MolMed S.p.A.
Company Overview

Leading the way in Cell & Gene therapy

November 12th, 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 £); **by Yescarta in UK 392,000 $ / patient** (300,000 £).

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 **engineering**
- ~ **200 scientists and support staff**
- Development and Manufacturing services: high profile network of partners and solid revenue grow (+39% 2011-17 CAGR)
- Growing and diversified **proprietary pipeline with autologous and allogeneic CAR therapies**
- GMP manufacturing authorization for Cell & Gene Therapies for its proprietary products as well as for third parties and/or in partnership.
- **2 Authorized GMP manufacturing facility** (4.800sqm) for GMP manufacturing, quality control activities for the production of clinical and commercial products.

Relevant achievements

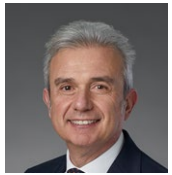
- ✓ **9 proprietary patent families** including 244 granted patents
- ✓ **Zalmoxis®, Aug 2016: EMA Conditional Market Authorization**, first negotiated price & reimbursement (Italy €149,000 per infusion)
- ✓ **Strimvelis, July 2016: EMA Market Authorization**, MolMed is the exclusive manufacturer for 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.

Management team and Scientific Advisory Board

Management Team



Carlo Incerti, *MD*
Chairman

- MolMed' Chairman
- 1991-2018 Head of Global Medical and Chief Medical Officer Affairs at **Sanofi Genzyme**
- Member of the **Board of EuropaBio**, the European Association for Bioindustries
- Member of the Governing Board of **IMI (Innovative Medicine Initiative)**

Riccardo Palmisano, *MD*
CEO

- Since 2015 **CEO at MolMed S.p.A**.
- Since 2016 President of **Assobiotec** (Italian biotech industries Trade Association)
- 2005 - 15 Vice President, Managing Director and GM at **Genzyme Italy**
- 2003 - 05 VP Commercial Retail Market at **GSK Italy**
- 2000 - 03 Managing Director and GM at **Shire Italy**

Luca Alberici, *PhD, MBA*
CBO

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

Salvatore Calabrese
CFO

- 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**

Fabio Ciceri, *MD*
Clinical Research Consultant

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

Scientific Advisory Board

Claudio Bordignon
MolMed Founder and SAB Chairman

- Chairman of the SAB
- Member of the Scientific Council of the European Research Council
- Full Professor of hematology at the University San Raffaele in Milan

Malcolm K. Brenner

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

Miguel-Angel Perales

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

Mohamad Mohty

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

Gianpietro Dotti

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

GMP Development and Manufacturing partners

Product/Therapy	Service	Partner	Preclinical	PhI/II	Pre-MAA	Market
MPS	LVV+HSC	Telethon	→			
GLD	LVV+HSC		→			
CGD	LVV+HSC		→			
Strimvelis	RVV+HSC	Orchard therapeutics	→	→	→	→
MLD	LVV+HSC		→	→	→	
WAS	LVV+HSC		→	→	→	
BTHAL	LVV+HSC		→	→		
MPS IIIA	Und.		→			
MPS IIIB			→			
Oncology	LVV	gsk	→	→		
FA	LVV	rocket pharma	→	→		
LAD	LVV		→			
UCART	LVV+T cells	cellectis	→	→		
Rare diseases	LVV	Boston Children's Hospital	→			
IFN	LVV+HSC	genenta science	→			

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)

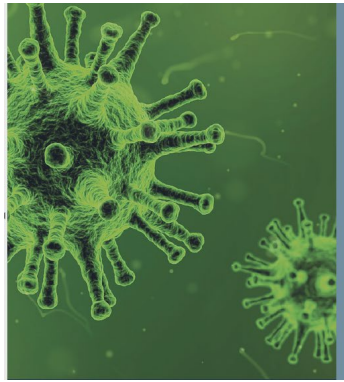
- **1,500 SQM (16,000 SQF)** and **9 grade B/C suites**
- Authorized GMP manufacturing facility since 2003 for **clinical programs**
- Authorized GMP manufacturing facility since 2015 for the **Commercial products**

Bresso New Facility

- **3,300 SQM (36,000 SQF)** 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**
- **Stream 2 opening in 2019/2020, for further services and new collaborations**

CDMO business experience in manufacturing of vector and modified cells

Viral Vector GMP Manufacturing



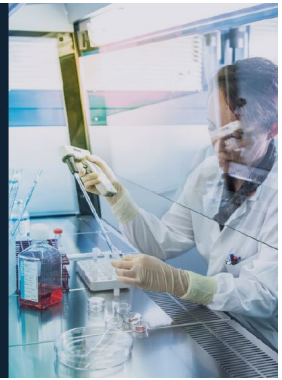
Up to **200L** Vector manufacturing
+ 150 GMP Vector batch produced

