MolMed S.p.A.
Company Overview

Leading the way in Cell & Gene therapy

March, 2018

Agenda

Bio-pharma scenario

- ✓ *The International Cell & Gene Industry*
- ✓ *The Cell & Gene momentum*

MolMed

- ✓ *The Company today: activities and results*
- ✓ *Financial Highlights*
- ✓ *Key Strenghts*
- ✓ *Shareholders structure*

MOLMED

Agenda

Bio-pharma scenario

- ✓ *The International Cell & Gene Industry*
- ✓ *The Cell & Gene momentum*

MolMed

- ✓ *The Company today: activities and results*
- ✓ *Financial Highlights*
- ✓ *Key Strenghts*
- ✓ *Shareholders structure*

MOLMED

Advanced Therapies: Current Global Sector Landscape

* *Source: 2017 ARM Industry Survey*

MOLMED

Advanced therapies: worldwide clinical trials overview

946
Clinical trials underway worldwide by end of 2017

Ph. I: 314
Ph. II: 550
Ph. III: 82

Number of Clinical Trials Utilizing Specific RM/AT Technology: 2017

GENE THERAPY

Total: 313
Ph. I: 113
Ph. II: 170
Ph. III: 30

GENE-MODIFIED CELL THERAPY

Total: 259
Ph. I: 106
Ph. II: 144
Ph. III: 9

CELL THERAPY

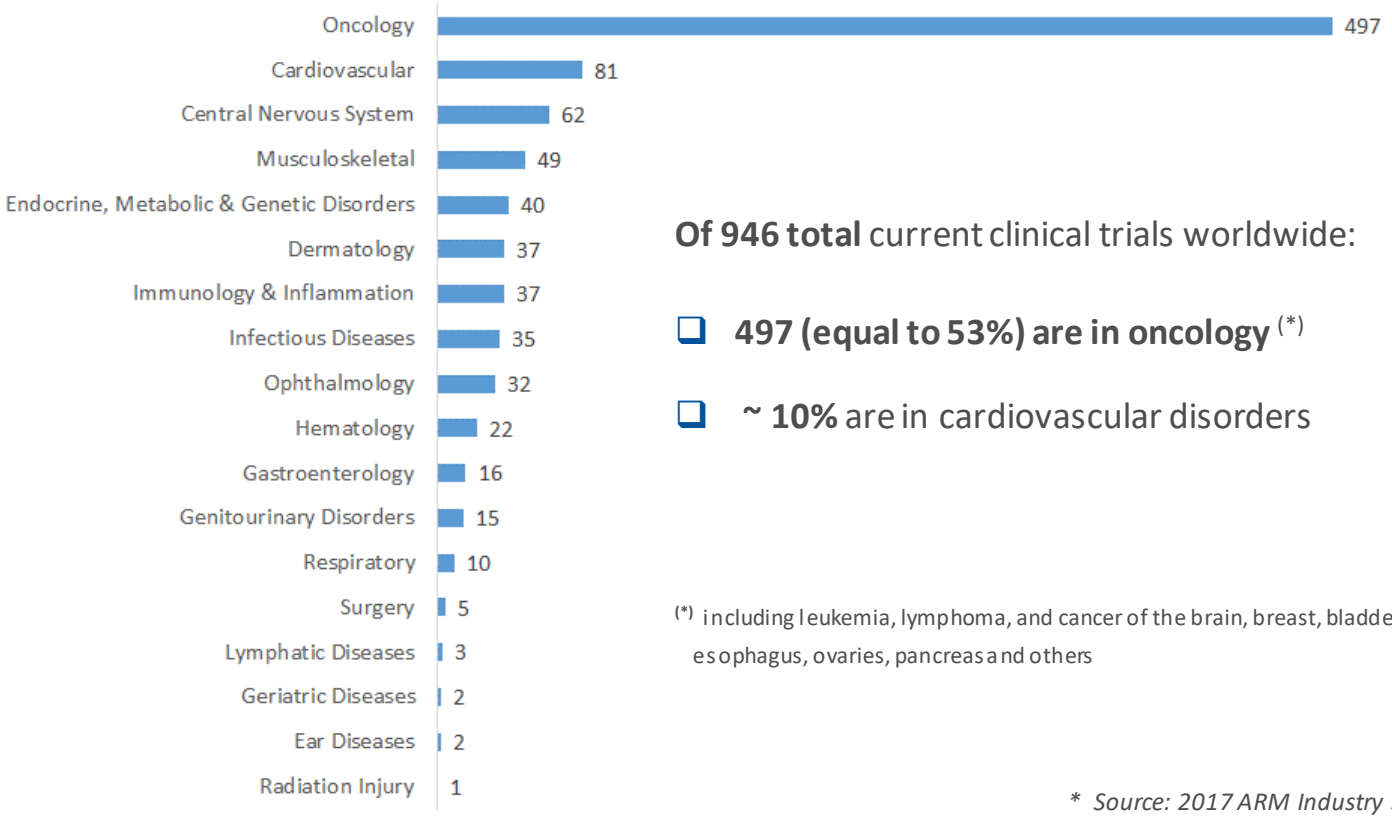
Total: 353
Ph. I: 90
Ph. II: 225
Ph. III: 38

TISSUE ENGINEERING

Total: 21
Ph. I: 5
Ph. II: 11
Ph. III: 5

* *Source: 2017 ARM Industry Survey*

Advanced therapies: clinical trials by indications in 2017



Of 946 total current clinical trials worldwide:

- **497 (equal to 53%) are in oncology** (*)
- **~ 10%** are in cardiovascular disorders

(*) including leukemia, lymphoma, and cancer of the brain, breast, bladder, cervix, colon, esophagus, ovaries, pancreas and others

* *Source: 2017 ARM Industry Survey*

Cell & Gene: key approvals and prices in 2017

FDA

NOVARTIS	*Aug 2017*	**Kymriah CAR T cell therapy** for children and young adults with ALL **475,000 USD**
Kite Pharma	*Oct 2017*	**Yescarta CAR T cell therapy** for the tadult patients with relapsed/refractory large B cell lymphoma after two or more lines of systemic therapy **373,000 USD**
Spark THERAPEUTICS	*Dec 2017*	**Luxturna gene therapy** for biallelic RPE65-mediated inherited retinal disease **425,000 USD/eye**

EMA

TiGENIX Living Medicines	*Dec 2017*	**TiGenix's Cx601 allogeneic cell therapy** for treatment of Crohn's received EMA CHMP endorsement

Cell & Gene: MolMed approved proprietary and CDMO products and prices

EMA

gsk	*July 2016*	**STRIMVELIS** gene therapy * for the treatment of ADA-SCID: EU market authorization **Reimbursed Price in Italy: 594,000 €**
MOLMED	*Aug 2016*	**Zalmoxis** patient-specific cell therapy obtained the EU market authorization form **Reimbursed price per infusion in Italy: 149,000 €** (MolMed negotiated a *flat price per patient*, considering a number of infusions between 1 and 4)

