GENE AND CELL THERAPY THERAPEUTIC MECHANISMS AND STRATEGIES SECOND EDITION REVISED AND EXPANDED FILE PDF

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Gene And Cell Therapy Therapeutic Mechanisms And Strategies Second Edition Revised And Expanded Introduction

Gene and Cell Therapy

This reference is completely revised and expanded to reflect the most critical studies, controversies, and technologies impacting the medical field, including probing research on lentivirus, gutless adenovirus, bacterial and baculovirus vectors, retargeted viral vectors, in vivo electroporation, in vitro and in vivo gene detection systems, and all inducible gene expression systems. Scrutinizing every tool, technology, and issue impacting the future of gene and cell research, it is specifically written and organized for laymen, scholars, and specialists from varying backgrounds and disciplines to understand the current status of gene and cell therapy and anticipate future developments in the field.

Gene and Cell Therapy

Since the publication of the second edition of this book in 2004, gene therapy and cell therapy clinical trials have yielded some remarkable successes and some disappointing failures. Now in its third edition, Gene and Cell Therapy: Therapeutic Mechanisms and Strategies assembles many of the new technical advances in gene delivery, clinical applications, and new approaches to the regulation and modification of gene expression. New Topics Covered in this Edition: Gene and Cell Therapies for Diabetes and Cardiovascular Diseases Clinical Trials Human Embryonic Stem Cells Tissue Engineering Combined with Cell Therapies Novel Polymers Relevant Nanotechnologies SiRNA Therapeutic Strategies Dendrimer Technologies Comprised of contributions from international experts, this book begins with a discussion of delivery systems and therapeutic strategies, exploring retroviral vectors and adenovirus vectors, as well as other therapeutic strategies. The middle section focuses on gene expression and detection, followed by an examination of various therapeutic strategies for individual diseases, including hematopoietic disorders, cardiovascular conditions, cancer, diabetes, cystic fibrosis, neurological disorders, and childhood-onset blindness. The final section discusses recent clinical trials and regulatory issues surrounding the new technology. This compendium is assembled by noted molecular biologist and biochemist Nancy Smyth Templeton. Baylor College of Medicine and several other institutions have used Dr. Templeton’s non-viral therapeutics in clinical trials for the treatment of lung, breast, head and neck, and pancreatic cancers, as well as Hepatitis B and C. She continues to work at the forefront of research in gene and cell therapies. Her contributions, as well as those contained in this volume, are sure to advance the state of the art of these revolutionary life-saving technologies.

Gene and Cell Therapy

The Most Comprehensive, State-of-the-Art Book on Using Gene and Cell Therapy in Clinical MedicineGene and Cell Therapy: Therapeutic Mechanisms and Strategies, Fourth Edition presents extensive background and basic information, state-of-the-art technologies, important achievements, and lingering challenges in the fields of gene and cell therapies. T

Gene Therapy

Second Generation Cell and Gene-Based Therapies: Biological Advances, Clinical Outcomes, and Strategies for Capitalisation serves as the only volume to the market to bridge basic science, clinical therapy, technology development, and business in the field of cellular therapy/cytotherapy. After more than two decades of painstaking fundamental research, the concept of therapeutic cells (stem cells, genes, etc.), beyond the concept of vaccines, is reaching clinical trial, with mounting confidence in the safety and efficacy of these products. Nonetheless, numerous incremental technical advances remain to be achieved. Thus, this volume highlights the possible R&D paths, which will ultimately facilitate clinical delivery of cutting edge curative products. The next waves of innovation are reviewed in depth for hematopoietic stem cells, mesenchymal stem cells, tissue engineering, CAR-T cells, and cells of the immune system, as well as for enabling technologies such as gene and genome editing. Additionally, deep dives in product fundamentals, history of science, pathobiology of diseases, scientific and technological bases, and financing and technology adoption constraints are taken to unravel what will shape the cytotherapy industry to the horizon 2025 and beyond. The outcome is not simply a scientific book, but a global perspective on the nascent field combining science, business, and strategic fundamentals. Helps readers learn about the most current trends in cell-based therapy, their overall effectiveness from a clinical prospective, and how the industry is moving therapies forward for capitalization \"Perspectives\" section at the end of each chapter summarizes key learnings, hypotheses, and objectives highlighted and combines scientific and business insights Edited and authored by scientists representing both basic and clinical research and industry, presenting a complete story of the current state and future promise of cellular therapies

Second Generation Cell and Gene-Based Therapies

Recent advances in stem cell biology, nanotechnology and gene therapy have opened new avenues for therapeutics. The availability of molecular therapeutics that rely on the delivery of DNA, RNA or proteins, harnessing enhanced delivery with nanoparticles, and the regenerative potential of stem cells (adult, embryonic or induced pluripotent stem cells) has had a tremendous impact on translational medicine. The chapters in this book cover a range of strategies for molecular and cellular therapies for human disease, their advantages, and central challenges to their widespread application. Potential solutions to these issues are also discussed in detail. Further, the book addresses numerous advances in the field of molecular therapeutics that will be of interest to the general scientific community. Lastly, the book provides specific examples of disease conditions for which these strategies have been transferred to the clinic. As such, it will be extremely useful for all students, researchers and clinicians working in the field of translational medicine and molecular therapeutics.

