

Specialist Discretion for Managing Specialty Pharmaceuticals: The Ethics Argument for Discretion

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Arguing against guidelines is a bit like arguing against motherhood and apple pie. Any reasonable person familiar with the way that health systems and managed care groups operate today will not reject the need for practice guidelines to improve appropriate care, reduce inappropriate care, and make the best use of human and financial resources. Indeed, the general case for guidelines is compelling and undeniable. However, to argue that the types of practice guidelines written for pharmaceuticals used in common diseases for which we have an enormous base of evidence can be applied whole cloth to guidelines for specialty pharmaceuticals is inappropriate. This

“argument by analogy” deserves to be challenged. Although guidelines are critically important management tools, they simply do not work in all situations.

Guidelines do not work well with specialty pharmaceuticals mainly because of the lack of an evidence base. Again, there is no question that diseases for which there is extensive clinical experience with existing medications (eg, diabetes, coronary artery disease, hypertension) are appropriately managed within practice guidelines. Typically, however, there is far less clinical history with injectables and biologics when they first reach the market. Even the evidence as reported in the product labeling for these specialty pharmaceuticals, such as etanercept and omalizumab, tends to be microscopic compared with the vast literature available on oral drugs for common long-term diseases. The number of patients studied in these registration trials—many of which have never been published in peer-reviewed journals—are usually quite small. Thus, even if the overall study design is valid (eg, rated 1a or 1b in terms of design) the statistical power of the resulting data is meager. This means that the expected range of variation in individual response to the new drug will be potentially greater. And this lack of predictability, in turn, implies that individual patients contemplating use of recently developed biologics and/or patients with less common diseases will do best when their treatment decisions are monitored on a case-by-case basis rather than by guidelines.

In these situations where written guidelines are not practical, specialists are the most reliable guides to proper therapy. These physician experts tend to keep up

with developments in their own fields. They stay on top of the literature and are even likely to be more current than the major treatment guidelines in their field since the large committees tend to meet infrequently. (These same specialists, remember, are also usually at the center of any committee that would meet to develop guidelines for the therapy in question.) Because they are typically ahead of the curve with many specialty society treatment recommendations, the specialist clinicians understand the process of using guidelines or package inserts as general roadmaps but make individual decisions for their patients and monitor the results carefully. In some cases, these specialists will also have personal early experience with an injectable or biologic and therefore be in a good position to adjust their treatment choices accordingly. This “familiarity with the unfamiliar” is what makes specialist physicians the natural pathfinders for the ongoing phase 4 clinical experience that is so critical for cautiously defining the proper use and diffusion of specialty pharmaceuticals.

In fact, allowing individual specialist physicians to make their own choices on specialty pharmaceuticals may be even more cost effective than attempting to create and enforce a narrow guideline. As just mentioned, individual specialists are more likely to be up to date with the actual evidence than any guideline panel instructions. This may explain why specialty drugs are less likely to be abused through overuse or inappropriate use than typical therapies. Prior authorization policies simply waste time and money in this situation. If the resources normally spent to incent specialists went instead into basic educational support for a more general prescribing policy, adherence would likely improve. At Kaiser Permanente, for example, specialists are given full clinical autonomy, including the ability to write a prescription for a drug off the formulary, and this has resulted in one of

the highest health system adherence rates in the country.

Granting patients and physicians full discretion in their use of specialty pharmaceuticals has an added ethical element that must also be part of this debate. Even in those situations where guidelines are routinely applied, it is imperative to have an appeals process in place. The application of population-based guidelines to patients with rare diseases has the potential to cause fundamental inequities in care. These patients and their specialists are known to fight more frequently for individual exceptions to prescribing rules. Thus, it is especially critical that health systems incorporate a consistent, fair, and transparent appeals process into any specialty guideline.

Finally, the lack of a deep evidence base for many of these specialty pharmaceuticals raises another ethical concern for health systems considering guidelines. Many patients have an inherent mistrust of the motives of managed care organizations in creating any guideline that tells clinicians how to practice. There is confusion, some of it justified, to whether the system’s real motivation is to reduce costs, limit dangerous therapy, or improve care. In fact, guidelines are often intended to increase the use of a certain drug to accomplish the twin goals of better care and reduced costs for hospitalizations or office visits. But whatever the underlying motivation for a specific guideline, the population-level evidence on which that guideline was founded provides the health organization with the moral ground to intervene with an individual patient—and even, in some cases, to incent clinicians to practice in a certain fashion.

With specialty pharmaceuticals we have less clinical evidence and therefore less moral ground on which to intervene in individual clinical decisions. This is the core of the argument against the use of guidelines—especially those with incentives—for specialty pharmaceuticals.