

**TABLE S1.** Patient Disposition (FAS)

| <b>Disposition/Reason</b>                      | <b>N = 124</b><br><b>n (%)</b> |
|--|--------------------------------|
| <b>Treatment phase</b>                         |                                |
| Ongoing <sup>a</sup>                           | 0                              |
| Discontinued from treatment phase              | 124 (100.0)                    |
| <b>Primary reason for discontinuation</b>      |                                |
| Completed <sup>b</sup>                         | 32 (25.8)                      |
| Progressive disease                            | 53 (42.7)                      |
| Adverse event                                  | 18 (14.5)                      |
| Death  | 10 (8.1)                       |
| Physician decision                             | 6 (4.8)                        |
| Subject/guardian decision                      | 2 (1.6)                        |
| No longer requires treatment                   | 1 (0.8)                        |
| Lost to follow-up                              | 1 (0.8)                        |
| Protocol deviation                             | 1 (0.8)                        |
| <b>Post-treatment phase</b>                    |                                |
| Ongoing  | 0                              |
| Discontinued post-treatment efficacy follow-up | 10 ( 8.1)                      |

Abbreviation: FAS, full analysis set.

<sup>a</sup>Patients ongoing at the time of the cutoff date January 22, 2018.

<sup>b</sup>Thirty-two patients had completed their study treatment as of the data cutoff, and they have been transferred into a separate roll-over study (25 patients) or commercial supply (7 patients).