Exon 13 Skip After QR-421a Single Dose Treatment Regimen Mimicking in vivo Situation in Wild-type Retinal Organoids

As a follow-up experiment, the treatment conditions mimicked stressful concentrations following administration of a single 15 µM injection. Retinal organoids were treated with 5, 10, and 20 µM QR-421a for 15 days, followed by biweekly administration of 20 µM for 56 days. Forty-two days post injection, M3 was quantified by ddPCR and percentage of exon 13 exclusion was calculated. Data is shown as mean ± SEM. QR-421a treatment resulted in dose-dependent exon skip in retinal organoids, and interestingly a significantly amount of exon 13 skip was noted even 2 weeks after reinitiating QR-421a from the medium.

Results (continued)

USH2A Exon 13 Skip After QR-421a Single Dose Treatment Regimen Mimicking in vivo Situation in Wild-type Retinal Organoids

As a follow-up experiment, the treatment conditions mimicked stressful concentrations following administration of a single 10 µM injection. Retinal organoids were treated with 5, 10, and 20 µM QR-421a for 15 days, followed by biweekly administration of 20 µM for 56 days. Forty-two days post injection, M3 was quantified by ddPCR and percentage of exon 13 exclusion was calculated. Data is shown as mean ± SEM. QR-421a treatment resulted in dose-dependent exon skip in retinal organoids, and interestingly a significantly amount of exon 13 skip was noted even 2 weeks after reinitiating QR-421a from the medium.