Orphan Drugs Understanding The Rare Disease Market And Its Dynamics Woodhead Publishing Series In Biomedicine

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Rare Diseases Epidemiology: Update and Overview - Manuel Posada de la Paz 2017-12-06 The fields of rare diseases research and orphan products development continue to expand with more products in research and development status. In recent years, the role of the patient advocacy groups has evolved into a research partner with the academic research

community and the bio-pharmaceutical industry. Unique approaches to research and development require epidemiological data not previously available to assist in protocol study design and patient recruitment for clinical trials required by regulatory agencies prior to approval for access by patents and practicing physicians.

Orphan Drug Amendments of 1991 - United States. Congress. Senate. Committee on Labor and Human Resources 1992

Does Misery Love Company? - Frank R. Lichtenberg 2003

With substantial fixed costs of drug development, more common conditions can support more products. If additional pharmaceutical products are beneficial, they will attract greater consumption and promote better health, e.g. greater longevity. We ask how market size measured by condition prevalence affects consumption and longevity. We document in condition cross sections that both the tendency to use a drug and longevity are higher for individuals with more prevalent conditions. We also make use of the 1983 Orphan Drug Act (ODA), which promoted development of drugs for the treatment of rare conditions. Longevity and drug use have grown more quickly for persons with rare diseases and even more quickly for persons with conditions with substantial orphan drug use.

Orphan Drugs - Faraz Farooq 2016-12-15

While drug discovery for common disorders is somewhat standard and based on the large potential markets, the small market for most orphan diseases means that effective therapies are uncommon. With next generation DNA sequencing, the pace of inherited novel rare disease gene identification has increased markedly. Drug discovery for rare diseases is somewhat

hampered by a lack of collaboration between academia and industry separating the necessary resources from skills. This volume discusses important aspects of orphan drug development such as regulatory affairs, public industry partnership, bench to bedside using examples of rare diseases, challenges of clinical trials and future directions.

Orphan Drugs - Carolyn H. Asbury 1985

The Problem of Herbal Medicines Legal Status - José Luis Valverde 1999

The main problem in the use of medicinal plants, discussed in this book, is that citizens are being persuaded that herbal medicine is based on its low or even absent toxicology. A good efficacy is assumed as self-evident, and therapeutic benefit without risks is expected. Many users prefer natural medicine instead of synthetic remedies. However, the number of reports on unwanted side effects of phytomedicines increased in the last years. In some instances, a lack of pharmaceutical quality was found. The unqualified recommendation of herbal medicines may represent a considerable risk to the user. The use of a herbal remedy with unproven efficacy can represent a risk for the user when a more effective and necessary treatment will therefore be stopped or omitted. These circumstances must be taken into account by the governments, inspection services, the doctors and the judges. The present approach to herbal medicines and fraud with these products do not receive the necessary punishment because it is believed that if the product does not have any therapeutic property, it cannot entail any harm either.

Biologics, Biosimilars, and Biobetters - Iqbal Ramzan 2021-02-03

A comprehensive primer and reference, this book provides pharmacists and health practitioners the relevant science and

policy concepts behind biologics, biosimilars, and biobetters from a practical and clinical perspective. Explains what pharmacists need to discuss the equivalence, efficacy, safety, and risks of biosimilars with physicians, health practitioners, and patients about Guides regulators on pragmatic approaches to dealing with these drugs in the context of rapidly evolving scientific and clinical evidence Balances scientific information on complex drugs with practical information, such as a checklist for pharmacists

Rare Disease Drug Development - Raymond A. Huml 2021-11-08

This book provides a broad overview of rare disease drug development. It offers unique insights from various perspectives, including third-party capital providers, caregivers, patient advocacy groups, drug development professionals, marketing and commercial experts, and patients. A unique reference, the book begins with narratives on the many challenges faced by rare disease patient and their caregivers. Subsequent chapters underscore the critical, multidimensional role of patient advocacy groups and the novel approaches to related clinical trials, investment decisions, and the optimization of rare disease registries. The book addresses various rare disease drug development processes by disciplines such as oncology, hematology, pediatrics, and gene therapy. Chapters then address the operational aspects of drug development, including approval processes, development accelerations, and market access strategies. The book concludes with reflections on the authors' case for real-world data and evidence generation in orphan medicinal drug development. Rare Disease Drug Development

is an expertly written text optimized for biopharmaceutical R&D experts, commercial experts, third-party capital providers, patient advocacy groups, patients, and caregivers.

