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

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.

The recent momentum and urgency around translating science and technology into health innovation is inspiring. It is transforming academia, too, as the rapidly-evolving world of health innovation has given rise to a new breed of academic - the academic entrepreneur - who works to move ideas from initial research to practical implementation. The work of these individuals is crucial to realizing the potential of investments in better care, and yet there existed no central repository for information and wisdom relevant to their mission; no place to house and explore the evolving knowledge base around translating evidence into impact.We aim to build one. In the spirit of collaboration, the Children's Hospital of Philadelphia (CHOP) Research Institute collaborated with the University of Pennsylvania's (Penn) Institute for Translational Medicine and Therapeutics (ITMAT) to seed fund a grassroots effort of editors, subject matter experts, and translational research students to create a free open education resource stored on ScholarlyCommons (University of Pennsylvania, Philadelphia, PA).Academic Entrepreneurship seeks to build a diverse community of empowered professionals who know how to bridge the worlds of academic research and commercialization to turn ideas and discoveries into innovations that provide value to patients, providers, and healthcare systems, thereby realizing full market potential and societal impact. This book is a repository of tools, advice, and best practices that establishes a foundation for academic researchers and innovators wherever they may reside.Recognizing that academic entrepreneurs are busy and bright, and have limited time to learn entrepreneurship, the chapters in this book were designed as an efficient and state-of-the-art source of guidance. With carefully curated content as a strong foundation, the reader will have quick introductions to key topics in academic entrepreneurship and innovations with a list of resources for those who wish to go further.This book was created as a limited print run of the first edition of the living content stored in the University of Pennsylvania's open access repository, ScholarlyCommons, as of 1/1/2020. As a living e-textbook, the content of Academic Entrepreneurship for Medical and Health Scientists is continuously enhanced and revised.

RARE CANCER AGENDA 2030 Ten Recommendations from the EU Joint Action on Rare Cancers 1. Rare cancers are the rare diseases of oncology 2. Rare cancers should be monitored 3. Health systems should exploit networking 4. Medical education should exploit and serve healthcare networking 5. Research should be fostered by networking and should take into account an expected higher degree of uncertainty 6. Patient-physician shared clinical decision-making should be especially valued 7. Appropriate state-of-the-art instruments should be developed in rare cancer 8. Regulation on rare cancers should tolerate a higher degree of uncertainty 9. Policy strategies on rare cancers and sustainability of interventions should be based on networking 10. Rare cancer patients should be engaged

Nearly every type of cancer passes through a precancer phase, during which it cannot metastasize or invade other tissues. While medicine is

not always successful in treating or curing advanced stages of cancers, recent advances in our understanding of carcinogenesis have helped us to develop strategies to prevent, diagnose, and treat many cancers at the precancer stage. Research in this field is escalating rapidly as the evidence increasingly shows that the number of annual cancer deaths could be drastically reduced through the effective treatment and cure of precancer lesions. This book begins by explaining why it has been so difficult to cure cancers, followed by a review of precancer biology, with descriptions of the most common precancer lesions. The final chapters provide practical socio-political and medical goals for precancer treatment, including discussions of the economics and politics of treating precancers.

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.

A primary concern of rare disease diagnosis is the lack of accurate information that may lead to delayed interventions, administering

inaccurate treatments, and social consequences. Health communication continues to be one-way and rely on the expertise from the health practitioner. In such a broad spectrum of rare diseases, patients may find it difficult to obtain timely information, accurate diagnosis, and appropriate treatments, surgeries, medications, or psychological counseling in their own countries. The use of information and communication technologies can create new communication channels that address this lack of knowledge. Communicating Rare Diseases and Disorders in the Digital Age is an essential reference source that uses computer-mediated communication to improve patient knowledge when afflicted or dealing with rare health conditions. Featuring research on topics such as support networking, eHealth management, and social computing, this book is ideally designed for health practitioners, physicians, patients, medical administrators, nurses, surgeons, infectious disease educators, hospital directors, world health organizations, academicians, students, and researchers seeking coverage on current advances in health communication, computer science, and epidemiology.

