The pharmaceutical industry faces a well-documented perfect storm: on the one hand, the patent cliff; the lack of new blockbusters and, on the other, economic pressure on pricing from markets with growing expectations and shrinking budgets. In the face of such pressure, traditional health economics models no longer seem appropriate and yet what do we have to replace them? The growing focus on ‘value’ and ‘cost effectiveness’ are evidence of new emerging thinking although, even here, with the shift from medicine as cure to medicine as palliative, as a treatment for chronic illness and with the growing emphasis on preventative approaches, the landscape is complex and challenging.

*The Future of Health Economics* offers a window into some of the most influential emerging issues in pharmacoeconomics; issues such as risk-sharing and alternative pricing models or the potential impact of radical new approaches such as personalized medicine; as well as exploring the changing role of government and regulators.

Ulf Staginnus and Olivier Ethgen, themselves two of the most well-regarded practitioners in this field, have brought together some leading-edge thinkers from industry and academia around the world to provide the industry, policy-makers, regulators, health practitioners and academics with the raw material for their future scenarios.

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List of Contributors

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management, and patient-centered clinical development. Dogan is a Doctor of Medicine, has a PhD in Public Health, an MSc in Health Economics and an MBA.

Amiram Gafni: Amiram is a professor in the Department of Clinical Epidemiology and Biostatistics at McMaster University in Ontario and a member of the Centre for Health Economics and Policy Analysis. He has more than 450 publications in peer-reviewed journals. In the 2011 World Bank rankings of health economics researchers (see _Journal of Health Economics_, 2012, 31:406–39 (Table 5)) he was ranked equal 35th in the world (equal 1st in Canada) based on the volume and impact of his publications. His research interests are in the area of economic evaluation of health care programs (both methods development and empirical applications), modeling of consumers’ health-care behavior, models of patient–physician decision making (e.g. shared decision making), policy analysis and risk and decision analysis in health.

Larry Gorkin: Trained as a PhD Clinical Psychologist, Larry was drawn to research and began his career as a grant writer to the National Institutes of Health and the pharmaceutical industry. At that time, he was associated with the Brown University School of Medicine, where he received his post-doctorate degree. In 1996, he was employed within health economics at Pfizer world headquarters in New York City. He spent the bulk of the next 13 years as a special advisor to the head of health economics at Pfizer, working on licensing opportunities, critical competitive challenges, etc. After leaving Pfizer in 2009, Larry began a small consulting firm focusing on health economics in drug development, and started writing reports that he placed on the internet gratis on reforming aspects of the industry.

Lamiae Grimaldi-Bensouda: Lamiae is one of the founders of the PGRx platform, a widely used pharmacoepidemiological information system. She has more than 12 years’ experience in clinical pharmacology (board-certified) and pharmacoepidemiology (PhD), notably in the conduct of real-world studies. Specializing in methodological development as directly applicable to real-world studies, her experience includes the design, conduct and analysis of major, landmark studies. She has the unique experience of both primary data collection from physicians and patients and of the analysis of electronic health care databases. Lamiae has conducted more than 20 major studies involving clinical, patient-reported and functional outcomes assessment internationally and an additional dozen studies using eHCD or EMR, and numerous surveys. She is part of the PROTECT program (Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium) coordinated by the European Medicines Agency (EMA). In 2006, Lamiae established a clinical and academic career as chief resident in clinical pharmacology at a university hospital in France where she ran the regional pharmacovigilance unit. She is now an Associate Professor at the Conservatoire National des Arts & Métiers where she directs a Master Program on Drug Evaluation, Market Access & Public Health and is a Fellow of the Pasteur Institute.

Eugenia Gruzglin: Eugenia has more than ten years’ experience in the health care industry. She has worked in a variety of therapeutic areas, including oncology, immune-oncology, cardiometabolic disorders and multiple sclerosis, to mention just a few. In addition, she is well familiar with novel technology platforms
and a variety of diagnostics. Eugenia received her PhD in molecular oncology from Mount Sinai, NYU, and completed her post-doctoral training at Memorial Sloan-Kettering Cancer Center in New York City. She has coauthored a number of peer-reviewed articles and presented her work at international meetings. Based on her PhD work, she holds a provisional patent in cancer diagnostics and cancer treatment.

**Eddie Hornby**: Eddie is an independent consultant with more than 30 years’ experience gained in the pharmaceutical industry internationally in roles including preclinical, biochemistry and pharmacology, clinical trials science and health economics. He has worked in most major disease areas and also in designing cross-functional processes for integrating payer and regulatory insights into early and full clinical plan development and market access planning.

**Claudio Jommi**: Claudio is Associate Professor of Management at the Department of Pharmaceutical Sciences, University of Novara. He is also Director of the Pharmaceutical Observatory at Cergas (Centre for Research on Health and Social Care Management), Bocconi University, and Professor at Bocconi School of Management, Public Management and Policy Department, where he coordinates the specialization in Pharmaceuticals and Medical Technologies, Master in International Health Management Economics and Policy (Mihmep). He is President of the Italian Health Economics Association. He has been visiting professor at Essec Business School, Cergy (Paris), at the Herivan K. Haub School of Business, St Joseph University, Philadelphia (USA), at the Andrija School of Public Health, Zagreb and the University of Claude Bernard Lyon 1 (European Market Access University Diploma). His research activity is focused on pharmaceutical economics, policy and management, health technology assessment and decision making in health care. He has published in many international and national journals, including *Drug Design Development and Therapy*, *European Journal of Health Economics*, *Health Policy*, *International Journal of Health Planning and Management*, *Journal of Medical Marketing*, *Pharmacoeconomics*, *PLOS Neglected Diseases* and *Public Money & Management*.

**Ning Lu**: Ning is a freelance consultant with more than ten years’ international consulting and biopharmaceutical experience. Her professional experience spans across pricing and market access, health care systems, hospital management, business analysis and strategy. She has conducted projects in Europe, Asia and the US covering central nervous systems, oncology, gastrointestinal disorders and rare diseases. Most recently she worked as Senior Manager Market Access at Biogen International, focusing on European Market Access. Ning received her MPH from Harvard University and her MBA from the University of Michigan.

**Kevin Marsh**: Kevin is Senior Director of Modeling and Simulation at Evidera. His research interests include the use of economic and decision analysis to inform health decisions, including pipeline optimization, authorization, reimbursement, and prescription decisions. He specializes in decision modeling, MCDA, and a range of economic valuation methods, such as stated preference value approaches. He actively contributes to the methodological development of these techniques. He is currently co-chairing the International Society for Pharmacoeconomics
and Outcomes Research Taskforce on the use of MCDA in Health Care Decision-Making. He has applied these and other research techniques for a range of organizations, including both regulatory and industry clients. Kevin completed his PhD at the University of Bath, specializing in economic valuation techniques. After a year at Oxford University, he joined the Matrix Knowledge Group in London, where he built the economics practice. Kevin is an active member of the Campbell and Cochrane Economic Methods Group.