Encyclopedia of Pharmacy Practice and Clinical Pharmacy - 2019-06-28

Encyclopedia of Pharmacy Practice and Clinical Pharmacy covers definitions, concepts, methods, theories and applications of clinical pharmacy and pharmacy practice. It highlights why and how this field has a significant impact on healthcare. The work brings baseline knowledge, along with the latest, most cutting-edge research. In addition, new treatments, algorithms, standard treatment guidelines, and pharmacotherapies regarding diseases and disorders are also covered. The book's main focus lies on the pharmacy practice side, covering pharmacy practice research, pharmacovigilance, pharmacoeconomics, social and administrative pharmacy, public health pharmacy, pharmaceutical systems research, the future of pharmacy, and new interventional models of pharmaceutical care. By providing concise expositions on a broad range of topics, this book is an excellent resource for those seeking information beyond their specific areas of expertise. This outstanding reference is essential for anyone involved in the study of pharmacy practice. Provides a 'one-stop' resource for access to information written by world-leading scholars in the field Meticulously organized, with articles split into three clear sections, it is the ideal resource for students, researchers and professionals to find relevant information Contains concise and accessible chapters that are ideal as an authoritative introduction for non-specialists and readers

from the undergraduate level upwards Includes multimedia options, such as hyperlinked references and further readings, cross-references and videos

Breakthrough Business Models - Institute of Medicine 2009-02-17

The process for developing new drug and biologic products is extraordinarily expensive and time-consuming. Although large pharmaceutical companies may be able to afford the cost of development because they can expect a large return on investment, organizations developing drugs to treat rare and neglected diseases are unable to rely on such returns. On June 23, 2008, the Institute of Medicine's Forum on Drug Discovery, Development, and Translation held a public workshop, "Breakthrough Business Models: Drug Development for Rare and Neglected Diseases and Individualized Therapies," which sought to explore new and innovative strategies for developing drugs for rare and neglected diseases.

Rare Diseases and Orphan Drugs - Jules J. Berman 2014-05-26

Rare Diseases and Orphan Drugs shows that much of what we now know about common diseases has been achieved by studying rare diseases. It proposes that future advances in the prevention, diagnosis, and treatment of common diseases will come as a consequence of our accelerating progress in the field of rare diseases. Understanding the complex steps in the development of common diseases, such as cancer, cardiovascular disease, and metabolic diseases, has proven a difficult problem. Rare diseases, however, are often caused by aberrations of a single gene. In rare diseases, we may study how specific genetic defects can trigger a series of events that lead to the expression of a particular disease. Often, the disease process manifested in a

certain rare disease is strikingly similar to the disease process observed in a common disease. This work ties the lessons learned about rare diseases to our understanding of common ones. Chapters covering the number of common diseases are minimized, while rare diseases are introduced as single diseases or as members of diseases classes. After reading this book, readers will appreciate how further research into the rare diseases may lead to new methods for preventing, diagnosing, and treating all diseases, rare or common. Makes rare diseases relevant to clinicians and researchers by tying lessons learned about the rare diseases to our understanding of the common diseases Stresses basic pathologic mechanisms that account for human disease (e.g., disorders of cell development, replication, maintenance, function and structure), that can be understood without prior training in pathology Discusses advanced concepts in molecular biology and genetics in a simple, functional context appropriate for medical trainees and new researchers Offers insights into how further research into rare diseases may lead to new methods for preventing, diagnosing, and treating all diseases.