Gene Therapy

This is a reference handbook for young researchers exploring gene and cell therapy. Gene therapy could be defined as a set of strategies modifying gene expression or correcting mutant/defective genes through the administration of DNA (or RNA) to cells, in order to treat disease. Important advances like the discovery of RNA interference, the completion of the Human Genome project or the development of induced pluripotent stem cells (iPSc) and the basics of gene therapy are covered. This is a great book for students, teachers, biomedical researchers delving into gene/cell therapy or researchers borrowing skills from this scientific field.

Gene and Cell Therapy: Biology and Applications

This book offers an updated overview of the most recent research advances in the field, a comparison of established techniques and methods, a discussion on current experimental and translational challenges, and a commentary on potential opportunities and future directions. Dedicated chapters address and review the preclinical and clinical state-of-the-art of gene therapies for the reconstructive and regenerative surgery of skin and wounds, pathological scars, cartilage, tendons, skeletal muscles, and bio-engineered flaps. A brief guide to developing gene therapy clinical trials in the context of reconstructive and regenerative surgery is also provided. Biomedical and technological innovations are transforming our capacity to use gene therapies to safely and effectively repair, reconstruct, and regenerate tissues that are deficient or have been damaged by trauma and diseases. The targeted and controlled modulation of gene expression in tissues represents a game-changing, next-generation therapeutic tool for the modern reconstructive surgeon, expanding the horizon of regenerative surgery and tissue engineering. Through gene therapies, surgeons can direct (stem) cell differentiation and cell function, modulate the release of growth/transcriptional factors, affect the biological properties of regenerative scaffolds, control tissue inflammation, or induce immune-suppression in composite tissue allotransplants and xenotransplants. Written by renowned reconstructive surgeons and leading experts in each of these fields - from top academic institutions around the globe, the book provides an initial practical guide for veteran and newcomer surgeons alike, as well as for researchers interested in exploring the latest gene-based therapeutic strategies for reconstructive and regenerative surgery.

A Handbook of Gene and Cell Therapy

This third edition provides new and updated chapters on gene therapeutic strategies of cancer. Chapters guide readers through suicide and oncolytic gene therapy, gene replacement and gene suppression therapy, vector development and refinement, immunogene therapy, TCR and CAR engineering, tumor vaccination using DNA or RNA vaccines, and antitumoral immune stimulation at different levels. Written in the format of the highly successful Methods in Molecular Biology series, each chapter includes an introduction to the topic, lists necessary materials and reagents, includes tips on troubleshooting and known pitfalls, and step-by-step, readily reproducible protocols. Authoritative and cutting-edge, Gene Therapy of Cancer: Methods and Protocols, Third Edition aims to be a useful and practical guide to new researchers and experts looking to expand their knowledge.

Gene Therapy in Reconstructive and Regenerative Surgery

The Second Edition of Gene Therapy of Cancer provides crucial updates on the basic science and ongoing research in this field, examining the state of the art technology in gene therapy and its therapeutic applications to the treatment of cancer. The clinical chapters are improved to include new areas of research and more successful trials. Chapters emphasize the scientific basis of gene therapy using immune, oncogene, antisense, pro-drug activating, and drug resistance gene targets, while other chapters discuss therapeutic approaches and clinical applications. This book is a valuable reference for anyone needing to stay abreast of the latest advances in gene therapy treatment for cancer. Key Features * Provides in-depth description of targeted systems and treatment strategies * Explains the underlying cancer biology necessary for understanding a given therapeutic approach * Extensively covers immune therapeutics of vaccines, cytokines, and peptide-induced responses * Presents translational focus with emphasis on requirements for clinical implementation * Incorporates detailed illustrations of vectors and therapeutic approaches ideal for classroom presentations and general reference

Gene Therapy of Cancer

About 7 million people worldwide are suffering from various inherited neuromuscular diseases. Gene therapy brings the hope of treating these diseases at their genetic roots. Muscle Gene Therapy is the only book dedicated to this topic. The first edition was published in 2010 when the field was just about to enter its

prime time. The progress made since then has been unprecedented. The number of diseases that have been targeted by gene therapy has increased tremendously. The gene therapy toolbox is expanded greatly with many creative novel strategies (such as genome editing and therapy with disease-modifying genes). Most importantly, clinical benefits have begun to emerge in human patients. To reflect rapid advances in the field, we have compiled the second edition of Muscle Gene Therapy with contributions from experts that have conducted gene therapy studies either in animal models and/or in human patients. The new edition offers a much needed, up-to-date overview and perspective on the foundation and current status of neuromuscular disease gene therapy. It provides a framework to the development and regulatory approval of muscle gene therapy drugs in the upcoming years. This book is a must-have for anyone who is interested in neuromuscular disease gene therapy including those in the research arena (established investigators and trainees in the fields of clinical practice, veterinary medicine and basic biomedical sciences), funding and regulatory agencies, and patient community.

