

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

**Jorge Rocha, Sandra Oliveira, Cláudia
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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 Boccutto, 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 antiphospholipid syndrome Rohan Willis, Ljudmila Stojanovich, Ahmet Cagkan Inkaya, 2023-03-24

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

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