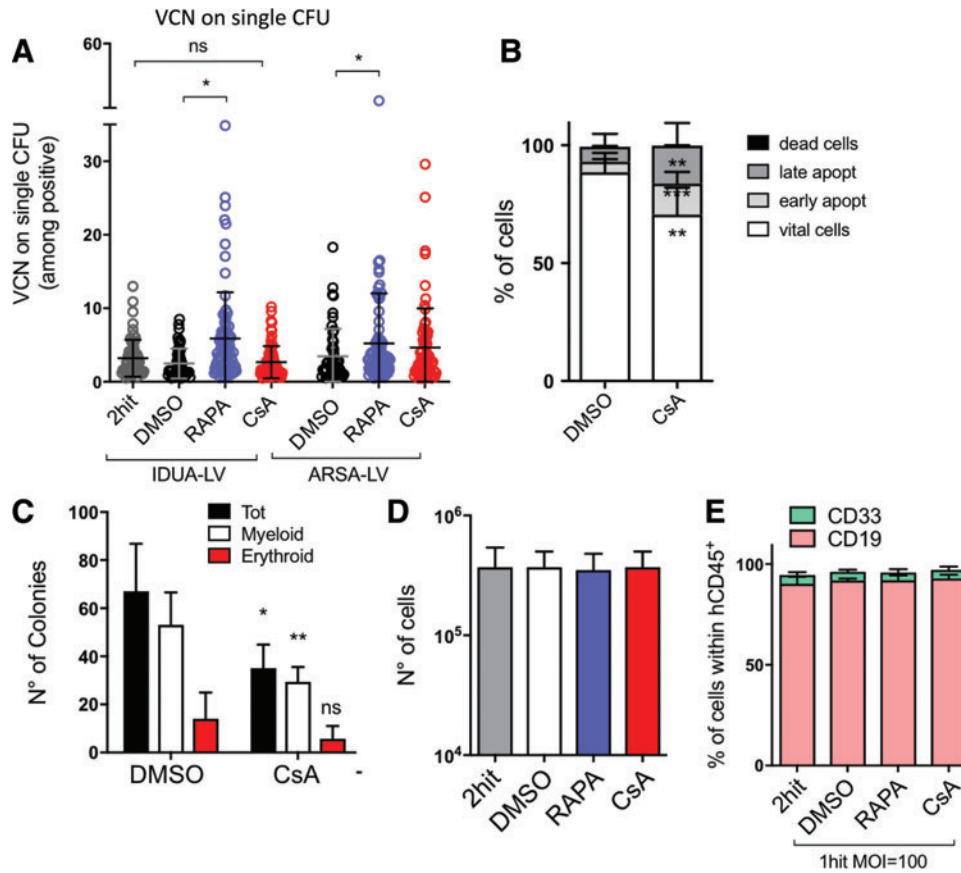


Supplementary Data



Supplementary Figure S1. (A) BM-derived CD34⁺ cells were transduced with *IDUA*-LV, as reported in Fig. 1A. VCN/human genome were evaluated, picking single colonies after 15 days in culture ($M \pm SD$, $n \geq 50$, Kruskal-Wallis with Dunn's multiple comparison test vs. each DMSO; ns, not significant; $*p \leq 0.05$). (B and C) mPB-CD34⁺ cells were transduced or not with a lab-grade PGK-*GFP* LV in the presence of DMSO/CsA, and apoptosis (B) or colonies count (C) were reported ($M \pm SD$, $n = 10$, Mann-Whitney test vs. each DMSO, $**p \leq 0.01$, $***p \leq 0.001$). (D) BM-CD34⁺ cells were counted at the time of transplantation in NSG mice, as reported in Fig. 1 for *IDUA*-LV. (E) Percentages of human B, T, and myeloid lineages within human cells were shown in the BM of mice from the experiments reported in Fig. 1 at 22 weeks post transplant.