MolMed S.p.A.
Company Overview

Leading the way in Cell & Gene therapy

September, 2018

Disclamer

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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Cell & Gene: 2017 – 18 momentum

$7.5 Billion Total Amount Raised in 2017*

$4.2 Billion raised in 2016

78% increase in **Global Investments**

Major 2017/18 M&A deals

GILEAD / Kite Pharma	*Oct 2017*	**Gilead Sciences** acquired **Kite Pharma**, Inc. for **11.9 $ Bn**
SANOFI / Bioverativ	*Jan 2018*	**Sanofi SA** agreed to buy **Bioverativ Inc.** to gain treatments for rare blood disorders ~**11.6 $ Bn**
Celgene / juno	*Jan 2018*	**Celgene Corporation** acquired **Juno Therapeutics** advancing Global Leadership in cellular Immunotherapy ~**9 $ Bn**

Source: 2017 ARM Industry Survey, Data provided by INFORMA
**Data does not include M&A transactions*

Cell & Gene: major regulatory approvals confirmed positive risk-benefit ratio

July 2016 — **STRIMVELIS** gene therapy* for the treatment of ADA-SCID: EU market authorization

Reimbursed Price in Italy: 594,000 € /patient

* **MolMed developed and manufactures Gsk' Strimvelis**

Aug 2016 — **Zalmoxis** patient-specific cell therapy obtained the EU market authorization form

Reimbursed price in Italy: 149,000 € /infusion

(clinical trial average dose ~ 2 infusions/patient)

Aug 2017 — **Kymriah CAR T cell therapy** for children and young adults with ALL **475,000 $ / patient** (**373,000 $ / patient** in DLBCL in May 2018)

Oct 2017 — **Yescarta CAR T cell therapy** for the adult patients with relapsed/refractory large B cell lymphoma after two or more lines of systemic therapy **373,000 $ / patient**

Dec 2017 — **Luxturna gene therapy** for biallelic RPE65-mediated inherited retinal disease **425,000 $/eye**

Aug 2018 — **CAR T cell therapies Kymriah and Yescarta** obtained EU market authorization: **price obtained by Kymryah in UK 361,750 $ / patient** (282,000 £).

MolMed: recognized leader in Cell & Gene research, development and manufacturing

- Biotechnology company focused on research, development, manufacturing and clinical validation of innovative anticancer and rare diseases therapies, listed on the main market (MTA) of the Milan Stock Exchange since 2008 (MLMD.MI)
- Pioneering research & development approach in viral vectors and cells engeneering
- Strong Leadership Team and ~ 200 scientists and support staff
- Development and Manufacturing services: high profile network of partners and solid revenue grow (+39% 2011-17 CAGR)
- Relevant pre-clinical, clinical and regulatory achievements
- Growing and diversified proprietary pipeline

MolMed's Leadership Team and Scientific Advisory Board

Management Team

- MolMed' Founder and Chairman
- Member of the Scientific Council of the European Research Council
- Full Professor of hematology at the University San Raffaele in Milan
- Chairman of the Scientific Advisory Board

Claudio Bordignon, *MD*
Founder & Chairman

- MolMed' Founder and Chairman
- Member of the Scientific Council of the European Research Council
- Full Professor of hematology at the University San Raffaele in Milan
- Chairman of the Scientific Advisory Board

Riccardo Palmisano, *MD*
CEO

- Since 2015 CBO at **MolMed**
- 2013-15 **Bain & Co**
- 2011-12 Research Associate at **Sanford B. P. Medical Discovery Institute** (La Jolla, CA)

Luca Alberici, *PhD, MBA*
CBO

- Since Sept 2018 CFO at **MolMed**
- 2014-18 General Manager at **Jazz Pharma Italy**
- 2005-14 COO and at **Gentium** (NASD)
- 2003-05 **Cell Therapeutics Europe**
- **1996-2003 PWC**

Salvatore Calabrese
CFO

- Clinical research coordinator and Head of Hematology and Hematopoietic Stem Cell Transplantation Unit at **Ospedale S. Raffaele**
- Full professor, **University San Raffaele**

Fabio Ciceri, *MD*
Clinical Research Consultant

Scientific Advisory Board

Director of the Center for Cell and Gene Therapy and Professor of Medicine and of Pediatrics at Baylor College of Medicine, Houston, TX

Malcolm K. Brenner

Professor of Hematology and Head of the Hematology and cellular therapy Department at the Saint-Antoine Hospital and University Pierre & Marie Curie, Paris

Mohamad Mohty

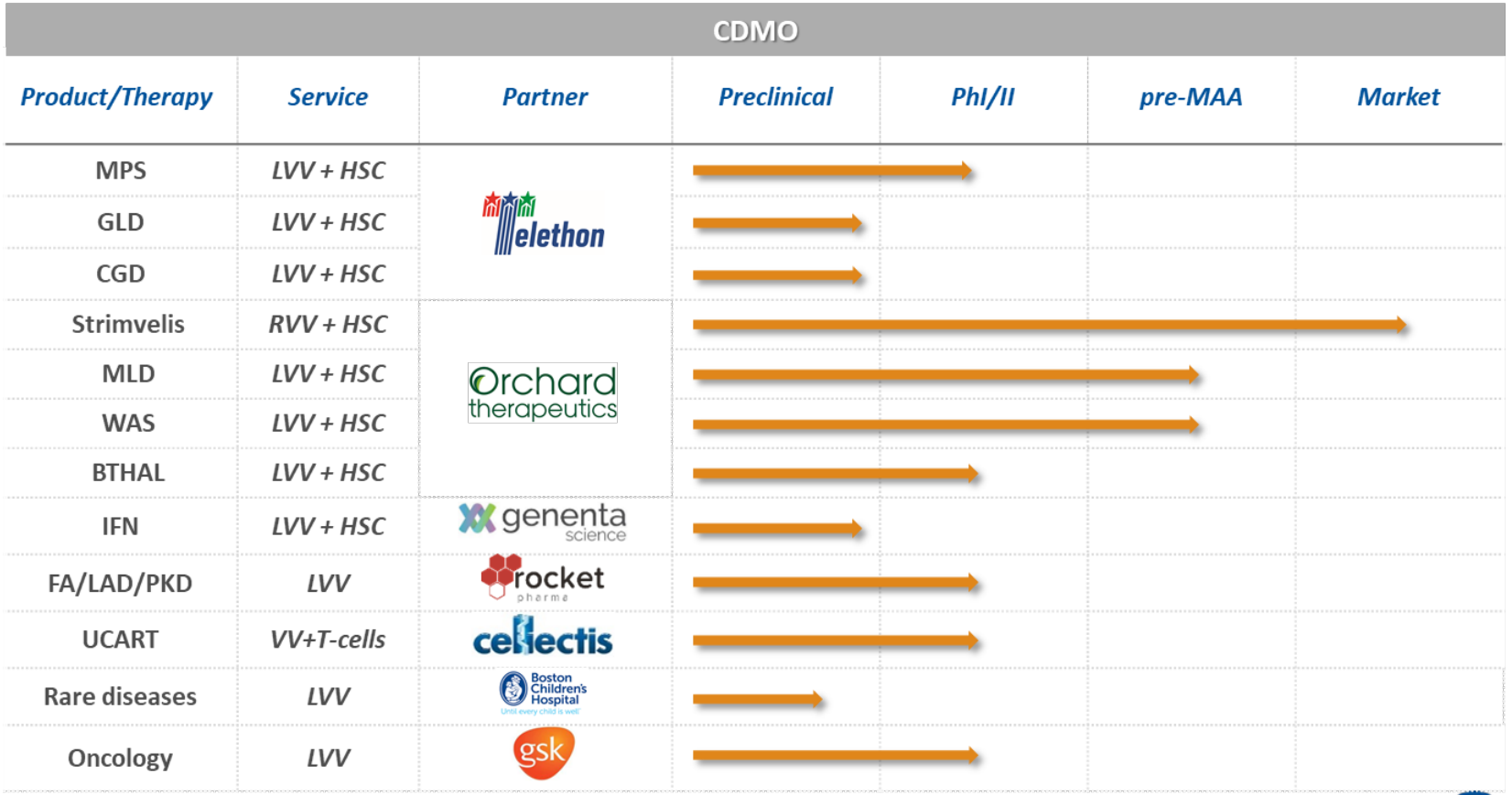
Deputy Chief, Adult Bone Marrow Transplant Service at Memorial Sloan Kettering Cancer Center, NY, USA

