16.1 INTRODUCTION
Economic spending on healthcare and demographic changes in usage trends impact an increasingly global cost-conscious healthcare environment that is focused on optimal patient outcomes. The US, in particular, is facing the challenge of exacting the highest value and best clinical outcomes from per patient spending that is expected to increase by approximately two-thirds in the next decade. Historically, low-level US healthcare expenditure growth trends since 2008 have been attributed to the economic recession, patient reduction in healthcare spending from higher deductibles in private plans, and lower Medicare and Medicaid payments to providers (Kaiser, 2015, para 1). The Kaiser Foundation (2015, para 1) 10-year forecasts from 2015 through 2025 project that healthcare expenditures are expected to grow at approximately 5% year over year from approximately $9000 to more than $15,000 per patient. By 2020, the US expects to spend over $4.0 trillion on healthcare, representing more than 20% of the national gross domestic product (Kaiser, 2015, para 1). Although the US spends more per capita on its population than any other country, the nation continues to strive towards achieving excellent patient outcomes compared to the rest of the developed world (Davis et al., 2014). Consequently, providers and payers are looking for better ways to justify health care utilization and value to address the continuous increase in healthcare costs and the challenges associated with producing optimal outcomes through health economics and outcomes research (HEOR).

Differing payment models have been utilized to incentivize improved outcomes and decrease costs, including unique payment models (eg, capitation, bundled payments, and quality programs) and leveraging purchasing power (eg, integrated delivery networks, accountable care organizations, group purchasing organizations). These programs have utilized a variety of incentives to improve quality of care, but have had marginal success in controlling costs, and future efforts are being made by shifting more risk to healthcare providers. This is demonstrated by Medicare’s quality programs, which link patient quality measures to hospital reimbursement amounts and penalties (CMS.Gov, 2016). For example, beginning in 2017, US Centers for
Medicare and Medicaid Services (CMS) will penalize hospitals when patients who undergo a coronary artery bypass graft procedure and have a readmit back to the hospital in under 30 days of initial discharge (CMS.Gov, 2016, para 5). As a consequence of an increased payer focus on quality and outcome, hospital healthcare administrators are focusing increasingly on the cost of new medical devices and the impact on cost and hospital margins. However, contrary to this belief, medical device costs relative to overall healthcare spending have remained relatively stable over the last 25 years, ranging from 5% to 7% of total healthcare costs (Donahoe and King, 2014, p. 2; Ferguson, 2014, p. A2). Alternatively, the major cost driver in the hospital setting remains labor costs, representing close to 66% of hospital expenses (American Hospital Association, 2012, para 3). As a consequence of this misperception, device companies have had to become more strategic in their product commercialization process and consider health economics and evidence needs earlier in the typical product development stage gate process (device product development process divided by five to six gated stages typically, including various inputs from different functional areas). Health economics and reimbursement needs are typically included in the stage gate process and will normally include a global strategy where evidence needs may range from clinical efficacy documentation to formal cost-effectiveness assessments. Those companies that are able to assess value request needs earlier in the stage gate process and produce adequate evidence to support a new technology stand a stronger chance of diffusion of their technology. This chapter will focus on health economics for medical devices and the value assessment processes that produce clinical outcomes data. Then we discuss various types of economic evaluation strategies for medical device companies to improve market access from the perspective of different stakeholders. There is a brief discussion on health economic proxy measures and dissemination strategies and tools to produce health economic evidence. Finally, we conclude with the practical application of these methods in a case study example.

16.2 HEALTH ECONOMICS FOR MEDICAL DEVICES

Health economics for medical device companies may represent a variety of strategic needs under certain conditions in which cost evidence may be required and, alternatively, other business conditions or circumstances in which they are not. Economics typically focus on scarcity of goods, priority setting, opportunity costs, marginal benefits, and incremental analysis (Shiell et al., 2002).

However, traditional health economics is defined as “the study of how scarce resources are allocated among alternative uses for the care of sickness and the promotion, maintenance and improvement of health, including the study of how healthcare and health-related services, their costs and benefits, and health itself are distributed among individuals and groups in society.