- Customization, development, qualification and validation activities carried out by top level Expertise
- Able to perform more than 100 analytical tests in-house, resulting in containment of material, costs and release timelines

GMP Cell Engineering

- Top level expertise from tech transfer to fill and finish for clinical and commercial use
- We offer proprietary processes for gene modification of HSC (CD34+) and T-cells

+ 250 Treated patients
+ 20 Clinical Trials



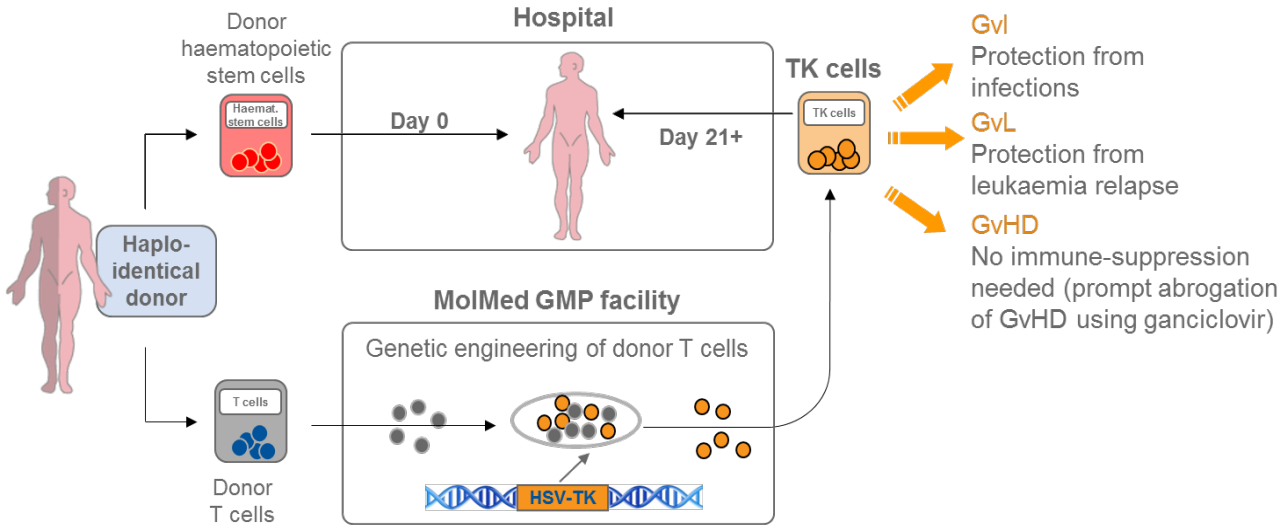
MolMed Onco-hematology proprietary pipeline

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

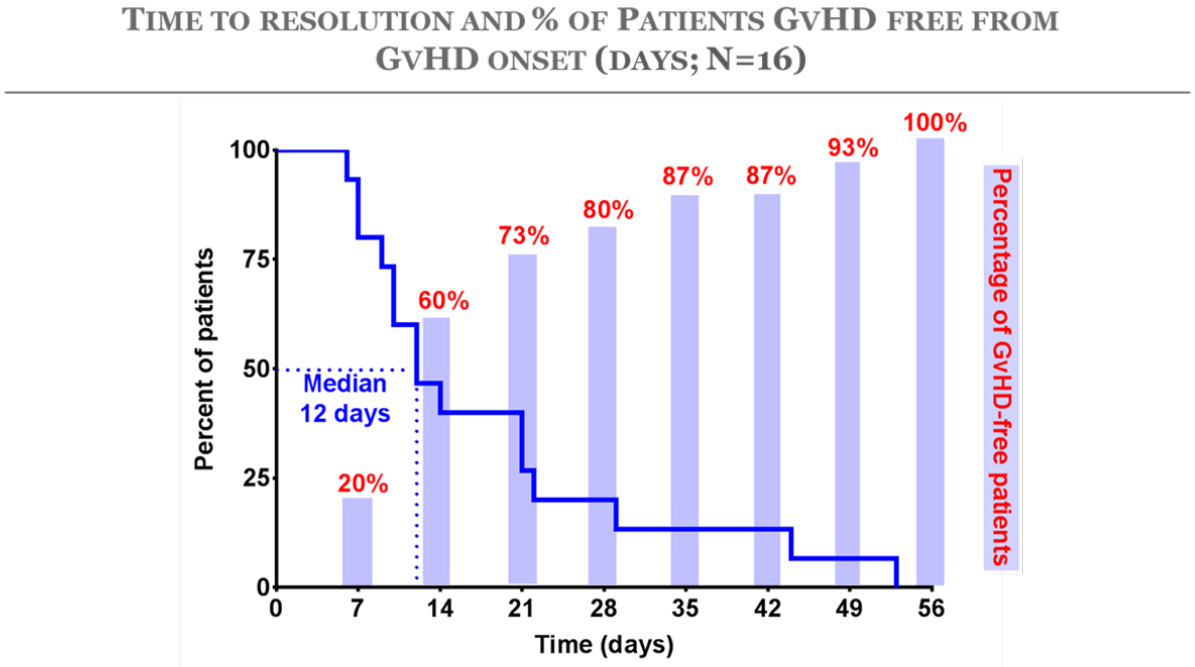
Product/ Therapy	*Indication*	*Disc/ Feas*	*Precl*	*PhI/II*	*PhIII*	*Market*
Zalmoxis®	Haplo identical Transplant in Hematological Malignancies	→	→	→	→	EU CMA
CAR-T CD44v6 hematological tumor	Acute Myeloid Leukemia; Multiple Myeloma	→	→			**AUTOLOGOUS**
CAR-T CD44v6 Solid tumor	Lung, breast adenocarcinomas, head and neck, ovary carcinomas	→				
CAR-T (2 undisclosed targets)	Undisclosed	→				
Allogeneic CAR-NK (3 undisclosed targets)	Undisclosed	→				**ALLOGENEIC**

