Enabling individualized cell and gene therapies through non-viral gene transfer with synthetic transposons

› Viral vector production is complex, costly, slow (>4 months), and difficult to personalize, limiting scalability of cell and gene therapies

› Personalized cell and gene therapies require a rapid, modular, and cost-efficient vector manufacturing process

SynTra is a non-viral gene transfer platform, combining the Sleeping Beauty technology with synthetic DNA production for efficient, scalable, personalized cell and gene therapies.
SynTra enables custom, clinical-grade vector production in less than a month, enabling truly individualized therapies

TECHNOLOGY

Non-viral, synthetic, modular gene delivery

› The Max Delbrück Center's proprietary Sleeping Beauty Transposase enables non-viral gene transfer with similar efficacies as viral vectors, but greatly reduced complexity (only DNA and RNA needed)

› Fully synthetic, cell-free DNA production process, allowing a flexible and patient-specific vector generation for individualized therapies

› Modular and fast pipeline, reducing custom vector production time from >4 months to <1 month

› Combination of Sleeping Beauty Transposase-mediated gene transfer and synthetic DNA-Transposons reduces the patient-specific cell production to less than 2 months

Development status – TRL 4

› DNA production pipeline allows small scale production of synthetic DNA transposons in less than a week

› Proof of principle demonstrated, synthetic vectors showing comparable efficacy to current gold-standard vectors

› Platform established and advancing toward automated, scalable manufacturing workflows

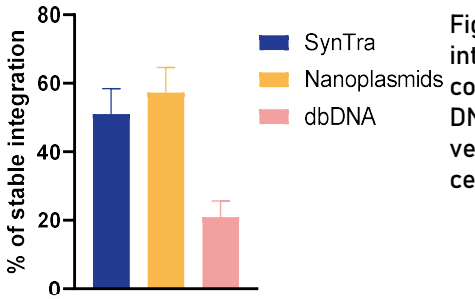

Figure 1: Gene integration rates compared to DNA Transposon vectors in Jurkat cells

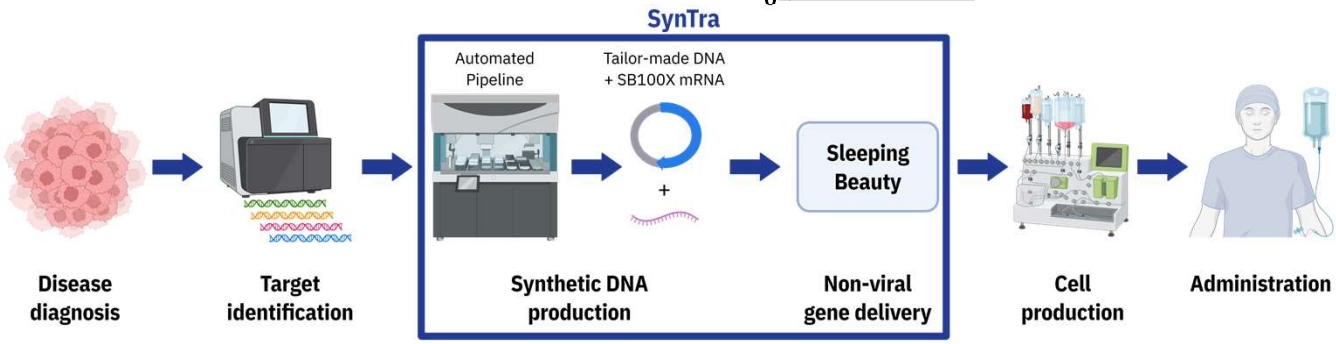

Figure 2: Personalised cell and gene therapy pipeline with SynTra as the central component

INTELLECTUAL PROPERTY

Stable Gene Transfer with Hyperactive Sleeping Beauty Transposase SB100X
WO2009003671A3
National phases: EP, US, AU, CA, DE, JP (all granted)

PARTNER WITH US

We are seeking

› Co-development opportunities

› Pilot partners

RESEARCH EXPERTISE

› Developed under the leadership of Dr. Michael Fichtner, biochemist with a background in cancer immunology, with strong expertise in non-viral gene delivery and transposon systems

› Supported by the European Regional Development Fund (ERDF), advancing automated manufacturing for synthetic DNA–based gene therapies

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Mátés, L., et al. (2009) - Nat Genet
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