

**TABLE 3 Adverse events, CTCAE version 5.0**

	Total cohort (N = 131)	Wild-type (n = 67)	Heterozygous (n = 47)	Homozygous (n = 17)
Hematological AE, grade IV, n (%)				
Anemia	3 (2.3)	2 (3.0)	1 (2.1)	0 (0.0)
Leukopenia	103 (79)	43 (64)	44 (94)	16 (94)
Neutropenia	127 (97)	63 (94)	47 (100)	17 (100)
Thrombocytopenia	38 (29)	20 (30)	16 (34)	2 (12)
Nonhematological AE, grade III–IV, n (%)				
DIC	1 (0.8)	1 (1.0)	0	0
FN	30 (23)	13 (19)	12 (26)	5 (29)
TLS	1 (0.8)	1 (1.0)	0	0
Ascites	2 (1.5)	2 (3.0)	0	0
Diarrhea	3 (2.3)	2 (3.0)	1 (2.1)	0 (0.0)
Nausea	7 (5.3)	4 (6.0)	3 (6.4)	1 (5.9)
Vomiting	5 (3.8)	4 (6.0)	1 (2.1)	0
BSI	8 (6.1)	5 (7.5)	1 (2.1)	2 (12)
CRBSI	3 (2.3)	2 (3.0)	0	1 (5.9)
Meningitis	1 (0.8)	0	0	1 (5.9)
AST elevation	5 (3.8)	3 (4.5)	1 (2.1)	1 (5.9)
ALT elevation	5 (3.8)	2 (3.0)	2 (4.3)	1 (5.9)
Amylase elevation	5 (3.8)	3 (4.5)	2 (4.3)	0
Hypofibrinogenemia	1 (0.8)	1 (1.0)	0	0
Seizure	1 (0.8)	0	0	1 (5.9)
Hypoxia	4 (3.1)	3 (4.5)	1 (2.1)	0

Abbreviations: CTCAE, common terminology criteria for adverse events; DIC, disseminated intravascular coagulation; FN, febrile neutropenia; TLS, tumor lysis syndrome; BSI, blood stream infection; CRBSI, catheter-related blood stream infection; AST, aspartate aminotransferase; ALT, alanine aminotransferase