

Recent Advances in the Cellular and Molecular Understanding of Myelodysplastic Syndromes: Implications for New Therapeutic Approaches

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Abstract: It has been more than 10 years since any new disease-modifying therapies have received regulatory approval for indications related to myelodysplastic syndromes (MDS). Advances in our collective biological understanding of MDS in the last decade, however, have made it possible to hope that effective therapeutics can be designed to improve MDS-associated cytopenias and patients' quality of life, and perhaps even delay clonal progression and extend survival. Classes of MDS-associated mutations and disordered biological pathways targeted by developmental therapeutics include the following: aberrant messenger RNA splicing, neomorphic enzymes in the citric acid cycle with oncogenic activity, overactivated tyrosine and serine-threonine kinases, epigenetic and chromatin remodeling alterations, abnormal telomere dynamics, and failed protection of DNA integrity. At present, treatments for MDS are usually administered as sequential monotherapy, but there is a trend toward clinical trials of combination therapies—in which new agents are added to a DNA hypomethylating agent backbone—for both upfront treatment and the treatment of relapsed/refractory disease. Agents in clinical trials for subsets of MDS include luspatercept, antibodies targeting CD33, isocitrate dehydrogenase inhibitors, deacetylase inhibitors, venetoclax, and immunotherapies designed to overcome immune checkpoint inhibition. These biologically based therapeutics, as well as the encouraging precedent of 7 new approvals by the US Food and Drug Administration in 2017 for the treatment of acute leukemia, offer the prospect that 10 more years will not elapse before another new therapy is approved for MDS.

Introduction and Epidemiology of Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) encompass a spectrum of bone marrow malignancies characterized by cytopenias and ineffective clonal hematopoiesis, as well as a risk for evolution to acute myeloid leukemia (AML) in up to 30% of cases. It has long been recognized

Key messages

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Recent Advances And New Syndromes

Ravi Fotedar, Bruce Phillips



Recent Advances And New Syndromes:

Recent Advances and New Species in Aquaculture Ravi Fotedar, Bruce Phillips, 2011-07-07 This comprehensive up to date text delivers the latest must have information on species new to aquaculture and documents the most important technological innovations of the past decade Every aspect of the growing field has been addressed with coverage spanning recent technological development new species recent changes and global trends More specifically you will find information on the culture of species such as barramundi cobia dolphin fish spiny lobsters slipper lobsters mud crabs penaeid prawns Nile tilapia yellow king fish abalone sea cucumber and sea urchin seaweed ornamentals and Indian major carps fugu mud skippers cephalopods and blue fin tuna The technological innovations and introduction of new species into aquaculture are critical to the evolution of the global aquaculture industry an industry which is rapidly becoming one of the fastest growing in the world having experienced huge advances across its many and diverse facets **Recent Advances and New Species in Aquaculture** focuses explicitly on the ever changing face of aquaculture providing core scientific and commercially useful information on the remarkable growth in aquaculture production and in the advancement of new technological tools Written by many well respected international figures and drawn together and edited by Ravi Fotedar Bruce Phillips this exciting book is an essential purchase for anyone involved in or about to enter into the aquaculture industry Libraries in all universities and research establishments where aquaculture fish biology aquatic and environmental sciences and fisheries are studied and taught will find this an important addition to their shelves **Recent Advances and New Species in Aquaculture** is sure to become a key companion for all those studying aquaculture and a valuable source of reference for all personnel involved in the industry