World Bank (2016, para 1)

For some medical device companies, health economics may be represented by a focus on formal assessment and tools, such as building budget impact analysis or
cost-effectiveness studies, to be used with government policymakers or payer groups to illustrate therapy value. Health economic initiatives for smaller organizations may focus more on sales tools and reimbursement guides to convince hospital administrators of the value of their therapy or diagnostic. Health economics for device companies characteristically focus on illustrating value for a medical technology by attempting to increase reimbursement and market access. In general, device companies may have different criteria for health economic needs than pharmaceutical companies (Ferguson et al., 2014). One criterion for staffing or outsourcing in a health economics area is the anticipated need to create economic value messages and dissemination strategies for a technology, such as those stemming from an innovative premarket approval (PMA) therapy or novel diagnostic with anticipated widespread use. Health economics evidence may be required for a PMA product in the following circumstances: (1) a highly anticipated utilization of technology in the health system; (2) a high average sales price and needed justification; (3) a sales model that focuses significantly on global markets outside the US, which require formal economic evaluation; (4) product offerings that require considerable differentiation; (5) an anticipated pressure from providers on device price; and (6) questions arising on the products’ clinical utility.

Since a majority of devices are cleared through the 510(k) process, which is based on predicate devices already on the market, there may not have a great need for health economics at the onset of development for Class II products. Depending on differential and market access, 510(k) products may or may not have a need for health economics support similar to a PMA. Nonetheless, as healthcare resources become scarce and more people require increasingly complex treatments, the need for device-related health economics to justify utilization is expected to grow.

16.3 VALUE ASSESSMENT PROCESS

Health economics and reimbursement planning needs should occur early in the stage gate product development process. This includes an initial focus on the global assessment of health economic and health technology assessment (HTA) needs to support reimbursement and market access for innovative and PMA type products that commercialize outside the US. The priority given to requirements for markets external to the US are driven by the European market, where countries may explicitly ask for cost-effectiveness or budget impact analysis data, especially first to market technologies (Stephens et al., 2012). European nations have very formal HTA processes where national regulatory agencies, such as the UK’s National Institute of Health and Care Excellence or the Institute for Quality and Efficiency in Healthcare in Germany, may perform cost-effectiveness analysis (CEA) as a condition of reimbursement. If a device is being used to diagnose or treat in a relatively new market, an initial burden of illness study may be helpful. Results from the burden of illness study can improve understanding of device application by relaying the overall resource use of the pathology and where a new device could make an economic impact. Questions
typically asked within geography when considering a health economics and reimbursement needs include:

1. What is the coding and coverage landscape for new technology?
   If no coding and coverage exist, what level and type of clinical evidence will be required to create codes and coverage?
2. What is timeline and cost to create needed evidence?
3. Is there a specialty society willing to work with the manufacturer to create new reimbursement codes or change existing reimbursement codes?
4. Will the payers require economic evaluation as a condition of reimbursement? If yes, for which geographies?
5. Will hospital purchasers and providers require an assessment, such as results from a value analysis committee, as justification for product use?
6. Will a health economic assessment aid in the sales process, such as allowing marketing claims, or be a differentiator?

CMS is not permitted to directly include results from CEA in their evaluation of a new technology. However, they may indirectly consider the economics of a therapy, such as the budgetary impact of a new therapy, as a criterion for a new innovative technology add-on payment (Neumann et al., 2005). Nevertheless, while consideration for health economics at the US national level is not currently applicable, other payers (eg, private, state governments, hospitals) have begun to include these assessments when performing new HTAs. There is an expectation that cost-effectiveness will become a more common policy tool to help determine which device technologies to reimburse or use in a clinical setting, given the increasing-aged population that continues to change the map of insurance coverage and the overarching limitation on finite resources available for healthcare.

16.4 CLINICAL AND OUTCOMES DATA
The availability of clinical data will impact the ability to accurately describe or monetize the value of a technology. Therefore, both clinical and economic evidence are important drivers to market access and an essential part of the evidence planning process. Evidence plans must also include an evidence map with prioritized claims for a given technology, appropriate end points, and resource capture to complete the value story to support claims.

Typical approaches to evidence planning to capture clinical and economic data include adding specific metrics to a clinical study for economic evaluation or the addition of a “piggy back” type study to the clinical evaluation. The International Society of Pharmacoeconomics and Outcomes Research (ISPOR) has recently published a methods paper describing how to best align planned clinical studies to include economic evaluation (Ramsey et al., 2015). In practical terms, multiple studies and inputs may be required to adequately support a strong economic evaluation.
Many economic evaluations will include an assessment of the economic gain (benefit) in relation to outcome improvement when comparing two therapies over time. Economic metrics may be captured in clinical studies either by using (1) actual resource costs (eg, charges or cost/charge ratios) of interventions from the hospital charge master or UB-92 forms completed during a hospital stay, and costs of other health resources, which are less efficient; or (2) an effort will be made to capture resource use (eg, procedure, hospital stay information, medications, rehabilitation) and then monetizing with reputable standard sources, such as Medicare Severity Diagnosis Related Group (MS-DRG) payments or Red Book pharmaceutical costs, which is more common (for more information on MS-DRG and Red Book, see https://www.cms.gov/Medicare/Coding/ICD10/ICD-10-MS-DRG-Conversion-Project.html or http://micromedex.com/redbook). It should be noted that costs represent costs of a hospital or caregiver to provide a service, whereas charges represent the costs plus markup, which range from multipliers that are 2.6–4.7 times that of true treatment costs (Shannon and Joynt, 2013).

