

# Development of CD123 CAR T cells against aggressive acute leukemia



1st Immuno-Oncology Research Days March 13 and 14, 2025 CHU Strasbourg



## CARLA Biotherapeutics was founded by entrepreneurs and experts in the key fields of cell therapy development (Hematology, Oncology, Cell Therapy & Drug Development)



## Zaki Sellam MSc, MBA Co-Founder and CEO

>20 years of international experience Creation of several biotech start-ups Several fund-raisings to his credit



Francine Garnache , Pharm.D, PhD
Co-Founder, Board Member
Doctor of Science in Hematology &
ImmunotherapyExpert in CD123+ pathologies in blood cancers



Mehdi Chelbi, MSc Co-Founder, Board member, COO

One patent and over 60 publications

20 years' industry experience Seasoned pharmaceutical industry professional CEO and co-founder of several healthcare companies



#### Olivier Adotevi, MD, PhD Co-Founder, Board Member Medical Expert

Doctor of Science in Immunology & Oncology Heads UMR Right (130 researchers)

Has led and directs several international clinical programs devoted to cancer immunotherapies - several patents and numerous publications



## Fanny Angelot-Delettre, Pharm.D, PhD Co-Founder, Board member

Pharmacist and Doctor of Science Director of EFS Besançon Expert in the production of cellular immunotherapies



## Maxime Fredon, PhD Lead Scientist, CSO

PhD in Immunology and CAR-T Cell in Oncology.