Gene Therapy of Cancer

Regenerative medicine – stem cell and gene-based therapy – offers a new approach for restoring function of damaged organs and tissues. This is the first book to cover the major new aspects and field of regenerative medicine. This title is therefore a timely addition to the literature. It brings together the major approaches to regenerative medicine in one text, which ensures that techniques learnt in one discipline are disseminated across other areas of medicine.

Muscle Gene Therapy

This unique advanced textbook provides a clear and comprehensive overview of gene delivery, gene therapy and genetic pharmacology, with descriptions of the main gene transfer vectors and a set of selected therapeutic applications, along with safety considerations. The second edition features new groundbreaking material on genome editing using the recently discovered CRISPR/Cas9 system and on cancer immunotherapy by CAR-T cells. It also presents the historical milestone of gene therapy application in the field of severe combined immunodeficiency, and other fields of gene therapy and molecular medicine.The use of gene transfer is exponentially growing in the scientific and medical communities for day-to-day cell biology experiments and swift development of gene therapy, which is already revolutionizing medicine. In this advanced textbook, more than 30 leading scientists come together to explore these topics.This educational introduction provides the background material needed to further explore the subject as well as relevant research literature. It is an invaluable resource to Master, PhD or MD students, post-doctoral scientists or medical doctors, as well as any scientist wishing to deliver a gene or synthetic nucleotide or develop a gene therapy strategy. The second edition's simple and synthetic content will be of value to any reader interested in the biological and medical revolution derived from the elucidation of the human genome.

Stem Cell and Gene-Based Therapy

This book discusses the different regulatory pathways for Advanced Therapy Medicinal Products implemented by national agencies in North and South America, Europe and Asia and by international bodies in the effort of international harmonization. This book represents an update of the first edition, as it covers regulatory novelties and accumulated experience in the regions already addressed. In addition, this new edition offers a wider international perspective: new chapters are included covering Advanced Therapy Medicinal Products regulations in India, Malaysia, Spain and Thailand, the European Pharmacopoeia texts for gene therapy medicinal products as well as international harmonization programs. Each chapter, authored by experts from various regulatory bodies throughout the international community, walks the reader through the applications of nonclinical research to translational clinical research to licensure and therapeutic use of these innovative products. More specifically, each chapter offers insights into fundamental considerations that are essential for developers of Advanced Therapy Medicinal Products in the areas of product quality, pharmacology and toxicology, clinical trial design and HTA pathways, as well as pertinent ‘must-know’

guidelines and regulations. Regulatory Aspects of Gene Therapy and Cell Therapy Products: a Global Perspective is part of the American Society of Gene & Cell Therapy sub-series of the highly successful Advances in Experimental Medicine and Biology series. It is essential reading for graduate students, clinicians, and researchers interested in gene and cell therapy and the regulation of pharmaceuticals.

Advanced Textbook On Gene Transfer, Gene Therapy And Genetic Pharmacology: Principles, Delivery And Pharmacological And Biomedical Applications Of Nucleotide-based Therapies (Second Edition)

Thoroughly updated to reflect major advances in the field of immuno-oncology, this second edition of Cancer Immunotherapy Principles and Practice, from the Society for Immunotherapy of Cancer (SITC), remains the definitive resource for information on tumor immunology and cancer immunotherapy treatments. An essential reference for both novice and experienced cancer researchers, oncologists, and related practitioners alike, the book not only guides readers through the fundamental scientific principles of the field all the way to translational and practical clinical applications for treating and managing oncologic disease, but also provides a comprehensive understanding of the regulatory processes that support the safe and effective delivery of immunotherapy to patients with cancer. The expanded and updated second edition now spans 68 chapters, including 12 new chapters, covering major topics and innovations that have shaped the rapid development of immunotherapy and its ascension into the standard of care as first-line treatment for a growing number of disease settings. New to this edition are chapters with deeper insight into our understanding of cancer genomics and determinants of response, immunogenic cell death, cancer and stromal cell-intrinsic pathways of immune resistance, cancer immune exclusion, adoptive cell therapy, metabolomics, tumor mutation burden, immunotherapy in combination with radiation therapy, synthetic biology, and more. Complete with detailed illustrations, tables, and key points for targeted reference, Cancer Immunotherapy Principles and Practice, Second Edition is the most comprehensive and authoritative resource for scientists and clinicians looking to expand their knowledge base of this dynamic field. Key Features: Offers key insights and perspectives on cancer immunology and immunotherapy treatments from renowned experts in the field Covers the basic principles and science behind cancer immunotherapy and tumor immunology Includes treatment strategies for a vast array of available immunotherapy classes and agents, such as cytokine therapies, oncolytic viruses, cancer vaccines, CAR T therapies, and combination immunotherapies Provides essential information on FDA-approved immunotherapies, including clinical management and outcome data related to response rates, risks, and toxicities Discusses special considerations for immunotherapy in the context of specific disease settings, including skin cancers, genitourinary cancers, gastrointestinal cancers, hepatocellular carcinomas, gynecologic malignancies, breast cancers, lung cancers, head and neck cancers, brain tumors, sarcomas, pediatric cancers, and treatments combined with radiation therapy Clarifies the complex regulatory aspects behind the development and approval of immunotherapy drugs