MOLMED * MolMed developed and manufactures Gsk' Strimvelis

Cell & Gene : 2017 and early 2018 momentum

M&A

GILEAD / Kite Pharma	*Oct 2017*	**Gilead Sciences** acquired **Kite Pharma**, Inc. for **11.9 USD Bn**
SANOFI / Bioverativ	*Jan 2018*	**Sanofi SA** agreed to buy **Bioverativ Inc.** (biotech spinoff of giant Biogen) to gain treatments for rare blood disorders for approx. **11.6 USD Bn**
Celgene / JUNO THERAPEUTICS	*Jan 2018*	**Celgene Corporation** acquired **Juno Therapeutics**, Inc. advancing Global Leadership in cellular Immunotherapy for approx. **9 USD Bn**

Partnership agreements

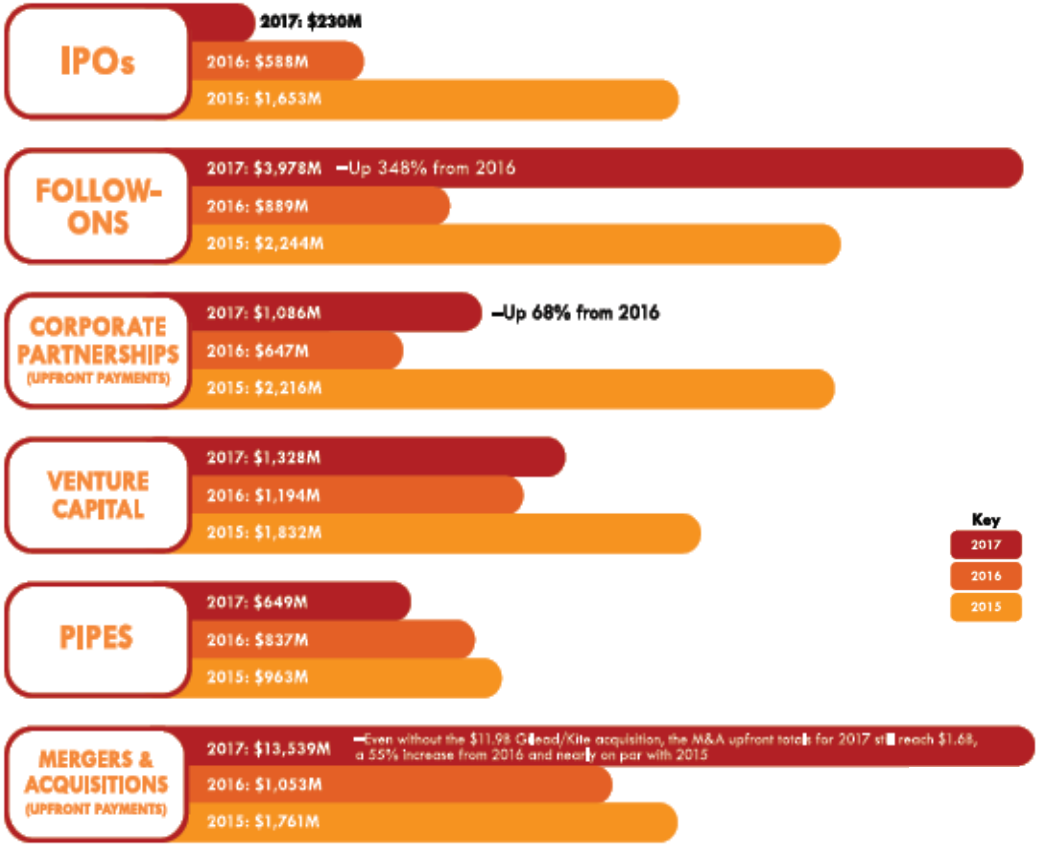
Pfizer / Sangamo	*May 2017*	**Pfizer** signs with **Sangamo Therapeutics** a collaboration for the hemophilia A gene therapy **545 USD M**
gsk / Adaptimmune	*Sep 2017*	**GlaxoSmithKline** licensed the rights to **Adaptimmune's** pioneering T-cell therapy program **63 USD M**
Janssen / Legend Biotech	*Dec 2017*	**Janssen Biotech e**nters into a global collaboration with **Legend Biotech** to develop, manufacture and market **Legend's** CAR T-cell therapy for multiple myeloma **350 USD M**

Cell & Gene: total 2017 Global Financing

TOTAL 2017 GLOBAL FINANCINGS

$7.5 Billion raised in 2017

78.5% increase from 2016



Source: 2017 ARM Industry Survey

*** Figures do not include M&A transactions*

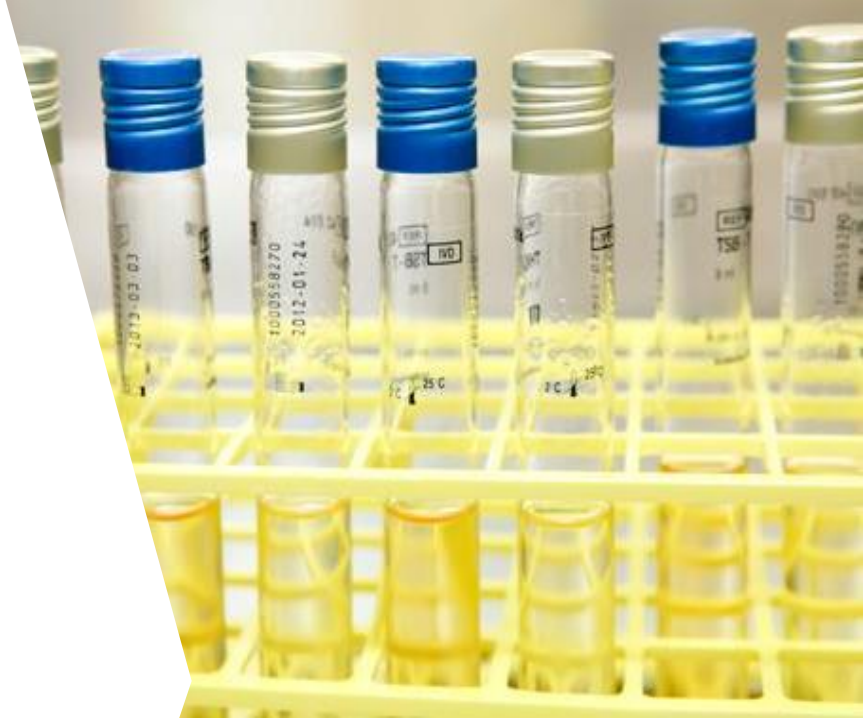
Agenda

Bio-pharma scenario

- ✓ *The International Cell & Gene Industry*
- ✓ *The Cell & Gene momentum*

MolMed

- ✓ *The Company today: activities and results*
- ✓ *Financial Highlights*
- ✓ *Key Strenghts*
- ✓ *Shareholders structure*



MOLMED

MolMed: an established dual business model leveraging common technological assets

