Avapritinib in unresectable or metastatic PDGFRA D842V-mutant gastrointestinal stromal tumours: long-term efficacy and safety data from the NAVIGATOR phase 1 trial

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Appendices

Table A1. Previous TKI exposure at baseline

	***PDGFRA* D842V population (*n* = 56)**	**Safety population (*N* = 250)**
Prior lines of TKI therapy, median (range)	1 (0–6)	3 (0–11)
Prior lines of TKI therapy, n (%)		
0	11 (20)	11 (4)
1	21 (38)	65 (26)
2	10 (18)	34 (14)
3	6 (11)	44 (18)
≥4	8 (14)	96 (38)

PDGFRA, platelet-derived growth factor receptor A; TKI, tyrosine kinase inhibitor.

Table A2. Treatment-related adverse events in ≥20% of the *PDGFRA* D842V and safety populations

Preferred term, n (%)	*PDGFRA* D842V population (*n* = 56)	Safety population (*N* = 250)
Nausea	33 (59)	148 (59)
Fatigue	31 (55)	135 (54)
Periorbital oedema	26 (46)	109 (44)
Anaemia	32 (57)	103 (41)
Diarrhoea	30 (54)	96 (38)
Vomiting	17 (30)	86 (34)
Increased lacrimation	17 (30)	81 (32)
Memory impairment	23 (41)	80 (32)
Decreased appetite	16 (29)	76 (30)
Peripheral oedema	18 (32)	68 (27)
Hair colour changes	16 (29)	61 (24)
Face oedema	13 (23)	56 (22)
Increased blood bilirubin	15 (27)	49 (20)
Dysgeusia	13 (23)	47 (19)
Neutropenia	14 (25)	29 (12)

PDGFRA, platelet-derived growth factor receptor A.

Table A3. Any-cause adverse events occurring in ≥20% of patients in the *PDGFRA* D842V population receiving <300 mg and 300/400 mg starting doses

	***PDGFRA* D842V population (n=56)**	
Preferred term, n (%)	**300/400 mg dose (*n* = 38)**	**<300 mg dose (*n* = 17)**
Nausea	28 (74)	9 (53)
Anaemia	26 (68)	11 (65)
Diarrhoea	25 (66)	11 (65)
Fatigue	22 (58)	13 (76)
Memory impairment	18 (47)	5 (29)
Periorbital oedema	17 (45)	10 (59)
Decreased appetite	15 (39)	7 (41)
Increased lacrimation	13 (34)	8 (47)
Peripheral oedema	12 (32)	9 (53)
Vomiting	12 (32)	8 (47)
Abdominal pain	12 (32)	6 (35)
Hypokalaemia	12 (32)	2 (12)
Increased blood bilirubin	12 (32)	3 (18)
Neutropenia	11 (29)	3 (18)
Face oedema	11 (29)	2 (12)
Dizziness	10 (26)	5 (29)
Headache	10 (26)	2 (12)
Hair colour changes	9 (24)	7 (41)
Dyspepsia	9 (24)	4 (24)
AST increase	9 (24)	2 (12)
Hypomagnesemia	9 (24)	2 (12)
URTI	9 (24)	2 (12)
Weight decrease	8 (21)	6 (35)

Dysgeusia	8 (21)	4 (24)
Hypophosphatemia	8 (21)	3 (18)
Constipation	7 (18)	5 (29)
Insomnia	6 (16)	4 (24)
Back pain	6 (16)	4 (24)
Rash	5 (13)	5 (29)
Increased blood creatinine	5 (13)	5 (29)
Influenza-like illness	3 (8)	4 (24)
Gastroenteritis	2 (5)	4 (24)

AST, aspartate aminotransferase; PDGFRA, platelet-derived growth factor receptor A; URTI, upper respiratory tract infection.

Table A4. Any-cause adverse events of special interest in the *PDGFRA* D842V and safety populations

Adverse event, n (%)	*PDGFRA* D842V population (*n* = 56)		Safety population (*N* = 250)	
	Any grade	**Grade ≥3**	**Any grade**	**Grade ≥3**
Cognitive effects[a]	32 (57)	1 (2)	115 (46)	12 (5)
Memory impairment	23 (41)	0	81 (32)	2 (<1)
Confusional state	8 (14)	0	17 (7)	4 (2)
Cognitive disorder	7 (13)	1 (2)	28 (11)	3 (1)
Encephalopathy	1 (2)	0	5 (2)	4 (2)
Intracranial bleeding[a]	3 (5)	3 (5)	7 (3)	4 (2)
Intracranial haemorrhage	2 (4)	2 (4)	3 (1)	2 (<1)
Cerebral haemorrhage	1 (2)	1 (2)	1 (<1)	1 (<1)
Subdural haematoma	0	0	3 (1)	1 (<1)

PDGFRA, platelet-derived growth factor receptor A.

[a]Combined term

Table A5. Deaths in the *PDGFRA* D842V population due to adverse events

Event, n (%)	*PDGFRA* D842V population (*n* = 56)
Schizophrenia	1 (2)[a]
Disease progression	3 (5)
General physical health deterioration	2 (4)
Cardiac failure	1 (2)
Hepatic failure	1 (2)
Sepsis	1 (2)

[a]Considered treatment-related.