**Thomas Morel:** Thomas is Research Fellow within KU Leuven’s Department of Pharmaceutical and Pharmacological Sciences, Belgium. His primary research focus is rare diseases and orphan drug policy. Thomas holds degrees in Economics and Law from the London School of Economics and Political Science and King’s College London.

**Cédric Popa:** Cédric is Partner at Deloitte and has led the valuation team in the financial advisory department since 2008. Cédric’s experience includes valuations of businesses and intangible assets for commercial and strategic purposes, in litigation contexts for tax purposes and also for financial reporting. Cédric has more than 14 years’ experience in various industries, including life sciences, technology, media and communications, manufacturing and food and beverage. In the life sciences sector, his projects have included valuation of the R&D portfolio in a €2 billion-plus pharma acquisition, benchmarking studies on the financial and operating performance of leading pharma companies, co-authoring an article on financial performance of orphan drug companies, market strategy advice to medical device and pharmaceutical companies, and more. He has spoken on valuation issues at several external and in-house seminars and is a member of the UK Society of Share and Business Valuers and of the Royal Institute of Chartered Surveyors. Cédric holds an EMBA from the University of Washington and a BA in Economics and Econometrics from the University of Kent at Canterbury.

**Antonio Ramirez de Arellano:** Antonio is Regional Manager of the Novo Nordisk HEOR European team. He was awarded his BSc degree in Economics from the University of Barcelona, his MSc degree in Health Economics from the University of Barcelona, his MSc degree in Economic and Social Policy Analysis from the Department of Economics, University of York and his PhD degree in Economics from the University of York. The subject of his doctoral thesis was data envelopment analysis (a non-parametric method for estimation of production frontiers). He was a lecturer of Macroeconomics, Microeconomics and Health Economics in the Department of Economic Theory at the University of Barcelona, and he also ran the *Spanish Journal of Health Economics*. From 2000 to 2004 he was the national health system advisor in the cabinet office of the Spanish prime minister. Antonio has worked as a health economist in the pharmaceutical industry (Roche, BMS, Ferrer International and Novo Nordisk).

**R. Ömer Saka:** Ömer leads the Deloitte Center of Excellence for Market Access Strategy, Health Economics. Ömer and his team support medical devices and pharmaceutical companies in three high-level health economics aspects, which include value generation, value illustration and value communication, with related activities spanning from portfolio management to health economics.
modeling, pricing, writing and adaptation of value dossiers, market access strategy as well as the design and the analysis of observational studies. In the past, Ömer worked for five years at the London School of Economics and Political Science and King’s College London. He later joined the National Audit Office of England to lead the health economics and decision modeling practices there. He produced influential work in the area of stroke, which resulted in the implementation of the 2007 UK National Stroke Strategy in a collaborative process with the Department of Health and academic organizations. Ömer has been involved in a number of consultancy projects for governmental organizations in the EU, the UK, Turkey and South Korea. He has published on issues such as the use of health technology assessment methods in orphan drugs policy and has research interests in the use of modeling methods in economic evaluation, comparative health policy analysis and efficient provision of services in hospitals. Ömer is a medical doctor by education.

Ed Schoonveld: Ed is a Managing Principal with ZS Associates in New York and is the leader of the firm’s market access and pricing practice. He has extensive experience in pharmaceutical marketing and pricing from both the corporate pharma and the consultancy perspective. His team advises drug companies on product pricing and market access strategy, global pricing policy, and internal organizational and process challenges. Most of these projects involve global payer and pricing research through a host of qualitative and quantitative methodologies. Ed’s expertise in global pricing and reimbursement is extensive – he has served both on the affiliate level as a general manager of a European affiliate and at corporate headquarters as the responsible leader for global pricing and health economics groups at Wyeth, Eli Lilly and BMS. Ed’s considerable experience in the pharmaceutical industry includes various sales, marketing and general management positions with Lederle, Wyeth, Eli Lilly and BMS in the US and Europe. Ed has also led pricing and reimbursement consulting practices for Analytica International, Cambridge Pharma/IMS and his own consultancy firm. Ed is the author of The Price of Global Health, a groundbreaking book on global drug pricing. The second edition was published in January 2015.

Steven Simoens: Steven is a Professor of Pharmacoeconomics at KU Leuven. He is a health economist and is involved in research and teaching of pharmacoeconomic aspects of medicines, medical devices and related products. He is the current head of the unit Clinical Pharmacology and Pharmacotherapy in the Department of Pharmaceutical and Pharmacological Sciences at KU Leuven. His research interests focus on issues surrounding policy and regulation of the pharmaceutical sector. He has worked extensively in the area of pricing, reimbursement and distribution of orphan medicines and of generic medicines in Europe. Steven also carries out economic evaluations of medicines and medical devices. He has been involved in multiple health technology assessments of antibiotics and of medical devices. He was involved as an expert in drafting the guidelines for pharmacoeconomic evaluations in Belgium. Steven has more than 220 publications in peer-reviewed journals and has given over 240 congress presentations. He is a member of the editorial board of multiple journals. He has lived and worked in England, France, Germany and Scotland.
Justine Slomian: Justine trained as a midwife and has professed in this area for three years. In 2013, she also obtained a Master’s degree in Public Health Sciences with epidemiology and health economics majors. She is now undertaking a PhD research program at the University of Liège in Belgium. She studies the usefulness and relevance of new information and communication technologies in the management of postnatal stress and depression. She has previously studied the influence of new information and communication technologies in various fields such as osteoporosis, menopausal women or cardiovascular diseases.

Baudouin Standaert: Baudouin has a 20-year career in industry (ten years at Amgen and ten at GSK Vaccines). Before that he worked in the public health sector as an epidemiologist on cancer and on infectious diseases as the head of the Provincial Institute of Hygiene in Antwerp, Belgium. He now works on the health economics evaluation of vaccines at a global level worldwide, promoting new ways to assess the total economic value of immunization programs. His main interest is to make complex programs more accessible to decision makers. Baudouin is also linked to the University of Groningen where he was recently promoted with a doctoral thesis on “New ways to explore the economic value of vaccines”.