Outcome metrics typically captured in clinical studies may include a generic quality of life (QOL) measure, such as the short form 12 (SF-12), SF-36, and the EuroQol (EQOL 5D), which allows calculation of a quality adjusted life year (QALY), or another clinical measure of importance, such as disease occurrence or pain pre- to postintervention (Table 16.1). The SF and EQOL-5D include several domains (eg, pain, function) and provide a general assessment of the QOL intervention. In addition to general QOL assessment, a QALY estimate could be calculated to allow

<table>
<thead>
<tr>
<th>Costs (Resources)</th>
<th>Outcomes (Value Drivers)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Procedure, device used, and hospital stay costs</td>
<td>Pain scores</td>
</tr>
<tr>
<td>Adverse events during procedure or in follow-up</td>
<td>Functional changes</td>
</tr>
<tr>
<td>Medication use during treatment or diagnosis</td>
<td>Quality adjusted life year assessed through short form (SF)-12, SF-36, EQOL-5D [general quality of life (QOL)]</td>
</tr>
<tr>
<td>Rehabilitation utilization following procedure</td>
<td>Major incidence rate (eg, stroke, myocardial infarction)</td>
</tr>
<tr>
<td>Repeat procedures or medical care required, including 30-day readmit rates</td>
<td>Repeat surgery or readmit rate</td>
</tr>
<tr>
<td>Indirect (eg, lost work time, caregiver time, transport costs)</td>
<td>Disease-specific QOL (eg, Oswestry Disability Index, Minnesota Living with Heart Failure Questionnaire, Western Ontario and McMaster Universities Arthritis Index</td>
</tr>
<tr>
<td></td>
<td>Patient satisfaction</td>
</tr>
</tbody>
</table>

Compiled from personal experience.
comparisons between different disease areas by quantifying a hypothetical amount of health gain with an intervention. The QALY is used frequently in assessing how to allocate healthcare resources (Weinstein et al., 2009). The SF instruments have been used more frequently in the US to estimate QALYS, whereas the EQOL-5D is more common in Europe. Choosing which outcome measure to utilize in an economic intervention is dependent on stakeholder need and the potential to demonstrate the most impact in a given situation. For example, if an intervention is thought to reduce stroke incidence, an economic evaluation might consider the costs associated with stroke reduction between two interventions. In contrast, a formal QALY assessment may be included if the goal is make an argument that the economic impact is less for one medical intervention compared to another.

The most frequent economic evaluation, CEA, normally compares two interventions to indicate differences in costs to significantly improve a clinical outcome. Most medical device economic studies focus on direct costs and less frequently on indirect costs (eg, time out of work, caregiver support) due the difficulty in reliable data capture.

16.5 TYPES OF ECONOMIC EVALUATION

There are several types of economic evaluations, and a targeted strategy should be employed when determining which types of evaluations to consider (Table 16.2). Several commonly utilized economic studies with medical devices will be discussed as well as Simoens (2009)’s essential review on health economic methods.

16.5.1 ADMINISTRATIVE CLAIMS DATA STUDY, EXAMPLE, AND IMPLICATIONS

The goal of the administrative claims study is to illustrate trends in outcomes with a large data set, procedure volumes, or even areas of unmet clinical need. A claims database typically will include diagnostic and procedure information, basic outcomes, and sometimes cost data. This study type is generally used under two conditions: first, when there is anticipation that the length of time to generate robust results from a clinical study will be substantial, and second, when certain items, such as safety or cost of treatment, are under evaluation.

There are several sources for claims studies, including the Medicare MedPAR data set, commercial payer data sets (Optum from UnitedHealthcare), or even group purchasing organizations representing hospitals, such as Premiere. ISPOR maintains multiple links to international databases (http://www.ispor.org/DigestOfIntDB/CountryList.aspx). Many specialty societies, including the American College of Cardiology and the American Association of Thoracic Surgery, retain member reported registries that are accessible by society members to utilize for studies. Each resource has its own strength and weakness.