Recent Advances in Pediatric Cancer Predisposition Syndromes Angela Mastronuzzi, Luigi Boccuto, Riccardo Masetti, 2021-05-03 We would like to acknowledge Dr Giada Del Baldo and Dr Mariachiara Lodi from IRCCS Bambino Gesù Children's Hospital have acted as coordinator and have contributed to the preparation of the proposal for this Research Topic *Recent advances in voltage-gated sodium channels, their pharmacology and related diseases* Mohamed Chahine, Jean-François Desaphy, It is our pleasure to co edit a Research Topic on voltage gated sodium channels pharmacology and related diseases We are in a process to inviting submissions of novel research article state of the art review papers and viewpoints on this topic All the papers will follow a peer review process according to the guidelines of Frontiers in Pharmacology Voltage gated sodium channel play a critical role in electrical signalling in many excitable cells such as neurons skeletal muscle cells and cardiac myocytes They are responsible for the initiation and the propagation of action potential allowing integration of higher processes They are formed by one alpha subunit that forms the pore of the channel and one or several regulatory subunits Sodium channels are the target for local anaesthetics antiarrhythmic drugs and anticonvulsants This class of ion channels is prime target to several toxins Recently a number of mutations in sodium channels that often results in alterations of rapid channel gating have been identified and linked to human diseases Such

channelopathies cause periodic paralysis myotonia long QT syndrome and other cardiac conductance disturbances pain hemiplegic migraine and epilepsy Sodium channels play also a negative role in malignant cellular proliferation chronic pain and other diseases Considering these crucial physiological and pathological implications it is not surprising that sodium channels have been and still are key targets for drug development This Research Topic of Frontiers of Pharmacology of Ion Channels and Channelopathies will focus on this important class of ion channels how they operate their pharmacology and their implication in diseases

Recent Advances in iPSC Disease Modeling Alexander Birbrair,2020-07-16 Recent Advances in iPSC Disease Modeling Volume One addresses how induced pluripotent stem cells can be used to model various diseases This new volume teaches readers about current advances in the field describing the use of induced pluripotent stem cells to model several diseases in vitro and thus enabling us to study the cellular and molecular mechanisms involved in different pathologies Further insights into these mechanisms will have important implications for our understanding of disease appearance development and progression The volume is written for researchers and scientists in stem cell therapy cell biology regenerative medicine and organ transplantation specialists In recent years remarkable progress has been made in the obtention of induced pluripotent stem cells and their differentiation into several cell types tissues and organs using state of art techniques Hence these advantages have facilitated the identification of key targets and further defining on the molecular basis of several disorders Provides an overview on the fast moving field of induced pluripotent stem cell technology regenerative medicine and therapeutics Covers the following diseases severe congenital neutropenia sickle cell and Diamond Blackfan anemias muscular dystrophies Bernard Soulier syndrome familial hypercholesterolemia type II A Werner syndrome lysosomal storage diseases and more Contains descriptions of cutting edge research on the development of disease specific human pluripotent stem cells

National Library of Medicine Current Catalog National Library of Medicine (U.S.),1971 First multi year cumulation covers six years 1965 70

Systemic Sclerosis - Recent Advances and New Perspectives Katja Perdan Pirkmajer,Blaž Burja,Katja Lakota,2024-05-15 Systemic sclerosis SSc is a multisystem autoimmune disease involving vasculopathy and immune activation that leads to the deposition of extracellular matrix and the development of fibrosis resulting in cardinal clinical problems Systemic Sclerosis Recent Advances and New Perspectives provides a comprehensive overview of the pathophysiology diagnosis and specific management of the clinical manifestations of SSc The book addresses the current understanding of the causes and consequences of inflammatory activation chromosomal instability and activating factors Special emphasis is placed on diagnosis from the recognition of very early systemic sclerosis and consideration of its treatment to diagnostic methods for microvascular and pulmonary involvement It also highlights the less life threatening but very common manifestations of bone and skeletal muscle involvement which affect patient quality of life

