

CRISPR targeting of viral genes

CAR-T cells Engineering the production of neutralizing antibodies

Gene Vaccination

Adrian Bot, Mihail Obrocea, Francesco M. Marincola

Gene Vaccination:

Gene Vaccination: Theory and Practice Eyal Raz, 2012-12-06 Vaccination has been established as an efficient procedure to prevent infections Over the past few years a new method of subunit vaccination has attracted the attention of immunologists Despite its popularity it is only recently that the basic mechanisms that drive the immune response to the encoded antigen have begun to unfold The multidisciplinary approach of this book outlines the basic characteristics of gene DNA vaccination the role of APCs or bone marrow derived cells in the induction of the immune response It points out the potential applications for various infectious and allergic diseases and describes the multifaceted properties of DNA in initiating and determining the subsequent immune responses to the encoded antigen Gene Vaccines Josef Thalhamer, Richard Weiss, Sandra Scheiblhofer, 2011-08-31 The induction of antigen specific immune responses after in vivo transfection with expression plasmids has triggered a revolution of vaccine research After a first hype evoked by the fascinating options of this method clinical studies did not reach the ambitious aims and a phase of disillusion ensued It became obvious that Gene vaccines displayed a weaker immunogenicity in humans than had been observed in the mouse models Meanwhile these hurdles have been overcome and gene vaccines undergo a renaissance The present book gives an update of the world of naked gene vaccines namely DNA and RNA vaccines Its content ranges from general mechanisms inherent immunostimulatory properties and the vast potential to modulate immune responses to recent successful clinical studies and approved veterinary gene vaccines Beyond the state of the art of genetic immunization the reader will be **DNA Vaccination/Genetic Vaccination** Hilary stimulated with a chapter addressing burning questions Koprowski, David B. Weiner, 2012-12-06 Genetic DNA immunization represents a novel approach to vaccine and immune therapeutic development The direct injection of nucleic acid expression cassettes into a living host results in a limited number of its cells becoming factories for production of the introduced gene products. This host inappropriate gene expression has important immunological consequences resulting in the specific immune activation of the host against the gene delivered antigen The recent demonstration by a number of laboratories that the induced immune responses are functional in experimental models against both specific infectious diseases and cancers is likely to have dramatic consequences for the develop ment of a new generation of experimental vaccines and immune therapies This technology has the potential to enable the pro duction of vaccines and immune based therapies that are not only effective immunologically but are accessible to the entire world rather than just to the most developed nations Vaccine Development Vaccination against pathogenic microorganisms represents one of the most important advances in the history of medicine Vaccines including those against polio measles mumps rubella hepatitis A hepatitis B pertussis and other diseases have dramatically improved and protected more human lives than any other avenue of modern medicine The vaccine against smallpox for example has been so successful that it is now widely believed that this malicious killer responsible for more deaths in the