The more robust the data set, typically, the higher the cost to license or use the data. For example, patients tracked in Medicare allow assessment of resource use
Table 16.2 Most Frequent Types of Medical Device Economic Evaluation

<table>
<thead>
<tr>
<th>Type of Evaluation</th>
<th>Description</th>
<th>Stakeholder</th>
</tr>
</thead>
<tbody>
<tr>
<td>Administrative claims data study</td>
<td>A retrospective analysis of a large data set with the purpose of assessing safety, effectiveness, or costs. Sample sizes of several thousands are common.</td>
<td>Provider, payer</td>
</tr>
<tr>
<td>Burden of Illness</td>
<td>Study attempting to illustrate incidence and/or prevalence of a medical condition and economic burden to treat condition in region or geography.</td>
<td>Payer</td>
</tr>
<tr>
<td>Budget impact</td>
<td>Estimation of the financial consequences of adoption of a new device within a specific healthcare setting (potentially replacing another). In particular, a budget impact analysis predicts how a change in the mix of devices and other therapies used to treat a particular health condition will impact the trajectory of spending on that condition.</td>
<td>Hospital, provider, payer</td>
</tr>
<tr>
<td>Cost-effectiveness analysis (CEA)</td>
<td>A comparison of the costs and health effects of an intervention to assess the extent to which the intervention can be regarded as providing value for money or possibly cost savings. A cost-effectiveness study will use an incremental cost effectiveness ratio, which compares the incremental difference of treatment costs between two interventions and differences in outcomes.</td>
<td>Provider, payer</td>
</tr>
<tr>
<td>Cost utility</td>
<td>A cost utility analysis is a form of CEA. The cost utility analysis utilizes quality adjusted life years (QALY) as the outcome metric. QALYS are typically assessed with validated patient reported questionnaires, such as the EQOL-5D or SF-36.</td>
<td>Provider, payer</td>
</tr>
<tr>
<td>Markov modeling</td>
<td>Markov modeling is form of decision analysis, which tracks probabilities and costs of patient health states over time. This allows for forecasting potential economic benefits of an intervention or diagnostic compared to another over a long time period.</td>
<td>Provider, payer</td>
</tr>
<tr>
<td>Time motion study</td>
<td>The time motion study assess the impact of a technology on efficiency gains, typically by measuring impact on time of specific activities in a medical process or workflow.</td>
<td>Hospital</td>
</tr>
</tbody>
</table>

regardless of where treated over time, whereas a data set such as Premiere only allows patients to be tracked who receive treatment within network during the course of their treatment. From a costing standpoint, an organization such as Premier allows more granularity when it comes to tracking episodic costs (UB-92 captured), versus Medicare, which utilizes various charges and charge-to-cost ratios. Overall, these types of administrative claims data have numerous uses and can serve as a bridge before formal clinical study data is available.

An example of a claims study can be found in a study conducted by Brown et al. (2008, p. 1980). This study examined the cost of complications for Medicare beneficiaries who survived coronary artery bypass surgery (n = 114,000+) without concomitant valve procedures. The authors found an average treatment cost of approximately $32,000 and an average length of stay (LOS) of 9.9 days. Those with serious complications had an incremental cost of $15,468 and added LOS of 5.3 days. This study allowed a snapshot of the financial impact of those patients experiencing complications with a complex procedure and established areas for healthcare improvement.

Further, the Brown et al. (2008) study allowed the assessment of costs of treatment as well as the incremental cost of complications of cardiac surgery. Policymakers and payers typically use this type of information to help make decisions related to therapy access, coverage policies, as well as evidence strength in HTAs. Medical device manufacturers could use claims studies such as this to help identify where better therapies could be developed to reduce complications, as well as illustrate the effectiveness of specific therapies to treat patients with heart failure. A claims study of this size would certainly obtain major attention at a cardiology specialty meeting and perhaps help drive uptake and market access with providers.

16.5.2 BURDEN OF ILLNESS STUDY, EXAMPLE, AND IMPLICATIONS

The burden of illness study has several goals, which include bringing an awareness of the (1) incidence and/or prevalence of a specific medical condition, (2) natural history of condition, (3) impact of condition on QOL, (4) mortality and morbidity of the condition, and (5) economic and resource cost of the condition. The burden of illness study may be performed in an effort to influence stakeholders if there is a perceived lack of awareness on the economic or clinical impact of a medical condition.