Miguel-Angel Perales

Member of the UNC Lineberger Comprehensive Cancer Center and Professor of the Microbiology and Immunology Director of the UNC Immunotherapy Program at the Univ. of North Carolina, NC

Gianpietro Dotti

MolMed GMP Development and Manufacturing partners



Excellent GMP capacity

MolMed obtained **the GMP manufacturing authorization** for Cell & Gene Therapies for its proprietary products as well as for third parties and/or in partnership

San Raffaele Facility (MI)

- **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**

Bresso New Facility

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

MolMed relevant achievements

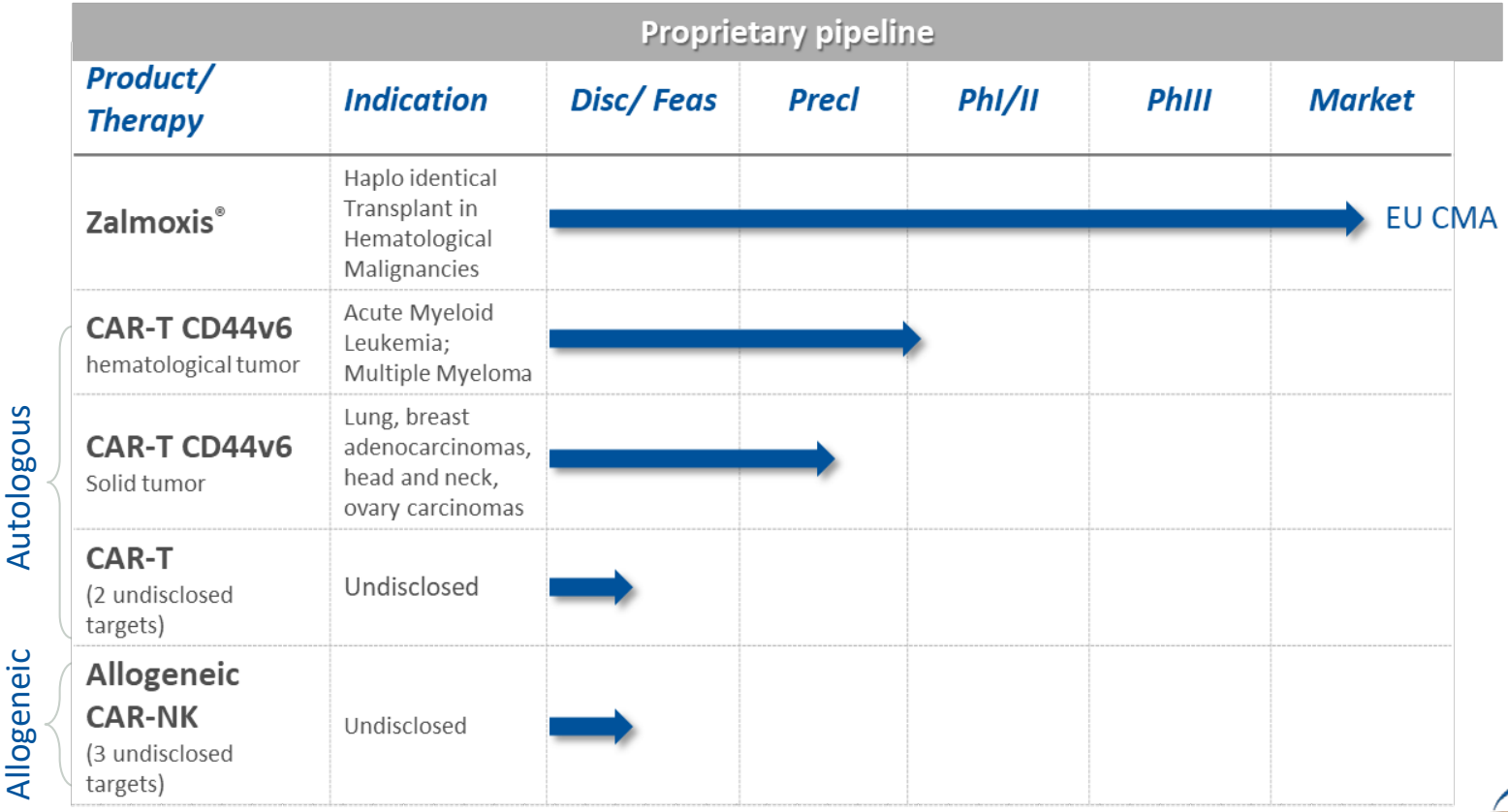
- ✓ **9 proprietary patent families** including 244 granted patents
- ✓ **Zalmoxis®, Aug 2016: EMA conditional market authorization**, first negotiated price & reimbursement (Italy €149,000 per infusion)
- ✓ **Strimvelis, July 2016: EMA market authorization**, MolMed is the exclusive manufacturer for vector and medicinal product
- ✓ **CAR T CD44v6, Aug 2018: IMPD submitted for 1st in-man clinical trial**

«MolMed Spa is uniquely endowed in the EU with the knowhow and experience necessary to meet this ambitious objective, as demonstrated by its unparalleled track record»

«To be successful, EURE-CART proposes the early involvement of National regulatory ***authorities for accelerating the approval of CAR T-cell immunotherapy, as well as the centralisation of its production by the MolMed Spa»*** Horizon 2020 EURECART Project funding commission.