twentieth century than World Wars I and II combined has been removed from the face of the earth Genetic **Immunization** Adrian Bot, Constantin A. Bona, 2013-03-14 Wolf's discovery demonstrating that a reporter gene is expressed in myocytes subsequent to injection of naked DNA was exploited by immunologists and vaccinologists to develop a new generation of vaccines This observation galvanized the research and in a short lapse of time an oceanic volume of knowledge has been accumulated The research carried out in a variety of animal models showed the efficacy of genetic immunization against viruses bacteria and some parasites by the ability to induce a strong priming effect resulting from long lasting persistence of plasmid as episomes Furthermore it was demonstrated that newborn or infant immune unresponsiveness to classical vaccines can be corrected by genetic immunization. The applications of genetic immunization for prophylaxis of infections was extended to immunotherapy namely cancerous auto immune and allergic diseases Immunologists have provided pertinent information on the cellular basis of the immune responses elicited by genetic immunization and molecular biologists have established the molecular basis of intrinsic adjuvant properties of plasmids **Nonviral Vectors for Gene Therapy, Part 2** Leaf Huang, Mien-Chie Hung, Ernst Wagner, 2005-08-01 The field of non viral vector research has rapidly progressed since the publication of the first edition This new edition is expanded to two separate volumes that contain in depth discussions of different non viral approaches including cationic liposomes and polymers naked DNA and various physical methods of delivery as well as a comprehensive coverage of the molecular biological designs of the plasmid DNA for reduced toxicity prolonged expression and tissue or disease specific genes New developments such as the toxicity of the non viral vectors and recent advances in nucleic acid therapeutics are fully covered in these volumes Nonviral Vectors for **Gene Therapy** Mien-Chie Hung, Leaf Huang, Ernst Wagner, 1999-07-01 Gene transfer within humans has been an obstacle until about 10 years ago At that time it was found that viral vectors were effective carriers of healthy genes into patients cells The problem however was that viral vectors proved unnecessarily harmful to humans subjects experienced inflamatory activity and negative immunological responses to the genes Viral vectors were also unable to meet the needs of the pharmaceutical community they were not reproducible in large scale proportions in cost effective ways Thus research was undertaken to find a safer way to transfer genes to patients without jeopardizing the safety of the patient And so non viral vectors were discovered This volume presents the various non viral vectors currently under development Although not methodologically oriented it will provide the necessary details behind the development of the vectors This information will prove useful to both researchers and clinicians Key Features Presents state of the art developments of nonviral vectors as tools for modern molecular medicine Covers all types of nonviral vectors from molecular structure to therapeutic applicationProvides a comprehensive review of synthetic vectors Includes contributions from major investigators and leading experts in the field **Advanced Gene Delivery** Alain Rolland, 2003-09-02 A practical resource for everyone involved in the gene therapy field and in the design of effective gene delivery systems this volume presents an overview and update of recent

advances in the field of non viral methods for the in vivo transfer of therapeutic genes to biological targets using conventional routes of administration Methods to control the spatial and temporal modulation of gene function in vivo as well as the level duration specificity and fidelity of gene expression are described. The rational design and the applications of a variety of non viral gene delivery systems such as cationic lipid polymer and poly peptide based systems are exemplified for the control of location of the appearing genes administered by various routes Current and potential clinical applications of gene based medicines are presented for the prevention correction or modulation of diseases Examples of current applications of plasmid based systems for genetic vaccination treatment of genetic disorders such as cystic fibrosis and treatment of acquired diseases such as cancer are also provided Biologic and Gene Therapy of Autoimmune Disease C. G. Fathman, A. N. Theofilopoulos, 2000-07 The clinical management of autoimmune diseases has proven to be extremely difficult Current therapies focus on trying to alleviate symptoms but fail to correct the fundamental immune defects that lead to pathology To achieve this goal it is necessary to understand much of the biology of antigen presentation lymphocyte activation and the effects of cytokines The articles in this book provide an up to date review of current innovative therapies using both biologic and gene therapy for the treatment of selected autoimmune diseases Therapeutical approaches discussed include oral tolerance the use of anti CD4 monoclonal antibodies IL 10 and anti TNFa antibodies DNA vaccination and gene therapy applied to organ specific autoimmune disease Although some of these techniques are still in their infancy their potential efficacy has been demonstrated in several animal models of autoimmune disease holding great promise for the future development of treatments Written by recognized experts in the field the chapters in this book illustrate the concept of technology transfer from bench to bedside and provide a valuable update for clinicians and scientists in clinical immunology

Gene and Cell Therapy Nancy Smyth Templeton,2003-12-17 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 Plasmids for Therapy and Vaccination Martin Schleef,2008-07-11 This is the first book specializing in plasmids and their biomedical use including all relevant aspects of production applications quality and regulations Readers will discover clinical applications for the wide range of preventive and therapeutic applications using plasmid DNA The book describes modified vector systems based on plasmids as well as the potency of genomic research and vector design by informatics Using the example of fish vaccination the application of DNA vaccination in veterinary health care is reviewed followed by a detailed overview of plasmid production technology on an industrial scale Finally the book considers regulatory

