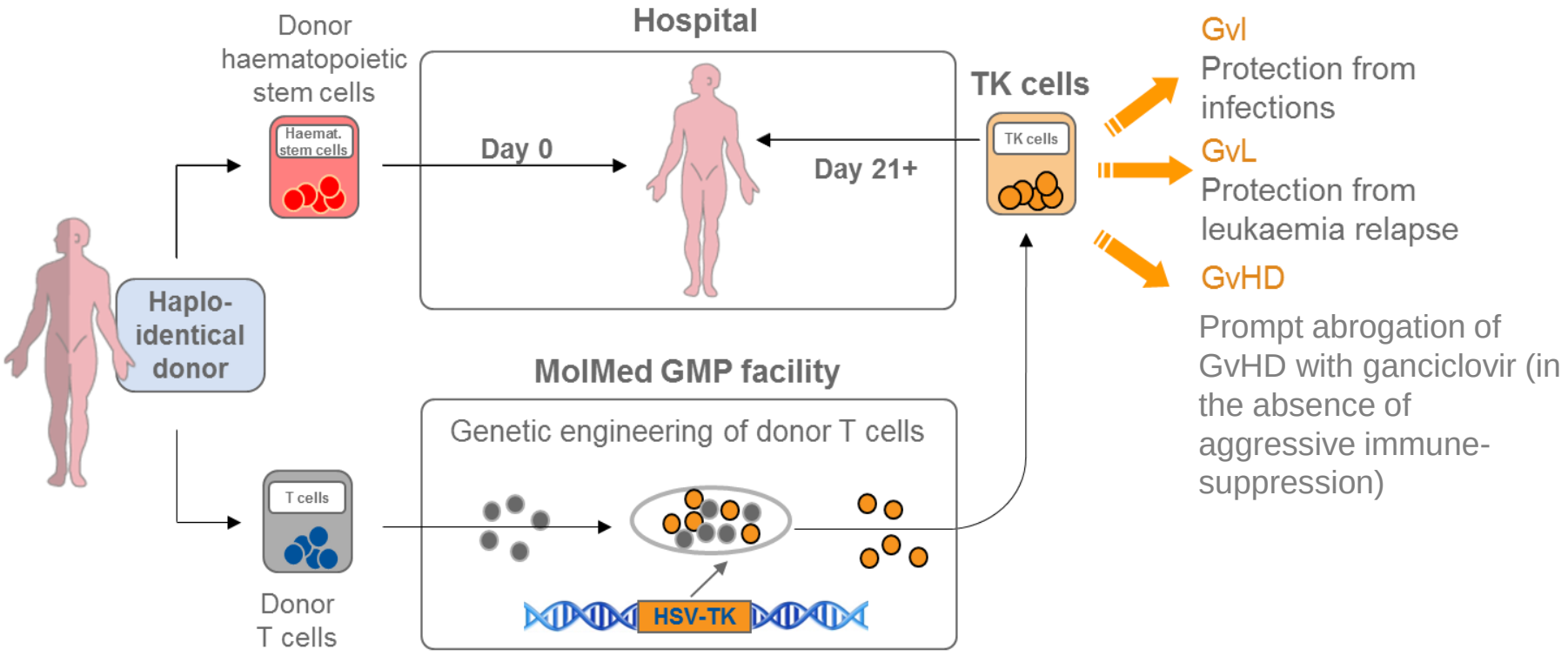
- There are two protocols currently used:

  - ✓ Ex vivo T-cell depleted haplo grafts without any donor cell therapy
    - delayed immune recovery and increased non-relapse mortality
  - ✓ T-cell replete haplo grafts followed by in vivo T-cell depletion and immunosuppression
    - increased GvHD and relapse risks

