The Future of Health Economic Modeling: Have We Gone Too Far or Not Far Enough?

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As clinicians, we have to remind ourselves of the real motivation for health-care modeling. The development of models—like all clinical research methods—is a tool to help patients, to help the health-care systems act more efficiently, and ultimately, to achieve the goals of providing high quality and value in health-care services.

Have models had an impact? There is some evidence, although scant, that modeling does impact guideline development and reimbursement decisions. One large challenge, however, is that ultimately most “do we, don’t we?” decisions are still being made by clinicians at the bedside. There is virtually no evidence suggesting that models alone affect what clinicians do. Thus, we should not be overly optimistic that “better” models will ultimately change how we practice medicine. As we look into the future, the question to be asked is: Will models, no matter how complex or how simple, ever be accepted as a real time decision-making tool? Can we ever rely on the computer alone, as in 2001: A Space Odyssey, and put the trialists out of business except for the sole purpose of being able to validate an existing model?

In my view, the motivation for “incremental” modeling acceptance is quite straightforward: address the questions for which rigorous evidence is not currently available. On the success side of the equation, modelers have used a synthesis of data from various sources to extend the duration of trials, to examine populations not included in trials, or to address those questions for which trial data do not exist. Clearly, one advantage is the relative cost of simulations compared with the real cost of collecting empiric data. As we acknowledge how expensive it is to do trials, it is important to remember that it may be quite complicated to obtain all this information for models, particularly for those clinical conditions that are less widely studied. Modeling in this context has been quite useful in defining or refining research questions. Many in the community are quick to recognize these exercises as hypothesis-generating when compared with hypothesis-testing.

After examining David Eddy’s review of Markov models and the newer calculus-driven models, a fundamental question about models that arises is “How good is good enough?” The answer depends on the specific clinical or policy question. In many situations, the answer can be found in a rather short period of time (i.e., back of the envelope), or at least approached fairly efficiently, with data already available. To determine a model’s relevance, we need to ask “What do we know already?” It is fascinating when people outside the clinical field query “Why are you spending so much time simulating those diseases where the best trials have already been done?” I think there is some credence to the argument that we have been inefficient in our efforts. Why not devote our attention to those areas of surgical interventions or other rare clinical scenarios where there really are little or no data? Some of my colleagues in clinical medicine are almost practicing in a data-free environment. We need to determine what resources are available and invest in those areas where the data needs are most pressing. While more efficient allocation of modeling resources may help, we are still unsure whether key stakeholders will be influenced by the results.

Given the lack of enthusiasm of current models, Dr Eddy suggests that the next generation of models must become more “realistic” and be validated to become widely accepted. So are reality-based models really the answer? I think the answer is “It depends.” It is my impression that certain questions do not require overly complex models. And even in situations where they would be helpful, there are concerns that data requirements may not be met. As is the case for cost-effectiveness analysis, it would be interesting to examine whether it is worth the incremental cost to go beyond
a well-accepted, somewhat validated Markov model and create a reality-based model that David Eddy mentions. While there may be disagreement regarding the relative value of even more complex models, there should be near universal acceptance that internal and external validation of existing models will raise the bar for all types of models. Validation will most certainly raise acceptance, hopefully in the payer community, where there is usually substantial resistance—and sometimes frank skepticism—about this methodology in general. My own experience in US managed care indicates that concerns regarding accepting models, at least on the first glance, are not about the methodology or the complexity of the programming (the “engine”), but are aimed at the model’s assumptions or specific inputs. As a result, I personally remain unconvinced that more sophisticated models from the simulation perspective will get us over a very high hurdle to acceptance.

A number of issues persist when considering the future health of economic modeling. First, and the most important, is the need for model transparency. The fairly prevalent “black box” model should become a thing of the past. A corollary concern is the growth of “pay for play” proprietary models that limit access to a user’s willingness and ability to pay. Will proprietary incentives lead to the development of multiple, and potentially inconsistent, models? Second is quantifying data acquisition costs for complex models. In the case of a well-studied condition such as diabetes, for example, it is relatively easy to draw out the information needed for a complex model. In other conditions, however, the data simply are not there and acquisition may be prohibitively expensive. The third issue is dealing with conflicting evidence. When the data are unequivocal, when we simply know or do not know, the approach is rather straightforward. When the data conflict, the appropriate steps are not as clear-cut. A timely example would be cyclooxygenase–nonsteroidal antiinflammatory drug (COX-2–NSAID) conundrum regarding cardiac adverse events. There is one placebo controlled randomized trial concluding that the remaining COX-2 selective NSAID on the market—celecoxib—causes an increase in adverse cardiovascular events. A second randomized controlled trial, with a similar design and a similar drug, indicates that there is no effect. It is not certain how clinicians or decision-makers would ultimately be able to use even higher level of complex models to help answer questions, if and when trial data are conflicting. Unfortunately, there are many clinical examples where the available data do not shed a clear-cut answer. The last issue worth mentioning is how best to handle new data and the development of innovative interventions ("the moving target"). When the new diagnostic imaging technique, or the new drug, or the new genomic-based intervention, appears on the horizon with very little data available regarding its impact in the short term or the long term, how will those innovations impact these models? Given these concerns, it is worth repeating the great value of models as hypothesis-generating tools, and highlighting the limits of using many models to yield definitive results.

In my view, if health economics modeling is to play an increased role in the future, the field must move forward with five principles in mind. 1) Models must be tailored to the specific question at hand and to a specific audience. We must not succumb to the request that models become a panacea for all stakeholders when important questions remain unresolved. 2) Validation should become the norm. 3) The ability of models to test hypotheses should not be overstated—emphasize hypothesis generation. 4) Transparency and proprietary concerns must be addressed. 5) The usefulness of models vis-à-vis their impact on decision-making should be routinely assessed. This last principle should not be forgotten. If there is no measurable positive impact on patients and the health-care system at large from modeling, then all of our efforts may go for naught.