In a study by Turakhia et al. (2015, p. 733), the authors examined the economic burden of undiagnosed nonvalvular atrial fibrillation (AF) in the US. The authors used two large administrative data sets and estimated the incremental costs of undiagnosed AF by comparing annual medical costs for patients with AF to propensity matched controls, and then they multiplied by prevalence in the same data sets. The authors found $10,355 yearly incremental health expenditure in the nondiagnosed AF patients as compared to similar patients without AF. Using US prevalence estimates of this population, the authors concluded that the US incremental direct medical cost of undiagnosed nonvalvular AF is approximately $3.1 billion annually.
The implication of their findings is profound because the study identifies an unmet medical need that could lead to actual savings for the US healthcare system simply by diagnosing these patients earlier. This study also provides information on the importance of this clinical problem for developers of new diagnostic products and payers who would also be mindful of identifying and using interventions that help to identify these patients earlier.

16.5.3 BUDGET IMPACT ANALYSIS, EXAMPLE, AND IMPLICATIONS

A budget impact analysis addresses the expected changes in the expenditure of a healthcare system after the adoption of a new intervention by estimating the likely financial consequences of that decision for a healthcare system (Sullivan et al., 2014). Commercial payers, government policymakers, and even hospitals perform budget impact analyses to assess impact on their yearly budgets. A real world question might include, “How much can we delegate to prevention versus treatment, and what is the potential impact on mortality or downstream healthcare costs?”

A budget impact analysis study by Saadi et al. (2011, p. 332) compared the budget impact of four drug-eluting stents (DES) for diabetic patients with cardiovascular disease from a commercial payer perspective. As part of the analysis, the authors observed that by substituting the CYPHER DES for other commercial DES with higher target lesion revascularization rates (3.2% versus those with ≥ rates than 6.9%) in a population of 200,000 beneficiaries, the results predicted annual cost savings of greater than $146 million per population per year ($733 per patient). The researchers were able to illustrate that by changing one type of treatment and reducing potential adverse events associated with the other DES, the potential monetary impact was significant.

The importance of the DES study illustrates potential cost savings when utilizing more effective technology. In this case, the avoidance of complications was a big factor in reduced per patient costs, highlighting how innovative technologies can reduce healthcare costs and improve outcomes. DES purchasers in this example might very well change their procurement decision based upon studies such as this, especially if clinical benefit is similar. Results may also elicit payers to make changes to coverage determinations.

16.5.4 COST-EFFECTIVENESS ANALYSIS/COST–UTILITY ANALYSIS, EXAMPLE, AND IMPLICATIONS

CEA is a comparative analysis of two or more alternative interventions in terms of both their health effects and cost (Noyes and Holloway, 2004), while the main purpose of the CEA is to identify strategies that maximize health gains with the resources at hand or to improve value for money (Young et al., 2013). The main metric of the CEA is the incremental cost-effectiveness ratio (ICER). The ICER is the ratio of the incremental cost of the new therapy compared to
the standard therapy divided by the incremental measure of the health benefit (Eze-Nilam et al., 2014).

\[ \text{ICER} = \frac{(C_1 - C_0)}{(B_1 - B_0)} \]

\( C_1 \) indicates total costs of treatment of the new therapy over an agreed-upon time horizon (study time period), and \( C_0 \) represents costs of the new therapy over that same time horizon. \( B_1 \) represents outcome/effectiveness during the study time horizon for the new therapy, whereas \( B_0 \) represents effectiveness for the standard therapy. Time horizons are chosen based upon availability of data, but typically range from 1 year, 2 years, 5 years, or the predicted future lifetime of the patient.

Effectiveness can be measured several different ways. In a true cost-effectiveness assessment, effectiveness is expressed by a clinically meaningful event, such as cost of stroke avoided, pain reduced, or heart attack detected. A form of CEA is cost utility, which utilizes QALYs as an effectiveness measure. The QALY takes into account the quality and quantity of a health intervention and allows generalizability between medical interventions. A QALY places a weight on time in different health states. A year of perfect health is worth one, and the most severe health state is zero (Philips, 2009, \textit{para 1}).

Use of QALYs as an effectiveness measure is the most common metric for effectiveness in CEA studies. Traditionally, a measure of success for CEA studies in the US are interventions that demonstrate an incremental improvement in health for less than $50,000 per QALY/stroke avoided, etc. (Neumann et al., 2014). The $50,000 threshold is not a solid rule, but instead an estimated figure based on society’s willingness to pay. Some experts suggest that a therapy should be considered cost-effective at thresholds greater than $100–150K per QALY gained, especially if the therapy is life saving (Neumann et al., 2014). Outside the US, other thresholds have been considered, such as €20,000–30,000/QALY in the UK (Young et al., 2013). Tufts Medical Center maintains a database of current studies that serves as a tool to assess which technologies are considered cost-effective (www.cearegistry.org).