**Passweg 2015*

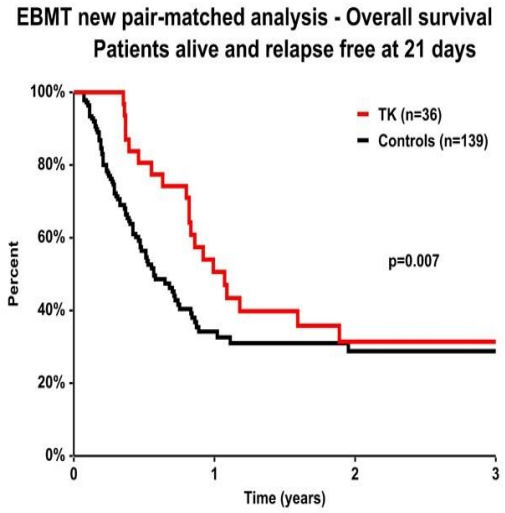
TK cells allow to preserve GvI and GvL effects

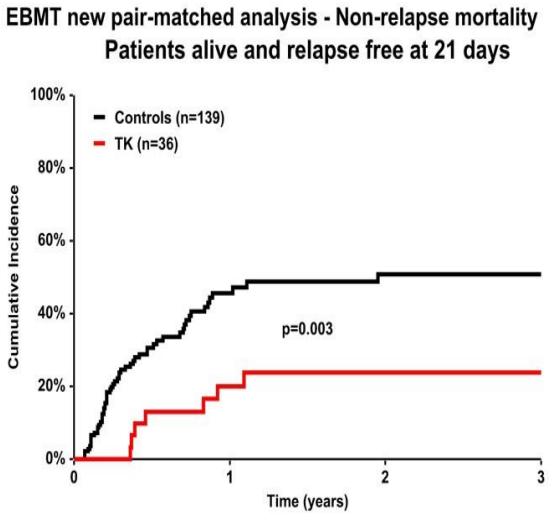
The TK haploidentical HSCT procedure makes a suitable donor available for any patient, without interfering with the timeframe of a normal transplantation

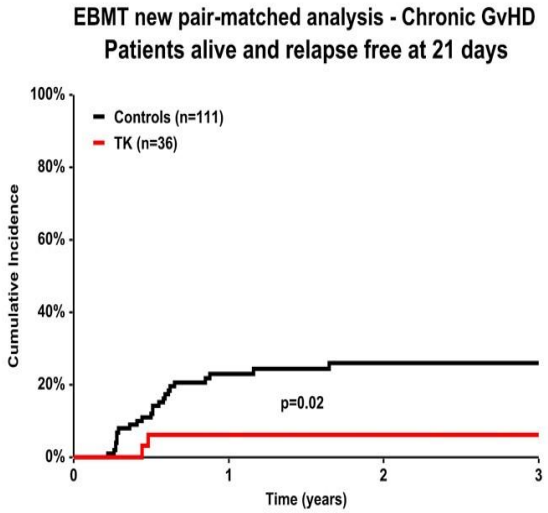


Bordignon, *Hum Gene Ther 1995;* Bonini, *Science 1997;* Bonini, *Nat Med 2003;* Traversari, *Blood 2007;* Ciceri, *Blood 2007;* Ciceri, *Lancet Oncol 2009*

