

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 inhibition, 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

Recent Advances And New Syndromes

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

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Handbook of Phobic and Anxiety Disorders in Children and Adolescents Thomas H. Ollendick,Neville J. King,W. Yule,2013-11-11 Reknowned authorities offer the first international handbook on anxiety and phobic disorders in children and adolescents Using DSM IV and ICD classifications this comprehensive and up to date volume addresses issues related to diagnostic classification epidemiolgy etiology assessment and treatment With its case studies this volume makes a practical reference for clinicians researchers and students Bericht ,1938 American Journal of Medical Genetics ,1984

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Congenital Cardiac Defects--recent Advances Daniel Bergsma,1972 **New York State Journal of Medicine** ,1961

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Recent Advances And New Syndromes Book Review: Unveiling the Magic of Language

In an electronic digital era where connections and knowledge reign supreme, the enchanting power of language has become more apparent than ever. Its power to stir emotions, provoke thought, and instigate transformation is truly remarkable. This extraordinary book, aptly titled "**Recent Advances And New Syndromes**," published by a highly acclaimed author, immerses readers in a captivating exploration of the significance of language and its profound impact on our existence. Throughout this critique, we shall delve to the book is central themes, evaluate its unique writing style, and assess its overall influence on its readership.

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