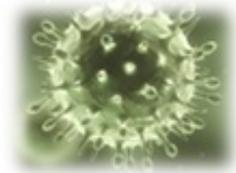


Master gene copy numbers in hematopoietic stem cells

Gene therapy safety improved by enhanced transduction efficiencies

[Transduction enhancers](#) help increase the chances of success for blood-cell targeted gene therapies. They fine-tune the number of gene copies entering [hematopoietic stem cells](#) (HSC) and help address heightened safety requirements set forth by regulatory institutions, as new data demonstrate.



A difficult gene therapy target...

In the absence of transduction enhancers, the low susceptibility of hematopoietic stem cells necessitates a high dose of [lentivirus](#) to even reach just a fraction of cells within the entire pool. While solid overexpression of gene elements can be advantageous for a number of therapeutic approaches, many vector strategies require a more refined approach. This is especially true for stem cells that may undergo several stages of proliferation and differentiation, making them highly susceptible to uncontrolled selection events.

...just got easier

Data from the Laboratory of [Prof. Hauber at the Heinrich Pette Institute](#) - Leibniz Institute for Experimental Virology (HPI) in Hamburg demonstrates that standard methods for transduction of hematopoietic stem cells frequently result in sub-optimal efficiencies. The research of Prof. Hauber and his team focusses on innovative [antiviral gene therapy strategies](#) to actively remove HIV genes from blood cells and to establish long-lasting resistance against the viral intruder.

In this context they were searching for a method to improve the transduction procedure that is also transferrable to a future clinical setting. When the team added [LentiBOOST™](#) transduction enhancer to the transduction protocol they perceived two effects.

- First, the fraction of transduction positive cells within the cell pool was significantly **increased up to 80%**.
- Second, the number of **vector copies per diploid genome** was between **3 and 5**, an **near-ideal value**.

*"These qualities are very helpful to **fine-tune** the effects of therapeutically relevant vectors that, if unchecked, could cause undesired effects. **Optimizing transduction rate and at the same time limiting vector integration numbers to these levels means improving both, safety and chance of success for future gene therapies.**"*

- Prof. Hauber commented.

LentiBOOST™ can be licensed from [SIRION Biotech](#), Munich for applications in clinical R&D. As world leading innovator of viral vector technologies the company also maintains projects to [improve AAV](#) dependent applications and is actively developing a new [AV serotype](#) that is applicable in vaccination and immune oncology.

SIRION Biotech is looking forward to discuss projects to develop and improve virus based technologies that [address the needs of a growing medical field](#).

About the company

Highest Technological Standards



SIRION Biotech is world leader for innovating virus vector technologies and also provides **custom services** to academic and industrial partners worldwide. SIRION is the only company mastering **all 3 major virus types** that are used regularly for genetic manipulation of cell systems.

- Customized [cell models](#) and [viral vectors](#)
- In shortest possible time frames
- Custom project management to fit your experimental setup

Technological features include

- Control gene expression and [knockdown](#)
- All-in-one [Lentivirus](#), [AAV](#) and [Adenovirus](#) vectors
- Inducible, tissue specific expression and [transduction boosters](#)

Licensing options for industrial use are available.

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A true dual-citizen of American and German descent, Carl has worked on both sides of the Atlantic. With his well based knowledge of cellular, neuro- and cardiac physiology and his unique bilingual background, he maintains fast and precise communications between the SIRION headquarters in Germany and US clients.

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[Please inquire](#) for your individual project proposal.



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