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**

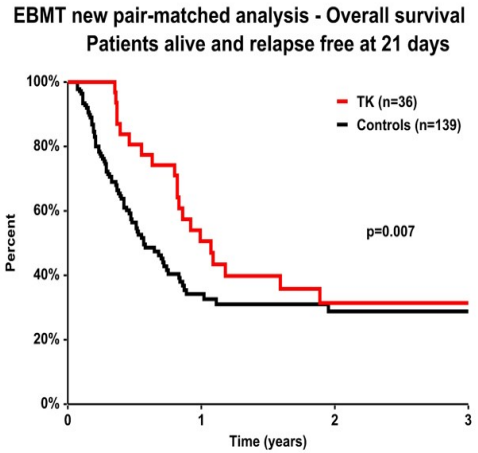


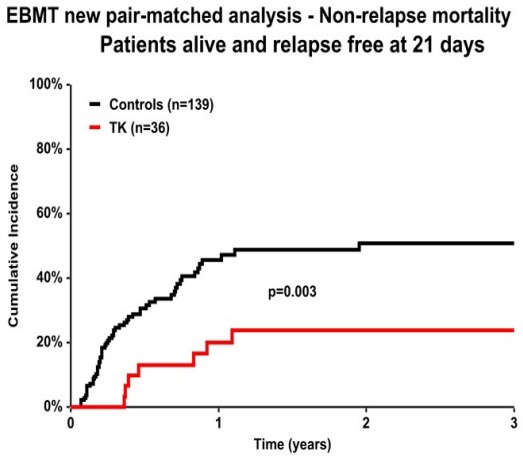
Zalmoxis® efficacy: 100% of acute GvHD resolution

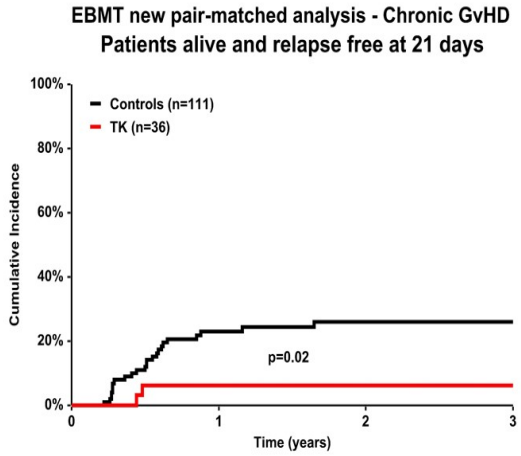


Note: Pulled data from TK007 and TK008 (experimental arm)
Source: ASH Meeting 2014, Abs. 2535

