

Online Table 1. Change in QOL-B Respiratory Symptoms Scores from Baseline to Day 14 for Patients in Different GRCQ Absolute Change Categories

| Mean (SD) change from baseline QOL-B Respiratory Symptom score at Day 14 |                |           |                   |           |                        |            |
|--|----------------|-----------|-------------------|-----------|------------------------|------------|
| <b>AIR-BX1</b>   |                |           |                   |           |                        |            |
| GRCQ Categories*   | AZLI (N = 124) |           | Placebo (N = 125) |           | All Patients (N = 249) |            |
|  | Mean (SD)      | n (%)     | Mean (SD)         | n (%)     | Mean (SD)              | n (%)      |
| No Change  | 0.5 (11.6)     | 53 (42.7) | 3.6 (10.3)        | 78 (62.4) | 2.4 (10.9)             | 131 (52.6) |
| Minimal Change   | 5.0 (15.0)     | 29 (23.4) | 8.6 (12.6)        | 27 (21.6) | 6.7 (13.9)             | 56 (22.5)  |
| Moderate Change  | 17.0 (22.9)    | 36 (29.0) | 8.7 (15.2)        | 18 (14.4) | 14.1 (20.8)            | 54 (21.7)  |
| Large Change   | 32.7 (11.3)    | 6 (4.8)   | 17.1 (-)          | 2 (1.6)   | 30.5 (11.9)            | 8 (3.2)    |
| <b>AIR-BX2</b>   |                |           |                   |           |                        |            |
| GRCQ Categories*   | AZLI (N = 124) |           | Placebo (N= 127)  |           | All Patients (N = 251) |            |
|  | Mean (SD)      | n (%)     | Mean (SD)         | n (%)     | Mean (SD)              | n (%)      |
| No Change  | 1.1 (14.3)     | 47 (37.9) | 2.4 (11.4)        | 74 (58.3) | 1.9 (12.6)             | 121 (48.2) |
| Minimal Change   | 14.1 (13.1)    | 44 (35.5) | 7.8 (13.6)        | 34 (26.8) | 11.4 (13.5)            | 78 (31.1)  |
| Moderate Change  | 11.1 (13.9)    | 22 (17.7) | 11.0 (16.5)       | 15 (11.8) | 11.1 (14.8)            | 37 (14.7)  |
| Large Change   | 16.5 (18.1)    | 11 (8.9)  | 27.8 (18.3)       | 4 (3.1)   | 19.5 (18.2)            | 15 (6.0)   |

\* GRCQ Respiratory Domain categories: no change in symptoms ( $\leq 0.5$ ), minimal change ( $> 0.5$  to  $1.5$ ); moderate change ( $> 1.5$  to  $2.5$ ), and large change ( $> 2.5$  to  $3.0$ ). GRCQ categories were based on the absolute value of GRCQ scores; the categories measured the magnitude of change in respiratory symptoms and included both improving symptoms (positive scores) and worsening symptoms (negative scores). The “N” for each column is the overall number of patients with GRCQ data; note that QOL-B Respiratory Symptoms scores were missing for 3 AZLI-treated patients (no change: 1; moderate change: 2) and 1 placebo-treated patient (large change) in AIR-BX1 and 2 AZLI treated (minimal and moderate change) patients in AIR-BX2. GRCQ = Global Rating of Change Questionnaire; QOL-B = Quality of Life-Bronchiectasis; SD = standard deviation

Online Table 2. Efficacy and Microbiological Measures: Change from Baseline to Day 14 of Treatment with AZLI/Placebo

|   | AIR-BX1  |             |                    |             | AIR-BX2         |             |                    |             |     |
|---|--|-------------|--------------------|-------------|-----------------|-------------|--------------------|-------------|-----|
|   | AZLI<br>(N=134)  |             | Placebo<br>(N=132) |             | AZLI<br>(N=136) |             | Placebo<br>(N=138) |             |     |
| Mean (SD) change from baseline at Day 14  |  |             |                    |             |                 |             |                    |             |     |
| QOL-B scales  | Points for change<br>of 1 answer category<br>for 1 item* | n           | n                  | n           | n               | n           | n                  | n           | n   |
| Respiratory Symptom   | 3.7  | 5.6 (19.1)  | 129                | 4.6 (12.2)  | 129             | 7.6 (16.3)  | 131                | 3.8 (14.3)  | 134 |
| Physical Functioning  | 6.7  | -1.9 (24.3) | 131                | 0.7 (15.2)  | 129             | -1.4 (21.7) | 133                | 1.2 (17.9)  | 133 |
| Vitality  | 11.1   | 0.5 (21.3)  | 132                | 1.6 (16.5)  | 129             | -0.6 (20.6) | 133                | 1.7 (16.7)  | 132 |
| Role Functioning  | 6.7  | -1.5 (20.1) | 131                | 2.2 (13.4)  | 129             | -2.0 (17.6) | 130                | -0.7 (15.0) | 133 |
| Health Perceptions  | 8.3  | 0.5 (18.4)  | 131                | -0.7 (14.3) | 129             | 0.8 (16.0)  | 132                | 1.8 (15.7)  | 134 |
| Emotional Functioning   | 8.3  | 0.1 (14.9)  | 132                | 0.0 (13.5)  | 129             | 1.4 (13.9)  | 133                | 3.1 (13.4)  | 132 |
| Social Functioning  | 8.3  | 1.2 (17.4)  | 131                | 1.7 (13.8)  | 129             | 0.4 (15.2)  | 130                | 2.1 (13.9)  | 134 |
| Treatment Burden  | 11.1   | -2.3 (18.4) | 123                | -4.6 (20.7) | 111             | -4.4 (18.1) | 114                | -7.1 (21.7) | 118 |
| FEV <sub>1</sub> % predicted, % change  |  | -1.0 (12.7) | 127                | -1.1 (8.0)  | 130             | -1.9 (11.9) | 131                | 0.2 (9.9)   | 134 |
| Log <sub>10</sub> CFU/g of sputum for target gram-negative pathogens <sup>†</sup> |  | -2.6 (2.8)  | 97                 | -0.2 (1.8)  | 102             | -2.7 (2.8)  | 101                | -0.2 (1.9)  | 104 |
| 6MWT, meters  |  | -3 (43.9)   | 125                | 6 (40.2)    | 127             | 4 (61.5)    | 128                | 3 (41.6)    | 131 |
| EQ-5D visual analog scale, points <sup>‡</sup>                                    |  | 3.1 (18.2)  | 125                | 0.1 (14.9)  | 128             | 2.0 (14.8)  | 128                | -0.7 (15.0) | 126 |