Recent Advances in Tryptophan Research Graziella Allegri,Carlo V.L. Costa,Antonella Bertazzo,2012-12-06 Wide and fascinating is the field of research on tryptophan a most versatile amino acid transformed as it

is in our organism into many biologically active substances This volume contains the proceedings of the Eighth International Meeting on Tryptophan Research held at the University of Padova Padova Italy from June 25 to 29 1995 under the auspices of the University of Padova National Research Council Italian Chemical Society Division of Pharmaceutical Sciences Region of the Veneto and City of Padova The meeting was held in Padova to commemorate Prof Luigi Musajo twenty years after his death and the editors dedicate this book to him in recognition of his pioneering work in tryptophan metabolism Prof Osamu Hayaishi delivered the Musajo Memorial Award Lecture Tryptophan oxygenase and sleep Figure I shows the ISTRY President Prof Simon N Young presenting the Musajo Memorial Medal to Prof Hayaishi during the Opening Ceremony Two hundred scientists from twenty two countries participated in the meeting These proceedings contain 121 papers encompassing a variety of topics and disciplines

Recent Advances in Pharmaceutical Innovation and Research Prati Pal Singh, 2023-09-11 This book covers several important aspects of pharmaceutical research and innovations It presents important topics on drug delivery novel microsphere nanocrystals polymeric nanoparticles peptide synthesis biopharmaceuticals pharmacodynamics yeast flocculation neuromodulators innovative drug discovery pharmacoinformatics aminoquinoline thiourea crystals for API synthesis FDCs and formulations research ayurveda and natural products and innovations to militate anti microbial resistance AMR A chapter is devoted to the applications of Artificial Intelligence and Machine Learning in diverse sectors of the pharmaceutical industry including drug discovery and development drug repurposing and improving pharmaceutical productivity The book also reviews the role of pharmacogenomics and pharmacogenetics in drug development and precision medicine Further the book presents an updated summary of recent advances in the fields of nanomedicines and nano based drug delivery systems This book is useful to pharmaceutical sciences students researchers educators and professionals in the pharmaceutical industry to understand the intricacies of new drug research and innovations

Recent advances in antiphospholipid syndrome Rohan Willis, Ljudmila Stojanovich, Ahmet Cagkan Inkaya, 2023-03-24

Current Catalog National Library of Medicine (U.S.), 1979 First multi year cumulation covers six years 1965 70

Recent Advances in Ophthalmology - 10 HV Nema, Nitin Nema, 2011-06 The tenth volume of Recent Advances in Ophthalmology contains some selected chapters on cornea uvea secondary glaucoma lens retina oculoplastic surgery and neuro ophthalmic disorders First time in the serial publication three editorials artificial cornea diabetic retinopathy and endophthalmitis are included to provide an over view of the problem to the reader Recent advances in ophthalmology have considerably improved the quality of medical and surgical management of the patients with eye diseases Besides editorial on artificial cornea five articles are included on cornea Sinha and coauthors have described corneal dystrophies in great detail covering prevalence international classification inheritance histopathology clinical features and treatment Hopefully the postgraduate students in ophthalmology and general ophthalmic practitioners will find the present volume helpful not only in the basic understanding of new developments in the subject but also in day to day clinical practice

and care of the patients Recent advances in understanding tourette syndrome, tic disorders and functional tics Amanda Ludlow,Seonaid Anderson,Kevin J. Black,Christine Conelea,2023-04-28 Time Series Analysis - Recent Advances, New Perspectives and Applications Jorge Rocha,Sandra Oliveira,Cláudia Viana,2024-05-22 Time series analysis describes explains and predicts changes in a phenomenon through time People have utilized techniques that add a distinctive spatial dimension to this type of analysis Major applications of spatiotemporal analysis include forecasting epidemics analyzing the development of traffic conditions in urban networks and forecasting backcasting economic risks such as those associated with changing house prices and the occurrence of hazardous events This book includes contributions from researchers scholars and professionals about the most recent theory models and applications for interdisciplinary and multidisciplinary research encircling disciplines of computer science mathematics statistics geography and more in time series analysis and forecasting backcasting Respiratory Disease: New Insights for the Healthcare Professional: 2011 Edition ,2012-01-09 Respiratory Disease New Insights for the Healthcare Professional 2011 Edition is a ScholarlyPaper that delivers timely authoritative and intensively focused information about Respiratory Disease in a compact format The editors have built Respiratory Disease New Insights for the Healthcare Professional 2011 Edition on the vast information databases of ScholarlyNews You can expect the information about Respiratory Disease in this eBook to be deeper than what you can access anywhere else as well as consistently reliable authoritative informed and relevant The content of Respiratory Disease New Insights for the Healthcare Professional 2011 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](http://www.ScholarlyEditions.com)