Common Assets

- **20+ year experience** in Cell & Gene Therapy Development and Manufacturing
- **140+ highly qualified scientist** and operators plus ~40 support staff
- 12 proprietary patent families including **256 granted patents** and 43 pending applications
- **2 GMP manufacturing facilities** of almost 5.000SQM, manufacturing of the **only 2** *ex-vivo* gene therapy products **approved in EU**
- **Strategical and commercial partnership** with primary big players of EU and US bio-pharma industry

Proprietary Pipeline

Research, development, manufacturing and clinical validation of proprietary products

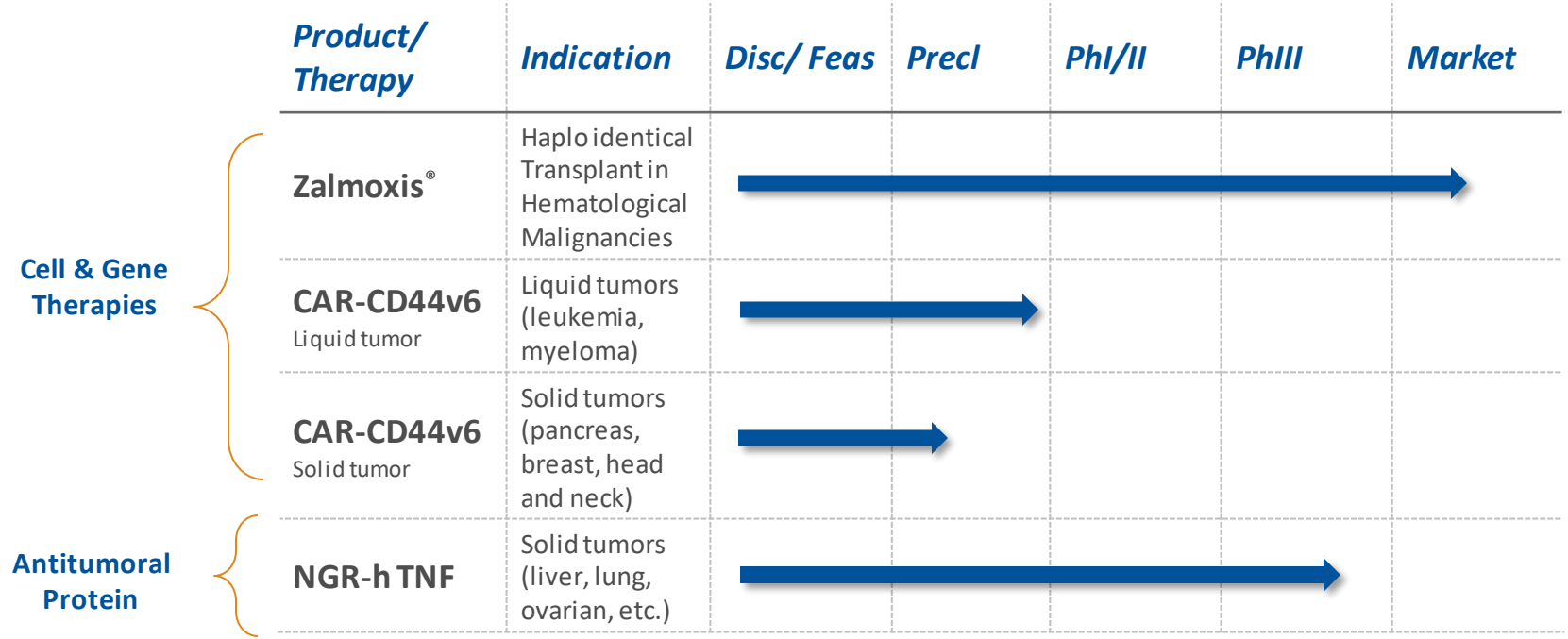
GMP Solutions (CDMO *)

Development and manufacturing for 3rd parties

* Contract Development and Manufacturing Organization

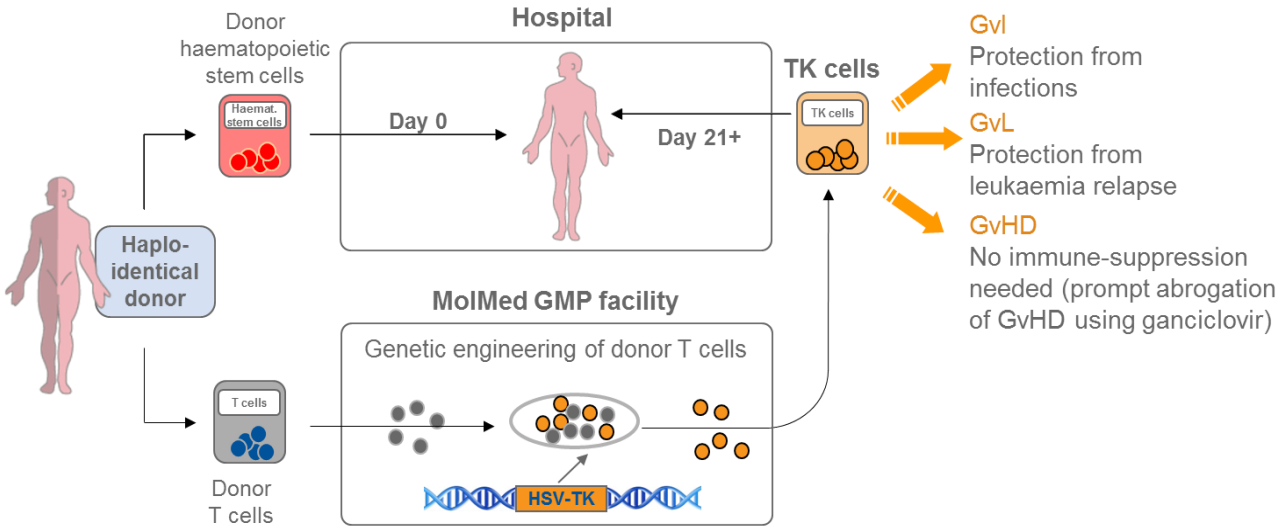
MolMed proprietary pipeline

MolMed product portfolio includes **proprietary anti-tumor therapies** in **clinical and preclinical** development:



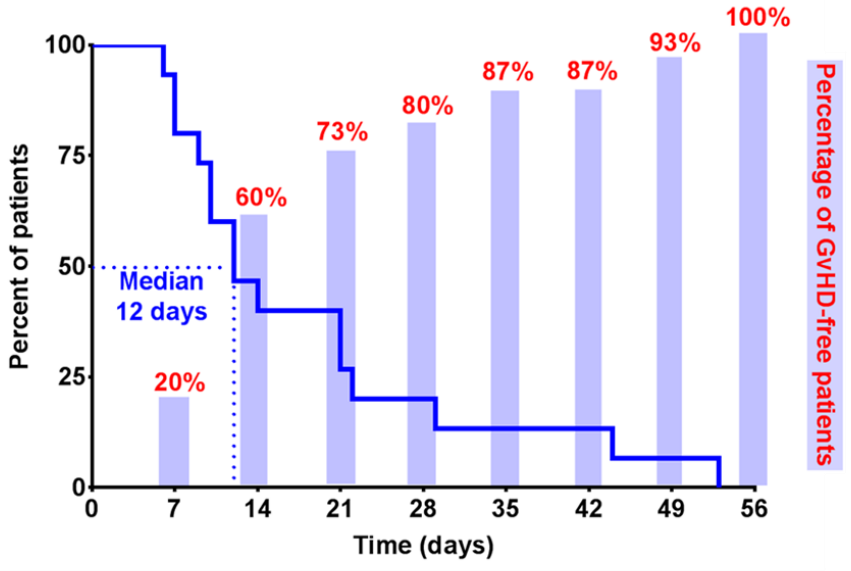
Zalmoxis®: addressing the limits of partially compatible stem cell transplantation