\* After standardizing scores on a 100-point scale; assuming responses were provided for all items on the scale (ie, no missing responses).

† Target gram-negative organisms included: *Achromobacter*, *Burkholderia*, *Citrobacter*, *Enterobacter*, *Escherichia*, *Klebsiella*, *Moraxella*, *Proteus*, *Pseudomonas*, *Serratia*, and *Stenotrophomonas* species. CFUs were imputed as 0 for patients with no pathogens present.

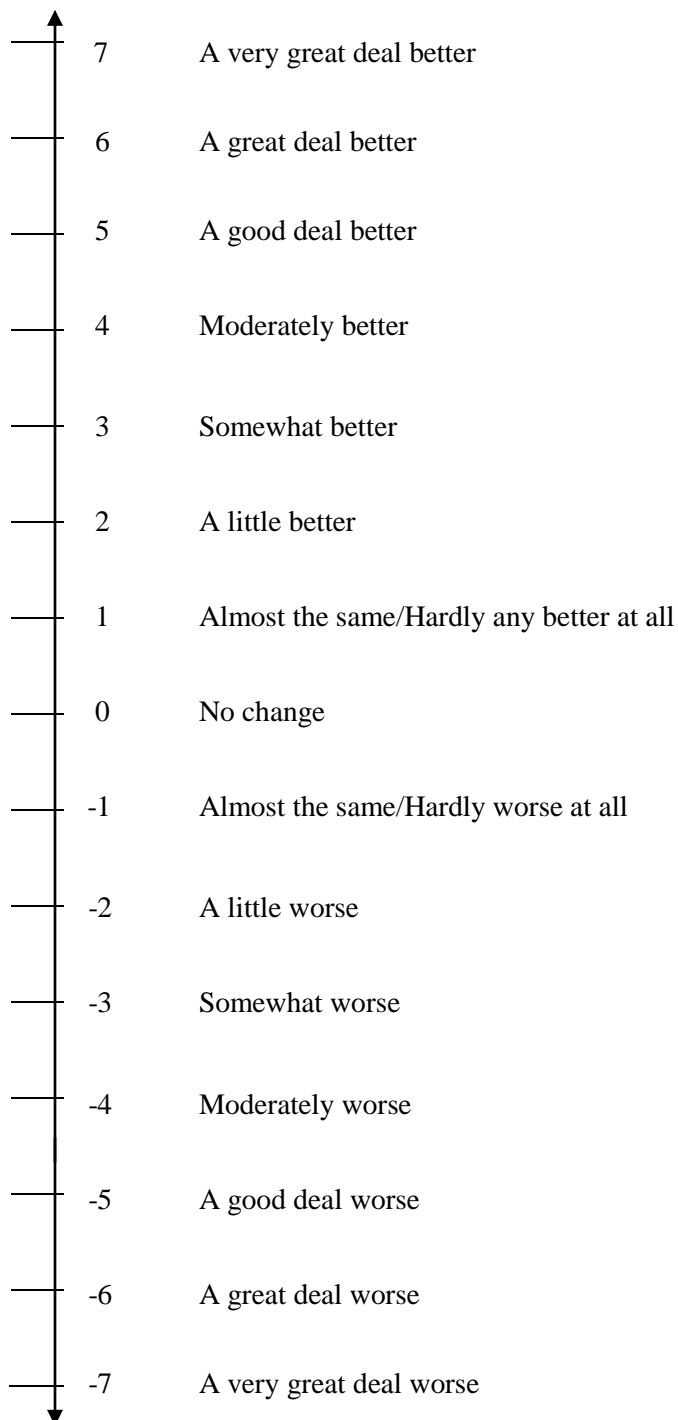
‡ EQ-5D VAS (possible range 0 to 100 points) was not administered at Day 14; these are data from Day 28, at the end of Course 1.

6MWT = 6-minute walk test; CFU = colony forming units; EQ-5D = Euro Quality of Life-5 Dimensions; FEV<sub>1</sub> = forced expiratory volume in 1 second QOL-B = Quality of Life-Bronchiectasis; SD = standard deviation

Online Figure 1. Respiratory Symptoms GRCQ (Global Rating of Change Questionnaire) Version 2.2 (A) and Version 3.0 (B).

A. GRCQ V2.2 for Respiratory Symptoms

In the last 2 weeks, have there been any changes in your RESPIRATORY symptoms (e.g., coughing, mucus production, wheezing) related to your bronchiectasis?



B. GRCQ V3.0 for Respiratory Symptoms

In the last 2 weeks, have there been any changes in your **respiratory symptoms** (e.g., coughing, mucus production, wheezing) related to your bronchiectasis?

My respiratory symptoms are...