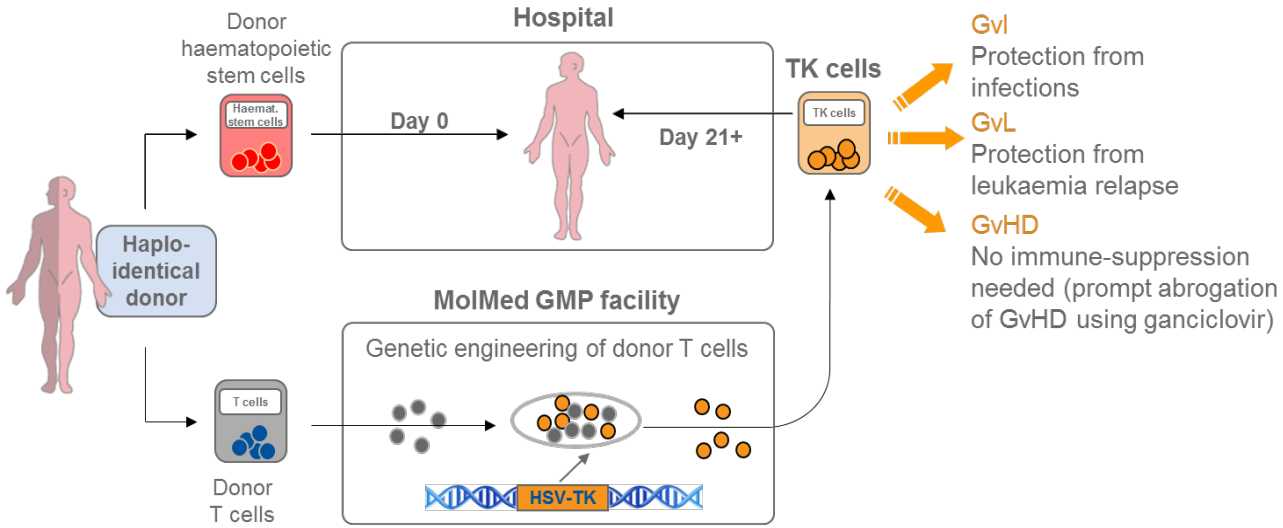
MolMed Onco-hematology proprietary pipeline

Product portfolio includes **proprietary anti-tumor cell & gene therapies** in **clinical and preclinical** development:



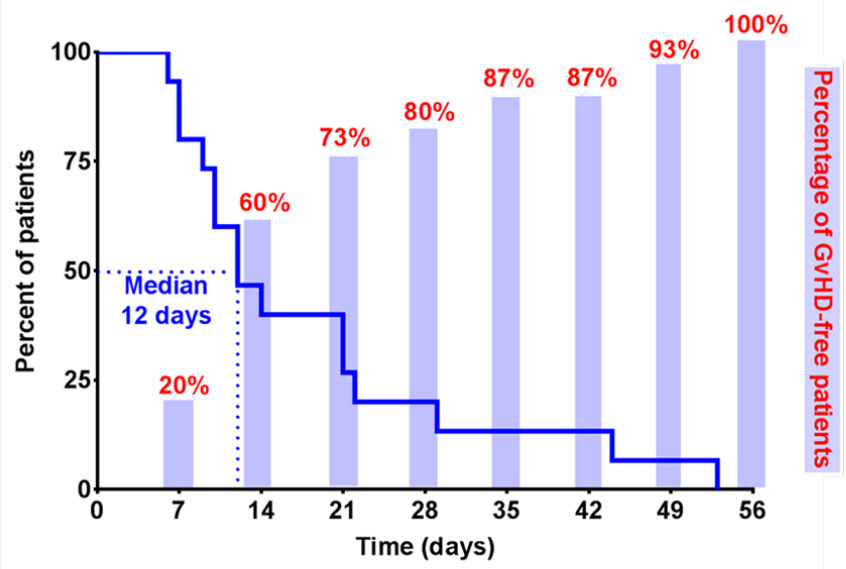
Zalmoxis®: a first in class orphan drug with a specific mechanism of action to address the limits of partially compatible stem cell transplantation

Zalmoxis® (TK) is an ex vivo cell therapy based on donor T cells genetically engineered to enable bone marrow transplants from **partially compatible donors,** inducing a rapid **immune reconstitution**



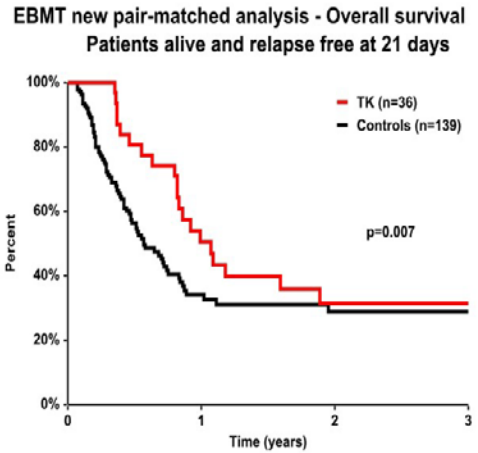
Zalmoxis® efficacy: 100% of acute GvHD resolution

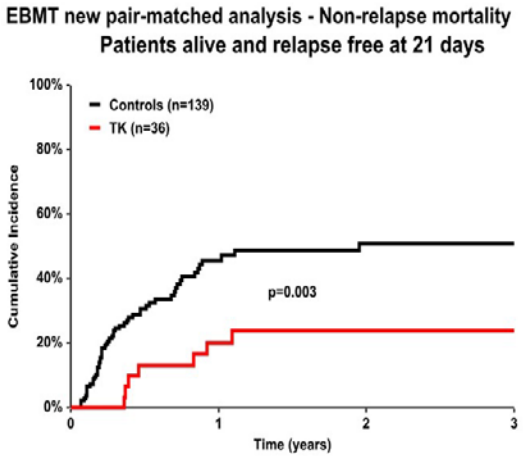
TIME TO RESOLUTION AND % OF PATIENTS GVHD FREE FROM GVHD ONSET (DAYS; N=16)

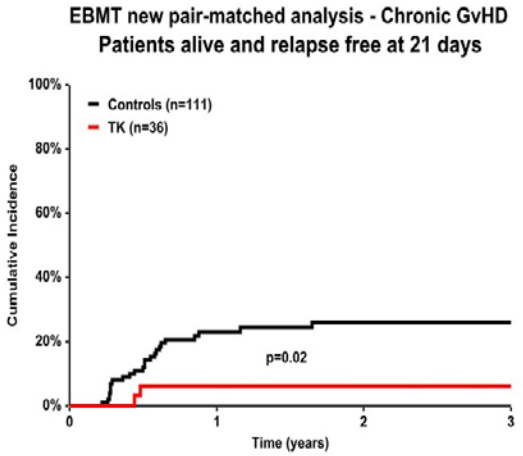


Note: Pulled data from TK007 and TK008 (experimental arm)
Source: ASH Meeting 2014, Abs. 2535