Precision Public Health - Tarun Weeramanthri 2018-06-25

Precision Public Health is a new and rapidly evolving field, that examines the application of new technologies to public health policy and practice. It draws on a broad range of disciplines including genomics, spatial data, data linkage, epidemiology, health informatics, big data, predictive analytics and communications. The hope is that these new technologies will strengthen preventive health, improve access to health care, and reach disadvantaged

populations in all areas of the world. But what are the downsides and what are the risks, and how can we ensure the benefits flow to those population groups most in need, rather than simply to those individuals who can afford to pay? This is the first collection of theoretical frameworks, analyses of empirical data, and case studies to be assembled on this topic, published to stimulate debate and promote collaborative work.

Drug Repurposing - David Cavalla 2022-02-09

Drug repurposing, or repositioning, is the development of existing drugs for new uses: given that 9 in 10 drugs that enter drug development are never marketed and therefore represent wasted effort, it is an attractive as well as inherently more efficient process. Three repurposed drugs can be brought to market for the same cost as one new chemical entity; and they can also be identified more quickly, an important benefit for patients whose diseases are progressing faster than therapeutic innovation. But repurposing also requires a fresh look at configuring pharmaceutical R&D, considering clinical, regulatory and patent issues much earlier than would otherwise be the case. In addition to new ways of thinking, the discovery of repurposing opportunities can take advantage of artificial intelligence techniques to match the perfect new use for an existing drug. This book provides an ideal introduction to the field of drug repurposing with contributions from world-renowned experts culminating in an excellent resource for any drug discovery or medicinal chemist.

Rare Diseases and Orphan Products - Institute of Medicine 2011-04-03

Rare diseases collectively affect millions of Americans of all ages, but developing drugs and medical devices to prevent,

diagnose, and treat these conditions is challenging. The Institute of Medicine (IOM) recommends implementing an integrated national strategy to promote rare diseases research and product development.

Orphan Drugs - Elizabeth Hernberg-Ståhl 2013-11-15

This authoritative and comprehensive book makes the reader familiar with the processes of bringing orphan drugs to the global market. There are between 5,000 and 7,000 rare diseases and the number of patients suffering from them is estimated to be more than 50 million in the US and Europe. Before the orphan drug legislation enacted in the US in 1983, there was a limited interest from industry to develop treatment for very small patient groups. One of the difficulties is, of course, that similar levels of investment are needed from a pharmaceutical company to bring a drug to the market for both small and large patient groups. The journey from application of an orphan drug designation to a reimbursed market-approved drug is long and many obstacles occur during the journey. After reading the book, readers will: Understand who the players/stakeholders are in the rare orphan disease field and their specific needs and concerns: patients and patient organizations, researchers and treating physicians within the field, industry, regulatory and reimbursement bodies* Understand the strong partnership between the different players and the various initiatives to improve and increase access to treatment for patients; minimizing the gap between numbers of known diseases, orphan designations, approved drugs and paid drugs. The book also provides short practical case stories from patients and researchers, as well as representatives from industry and authorities on the challenges they

came across in developing orphan drugs or getting access to orphan drugs. A comprehensive overview of strategy, key activities and considerations of how to bring an orphan drug from concept to the market and make it available to patients A source of updated information, news and trends for those who are already active in this fast-evolving field Covers the global definitions and the criteria for getting an orphan drug designation in, for example, the US and Europe

Access to Orphan Drugs in Guatemala - Yolanda Cabrera-Sybesma 2016-09-30

In this series Access to Medicines - Orphan Drugs; Volume 1: Guatemala, Central America provides a A Gap Analysis from an International Perspective which highlights the rarely talked about topic of Access to Orphan Drugs for the treatment of rare diseases in this Central American Country. This updated version provides the latest data trends specific to Guatemala and looks at the trends from an international perspective. Series: Access to Medicines - Orphan Drugs Volume 1- Central America (Guatemala).

Orphan Drugs and Rare Diseases - David C Pryde 2014

This book provides an up-to-date monograph on the drug discovery and regulatory elements of therapeutics used to treat rare or "orphan" diseases.