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



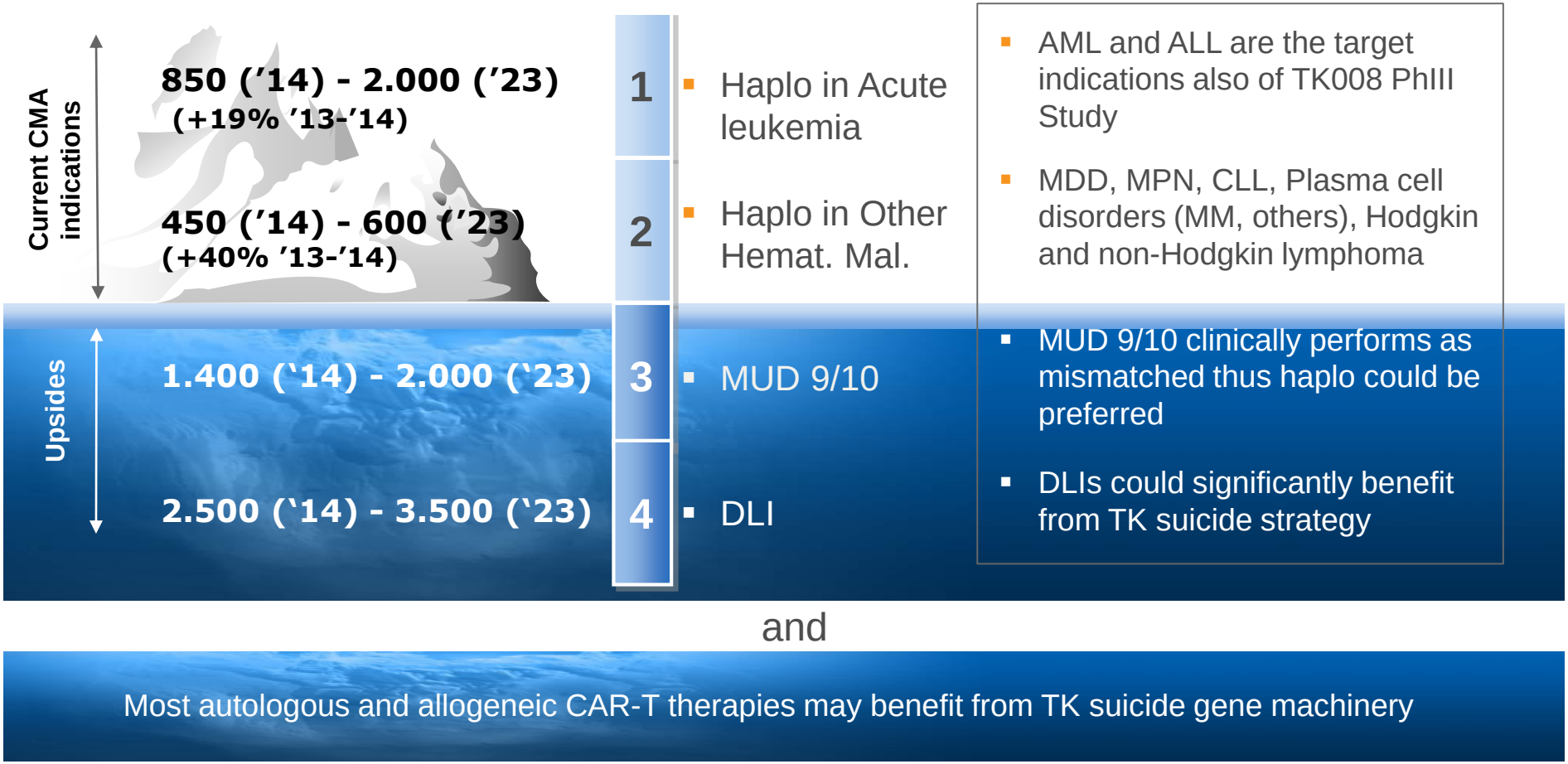




New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	**46%**	**34%**	**23%**
Zalmoxis (n=36)	**20%**	**51%**	**6%**
p-value^	***0.003***	***0.007***	***0.02***