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New Advances in Crop Disease Management Sangita Sahni,Ganesh Thapa,Bishun Deo Prasad,2024-11-08 Crop

protection is becoming increasingly important due to the need for food security and to meet growing environmental challenges This new book *New Advances in Crop Disease Management* provides readers with an updated and comprehensive overview of diverse crop protection strategies with special emphasis on the recent advances in crop protection approaches fundamental concepts principles methodology relationships and benefits for crop protection and human health It covers a variety of disciplines such as plant pathology biochemistry cell and molecular biology plant physiology host pathogen interactions precision agriculture decision analysis disease risk new ways of exploiting host genetic diversity including host resistance deployment biological control and microbial interactions surveillance and detection technologies and the consequences of climate change and signaling The book contains twenty chapters and begins with the role of biotechnology and its implications in crop disease detection and protection It then discusses the plant response to pathogen attack molecular basis of host pathogen interaction and induction of defense response viz R gene phytoalexins etc and advancement in early detection methodologies It also delves into induced mutation as new technology for resistant crop development The last part details the recent approaches and applications of resistant crop lines development including resistant pulses development approaches mining and mapping of R genes gene pyramiding lactic acid bacteria as biocontrol agents effecteromics and nanotechnology in crop disease management and the applications of phytochemicals and CRISPR Cas in the development of disease resistant crops for future *New Advances in Crop Disease Management* will be a key reference in the field of crop science and plant pathology agriculture for faculty and industry professionals as well as a valuable textbook reference for undergraduate and graduate students

New Advances in Pediatric Neurologic and Developmental Disorders in the Era of Genomics, An Issue of Pediatric Clinics of North America Gyula Acsadi, 2015-06-25

Neurodevelopmental diseases affect three percent of children and the specific cause is difficult to determine in many cases Genetic research uncovered many loss or gain of function mutations in genes that are associated with synaptic development and or remodeling However more and more data are accumulating regarding the importance in gene regulations in neurodevelopmental diseases This issue addresses many aspects of the genomics of neurodevelopmental diseases including articles devoted to genomic variations of brain malformations neuroimaging of brain development update on autism evaluation and genetics prader willi and angelman syndromes tuberous sclerosis complex genetics of pediatric epilepsy pediatric leukodystrophies autoimmune encephalopathies inherited neuropathies and motor neuron diseases and muscular dystrophies and congenital myopathies

Technology in Sports - Recent Advances, New Perspectives and Application Thomas Wojda, 2024-05-02 Explore the dynamic intersection of technology and sports in *Technology in Sports Recent Advances New Perspectives and Application* This comprehensive volume covers a wide array of topics from the biomedical and legal aspects of nutrition for athletes to cutting edge developments in sports analytics and talent identification Discover how computer simulations are revolutionizing training strategies and delve into the role of ECG in

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Myelodysplastic Syndromes: New Insights for the Healthcare Professional: 2011 Edition ,2012-01-09 Myelodysplastic Syndromes New Insights for the Healthcare Professional 2011 Edition is a ScholarlyBrief that delivers timely authoritative comprehensive and specialized information about Myelodysplastic Syndromes in a concise format The editors have built Myelodysplastic Syndromes New Insights for the Healthcare Professional 2011 Edition on the vast information databases of ScholarlyNews You can expect the information about Myelodysplastic Syndromes in this eBook to be deeper than what you can access anywhere else as well as consistently reliable authoritative informed and relevant The content of Myelodysplastic Syndromes New Insights for the Healthcare Professional 2011 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>

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