Mondher Toumi: Mondher is a medical doctor by training, with an MSc in Biological Sciences and a PhD in Economic Sciences. He is Professor of Public Health at Aix-Marseille University. After working for 12 years as Research Manager in the laboratory of pharmacology at the University of Marseille, he joined the Public Health Department in 1993. He worked from 1995 in the pharmaceutical industry for 13 years. Mondher was appointed Global Vice President at Lundbeck A/S in charge of health economics, outcome research, pricing, market access, epidemiology, risk management, governmental affairs and competitive intelligence. In 2008, he founded Creativ-Ceutical, an international consulting firm dedicated to supporting health industries and authorities in strategic decision making. In February 2009 he was appointed Professor at Lyon I University in the Department of Decision Sciences and Health Policies. The same year, he was appointed Director of the Chair of Public Health and Market Access. He conducted the first European University Diploma of Market Access (EMAUD) in Paris, France. Additionally, he recently created the Market Access Society to promote research and scientific activities around market access, public health and health economics assessment. Since 2009, he has also chaired the Annual Market Access Day, a purely academic event sponsored by EMAUD that has become a reference event in the area. In September 2014 he joined the research unit EA3279 of the Public Health Department, at Aix-Marseille University (France) as Professor. Mondher is also visiting professor at two famous Chinese universities: Shenyang Pharmaceutical University and Beijing University (Third Hospital). In addition to contributing as a reviewer on several journals, he is Chief Editor at the online Journal of Market Access and Health Policy. He did two mandates as Co-Chair of the Research Review Committee ISPOR, in 2012 and 2013. He is a recognized expert in drug development and an authority on market access and risk management. He has more than 200 scientific publications and oral communications, and has contributed to several books. He just finished a book on market access, to be published soon.
Janine van Til: Janine is a health scientist by training. Janine started her work in preference research with her PhD project called “Integrating preferences into decision making”, which was focused on including values in decision making in the treatment of ankle-foot impairments in stroke patients. Janine is currently working as an Assistant Professor in the department of Health Technology and Services Management at the University of Twente in the Netherlands. Her research is focused on using preference methods, mainly discrete choice experiments, best-worst scaling and multi-criteria decision analysis methods to include the stakeholder perspective in health care decisions in the clinical, management and societal context. She has more than ten years’ experience in the design and analysis of stated preference surveys, mainly in the field of neurology, oncology and cardiology. Janine is the main supervisor of two PhD students in the field of patient preference research, and is involved in the training of more than five other PhD students. Over the last five years she has successfully written multiple grant proposals, project managed national and international projects, and worked as a consulting researcher on international projects. She is also the main lecturer of the course “Patient Preference Modelling” in the Master Health Sciences at the University of Twente. In her non-research time, Janine likes to read books on the psychology of decision making and fantasy novels, imagine herself doing lots of physical outside activities and taking holidays, and is the proud mother of two very sassy boys.

Mel Walker: Mel, BPharm, MRPharmS, PhD, joined Otsuka’s European executive team in 2012 with the task of building a cutting-edge patient access function and developing Otsuka’s organizational capability to meet the needs of health care systems in Europe. Mel is passionate about the role that a highly ethical and patient-focused health care company can play in providing better health care for patients. He believes that value can be delivered not only by bringing medicines of real benefit, but also by gaining a better understanding about how medicines should be used within health care systems and using this knowledge to optimize the value delivered to patients at both an individual and a population level. Mel is also passionate about helping health care systems to use data, analytics and change management expertise to improve clinical practice and believes that a holistic approach is essential for a company that wishes to become a trusted and valued health care partner. Mel previously worked for GSK, where he held a variety of senior roles, including leading a team responsible for the delivery of health outcomes plans and reimbursement strategies for the oncology portfolio. He played a crucial role in driving engagement with international value experts involved in the appraisal, reimbursement and access for new medicines as part of the Access to Medicines Leadership, and helped to build broad access capability and drive customer-centric approaches across the CE region as a member of the executive team. Mel has co-authored more than 30 research papers and abstracts, as well as two book chapters, and worked in several therapeutic areas, including oncology, haematology, osteoporosis, rheumatoid arthritis, nephrology, transplantation, obesity, HIV and Alzheimer’s disease. He regularly chairs and contributes to international conferences in the areas of market access, the HTA regulatory interface, conditional reimbursement and
customer-centric approaches. He is affiliated to the Centre for Socioeconomic Research (University of Wales), sits on the Steering Committee for the Centre for Innovation in Regulatory Science, co-founded the HTAi ISG on HTA Regulatory Interactions and is a member of TOPRA’s Advisory Council.

**Michael Wonder:** Michael gained his BSc (Hons) in Biochemistry and Pathology from the University of Melbourne and a B Pharm from the Victorian College of Pharmacy, before working as a hospital pharmacist for five years. He then moved to Novartis Pharmaceuticals Australia as its first Health Economics Manager, working on strategic development and preparation of reimbursement and/or pricing submissions, before being appointed Director of Global Pricing and Market Access Operations for Novartis Pharma AG. Since 2011 Michael has been an independent consultant on biopharmaceutical reimbursement and pricing matters. He has also acted as an expert advisor to the Masters of Science in International Health Technology Assessment, Pricing and Reimbursement at the University of Sheffield and the Distance Learning Certificate Program in Health Economics and Outcomes Research at the University of Washington.

**Julie Zard:** Being an MD, Julie holds a specialized master in “Marketing management for the pharmaceutical industry and biotechnologies” from ESCP – Europe business school in Paris, and has completed the European University Diploma of Market Access from the University of Lyon I. She worked at Takeda France in the public and economic affairs department where she worked on preparation of transparency dossiers for the Transparency Committee and she managed team work with KOLs and consultants. She has worked on several market access topics: HTA overview with an international comparison analysis, products’ value stories, core value dossier on products in infectious diseases, payer researches interviewing KOLs, research on risk and market access opportunities in cardiovascular area. She has contributed to a research publication on market access agreements and has strong medical writing skills. Julie has participated in several Global Payer Advisory Boards, producing minutes and slide decks and executive summaries summarizing the meeting outcomes and recommendations. Julie is now pursuing a career as a medical practitioner.
The discipline of health economics has progressed substantially. In the 1960s, it began with cost–benefit analyses of public health programs, such as vaccination, and has evolved into a large discipline of formal health technology assessment (HTA). Cost-effectiveness analysis has become the centrepiece of HTA and is currently performed virtually for each new health care technology. The biopharmaceutical industry and health care payers increasingly rely on cost-effectiveness to assess the value of new technologies and to define their pricing and coverage decision making.

Nonetheless, the affordability criterion of the payers’ side is increasing and fuelled by the availability of costly technologies, the growing incidence of chronic diseases and a relatively gloomy economic outlook. In this context, the pricing and market access of medical innovations has without a doubt become a hot topic in recent years. Medical innovation usually occurs in incremental steps and comes at an additional cost to health care systems. Choice and prioritization became unavoidable. The cost debate has now reached a turning point in many health care systems.

