|  |  |  |  |
| --- | --- | --- | --- |
| **Experiment** | **Mutation** | **WASp expression (% CD14+) Pre/ edited/ gene therapy** | **Vector copy number edited/ gene therapy** |
| 1 | c.97C>T^ | 1.8/ 65/ 54 | 1/ 1.8 |
| 2 | 1483 Del G | 1.7/ 78/ 70 | 1/ 0.8 |
| 3 | c.97C>T^ | 1.9/ 81/ 24 | 1/ 1.75 |
| 4 | T302>C | 0.3/ 90/ 70 | 1/ 0.76 |

Figure 1- source data 2: Molecular details of patient stem cell-derived macrophages used for in vitro WAS correction

WASp, Wiskott Aldrich syndrome protein.

^ same patient