Zalmoxis®: clinical outcome as additional life saving therapy

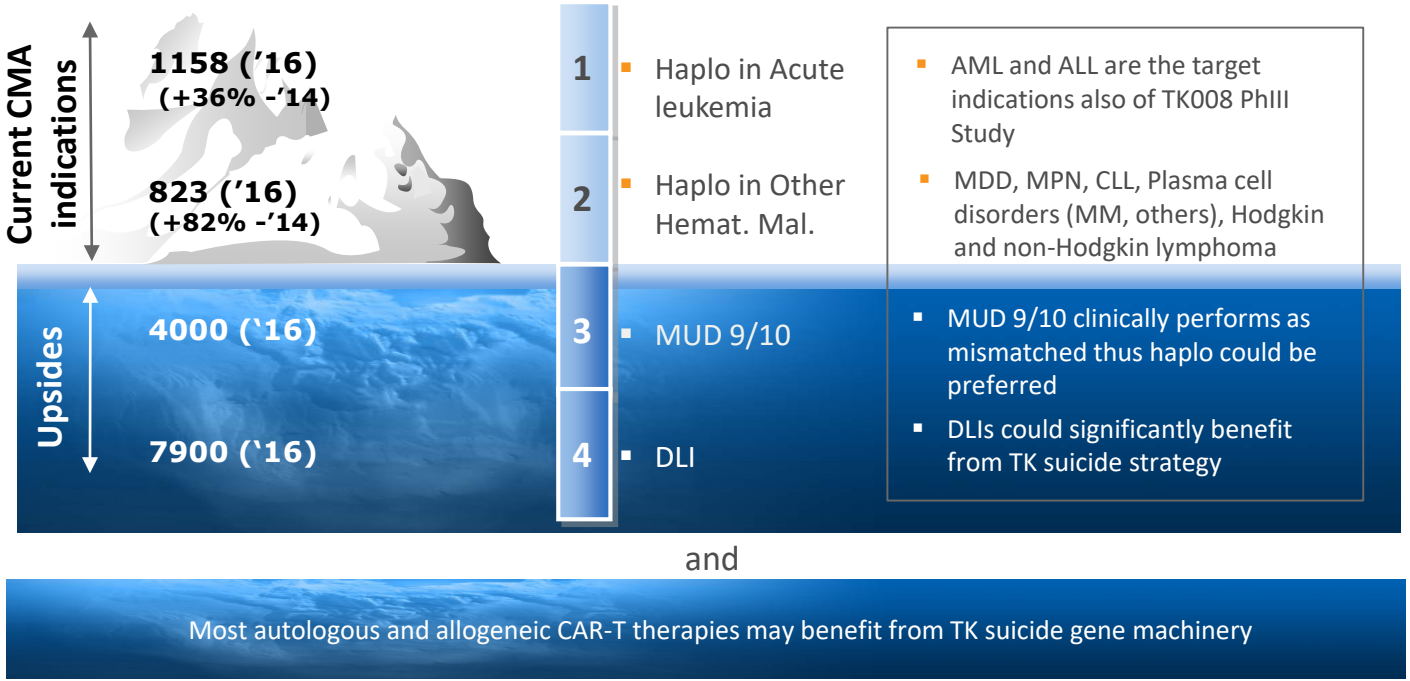






New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	46%	34%	23%
Zalmoxis (n=36)	20%	51%	6%
*p-value^*	*0.003*	*0.007*	*0.02*

European target population: haploidentical transplants



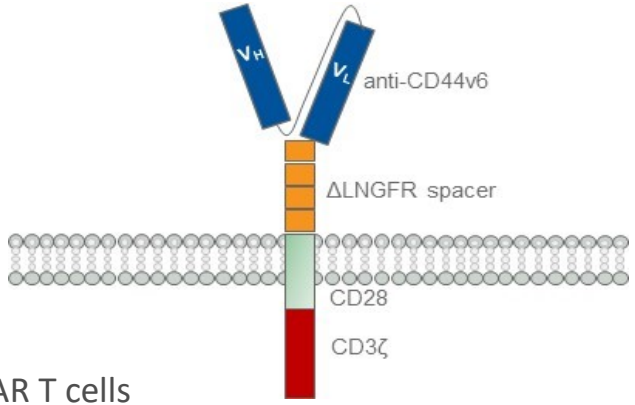
Source: Company and EBMT (Passweg J et al ,Bone Marrow Transpl 2018)

