

Accepted Manuscript

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PII: S0006-291X(16)30272-8

DOI: [10.1016/j.bbrc.2016.02.083](https://doi.org/10.1016/j.bbrc.2016.02.083)

Reference: YBBRC 35386

To appear in: *Biochemical and Biophysical Research Communications*

Received Date: 13 February 2016

Accepted Date: 19 February 2016

Please cite this article as: J. Pang, Y. Wu, Z. Li, Z. Hu, X. Wang, X. Hu, X. Wang, X. Liu, M. Zhou, B. Liu, Y. Wang, M. Feng, D. Liang, Targeting of the Human *F8* at the Multicopy rDNA Locus in Hemophilia A Patient-Derived iPSCs Using TALENickases, *Biochemical and Biophysical Research Communications* (2016), doi: 10.1016/j.bbrc.2016.02.083.

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Abstract

Hemophilia A (HA) is a monogenic disease due to lack of the clotting factor VIII (FVIII). This deficiency may lead to spontaneous joint hemorrhages or life-threatening bleeding but there is no cure for HA until very recently. In this study, we derived induced pluripotent stem cells (iPSCs) from patients with severe HA and used transcription activator-like effector nickases (TALENickases) to target the factor VIII gene (*F8*) at the multicopy ribosomal DNA (rDNA) locus in HA-iPSCs, aiming to rescue the shortage of FVIII protein. The results revealed that more than one copy of the exogenous *F8* could be

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