Zalmoxis®: clinical outcome as additional life saving therapy

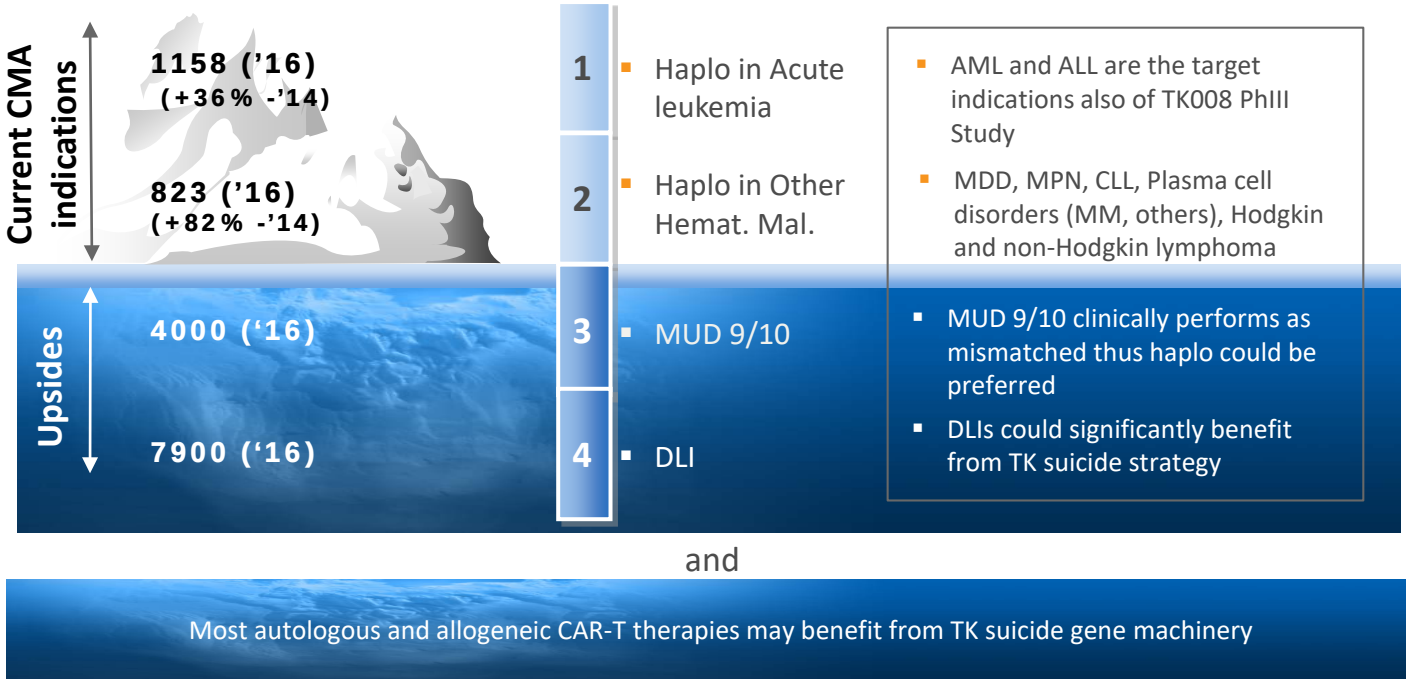






New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	46%	34%	23%
Zalmoxis (n=36)	20%	51%	6%
*p-value^*	*0.003*	*0.007*	*0.02*

European target population: haploidentical transplants



High unmet medical need opportunity

Source: Company and EBMT (Passweg J et al ,Bone Marrow Transpl 2018)

Zalmoxis® journey

Aug 2016

European Commission CMA
(Conditional Marketing Authorization)

- **Robust Core Value Dossier**
- **Clear pricing policy and corridor**

Apr and Jun 2017

MOLMED
TTY Biopharm MegaPharm

Exclusive commercial agreement

- TTY Biopharm **(Asian countries)**

Upfront + Milestones: 13.5M€

- TTY Biopharm **(Israel)**

Jul 2017

MOLMED

MolMed - Dompé

Strategic commercialization and Supply Agreements for **Europe**

Upfront + Milestones: 43.5M€
Royalties included in transfer price

Dec 2017

AIFA Reimbursement price
Eur 149,000 / infusion
(Flat price per patient)

Jan 2018

P&R* dossier Submission
Reimbursement price
Eur 163,900 / infusion

Feb 2018

Dompé exercises the option to undertake market access activities in **Switzerland, Turkey** and **Australia**

Next Steps

UNDER CONSIDERATION

FDA Marketing Authorization

ONGOING

Price/ Marketing Authorization

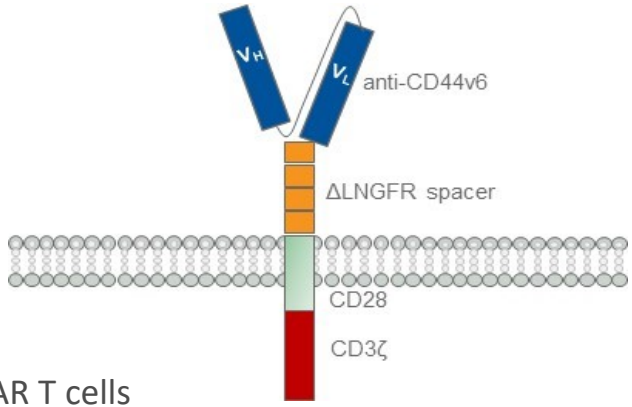
** P&R: Pricing & Reimbursement*

CD44v6 CAR T cells: an original late preclinical stage therapy, targeting both hematological and solid tumors

CAR-T family: **lymphocytes armed with chimeric receptors** that have demonstrated high anti-tumor potential, also against tumors, above all hematological, particularly aggressive and resistant to traditional therapies

CAR T CD44v6 features

- **Variant v6 of antigen CD44** is over-expressed in MM and AML
- **High safety profile** (low skin toxicity and suicide gene)
- **High therapeutic potential also in hematological and solid tumors**: it specifically recognizes variant 6 (v6) of the antigen CD44 (CD44v6)
- The **LNGFR spacer** allows **selection** and ***in vivo* tracking** of CD44v6 CAR T cells
- Generation of **CD44v6 antigen-loss variants** is circumvented by the reduced growth of CD44v6 negative tumor cells



