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Long-term survival case reports of two pediatric relapsed or refractory acute myeloid leukemia patients treated with bisantrene combination therapy

Amir Sharaf
CarthaGenetics® sarl, Switzerland

Background: Bisantrene, an anthracene derivative topoisomerase II and telomerase-inhibitor, macrophage-activator without anthracyclines' induced-cardiotoxicity or MDR demonstrated historical CR in recurrent or refractory AML: 23% (Marty, 1985), 50% (Marty, 1987; Bezwoda, 1989) and 72% (Spadea, 1993). In pediatrics (Leblanc, 1994), 46% historical CR was reached in heavily pretreated patients. We report, two AML survivors cases, decades following the study.

Case 1: Patient S (female) born January 1977, diagnosed with AML type M3 in January 1984. Initially treated with multiple lines (cytarabine, daunorubicin, 6-mercaptopurine, methotrexate), relapsed in November 1984, treated in December 1984 with bisantrene 250mg/m2/day (7 days) followed by 6 consolidation cycles of amsacrine, cytarabine, reached historical CR in January 1985. Patient underwent two autologous BMT in 1985. Normal Cardiac ultrasound in May 1985. Post transfusion HIV infection treated in September 1997. Alive today, mother of 3 children.

Case 2: Patient A (female) born June 1977, diagnosed with AML type M1, t(8,21) in 1990, had multiple lines (cytarabine, mitoxantrone, VP-16, daunorubicin). BM cryopreservation in June 1991. Relapsed in November 1991. Following additional failed chemotherapy, received bisantrene 200mg/m_2/day (5 days) + VP16 100mg/m_2/day (5 days) + carboplatin 150mg/m_2/day (5 days) in December 1991, had historical CR in January 1992 with normal cardiac ultrasound. Received BM autograft after fractioned irradiation and cyclophosphamide in February 1992. Alive today, gave birth in June 2015.

Conclusions: Long-term case reports and published bisantrene efficacy results from prior salvage studies, support renewed interest in its clinical development as candidate with unique safety profile particularly appropriate in pediatric AML.

Biography

Amir Sharaf is a Medical Doctor with additional experience in European Market Access of innovative pharmaceuticals, including early access of drug candidates in development for orphan indications and patients with rare diseases. His research interests include Immunology, Internal Medicine and Oncology. His research works focus on antibodies' diagnostic value in connective tissue diseases, as well as regulatory and economic requirements for the Market Access of Orphan and Innovative Therapeutics including Advanced Therapeutic Medicinal Products (ATMPs) in the EU. He has also been involved in the pricing of different molecules in Oncology and vaccination. Amir is CarthaGenetics® International Medical Manager and Business Development Director. He provides targeted methodology approaches for CarthaGenetics® projects, as well as scientific communications to physicians and health authorities worldwide. CarthaGenetics® is a company dedicated to the support of innovative treatments development and their access to patients in need worldwide. It was founded by Philippe Carteron de Balmont in 2004 based on his personal commitment to find innovative early patients access to Orphan Drugs. CarthaGenetics® is now a pioneer with one of the broadest experience in the field of Rare Diseases.

sharaf@carthagenetics.com

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