Anticompetitive Abuse of the Orphan Drug Act - United States. Congress. Senate. Committee on the Judiciary. Subcommittee on Antitrust, Monopolies, and Business Rights 1992

Pediatric Drug Development - Andrew E. Mulberg 2013-05-20

Pediatric Drug Development, Second Edition, encompasses the new regulatory initiatives across EU, US and ROW designed to

encourage improved access to safe and effective medicines for children. It includes new developments in biomarkers and surrogate endpoints, developmental pharmacology and other novel aspects of pediatric drug development.
Orphan Products Development – 1998

Financial and Economic Implications of Orphan Drugs – John McGuire 2014
In a free market, the pricing of prescription drugs is set by supply and demand. Under these conditions, low demand for orphan drugs (drugs used to treat rare diseases) would have prohibitively high prices for buyers and discourage investment in research and development for new orphan drugs by pharmaceutical manufacturers. However, orphan drug legislation designed to encourage the development and distribution of these drugs in various international jurisdictions has supported the financial profitability of the pharmaceutical manufacturing industry. In many developed economies owing to government intervention, health care does not always behave as a normal good. Consequently, these interventions have been made to provide access to orphan drugs while balancing recognition of the investments made in research and development by pharmaceutical manufacturers. Using Canada as a base case and expanding the analysis to several other developed economies, the authors first demonstrate that currently, there is no legislation to regulate the pricing of orphan drugs in Canada. Accordingly, the financial and economic implications of orphan drug pricing are significant from the perspectives of suppliers and buyers, as well as the federal and provincial governments in Canada, as well as several other countries.

Proceedings of 4th World Congress on Rare Diseases and Orphan Drugs 2018 - ConferenceSeries June 11-12, 2018 | Dublin, Ireland Key Topics : Neglected Tropical Diseases, Rare Pulmonary Diseases, Rare Diseases in Neurology, Rare Genetic Diseases, Scope of Orphan Drugs, Rare diseases of Endocrine System, Rare diseases of Immune System, Rare Cardiac Diseases, Rare Eye and Ear Diseases, Orphan Drugs Treatment for Rare Diseases, Rare Oral Diseases, Rare Hepatic Diseases, Rare Gastrointestinal Diseases, Rare Bacterial, Viral and Fungal infections, Rare diseases of Genitourinary System, Rare diseases in Nephrology, Rare Skin Diseases, Clinical Research on Orphan Drugs, Rare Morphological Diseases, Development of Orphan Products, Rare Diseases in Oncology, Rare Diseases in Anaesthesiology, Rare Diseases in Haematology, Orphan Drugs Market Research, Rare Gynaecological and Obstetrical Diseases, Pediatric Rare Diseases, Current Rare Diseases Research, Rare Diseases of Sexual Health, Rare Hereditary Diseases, Diagnosis and Treatment for Rare Diseases, Clinical case studies on Rare Diseases, Imaging of Rare Diseases, Other Rare Diseases,

Drug Repurposing - Farid A. Badria 2020-12-02 Drug repurposing or drug repositioning is a new approach to presenting new indications for common commercial and clinically approved existing drugs. For example, chloroquine, an old antimalarial drug, showed promising results for treating COVID-19, interfering with MDR in several types of cancer, and chemosensitizing human leukemic cells.This book focuses on the hypothesis, risk/benefits, and economic impacts of drug repurposing on drug discovery in dermatology, infectious

diseases, neurological disorders, cancer, and orphan diseases. It brings together up-to-date research to provide readers with an informative, illustrative, and easy-to-read book useful for students, clinicians, and the pharmaceutical industry.

Orphan Drug Act - United States. Congress. House. Committee on Energy and Commerce. Subcommittee on Health and the Environment 1990

Inequalities in Health - Nir Eyal 2013-10
Which inequalities in longevity and health among individuals, groups, and nations are unfair? And what priority should health policy attach to narrowing them? These essays by philosophers, economists, epidemiologists, and physicians attempt to determine how health inequalities should be conceptualized, measured, ranked, and evaluated.