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**



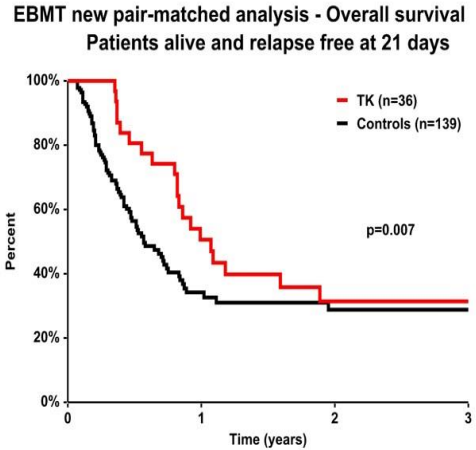
Clinical safety of Zalmoxis®: 100% of acute GvHD resolution

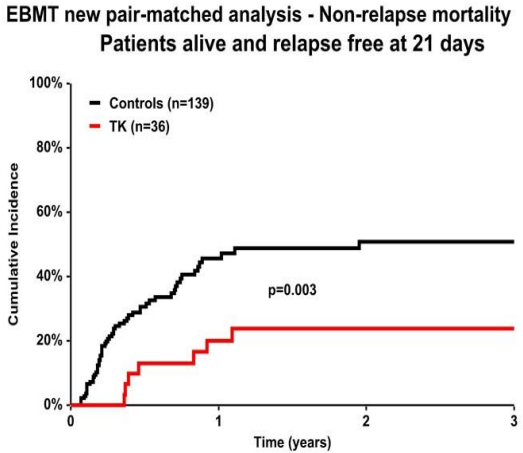
TIME TO RESOLUTION AND % OF PATIENTS GVHD FREE FROM GVHD ONSET (DAYS; N=16)

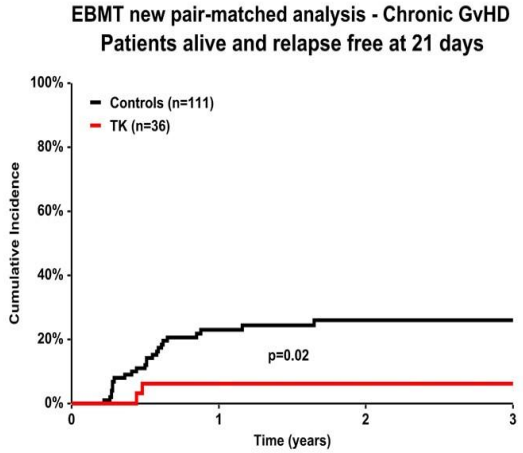


Note: Pulled data from TK007 and TK008 (experimental arm)
Source: ASH Meeting 2014, Abs. 2535

Zalmoxis®: clinical outcome

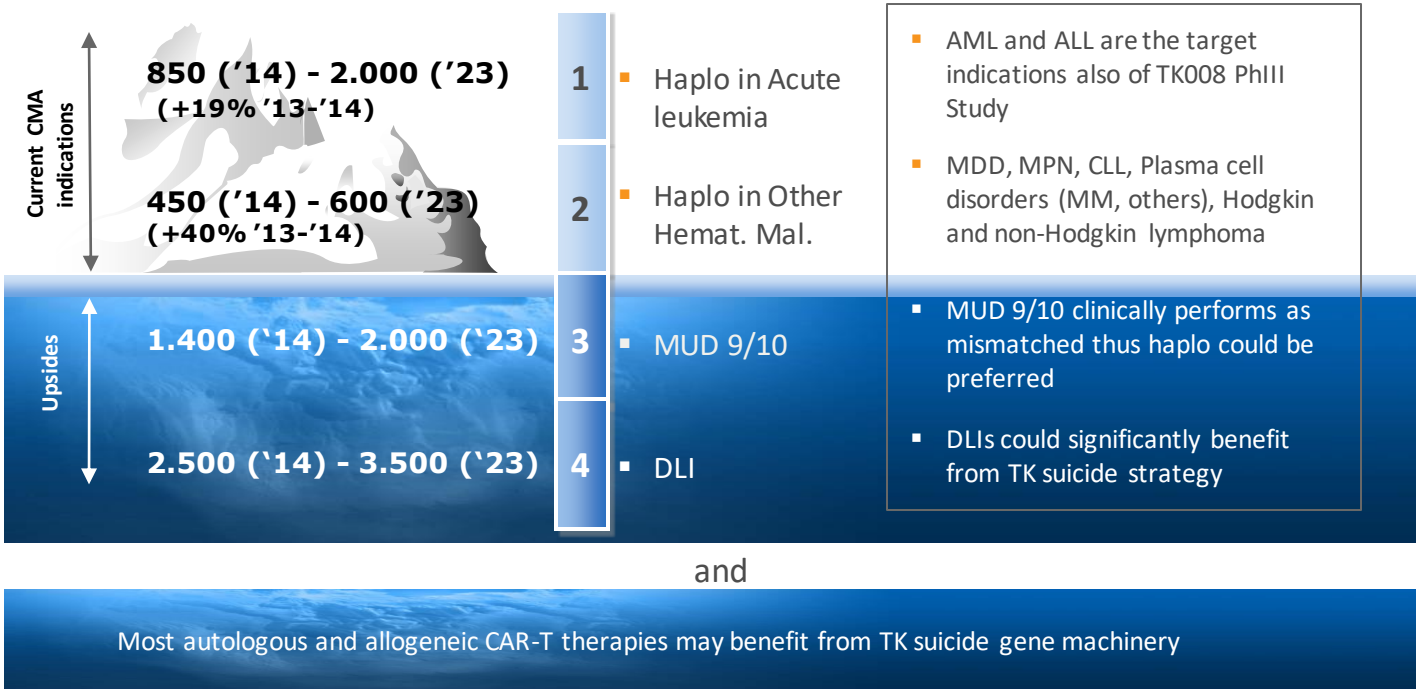






New pair-matched analysis 1-year outcomes Alive and relapse free at 21 days	Non-relapse mortality (NRM)	Overall survival (OS)	Chronic GvHD
Controls (n=139)	46%	34%	23%
Zalmoxis (n=36)	20%	51%	6%
*p-value^*	*0.003*	*0.007*	*0.02*