*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)*

European market potential analysis: strong growth and relevant upsides*

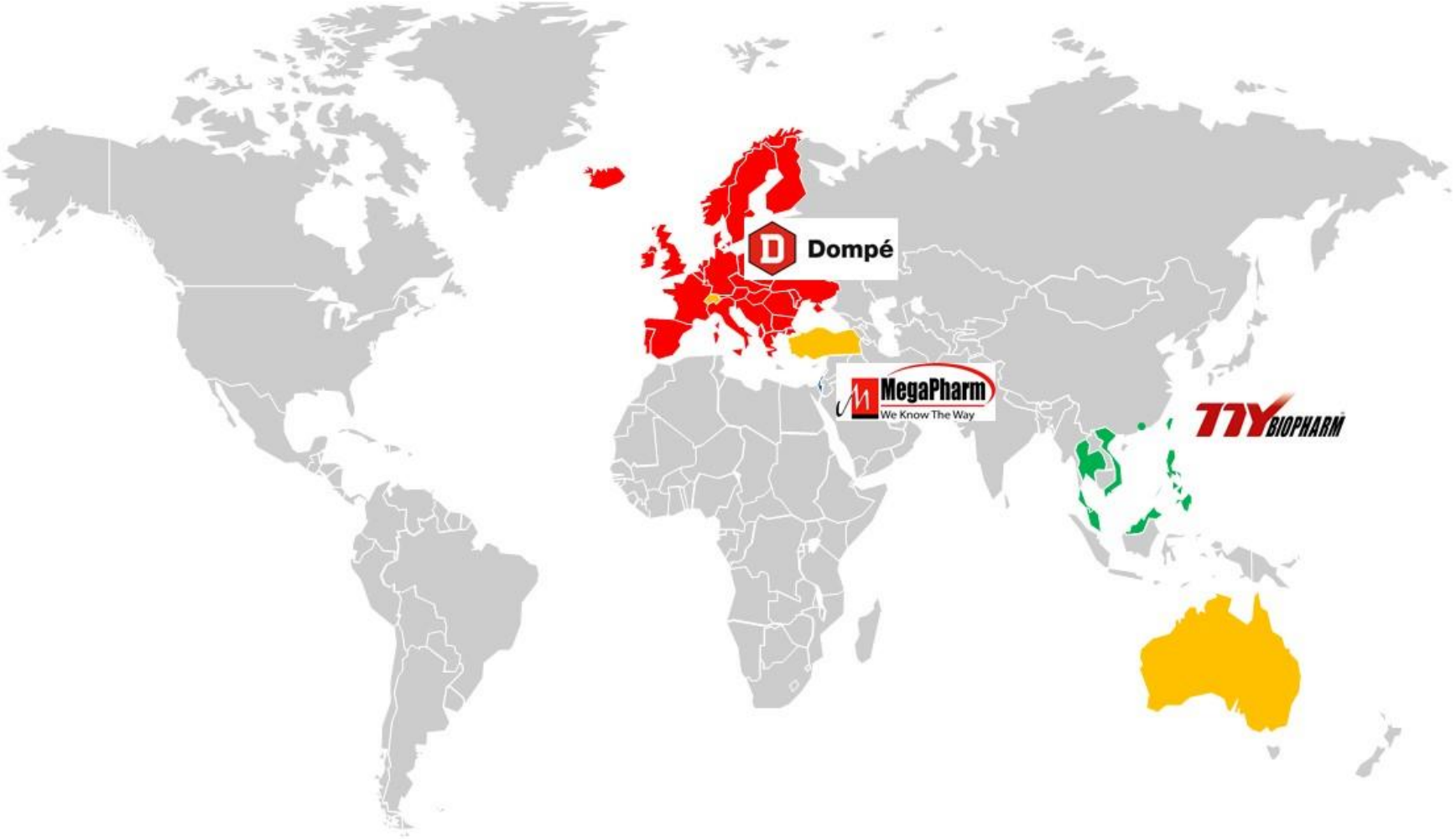


and

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

** Source: Company and EBMT*

Network of partners for the commercialization of Zalmoxis®



Following CMA, MolMed started several market access activities

- Definition of the pricing policy
- Drafting of the Core Value Dossier
- Definition of a pricing corridor

- Submission of the Pricing Dossier (Dec 2016)
- Ongoing discussion with CTS preparatory for CPR negotiation
- Definition of cost offset

- Activities propaedeutic to NUB application
- Ongoing drafting of AMNOG Dossier

- Activities propaedeutic to TC application
- Ongoing drafting of Pricing Dossier