Health economic studies in the US have been viewed as a guide in policymaking, whereas in other geographies, CEAs have been used as part of the formal decision-making process on coverage determination. However, there are specific regions and HTA groups in the US that consider economic value in their decision-making progress, such as in Washington state.

There are also other ways of illustrating CEA benefits in studies, including the CEA plane (Fig. 16.1) or cost-effectiveness acceptability curves (Eze-Nilam et al., 2014). A complete discussion of these graphic alternatives is beyond the scope of this chapter. However, the difference in cost and effectiveness between interventions is commonly represented on 1 of 4 planes with a scatterplot or “scattergram of distribution.” Depending on distribution within a specific quadrant, a new intervention may be more or less cost-effective and helps to determine if a new therapy is dominant over the comparator or the existing technology. For example, if points fall in quadrant 4, the comparator dominates the new technology and comparator should be adopted. Conversely, if points fall in quadrant 2, the new therapy is dominant over the comparator and should be adopted.
Types of Economic Evaluation

A second way of illustrating the quadrant concept is through plotting willingness to pay thresholds on the x-axis and probability that the new therapy has an ICER below the threshold on the y-axis. The strength of using an acceptability curve is that there is a better understanding of the willingness to pay for a therapy versus the use of the default $50,000/effectiveness measure (Eze-Nilam et al., 2014). Given that cost-effectiveness is the most frequent economic evaluation of medical devices, manufacturers should expect to see more requests to show cost-effectiveness value of their therapies.

A good example of a cost-effectiveness/utility assessment was conducted by Reddy et al. (2015, p. 2728), examining the time to cost-effectiveness following stroke reduction strategies in AF, comparing Warfarin versus novel anticoagulants (NOACs) versus percutaneous left atrial appendage closure (LAAC). Relative to Warfarin, LAAC was cost-effective at 7 years ($42,994/QALY), while NOACs were cost-effective at 16 years ($48,446/QALY). LAAC was dominant over NOACs by year 5 and Warfarin by year 10. The authors concluded that both NOACs and LAAC with the WATCHMAN device were cost-effective relative to Warfarin, but LAAC was also found to be cost-effective and to offer better value relative to NOACs (Reddy et al., 2015).

Cost-effectiveness studies are the most common economic evaluations of new medical technology. Many countries use this method as part of their coverage decision process. The referenced study illustrated the benefit of managing the left atria with a percutaneous device as compared to medical management and the cost-effectiveness over a period of several years (Reddy et al., 2015). The cost-effectiveness was below the $50,000/QALY threshold but only after 7 or more years. This may be a good purchase in societies in which a single payer system is present. However, societies in which there is a combination of commercial and government payers, where patients frequently change payers, a longer time horizon could prove
more challenging for coverage. Nonetheless, cost-effectiveness will become more prominent over time, given the finite resources and need to illustrate value. This type of study also illustrates the type of evidence several stakeholders will expect with new technology that has a large potential utilization or high cost.

16.5.5 MARKOV MODELING, EXAMPLE, AND IMPLICATIONS

Markov modeling is a form of decision analysis that assigns disease states for patients (e.g., healthy, adverse event, disability, death) over a patient’s life span, including probabilities and costs for each disease state. Markov models typically are used in combination of cost-effectiveness models and allow for a long-term estimation of potential economic impact.

The key features of a Markov model are:

1. that individuals reside in one of a finite set of mutually exclusive health states;
2. that time is represented by discrete periods called cycles, and individuals move between health states, or remain in their current health states, at the end of each cycle; and
3. that movements are governed by transition probabilities that are specific to each health state and each cycle.

The main advantage of incorporating Markov modeling into a decision analysis is because the method allows for the simulation of more complex consequences of an option, a greater number of possible events to be included in simulation, and the events can be simulated for lengthier periods downstream (Ademi et al., 2013).

A study by Cher et al. (2015) examined the cost-effectiveness of minimally invasive sacroiliac joint fusion surgery. Data from two clinical studies were used to build a Markov cost–utility model to evaluate the cumulative impact of 5-year health quality and costs after minimally invasive sacroiliac fusion or nonsurgical treatment. Sacroiliac fusion was associated with a gain of approximately 0.74 QALYs at a cost of US $13,313 per QALY gained (Cher et al., 2015, p. 1). The procedure provided potential cost savings per QALY gained compared to nonsurgical treatment with a time horizon of greater than 13 years. The study illustrated with modeling that there may be a cost benefit with a long time period, as compared to nonsurgical treatment.