Regulatory Aspects of Gene Therapy and Cell Therapy Products

Hemoglobin defects, specifically sickle cell disease & thalassemia, combined, constitute the most common monogenic disorders in the world. In fact, nearly 2% of the world’s population carries a globin gene mutation. The transfer of the corrective globin gene through the HSC compartment by allogeneic HSC transplantation (HSCT) has already proven curative in both SCD and thalassemia patients, and provides the proof of concept that genetic manipulation of the defective organ might be equally therapeutic. However, procedural toxicities and the requirement of an HLA-matched sibling donor limit this approach to a fraction of affected individuals. The editors review the progress & the state of the field in HSCT for hemoglobinopathies & shed light on the major changes expected in the next decade. Although allogeneic HSCT is a curative option, it is limited by the availability of matched donors, which are often available only to 15-20% of patients. An alternative to allogeneic HS CT is genetic correction of autologous HSCs, to overcome donor availability & immune side effects. This Book reviews the progress made on additive gene therapy approaches & the current state of the field. Finally, targeted genetic correction is emerging as a novel

therapeutic strategy in the hemoglobinopathies. Although ideal, the inefficiency of targeted correction was rate limiting for translation of this technology to the clinic. With advancements in zinc finger nucleases and TALE endonuclease mediated targeted correction, correction frequencies in hematopoietic stem cells is now reaching levels that may become clinically relevant. Furthermore, the ability to generate autologous embryonic stem cell like cells from primary somatic cells (skin fibroblasts or hematopoietic cells) of the affected individual has allowed for the potential application of genetic correction strategies.This Book reviews upcoming genetic strategies to reactivate fetal hemoglobin production and research advances.

Cancer Immunotherapy Principles and Practice, Second Edition

A Roadmap to Non-hematopoietic Stem Cell-Based Therapeutics: From the Bench to the Clinic is a resource that provides an overview of the principles of stem cell therapy, the promises and challenges of using stem cells for treating various clinical conditions, and future perspectives. The overall goal is to facilitate the translation of basic research on stem cells to clinical applications. The properties of stem cells from various sources are reviewed and the advantages and disadvantages of each for clinical use are discussed. Modifying stem cell properties through preconditioning strategies using physical, chemical, genetic, and molecular manipulation to improve cell survival, increase cell differentiation potential, enhance production of paracrine factors, and facilitate homing to the site of injury or disease upon transplantation are reviewed. Various routes of stem cell administration and dosing, and the duration of effects, are explored. Individual chapters are written by experts in the field and focus on the use of stem cells in treating various degenerative diseases, autoimmune diseases, wound healing, cardiovascular disease, spinal cord injury, oral and dental diseases, and skeletal disorders. Finally, experts in the regulatory arena discuss mechanisms used in different countries for approving the use of stem cells to treat diseases and many common issues that are typically encountered while seeking approval for this class of therapeutic agent. Offers advanced students, as well as new researchers, an overview of the principles of stem cell therapy Discusses a wide array of pressing clinical issues with stem cell-based therapies so that new ideas in the laboratory can be efficiently translated to the clinic through better designed clinical trials Helps clarify current regulatory mechanisms so that the safe use of stem cells for treating a variety of diseases can move forward Fosters cross-disciplinary dialogue between research scientists and physicians to accelerate the safe implementation of efficacious cell therapies

Gene and Cell Therapies for Beta-Globinopathies

Recent advances in stem cell biology, nanotechnology and gene therapy have opened new avenues for therapeutics. The availability of molecular therapeutics that rely on the delivery of DNA, RNA or proteins, harnessing enhanced delivery with nanoparticles, and the regenerative potential of stem cells (adult, embryonic or induced pluripotent stem cells) has had a tremendous impact on translational medicine. The chapters in this book cover a range of strategies for molecular and cellular therapies for human disease, their advantages, and central challenges to their widespread application. Potential solutions to these issues are also discussed in detail. Further, the book addresses numerous advances in the field of molecular therapeutics that will be of interest to the general scientific community. Lastly, the book provides specific examples of disease conditions for which these strategies have been transferred to the clinic. As such, it will be extremely useful for all students, researchers and clinicians working in the field of translational medicine and molecular therapeutics.