- Definition of a cost-effectiveness model

- Strategy definition

Responsibility of Dompè

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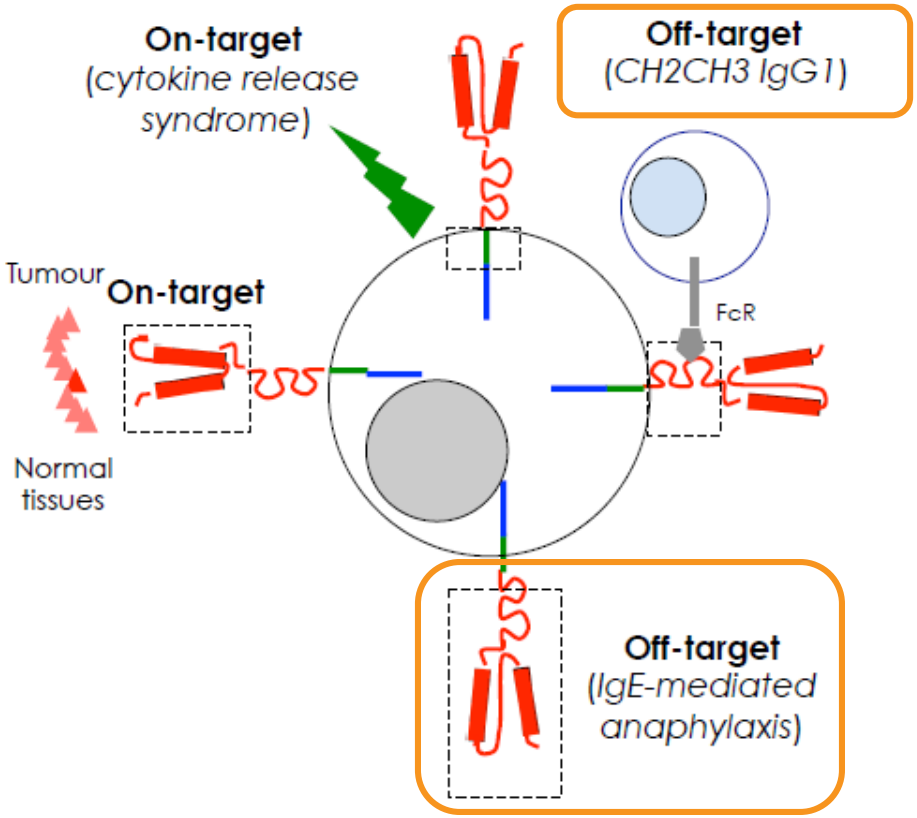
CD44v6 is expressed by several blood and solid cancers

- MolMed's CAR-CD44v6 is specific for variant v6 of antigen CD44 which is minimum expressed in normal tissue, while is over-expressed in haematological and epithelial tumours
  - 60% of AML and 90% of MM express CD44v6
  - Historically known 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

Off-target toxicities might be managed by exploiting the combination with 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

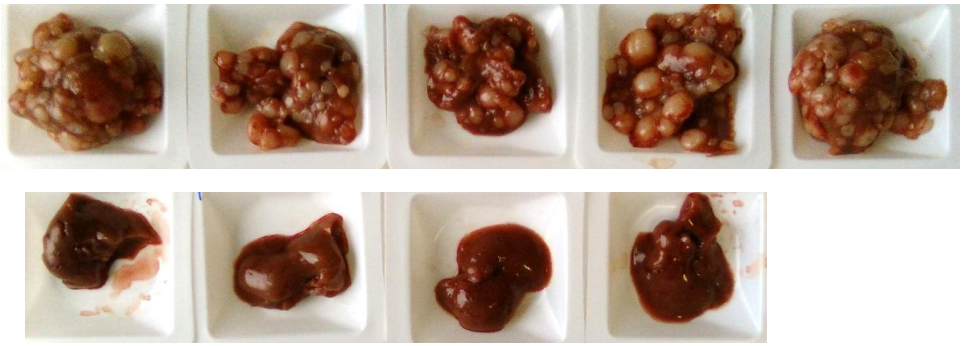
CAR-CD44v6: in vivo activity in a high tumor burden model of AML-M5 (THP-1)

