Ponatinib is a pan TKI with particular activity on BCR-ABL1 fusion protein, VEGF and FLT3, and a high number of collateral activity on immunogenic cell death and environment. Ponatinib is able to protect against the emergence of BCR-ABL1 mutations. Ponatinib was used in new-onset and relapse-refractory Ph+ ALL. The use of ponatinib may be further expanded in Ph-like/3C-UP ALL and in subcategories of AML.

Gilteritinib is an FLT3, AXL, and ALK inhibitor with single-agent activity in R/R AML. Gilteritinib multikinase inhibition and differentiation effects will be explored, together with combination with chemotherapy.

MDM2 and Menin inhibition are appealing strategies in the treatment of predefined subsets of AML, preliminary laboratory data will be presented.

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Sp12

HAEMOPHILIA AND NURSING CARE

Marcela Ganzella

Hemophilia is a rare, inherited, X-chromosome-linked bleeding disorder resulting from a deficiency of clotting factor VIII (hemophilia A) or factor IX (hemophilia B). In the world, according to the World Federation of Hemophilia 2019, there are currently approximately 157,517 people diagnosed with Hemophilia A and 31,997 Hemophilia B. Nurses may be involved in providing direct clinical care, education, support and self-management for patients and their families. In this presentation we will talk about important aspects of hemophilia: pathophysiology, nursing care and concern, treatment pathway and patient education

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Sp13

RHABDOMYOSARCOMA

Mehmet Fatih Okçu

Rhabdomyosarcoma is the most common soft tissue sarcoma in childhood. While based on the cooperative group work from US and Europe diagnosis and treatment guidelines exist management controversies exist for newly diagnosed intermediate and high risk disease and in patients with relapses. The presentation will discuss further details on management of these patient groups in the light of recent published work.

Non-rhabdomyosarcomatous soft tissue sarcomas (NRSTS)

NRSTS are large group of heterogenous group of soft tissue sarcoma diagnoses representing half of all childhood soft tissue sarcomas. In this presentation we will review standard approach in general on diagnosis and management of soft tissue tumors and further discuss how recent molecular work informs diagnosis and management of subgroup of patients.

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Sp14

THE APPROACH AND DIAGNOSIS OF COOMBS NEGATIVE HEMOLYTIC ANEMIAS

Achille Iolascon

Anemia affects 1.6 billion of people worldwide, about 10% of these individuals are affected by rare anemias of which 80% are hereditary.[1] Hereditary anemias (HA) embrace a highly heterogeneous group of disorders characterized by anemia of variable degree and by complex genotype-phenotype correlations. Differential diagnosis, classification, and patient stratification among HA are often very difficult.

To date, the major current application of next generation sequencing (NGS) in diagnostics is through disease-targeted tests for which multiple causal genes are known. Some studies have already demonstrated the utility of targeted-NGS (t-NGS) approach in the study of specific subtypes of HA patients. Here, we described the diagnostic workflow based on t-NGS that we developed for the diagnosis of patients affected by HA. Within this wide group of disorders, we included: (1) hyporegenerative anemias, as congenital dyserythropoietic anemias (CDA); (2) hemolytic anemias due to red cell membrane defects, as hereditary spherocytosis (HS) and stomatocytosis (HSt); hemolytic anemias due to enzymatic defects, as pyruvate kinase (PK) deficiency.[1–5]

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