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

CAR-CD44v6

hematological tumours

Day 1
AML

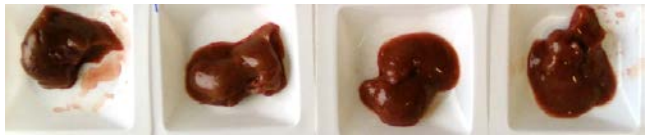
Day 15
T cells

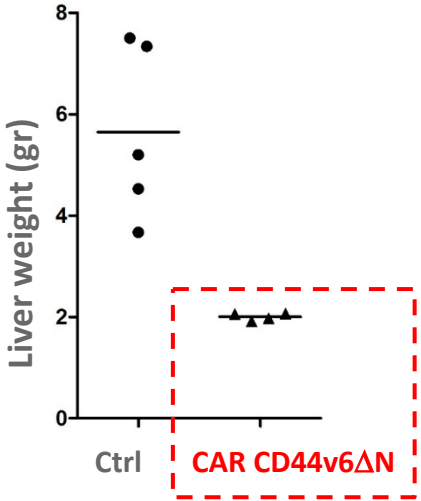
Day 16
T cells

Day 40
Sacrifice

CAR-CD44v6 cures aggressive leukemia in a mouse model: liver

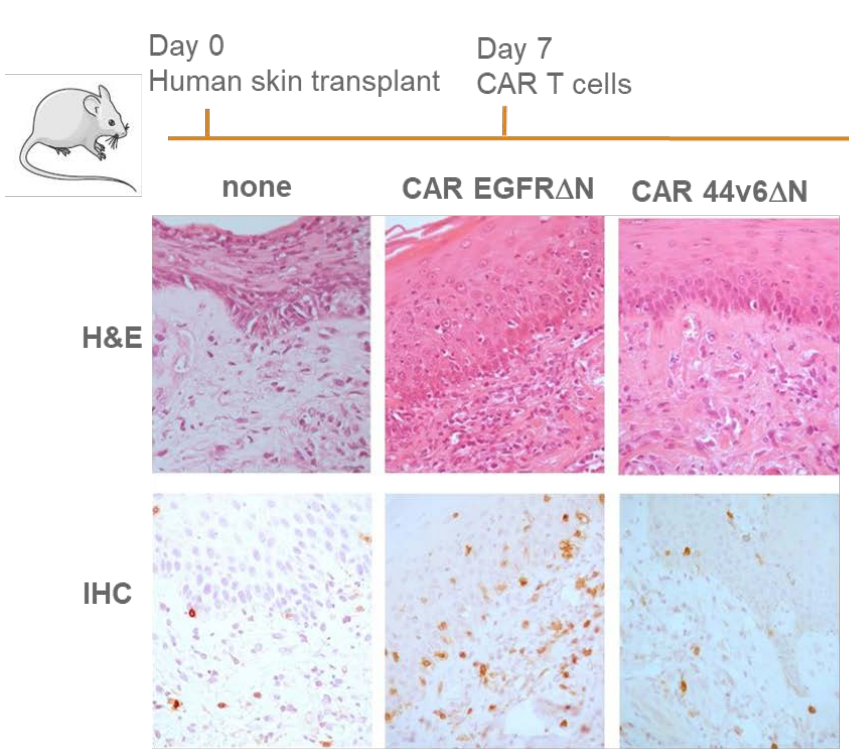
CD44v6

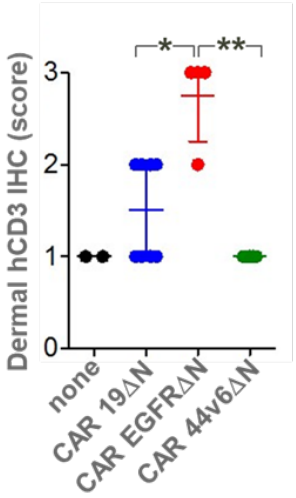




MolMed published results

CD44v6 CAR-T cells do not significantly infiltrate the skin: low expected toxicity





Use in combination with MolMed' proprietary patent TK suicide gene

CARTCD44v6 clinical trial within the EU-funded EURE-CART project

CAR-CD44v6

hematological tumours

Multi-centre, first-in-man Phase I/IIa clinical trial to demonstrate the safety and the efficacy of CAR-CD44v6 T-cell immunotherapy in:

- *Acute Myeloid Leukemia (**AML**)*
- *Multiple Myeloma (**MM**)*

Phase I - Dose escalation
Objectives: ***Maximum Tolerated Dose and Clinical Activity***
18 Pts (3 dose levels) **up to 30 Pts** (BOIN Adaptive design)

Q418 / QI19

Phase II - Dose expansion
Objectives: ***Confirmation of Clinical Activity and Safety Profile***
14 Pts (1 Dose level selected in Phase I) **per indication** (Simon design)

Q1-Q2 2020

MolMed leads a Team of clinical experts in oncology and pioneers in the field of Cell & Gene therapy:

EURE CART

- IRCCS Ospedale San Raffaele (Italy)
- Universitätsklinikum Würzburg (Germany)
- Ospedale Pediatrico Bambino Gesù (Italy)
- L'Hospital de la Santa Creu i Sant Pau (Spain)
- University Hospital Ostrawa (Czech Republic)
- Istituto Superiore della Sanità (Italy)
- Acromion GMBH (Germany)
- ARTTIC SAS (France)

CARTCD44v6 targeting severe unmet clinical needs in hematological tumours

Acute Myeloid Leukemia (AML)

- HSCT remains the most effective long-term treatment, yielding cure in 50–60% of patients (*Cormelissen et al., 2015; Passweg et al., 2016*)
- Transplant eligibility decrease with age and comorbidities
- Elderly pts are cured in only 10-20%
- Primary chemo resistant AML represent 20-30% of cases and dismal prognosis
- ***CAR T-cells may improve outcome in chemo resistant AML and in patients not eligible to transplantation (Medlinger et al., 2016)***

Multiple Myeloma (MM)

