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Genotoxic effects of base and prime editing in human hematopoietic stem cells

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Supplementary Figures



Supplementary Fig. 1. Percentage of human engraftment and editing of treated cells in xenotransplant experiments. **a,b**, Percentage of human cells engraftment (**a**) and editing of treated cells within human graft (**b**) in mice transplanted with CB/mPB HSPCs after B2M exon 2/AAVS1 editing at day 3 post-thawing from Fig. 3b (right), 3g, Extended Data Fig. 2g, 2n for (**a**) and from Fig. 3c (right), 3h, Extended Data Fig. 2h, 2o (n= 18,18,19,20). Median with IQR. LME followed by post hoc analysis. All statistical tests are two-tailed. n indicate independent animals.



Supplementary Fig. 2. Abundance and inter-/intra-mouse sharing of BARs from clonal tracking analyses. Heatmap of the abundance (red-scaled palette) of BARs (rows) in PB at indicated times

after transplant, hematopoietic organs and lineages (columns) in mice from Fig. 3i. All statistical tests are two-tailed. n indicate independent animals.



Supplementary Fig. 3. Gating strategies for flow cytometry analyses used for in vitro samples. Gating strategies for the analysis of: **a**, B-lymphoblastoid cells at 7 days after treatments; **b**, Human T cells at 7 days after treatments; **c**, Human HSPCs (CB-/mPB-derived) at 7 days after treatments.



а





Supplementary Fig. 4. Gating strategies for flow cytometry analyses used for PB, BM and SPL of mice. Gating strategies for the analysis of: **a**, human cells in PB of transplanted NSG mice; **b**,

human cells within BM of transplanted NSG mice at the end of the experiment; \mathbf{c} , human cells within SPL of transplanted NSG mice at the end of the experiment.