Extensive experience immune cell therapies
Thesis on CARLA001



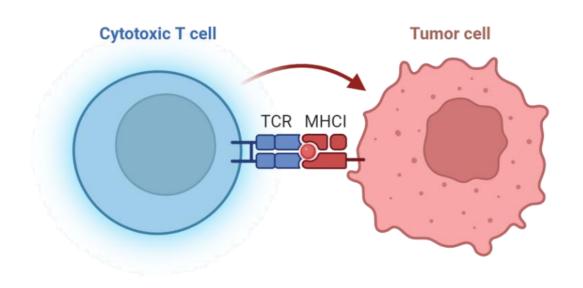


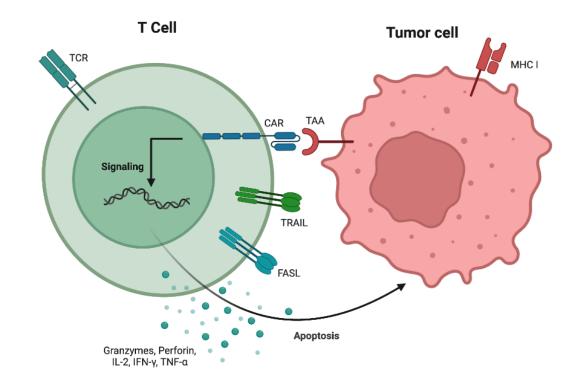




#### **CAR T cells vs T cells**

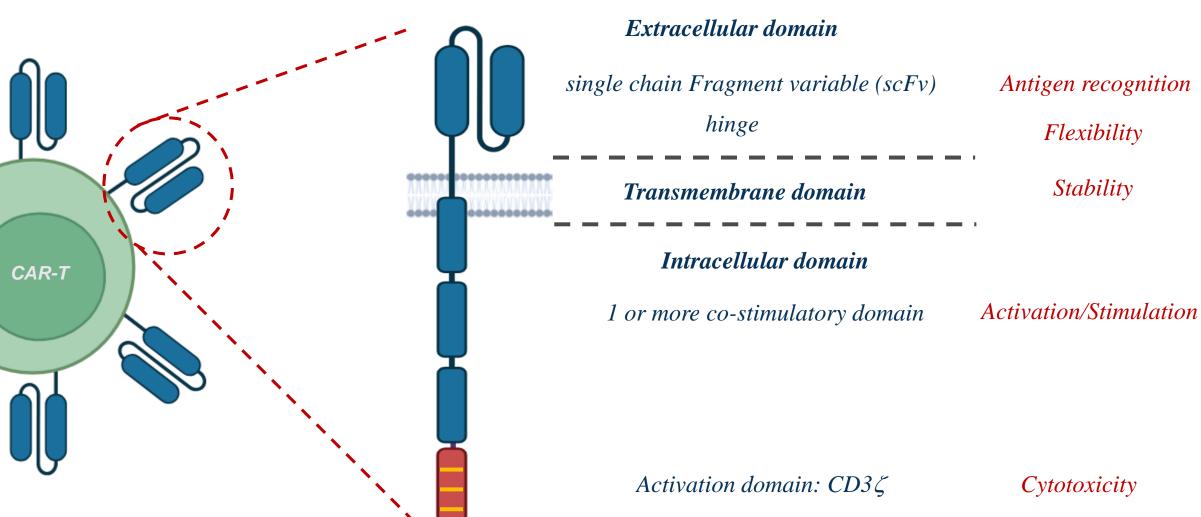
CAR: Chimeric Antigen Receptor







#### **CAR** construct: different components that have their importance

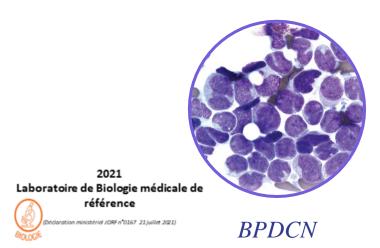


4 CAR T cell immunotherapy for human cancer. **Science**. Published: March 2018.



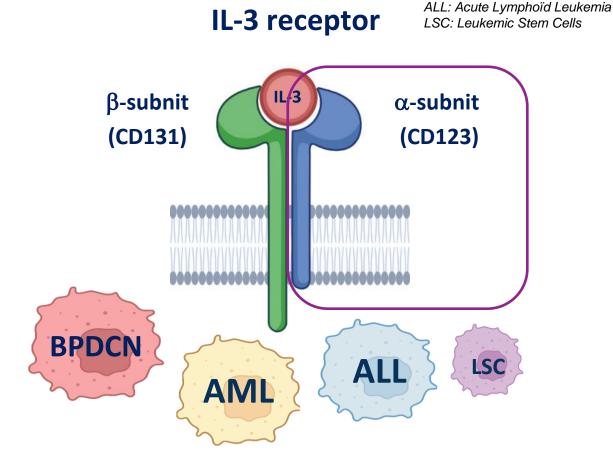
#### CD123: a target of interest for treatment of acute leukemia

BPDCN: Blastic Plasmacytoid Dendritic Cell Neoplasm



Median overall survival: 8-14 months Overall survival at 1 year = 10-15% Often misdiagnosed and/or under-reported Tagraxofusp but efficacy is limited and 100% of patient relapse