European market potential analysis: strong growth and relevant upsides



Source: Company and EBMT 2016

MOLMED

Zalmoxis® journey

European Commission CMA
(Conditional Marketing Authorization)
- **Robust Core Value Dossier**
- **Clear pricing policy and corridor**

Aug 2016

MolMed - Dompé
Strategic commercialization and Supply Agreements for **Europe**
Upfront + Milestones: 43.5M€
Royalties included in transfer price

Jul 2017

Exclusive commercial agreement
- TTY Biopharm **(Asian countries)**

Upfront + Milestones: 13.5M€
- TTY Biopharm **(Israel)**

Apr and Jun 2017

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

Dec 2017

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

Jan 2018

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

Feb 2018

Next Steps

UNDER CONSIDERATION

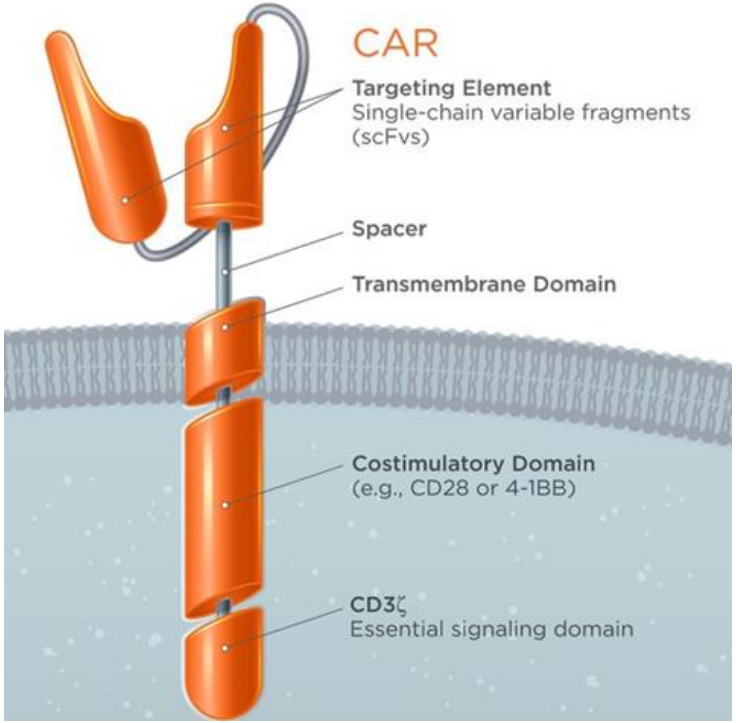
FDA Marketing Authorization

ONGOING

Price/ Marketing Authorization

* *P&R: Pricing & Reimbursement*

CAR T: a new powerfull anti-cancer strategy



CAR T Cells (Chimeric Antigen Receptors Cells):
Artificial receptor combining antibody specificity with lymphocytes efficacy.

In 2017, two CAR T-cell therapies were approved by the FDA: one for the treatment of children with acute lymphoblastic leukemia (ALL) and the other for adults with advanced lymphomas.

Nevertheless, researchers question about whether they will ever be effective against solid tumors like breast and colorectal cancer.

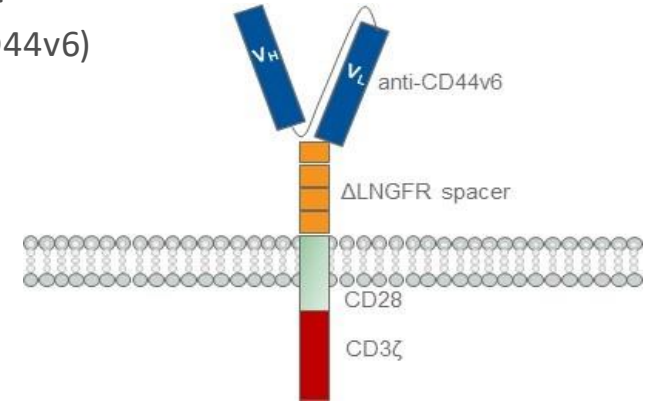
Source: NIH National Cancer Institute

MolMed CAR T CD44v6 uniqueness

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

- **High therapeutic potential also in several solid tumors**, as it specifically recognizes variant 6 (v6) of the antigen CD44 (CD44v6)
- **Specifically infiltrating** the tumor
- **High safety profile** (low skin toxicity)



CD44v6 is expressed by several blood and 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 (liquid tumor)**
- **Variant v6 of antigen CD44** is over-expressed in several hematological malignancies and solid epithelial tumors:

Liquid Tumor

- 60% of AML*
- 90% of multiple myeloma MM*

Solid Tumor

- breast cancer (triple negative)
- pancreatic adenocarcinoma
- head & neck cancer
- brain tumor stem cells
- colon cancer stem cells