The Markov model provides an example of how value of a therapy can be modeled over a number of years, even with a limited amount of data. This type of analysis allows illustration of long-term value and potential economic implications for payers and clinicians. The authors in this study were able to illustrate the long-term potential benefit of fusion surgery even though their model did not include 5 years of data due to lack of availability.

This approach is frequently used in health economics and has a big impact that is most evident in countries outside the US that utilize economics as part of formal
16.6 OTHER HEALTH ECONOMICS PROXY MEASURES

The performance of health economic evaluation is not always practical due to the length of time necessary to develop adequate clinical and economic data for models to make a significant impact. In these instances, there are examples of proxy measures at both the macro- and microeconomic levels. At the macro level, for example, measures of healthcare spending per person may be considered a proxy for aggregate healthcare demands. At the patient or micro level, items such as resource use, medication use, medical procedures, hospital readmits, clinical staff time, bleed rates, or adverse events have been utilized (Glassman et al., 2006). A proxy measure allows the articulation of value for a given technology in the event that it is not possible to monetize and/or express per unit of outcome gain. Healthcare payers and providers recognize that increased resource use equates to increased spending and/or time, and in many instances, a compelling argument can be made to impact coverage determination based upon this type of information. When rationalizing the use of proxy measures in clinical studies, there are important steps to consider, such as assessing stakeholders who would be impacted by resource use (eg, hospital, payer, provider), how the therapy would benefit the stakeholder, and the type of value metric that would be required (Giuliano et al., 2012).
16.7 DISSEMINATION STRATEGIES AND TOOLS FOR HEALTH ECONOMIC EVIDENCE

There are several strategies to disseminate and use healthcare economic value messages. Traditionally, companies develop economic studies and either present economic model findings at a conference or publish in a peer-reviewed medical journal with a key opinion leader (KOL). More sophisticated types of technologies have been utilized to illustrate economic models. These include developing models using standard spreadsheet and statistical analysis software, such as Microsoft Excel, and utilizing Apple iPad to illustrate the budget impact of a technology for purchasers of that item. An interesting example of this is illustrated by Yan et al. (2014). The authors examined the budget impact of a new dressing on leg wound ulcers and were able to demonstrate a sizeable cost savings with an Excel-generated model (Yan et al., 2014). In addition to models, simple economic calculators are sometimes used to illustrate value of therapy commonly used by hospital financial administrators (Trogdon et al., 2015). Finally, many use economic white papers that can deliver economic messages. This documents also serve as a “leave behind” that could be useful for hospital value analysis committees.

Many companies do not build calculators or models described from concept but elicit the support of external consultants. Companies such as BaseCase (Berlin, Germany), Abacus (Oxfordshire, UK), and others have perfected the ability to place models on iPad technology to allow sales representatives or reimbursement field people to utilize real-time health economics information. In addition to budget impact models, interactive economic models on iPads may also allow the assessment of procedure volumes, outcomes data, regional reimbursement information, and benchmarking with other hospitals. The right dissemination tool should be chosen based on anticipated use and with the primary stakeholder(s) in mind.

16.8 CONCLUSION

In conclusion, HEOR strategies are increasingly important to support reimbursement and market access with existing and newly introduced medical technologies. Those companies that illustrate value for their technologies stand a better chance at increasing market access and, ultimately, technology diffusion. Companies should assess stakeholder evidence needs, especially from payers and hospitals, in the process of determining their health economic needs. Various therapies that are novel, PMA/significant risk, high cost, or diagnose/treat a high incidence will most likely require some type of health economics support. Evidentiary support should be included in the overall global evidence plan. This may include tactics that range from building in appropriate clinical study end points to full-blown economic models that can be disseminated as publications or real time on iPads for field personnel. For smaller companies who may not have internal personnel with an expertise in this area, several excellent healthcare economic vendors can support needs in the US (eg, Boston Health Care, Covance, Technomics, PHAR) or outside the US (eg, Synergus, GfK Bridgehead, or MediClever). These vendors typically are well
versed in the particular requirements in different geographical regions and can draw upon a vast array of specific medical device experiences. Going forward, manufacturers must understand that health economics, that is, illustrating evidence-based value, will be as important as any other part of the product development process.