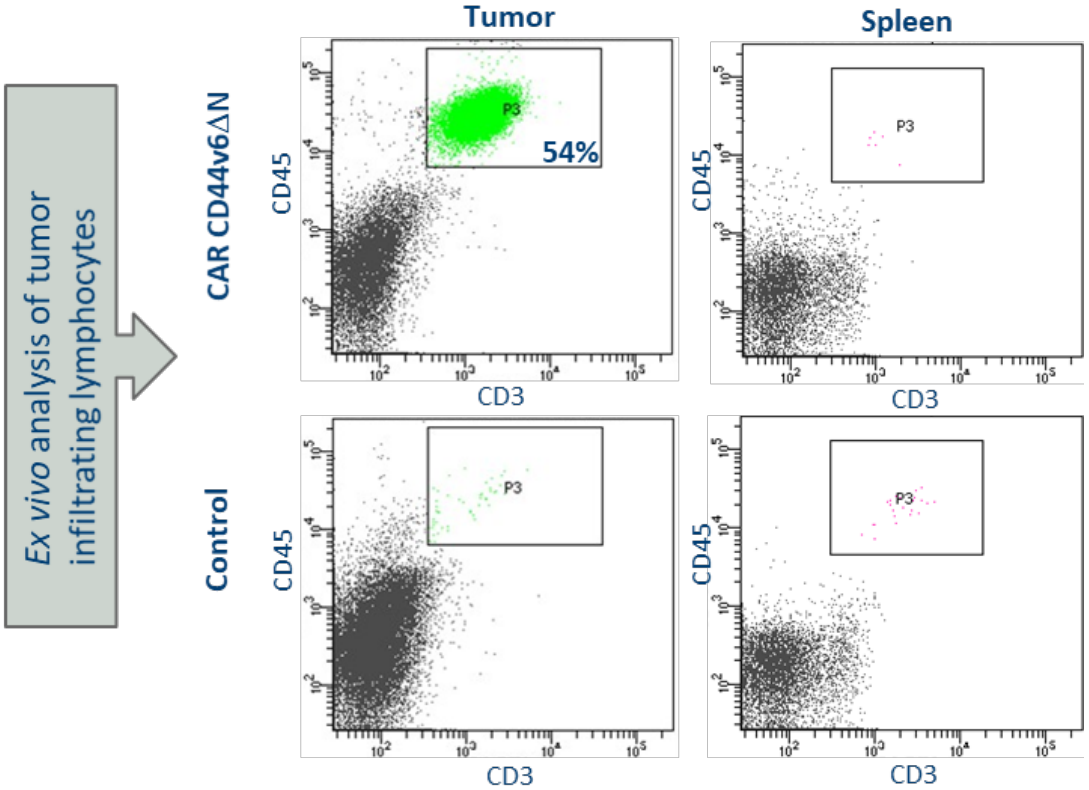
- In the last few years, novel drugs have improved both progression free (up to ~30 months) and median overall survival (~4.7 years) (*Warren et al., 2013; San Miguel et al., 2008*).
- Despite these recent achievements with novel drugs in the treatment of MM, duration of response decreases with successive relapses until resistant relapse develop
- ***Clinical need of a potentally curative approach is high in MM***
- ***CAR-T are being under investigation as a strategy to treat relapsing/refractory disease after failure of at least three different regimen***

CD44v6: expressed by several solid cancers

- Most of the clinical studies conducted to date have used CAR specific for CD19 antigen, **limiting its use in patients with hematologic B cell malignancies (hematological tumor)**
- **Variant v6 of antigen CD44** is over-expressed also in several solid **epithelial tumors**:
  - Squamous Cell Carcinomas mainly from **head & neck, esophagus, skin, and lung, ovary**
  - Adenocarcinomas mainly from **breast, lung, pancreas and colon**
  - **Sarcomas**
- Antitumor activity of CD44v6CAR T cells has successfully been demonstrated in **preclinical models of human lung and ovary carcinomas**

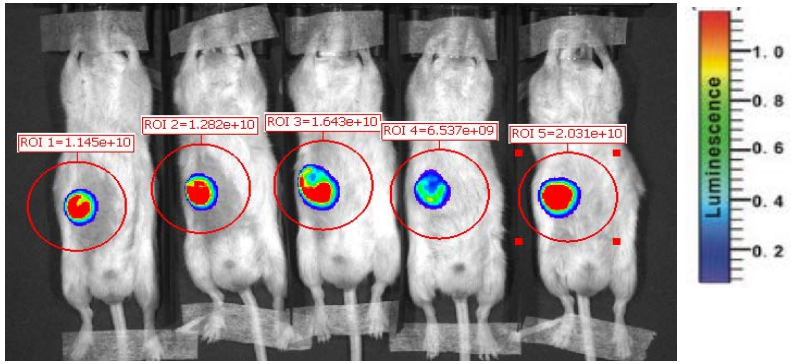
STRONG RATIONALE TO DESIGN AND IMPLEMENT A BASKET TRIAL BY 3Q 2019

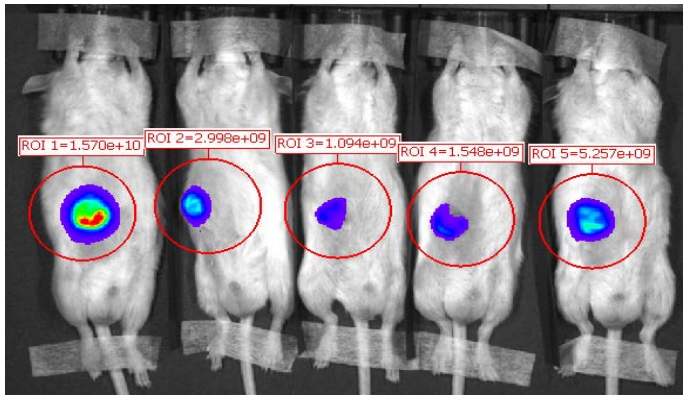
CD44v6 in vivo activity in solid tumors: a human lung adenocarcinoma model

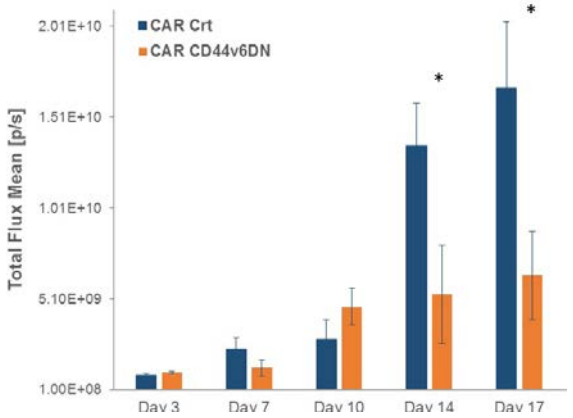


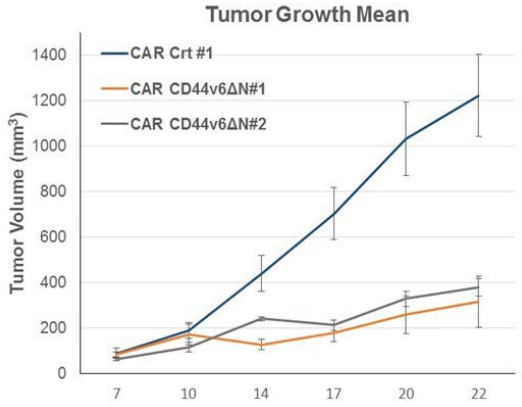
CAR T CD44v& highly infiltrates the tumor

CD44v6 in vivo activity in solid tumors: effect against human lung adenocarcinoma









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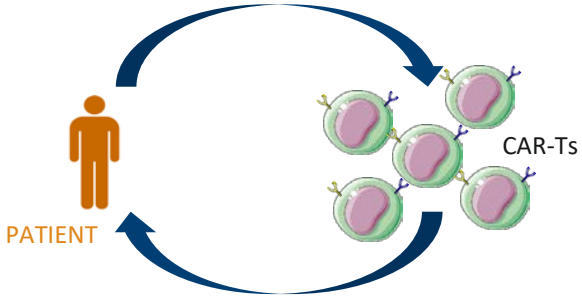
MolMed approach to develop new-generation CAR therapies proprietary pipeline

