

Commentary: Some Speculative Thoughts on Money and Healthcare for Cystic Fibrosis

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THE CASE REPORT from Castro-Elias et al. describes the apparently successful use of a recently approved medication as a rescue therapy for a desperately ill child who meets some but not all of the criteria established in clinical trials. Such a case report is to be expected; all new therapies will be studied in specific populations at first, and there will always be cases in which a therapy should be considered for those who have not been studied. In cystic fibrosis (CF), we have a long history of “off-label” use of medications in routine care and when a patient is in trouble. This is even more the case for therapies that are not in themselves burdensome and which hold the potential for significant benefit to the patient. So why is this case report different?

The difference here is a subject that the authors leave completely unaddressed: money. Were the medication under question not Ivacaftor, a therapy for CF distinguished by its novel biological approach and its extraordinary cost, this case report might attract no notice. But Ivacaftor is expensive, dramatically so, with costs approaching USD \$300,000 per year every year for life.

Nowadays the high cost of a new medication is not by itself news. What is novel is the introduction of such a high-cost medication into the existing pattern of care for patients with CF in the United States.

Ivacaftor, for all its promise, is yet another approach in a long line of additive incremental therapies that modify but do not cure the disease. Yes, the model of action is new and is directed to the currently accepted theories of the pathogenesis of the disease. But the size of the reported effects of Ivacaftor directly echo the effects of so many other innovations in CF care, not all of which are biological. The widespread introduction of the CF Care Model—the regular preventive, interdisciplinary approach to children with a chronic disease—achieved as much if not more sustained improvement in life expectancy and quality of life as the introduction of any single medication.

The introduction of Ivacaftor should prompt us in the U.S. CF community to consider *all* the potentially modifiable biological and nonbiological factors influencing outcome and quality of life. It ought to prompt us toward a few thought experiments about the work we do. To wit, if we had \$300,000 per year each year to spend on the healthcare of a child or adult with CF, what should we spend it on?

Now this is admittedly an odd question in the U.S. healthcare system of finance. No U.S. health insurance company would hand a clinician and patient such a sum of money to spend as they please, would they? These companies appear so far to be paying for Ivacaftor, but if I were the executive of a health insurance company, I might begin to consider other options. I might very well go to the head of the local CF center and ask: could you achieve a clinical improvement in this patient similar to Ivacaftor—for argument’s sake, a 15% improvement in forced expiratory volume in one second (FEV1)—with \$50,000 per year to spend as you please, every year for the next five years? In other words, doctor, can you get the same bang for less buck?

I think any CF clinician in the country could say yes. We need only to look at the quality improvement work done by the CF Foundation at far, far less cost—*astronomically* less cost—to imagine how this would be achievable. Every CF center in the country could achieve a substantial improvement in quality of care, in daily adherence, in prevention of hospitalization, in decreased days of school and work lost, and in almost any outcome you can think of if they had that money to spend. We could close the gap in outcome between CF centers in a heartbeat. We could achieve every goal of the Foundation’s quality improvement program within 1 or 2 years. If we had \$100,000 per year per patient to spend, we could do even more—and at such savings compared to Ivacaftor! And if, as expected, more therapies like Ivacaftor are shown to be effective for the majority of common CF genotypes, the money available to spend per patient is going to skyrocket!

Yes, this is speculation. And no, I do not think that insurance company executives will take me up on my plan, for obvious reasons. And it isn’t only money that prevents us from making these changes right now—although money on the scale of what we seem willing to spend on Ivacaftor would make a tremendous difference. And we ought not to assume that the current high cost of Ivacaftor is a settled issue. But case studies like Castro-Elias et al.’s ought to make us pause and think. What would we do if money on this scale were available for other uses?

We in CF already have so many advantages over other disease-specific communities in the United States. We have a long history of committed multidisciplinary clinicians

working under an effective model of care for which we take responsibility, as well as a strong community of basic and clinical researchers, and arguably the most effective disease research-advocacy foundation in the nation. We have a history of leading the way.

And so let me suggest that now is the time for us as a community to consider leading again. Let us take this opportunity to re-examine how we operate within the constraints of the U.S. healthcare system, to examine those factors that affect outcomes in CF that are not simply the addition of yet another incremental therapy, no matter how effective. At the very least, let us reconsider how we spend the resources we have, be they financial or otherwise. We

may come to the conclusion that things are just as they should be, but we might see a way to change the way we care for CF patients for the better.

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