* AML: Acute Myeloid Leukemia; MM: Multiple Myeloma

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

Day 1
AML

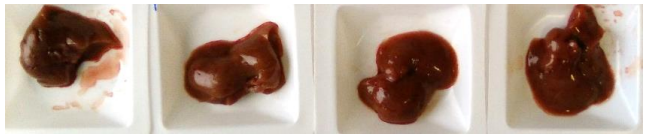
Day 15
T cells

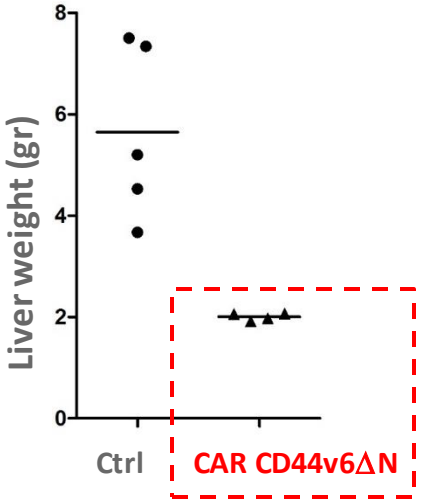
Day 16
T cells

Day 40
Sacrifice

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

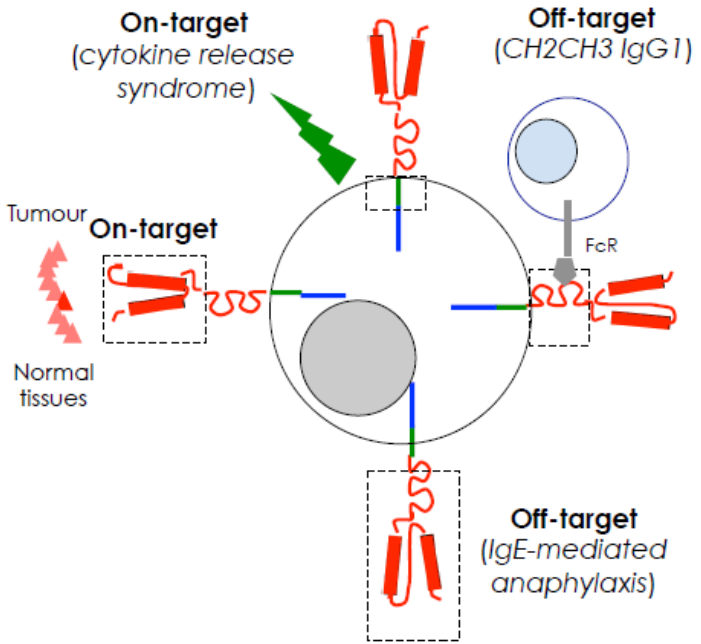
CD44v6





MolMed published results

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



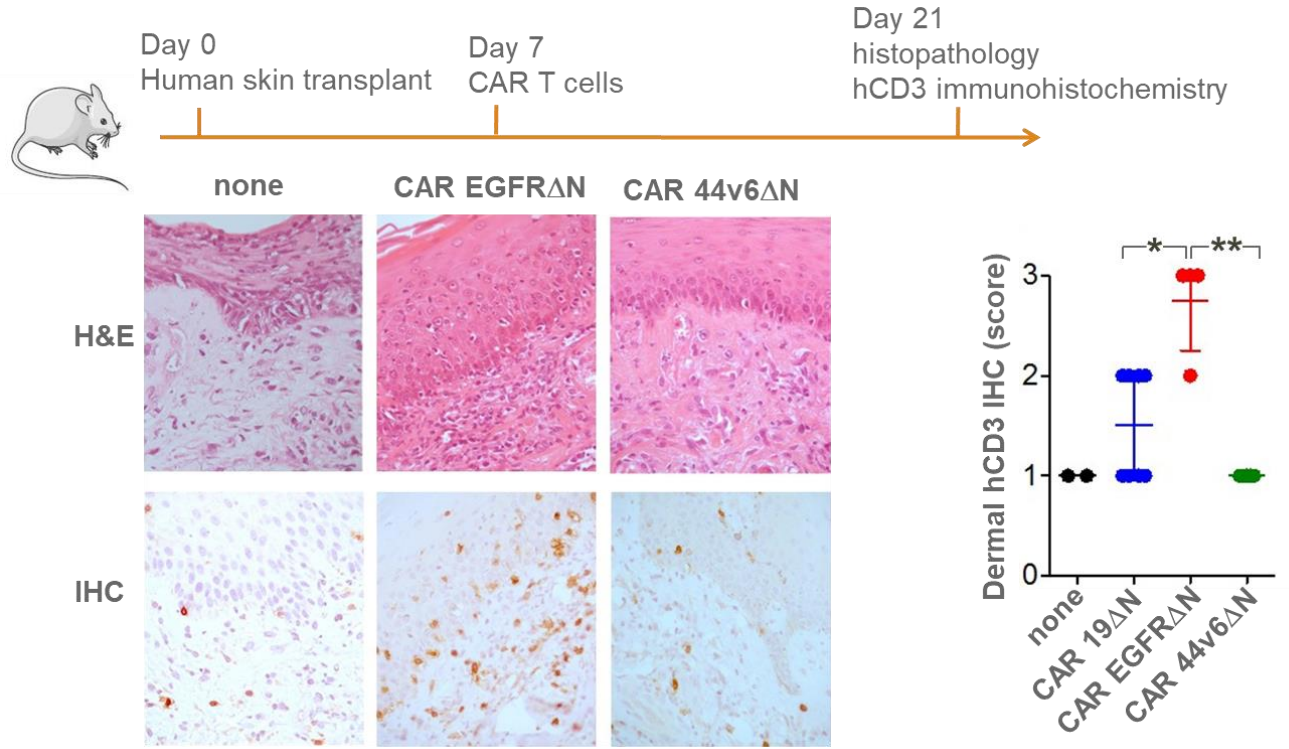
***Source**: Casucci et al, Cancer Immunol Immunother*

OFF-TARGET TOXICITIES

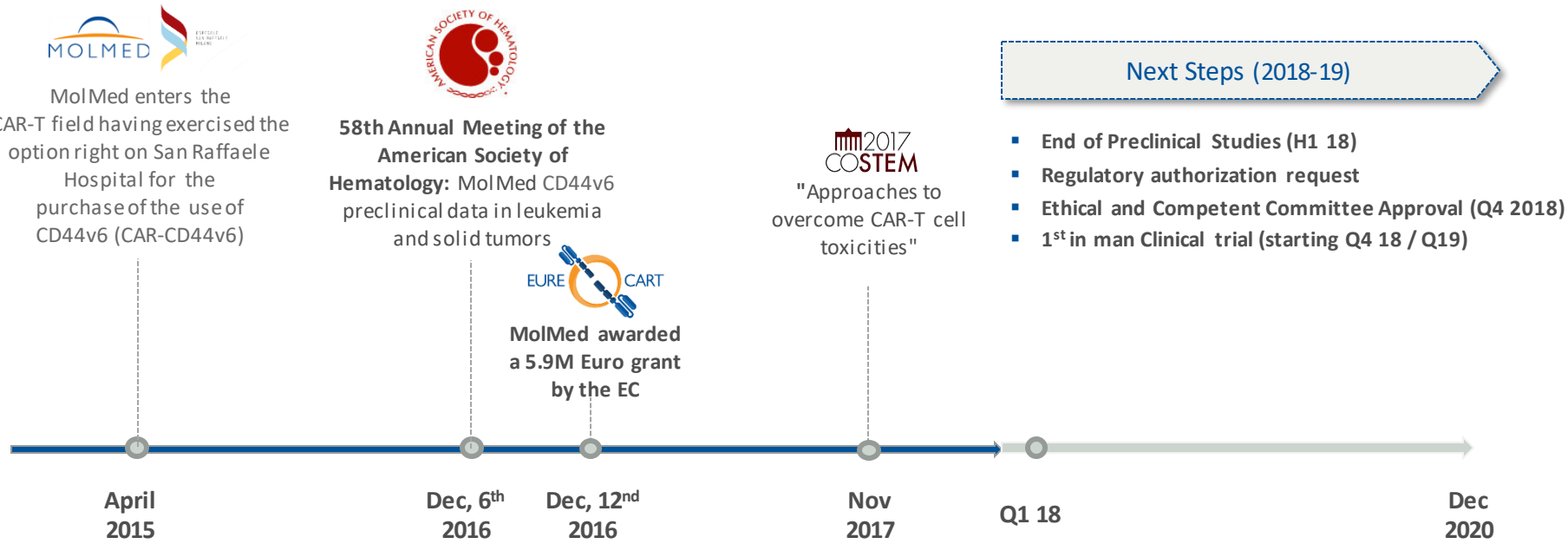
- CD44v6 expression by normal cells
- Cytokine release syndrome
- Non-specific spacer-mediated activation
- CAR-CD44v6 expression on effector cells

Use in combination with TK suicide gene

Low skin toxicity: EGFR CAR-T cells, but not CD44v6 CAR-T cells significantly infiltrate the skin



