Table S1. Impact of metabolite interference on the quantitation of ASO-001, when M1 (3’ n-1), M2 (3’ n-10), M3 (5’ n-10), M4 (3’ n-15), and M5 (5’ n-15) were co-spiked at 20% of ASO-001 concentrations.

|  |  |  |
| --- | --- | --- |
| Replicates | Low QC | High QC |
|  | 1.50 ng/mL | 375 ng/mL |
| 1 | 1.38 | 394 |
| 2 | 1.36 | 403 |
| 3 | 1.37 | 399 |
| 4 | 1.34 | 410 |
| 5 | 1.38 | 408 |
| 6 | 1.27 | 403 |
| Mean | 1.35 | 403 |
| S.D. | 0.0420 | 5.85 |
| %CV | 3.1 | 1.5 |
| %Bias | -10.0 | 7.5 |
| n | 6 | 6 |

Table S2. Intra- and inter-run precision and accuracy of ASO-001 in A) treated artificial cerebrospinal fluid (aCSF) and B) treated monkey cerebrospinal fluid (CSF), quantitated using calibration curves prepared in treated aCSF.

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| A |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  |  | LLOQ QC | | | Low QC1 | | | Mid QC2 | | | High QC3 | | |
|  | Nominal (ng/mL) | 0.500 | | | 1.50 | | | 250 | | | 375 | | |
| Intra-run P&A | Mean ± SD (ng/mL) | 0.516 | ± | 0.0528 | 1.45 | ± | 0.143 | 259 | ± | 5.61 | 403 | ± | 12.7 |
| %CV | 10.2 | | | 9.9 | | | 2.2 | | | 3.2 | | |
| %Bias | 3.2 | | | -3.3 | | | 3.6 | | | 7.5 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Mean ± SD (ng/mL) | 0.512 | ± | 0.0452 | 1.50 | ± | 0.126 | 268 | ± | 2.97 | 415 | ± | 5.49 |
| %CV | 8.8 | | | 8.4 | | | 1.1 | | | 1.3 | | |
| %Bias | 2.4 | | | 0.0 | | | 7.2 | | | 10.7 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Mean ± SD (ng/mL) | 0.536 | ± | 0.0173 | 1.59 | ± | 0.0789 | 268 | ± | 5.43 | 386 | ± | 6.71 |
| %CV | 3.2 | | | 5.0 | | | 2.0 | | | 1.7 | | |
| %Bias | 7.2 | | | 6.0 | | | 7.2 | | | 2.9 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Inter-run P&A | Mean ± SD (ng/mL) | 0.521 | ± | 0.0402 | 1.51 | ± | 0.128 | 265 | ± | 6.52 | 402 | ± | 14.8 |
| %CV | 7.7 | | | 8.5 | | | 2.5 | | | 3.7 | | |
| %Bias | 4.2 | | | 0.7 | | | 6.0 | | | 7.2 | | |
| n | 18 | | | 18 | | | 18 | | | 18 | | |
|  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| B |  |  |  |  |  |  |  |  |  |  |  |  |  |
|  |  | LLOQ QC | | | Low QC1 | | | Mid QC2 | | | High QC3 | | |
|  | Nominal (ng/mL) | 0.500 | | | 1.50 | | | 250 | | | 375 | | |
| Intra-run P&A | Mean ± SD (ng/mL) | 0.518 | ± | 0.0291 | 1.45 | ± | 0.0557 | 256 | ± | 8.1 | 396 | ± | 10.6 |
| %CV | 5.6 | | | 3.8 | | | 3.2 | | | 2.7 | | |
| %Bias | 3.6 | | | -3.3 | | | 2.4 | | | 5.6 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Mean ± SD (ng/mL) | 0.533 | ± | 0.0332 | 1.49 | ± | 0.0916 | 264 | ± | 5.68 | 408 | ± | 9.89 |
| %CV | 6.2 | | | 6.1 | | | 2.2 | | | 2.4 | | |
| %Bias | 6.6 | | | -0.7 | | | 5.6 | | | 8.8 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Mean ± SD (ng/mL) | 0.564 | ± | 0.0589 | 1.68 | ± | 0.0774 | 264 | ± | 4.84 | 388 | ± | 4.88 |
| %CV | 10.4 | | | 4.6 | | | 1.8 | | | 1.3 | | |
| %Bias | 12.8 | | | 12.0 | | | 5.6 | | | 3.5 | | |
| n | 6 | | | 6 | | | 6 | | | 6 | | |
| Inter-run P&A | Mean ± SD (ng/mL) | 0.538 | ± | 0.0444 | 1.54 | ± | 0.126 | 261 | ± | 7.02 | 397 | ± | 12.0 |
| %CV | 8.3 | | | 8.2 | | | 2.7 | | | 3.0 | | |
| %Bias | 7.6 | | | 2.7 | | | 4.4 | | | 5.9 | | |
| n | 18 | | | 18 | | | 18 | | | 18 | | |