Global Pediatric Development of Drugs, Biologics, and Medical Devices - Jocelyn Jennings 2021-11-26

Orphan - Philip Reilly 2015
"This book is about the struggle to save the lives of children who, because of a roll of the genetic dice, are born with any one of more than several thousand rare genetic disorders. It recounts the now century long effort of small groups of physicians and scientists to take on some of these genetic diseases. In many cases just a few physician-scie

Innovative Methods for Rare Disease Drug Development - Shein-Chung Chow 2020-11-11
In the United States, a rare disease is defined by the Orphan Drug Act as a disorder or condition that affects fewer than 200,000 persons. For the approval of "orphan" drug products for rare diseases, the traditional approach of power analysis for sample size calculation

is not feasible because there are only limited number of subjects available for clinical trials. In this case, innovative approaches are needed for providing substantial evidence meeting the same standards for statistical assurance as drugs used to treat common conditions. Innovative Methods for Rare Disease Drug Development focuses on biostatistical applications in terms of design and analysis in pharmaceutical research and development from both regulatory and scientific (statistical) perspectives. Key Features: Reviews critical issues (e.g., endpoint/margin selection, sample size requirements, and complex innovative design). Provides better understanding of statistical concepts and methods which may be used in regulatory review and approval. Clarifies controversial statistical issues in regulatory review and approval accurately and reliably. Makes recommendations to evaluate rare diseases regulatory submissions. Proposes innovative study designs and statistical methods for rare diseases drug development, including n-of-1 trial design, adaptive trial design, and master protocols like platform trials. Provides insight regarding current regulatory guidance on rare diseases drug development like gene therapy.

Rare Diseases Epidemiology - Manuel Posada de la Paz 2012-11-07

In our etiologic research, we epidemiologists need to leave behind the concepts of ‘cohort’ study and ‘case-control’ study and adopt that of the etiologic study as the singular substitute for these. With this sentence, the famous epidemiologist Professor Olli S. Miettinen began his personal re ection on the future of the epidemiology [1]. He sought to highlight the

fact that the role of the epidemiologist should be mainly focused on aetiological research. Nevertheless, the widespread idea still exists that epidemiology is limited to purely providing gures and descriptive data on the frequency and distribution of disease. Indeed, it is more than likely that the precise aim of those rst classic epidemiological steps, i. e. , methods essentially based on describing the distri- tion of a given disease, is still not all that well understood by many scientists, let alone the general public. Such descriptions seek to generate hypotheses and afford explanations for key factors (be these risk factors or the presumable causes th- selves), which might justify differences in terms of persons, time or place and, in turn, ultimately serve to develop preventive measures and/or gain quality-adjusted life years. To restrict the goals of epidemiology to activities exclusively concerned with reporting gures or even complex statistical results is a great mistake, one that renders it dif cult to take full advantage of the epidemiologist's true role, which is "to study disease determinants and to assess the actual impact of factors involved in their development, distribution and dissemination".

Health Technology Assessment in Japan - Isao Kamae 2019-09-03

Representing the first book on the topic, this work offers the reader an introduction to the Japanese systems for health technology assessment (HTA) officially introduced by the Ministry of Health, Labour and Welfare (MHLW) in 2016. Policy and guidelines are discussed, with the relevant methods and conditions of cost-effectiveness analysis explained alongside. Numerous instructive examples and exercises, ranging from basic to

advanced, impart valuable knowledge and insight on the quantitative methods for economic evaluation, which will appeal to both beginners and experts. This guidebook is authored by Japan's foremost expert in HTA and pharmacoeconomics, with a view to strengthening the reader's expertise in value-based healthcare and decision-making. The methods presented are essential to informing regulatory, local and patient decisions; as such, the book is equally recommended to industry and government, as well as academia, and anyone with an interest in Japanese HTA.

Our Bodies, Our Data - Adam Tanner 2017-01-10

How the hidden trade in our sensitive medical information became a multibillion-dollar business, but has done little to improve our health-care outcomes Hidden to consumers, patient medical data has become a multibillion-dollar worldwide trade industry between our health-care providers, drug companies, and a complex web of middlemen. This great medical-data bazaar sells copies of the prescription you recently filled, your hospital records, insurance claims, blood-test results, and more, stripped of your name but possibly with identifiers such as year of birth, gender, and doctor. As computing grows ever more sophisticated, patient dossiers become increasingly vulnerable to reidentification and the possibility of being targeted by identity thieves or hackers. Paradoxically, comprehensive electronic files for patient treatment—the reason medical data exists in the first place—remain an elusive goal. Even today, patients or their doctors rarely have easy access to comprehensive records that could improve care. In the evolution of medical data, the instinct for profit has outstripped patient needs. This book