MolMed is one of the few biopharma worlwide having a diversified pipeline in both autologous and allogeneic CARs

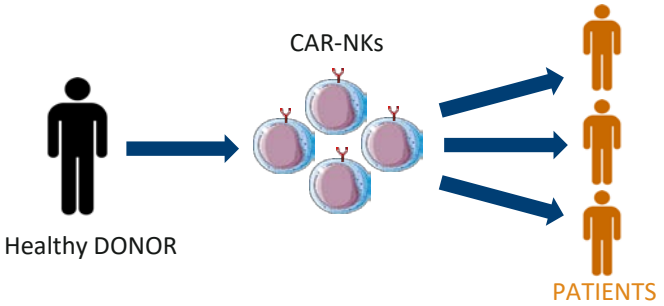
Autologous CAR-T Platform



- **No GvHD risk**
- **Proven clinical efficacy**
- **High production cost (1 batch = 1 patient)**

June 28th 2018: ***3ys Master Agreement with AbCheck*** *for the development of new CARs targeting* ***novel tumor antigens***

Allogeneic CAR-NK Platform



- **NK cells exclude GvHD**
- **Lower COGS/patient** (significant benefits from both a technical and logistic point of view)
- **Wider market potential** (1 batch = multiple patients)

May 31st 2018: ***binding term sheet with Glycostem*** *for the development and manufacturing of* ***allogeneic CAR-NK therapies***

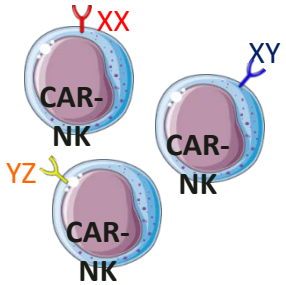
MolMed criteria to select new targets for autologous CAR T therapies

- ✓ Target **unmet clinical needs**
- ✓ Target both **hematological and solid tumors**
- ✓ Chose **targets with safe and/or moderate risk expression** profile
- ✓ **No follower approach** on CD19 or other targets in advanced clinical development by large companies
- ✓ Evaluate **IP freedom to operate**
- ✓ Target selection endorsed by **Scientific Advisory Board**

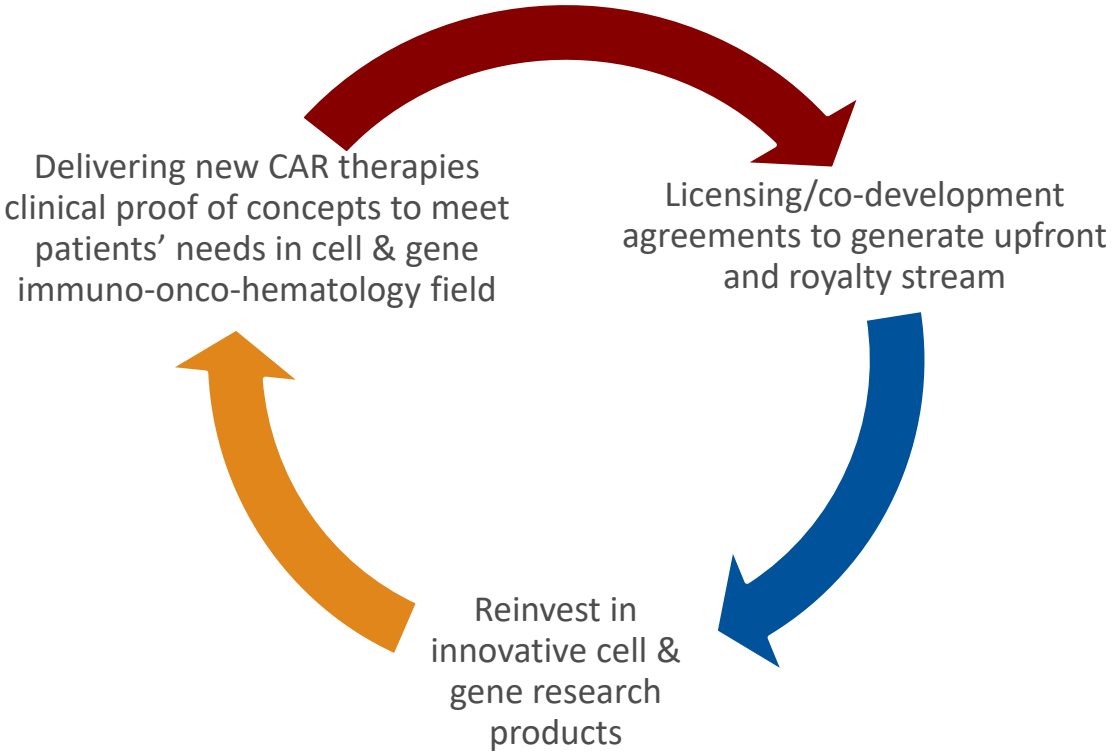
MolMed approach to select a new allogeneic CAR platform

- ✓ CAR-NK cells are one of the **most innovative pre-clinical investigations** in cellular immunotherapy with much less competition compared to autologous CAR-T
- ✓ NK cells are cells of the innate immune system, capable to mediate anti-cancer **effects without the risk of inducing graft-versus-host disease (GvHD)**
- ✓ NK cells are **well suited as off-the-shelf therapy capable**, starting from a single batch produced by a healthy donor, to treat a large number of patients with cancer
- ✓ **MolMed has defined 3 specific targets products**, using 3 different tumor antigen receptors, in order to enlarge possible cancer indications, targeting both hematological and solid tumors and place itself in a leadership position inside the CAR-NK field

«***NK cells are going to be the next big thing in CAR therapy***»
Carl June, MD, PhD; ASCO 2018



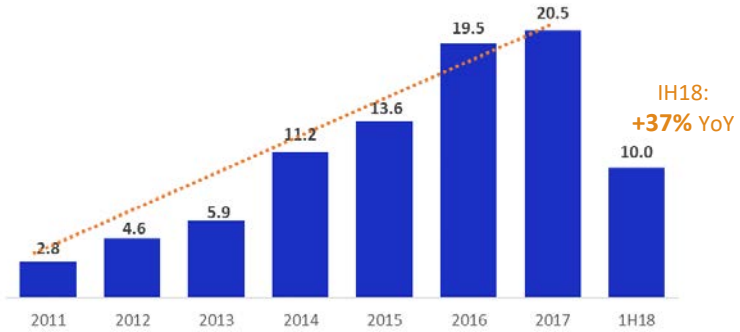
MolMed' CARs platform strategic vision



MolMed: an established dual business model leveraging common technological assets

GMP Solutions (CDMO *)