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

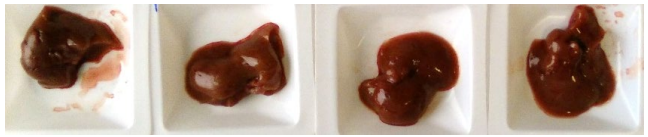
Day 1
AML

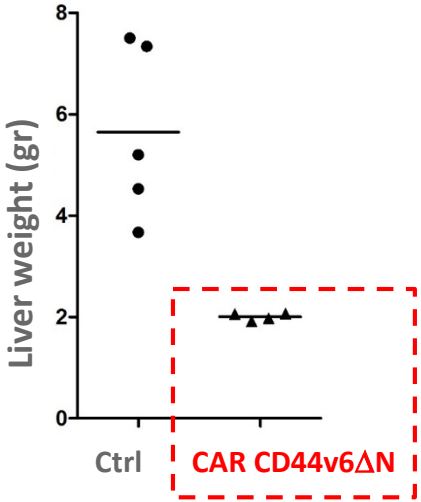
Day 15 Day 16
T cells T cells

Day 40
Sacrifice

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

CD44v6

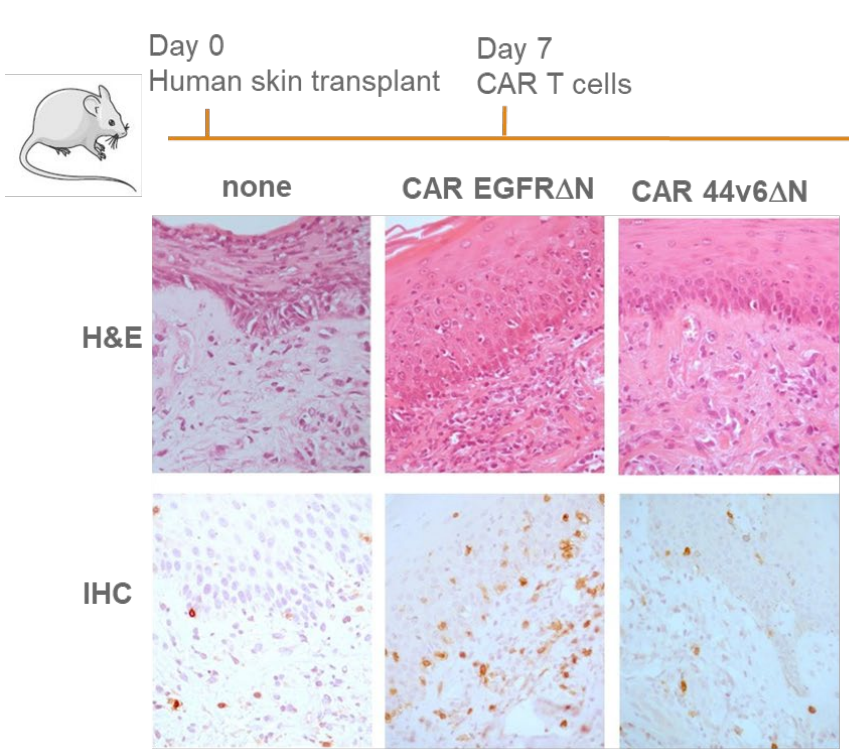


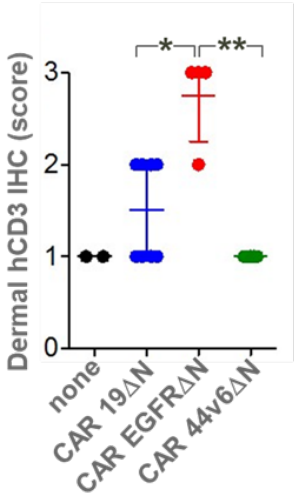


MolMed published results

MOLMED

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-center, 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)

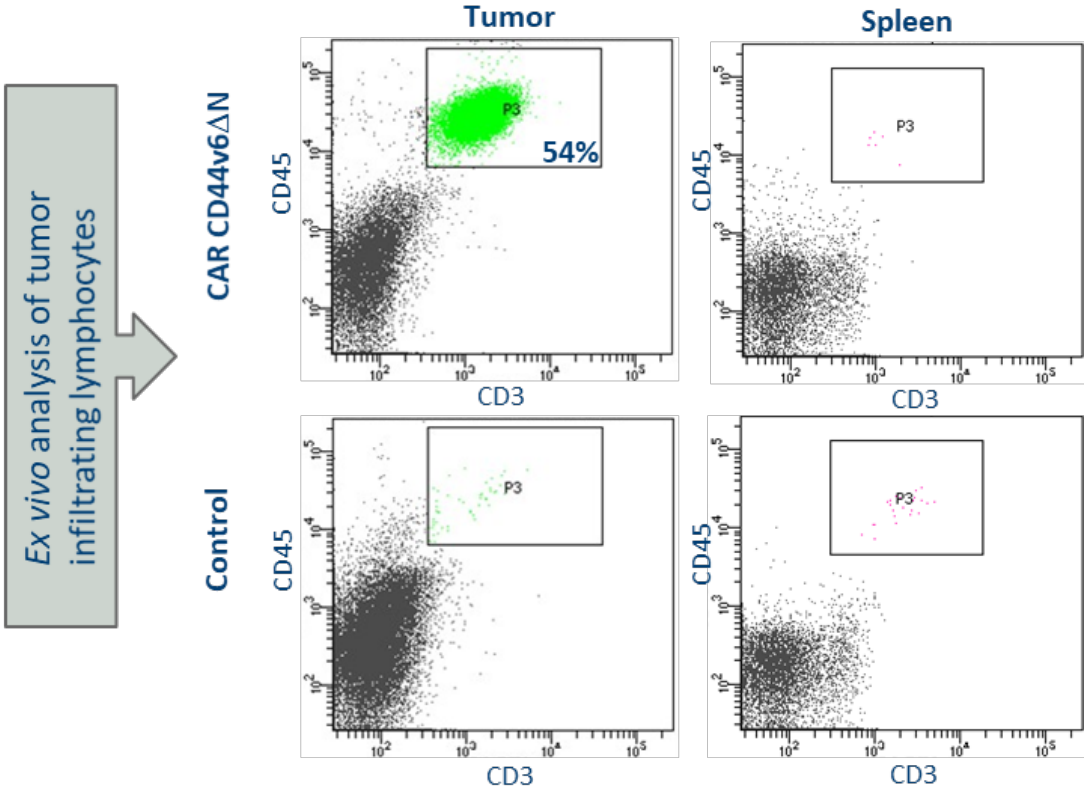
- In the last few years, novel drugs have improved both progression free (up to ~30 months) and median overall survival (~4.7 years) (*Warren et al., 2013; San Miguel et al., 2008*).
- Despite these recent achievements with novel drugs in the treatment of MM, duration of response decreases with successive 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**