A Roadmap to Nonhematopoietic Stem Cell-Based Therapeutics

Over the past decade, significant efforts have been made to develop stem cell-based therapies for difficult to treat diseases. Multipotent mesenchymal stromal cells, also referred to as mesenchymal stem cells (MSCs), appear to hold great promise in regards to a regenerative cell-based therapy for the treatment of these diseases. Currently, more than 200 clinical trials are underway worldwide exploring the use of MSCs for the treatment of a wide range of disorders including bone, cartilage and tendon damage, myocardial infarction, graft-versus-host disease, Crohn’s disease, diabetes, multiple sclerosis, critical limb ischemia and many

others. MSCs were first identified by Friendenstein and colleagues as an adherent stromal cell population within the bone marrow with the ability to form clonogenic colonies in vitro. In regards to the basic biology associated with MSCs, there has been tremendous progress towards understanding this cell population's phenotype and function from a range of tissue sources. Despite enormous progress and an overall increased understanding of MSCs at the molecular and cellular level, several critical questions remain to be answered in regards to the use of these cells in therapeutic applications. Clinically, both autologous and allogenic approaches for the transplantation of MSCs are being explored. Several of the processing steps needed for the clinical application of MSCs, including isolation from various tissues, scalable in vitro expansion, cell banking, dose preparation, quality control parameters, delivery methods and numerous others are being extensively studied. Despite a significant number of ongoing clinical trials, none of the current therapeutic approaches have, at this point, become a standard of care treatment. Although exceptionally promising, the clinical translation of MSC-based therapies is still a work in progress. The extensive number of ongoing clinical trials is expected to provide a clearer path forward for the realization and implementation of MSCs in regenerative medicine. Towards this end, reviews of current clinical trial results and discussions of relevant topics association with the clinical application of MSCs are compiled in this book from some of the leading researchers in this exciting and rapidly advancing field. Although not absolutely all-inclusive, we hope the chapters within this book can promote and enable a better understanding of the translation of MSCs from bench-to-bedside and inspire researchers to further explore this promising and quickly evolving field.

Gene and Cell Therapy

R.E. Nordon and K. Schindhelm, Introduction. -- L. Robb, A.G. Elefanty, and C.G. Begley, Transcriptional Control of Hematopoieses. -- R. Starr and N.A. Nicola, Cell Signaling by Hemopoietic Growth Factor Receptors. -- P.J. Simmons, D.N. Haylock, and J.-P. Lévesque, Influence of Cytokines and Adhesion Molecules on Hematopoietic Stem Cell Development. -- P.A. Rowlings, Allogeneic Hematopoietic Stem Cell Transplantation. -- U. Hahn and L.B. To, Autologous Stem Cell Transplantation. -- M.R. Vowels, Cord Blood Stem Cell Transplantation. -- S.R. Riddell, E.H. Warren, D. Lewinsohn, C. Yee, and P.D. Greenberg, Reconstitution of Immunity by Adoptive Immunotherapy with T Cells. -- L.Q. Sun, M. Miller, and G. Symonds, Exogenous Gene Transfer into Lymphoid and Hematopoietic Progenitor Cells. -- C. Dowding, T. Leemhuis, A. Jakubowski, and C. Reading, Process Development for Ex Vivo Cell Therapy. -- R.E. Nordon and K. Schindhelm, Cell Separation. -- P.W. Zandstra, C.J. Eaves, and J.M. Piret, Environ ...

Mesenchymal Stem Cell Therapy

This text covers all aspects of gene therapy, including basic principles, viral and nonviral delivery systems, targeted diseases, regulatory issues, and FDA requirements. It investigates genetic bullets to block HIV-1 replication and genetic guns to deliver antiviral agents.

Ex Vivo Cell Therapy

Biopharmaceuticals—Advances in Research and Application: 2013 Edition is a ScholarlyEditions™ book that delivers timely, authoritative, and comprehensive information about Enzyme Therapies. The editors have built Biopharmaceuticals—Advances in Research and Application: 2013 Edition on the vast information databases of ScholarlyNews.™ You can expect the information about Enzyme Therapies in this book to be deeper than what you can access anywhere else, as well as consistently reliable, authoritative, informed, and relevant. The content of Biopharmaceuticals—Advances in Research and Application: 2013 Edition has been produced by the world's leading scientists, engineers, analysts, research institutions, and companies. All of the content is from peer-reviewed sources, and all of it is written, assembled, and edited by the editors at ScholarlyEditions™ and available exclusively from us. You now have a source you can cite with authority, confidence, and credibility. More information is available at http://www.ScholarlyEditions.com/.

Gene Therapy

This book offers current data on delivery systems and therapeutic strategies, gene expression and detection, and disease targets and therapeutic strategies. It presents results and prospects in gene therapy clinical trials, other cell-based therapies and trials, and regulatory issues. Topics include optimization of non-viral gene therapeutics, gene therapy for cancer, and DNA vaccines.

Biopharmaceuticals—Advances in Research and Application: 2013 Edition

This unique advanced textbook provides a clear and comprehensive overview of gene delivery, gene therapy and genetic pharmacology, with descriptions of the main gene transfer vectors and a set of selected therapeutic applications, along with safety considerations. The second edition features new groundbreaking material on genome editing using the recently discovered CRISPR/Cas9 system and on cancer immunotherapy by CAR-T cells. It also presents the historical milestone of gene therapy application in the field of severe combined immunodeficiency, and other fields of gene therapy and molecular medicine. The use of gene transfer is exponentially growing in the scientific and medical communities for day-to-day cell biology experiments and swift development of gene therapy, which is already revolutionizing medicine. In this advanced textbook, more than 30 leading scientists come together to explore these topics. This educational introduction provides the background material needed to further explore the subject as well as relevant research literature. It is an invaluable resource to Master, PhD or MD students, post-doctoral scientists or medical doctors, as well as any scientist wishing to deliver a gene or synthetic nucleotide or develop a gene therapy strategy. The second edition's simple and synthetic content will be of value to any reader interested in the biological and medical revolution derived from the elucidation of the human genome.

