Preface

Many people have asked me to recommend a book about global drug pricing. I never had an answer. Others have suggested that I write that book. Well, here it is …

It is puzzling that there are hardly, if any, books written about global drug pricing. The topic is certainly garnering interest and emotion from politicians, healthcare professionals, drug industry professionals and the public. Also, inside pharmaceutical companies there is a great need for a better understanding of the topic. As a client recently noted:

> many drug marketing people are not proficient in pharmaceutical access and pricing, yet, it is probably the most critical success factor in today's market.

Global drug pricing is a very complex topic, partly because companies rely on a period of market exclusivity through patents. It is also different from most other industries since government payers have a lot of buying power. In economic terms it could be typified as "monopoly vs. monopsony" that causes a unique and interesting dynamic, particularly when considering the situation on a larger global scale. Payers and politicians sometimes complain about lack of competition for a new drug category. Drug manufacturers complain about government controls in drug pricing. In any case, in the pharmaceutical market dynamic, general pricing principles do not directly apply without significant customization. Therefore, general pricing textbooks are essentially useless for application in pharmaceutical pricing cases.

In contrast to drug pricing, many books have been written about health economics, a discipline that provides a systematic methodology to make health resource decisions under budget constraints. Health economics is used, for example in the U.K., to decide whether a new anti-cancer drug should

be recommended for inclusion in their drug formulary or whether liver transplants should be reimbursed for every eligible patient. In most countries, however, payers and politicians are struggling to strictly base drug coverage decisions on a calculation that is only understood in detail by academics and that does not connect well with their organizational success metrics. Only a few payers strictly use health economics principles in pricing and access decision making. Global drug pricing includes health economics and health outcomes considerations but is in reality even broader and more complicated.

As a leader in global pricing and health economics and outcomes research disciplines in three large global drug companies and serving as a consultant to many others, I have had the privilege of observing the evolving roles of pricing and health economics over the years. It is particularly interesting that the two disciplines that focus on payer decision-making are often reporting into different corporate branches, that is commercial and research and development. It makes successful collaboration of the two areas heavily dependent on having similar viewpoints among the leaders of both fields, in a setting where even a joint textbook on best practices did not exist until this present work.

Healthcare is a matter that is and should be near and dear to all of us. In times of a health scare, for example caused by Ebola or Anthrax, we call for miracle solutions to protect ourselves from harm. Whether during recent debates on U.S. drug pricing or previous ones on pricing of HIV/AIDS drugs or patient co-payments in a European country, the public seems to be very engaged, yet very poorly informed about the topic. Politicians who go to bat for drug pricing issues are often equally poorly informed and are driven by short-term political motives rather than a long-term societal perspective. Welcome to the age of the sound bite.

The drug industry is often mentioned for its lobbying muscle. However, with all its capabilities, it has certainly not managed to gain the heart of the public. For an industry that is saving lives and improving patient wellbeing, to be outperformed in gaining public sympathy by the gun and tobacco industries is remarkable. Yes, there are several factors that make the pharmaceutical company story complicated. These are outlined in this book. The full story is worth telling, as the interested public deserves more than sound bites.

High prices for new biotechnology drugs can create a lot of issues for individual patients, as they may not be able to afford the cost or co-payment for the treatment. There are patient assistance programs in place to offset some

of that pain, but these cannot eliminate the issue entirely, particularly not in emerging countries, such as China. However, the ability to charge these prices within reason, is essential to ensure that we are able to address future healthcare challenges, such as the next Ebola or coronavirus epidemic, emerging resistant MRSA infected patients, multi-drug resistant HIV/AIDS patients, to continue to improve treatment and compliance of common conditions such as diabetes, and to find new treatment approaches for rare diseases that currently don't have drug solutions.

Scientific innovations in areas such as gene therapy and immuno-oncology, together with our poor dietary and sedentary lifestyle are likely to further increase the cost of healthcare. Healthcare reform in any country is unlikely to address that effectively without increases in funding or significant reductions in healthcare coverage. Many governments are choosing price control mechanisms to address their funding issues. History has shown that failing controls lead to more controls, resulting in a patchwork of government bureaucracy, which is creating more problems rather than restoring a market mechanism. I have frequently challenged and will continue to challenge government payers to explore ways of restoring market mechanisms rather than putting another layer on top of all existing controls. It is in the interest of all of us, to find acceptable solutions to healthcare funding, while securing sufficient innovative research efforts to be ready to battle the next healthcare challenge in addition to all the existing ones.