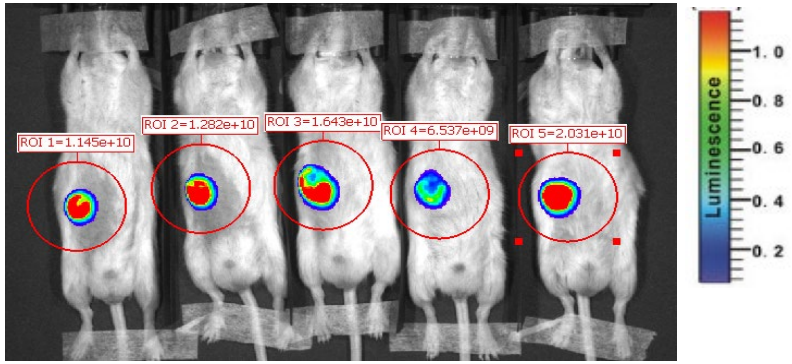
STRONG RATIONALE TO DESIGN AND IMPLEMENT A BASKET TRIAL BY 3Q 2019

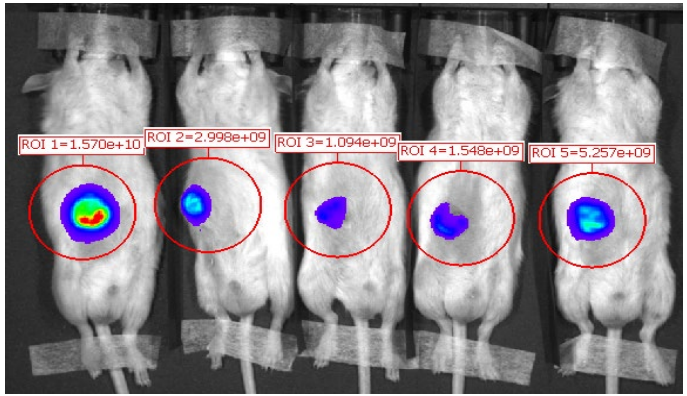
CD44v6 in vivo activity in solid tumors: a human lung adenocarcinoma model

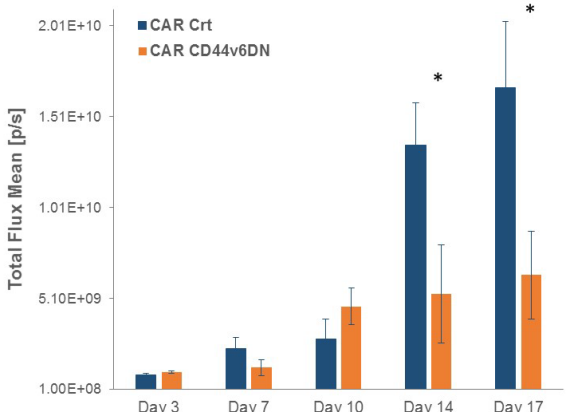


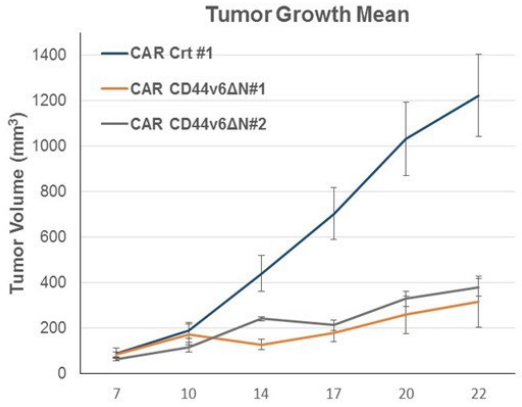
CAR T CD44v& highly infiltrates the tumor