This User's Guide is intended to support the design, implementation, analysis, interpretation, and quality evaluation of registries created to increase understanding of patient outcomes. For the purposes of this guide, a patient registry is an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more predetermined scientific, clinical, or policy purposes. A registry database is a file (or files) derived from the registry. Although registries can serve many purposes, this guide focuses on registries created for one or more of the following purposes: to describe the natural history of disease, to determine clinical effectiveness or cost-effectiveness of health care products and services, to measure or monitor safety and harm, and/or to measure quality of care. Registries are classified according to how their populations are defined. For example, product registries include patients who have been exposed to biopharmaceutical products or medical devices. Health services registries consist of patients who have had a common procedure, clinical encounter, or hospitalization. Disease or condition registries are defined by patients having the same diagnosis, such as cystic fibrosis or heart failure. The User's Guide was created by researchers affiliated with AHRQ's Effective Health Care Program, particularly those who participated in AHRQ's DEcIDE (Developing Evidence to Inform Decisions About Effectiveness) program. Chapters were subject to multiple internal and external independent reviews.

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

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.

This report reviews the important role of medicines in health sytems, describes recent trends in pharmaceutical expenditure and financing, and summarises the approaches used by OECD countries to determine coverage and pricing.

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”.

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.

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.

Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Department of Health and Human Services Regulation) (HHS) (2018 Edition) The Law Library presents the complete text of the Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Department of Health and Human Services Regulation) (HHS) (2018 Edition). Updated as of May 29, 2018 HHS is issuing this final rule to clarify how section 340B(e) of the Public Health Service Act (PHSA) will be implemented. The final rule applies section 340B(e) of the PHSA only to drugs transferred, prescribed, sold, or otherwise used for the rare condition or disease for which the orphan drug was designated under section 526 of the Federal Food, Drug, and Cosmetic Act (FFDCA). The final rule also sets forth that it is the responsibility of the 340B covered entity to maintain auditable records that demonstrate compliance with the terms of the orphan drug exclusion requirements. This rule will provide clarity in the marketplace, maintain the 340B savings for newly-eligible covered entities, and protect the financial incentives for manufacturing orphan drugs designated for a rare disease or condition as indicated in the Affordable Care Act and intended by Congress. This book contains: - The complete text of the Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Department of Health and Human Services Regulation) (HHS) (2018 Edition) - A table of contents with the page number of each section

This report discusses the need for an integrated and cyclical approach to managing health technology in order to mitigate clinical and financial risks, and ensure acceptable value for money. The analysis considers how health systems and policy makers should adapt in terms of development, assessment and uptake of health technologies. The first chapter provides an examination of adoption and impact of medical technology in the past and how health systems are preparing for continuation of such trends in the future. Subsequent chapters examine the need to balance innovation, value, and access for pharmaceuticals and medical devices, respectively, followed by a consideration of their combined promise in the area of precision medicine. The final chapter examines how health systems can make better use of health data and digital technologies. The report focuses on opportunities linked to new and emerging technologies as well as current challenges faced by policy makers, and suggests a new governance framework to address these challenges.

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

"The inspiring memoir of a young doctor and former college athlete who became a champion for people suffering from rare, under-researched diseases--all while battling his own. A former Georgetown quarterback nicknamed "The Beast," David Fajgenbaum was also a force in medical school, where he was 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 over a condition they had yet to even diagnose; floating in and out of consciousness, Fajgenbaum prayed for the equivalent of game day overtime: a second chance. Miraculously, Fajgenbaum survived, but 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 disease. When he relapsed 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 proposed to align them himself. More than five years later and now married to his college sweetheart, his hard work has paid off: a treatment that 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 forces of determination, love, family, faith and serendipity collide"--

Rare diseases are a group of genetic disorders occurring in a small percentage of the population with the conditions being chronic but incurable. Approximately 7000 to 8000 different types have been identified and about 350 million people globally are affected in childhood and adulthood, resulting in enormous physical, mental, and psychological suffering and financial burden. It is imperative for medical scientists, clinicians, communities, and societies to ensure appropriate care is applied to ease the suffering of such patients. The

extraordinary and unprecedented hallmark in the field of rare diseases has revolutionized modern human medicine with exciting and advancing developments of the genomic era over the last two decades. Patients with rare diseases have been receiving increasing benefits in care and life quality improvements than ever before. This book intends to share and exchange the advancing knowledge and experiences from the authors, who have the necessary expertise within the various topics and subjects in the research, diagnosis, and management of rare diseases. It is hoped they are able to provide further benefits to patients and families with the development of early and accurate diagnosis and effective therapies.