Hopefully this book will contribute in the ability to educate professionals, students, policy makers, politicians and the broader public about global drug pricing, its challenges and potential solutions. If anything, it would be great to at least achieve a common understanding on the issues. For the pharmaceutical industry, this book will hopefully form a basis for a more structured approach to addressing access and pricing challenges and to build a solid working relationship between the pricing and access function and other commercial and scientific functions. A good understanding of this field is critical for the survival of any pharmaceutical company.

Third edition

Given the fundamental changes in the prescription drug market I felt that it was time for an overhaul of the structure of the book to reflect the change in decision making dynamics. Today's "Age of Value and Affordability" is characterized

by a significantly increased involvement in drug pricing and access decisions of medical community, provider organizations, media, patient organizations and the broader public. As a result, any successful commercial campaign needs to consider how these stakeholders participate in what we introduce as the "Access Journey."

The new third edition has ten new chapters, including six of the first ten chapters, introducing the Access Journey as an organizing framework and highlighting "fair pricing" considerations, which dominate pharmaceutical pricing discussions in higher income countries as well as in lower- and middle-income countries. All chapters have been substantially restructured or updated to reflect recent environmental changes and with current examples where possible. All country chapters have been updated and expanded to add more detail. Detailed summaries have been added to all chapters in Parts A–F.

COVID-19

While finalizing the content of this third edition in early 2020, the COVID-19 pandemic changed our lives with lockdowns and virtual meetings. It caused healthcare system challenges, high mortality, unemployment and disrupted financial markets. The COVID-19 pandemic further accelerated the need for pharma to reorganize itself to demonstrate value and address affordability challenges from governments and patients. However, it also created significant opportunities to fix its severely damaged public reputation.

- Government budgets face more pressure than before. Tighter drug budgets will force payers to force lower prices and stricter access requirements. It will elevate the urgency of addressing evolving value and evidence requirements by all access journey stakeholders as outlined in Chapters 2 and 3. The need for changes in development decision making (Chapter 17) and organizational structure (Chapter 25) is even more imminent.

- Patient affordability may become an even larger challenge as average incomes stagnate globally under a recession. More patients will struggle with drug cost or higher copays and deductibles when they lose employer sponsored health insurance. With the concern over patient affordability already being high in both the United States and lower- and middle-income countries, addressing "Fair Pricing" related issues (Chapter 4) is crucial.

- Disruptions in clinical trial data gathering will cause some delay and may result in some data gaps over the COVID-19 pandemic period. Working with access decision makers to address these gaps and potentially bridge them with real world data and innovative agreements will be important (see Chapter 27).

- Urgency of finding new treatments and vaccines for emerging health challenges creates unique opportunities for pharma to forge new partnerships and change negative public perceptions of the industry (Chapters 4 and 28).

Pharma has a unique opportunity to improve its public perception and demonstrate value by making some fundamental changes that are highlighted in this third edition.

Introduction

The pharmaceutical industry is under unprecedented pressure due to a combination of declining R&D productivity, payer/provider demands for better value and public pressures to show pricing restraint.

The rapidly increasing cost of healthcare, shifts from fee-for-service to value-based reimbursement, public pressure on drug pricing and an increasingly vocal medical community have empowered public and private payers worldwide to be more demanding on evidence of value for the prescription drugs that are brought to market.

Pharmaceutical companies have often failed to deliver evidence of patient value, as development decision-making is overly focused on *speed to FDA approval* rather than *speed to commercial success* by effectively addressing the many "Access Journey" obstacles that typify today's much changed pharmaceutical environment. A confirmation of a need for change is found in an annual analysis of R&D productivity of the pharmaceutical industry (Deloitte, 2018), which indicates a continuous decline in return on investment from 10.1% in 2010 to only 1.9% in 2018. While celebrating an all-time high of FDA approvals in 2018, we need to consider whether this metric of success is meaningful to patients or shareholders if these products fail to deliver value.

Today, even in the U.S., which has historically been more sympathetic towards the pharmaceutical industry, heavily polarized republican and democratic parties seem to have very similar frustrations over drug pricing and feel that they need to take tough action to satisfy their constituents.

It's ironic that we don't seem to be able to reach political consensus on how to end gun violence as evidenced by the 26 mass shootings that took place between Memorial Day and Labor Day in 2019. However, as a country we are united in our anger towards an industry that is inventing numerous lifesaving products. It begs the questions:

How did the drug industry, with its lifesaving innovations, manage to earn a public image that is much worse than industries with products that kill, such as the gun and tobacco industries?

What does it take to fix it?

