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Supplementary appendix

This appendix formed part of the original submission and has been peer reviewed. We post it as supplied by the authors.

Supplement to: Garcia-Manero G, Roboz G, Walsh K, et al. Guadecitabine (SGI-110) in patients with intermediate or high-risk myelodysplastic syndromes: phase 2 results from a multicentre, open-label, randomised, phase 1/2 trial. *Lancet Haematol* 2019; published online May 3. http://dx.doi.org/10.1016/S2352-3026(19)30029-8.

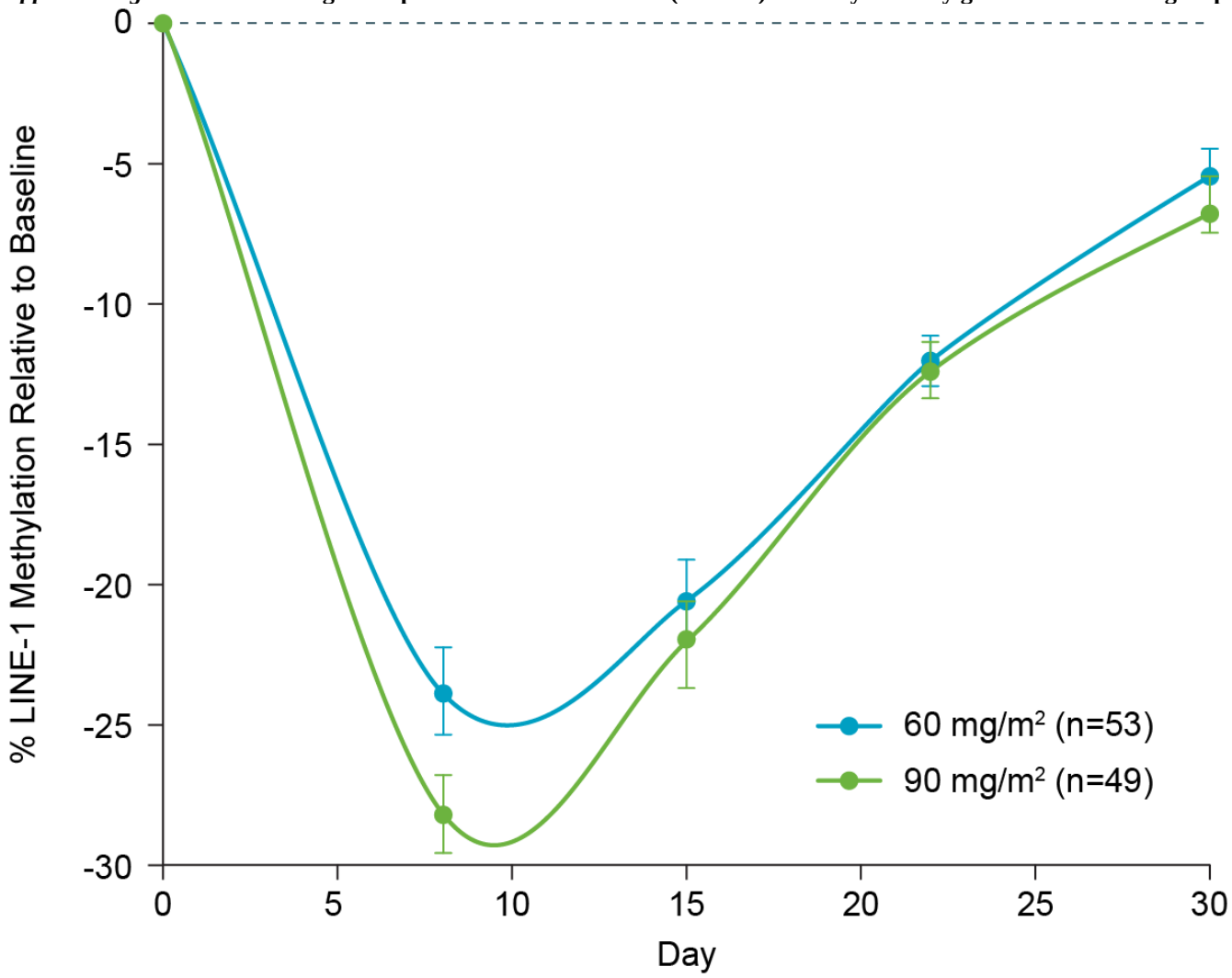
Supplementary Appendix

Centre #	Principal investigator	Study centre	Patients enrolled (N=105), n	Patients treated (N=102), n
3005	Hagop Kantarjian, MD (replaced Jean-Pierre J Issa, MD, 1 Sept 2011) Guillermo Garcia-Manero, MD*	The University of Texas MD Anderson Cancer Center, Houston, USA	31	31
3001	Gail Roboz, MD	New York-Presbyterian/Weill Cornell Medical Center, New York, NY, USA	16	16
3014	Katherine Walsh, MD (replaced William Blum, MD, 27 April 2013)	The Ohio State University, Columbus, OH, USA	11	10
3035	Jesus Berdeja, MD (replaced Michael R Savona, MD, 4 Feb 2014)	Sarah Cannon, Nashville, TN, USA	9	9
3036	Patricia Kropf, MD	Fox Chase Cancer Center, Philadelphia, PA, USA	8	8
3007	Casey O'Connell, MD	Keck School of Medicine of USC, Los Angeles, CA, USA	8	7
3008	Raoul Tibes, MD, PhD	Mayo Clinic, Scottsdale, AZ, USA	6	5
3035	Scott Lunin, MD (replaced Thomas Ervin, MD, 17 Jan 2014)	Florida Cancer Specialists & Research Institute, Fort Myers, FL, USA	5	5
3039	Todd Rosenblat, MD	Columbia University Irving Medical Center, New York	3	3
3027	Karen Yee, MD	Princess Margaret Cancer Centre, Toronto, Ontario, Canada	3	3
3032	Wendy Stock, MD	The University of Chicago Medicine Comprehensive Cancer Center, IL, USA	2	2
3026	Elizabeth Griffiths, MD	Roswell Park Comprehensive Cancer Center, Buffalo, NY	1	1
3038	Joseph Mace, MD	Florida Cancer Specialists & Research Institute, St Petersburg, FL	1	1
3040	Nikolai Podoltsev, MD	Yale School of Medicine, New Haven, CT, USA	1	1
Total no. of centres: 14				