NORD Guide to Rare Disorders is a comprehensive, practical, authoritative guide to the diagnosis and management of more than 800 rare diseases. The diseases are discussed in a uniform, easy-to-follow format--a brief description, signs and symptoms, etiology, related disorders, epidemiology, standard treatment, investigational treatment, resources, and references.The book includes a complete directory of orphan drugs, a full-color atlas of visual diagnostic signs, and a Master Resource List of support groups and helpful organizations. An index of symptoms and key words offers physicians valuable assistance in finding the information they need quickly.

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.

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.

Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Health Resources and Services Administration Regulation) (HRSA) (2018 Edition) The Law Library presents the complete text of the Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Health Resources and Services Administration Regulation) (HRSA) (2018 Edition). Updated as of May 29, 2018 HHS is issuing this final rule to clarify how section 340B(e) of the Public Health Service Act (PHSA) will be implemented. The final rule applies section 340B(e) of the PHSA only to drugs transferred, prescribed, sold, or otherwise used for the rare condition or disease for which the orphan drug was designated under section 526 of the Federal Food, Drug, and Cosmetic Act (FFDCA). The final rule also sets forth that it is the responsibility of the 340B covered entity to maintain auditable records that demonstrate compliance with the terms of the orphan drug exclusion requirements. This rule will provide clarity in the marketplace, maintain the 340B savings for newly-eligible covered entities, and protect the financial incentives for manufacturing orphan drugs designated for a rare disease or condition as indicated in the Affordable Care Act and intended by Congress. This book contains: - The complete text of the Exclusion of Orphan Drugs for Certain Covered Entities under 340B Program (US Health Resources and Services Administration Regulation) (HRSA) (2018 Edition) - A table of contents with the page number of each section

Publisher description

Orphan drugs are designated drug substances that are intended to treat rare or ‘orphan’ diseases. More than 7000 rare diseases are known that collectively affect some 6-7% of the developed world’s population; however, individually, any single, rare disease may only affect a handful of people making them commercially unattractive for the biopharmaceutical industry to target. Ground breaking legislation, starting with the Orphan Drug Act that was passed in the US in 1983 to provide financial incentives for companies to develop orphan drugs, has sparked ever increasing interest from biopharmaceutical companies to tackle rare diseases. These developments have made rare diseases, and the orphan drugs that treat them, sufficiently attractive to pharmaceutical development and many pharmaceutical companies now have research units dedicated to this area of research. It is therefore timely to review the area of orphan drugs and some

of the basic science, drug discovery and regulatory factors that underpin this important, and growing, area of biomedical research. Written by a combination of academic and industry experts working in the field, this text brings together expert authors in the regulatory, drug development, genetics, biochemistry, patient advocacy group, medicinal chemistry and commercial domains to create a unique and timely reference for all biomedical researchers interested in finding out more about orphan drugs and the rare diseases they treat. Providing an up-to-date monograph, this book covers the basic science, drug discovery and regulatory elements behind orphan drugs and will appeal to medicinal and pharmaceutical chemists, biochemists and anyone working within the fields of rare disease research and drug development or pharmaceuticals in industry or academia.

This text focuses on various factors associated with orphan diseases and the influence and role of health information technologies. Orphan diseases have not been adopted by the pharmaceutical industry because they provide little financial incentive to treat or prevent it. It is estimated that 6,000-7,000 orphan diseases exist today; as medical knowledge continues to expand, this number is likely to become much greater. The book highlights the opportunities and challenges in this increasingly important area. The book explores new avenues which are opened by information technologies and Health 2.0, and highlights also economic opportunities of orphan disease medicine. The editors of this new book have international experience and competencies in the key areas of patient empowerment, healthcare and clinical knowledge management, healthcare inequalities and disparities, rare diseases and patient advocacy.

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.

Searching for Magic Bullets reveals the quest of consumers, health professionals, and drug developers to find safer and faster methods of bringing new medications to the marketplace. Authors Basara and Montagne explore the current drug development and approval processes, their strengths and weaknesses, and the mechanisms by which patients and organizations evade these processes. Readers learn about the fundamentals of traditional and nontraditional drug discovery and development as they occur in the U.S., as well as the views of consumers, patients, and health professionals. Specific case studies of non-traditional drug development and acquisition strategies are highlighted, including AIDS medications, orphan drugs, and patient importation of medications. Basara and Montagne establish the differences in both knowledge and opinions of health consumers and health professionals regarding drug development, as well as how these differences often lead to frustration, dissatisfaction, and misappropriation of resources. The authors pinpoint the need for consumers and patients to know much more about the discovery and development of medicines, and for health professionals and students to understand patients’concerns, needs and beliefs, including their reasons for considering alternative methods of drug development and acquisition. Searching for Magic Bullets is a springboard from which consumers, health professionals, and students can discuss, debate, and resolve these issues and begin to develop more capable drug development and approval systems. This groundbreaking new book enlightens health professionals about patients’views regarding medication discovery and development and informs consumers and patients about the sometimes conflicting views