and quality assurance aspects of such new drugs plus thire market potential **Gene Therapy in Inflammatory Diseases** C. H. Evans, 2000-02-14 Gene therapy for inflammatory diseases is a new burgeoning field of medicine Edited by the undisputed pioneers of this area of research this volume is the first devoted to its topic It contains thirteen chapters each written by leaders in their respective fields that summarize the state of the art in developing novel gene based treatments for inflammatory diseases As well as providing an introduction to the basic concepts of gene therapy and the use of naked DNA approaches the book describes the advances that have been made in applying them to arthritis lupus multiple sclerosis diabetes Sjogren s syndrome and transplantation One chapter is devoted to discussing the first human clinical trials that apply gene therapy to the treatment of an inflammatory disease As well as providing novel therapeutic approaches gene therapy facilitates the development of new and improved animal models of disease a chapter describing these advances is also included As an up to date timely book written by th **Gene Therapy of Cancer** Stanton L. Gerson, Edmund C. Lattime, 2002-04-04 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 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 The Skin and Gene Therapy Ulrich R. Hengge, Beatrix Volc-Platzer, 2001 Basic Aspects 1 The Epidermal Barrier and Strategies for Surmounting It An Overview 2 Stem Cells Differentiation and Renewal Kinetics of Keratinocytes Implications for Cutaneous Gene Therapy 3 Relevant Animal Models for Skin Gene Therapy 4 Nonviral Gene Transfer into the Skin 5 Safety and Pharmacokinetics of Naked Plasmid DNA Studies on Dissemination and Ectopic Expression 6 Uptake of DNA by Keratinocytes Treatment of Skin Diseases Gene Therapy of Inherited Skin Diseases Gene Transfer Strategies in Tissue Repair Systemic Effects of Skin Gene Therapy The Use of Skin Directed Gene Therapy in the Treatment of Systemic Diseases Keratinocyte Gene Therapy Using Cytokine Genes Genetic Vaccination Using the Skin Principles of Genetic Immunization Systematic Modulation of Immune Responses by CpG DNA Genetic and Dendritic Cell Vaccination as a Novel Therapy for Melanoma Molecular Strategies Interfering with Tumor Progression of Melanoma and Improving Anti Tumor Immunity Prophylactic and Therapeutic DNA Vaccines Against Infectious Diseases Gene Therapy of Cancer Wolfgang Walther, Ulrike Stein, 2008-02-01 Since the

discovery of the molecular structure of genes and the unveiling of the molecular basis of numerous human diseases scientists have been fas nated with the possibility of treating certain diseases by transducing foreign DNA into the affected cells Initially it was proposed that the foreign DNA could either replace defective nonfunctional genes or code for therapeutic proteins This concept has evolved into the rapidly growing field of gene therapy Even though surgery radiotherapy and chemotherapy are widely ava able and routinely used for cancer treatment these therapies fail to cure approximately 50 percent of cancer patients Therefore since it is a disease characterized by aberrant gene expression cancer has been a target of gene therapy research since the inception of this treatment modality Numerous cancer gene therapy strategies are currently being investigated including gene replacement therapy the regulation of gene expression to modulate immu logical responses to tumors the direct killing of tumor cells and direct int ference with tumor growth In this context gene transfer systems tumor specific expression vectors and novel therapeutic genes have been extensively stied All these strategies aim for the selective destruction of human malignant disease while circumventing the destruction of nonmalignant cells and tissues thereby minimizing toxicity to the patient Therapeutic Vaccination Strategies P. Walden, W. Sterry, H. Hennekes, 2013-03-14 The induction of immune responses against tumor cells by vaccination is rapidly evolving as a therapeutic modality with new potentials for the treatment of cancer It is based on the fact that our immune system can identify tumor cells and once activated is capable of developing specific immunity against the neoplastic cells Numerous observations and intense research clearly document the major contribution of the immune system to the prevention of cancer And there are many reports of patients suffering from malignant melanoma or other tumors who mount a spontaneous immune response against their tumor cells that results in tumor regression Based on the recent advances in our understanding of the compo nents of our immune system their interactions and the regulation of immune responses we are now able to design vaccination strategies that induce or enhance cell mediated immunity against tumors A ma jor advancement came with the identification and characterization of relevant tumor antigens which are suitable target structures for anti tu mor immune response First clinical trials using such vaccine strategies have yielded encouraging results in patients However in spite of many reported cases of successful therapy of cancer by vaccination many patients still do not experience relief after such treatments These initial clinical trials and the accompanying investigations have revealed a number of important results that indicate the direction of future re search and development in the field Molecular <u>Vaccines</u> Matthias Giese, 2013-11-08 This title discusses all aspects of non infectious and non cancer so called NINC vaccines Hypertension diabetes and allergy vaccine development are referred to as well as the use of adjuvants and nanotechnology in vaccine development The way of novel vaccines from bench to preclinical to clinical studies and launch to the market under EMEA European Medicines Agency and FDA Food and Drug Administration guidelines are described in depth The book is therefore of interest for researchers and clinicians engaged in vaccine development and molecular vaccine application

