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Down Syndrome, Rasopathies, and Other Rare Syndromes

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DOWN SYNDROME, RASOPATHIES, AND OTHER RARE SYNDROMES

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KEY MESSAGES

- 1. Watch and wait in NS-related MPD and CBLS-related JMML, HSCT in patients with NF1related JMML
- 2. The myeloid leukemias of DS are uniquely sensitive to Ara-C. Low-dose Ara-C may be needed for control of clinically advanced congenital transient myeloproliferative disorder.
- Clinical studies examining the benefits of monitoring of GATA1s levels and prophylactic ARA-C therapy in infants with DS are warranted

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