Researchers create new protocol to improve gene therapy tool production

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A method to create a faster and lower cost alternative for a gene therapy tool has been developed by Boston University School of Medicine (BUSM) researchers.

Gene therapy is a clinical technique that introduce genes to treat disease. One approach is to use adeno-associated virus (AAV) as a tool to deliver the gene, but production of large quantities of AAV tends to be complicated and costly.

Now for the first time, BUSM researchers have developed an advanced protocol to produce large quantities of AAVs, viral vectors that can deliver a specific gene into humans and animals.

According to the researchers, AAVs are also powerful research tools when combined with modern gene-editing technologies and can serve as a practical alternative to genetically modified animal models. However, a major drawback has been the time and cost to produce quantities of AAV to be used for animals in the lab. This advanced technique bypasses developmental effects that can arise from conventional gene manipulation in animals, while saving time, reducing the numbers of animals used in research and eventually research cost.

"Our protocol helps to produce AAVs efficiently and economically in regular laboratories so that researchers can easily conduct a pre-clinical trials for gene therapy," explained co-corresponding author Markus Bachschmid, Ph.D., assistant professor of medicine at BUSM.

"Several labs in the Boston area and Japan have already tested this new protocol and found it useful," said co-corresponding author Reiko

Matsui, MD, assistant professor of medicine at BUSM. "Our hope is that many laboratories can adapt these procedures to accelerate research and promote gene therapy."

Gene therapy using AAV is a rapidly emerging field in clinical therapy. The recent release of the FDA approved AAV-based drug Zolgensma for treating spinal muscular atrophy is a landmark in human gene therapy and demonstrates the high potential of AAV.

These findings appear online in *Scientific Reports*.

More information: Toyokazu Kimura et al, Production of adeno-associated virus vectors for in vitro and in vivo applications, *Scientific Reports* (2019). DOI: 10.1038/s41598-019-49624-w

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