tells the human, behind-the-scenes story of how such a system evolved internationally. It begins with New York advertising man Ludwig Wolfgang Frohlich, who founded IMS Health, the world's dominant health-data miner, in the 1950s. IMS Health now gathers patient medical data from more than 45 billion transactions annually from 780,000 data feeds in more than 100 countries. Our Bodies, Our Data uncovers some of Frohlich's hidden past and follows the story of what happened in the following decades. This is both a story about medicine and medical practice, and about big business and maximizing profits, and the places these meet, places most patients would like to believe are off-limits. Our Bodies, Our Data seeks to spark debate on how we can best balance the promise big data offers to advance medicine and improve lives while preserving the rights and interests of every patient. We, the public, deserve a say in this discussion. After all, it's our data.

Orphan Lung Diseases - J.-F. Cordier 2014-05-14

Orphan lung diseases differ from the more common pulmonary disorders, due to the fact that the respiratory physician will only see a few of them each year or even during their career. However, as a specialist, it is necessary to identify and confirm such a diagnosis in a patient. This Monograph comprehensively covers the most common and/or complex of these orphan lung diseases. This Monograph should be seen as a solid companion for the respiratory specialist each time they need to consider a diagnosis of one of these orphan diseases.

The Social Construction of the Orphan Drug Industry - Qing Ying Low (Timothy) 2018

"Sociologists have noted that markets are not always formed "naturally", and the

creation of markets would require social actors such as the legislator to legitimate it and policies by the state to support its establishment. Using legitimation and choice-within-constraints framework, this would also seem to be the case for the orphan drug industry. Due to the nature of orphan drug being used to treat a small population of patients with rare diseases, legislators in the United States have created new justifications to pass the Orphan Drug Act in 1983. While the act has encouraged pharmaceutical companies to develop orphan drugs, scholars have noted that little has been done to reduce orphan drug prices to ensure that patients have access to these drugs. This paper will argue that during the legitimation of the Orphan Drug Act, legislators have been committed and constrained to a set of "free market" ideas when debating and proposing solutions. The justifications for the orphan drug act had generally been embedded within "free market" ideas. While there were instances that legislators used arguments such as "basic human right" to pass the act, these justifications became less relevant in future debates. Justification tied to "free market" ideas that were created for the orphan drug act has also become constraints to future legislators, resulting in Congress to pay little attention to the issue of high orphan drug prices and access of orphan drug for rare disease patient" -- Abstract.

Introduction to Basics of Pharmacology and Toxicology - Gerard Marshall Raj 2019-11-16 This book illustrates, in a comprehensive manner, the most crucial principles involved in pharmacology and allied sciences. The title begins by discussing the historical aspects of drug discovery, with up

to date knowledge on Nobel Laureates in pharmacology and their significant discoveries. It then examines the general pharmacological principles – pharmacokinetics and pharmacodynamics, with in-depth information on drug transporters and interactions. In the remaining chapters, the book covers a definitive collection of topics containing essential information on the basic principles of pharmacology and how they are employed for the treatment of diseases. Readers will learn about special topics in pharmacology that are hard to find elsewhere, including issues related to environmental toxicology and the latest information on drug poisoning and treatment, analytical toxicology, toxicovigilance, and the use of molecular biology techniques in pharmacology. The book offers a valuable resource for researchers in the fields of pharmacology and toxicology, as well as students pursuing a degree in or with an interest in pharmacology.