CAR T - CD44v6 journey



MolMed leads the EU-funded EURE-CART project

EURE-CART brings together clinical experts in oncology, and pioneers and leaders in the field of Cell & Gene therapy to conduct a **multi-centre, first-in-man Phase I/II clinical trial to demonstrate the safety and the efficacy of CAR-CD44v6 T-cell immunotherapy in acute myeloid leukemia and multiple myeloma**

- 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)

ROLE of MolMed: project coordinator and Phase I/II trial SPONSOR

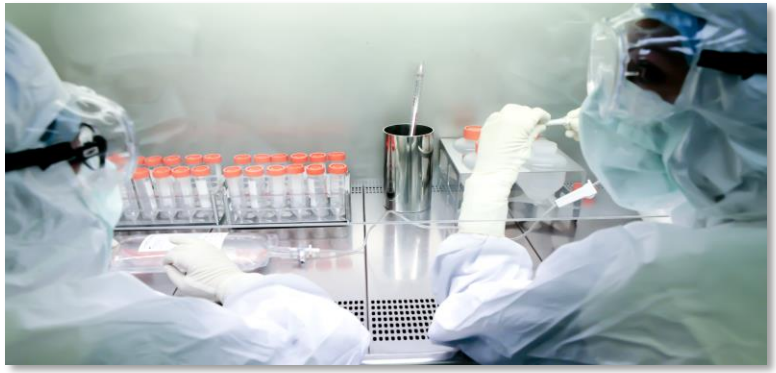
«MolMed Spa is uniquely endowed in the EU with the knowhow and experience necessary to meet this ambitious objective, as demonstrated by its unparalleled track record»

«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»

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)



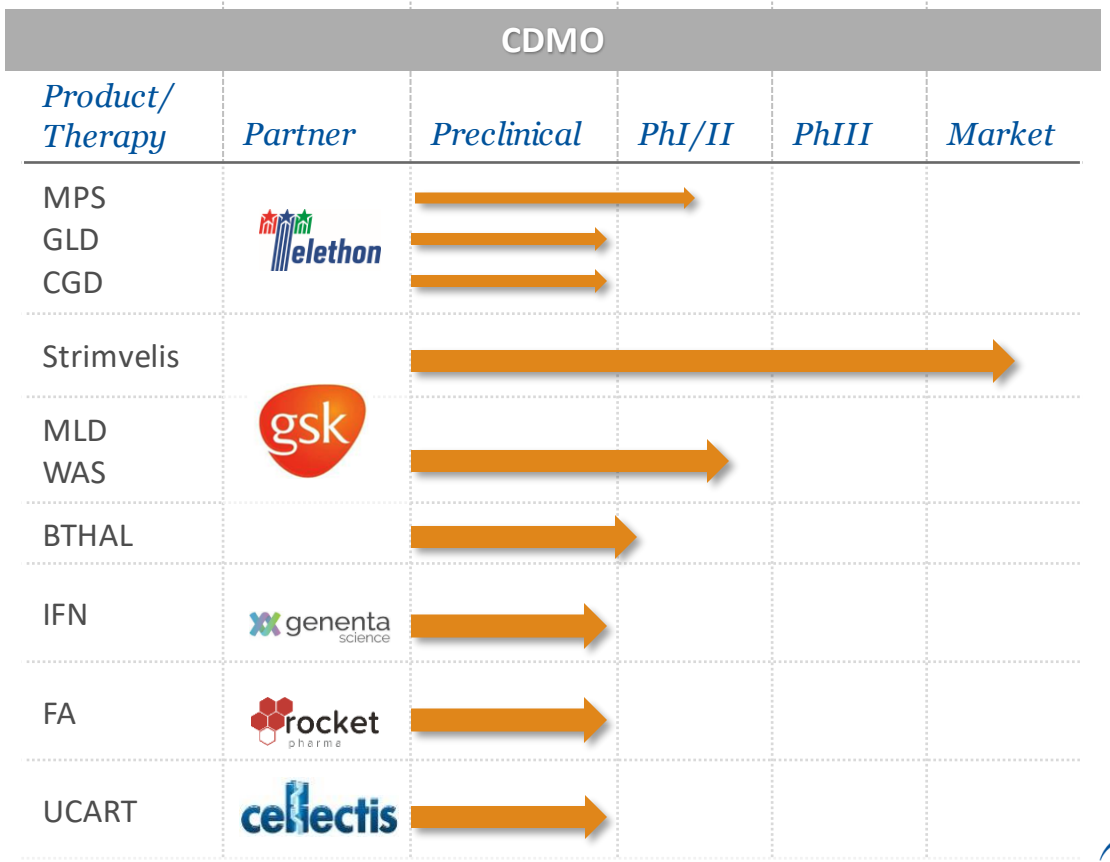
- **1,500 SQM** and **8 grade B/C suites**
- Authorized GMP manufacturing facility since 2003 for **clinical programs**
- Authorized GMP manufacturing facility since 2015 for the **Commercial products**

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 TK cells used in clinical trials

GMP Development and Manufacturing service agreements

GMP clients and partnership portfolio includes **primary European and US biopharmaceutical companies**