The issue of affordability and financial sustainability has triggered the need to think about new concepts going beyond the sole consideration of the “price-per-pill” and the incremental cost-effectiveness ratio. How can health care systems reward innovation and sustain research and development (R&D) investments while also balancing affordability and equitable access to innovation? What assessment criteria should be applied in deciding access and reimbursement? What are the overall value elements that a new treatment brings to patients and society as a whole? What are the perspectives in assessing the cost versus the benefit of innovation? There are many unanswered questions.

The value of treatment continues to be debated intensively at each introduction of innovative products. In recent decades, developments have been made in many areas such as orphan diseases, infectious diseases and cancers. The launch of drugs such as Imatinib in chronic myeloid leukaemia or Sofosbuvir in hepatitis C has brought major advancements for patients. At the same time, these products intensified the debate about the cost of innovative health care technologies.

These debates have not brought forward a single definition of value agreed on by all stakeholders. Therefore, the question arises as to whether all elements that determine the value of a new technology are sufficiently captured in a cost-effectiveness analysis or whether complementary assessment criteria should be considered within a multi-criteria decision analysis framework.
The identification of populations that are expected to benefit the most from a particular treatment, so-called stratified or personalized medicine, is also clearly sought by health care payers. The use of companion diagnostics carries the hope of streamlining clinical decisions and resource allocation with the identification of patient populations most likely to benefit from companion therapies. However, questions remain in the value assessment of these technologies.

Progress in information technology (IT) and the power of the internet have reached the health care industry in a way never observed before. “Big data”, “mHealth” or “real-world evidence” are the buzzwords in recent years. It is indeed critical that real-world evidence is collected, processed and analyzed to direct innovation to the populations most likely to benefit and to ensure a wise investment of resources. An increasing number of countries are developing guidance to generate evidence beyond pivotal trials. In many diseases, notably rare diseases, this approach is often the only way to amass significant data for decision making.

There is also the tendency to link real-world outcomes to reimbursement and pricing in pay-for-performance type contracts. We have all witnessed the rise of risk-sharing and other market access agreements between manufacturers and payers. In theory, these agreements can address some of the above issues. They can facilitate faster market access for new medicines while additional real-world evidence is still being generated.

Emerging markets around the world have also been learning from the instituted HTA countries. They have started to introduce guidelines on health economics evaluation and to establish pricing and reimbursement procedures based on economic criteria and relative effectiveness evaluations.

Many books have been written about the theory and methods of health economics. Our aim was not to edit another book further emphasizing the need for cost-effectiveness analysis; instead, we aimed to bring together the most advanced and perhaps unconventional thinkers in the area, from academia to industry, in an attempt to provide forward-thinking ideas on the aforementioned issues.

We have divided the book into three parts:

I. Innovation and market access
II. Technological changes and demographics
III. Decision making and assessment

The first part is devoted to discussions about market access issues of health care innovation.

- Ed Schoonveld starts by insisting on the importance of understanding and defining value in preparing for market access and pricing negotiations. The following questions, among others, are discussed: How do payers perceive value? How can health outcomes and health economics evidence play a role in demonstrating value?
- Then, Larry Gorkin and Eugenia Gruzglin notice that the rapidly improving productivity in drug development will produce a tipping point, in which the unabated approach to ever-increasing drug costs is unsustainable. They
advocate a return to value-based pricing to recalibrate the balance between pricing and value.

- Admittedly, for a new health care technology to fulfil its therapeutic and commercial potential, it is no longer a matter of generating evidence to demonstrate its quality, safety and efficacy to a standard expected by regulators. Michael Wonder and Edward Hornby thus discuss the issues associated with obtaining scientific advice directly from payers. Until relatively recently, this process was considered impossible.

- Market access agreements have been increasingly proposed and implemented during the past few years. Mondher Toumi, Julie Zard, Imene Ben Abdallah, Claudio Jommi and Joshua Cohen provide a thorough description of the emerging market access agreements between the industry and health care payers.

- Among the drugs brought to patients, orphan drugs represent particular challenges. They are produced at a low level but have relatively high prices, in combination with uncertainty over their effectiveness. Thomas Morel and Steven Simoens debate the coverage of orphan drugs, knowing that such drugs are unlikely to be cost-effective.

- Times have changed dramatically for the pharmaceutical industry during the past few years. Market access capabilities, as well as a patient-centred approach to business strategy, have become critical. In this context, Ulf Staginnus suggests how to successfully continue innovating and addressing HTA requirements.

The second part of the book examines a few of the health economics issues that are conveyed by ongoing technical changes and demographics.

- Antonio Ramirez de Arellano provides an overview of experiences in conducting a health economics evaluation of the combination of genetic testing and drugs. The chapter is illustrated with specific examples in which the application of pharmacogenomic testing may be regarded as promising in terms of efficiency.

- Olivier Ethgen, Justine Slomian and Mel Walker introduce a series of health information technology concepts and offer a discussion of the promises of these emerging technologies for health economics in the future.

- Stephen Birch and Amiram Gafni discuss how cost control (and hence the sustainability of healthcare systems) depends on the methods used for planning and evaluating healthcare resource use. They challenge the notion that equitable access to healthcare is unsustainable and show that the continuous increases in healthcare costs are the result of inappropriate assumptions underlying the methods for planning and evaluating healthcare resources. They offer alternative approaches that relate directly to the needs of populations.

- Mark Connolly argues that the typical economic framework for evaluating health conditions ignores the fiscal externalities of health or changes in health status. He then introduces the intergeneration economic framework and shows how the government can expect a return on investment from health.
Introduction

• To conclude this section, Ning Lu and Eun-Young Bae select two representative Asian countries, China and South Korea, to debate the adoption of health economics. They question whether the need to manage rising health care costs has reached a critical mass and discuss the level of resource infrastructure/capacity development needed for health economics adoption.

The third part of the book comprises a few reflections on how to complement cost-effectiveness to more comprehensively support decision making.

• Janine van Til and John Bridges bring forward the patient perspective. They introduce preference-based methods that enable valuation of healthcare innovation within the context of HTA, assuming that the patient is the best judge of its value trade-offs.
• Olivier Ethgen and Kevin Marsh present the multi-criteria decision analysis framework as an aid to decision making in health care. They describe the necessary steps to implement a multi-criteria decision analysis and provide a few applications to support health care decision making as well as the lessons and challenges identified through these experiences.
• Real-world evidence is increasingly required to defend reimbursement or to keep a product on the market. It is also becoming a critical piece of evidence to anticipate the real-world effectiveness drivers, to predict the potential public health impact and to identify a target population. Billy Amzal, Roman Casciano, Lamiae Grimaldi-Bensouda and Lucien Abenhaim describe the concepts and the practice of relative effectiveness studies.
• Nadia Demarteau, Baudouin Standaert and Stephanie Earnshaw ascertain the limit of cost-effectiveness and introduce the mathematical programming approach to optimize the allocation of health care interventions. The contributors present two applications to cervical cancer prevention and type 2 diabetes.
• Finally, E.I. Hervé Akpo, Cédric Popa, Dogan Fidan and Ömer Saka describe how commercial, research and development (R&D) and health economics models can be interconnected within a framework of product portfolio management under budgetary limitations. They then argue that the use of capital investment appraisal techniques in addition to health economics methods would enhance the efficiency of an investment portfolio decision-making process.