Economic Dimensions of Personalized and Precision Medicine - Ernst R. Berndt 2019-04-22

Personalized and precision medicine (PPM)—the targeting of therapies according to an individual's genetic, environmental, or lifestyle characteristics—is becoming an increasingly important approach in health care treatment and prevention. The advancement of PPM is a challenge in traditional clinical, reimbursement, and regulatory landscapes because it is costly to develop and introduces a wide range of scientific, clinical, ethical, and socioeconomic issues. PPM raises a multitude of economic issues, including how information on accurate diagnosis and treatment success will be disseminated and who will bear the cost;

changes to physician training to incorporate genetics, probability and statistics, and economic considerations; questions about whether the benefits of PPM will be confined to developed countries or will diffuse to emerging economies with less developed health care systems; the effects of patient heterogeneity on cost-effectiveness analysis; and opportunities for PPM's growth beyond treatment of acute illness, such as prevention and reversal of chronic conditions. This volume explores the intersection of the scientific, clinical, and economic factors affecting the development of PPM, including its effects on the drug pipeline, on reimbursement of PPM diagnostics and treatments, and on funding of the requisite underlying research; and it examines recent empirical applications of PPM.

Orphan Diseases and Orphan Drugs - I. Herbert Scheinberg 1986

The Oxford Handbook of the Economics of the Biopharmaceutical Industry - Patricia M. Danzon 2012-04-12

The biopharmaceutical industry has been a major driver of technological change in health care, producing unprecedented benefits for patients, cost challenges for payers, and profits for shareholders. As consumers and companies benefit from access to new drugs, policymakers around the globe seek mechanisms to control prices and expenditures commensurate with value. More recently the 1990s productivity boom of new products has turned into a productivity bust, with fewer and more modest innovations, and flat or declining revenues for innovative firms as generics replace their former blockbuster products. This timely volume examines the economics of the biopharmaceutical industry, with eighteen chapters by leading

academic health economists. Part one examines the economics of biopharmaceutical innovation including determinants of the costs and returns to new drug development; how capital markets finance R&D and how costs of financing the biopharmaceutical industry compare to financing costs for other industries; the effects of safety and efficacy regulation by the Food and Drug Administration (FDA) and of price and reimbursement regulation on incentives for innovation; and the role of patents and regulatory exclusivities. Part two examines the market for biopharmaceuticals with chapters on prices and reimbursement in the US, the EU, and other industrialized countries, and in developing countries. It looks at the optimal design of insurance for drugs and the effects of cost sharing on spending and on health outcomes; how to measure the value of pharmaceuticals using pharmacoeconomics, including theory, practical challenges, and policy issues; how to measure pharmaceutical price growth over time and recent evidence; empirical evidence on the value of pharmaceuticals in terms of health outcomes; promotion of pharmaceuticals to physicians and consumers; the economics of vaccines; and a review of the evidence on effects of mergers, acquisitions and alliances. Each chapter summarizes the latest insights from theory and recent empirical evidence, and outlines important unanswered questions and areas for future research. Based on solid economics, it is nevertheless written in terms accessible to the general reader. The book is thus recommended reading for academic economists and non-economists, and for those in industry and policy who wish to understand the economics

of this fascinating industry.

Chasing My Cure - David Fajgenbaum 2019-09-10 LOS ANGELES TIMES AND PUBLISHERS WEEKLY BESTSELLER • The powerful memoir of a young doctor and former college athlete diagnosed with a rare disease who spearheaded the search for a cure—and became a champion for a new approach to medical research. “A wonderful and moving chronicle of a doctor’s relentless pursuit, this book serves both patients and physicians in demystifying the science that lies behind medicine.”—Siddhartha Mukherjee, New York Times bestselling author of The Emperor of All Maladies and The Gene David Fajgenbaum, a former Georgetown quarterback, was nicknamed the Beast in medical school, where he was also known for his unmatched mental stamina. But things changed dramatically when he began suffering from inexplicable fatigue. In a matter of weeks, his organs were failing and he was read his last rites. Doctors were baffled by his condition, which they had yet to even diagnose. Floating in and out of consciousness, Fajgenbaum prayed for a second chance, the equivalent of a dramatic play to second the game into overtime. Miraculously, Fajgenbaum survived—only to endure repeated near-death relapses from what would eventually be identified as a form of Castleman disease, an extremely deadly and rare condition that acts like a cross between cancer and an autoimmune disorder. When he relapsed while on the only drug in development and realized that the medical community was unlikely to make progress in time to save his life, Fajgenbaum turned his desperate hope for a cure into concrete action: Between hospitalizations he studied his own charts and tested his own blood