of health professionals. It is divided into three sections: drug development and approval in the U.S., a case study of orphan drugs, and risky and sometimes illegal ways in which consumers evade the traditional drug development and approval systems. An Overview of the Chapters: A Review of the Drug Development Process of the Pharmaceutical Industry: Presents the steps that must be taken when researching and developing a new medication. The Food and Drug Administration and the Drug Approval Process: Describes the history and scope of the FDA, the steps involved in acquiring drug approval, and the various stages of clinical testing. Orphan Drug Legislation: A review of the Orphan Drug Act of 1983 and the changes that have recently been proposed by Congress. The impact of the Act is highlighted through a description of products that have been made available since the legislation was enacted. Issues of controversy are also highlighted. Non-traditional Methods of Drug Development: The role of patients and consumers in drug development and evaluation is discussed, with an emphasis on the perceived shortcomings of the formal system. Patient Influence on Drug Development and Regulation: The influence of patient advocacy groups and consumers is discussed in relation to the development and approval of orphan drugs, the fast-tracking of specific medications, and the use of unapproved and alternative therapies. Prescription Drug Importation: Clarifies the current drug importation regulations, as well as provides specific directions for patients wishing to receive such products or learn more about FDA importation laws. The final chapter summarizes safe and rational techniques that empower consumers in their search for beneficial drug therapies. Resources and strategies for obtaining and using information are provided as a reference for readers. A glossary of terms, acronyms, and a directory of supplemental information sources strengthens the reader's understanding of the information presented. Who Benefits From This Book? Consumers and patients can use Searching for Magic Bullets as an accurate source of information about significant but often confusing medical issues. The FDA and the way medications are developed are easily misunderstood, while alternative therapies and medication sources are often believed to be the only options. Patients will learn the viewpoints of the pharmaceutical industry, the government, and their health care professionals; the rationale for various steps in the drug development process; the risks and benefits of participation in clinical trials; how to obtain the highest quality care, make informed health decisions, and reduce health care costs; and finally, how to cope with a rare disease and/or limited access to approved medications. The result is an informed, influential, and active patient. For health professionals, this book reviews the steps of drug development and approval and provides explanations for drug development decisions; drug approval time lag; and patient frustrations, misinterpretations, and expectations. It is critical for health professionals to understand the needs of patients and to determine how they can work with patients to find acceptable solutions. The literature references and medical information sources are invaluable in this regard. Pharmaceutical industry executives, product managers, clinical researchers, and sales representatives will find a concise and timely examination of the ways in which medications are discovered, developed, marketed, and used by patients. Discussions of orphan drug development, biotechnology products, and patient issues may also provide new insights into these often misunderstood areas. Pharmacy, medical, nursing, and other students will find this book a consolidated reference source and guidebook for information about the primary issues surrounding drug development and the FDA approval process. Patients’knowledge of alternative medical therapies will only increase and health care curricula must include material that helps students understand patients’perceptions of the medication development and approval systems, as well as the importance of patients in health care decisionmaking. The disadvantages of current drug development and approval systems are described with the hope that future health professionals can amend these processes and ultimately enhance patient care.

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.

There are 7,000 rare diseases affecting 6%–8% of the global population. That's 3.5 million people in the UK alone. Yet only 200 rare diseases have approved treatments. In recent years, there has been a surge of interest from business and social entrepreneurs in the field of health – including looking at ways to treat rare disease patients better and faster. This book presents some of the latest developments in the world of rare disease entrepreneurship from a global group of experts. It examines the topic from the business angle, considering the drug development process and providing case studies of successful orphan drug enterprises. It also looks at rare diseases from the perspective of the patient, analysing the growing rare disease patient movement, a successful patient group that uses social enterprise techniques, and chapters on key requirements for helping patients with rare diseases through registries and centres of excellence. The book will be an essential toolkit for social and business entrepreneurs who are interested in the world of rare/orphan diseases. It has the rigour of an academic publication, along with the clarity of a lay publication. An original and timely book, Rare Diseases will help to add knowledge and awareness to a vastly under-published subject.