Gene and Cell Therapy

Adenoviral Vectors for Gene Therapy, Second Edition provides detailed, comprehensive coverage of the gene delivery vehicles that are based on the adenovirus that is emerging as an important tool in gene therapy. These exciting new therapeutic agents have great potential for the treatment of disease, making gene therapy a fast-growing field for research. This book presents topics ranging from the basic biology of adenoviruses, through the construction and purification of adenoviral vectors, cutting-edge vectorology, and the use of adenoviral vectors in preclinical animal models, with final consideration of the regulatory issues surrounding human clinical gene therapy trials. This broad scope of information provides a solid overview of the field, allowing the reader to gain a complete understanding of the development and use of adenoviral vectors. Provides complete coverage of the basic biology of adenoviruses, as well as their construction, propagation, and purification of adenoviral vectors Introduces common strategies for the development of adenoviral vectors, along with cutting-edge methods for their improvement Demonstrates noninvasive imaging of adenovirus-mediated gene transfer Discusses utility of adenoviral vectors in animal disease models Considers Federal Drug Administration regulations for human clinical trials

Advanced Textbook on Gene Transfer, Gene Therapy and Genetic Pharmacology

This 21st Century Nanoscience Handbook will be the most comprehensive, up-to-date large reference work for the field of nanoscience. Handbook of Nanophysics by the same editor published in the fall of 2010 and was embraced as the first comprehensive reference to consider both fundamental and applied aspects of nanophysics. This follow-up project has been conceived as a necessary expansion and full update that considers the significant advances made in the field since 2010. It goes well beyond the physics as warranted by recent developments in the field. This eighth volume in a ten-volume set covers nanopharmaceuticals, nanomedicine, and food nanoscience. Key Features: Provides the most comprehensive, up-to-date large reference work for the field. Chapters written by international experts in the field. Emphasises presentation and real results and applications. This handbook distinguishes itself from other works by its breadth of coverage, readability and timely topics. The intended readership is very broad, from students and instructors

to engineers, physicists, chemists, biologists, biomedical researchers, industry professionals, governmental scientists, and others whose work is impacted by nanotechnology. It will be an indispensable resource in academic, government, and industry libraries worldwide. The fields impacted by nanophysics extend from materials science and engineering to biotechnology, biomedical engineering, medicine, electrical engineering, pharmaceutical science, computer technology, aerospace engineering, mechanical engineering, food science, and beyond.

Adenoviral Vectors for Gene Therapy

This book delivers a collection of organized and succinct reviews in the field of therapeutic genetic medicine presented by a carefully selected group of top experts. Each chapter focuses on a single, current topic and explains issues in the development of genetic treatments, critical challenges and strategies for implementing them, and future directions in research and translational applications. The book is complemented with uniformly designed illustrations and a supplementary Web site with a PowerPoint presentation for educators.

21st Century Nanoscience – A Handbook

This new book, from the editor of the highly successful Pharmaceutical Analysis, sets out to define the area of pharmaceutical chemistry as distinct from medicinal chemistry. It focuses less on prototypes of drugs that perhaps never came to market and more on the drugs currently in use. The emphasis in the book is on the physicochemical properties of drug molecules and, in so far as they are known, the way that these properties govern the interaction of the drug with its target. Important physicochemical properties include pKa and partition coefficient and the properties of the structural elements within the drug which provide interactions with the target via a range of intermolecular forces. The last fifteen years has seen a great advance in the knowledge of protein structures and a strong emphasis is given to the interaction of drugs with proteins which shape the majority of drug mechanisms. Features: Focus on intramolecular actions Mechanisms of action richly illustrated Self-assessment included Comprehensive chapters on vitamins and biotechnological products This new book, from the editor of the highly successful Pharmaceutical Analysis, sets out to define the area of pharmaceutical chemistry as distinct from medicinal chemistry. It focuses less on prototypes of drugs that perhaps never came to market and more on the drugs currently in use. The emphasis in the book is on the physicochemical properties of drug molecules and, in so far as they are known, the way that these properties govern the interaction of the drug with its target. Important physicochemical properties include pKa and partition coefficient and the properties of the structural elements within the drug which provide interactions with the target via a range of intermolecular forces. The last fifteen years has seen a great advance in the knowledge of protein structures and a strong emphasis is given to the interaction of drugs with proteins which shape the majority of drug mechanisms. Features: Focus on intramolecular actions Mechanisms of action richly illustrated Self-assessment included Comprehensive chapters on vitamins and biotechnological products

Concepts in Genetic Medicine

The three sections of this volume present currently available cancer gene therapy techniques. Part I describes the various aspects of gene delivery. In Part II, the contributors discuss strategies and targets for the treatment of cancer. Finally, in Part III, experts discuss the difficulties inherent in bringing gene therapy treatment for cancer to the clinic. This book will prove valuable as the volume of preclinical and clinical data continues to increase.

