International Journal of Hematology
https://doi.org/10.1007/s12185-021-03248-x

ORIGINAL ARTICLE

A retrospective analysis of azacitidine treatment for juvenile myelomonocytic leukemia

Yuko Honda[1] · Hideki Muramatsu[2] · Yuka Nanjo[3] · Shinsuke Hirabayashi[4] · Toru Meguro[5] · Nao Yoshida[6] · Harumi Kakuda[7] · Shuichi Ozono[8] · Manabu Wakamatsu[2] · Hiroshi Moritake[9] · Masahiro Yasui[10,11] · Hideki Sano[12] · Atsushi Manabe[4] · Kazuo Sakashita[13]

Received: 6 July 2021 / Revised: 16 October 2021 / Accepted: 19 October 2021

Abstract

Juvenile myelomonocytic leukemia (JMML) is a pediatric hematological malignancy with a poor prognosis. Although several case series have been published describing hematological and molecular responses to azacitidine (AZA) treatment in patients with JMML, the efficacy and safety profile of AZA is not well investigated, especially in Asian children and children undergoing hematopoietic stem cell transplantation (HSCT). We retrospectively analyzed 5 patients who received a total of 12 cycles (median 2 cycles) of AZA treatment in Japan. All five patients were boys and their ages at the time of treatment were 21, 23, 24, 26, and 46 months, respectively. All five patients tolerated AZA treatment, including four patients who received AZA after HSCT. Therapeutic toxicity with AZA was mostly limited to hematological toxicity. The only serious non-hematological adverse event was hyperbilirubinemia (grades III–IV) observed in a patient who received AZA after a second HSCT. Two out of five patients treated with AZA achieved a partial response (PR), while three patients treated for post-transplant relapse did not have an objective response. Future prospective studies should be conducted to develop combination therapies with AZA and other molecular targeted drugs for high-risk patients.

Keywords Juvenile myelomonocytic leukemia · Azacitidine · Hematopoietic stem cell transplantation · DNA hypomethylating agents

✉ Yuko Honda
h-yukoc@med.uoeh-u.ac.jp

[1] Department of Pediatrics, School of Medicine, University of Occupational and Environmental Health, 1-1 Iseigaoka, Yahatanishi-ku, Kitakyushu 807-8555, Japan

[2] Department of Pediatrics, Nagoya University Graduate School of Medicine, Nagoya, Japan

[3] Department of Hematology and Oncology, Miyagi Children's Hospital, Sendai, Japan

[4] Department of Pediatrics, Hokkaido University Hospital, Sapporo, Japan

[5] Department of Pediatrics, Yamagata University School of Medicine, Yamagata, Japan

[6] Department of Hematology and Oncology, Children's Medical Center, Japanese Red Cross Nagoya First Hospital, Nagoya, Japan

[7] Department of Hematology/Oncology, Chiba Children's Hospital, Chiba, Japan

[8] Department of Pediatrics and Child Health, Kurume University School of Medicine, Kurume, Fukuoka, Japan

[9] Division of Pediatrics, Faculty of Medicine, University of Miyazaki, Miyazaki, Japan

[10] Department of Hematology and Oncology, Children's Medical Center, Kitakyushu City Yahata Hospital, Kitakyushu, Japan

[11] Department of Hematology/Oncology, Osaka Women's and Children's Hospital, Izumi, Japan

[12] Department of Pediatric Oncology, Fukushima Medical University Hospital, Fukushima, Japan

[13] Department of Pediatric Hematology and Oncology, Nagano Children's Hospital, Azumino, Japan

Published online: 29 October 2021