**CASE STUDY**

A case study is presented to give perspective on how formal health economics and outcomes research (HEOR) can support market access and the sales process of a new medical technology. In this example, we will assume a company is bringing a new hypothetical PMA cardiac therapeutic product to market. The clinical indication is for a new heart treatment device that is a high-risk Class III designation according to the FDA because there are no existing market predicates. Thus, the therapy is considered a breakthrough technology with little clinical evidence. The product is in process of Conformité Européenne (CE) marking in the EU. The average US list sales price will be approximately $18,000, and the device has neither reimbursement codes nor existing overage. The company has completed a pilot study where the clinical data shows 85% effective in their cardiac-oriented primary end point. This is a medical condition that is normally treated with medical management and has about 40–50% efficacy. The clinical incidence and prevalence of the condition affected is high with a high probability of long-term treatment success. Following CE marking in Europe, the company gains FDA approval and decides to embark with a PMA clinical trial for a new label indication. A health economic evidence assessment is completed to determine strategies to increase market access as part of the reimbursement assessment. What would be an appropriate set of health economic strategies to support this new therapy? Take a moment to review the information provided in this document and to consider other sections of this book in order to determine what options may apply to this scenario.

Potential strategies to support reimbursement, market access, and ultimately an uptick in sales volume include the following:

- Build planned evidence map by geography for clinical and economic dissemination needs.
- Following CE marking, consider conducting an additional clinical study outside the US to generate outcomes or proxy measures that can illustrate economic value in peer-reviewed publications as well as real world use data.
- As part of FDA Investigational Device Exempt (IDE) trial, collect outcomes to allow illustration of economic value (proxy or more formal economics) and include formal preplanned subordinate analyses for economic metrics.
- Consider a pre-IDE study payer meeting to discuss evidence requirements to support coding and coverage needs, possibly both CMS and representatives from private payers.
- Work with clinical provider KOLs to present clinical results at impactful society meetings as data becomes available in the FDA trial and outside US studies.
- Develop health economic models (cost-effectiveness or budget impact) to support differing geographic needs, with a big focus on outside the US.
- Work with specialty societies with available evidence to support new Current Procedural Terminology application to the American Medical Association, if required, including guideline development inclusion and CMS for new MS-DRGs, if required.
- Share clinical and economic data, including budget impact to commercial payer medical directors to increase chance of coverage support.
- Continue to assess HTA requirement in key geographies and global evidentiary needs that are outside the US (OUS).
- Determine and build sales support tools, ie, value add committee material and slides.
- As more data is developed, build a clinical and economic dossier that allows use as part of the technology assessment and coverage reviews.
- Assess impact of health economic build through reimbursement availability, sales support, unit sales, etc.
Did the Strategies You Suggested Match Up With the Proposed Items?

The strategies listed above may not all be necessary to drive reimbursement, market access, and ultimately sales, but have the potential to contribute to accelerating product diffusion. However, there are plenty of examples that illustrate completing all of the above items and still not securing strong coverage and reimbursement. Payers are increasingly requiring more safety and effectiveness data as a condition of coverage, and even the best strategies face increasing difficulties to obtaining simultaneous full coverage in multiple geographies.

DEFINITIONS

Costs  Represents monetary costs of a health care diagnostic, intervention, or service associated with healthcare.

Cost-effectiveness Illustration of one technology demonstrating better economic value in relation to clinical measure over a period of time.

Charges  Represents hospital or provider costs plus markup, typically 300–1000% of true costs in healthcare settings.

Clinical outcomes Clinical measure to assess clinical benefit and success of intervention.

Comparator An existing medical diagnostic or intervention that is used as a comparison arm with the new technology, typically in form of a study.

End points Typically a major measure of clinical or economic success of an intervention.

Health economics The use of economic data or proxy information to articulate the value proposition of a technology.

Monetize The process of assigning monetary value to a resource or health service.

PMA Premarket approval or process that a significant risk device; Class III device undergoes to obtain FDA approval; typically an IDE study is required to illustrate safety and efficacy of the technology in this classification.

Proxy measure A surrogate measure representing healthcare resource use; the proxy measure allows inferences about potential cost impacts associated with a technology.

Value Use of economic or financial data to illustrate that there is an acceptable clinical outcome for the resources or dollars expended with a technology.

Value analysis committee Hospital committee that evaluates the appropriateness of a new technology to be used at the hospital, typically based on clinical and economic value.

REFERENCES


RECOMMENDATIONS FOR ADDITIONAL READING