**Characteristic: overexpression of CD123** 





CD123, an optimal acute leukemia target



AML: Acute Myeloid Leukemia

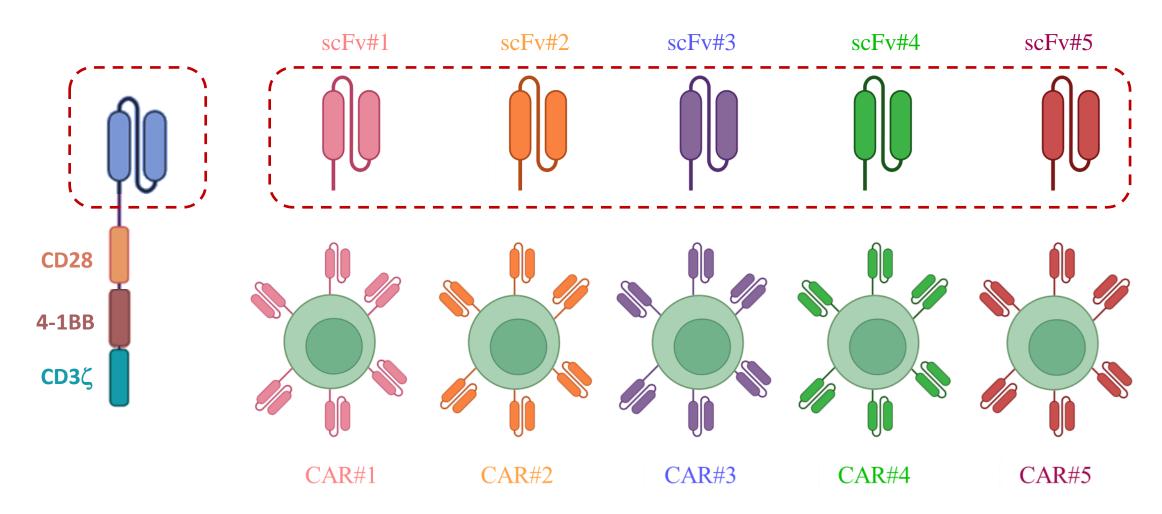


## on-target/off-tumor effects



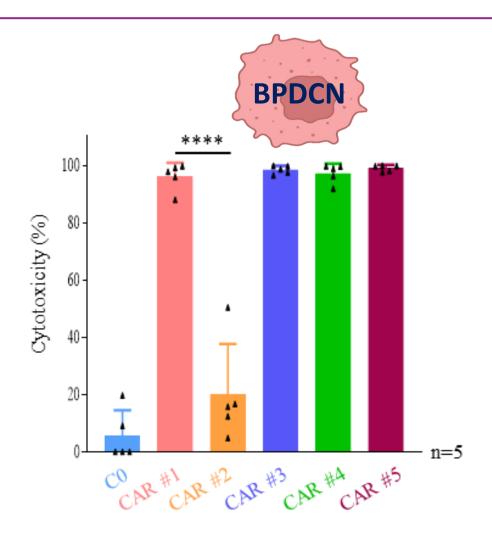


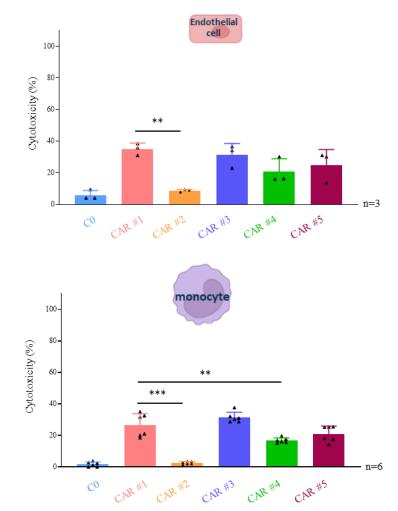
#### Impact of scFv substitution on the functionality/safety balance of a CD123 CAR T cells



Carla biotherapeutics

## In vitro, CD123 CAR-Ts preferentially eliminate BPDCN cells

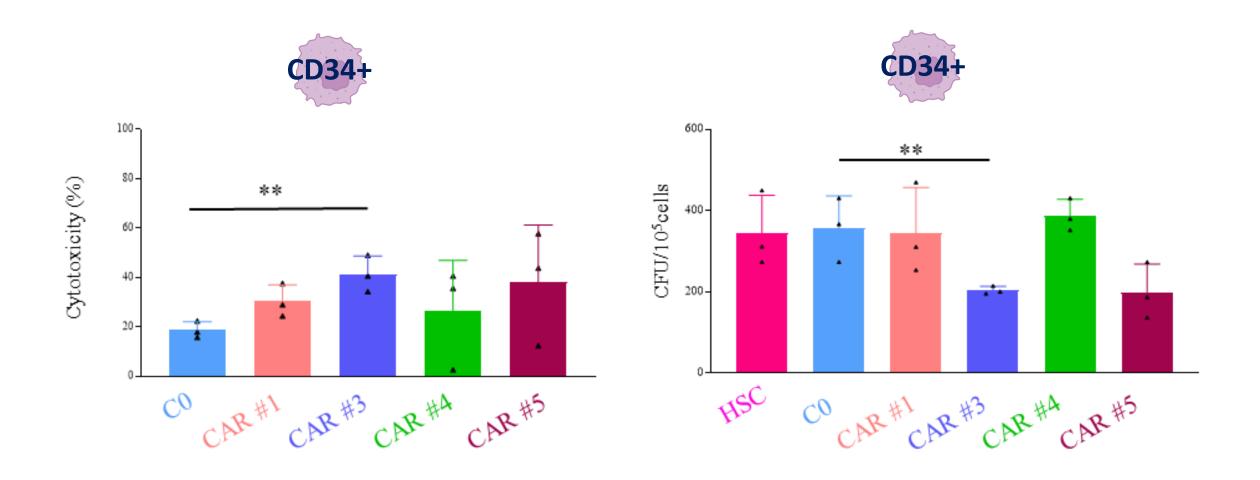






Impact of scFv on functionality and safety of third generation of CAR T cells. Fredon et al., Cancer Immunol Res.

