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A prospective registration of children with myelodysplastic syndromes (MDS) and myeloproliferative disorders (MPD) in Japan

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The diagnosis and the treatment of children with MDS and MPD are difficult mainly because the number of patients is very small. We organized the MDS Committee in the Japanese Society of Pediatric Hematology in 1997 and conducted a retrospective survey of patients with the diseases diagnosed between 1990 and 1999. The diagnosis was individually made in each institute. Of total, 293 patients were enrolled: 174 primary MDS (36 refractory anemia [RA], 20 RA with excess of blasts [RAEB], 29 RAEB in transformation [RAEBT], 15 chronic myelomonocytic leukemia [CMML], 60 juvenile myelomonocytic leukemia [JMML], 14 others), 48 MDS with constitutional abnormalities (18 Down syndrome, 11 Fanconi anemia, 4 neurofibromatosis-1, 15 others), and 71 secondary MDS (30 post therapy for aplastic anemia, 41 post therapy for malignancy. Overall survival (Standard Error) at 4 years for all the patients was 48.2 (3.1) % as a whole, and 56.3 (4) % for primary MDS, 49.4 (7.8) % for MDS with

constitutional abnormalities, 28.8 (5.6) % for secondary MDS. Bone marrow biopsy was performed in only 10% of the cases. The estimated frequency of the diseases was 8% of all the children with leukemia. We next started a prospective registration employing a central review of pathological specimens in 1999. The smears of peripheral blood and bone marrow were reviewed by 2 independent investigators and the biopsy specimens were reviewed by a qualified pathologist. As of May in 2004, 250 children were enrolled. The diagnosis of the first 150 patients included primary MDS other than JMML in 48, JMML in 35, and secondary MDS in 12. There were 34 patients with other diagnoses than MDS or MPD. Bone marrow biopsy was performed in 38% of the cases. We also initiated a prospective treatment program in 1999 and over 60 patients have been enrolled. The standardized diagnosis is essential to facilitate a good clinical trial in this rare disease. An international collaborative study is warranted at the next stage.