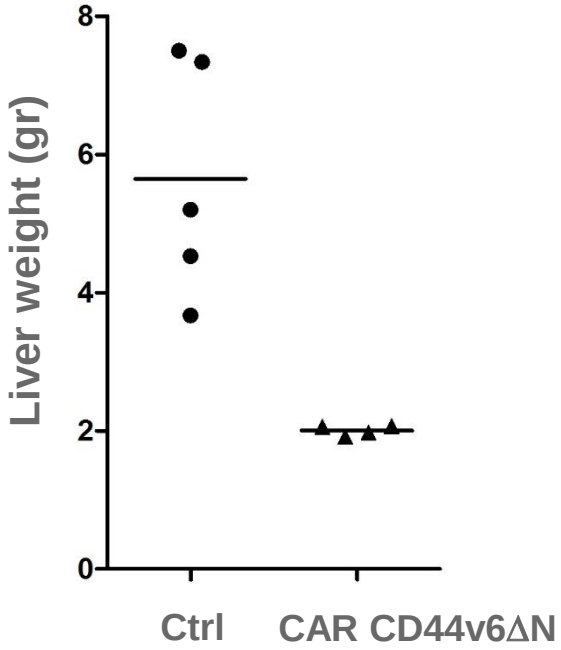
Day 1
AML

Day 15
T cells

Day 16
T cells

Day 40
Sacrifice





MolMed unpublished results

CAR-CD44v6: in vivo activity in a lung adenocarcinoma model (MR232)