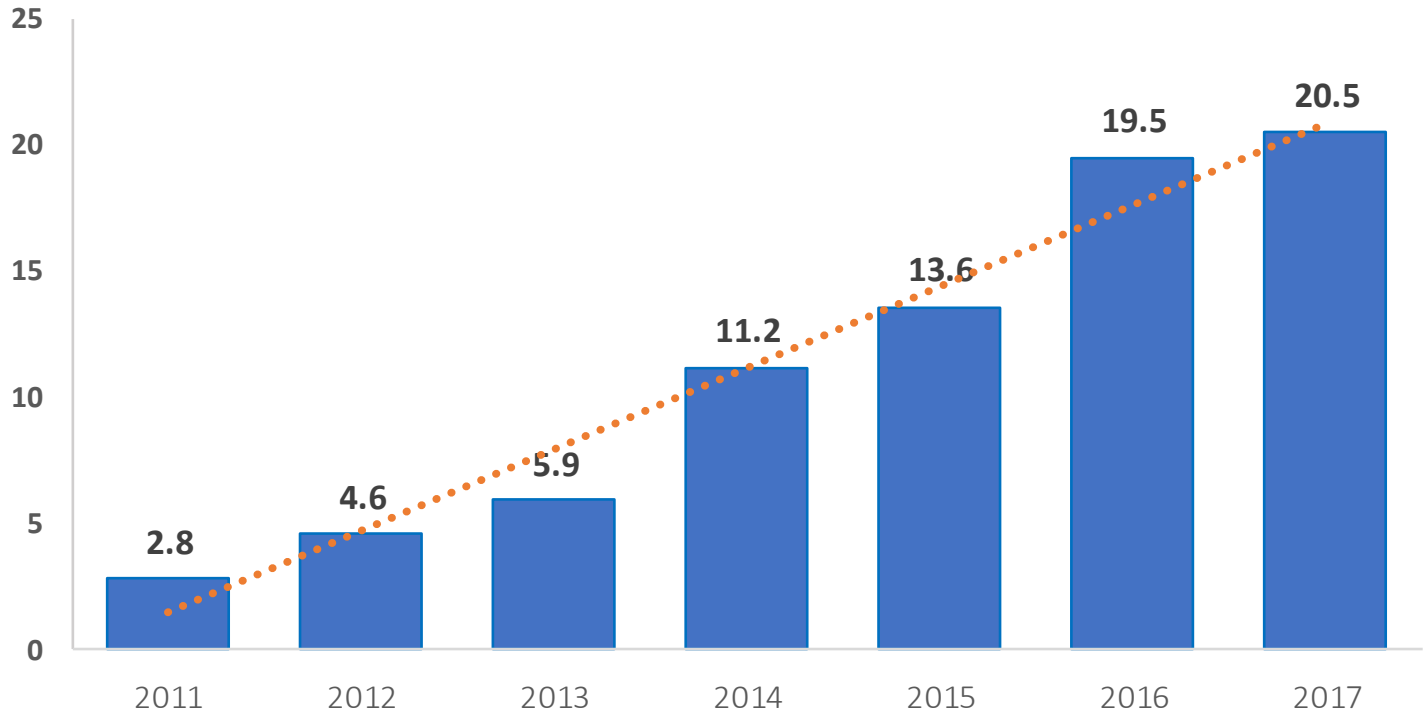


Key Financials: financial results improved significantly in the last three years

- **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***	***9***	

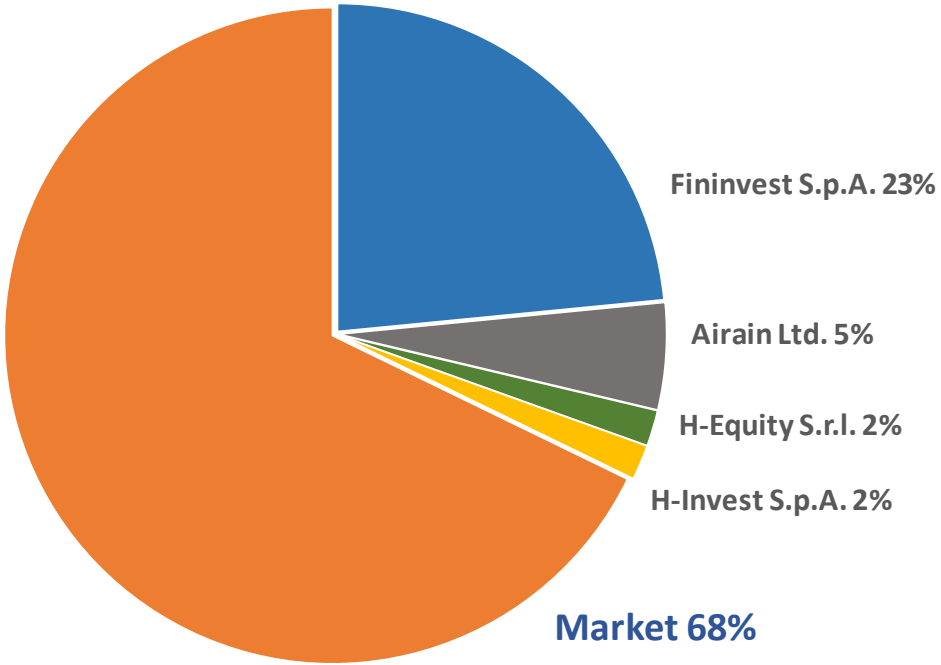
Significant Revenue growth from Development & Manufacturing Services



Shareholders' structure

Market cap ~ 250M € (Feb 16th, 2018)

Outstanding shares 456,962,393



MolMed's Board of Directors

Claudio Bordignon
Founder & Chairman

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

Riccardo Palmisano
CEO

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

Laura Iris Ferro *Independent Director*	Founder Chairman & CEO of the biotech Gentium, she led the company throughout a successfull IPO process on Amex and later on Nasdaq exchange markets
Carlo Incerti *Independent Director*	Head of Global Medical Affairs at Sanofi Genzyme and globally responsible for the medical management in the 4 therapeutic areas: oncology, immunology, multiple sclerosis and rare diseases. President of EuropaBio and board member of IMI (Innovative Medicine Initiative)
Mario Masciocchi *Independent Director*	BoD member and general manager in primary Italian companies in different industries (Montedison, Farmitalia, RCS, Bitron, Rank Xerox Italy; Capgemini Italy, Borbonese)
Elizabeth Robinson *Independent Director*	Ph.D. in biotechnology, also at MIT. Co-founder and president of NicOx S.A. and NicOx Reaserch Institute s.r.l. since January 2006. Since 2016 Investment Director Venture Capital of Quadrivio Capital SGR
Raffaella Ruggiero *Independent Director*	Lawyer Cassationist, working in the civil law, in 2006 she was elected by the Parliament as Judge Associate in the arraignment in front of the Constitutional Court
Didier Trono *Independent Director*	Deputy Director of the Swiss National Science Foundation's "Frontiers in Genetics" area; Professor and Dean of the School of Life Sciences at the Ecóle Polytechnique Fédérale inLausanne
Alfredo Messina	BoD member of the main companies of the I.R.I. group In 1990 he joined the Fininvest group as GM; later he was CEO for the group administration and control area. Currently he holds the positions of director of Mediaset España S.A. and Mondadori S.p.A., and is a consultant for Fininvest SpA He has been a senator of the Italian Republic since April 2008
Alberto Luigi Carletti	CFO of Fininvest S.p.A. and Board Member in other companies of Fininvest Group
Sabina Grossi	BoD and the HR committee member of Luxottica Group S.p.A., formerly Head of IR of the Group (1996 - 2012)

MolMed's Scientific Advisory Board (SAB)

Claudio Bordignon
Chairman

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

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, France

Malcolm K. Brenner

- Director of the Center for Cell and Gene Therapy at the Baylor College of Medicine, Houston, Texas, USA
- Professor of Medicine and of Pediatrics at Fayez S. Sarofim (Baylor College of Medicine), Houston, Texas, USA

Gian Pietro Dotti

Member of the UNC Lineberger Comprehensive Cancer Center
Professor of the Department of Microbiology and Immunology
Director of the UNC Lineberger Immunotherapy Program at the University of North Carolina - Chapel Hill, NC, USA

Miguel-Angel Perales

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

MolMed Key Strenghts

- *International leadership in Cell & Gene industry*
- *2 proprietary Cell & Gene therapies with one, Zalmoxis®, already authorized for the market in Europe and rewarded with valuable reimbursement price in two main EU countries*
- *Recognized GMP capability with the 1st facility in Europe to obtain the GMP manufacturing authorization for the market*
- *Value and Growth dual business model combining a proprietary pipeline with robust source of revenues from GMP services*
- *Established partnerships with primary biopharma international players for both proprietary therapies and GMP products*
- *Corporate Governance with extensive experience from complementary fields and Scientific Advisory Board with strong track record in biotech field*

Contacts

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