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





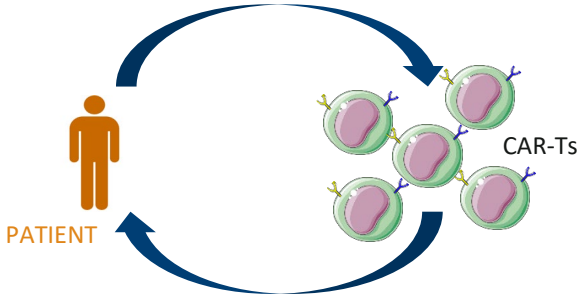




MolMed approach to develop new-generation CAR therapies proprietary pipeline

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

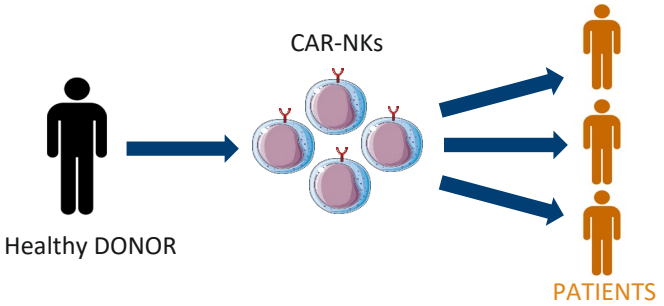
Autologous CAR-T Platform



- **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***

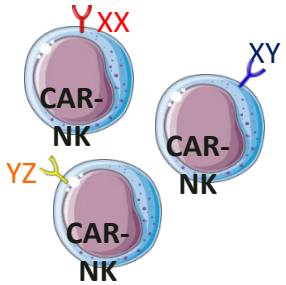
MolMed criteria to select new targets for autologous CAR T therapies

- ✓ 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**

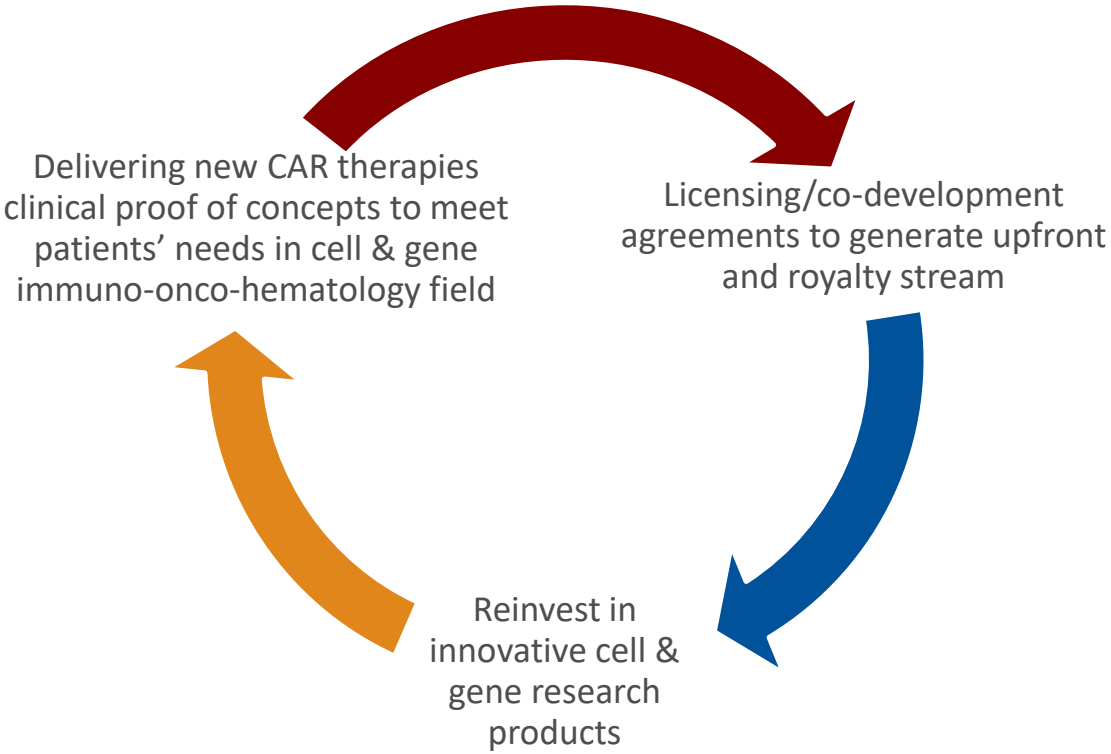
MolMed approach to select a new allogeneic CAR platform