Day 1
MR232 *sc*

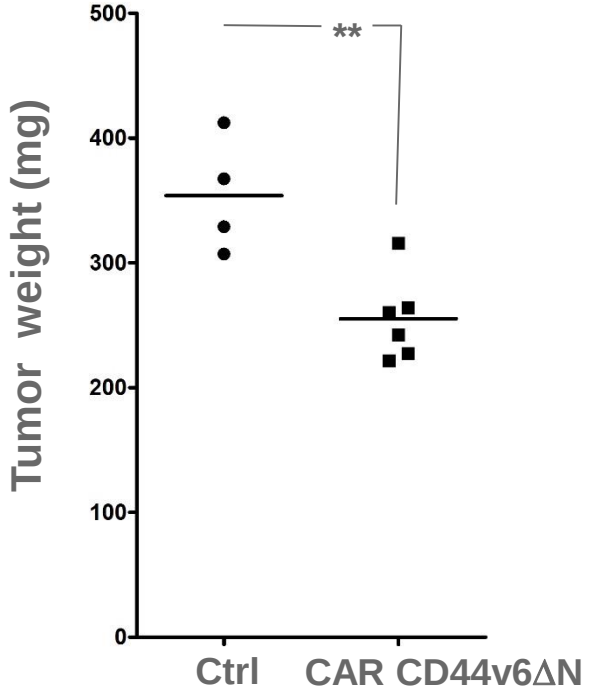
Day 3
5x10^6 T cells *iv*

Day 7
7x10^6 T cells *iv*

Day 14
Sacrifice

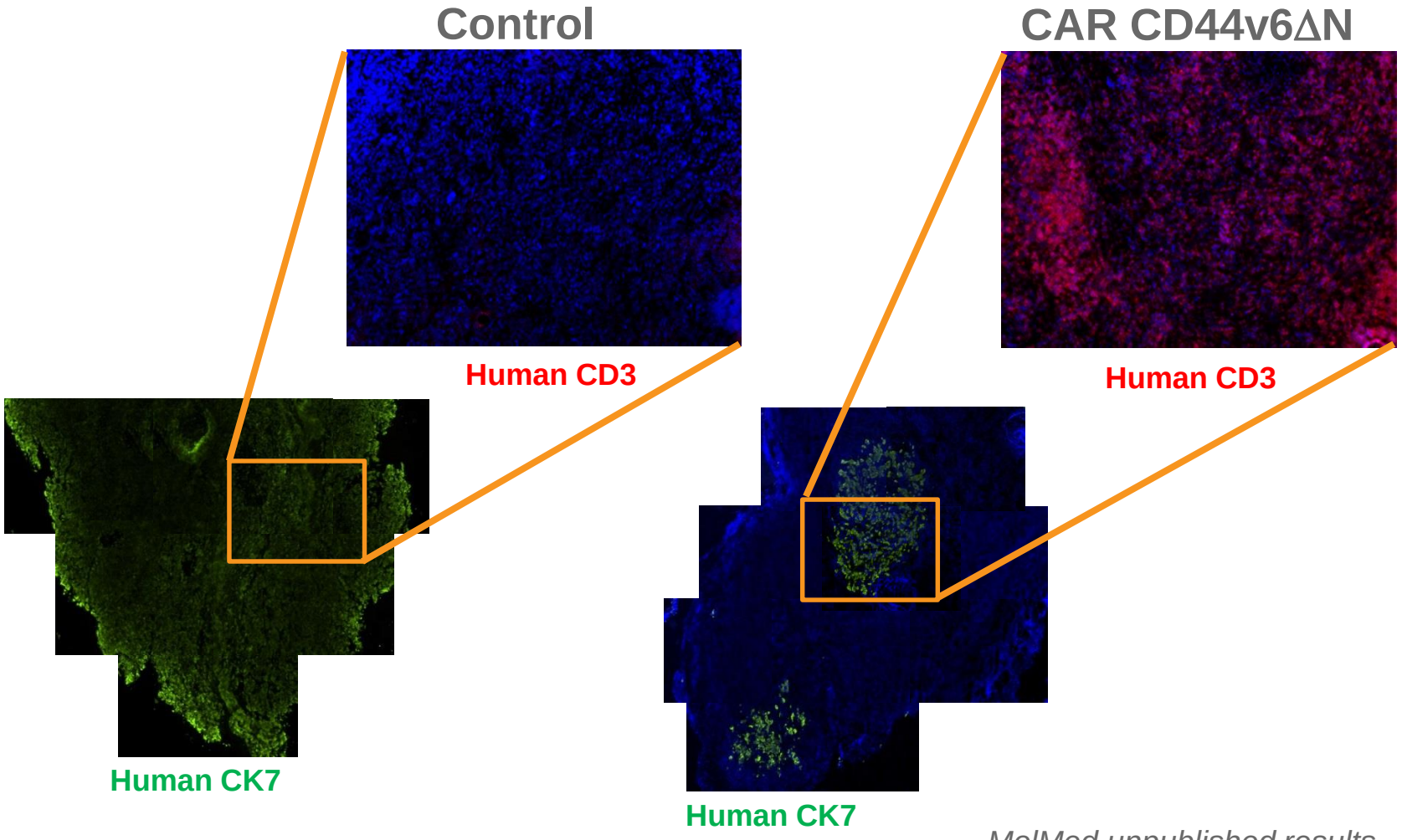
Ctrl

CAR CD44v6ΔN



MolMed unpublished results

Immunostaining of tumor sections from CAR CD44v6ΔN treated mice



MolMed unpublished results

MolMed leads the EU-funded EURE-CART project

- MolMed is **coordinator** of the the EURE-CART project
- EURE-CART's main object is to conduct a multicentre, **first-in-man Phase I/IIa clinical tria**l to demonstrate the safety and the efficacy of CAR-CD44v6 T-cell immunotherapy in **acute myeloid leukaemia and multiple myeloma**
- EURE-CART involves a consortium of **nine partners** from five different EU countries, including clinical, scientific and industrial groups clearly representing excellences in their fields. MolMed's partners are:
  - Ospedale San Raffaele (Italy)
  - Universitätsklinikum Würzburg (Germany)
  - Ospedale Pediatrico Bambino Gesù (Italy)
  - Fundacio Privada Institut de Recerca de L' Hospital de la Santa Creu i Sant Pau (Spain)
  - Fakultni Nemocnice S Poliklinikou Ostrava Foundation (Czech Republic)
  - Istituto Superiore di Sanità (Italy)
  - Acromion GMBH (Germany)
  - ARTTIC SAS (France)

Key financials – 1st half 2017

Key Income Statement:

- ✓ *total revenues of Euro 9.8 million, of which Euro 8.9 million from sales, up by 2.9% respect to Euro 8.7 million of first half 2016*
- ✓ *Operating and net results considerably improved by 21.2% and 22.2% respectively, against first half 2016*