We hope this book contributes new thoughts and perspectives that will advance the field of health economics. The relatively exclusive and narrow microeconomic application of cost-effectiveness analysis that we have observed so far might well be insufficient based on the challenges ahead. New approaches, or at least more comprehensive and complementary approaches, are needed to value innovative technologies. Hopefully, the debates in this book will inspire new ideas and practices among industry practitioners, policy makers, researchers and students.

Olivier Ethgen and Ulf Staginnus
Part I

Innovation and Market Access
Chapter 1

The Role of Value in Market Access and Pricing Negotiations

Ed Schoonveld

Introduction

Health economists sometimes seem to be their own worst enemy as they tend to insist on presenting evidence of what they think payers should be interested in, rather than focusing on what payers really act on.

How do payers perceive value? How can health outcomes and health economics evidence play a role in demonstrating value? What information can have an impact on market access and pricing decisions? In order to understand this, we must examine the decision-making processes that underlie drug prescribing and funding within each country’s health care system. Some global payer systems use cost-effectiveness methodologies for funding decisions, but most don’t. Many payers use “benefits”-based drug evaluations that are less directly tied to a cost impact benchmark. Health outcomes data can still have a substantial impact as a means of demonstrating value. It does mean, however, that we need to thoroughly understand the particular way in which we can guide decision making in each specific system. Before we look at the topic in more depth, let us first consider why governments feel the need to interfere in drug pricing and what their mindsets are in doing so.

The drug industry is very different from other industries due to cost structure, intellectual property-related aspects and ethical “right to health care” philosophies. A particularly important aspect is that the drug purchasing decision is not a simple buyer versus seller situation but a more complicated interchange between payer, physician and patient. Whether and how various stakeholders interact and influence drug use is very dependent on the payer system. Understanding this process and its implications goes a long way in the identification of critical success factors for market access and pricing of prescription drugs.

The Drug Purchasing Process

When you go to a grocery store to buy fruit, you may rest your eyes on a ripe mango and ask the grocer for the price. If acceptable, you may decide to buy the mango. If too expensive, you may decide to buy cheaper oranges instead. Now suppose you tell the grocer that you are still not convinced that the mangos are tastier than the oranges and insist that he lowers the price of the mangos. The angry response from the grocer may be inappropriate for printing, but will give reference to the fact that if I don’t like the price of the mangos, I should not buy them. Reading this example
you may find the suggestion ridiculous; however, this is exactly what is happening for prescription drugs in many countries. Governments in many cases demand that a drug price be lowered rather than deciding not to buy or reimburse it. Why do governments think that it is necessary to insist on a lower price for a product for which they don’t appreciate the value that is claimed by the seller?

The situation in the prescription drug purchasing process was once described to me as “Dinner for Three” (see Figure 1.1). Imagine three people, Bob, Ben and Betty, go to a restaurant, where Bob orders a meal, Betty eats and Ben pays the bill. You can imagine the conversation where payer Ben suggests that lobster was perhaps a bit excessive. He would be concerned that absent of an obligation to pay, diner Betty may not be sufficiently cost sensitive and might waste his money. The thought of buying a hot dog on the street corner may very well appeal to Ben. He may even claim that hot dogs are not proven to be less healthy. This example may seem far-fetched, but it illustrates the impact of a situation where a natural balance in decision making is distorted. Many governments have felt compelled to intervene in the pricing and reimbursement process for prescription drugs, particularly as drug cost impacts their budget directly. Unfortunately, as many of these controls are ineffective, they tend to lead to new controls, thus moving further and further away from a balanced system, rather than restoring a form of controlled market. A detailed discussion of these and other factors in the economics of the pharmaceutical industry and global drug pricing can be found in Schoonveld (2015).

**Understanding Payers**

Government and private payers, whether central, regional or local, often play a key role in the availability of prescription drugs for physician prescribing. Payers and their systems vary significantly globally. When we try to convince payers of the need to allow broad usage of our product at a reasonable price, we need to try to understand how a payer evaluates our drug and its value proposition within the context of the system, its cost-management practices, and the decision makers’ individual priorities.
How can the drug help a payer reach his or her objectives, and what evidence would he or she like to see to buy into any benefit claims that a manufacturer may put forward? As all payers are not the same, we need to consider how different payers might have different reactions to our value proposition. Hospital payers in Germany might have a different view on your drug’s value than a US managed care pharmacy director or the national Pharmaceutical Benefits Advisory Committee (PBAC) pricing authorities in Australia. The underlying reason for a different perspective on our value proposition can have various causes:

1. National vs. regional or local hospital scope of budget responsibilities
2. Differences in responsibilities with respect to the elements of health care under their remit, such as total medical cost (UK National Health Service or German sick fund) vs. pharmacy cost (for example US Medicare Part D or provincial drug budget holder in Canada)
3. Political and cultural differences between countries, such as a strong “market” philosophy in the US vs. a social equity-driven mindset in Europe
4. Legal differences, for example an obligation for health insurance companies to cover anti-cancer drugs in many US states
5. Differences in the decision-making process and the underlying cost-management principles that payers use in pricing and market access decisions
6. Specific local preferences and priorities, for example due to a particularly high or low incidence of a condition or cost of an intervention in comparison with other locations

The many differences in situations and perspectives between countries has caused every payer system to be unique, which makes the resulting global drug market access and pricing environment very complex. However, upon closer examination one can see that payers use a limited number of underlying cost-management principles to address their drive to ration healthcare utilization within their budget, as a controlling mechanism of an imperfect market for prescription drugs and to enforce their interests as a buyer.

Global Payer Archetypes and Segments

Payers around the world have found different ways to address their concerns with respect to pricing and/or reimbursement of prescription drugs, as described in the previous section. In the United States, private payers have instituted formularies, prior authorizations and step edits, together with co-pay and co-insurance rates, to incentivize patients to use generics and preferred brands. In France, the government has instituted a structured evaluation process of the therapeutic benefit of new drugs over existing “comparators” and is controlling price and use of the drugs through price controls and volume agreements. In the United Kingdom, the National Institute for Health and Care Excellence (NICE) reviews the cost-effectiveness of drugs and some other medical treatments, and then provides guidance to the local budget holders with respect to whether they should make the drug available for their patients. In China, the government has a limited number of drugs (usually low-cost options) on national and provincial formularies, whereas many high-cost branded drugs are available to
patients on a cash-pay basis only, leaving the funding decision for higher-cost drugs to the individual patient and his/her advising physician. The examples mentioned constitute substantially different systems of market access and pricing control. In examining other countries, we find other variations and methods of control as every country has individual decision-making power. Even within the European Union, although jointly reviewing and approving market authorization through the European Medicines Agency (EMA), individual countries control drug pricing and reimbursement with various control mechanisms. The EMA has no role in these decisions.