- ✓ 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



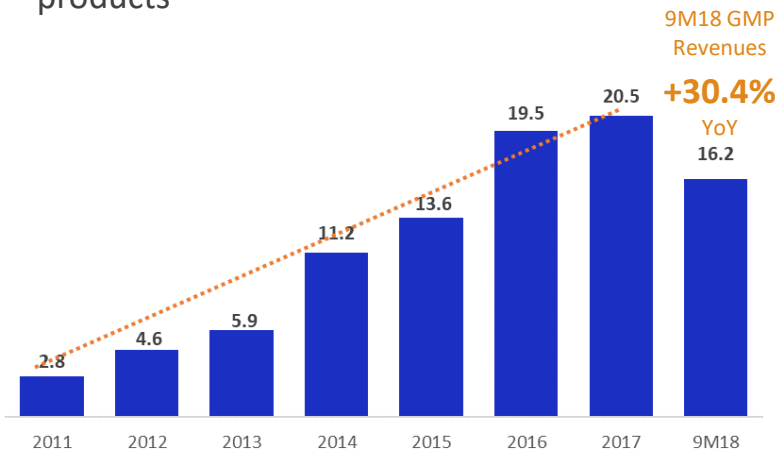
MolMed' CARs platform strategic vision



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



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*	*PhI/II*	*PhIII*	*Market*
Zalmoxis®	Haplo identical Transplant in Hematological Malignancies	→	→	→	→	EU CMA
CAR-T CD44v6 hematological tumor	Acute Myeloid Leukemia; Multiple Myeloma	→	→			**AUTOLOGOUS**
CAR-T CD44v6 Solid tumor	Lung, breast adenocarcinomas, head and neck, ovary carcinomas	→	→			
CAR-T (2 undisclosed targets)	Undisclosed	→				
Allogeneic CAR-NK (3 undisclosed targets)	Undisclosed	→				**ALLOGENEIC**

MOLMED

Solid financial position with continuously improving trend (9M18 vs 9M17)

- **Total 9M18 Revenues** of 20.0€ M, with Revenues from sales equal to Euro 19.4€ M, increased by 30.1% compared to 9M17
- **Operating and Net Results** considerably improved by 41.5% and 40% respectively, compared to 9M17
- **MolMed evergreen losses carried forward** amounting to about 204 Euro/million

€/000	9M18	9M17	Δ 9M18 vs 9M17 €	Δ 9M18 vs 9M17 %	FY17
Operating Revenues	**20,012**	**15,641**	***4,371***	*27.9%*	**23,987**
Revenues from sales	19,436	14,936	*4,500*	*30.1%*	23,000
Other operating income	576	705	*(129)*	*(18.3%)*	987
Operating costs	(24,640)	(23,549)	*1,091*	*4.6%*	32,135
Operating Result	**(4,628)**	**(7,908)**	***3,280***	***41.5%***	**(8,148)**
Net Result	**(4,873)**	**(8,102)**	***3,229***	***40.0%***	**(8,497)**
Net Financial Position(*)	**15,757**				**18,111**

- **Financial Position** equal to 15.7€ M cash and cash equivalents and current financial receivables, with no financial debt

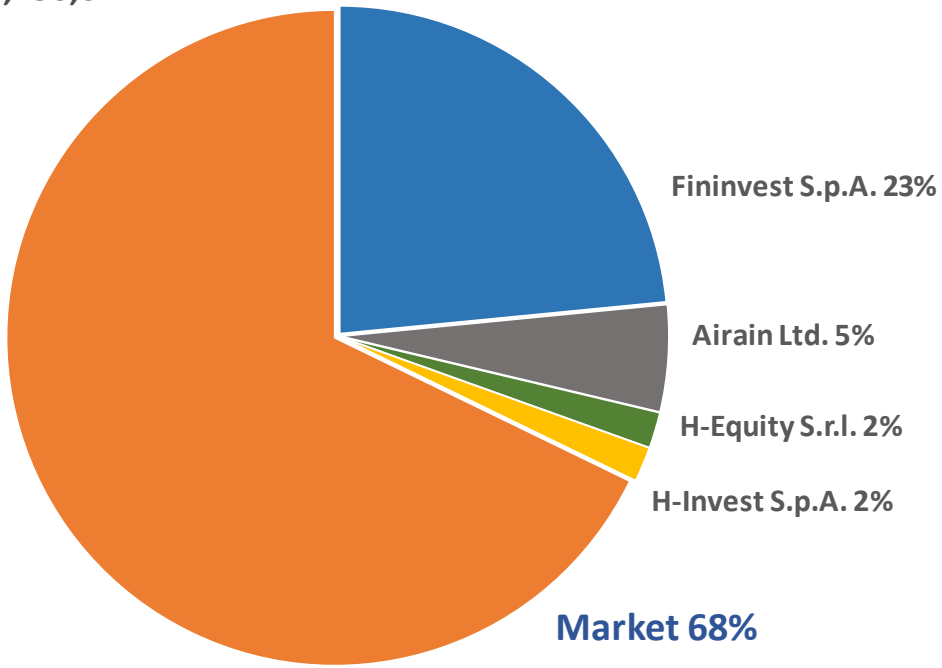
Financial results improved significantly in the last three years

- **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***	

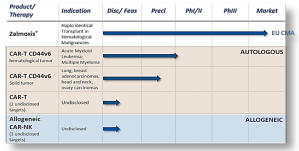
Shareholders' structure

- MolMed is listed on the main market (MTA) of the **Milan Stock Exchange** since 2008 (**MLMD.MI**)
- **Market cap ~ 140M €** (as of November 13th, 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

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