(amounts in € thousand)	1st half 2017	1st half 2016	Variation €	Variation %
Operating Revenues	9,819	10,221	(402)	(3.9%)
Revenues	8,935	8,681	254	2.9%
Other revenues	884	1,540	(656)	(42.6%)
Operating costs	16,320	18,457	(2,137)	(11.6%)
Operating result	(6,501)	(8,236)	1,735	21.1%
Net result for the period	(6,522)	(8,379)	1,857	22.2%

Key financials – 1st half 2017

Net Financial Position (cash and cash equivalents, as the Company has no indebtedness)

(amounts in € thousand)	**June 30, 2017**	**Dec 31st, 2016**
Net Financial Position	12,461	19,702

Additional resources – Standby Equity Facility Agreement

At the end of 2016, the Company signed a SEF Agreement with Société Générale; the bank has undertaken to subscribe the Share Capital Increase upon submission of discretionary requests from the Company at the terms and conditions specified in the agreement (3 days VWAP, 5% discount).

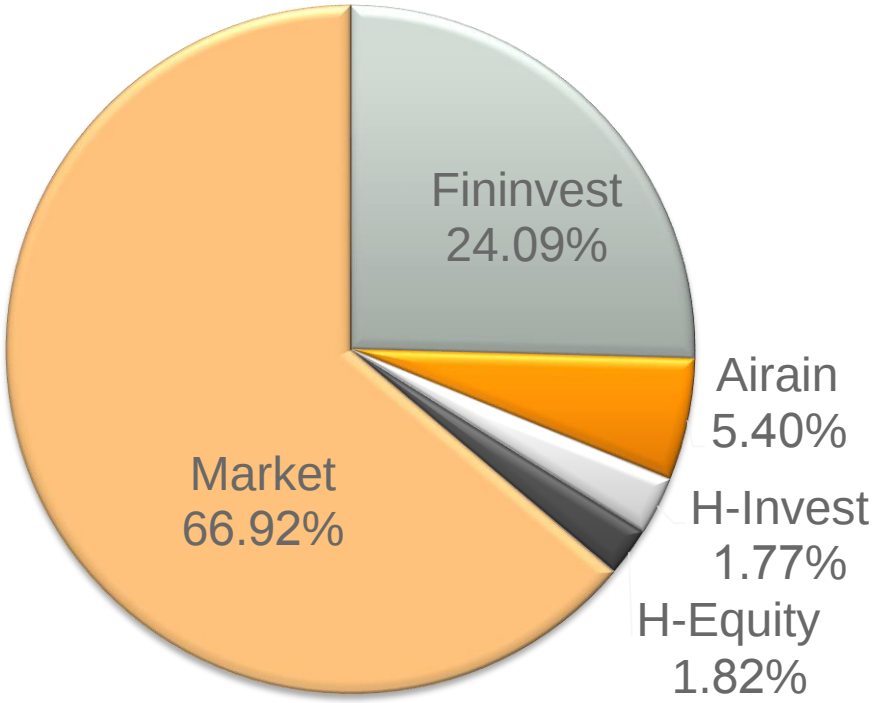
The agreement (2 year length till October 2018) involves a total of n. 42,000,000 of new MolMed's shares, of which already issued n. 23,5 million (**gross proceeds of about € 9,8 million**).

Capex

(amounts in € thousand)	**1st half 2017**	**1st half 2016**
Capex	741	933

MolMed's shareholders' structure

Market cap: ~200 M € (at Oct 27, 2017)



Objectives for 2017

Zalmoxis®

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

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 ☑
  - ✓ Cellectis on UCART ☑
- Gradual entry into operation of the new GMP facility ☑

Target for 2018

Zalmoxis®	CAR-T CD44v6	GMP Solutions
▪ Price in main EU markets ▪ Advancement of PhIII TK008 ▪ Growing stream of revenues from EU sales	▪ Starting of PhI/II on hematological malignancies ▪ Completion of preclinical efficacy and safety testing on solid tumors ▪ Exploratory partnering discussion ▪ Further expand the pipeline	▪ Finalization of the authorization process for the Stream 1 of the new GMP facility ▪ New partnerships or expansion of existing agreements for development and manufacturing of vectors and cells

Contacts

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