What are the commonalities among all the different pricing and reimbursement systems across countries? Despite all being different, there are some fundamental cost-management principles that can serve as descriptive archetypes and which can form a basis for a global segmentation of payer systems. Figure 1.2 shows an approach towards segmenting global payer systems that has proven to be particularly helpful in evaluating payer system reactions to typical outpatient prescription drugs by defining some archetypes. It is not suggested that every country perfectly matches one archetype. As a matter of fact, countries such as Canada exhibit characteristics of more than one archetype. For the purposes of this analysis, countries have been classified by what is generally considered their most restrictive characteristic. Here are the four archetypes, in order of increasing restrictiveness:

1. Emerging cash market
2. Competitive insurance-based market
3. Therapeutic referencing market
4. Health economics-driven market

Now let’s have a closer look at each of the archetypes and consider how the global prescription drug market can be segmented on the basis of the predominantly exhibited archetype in each country.

Figure 1.2 Global payer segments
Emerging Cash Markets

The emerging cash market is, as the title suggests, representing a large majority of emerging markets, where for many branded drugs there tends to be limited or no health care system coverage. Countries such as China and India fall in this category as the great majority of patients are paying for branded prescription drugs out of pocket without any reimbursement. Since these are prescription drugs, the physician has a strong role in drug choice, but patient affordability and willingness to pay are likely to feature significantly in the physician’s decision making. Providing universal health care coverage is on the political wish list of many emerging markets. However, most existing coverage programs do not have sufficient funding to go beyond some form of “essential” drug coverage, usually including generically available drugs only. Selected populations may enjoy broader coverage through employers, but this is typically only a fraction of the population, for example about 5% in India. It is important to have a realistic view on how drug coverage may evolve in the future and to examine this periodically. It is also important to realize that a future introduction or broadening of universal health coverage is likely to lead to more cost controls, whereby a country can look more like one of the other archetypes.

Competitive Insurance-Based Markets

Competitive insurance-based markets include most of the US market, where health insurance companies are competing to cover patient healthcare needs for a monthly insurance premium. Insurance companies decide on their drug formulary and related co-pays/co-insurance rates, prior authorizations and step edit restrictions on the basis of clinical value and terms offered by competing drug companies. Over the last 10 years, the US market has gradually undergone significant change, as the system has been and continues to be under reform. Insurance plans, in an effort to control their rapidly rising premiums, have continually increased patient co-pay and co-insurance burdens to limit cost and utilization of high-cost biologics and branded drugs with generic “equivalents”. Drug companies have utilized various co-pay offset programs to try to mitigate the negative impact of an unfavorable formulary tier placement and associated patient objections. In Medicare Part D, which is managed by private health plans, coupons cannot be used, which is one of the reasons why contracting in this segment has become more aggressive. Since Medicare Part D is a drugs-only coverage plan, insurance companies are incentivized to focus on drug cost only. These patients have their non-drug medical coverage under the traditional Medicare Parts A and B mechanisms. Medicare Part C or Medicare Advantage Plans have drug and other medical care integrated.

The Patient Protection and Affordable Care Act (ACA) or “ObamaCare” has introduced additional insurance plans, targeted at the uninsured population that does not have access to employer-sponsored plans and cannot afford other private options. Federal subsidies have resulted in low monthly premiums for lower-income populations, but the plans, which are offered through a “marketplace” or “exchange”, have relatively high deductibles and co-payments (often 50%). The introduction of these plans has further fragmented the US market.
The state-managed Medicaid programs have been much more restrictive in allowing branded drug options on a favorable formulary position. Many state Medicaid programs behave more like a therapeutic referencing market, which is described below.

Over the past few years, Brazil has seen substantial growth in its supplementary private insurance market. United Health Group acquired a 90% stake in Amil, the largest Brazilian private health insurance company. Today, close to 50% of drugs are funded through these private plans, which show similarities to the US managed care model.

**Therapeutic Referencing Markets**

Therapeutic referencing markets make their pricing or coverage decisions on the basis of the selection of a “comparator” and a rating of importance of innovativeness or clinical benefits proven over that comparator. Although implemented in slightly different ways, this methodology is used extensively in ex-US countries such as Japan, France, Germany, Italy and Spain. Most payers in these systems use strict criteria for granting favorable benefits or innovativeness ratings, as they are also frequently referred to. Head-to-head superiority data with statistically and clinically significant differences on meaningful outcomes are usually demanded. This is particularly critical when the selected comparator is an inexpensive drug, for example a generic. Many countries in this segment directly control drug price through this methodology. Germany has moved from reimbursement control to price control through introduction of its AMNOG legislation in early 2011. In some countries there is a theoretical possibility to opt out of reimbursement at a higher price; however, in reality this is often not a feasible commercial route.

**Health Economics-Driven Markets**

Health economics-driven markets are using cost-effectiveness as the primary metric for pricing or reimbursement decision making. Markets such as the United Kingdom, Australia, Canada, Sweden, South Korea and the public system in Brazil are typical examples of this approach. Payers in these systems tend to use a cost per QALY (quality-adjusted life years) approach. Some markets, such as the Netherlands, and since 2013 France, use cost-effectiveness data in addition to therapeutic referencing, but tend to use less strictly defined cost-effectiveness criteria.

**Payer Perspective on Health Outcomes, Health Economics, Value and Price**

Health economics theory provides guidance on how, in a situation with resource constraints, a rational trade-off can be made between value or utility of a drug or treatment and its associated impact on cost. That allows, at least theoretically, for making rational choices between health care spending options. Health economists usually measure the value of changes in patient condition through quality of life (QOL) scales, which allow for weighing the value of life years gained for different situations, such as curing a cancer patient vs. saving the life of a stroke victim. How functional the stroke victim is, for example – fully functional or partly or severely
impaired—will have a significant impact on the QOL metric. Academically it makes perfect sense, but not every society and payer within that society is comfortable with handling some of the ethically complex trade-offs that go with such a mechanic decision-making model. In the UK, a lot of debate has emerged over the introduction of value-based assessments, introducing “societal value” as a factor to accept higher or lower cost-effectiveness standards. Not many people will object to spending extra money on saving an infant’s life, but translating this to more restrictive coverage for the elderly makes the debate more difficult and may draw comparisons with the US “death panel” fears at the introduction of ObamaCare. These elements are probably the most important reasons why cost-effectiveness is serving as a strict basis for pricing and market access decision making in only about 10% of the global pharmaceutical market (see Figure 1.3).

