Supplementary Table 1 Definitions of response categories

		For patients who received micafungin as empirical antifungal therapy	For patients who received diagnostic driven antifungal therapy	For patients with invasive candidiasis and candidemia	For patients with invasive aspergillosis
Overall Success	Complete response	Resolution of fever symptoms	Survival within the observation period AND resolution of all attributable symptoms and signs of disease and radiological abnormalities AND		
			Survival within the observation period AND mycological evidence of eradication of disease	Fungal clearance confirmed in the blood OR at the original infected site by a second biopsy/repeated biopsies	Disappearance of imaging abnormalities, or only a continuous scar or post-operative imaging change AND evidence of pathogen clearance at the site of infection by secondary culture
				For patients whose samples are difficult to re-culture[a], disappearance of all symptoms and signs of infection and imaging abnormalities is required	
	Partial response	Improvement in fever symptoms	Survival within the observation period AND improvement in attributable symptoms and signs of disease and radiological abnormalities AND		
			Survival within the observation period AND evidence of clearance of cultures or reduction of fungal burden, as assessed by a quantitative and validated laboratory marker	Fungal clearance confirmed in the blood OR at the original infected site by a second biopsy/repeated biopsies	Lesion diameter reduced by >25% AND
				For patients whose samples are difficult to re-culture[a], disappearance of all symptoms and signs of infection and imaging abnormalities is required	A second culture provides evidence of pathogen clearance at the site of infection
					Relief of overall clinical symptoms and signs in

	patients with stable imaging[b] Stable imaging[b], no mycelium and negative culture confirmed in biopsy of infected sites
Failure	Patients with stable response, progression of disease and death
Stable response	Survival within the observation period and no improvement in fungal disease, but no evidence of progression, as determined based on a composite of clinical, radiological, and mycological criteria
Progression of disease	Evidence of progressive fungal disease based on a composite of clinical, radiological, and mycological criteria
Death	Death during the prespecified period of evaluation, regardless of attribution

[a]e.g. patients with internal organ infections

[b]lesion diameter <25%

Supplementary Table 2 Distribution of patients according to organism causing the infection, type of disease and the duration of treatment (PPS)

Index	**(*n* = 2555)**
PPS, *n* (%)	1457 (57.0)
Type of IFI, *n* (%)	
Aspergillus infected patients	17 (1.2)
Candida infected patients	217 (14.9)
Not applicable	1223 (83.9)
Diseases, *n* (%)	
Gastrointestinal mycosis	11 (0.8)
Fungemia	34 (2.3)
Respiratory mycosis	642 (44.1)
Not applicable	770 (52.9)
Duration of treatment, *n* (%)	
≥ 4 weeks	100 (6.9)
≥ 2 weeks to < 4 weeks	508 (34.9)
≥ 1 week to < 2 weeks	849 (58.3)

IFI, invasive fungal infection; *PPS*, per-protocol set

Supplementary Table 3 Rates of fever resolution, resolution of other signs and symptoms, and mycological evidence of pathogen clearance (FAS)

	Number of patients (%)	**95% CI[a]**
Resolution of fever symptoms	(*n* = 1403)	
Not applicable	17 (1.2)	0.6–1.8
Fever symptoms not resolved	290 (20.7)	18.7–22.8
Resolution of fever symptoms	1096 (78.1)	76.0–80.3
Resolution of other signs and symptoms	(*n* = 2467)	
Resolution	113 (4.6)	3.8–5.4
Improvement	1284 (52.1)	50.1–54.0
No improvement	436 (17.7)	16.2–19.2
Deterioration	284 (11.5)	10.3–12.8
Not applicable	350 (14.2)	12.8–15.6
Resolution of imaging abnormalities	(*n* = 1314)[b]	
Disappeared	9 (0.7)	0.2–1.1
Improved	259 (19.7)	17.6–21.9
Stabilized	124 (9.4)	7.9–11.0
Aggravated	92 (7.0)	5.6–8.4
New lesions	18 (1.4)	0.7–2.0
Uncertain radiographic findings	812 (61.8)	59.2–64.4[c]
Mycological evidence of pathogen clearance	(*n* = 422)	
Clear	110 (26.1)	21.9–30.3
Unclear	40 (9.5)	6.7–12.3
Not applicable	272 (64.5)	59.9–69.0[d]

[a]Asymptotic 95% CIs of the percent response rate calculated

[b]Number of patients with ≥1 imaging abnormality from any baseline assessment

[c]Judgments could not be made for some assessments due to inconsistent imaging modalities before and at the end/discontinuation of treatment; for assessments of whether fungal lesions shrank by >25%, 99.9% were uncertain

[d]Patients could not be reassessed because they were not re-examined or because inconsistent mycological examination methods were applied before and after treatment

CI, confidence interval; *FAS*, full analysis set

Supplementary Table 4 Effectiveness results (PPS [*n* =1457])

Comprehensive assessment of treatment, *n* (%); 95% CI	
Complete response	302 (20.7); 18.7–22.8
Partial response	705 (48.4); 45.8–51.0
Stable response	233 (16.0); 14.1–17.9
Progression of disease	152 (10.4); 8.9–12.0
Death	65 (4.5); 3.4–5.5

Asymptotic 95% CIs of the percent response rate calculated

CI, confidence interval; *PPS*, per-protocol set

Supplementary Table 5 AEs reported in > 1% of patients treated with micafungin (SAS [*n* = 2555])

	Number of patients (%)	**Number of events**
Events by System Organ Class and Preferred term	925 (36.2)	1989
Investigations	250 (9.8)	395
Platelet count decreased	35 (1.4)	35
Blood albumin decreased	26 (1.0)	26
Gastrointestinal disorders	248 (9.7)	322
Diarrhea	56 (2.2)	57
Vomiting	47 (1.8)	47
Abdominal pain	30 (1.2)	30
Abdominal distension	30 (1.2)	30
Respiratory, thoracic and mediastinal disease	218 (8.5)	282
Dyspnea	58 (2.3)	61
Respiratory failure	43 (1.7)	43
Chest discomfort	27 (1.1)	27
Cough	26 (1.0)	26
General disorders and administration site conditions	193 (7.6)	218
Fever	51 (2.0)	51
Multi-organ failure	38 (1.5)	38
Edema peripheral	33 (1.3)	34
Fatigue	30 (1.2)	30
Infections and infestations	99 (3.9)	118
Pulmonary infection	55 (2.2)	55
Septic shock	32 (1.3)	32
Nervous system disorders	86 3.4)	95

Cardiac disorders	80 (3.1)	91
Skin and subcutaneous tissue disorders	77 (3.0)	80
Rash	34 (1.3)	34
Blood and lymphatic system disorders	61 (2.4)	65
Anemia	37 (1.5)	37
Hepatobiliary disorders	56 (2.2)	58
Hepatic function abnormal	43 (1.7)	43

AEs, adverse events; *SAS*, safety analysis set

Supplementary Fig. 1 Overall success rates (proportion of patients with complete or partial response) at the end of treatment according to: diagnostic certainty (**a**); the organism causing the infection (**b**); type of disease (**c**); treatment duration (**d**) and the daily dose (**e**) in the PPS[a]

[a]Patients receiving micafungin for < 1 week were excluded from the PPS. *P*-values indicate significant differences between treatment groups (providing there is also a clear difference in the proportion of patients with a complete or partial response); error bars represent asymptotic 95% confidence intervals

PPS, per-protocol set