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abstract

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abstract

Chemotherapy-free Treatment: New Approach for Pediatric APL

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Introduction: APL is a subtype of AML, characterised by recurrent cytogenetic abnormalities that involve the chimeric gene PML/RARalpha. The average age of onset is 9 years, and it mainly affects females. The prognosis is good, with a survival rate that exceeds 90% of cases. The treatment historically involved a polychemotherapy based on the combination of anthracyclines, inevitably associated with serious side effects such as cardiotoxicity, myelosuppression, and hepatic repercussions.
For these reasons, a lot of studies evaluated the possibility of limiting the use of chemotherapy, replacing it completely with the combination of ATRA and ATO (arsenic trioxide). For patients classified as high risk is also added Gemtuzumab-Ozogamicin. With our work, we compared the data of patients treated with the previous chemotherapy approach according to the AIEOP ICC APL Study 01 protocol with those of patients treated with the current AIEOP ICC APL Study 02 chemotherapy-free protocol. The main goal was to verify the non-reduction of the efficacy of the new treatment and any other differences in side effects and quality of life between the two protocols.

Methodology: The study included 7 patients followed at the Children's Hospital of Brescia from 2009 to the present and 8 patients treated at the San Gerardo Hospital of Monza from 2020 to 2021.
In our sample, the incidence was characterized by males, with an average age of 9.8 years, without pathological BMIs, without major episodes of hemorrhagic diathesis at diagnosis, and who belonged predominantly to the standard risk class.

Results: The times necessary to achieve hematological and bone marrow remission were approximately the same for both groups of patients. Furthermore, the polychemotherapy resulted in greater cardiotoxicity; more episodes and higher severity of hepatotoxicity; greater myelosuppression; the differentiation syndrome was also characterized mainly by the course of patients subjected to polychemotherapy, while pseudotumor cerebri the protocol with ATRA-ATO combination.
About quality of life, the chemotherapy treatment lasts 28 and a half months, compared to only 8 months in total foreseen in the ICC APL Study 02 protocol. Therefore, although the total number of days of hospitalization is significantly higher in the ATRA-ATO treatment, overall, this treatment lasts less with fewer repercussions for the young patients. At last, alopecia is a specific consequence of chemotherapy.

Conclusion: For all these reasons, the data support us in continuing the treatment of Acute Promyelocytic Leukemia in pediatric age at our facility according to the ICC APL Study 02 chemotherapy-free protocol.