A particularly difficult challenge is how the relatively academic principles of health economics can be applied in an ethically acceptable way, also considering that at time of launch of a new drug, only data is available on its performance in a controlled clinical setting as defined by Food and Drug Administration (FDA) and EMA type trial requirements. Demanding data on the real-life performance of a drug, before it can be broadly accepted as a viable alternative, can choke off promising new treatment options, much like it is difficult to teach a child to ride a bicycle without at least some risk of falling. Particularly in a field such as oncology, it can be unethical to test a new drug in earlier treatment stages before showing its potential in late-stage disease, where it is much less likely to be cost-effective than in a curative setting. Many drugs that have been initially used for palliative treatment for end-stage cancer patients have over time proven their utility in extending lives in earlier lines of therapy.

In order to truly understand how health outcomes and health economics support pricing and market access decision making, we must first take a closer look at each of these terms and what they entail. We can then evaluate how they can be relevant in each of the global payer archetypes and segments that we identified in the previous section.

![Figure 1.3 Global payer segments by sales and population](image-url)
**Health Outcomes**

Utilizing health outcomes to more effectively illustrate the impact of a treatment on a patient’s well-being can be beneficial in any payer system. The general debilitating nature of an untreated or insufficiently treated condition, or the toxicity and side effects of older treatment options, are frequently underestimated. One reason is that physicians tend to not embellish on issues with current treatments unless there are better options. After all, why raise an issue and upset a patient when there is no alternative solution available?

Showing improved long-term outcomes, such as a reduction in cardiovascular events or improved overall survival, is particularly powerful in payer discussions. In general, long-term outcomes are much more important than shorter-term surrogate endpoints in the impact on overall patient health and associated long-term medical cost. For a lipid-lowering drug, reductions in cardiovascular events will be strongly valued over surrogate low-density lipoprotein (LDL) reduction claims. Similarly, in oncology, overall survival claims are more powerful than tumor response rates. Surrogate endpoints can play an important role in patient management, but are much less meaningful as a measure of value for a drug or treatment unless the relationship with longer-term outcomes can be demonstrated or seems reasonably certain. Payers often simply reject surrogate endpoints on that basis.

Softer health outcomes endpoints, such as non-specific QOL improvement claims, better compliance and fewer side effects, are frequently met with skepticism by payers, unless the claims are clearly linked to long-term outcomes and/or cost savings, and are supported with strong data. In that sense, the term QOL is not of interest to many payers, as it lacks specificity. It is neither emotionally nor economically compelling as it is a very generic term.

**Health Economics**

Health economics information that is often considered by private and government payers is cost-effectiveness, usually as a cost per QALY, and/or budget impact. Cost-effectiveness data is required by a minority of healthcare systems. The British National Health Service (NHS) bases many reimbursement decisions on an evaluation by NICE. Within NICE guidelines, drugs are typically not recommended for formulary inclusion unless they are both effective and cost-effective, as defined by NICE standards. Australia, Canadian provinces, Sweden, South Korea and a number of smaller countries use health economics in a similar way in pricing or reimbursement decision making. Some countries mention cost-effectiveness as a reimbursement criterion, but in many cases this is not actually used in their decision making.

Most countries will consider the impact of a drug or treatment on medical and/or drug budget, even if they do not evaluate cost-effectiveness. New drugs that are selectively used for appropriate patients and don’t “break the bank” tend to be reviewed in a much more lenient fashion than new potential blockbuster drugs. For these drugs, the discussion frequently focuses on the identification of “appropriate patients” for the new treatment. By selecting only a fraction of the population for which a drug is approved in the FDA or European Medicines Agency (EMA) label, a payer can allow a new treatment, while
securing the use of less expensive, perhaps generically available, treatment options first. In reality, this can work well when a payer has the ability to control utilization, for example through the use of step edits, as is done frequently in the United States. In countries where payers have little control over utilization within a relatively broad label, this can become a serious negotiation hurdle.

The drug industry generally experiences health economic requirements as a significant hurdle to patient access. Particularly since it is often practically impossible to have a strong health economic dossier at product launch, health economic requirements at launch indeed have the practical impact of an additional hurdle, in terms of both time and resources required to satisfy rigidly formulated health economic requirements. Moreover, payers tend to mistrust or even reject any health economic data gathered by drug manufacturers.

**Actual Use of Health Outcomes and Economics in Payer Decision Making**

**Health Economics-Driven Markets**

As suggested by the segment name, health economics-driven markets place the utmost significance on health economic data. In particular cost-effectiveness data, in the form of an incremental cost-effectiveness ratio, is central to coverage decision making. In the UK, the cost per QALY is fairly directly related to the coverage advice of NICE. However, it should be realized that NICE is merely advising on coverage; the actual coverage decisions are made by clinical commissioning groups (CCGs), the local budget holders. CCGs often argue that their financing is inadequate to fund for all NICE-endorsed prescription drugs.

Figure 1.4 illustrates a typical view of cost vs. added outcomes benefit and the related cost-effectiveness plane as used in these markets. It refers to the UK, but will be similar for other health economics-driven markets.

In Canada, the Common Drug Review (CDR) is a joint review from most provinces (excluding Quebec) to evaluate effectiveness and cost-effectiveness of new drugs. A similar review exists for oncology drugs. Provinces tend to use a positive CDR review as a basis for formulary decision making; however, provinces often negotiate with drug companies for confidential provincial agreements after a CDR review is negative. By the CDR’s own statistics, roughly half the evaluations end up in a positive advise; however, when considering the large number of partial or conditional coverage approvals, the scorecard looks much more negative. This is not surprising, since Canada has a history of compulsory licensing and subsequent price controls, resulting in average drug price levels that are below those of the United States. Drug companies have been hesitant to allow Canadian prices at much below US levels and have sometimes opted to forego the Canadian market rather than face intense scrutiny over pricing differentials. Provincial listing agreements and more recent pan-Canadian price negotiations have provided opportunities to engage in confidential deals to circumvent the issue.

The Australian PBAC is using cost-effectiveness for national coverage decision making under its Pharmaceutical Benefits Scheme (PBS) drug-listing system. Drug companies need to qualify for formal consideration of cost-effectiveness in the evaluation of the
reimbursed price. When benefits of a new drug are deemed insignificant, cost-minimization vs. a reference treatment is used as a basis for reimbursement price determination.