The goal of this book is three-fold:

1. To give some answers to many questions related to prescription drug development, marketing and pricing. A deeper understanding of the industry's decision-making processes and challenges can hopefully form a modest contribution to a better understanding and hopefully meaningful dialog to further a common goal: bringing much needed new treatments to patients worldwide.

2. To challenge the prescription pharmaceutical industry to adjust to the needs and requirements of a rapidly evolving market. Patients, medical community, payers, and the broader public demand products with more clearly demonstrated value, as well as an open mind to partner in addressing affordability challenges.

3. Provide professional guidance for pharmaceutical industry professionals that will enable them to work with other disciplines to create winning value strategies and develop breakthrough therapies that payers will cover, patients can afford and allows for a reasonable return on investment.

The innovative biopharmaceutical industry is uniquely different from any other industry through a combination of factors:

- High research and development investments are required for every product, thus requiring strong patent protection and a substantial margin over manufacturing cost to sustain a business.

- A unique purchasing dynamic between decision maker (physician), payer (public or private health insurance company) and product user (patient), which is different from a one-on-one buyer seller relationship that is typical for most products.

- Ethical aspects of commercializing products that are deemed essential for people's health, particularly where a market price may not be "affordable" for a substantial part of the population.

The Price of Global Health is the first book of its kind: an in-depth but straightforward exploration of the pricing process and its implications. The book is designed to help a wide range of audiences gain a better understanding of this complex and emotionally charged field.

This book is an invaluable resource for anybody who is interested, involved in or affected by the development, funding and utilization of prescription drugs. It is of critical importance to pharmaceutical company executives and other leaders and professionals in drug development and commercialization, including marketing, business development, access and pricing, clinical development, drug discovery, regulatory affairs, market research and public affairs.

Consumers will gain an understanding of drug company decisions and how they impact the emergence of new and innovative therapies; better appreciate how pharmaceutical prices are determined and what factors influence the process; form an opinion on how various healthcare reform proposals may impact their ability to obtain future drug treatments.

Legislators will understand reasons for differences between global healthcare systems and drug pricing and access controls; grasp the advantages and pitfalls of seemingly attractive control mechanisms employed in various countries; be able to propose healthcare legislation that ensures appropriate healthcare coverage of patients and allows for future exploration of new therapies.

Pharmaceutical company leaders will learn how to optimize their drug development efforts and avoid hundreds of millions of dollars in misdirected drug development investments, by ensuring that they are optimally directed at today's critical decision makers in global markets; be able to identify real commercial potential of development compounds and licensing opportunities, thus allowing for better decisions and avoiding investments in compounds with rapid FDA approval times, but with poor commercial prospects.

Access and pricing professionals will gain insight into how to analyze pricing and access opportunities for new drugs with some well-structured analytical frameworks, thus enabling them to bring blockbuster and specialty drugs to their full potential; understand how to develop a meaningful value story for payers and their advisers, thus enabling broader access and avoiding large discounts and rebates that can eliminate profit margins; avoid common pitfalls in payer and pricing research that is done to optimize and validate pricing and access strategies.

This book consists of seven parts and 41 chapters. There is a logical sequence to the chapters, but each can be read individually to suit each reader's interests. Readers with a general (non-specialist) interest in pharmaceutical pricing issues are recommended to read Chapters 1 to 8 and 13 for an initial overview and then select further chapters depending on their specific interest.

Part A provides a basic overview of the pharmaceutical access and pricing environment, recent trends and related corporate challenges. It describes the impact of the current "Age of Value and Affordability" on access and prescribing decision making and introduces the "Access Journey" as an organizing framework to design effective development and commercialization strategies that help companies to sustain success throughout these changes.

Part B describes the roles of important Access Journey stakeholders (payers, medical community, provider organizations and patients), their values and the evidence that may sway their decisions.

Part C discusses methodologies that payers worldwide employ to manage pricing and prescription drug utilization within their budgets. Chapters are included about fundamentals of pricing, referenced based pricing, health economics and outcomes research, and an introduction to the benefits pyramid.

Part D focuses on the drug development process and some structured approaches to determine viable pricing options based on an analysis of the market and an assessment of the benefits and evidence provided.

Part E discusses global pricing strategies and their public policy and ethical considerations, as well as market specifics for oncology, gene therapies and biosimilars.

Part F addresses implementation, including negotiations, value-based agreements, deal making and beyond-the-pill offerings.

Part G contains details descriptions of 13 global healthcare systems with a description of pricing and reimbursement systems and their requirements.