samples, looking for clues that could unlock a new treatment. With the help of family, friends, and mentors, he also reached out to other Castleman disease patients and physicians, and eventually came up with an ambitious plan to crowdsource the most promising research questions and recruit world-class researchers to tackle them. Instead of waiting for the scientific stars to align, he would attempt to align them himself. More than five years later and now married to his college sweetheart, Fajgenbaum has seen his hard work pay off: A treatment he identified has induced a tentative remission and his novel approach to collaborative scientific inquiry has become a blueprint for advancing rare disease research. His incredible story demonstrates the potency of hope, and what can happen when the forces of determination, love, family, faith, and serendipity collide. Praise for Chasing My Cure "A page-turning chronicle of living, nearly dying, and discovering what it really means to be invincible in hope."—Angela Duckworth, #1 New York Times bestselling author of Grit "[A] remarkable memoir . . . Fajgenbaum writes lucidly and movingly . . . Fajgenbaum's stirring account of his illness will inspire readers."—Publishers Weekly

Small Market Drugs, Big Price Tags - United States. Congress. Joint Economic Committee 2009

Value Assessment of Orphan Drugs and Treatments for Rare Diseases - Catherine M. Lockhart 2016 OBJECTIVES: In 1983 the US Orphan Drug Act was passed to facilitate commercialization of drugs to treat rare diseases. The market value for orphan drugs in the US reached $90 billion annually in 2014, with worldwide sales forecast at $176 billion. Payers and

policymakers need robust methodology for evaluation of health technology in this growing landscape of expensive treatments for rare diseases. Here I present a systematic review of current practices in value-based evaluation of orphan drugs from a global perspective. I also propose a potential new framework to be developed as new metric for assessing the value of orphan drugs, the Orphan Drug Index Estimate (ODIE). METHODS: For the systematic review, searches were conducted in December 2015 in PubMed®, EMBASE®, and Web of Science® databases using the following keywords: orphan drug, rare disease treatment, economics, resource utilization, cost, cost effectiveness, questionnaire, and value. Only references published in English were included. Manuscripts that solely reported one of the following were excluded: clinical or patient care, policy or legislation on orphan drugs particularly relating to research incentive, opinion or editorial, preclinical studies, drug-development, unrelated to rare diseases or healthcare, reviews other than systematic reviews for health technology assessment. RESULTS: A total of 2513 unique references were obtained, and screened by title and abstract according to exclusion criteria. After exclusion, 333 references remained for full evaluation. Of those, an additional 296 were excluded, but 51 additional studies were included from the reference lists of included articles. A total of 88 articles were included in the complete analysis. Overall, the methodology employed for conducting cost-effectiveness assessments followed traditional techniques including decision analysis and Markov modeling techniques. The

reported incremental cost effectiveness ratios (ICERs) ranged from dominant treatments to a high of €6.1 million per quality adjusted life year (QALY). Interpretation of the results was more challenging, with 43% of studies reporting ICERs that would not be considered cost-effective under a willingness-to-pay threshold of $50,000 per QALY. In spite of the lack of cost-effectiveness, the majority of authors agreed that since the treatment under review is for a rare condition, there is an obligation to cover the costs. In light of these analyses, there is an evident need for a method of analysis that is more comprehensive than the ICER, and more appropriate for addressing the uniqueness of orphan drugs, including variables related to the rarity and severity of disease, and a broader societal perspective on costs, including societal burden and identifiable opportunity costs. In response, here I propose a potential new metric based on multicriteria decision analysis (MCDA) techniques to provide a more comprehensive evaluation of orphan drugs. CONCLUSIONS: There is a global consensus of a need to develop appropriate methodology, analysis techniques, and related policies to address management of expensive treatments. It is not yet clear how best to evaluate the value of orphan drugs. More thorough evaluation and validation of novel modeling techniques, analytic rationale and proactive policy changes are needed to redefine the status quo of health technology assessment of rare disease treatments. I propose a new metric to overcome some limitations of the ICER in evaluation of rare diseases. Continued research is needed in detailed development of a valid, quantifiable, and reproducible metric;

however, the work presented here provides a foundation for the development process.