Pluripotent Cells for Stroke: from Mechanism to Therapeutic Strategies

Lung cancer is the leading cause of cancer related mortality in Canada and USA. Majority of the patients present in advanced stage of the disease and of these only about 2% will be alive at 5 years. NSCLC is the most common form of lung cancer, accounting for approximately 87% of cases. Systemic chemotherapies

have been used to treat metastatic NSCLC for decades, but the improvements of outcomes have reached a plateau. Recent advances in understanding signalling pathways for malignant cells, their interconections,the importance of various receptors and biomarkers and the interplay between various oncogenes have led to the development of targeted treatments that are improving both efficacy and safety of the treatments. Knowledge about the advantages of treatments with the targeted agents in metastatic NSCLC is growing rapidly. Combining various targeted agents or sequencing them properly will be important in the era of personalised medicine and overcoming development of the resistence to various targeted agents will be challenging. The importance of a team work,from the diagnosis through various treatments, to supportive care, from the interventional radiologists, pneumologists or surgeons, who have to obtain a satisfactory tumor tissue specimen, to pathologists, radiation and medical oncologists, to supportive care specialists, will be described in our publications. We will cover completely present and future approaches to personalised medicine in this rapidly evolving field of metastatic NSCLC.

Pharmaceutical Chemistry E-Book

Basic scientific background Breast cancer is one of the most common cancer and the most frequent cause of cancer death among women worldwide. Currently, subtyping breast cancers into hormone receptor (HR) positive, human epidermal growth factor receptor-2 overexpressing (HER2+), and triple negative breast cancer (TNBC) is the basis of diagnosing and treating this disease. The main treatment strategies for breast cancer include surgery, endocrine therapy, molecular targeted therapy, chemotherapy, radiotherapy, immunotherapy and gene therapy. However, resistance of breast cancer cells to chemotherapeutic agents, molecular targeted therapies and immunotherapy may occur either intrinsically or de nova, and is often ultimately responsible for treatment failure. Therefore, drug resistance poses a major challenge to breast cancer treatment. Current developments: Drug resistance in breast cancer is a complex clinical condition originating from a wide range of molecular alterations. The development of endocrine therapy resistance is believed to be associated with many cellular changes, such as ESR1 gene mutations, bypassing estrogen signaling pathway and altered tamoxifen metabolism. Meanwhile, changes in immune response, alternation of drug-binding property and downstream pathways are involved in the mechanisms of drug resistance in HER2+ breast cancer. In addition, resistance to chemotherapeutic agents predominantly arises from increased drug efflux and cross resistance. Current studies suggest that treatment strategies and therapeutics have to be designed specifically to each patient in different clinical situations. The use of modern genomic, proteomic and functional analytical techniques has contributed to identify novel genes and signaling networks involved in breast cancer drug resistance. Moreover, the use of high-throughput techniques in combination with bioinformatics and systems biology approaches has aided the interrogation of clinical samples and allowed the identification of molecular signatures and genotypes that predict responses to certain drugs. Despite much progress has been made in the field of breast cancer drug resistance, such as combination therapy and drug-loaded nanoparticles, the complexity and variability of drug resistance mechanism still inevitably lead to the continuous occurrence of drug resistance. Therefore, with the increasing amounts of anti-breast cancer agents, there are now unprecedented opportunities to understand and overcome drug resistance through further research into mechanisms and corresponding strategies, which will help achieve lasting disease control and bring survival benefits to patients with advanced cancer. Papers of interest: The current Research Topic of Frontiers in Pharmacology focuses on publishing Original Research, Review articles and Case Reports focusing on (a) elucidating mechanisms of drug resistance in breast cancer, target mutations, tumor microenvironment, undiscovered genes and signaling pathways; (b) promising drug delivery systems that can enhance the sensitivity of anti- breast cancer agents to various tumors; (c) strategies that can improve patient care during bio-chemotherapeutic treatments; (d) small molecule compounds that are effective against drug-resistant breast tumors (e) biomarkers of chemotherapy resistance in breast cancer patients and (f) in vitro and in vivo models. Guidelines for article of submission: - Authors must stick to the set guidelines for ethical practices by the Frontiers journals. - The main content of the article must have certain innovation and research significance. - The authors should describe the construction method of drug-resistant cell lines when using them for experiments in the article.