Enrolled = signed informed consent form and not screen failure; Treated = enrolled and received ≥1 dose. *Coordinating investigator; Dr Garcia-Manero was subinvestigator under Dr Kantarjian for overall study, but acted as coordinating investigator for MDS portion of study and CSR (SGI-110-01-D).

Appendix Table A1: **Principal investigators and study centres**

Appendix Figure A1. **Mean long interspersed nuclear element-1 (LINE-1) demethylation by guadecitabine dose group.**



	Overall response rate						
	Relapsed/refractory MDS			Treatment-naïve MDS			
	60 mg/m^2 (n=23)	90 mg/m^2 (n=23)	Total (n=46)	60 mg/m^2 (n=26)	90 mg/m^2 (n=21)	Total (n=47)	Total (n=93)
DNMT3A negative	6/20 (30)	9/18 (50)	15/38 (39)	10/20 (50)	10/18 (56)	20/38 (53)	35/76 (46)
DNMT3A positive *†	1/3 (33)	3/5 (60)	4/8 (50)	3/6 (50)	2/3 (67)	5/9 (56)	9/17 (53)
TET2 negative	4/15 (27)	11/21 (52)	15/36 (42)	9/18 (50)	11/19 (58)	20/37 (54)	35/73 (48)
TET2 positive*†	3/8 (38)	1/2 (50)	4/10 (40)	4/8 (50)	1/2 (50)	5/10 (50)	9/20 (45)
TP53 negative	7/19 (37)	10/17 (59)	17/36 (47)	13/25 (52)	8/16 (50)	21/41 (51)	38/77 (49)
TP53 positive*	0/4 (0)	2/6 (33)	2/10 (20)	0/1 (0)	4/5 (80)	4/6 (67)	6/16 (38)

Data expressed as n/N (%). "Positive" means mutation was present; "negative" means mutation was absent. Nine patients without baseline gene mutation data were excluded. MDS=myelodysplastic syndrome. DNMT3A=DNA methyltransferase 3α. TET2=tet methylcytosine dioxygenase 2. TP53=tumour protein P53.CR=complete response. HI-N= haematologic improvement-neutrophil. *1 relapsed/refractory patient was positive for DNTM3A and TET2, and a nonresponder. †1 treatment-naive patient was positive for DNTM3A and TP53 (and had a complete response) and another treatment-naïve patient was positive for DNTM3A and TET2 (and had responses of haematologic improvement and haematologic improvement in neutrophils.

Appendix Table A2: **Overall response by gene mutation status in myelodysplastic syndrome by disease cohort**

	Guadecitabine dose level		Disease cohort		All patients (n=102)
	60 mg/m^2 (n=53)	90 mg/m^2 (n=49)	Treatment naïve (n=49)	Relapsed/refractory (n=53)	
30-day mortality	0	1 (2)	0	1 (2)	1 (1)
60-day mortality	2 (4)	2 (4)	3 (6)	1 (2)	4 (4)
90-day mortality	3 (6)	6 (12)	4 (8)	5 (10)	9 (9)

Data expressed as n (%) of patients.

Appendix Table A3: **All-cause mortality**