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Cystic Fibrosis And Ivacaftor Use: The Authors Reply

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Almut Winterstein and Amir Sarayani have three concerns regarding our article (May 2018). Their first concern is mortality ("likely while hospitalized"). Of the 199 patients enrolled twelve months before taking ivacaftor, 143 had twelve months of enrollment after treatment, and of the other 56, 1 died during a hospitalization in the twelve months after treatment. A twelve-month mortality rate of 1 in 199 (0.5 percent) patients is not surprising. In the Cystic Fibrosis Foundation's Patient Registry, 10 of 1,779 (0.6 percent) patients who first took ivacaftor during 2012–15 died within twelve months (unpublished data).

Second, the authors noted discrepancies in the numbers of patients in exhibit 2, which were indeed incorrect; the article has been corrected online. There were 56 patients with 3–9 fills and 81 patients with 10–12 fills, for a total of 137 patients. Among those with 3–9 fills, admissions dropped from 43 (0.77 per year) before initiation of ivacaftor to 27 (0.48 per year) after initiation—a 37 percent decrease. For those with 10–12 fills, admissions dropped from 35 (0.43 per year) before initiation to 10 (0.12 per year) after initiation—a 71 percent decrease. These changes do not alter the conclusion that the reduction in hospitalizations was greater among those who were adherent to treatment. We excluded 6 patients with 1–2 fills because the cell size was too small to calculate admission rates.

Third, the authors speculate that in-hospital receipt of ivacaftor could have caused reverse causation—that is, more hospital days leading to fewer outpatient-filled prescriptions because inpatient medication use substituted for outpatient prescriptions. That hypothesis is implausible. The average length-of-stay for an admission in these data was seven to eight days. Even if a hospital were to administer daily ivacaftor to patients, that would not appreciably reduce the need for outpatient pharmacy refills upon discharge.