Advanced Vaccination Technologies for Infectious and Chronic Diseases Vasso Apostolopoulos, Lalitkumar K. Vora, Vivek P. Chavda, 2024-03-25 The role of vaccines is emerging and even critical to ending infectious and chronic diseases and pandemics alike The design and development of new vaccines could lead to improved health Handbook on Advanced Vaccination Technologies for Infectious and Chronic Disease discusses these new developments and introduces the reader to the current state of the science and the outlook going forward from the discovery of vaccines to the clinical trials of personalized vaccines Handbook on Advanced Vaccination Technologies for Infectious and Chronic Diseases is a valuable reference for occupational health professionals whose role involves supervision of immunization programs such as those working in the National Health Service some sectors of higher education and the pharmaceutical industry Offers comprehensive coverage of different vaccine platforms and their development Includes information on the regulatory perspective of vaccine development Describes different delivery approaches for vaccinology Explains the clinical development of vaccines along with novel platforms Covers all recent developments of vaccine production technologies new types of vaccines and ongoing research that could prevent future pandemics Cancer Vaccines Adrian Bot, Mihail Obrocea, Francesco M. Marincola, 2015-05-06 Recent advances in immunology and biology have opened new horizons in cancer therapy included in the expanding array of cancer treatment options which are immunotherapies or cancer vaccines for both solid and blood borne cancers Cancer Vaccines From Research to Clinical Practice is the first text in the field to bring immunotherapy treatments Rosenberg's Molecular and Genetic Basis of Neurological and Psychiatric Disease Roger N. Rosenberg, Juan M. Pascual, 2020-06-24 Rosenberg's Molecular and Genetic Basis of Neurologic and Psychiatric Disease Sixth Edition Volume Two provides a comprehensive introduction and reference to the foundations and practical aspects relevant to the majority of neurologic and psychiatric disease This updated volume focuses on degenerative disorders movement disorders neuro oncology neurocutaneous disorders epilepsy white matter diseases neuropathies and neuronopathies muscle and neuromuscular junction disorders stroke psychiatric disease and a neurologic gene map A favorite of over three generations of students clinicians and scholars this new edition retains and expands on the informative concise and critical tone of the first edition This is an essential reference for general medical practitioners neurologists psychiatrists geneticists related professionals and for the neuroscience and neurology research community at large The content covers all aspects essential to the practice of neurogenetics to inform clinical diagnosis treatment and genetic counseling Provides comprehensive coverage on the neurogenetic foundation of neurological and psychiatric disease Presents detailed coverage of genomics animal models and diagnostic methods with new coverage on evaluating patients with biochemical abnormalities or gene mutations Includes new chapters on the pharmacogenomics of epilepsy and the most recent updates in molecular genetics focusing on neurodegenerative and psychiatric diseases *DNA Vaccines* Douglas B. Lowrie, Robert Whalen, 2008-02-01 The field of DNA vaccines has undergone explosive growth in the last few years As usual

some historical precursors of this approach can be d cerned in the scientific literature of the last decades However the present state of affairs appears to date from observations made discreetly in 1988 by Wolff Malone Felgner and colleagues which were described in a 1989 patent and published in 1990 Quite surprisingly they showed that genes carried by pure plasmid DNA and injected in a saline solution hence the epithet naked DNA could be taken up and expressed by skeletal muscle cells with a low but reproducible frequency Such a simple methodology was sure to spawn many applications In a separate and important line of experimentation Tang De Vit and Johnston announced in 1992 that it was indeed possible to obtain humoral immune responses against proteins encoded by DNA delivered to the skin by a biolistic device which has colloquially become known as the gene gun The year 1993 saw the publication of further improvements in the me ods of naked DNA delivery and above all the first demonstrations by several groups of the induction of humoral and cytotoxic immune responses to viral antigens expressed from injected plasmid DNA In some cases protection against challenge with the pathogen was obtained The latter result was questionably the touchstone of a method of vaccination worthy of the name

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