- A growing source of revenues to fund internal R&D, but also…
- ..a cutting edge technological asset to grant robust development and manufacturing of internal products



* Contract Development and Manufacturing Organization

Proprietary Pipeline

- Ability to manage from pure research to clinical, manufacturing, regulatory authorization, market access and pricing & reimbursement
- A growing and diversified clinical and pre-clinical stage onco-hematology cell & gene products pipeline

Product/ Therapy	Indication	Disc/ Feas	Precl	Phl/II	PhIII	Market
Zalmoxis®	Haplo identical Transplant in Hematological Malignancies					
CAR-T CD44v6 Liquid tumor	Liquid tumors (leukemia, myeloma)					
CAR-T CD44v6 Solid tumor	Solid tumors (pancreas, breast, head and neck)					
CAR-T (2 undisclosed targets)	Undisclosed					
Allogeneic CAR-NK (3 undisclosed targets)	Undisclosed					

MolMed: solid financial position with significant improvements vs IH17

- **Total IH18 Revenues** of 12.7€ M, with Revenues from sales equal to Euro 12.2€ M, increased by 36.9% compared to IH17
- **Operating and Net Results** considerably improved by 56.1% and 52.7% respectively, compared to IH17
- **MolMed evergreen losses carried forward** amounting to about 204 Euro/million

€/000	IH18	IH17	Δ IH18 vs IH17 €	Δ IH18 vs IH17 %	FY17
Operating Revenues	**12,712**	**9,819**	***2,893***	*29.5%*	**23,987**
Revenues	12,234	8,935	*3,299*	*36.9%*	23,000
Other operating income	478	884	*(406)*	*(45.9%)*	987
Operating costs	(15,563)	(16,320)	*757*	*4.6%*	32,135
Operating Result	**(2,851)**	**(6,501)**	***3,650***	***56.1%***	**(8,148)**
Net Result	**(3,085)**	**(6,522)**	***3,437***	***52.7%***	**(8,497)**
Net Financial Position(*)	**18,098**				**18,111**

- **Financial Position** equal to 18.1€ M cash and cash equivalents and current financial receivables, with no financial debt

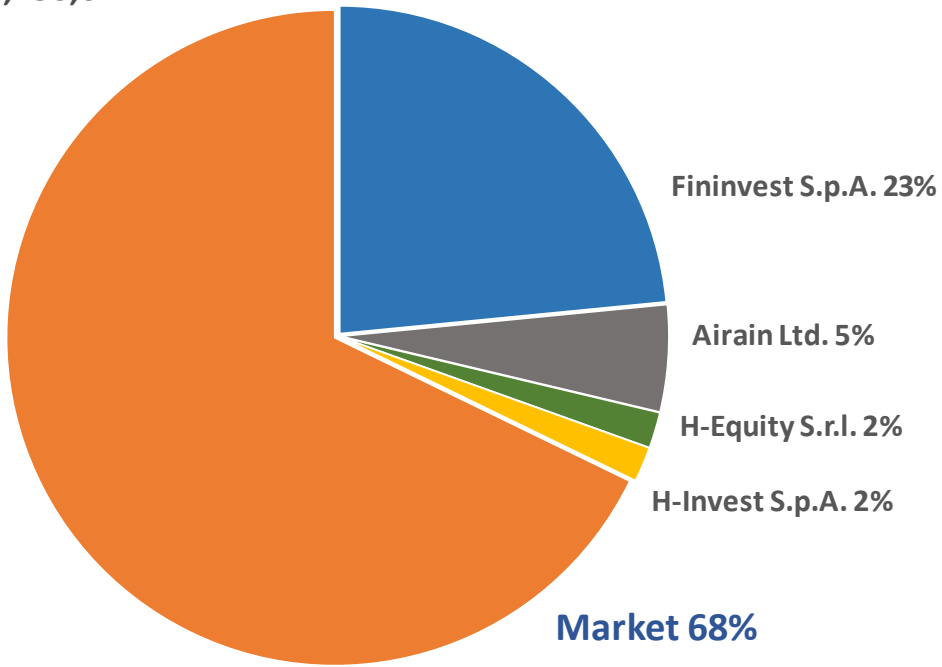
MolMed: financial results improved significantly in the last three years

- **Total FY17 Revenues** of 24€ M, with Revenues from sales equal to Euro 23€ M, increased by 18.0% compared to 2016
- **Operating and Net Results** considerably improved by 40% and 38.8% respectively, compared to 2016
- **Human resources** increased year by year, from 152 employees at the end of 2016 to 186 as of December 31st, 2017

€/000	FY17	Δ FY17 vs FY16 €	Δ FY17 vs FY16 %	FY 2016	FY 2015	Δ FY16 vs FY15 €	Δ FY16 vs FY15 %
Operating Revenues	**23,987**	***1,162***	*5.1%*	**22,825**	**16,764**	***6,061***	***36,2%***
Revenues	23,000	*3,516*	*18.0%*	19,484	13,576	*5,908*	*43,5%*
Other operating income	987	*(2,354)*	*(70.5%)*	3,341	3,188	*153*	*4,8%*
Operating costs	32,135	*(4,276)*	*(11.7%)*	36,411	37,302	*(891)*	*(2,4%)*
Operating Results	**(8,148)**	***5,438***	***40.0%***	**(13,586)**	**(20,538)**	***6,952***	***33.8%***
Net Result for the period	**(8,497)**	***5,379***	***38.8%***	**(13,876)**	**(20,784)**	***6,908***	***33.2%***
Net Financial Position	**18,111**			**19,702**	**29,938**		
Work Force (#)	***186***	***5***		***181***	***152***	***29***	

MolMed Shareholders' structure

- MolMed is listed on the main market (MTA) of the **Milan Stock Exchange** since 2008 (**MLMD.MI**)
- **Market cap ~ 189M €** (as of September 4th, 2018)
- **Outstanding shares: 463,450,672**



MolMed strategic goals and upcoming milestones

Proprietary Product Pipeline

- ***Zalmoxis®**: place in therapy and geography development*
- ***CAR44v6 (hematological tumors)***
  - ***4Q18 - IQ19**: 1st in man clinical trial*
  - ***Q1 - Q2 2020:** Confirmation of Clinical Activity and Safety Profile*
- ***CAR44v6 (solid tumors)***
  Following hematological trial IMPD:
  - ***3Q19**: solid trial IMPD*
  - ***4Q19 – IQ20:** 1st in man clinical trial*
- ***New CAR pipeline**: **4Q19:** preclinical data*

Contract Development and Manufacturing

- ***Bresso Facility** Stream 2 authorization (**2019/20**)*
- *Further expansion of client base and revenue **double digit growth***
- ***Services range** and technological platforms enlargement*

Contacts

Ilaria Candotti
Investor Relations & Communication Manager
e-mail: investor.relations@molmed.com
MolMed S.p.A.
phone: +39 02 21277.205