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Letter to Editor

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Dear Editor,

On October 7th, we got the news on Nobel Prize in Chemistry, awarded for CRISPR-Cas9 genetic scissors, the so-called game changer technology, which was first described in 2012. The prize was given to Emmanuelle Charpentier, director of the Max Planck Unit for the Science of Pathogens, in Berlin, Germany and Jennifer A. Doudna, professor at UC Berkeley and faculty scientist at Howard Hughes Medical Institute. The "First-all Female Scientists Team" promoted a huge development in science and opened doors to new discoveries in basic science, agriculture and medicine.

CRISPR-Cas9 gene editing tool has been adapted to a genome editing system that occurs naturally in bacteria. The bacteria create the so called CRISPR arrays, by capturing fragments of DNA from invading viruses. Via these arrays, the bacteria remember the virus and/or similar fragments of the virus. CRISPR-Cas9 technique has been worldwide used by researchers to develop new crops that withstand drought and pests, clinical trials for curing cancer, and there are hopes to help cure inherited diseases.

The recent achievements clearly show that humankind is benefiting from this technology. In clinical trials, some preliminary results showed that the altered genome of immune cells of three cancer patients has been well-tolerated and this could provide evidence of safety and feasibility in using CRISPS-Cas9 in treating human diseases. However, announcements like the birth of twin girls from genome edited embryos in November 2018 in China, raise up ethical, social, safety and efficacy concerns. Despite the main aim of the genome editing experimental trials being to fix genes with defects or make other edits in the genome of the embryo, it has been shown that unwanted changes of the genes can be generated. As a consequence, these changes lead to different outcomes in the cells of the same embryo. Such examples lead the scientific societies to conclusions, that the genome editing technology is not ready to be used in human embryos.

The countless applications are benefiting all of us in our research centres and we will most probably continue to benefit from this technology in the future. The number of CRISPR gene editing studies entering clinical trials is rapidly evolving for the treatment of various diseases. The first clinical trials in cancer patients involve the use of CRISPR-engineered T cells for cancer immunotherapy treatment with results supporting the effectiveness and general safety. Recent publications show early success of using genome editing tools to treat sickle-cell anaemia and ß-thalassaemia. The *BCL11A* erythroid-specific enhancer was successfully targeted by CRISPR-Cas9.

There are still questions that need to be answered: what will happen with the human genome editing in the long run? When will genome editing be proven to be safe and efficient to be used in editing human embryos? How many more experiments are necessary to be done on embryos to get an answer? Are there going to be rules and authorization in using gene-therapy kits in human genome editing, to

prevent misuse of the technology? When will CRISPR-based gene editing be routinely used in clinic? We still need more time to get an answer to these questions and learn more about this technology. Let's see together what surprising outcomes genome editing science CRISPR-Cas9 will bring us.

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