

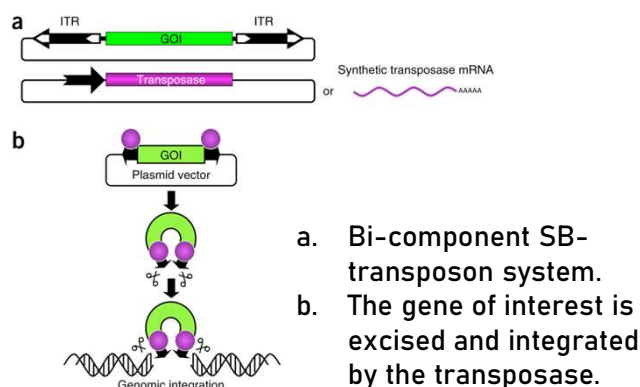
- › Cost of current CAR-T therapies: 250–400k € per patient (viral gene delivery)
- › Bottlenecks in manufacturing due to complex GMP virus production
- › Regulatory pressure: need for safe and less costly non-viral alternatives

### The Sleeping Beauty Transposase System (SB100X)

- › Virus-free integration: ~80% efficiency in T cells
- › Improved safety: almost random integration profile, no hotspots
- › Reduced manufacturing costs: >10x cheaper than viral vectors

## TECHNOLOGY

### HOW IT WORKS



### Main features

- › Mechanism: cut and paste (no-reverse transcription)
- › Cargo capacity: no upper limit +kb
- › Immunogenicity: similar to plasmid-based expression vectors
- › Tropism: dividing and non-dividing cells
- › Integration profile: improved biosafety transposases
- › Stable expression: copy number dependent, non-silenced
- › Efficacy: in certain cells comparable to lentivirus
- › Transcriptional activation: no promoter/enhancer activity

### Development status

- › Clinical phase I/II studies: e.g. ex vivo CAR-T CARAMBA by University of Würzburg
- › ex vivo engineered B cell therapy
- › in vivo gene insertion therapy

## INTELLECTUAL PROPERTY

A developing portfolio from the foundational discovery to a high-precision genome engineering system with improved biosafety

*Hyperactive variants of the transposase protein of the transposon system Sleeping Beauty*

Priority date	New variant improvement
2022	Safer integration profile
2022	~2× activity
2023	Shorter half-life
2025	Targeting genomic safe harbors

## APPLICATIONS

### Cell therapy: Oncology / Immunotherapy

- › Development of CAR-T and TCR-T cells, gene therapy; in vivo applications
- › Leukemia, lymphomas, and solid tumors; rare diseases (X-CDG, MPS I)
  - › Need for system with **safety**

### Bioproduction

- › Generation of stable cell lines for biologics manufacturing
- › High efficiency for sustained protein expression
  - › Need for system with **efficiency**

### Research tool

## PARTNER WITH US

We are seeking

- › Co-development opportunities
- › Licensing partners

## RESEARCH EXPERTISE

- › This technology was developed by the research group “mobile DNA” of Dr Zsuzsanna Izsvak who has 20+ years of experience in studying transposable elements.
- › The Sleeping Beauty 100x transposon system (transposase enzyme SB100x and transposon vector pT4) was further developed and de-risked for a variety of gene and cell engineering applications in the Helmholtz Innovation Lab MDCcell.



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