Sweden is using cost-effectiveness in a similar way to support coverage decision making by its regional councils, but is taking a broader societal view on cost, allowing for patient economics to be included rather than only department of health expenses. South Korea has adopted cost-effectiveness as a means of controlling drug pricing. It took the South Korean government a long time before its Health Insurance Review and Assessment Service (HIRA) agency developed sufficient expertise to effectively apply the technology. Today, it has been fully adopted; however, it now serves merely as a starting point for price negotiations as the government attempts to further curtail drug spending.

**Therapeutic Referencing Markets**

Most therapeutic referencing markets consider health economics data only to inform budget impact of a drug coverage-related decision. Typically, pricing and reimbursement authorities state that the company can submit cost-effectiveness evaluations, but many do not have the internal capability to review the data, and their process often does not offer an opportunity for cost-effectiveness data to impact the pricing or reimbursement decision. Although there is no fixed formula that is valid for all countries, payers in these systems tend to look at budget impact, particularly when this is expected to be high and where there are opportunities to limit use of a certain drug for more “appropriate” populations. Providing information on effectiveness of a drug in sub-populations can strengthen the value proposition for that population,
but can also lead to a restriction of use for that specific population. Treatment cost offsets are important to demonstrate, but non-drug medical cost offsets will be convincing only for payers with a broader medical perspective.

The main impact of health outcomes and health economics information on decision making in therapeutic referencing markets is through the assessment of benefits and related “innovativeness” or “value added” rating. This is where patient outcomes data rather than economic data can be leveraged to support value claims. However, it is important that the outcomes data are relevant to the payer and demonstrate, for example, meaningful impact on mortality, avoidance of severe cardiovascular events, reduction in hospitalization days or other clinically and/or economically relevant improvements. Quality of life as a term tends to not impress most payers in the therapeutic referencing market space. The term is too generic for them and without more meaningful specificity it is generally considered irrelevant for coverage decision making.

Figure 1.5 shows a therapeutic referencing market version of the cost-effectiveness plane, discussed earlier in this chapter. As indicated, value is expressed in some form of benefits assessment or innovativeness rating. In this example, the French Amélioration du Service Médical Rendu (ASMR) rating is used, which ranges from I (major benefit) to VI (no benefit, i.e. not reimbursed). The actual reimbursement price premium over the comparator that will be granted or allowed under national law cannot be calculated through a pre-determined formula. However, a small premium is feasible under ASMR IV, whereas a discount is to be expected under ASMR V.
Price premiums for ASMR ratings I–III are much more favorable but more complicated to determine and are influenced by prices in other EU markets. In other therapeutic referencing markets, a similar system of benefits rating exists. In Japan, the relationship is much more defined than in Germany, Italy and Spain. It may be clear from the above that the approach in therapeutic referencing markets is very structured, but the impact on reimbursable price is less mechanistic than the cost-effectiveness assessment under NICE and similar health economics-driven systems. In some markets, such as France since October 2013, cost-effectiveness considerations can play a role in decision making next to the benefits analysis; however, cut-off points in terms of a maximum cost/QALY are usually not defined in these markets.

The use of health economics and health outcomes has some history in the largely private US market. Contrary to the United Kingdom and Canada, the use of strict cost-effectiveness rules and linked-coverage decision making has been met with a lot of resistance among US decision makers and the public, as it was generally felt to be objectionable to determine access to health care through cost cut-offs that are related to an assessment of the value of life. However in, a slightly different way, various health economics and health outcomes aspects influence decision making.

Private payers, such as United Healthcare, Humana or Aetna, review full clinical packages as a primary guide towards an initial decision as to whether, medically speaking, a new treatment should be included in the benefits package. Effectiveness and safety are the primary drivers of this decision. Provided that the medical review has yielded a positive answer, formulary decisions are made on the basis of cost-related issues and concerns. Should utilization be encouraged through a lower patient co-pay? Should prior authorizations and step edits be instituted to avoid overly broad and what the payer would consider “inappropriate” use? Are the restrictions medically justifiable? Do the costs of these programs justify the means? Is managing the drug category important in the context of the per-patient-per-month (pppm) cost to the payer?

The perspective and time horizon of the payer are important considerations in the US. First, some payers are responsible for drug budget only. For example, a private plan, managing Medicare Part D drugs, will not be particularly motivated to accept increased drug expenditures in order to achieve cost offsets through reduced hospitalizations or other medical interventions. These payers are unlikely to bluntly state that they don’t care about medical consequences of sub-optimal treatment. However, since their incentives are not aligned with it, we need to ensure broad awareness with employers and the public to overcome payer resistance and “skepticism” of claims and data presented. Second, US payers tend to be fairly short-term oriented. Cost offsets after three or five years are likely to be beyond the time horizon of consideration in most cases.

For most US payer decisions, budget impact considerations can play a substantial role, but cost-effectiveness does not. They have been more sensitive to direct savings incentives, such as offered through shared savings for Accountable Care Organizations. Payers may also choose to take advantage of the gradually starting incorporation of cost in treatment guidelines, issued by medical associations, to support rejection of expensive treatment options. However, lacking a direct impact on their financials, the role of cost-effectiveness in future decision making seems highly uncertain for most health care plans. Few plans, for example Blue Cross Blue Shield, have instituted such reviews for drugs and other treatments.
Emerging Cash Markets

As described earlier in this chapter, funding decisions in emerging cash markets are subject to dialog between healthcare provider, typically the treating physician and the patient, who will have to pay for the treatment. National health departments tend to have limited interference with drug pricing, as they do not feel a budgetary impact. In some countries, the government does impose pricing restrictions, often based on the prices in a basket of international markets.

Cost-effectiveness evaluations have little merit in communications with physicians and patients, although on a more generic level physicians may claim to look at cost-effectiveness of a treatment. In reality this is seldom the case, or only in very general terms as “cost conscious” rather than driven by detailed cost-effectiveness models. The type of models that are prepared for NICE and SMC in the UK, CDR in Canada and PBAC in Australia have little merit in this setting. By the same token, budget impact assessments are meaningless unless in an exceptional case one would want to educate a patient on long-term personal cost savings for a drug treatment.

Conclusion

The application of health outcomes and health economics is very different from country to country, as healthcare systems have varying methods of evaluating the value and need for reimbursement coverage of a prescription drug. Cost-effectiveness has only limited application, primarily in health economics-driven systems. However, health outcomes, as a means of demonstrating tangible value, and budget impact analyses have a much broader application globally. To be successful in influencing payers and their decisions, health economists need to always carefully consider the utility of their analyses in the context of the payer system rules and demonstrated past behaviors. Understanding payer systems, rules and the way in which actual decisions are made is the key step in ensuring that health outcomes and economics are adding value in the process. Those who don’t understand them are destined to live their lives in oblivion.

Note

1 More detailed information on global drug pricing and market access issues and the preferences and needs of payers can be found in Schoonveld (2015).

Reference