Ferroptosis in malignant brain tumors

Recognition and killing of aberrant, infected or tumor targets by Natural Killer (NK) cells is mediated by positive signals transduced by activating receptors upon engagement of ligands on target surface. These stimulatory pathways are counterbalanced by inhibitory receptors that raise NK cell activation threshold through negative antagonist signals. While regulatory effects are necessary for physiologic control of autoimmune aggression, they may restrain the ability of NK cells to activate against disease. Overcoming this barrier to immune surveillance, multiple approaches to enhance NK-mediated responses are being investigated since two decades. Propelled by considerable advances in the understanding of NK cell biology, these studies are critical for effective translation of NK-based immunotherapy principles into the clinic. In humans, dominant inhibitory signals are transduced by Killer Immunoglobulin Like Receptors (KIR) recognizing cognate HLA class I on target cells. Conversely, KIR recognition of “missing self-HLA” - due to HLA loss or HLA/ KIR mismatch - triggers NK-mediated tumor rejection. Initially observed in murine transplant models, these antitumor effects were later found to have important implications for the clinical outcome of haplotype-mismatched stemcell transplantation. Here, donor NK subsets protect against acute myeloid leukemia (AML) relapse through missing self recognition of donor HLA-C allele groups (C1 or C2) and/or Bw4 epitope. These studies were subsequently extended by trials investigating the antileukemia effects of adoptively transferred haplotype-mismatched NK cells in non-transplant settings. Other mechanisms have been found to induce clinically relevant NK cell alloreactivity in transplantation, e.g., post-reconstitution functional reversal of anergic NK cells. More recently, activating KIR came into the spotlight for their potential ability to directly activate donor NK cells through in vivo recognition of HLA or other ligands. Novel therapeutic monoclonal antibodies (mAb) may optimize NK-mediated effects. Examples include obinutuzumab (GA101), a glyco-engineered anti-CD20 mAb with increased affinity for the Fc?RIIIA receptor, enhancing antibody-dependent cellular cytotoxicity; lirilumab (IPH2102), a first-in-class NK-specific checkpoint inhibitor, blocking the interaction between the major KIR and cognate HLA-C antigens; and elotuzumab (HuLuc63), a humanized monoclonal antibody specific for SLAMF7, whose anti-myeloma therapeutic effects are partly due to direct activation of SLAMF7-expressing NK cells. In addition to conventional antibodies, NK cell-targeted bispecific (BiKEs) and trispecific (TriKEs) killer engagers have also been developed. These proteins elicit potent effector functions by binding target ligands (e.g., CD19, CD22, CD30, CD133, HLA class II, EGFR) on one arm and NK receptors on the other. An additional innovative approach to direct NK cell activity is genetic reprogramming with chimeric antigen receptors (CAR). To date, primary NK cells and the NK92 cell line have been engineered with CAR specific for antigens expressed on multiple tumors. Encouraging preclinical results warrant further development of this approach. This Research Topic welcomes contributions addressing mechanisms of NK-mediated activation in response to disease as well as past and contemporary strategies to enhance NK mediated reactivity through control of the interactions between NK receptors and their ligands.

Gene Therapy for Cancer

Virtually any disease that results from malfunctioning, damaged, or failing tissues may be potentially cured through regenerative medicine therapies, by either regenerating the damaged tissues in vivo, or by growing the tissues and organs in vitro and implanting them into the patient. Principles of Regenerative Medicine discusses the latest advances in technology and medicine for replacing tissues and organs damaged by disease and of developing therapies for previously untreatable conditions, such as diabetes, heart disease, liver disease, and renal failure. Key for all researchers and instituions in Stem Cell Biology, Bioengineering, and Developmental Biology The first of its kind to offer an advanced understanding of the latest technologies in regenerative medicine New discoveries from leading researchers on restoration of diseased tissues and organs

The Treatment of Metastatic Non-small Cell Lung Cancer (NSCLC) in New Era of Personalised Medicine

Molecular Biology of B Cells, Second Edition is a comprehensive reference to how B cells are generated, selected, activated and engaged in antibody production. All of these developmental and stimulatory processes are described in molecular, immunological, and genetic terms to give a clear understanding of complex phenotypes. Molecular Biology of B Cells, Second Edition offers an integrated view of all aspects of B cells to produce a normal immune response as a constant, and the molecular basis of numerous diseases due to B cell abnormality. The new edition continues its success with updated research on microRNAs in B cell development and immunity, new developments in understanding lymphoma biology, and therapeutic targeting of B cells for clinical application. With updated research and continued comprehensive coverage of all aspects of B cell biology, Molecular Biology of B Cells, Second Edition is the definitive resource, vital for researchers across molecular biology, immunology and genetics. Covers signaling mechanisms regulating B cell differentiation Provides information on the development of therapeutics using monoclonal antibodies and clinical application of Ab Contains studies on B cell tumors from various stages of B lymphocytes Offers an integrated view of all aspects of B cells to produce a normal immune response

Molecular Mechanisms of Drug Resistance And Strategies of Sensitization in Breast Cancer, 2nd edition

As human gene therapy becomes a clinical reality, a new era in medicine dawns. Novel and innovative developments in molecular genetics now provide opportunities to treat the genetic bases of diseases often untreatable before. Somatic Gene Therapy documents these historical clinical trials, reviews current advances in the field, evaluates the use of the many different cell types and organs amenable to gene transfer, and examines the prospects of various exciting strategies for gene therapy.

Kr?tika chronika

Tailoring NK Cell Receptor-Ligand Interactions: an Art in Evolution, 2nd Edition