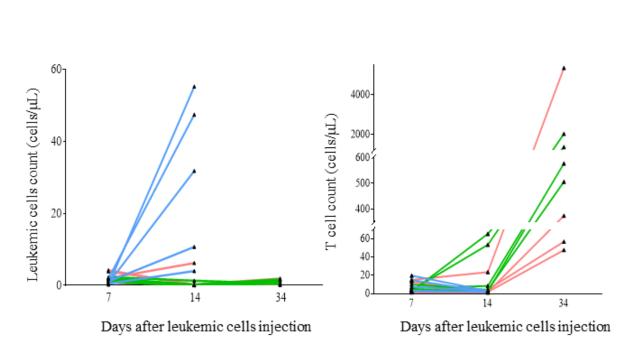
## Low/no impact in hematopoiesis

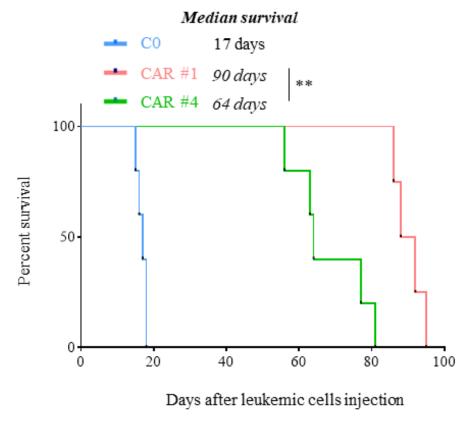




Impact of scFv on functionality and safety of third generation of CAR T cells. Fredon et al., Cancer Immunol Res.

## Efficacy of CD123 CAR-Ts in BPDCN PDX xenografts mouse model





**Tumor uptake control** 

**CAR T cells expansion** 

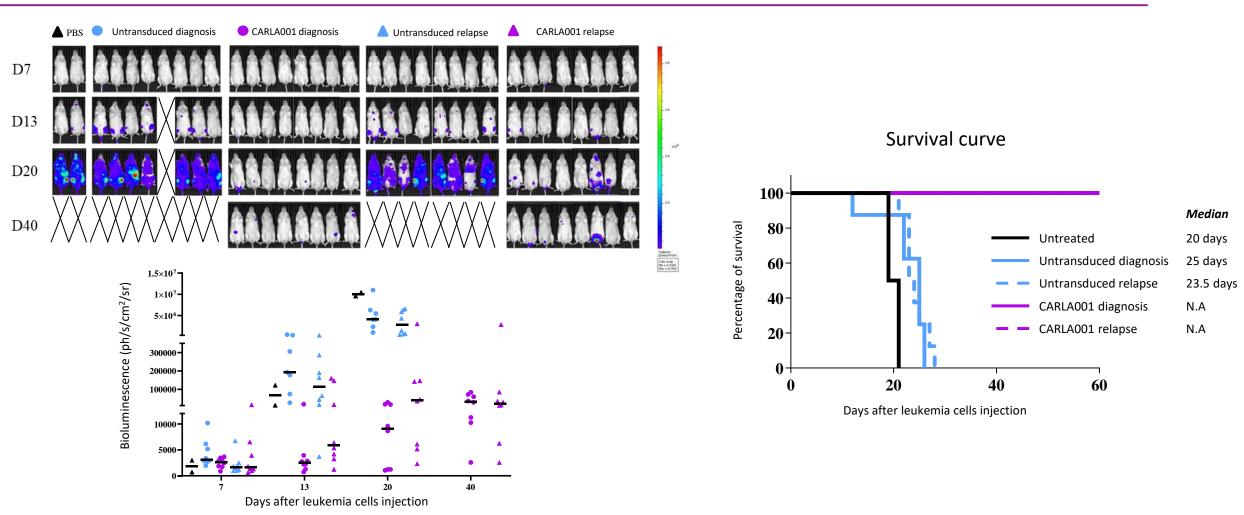
Increase overall survival in mice



**CAR#1: drug candidate (CARLA001)** 



### **CARLA001** produced from T cells of even relapsed patients



CARLA001 produced with patient T cells, even in relapse, retains strong functionality

biotherapeutics

Impact of scFv on functionality and safety of third generation of CAR T cells. Fredon et al., Cancer Immunol Res







REGION BOURGOGNE FRANCHE COMTE











## Thank you

## Maxime Fredon, PhD

maxime.fredon@carla-biotherapeutics.com CARLA biotherapeutics 18 rue Alain Savary Besançon, F-25000



