



Press release - science

Leuven, Belgium – 30 april 2009

Moving gene therapy forward with mobile DNA

Leuven, Belgium - VIB researchers connected to the Katholieke Universiteit Leuven (Belgium) in collaboration with colleagues at the Max Delbrück Center in Berlin (Germany) have developed a new non-viral gene technology approach. This approach may overcome side-effects associated with the current viral vectors, such as inflammation or the development of cancer. The result offers new hope for optimizing gene therapy as a possible cure for specific diseases, such as genetic disorders and cancer.

Gene therapy

Gene therapy is the introduction of genetic material into a patient's cells resulting in a cure or a therapeutic effect. In recent years, it has been shown that gene therapy is a promising technology to treat or even cure several fatal diseases for which there is no attractive alternative therapy. Gene therapy can be used for hereditary diseases, but also for other diseases that affect heart, brain and even for cancer. Indeed, recent results suggest that gene therapy can be beneficial for patients suffering from aggressive brain cancer that would otherwise be lethal.

A safe delivery of the genes?

Despite the overall progress, there is still a need to develop improved and safer approaches to deliver genes into cells. The success of gene therapy ultimately depends on these gene delivery vehicles or *vectors*. Most vectors have been derived from viruses that can be tailor-made to deliver therapeutic genes into the patients' cells. However, some of these viral vectors can induce side-effects, including cancer and inflammation.

Marinee Chuah, Thierry VandenDriessche, Eyayu Belay and their fellow VIB researchers at K.U. Leuven in collaboration with **Zsuzsanna Iszvak** and **Zoltan Ivics** and colleagues at the Max Delbrück Center in Berlin (Germany) have now developed a new non-viral approach that overcomes some of the limitations associated with viral vectors.

Lessons from evolution

Using the principles of evolution and natural selection, that were initially conceived by Charles Darwin, they have now developed an efficient and safe gene delivery approach based on non-viral genetic elements, called transposons. Transposons are mobile DNA elements that can integrate into 'foreign' DNA via a 'cut-and-paste' mechanism. In a way they are natural gene delivery vehicles. The researchers constructed the transposons in such a way that they can carry the therapeutic gene into the target cell DNA. Doing so, they obviate the need to rely on viral vectors.

'We show for the first time that it is now possible to efficiently deliver genes into stem cells, particularly those of the immune system, using non-viral gene delivery', says **Marinee Chuah**. 'Many groups have tried this for many years but without success. We are glad that we could now overcome this hurdle' claims **Thierry VandenDriessche**. **Zsuzsanna Iszvak** and **Zoltan Ivics** concur: 'This transposon technology may greatly simplify the way gene therapy is conducted, improve its overall safety and reduce the costs'.

The VIB researchers are further testing this technology to treat specific diseases including cancer and genetic disorders, in anticipation of moving forward and treat patients suffering from these diseases.



Questions

Given that this research can raise a lot of questions for patients, we ask you to please refer questions in your report or article to the email address that VIB makes available for this purpose: patienteninfo@vib.be. Everyone can submit questions concerning this and other medically-oriented research directly to VIB via this address.

Relevant scientific publication

This research appears in the authoritative journal *Nature Genetics* (Mátés *et al.*, Molecular evolution of a novel hyperactive sleeping beauty transposase enables robust stable gene transfer in vertebrates).

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Info for the editor

This research has been performed by **Marinee Chuah** and **Thierry Vandendriessche**, VIB Vesalius Research Center, K.U.Leuven

(www.vib.be/Research/EN/Research+Departments/Vesalius+Research+Center/Thierry+Vandendriessche+--+Marinee+Khim+Chuah)

VIB

VIB, the Flanders Institute for Biotechnology, is a non-profit research institute in the life sciences. Some 1100 scientists and technicians conduct strategic basic research on the molecular mechanisms that control the functioning of the human body, plants, and micro-organisms. Through a close partnership with four Flemish universities – Ghent University, the Katholieke Universiteit Leuven, the University of Antwerp, and the Vrije Universiteit Brussel – and a solid investment program, VIB unites the forces of 65 research groups in a single institute. Their research aims at fundamentally extending the frontiers of our knowledge. Through its technology transfer activities, VIB strives to convert the research results into products for the benefit of consumers and patients. VIB also develops and distributes a broad range of scientifically substantiated information about all aspects of biotechnology. More info at: www.vib.be.

K.U.Leuven

The University of Leuven is Belgium's largest university and one of the oldest universities in Europe, founded in 1425. It is a comprehensive university with 14 faculties, with a long tradition of high-quality interdisciplinary research and teaching. The University of Leuven has over 33,000 students (12 percent international) and over 17,000 staff members (8,600 in the various university departments and 8,700 at UZ Leuven, the university hospital). More info at: www.kuleuven.be

Max Delbrück Center

Located in the northeast corner of Berlin, the Max Delbrück Center for Molecular Medicine (MDC) Berlin-Buch combines basic research in molecular biology and genetics with clinical research so that the latest medical discoveries can be brought to the bedside as quickly as possible. For this reason the scientists of the MDC collaborate closely with clinicians of two clinics in its vicinity, which are part of the Charité, Universitätsmedizin Berlin/Helios Klinikum Berlin - namely, with the Robert Rössle Cancer Clinic (RRK) and the Franz Volhard Clinic for Cardiovascular Diseases (FVK) in Berlin-Buch. The links between the MDC and these Berlin-Buch clinics are many and varied: Clinicians head research groups at the MDC, MDC researchers and clinicians pursue mutual research projects and the MDC supports research in the clinics in many ways including offering young clinicians a special training program in basic research. More info at: www.mdc-berlin.de/en

Mention both VIB and the university

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For more information

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