Table E1. Characteristics of the studies included in the meta-analysis

First author (year)	Study design	Follow-up periods (months)	Response criteria	Definition of PsP	Category of PsP	Tumor*	Agent(s)	Total no. of patients	No. of patients with PsP
Wolchok et al (2009)	Pooled analysis of two trials (NCT00289627, NCT00289640)	NA	irRC	Among patients with WHO PD, patients with irPR and irSD on irRC	PD- irSD/PR/CR on irRC	Melanoma	Ipilimumab	227	22 (9.7%) <sup>a</sup>
Topalian et al (2014)	Posthoc analysis of a clinical trial (NCT00730639)	14–52 months	RECIST 1.0	Persistent reduction in target lesions in the presence of new lesions, regression after initial PDb	PD-SD/PR/CR on RECIST 1.0	Melanoma	Nivolumab	107	4 (3.7%)
Weber et al (2015)	Randomized clinical trial (NCT01721746 [CheckMate 037])	8.4 months in median	RECIST 1.1	A greater than 30% reduction in the sum of the longest diameters of target lesions, consistent with an unconventional, immune-related response following PD	PD-PR/CR on RECIST 1.1	Melanoma	Nivolumab	120	10 (8.3%)
Gettinger et al (2015)	Posthoc analysis of a clinical trial (NCT00730639)	39 months in median	RECIST 1.0	Persistent reduction in target lesions in the presence of new lesions, regression after following initial PD <sup>b</sup>	PD-SD/PR/CR on RECIST 1.0	NSCLC	Nivolumab	129	6 (4.7%)
Borghaei et al (2015)	Randomized clinical trial (NCT01673867 [CheckMate 057])	13.2 months in minimum	RECIST 1.1	■ Appearance of a new lesion followed by decrease from baseline of at least 10% in sum of target lesions ■ Initial increase from nadir ≥ 20% in sum of target lesions	PD-SD/PR/CR on RECIST 1.1	NSCLC	Nivolumab	287	16 (5.6%)

				followed by reduction from					
				baseline of at					
				least 30% ■ Initial increase	-				
				from nadir ≥					
				20% in sum of					
				target lesions or					
				appearance of					
				new lesion followed by at					
				least 2 tumor					
				assessments					
				showing no					
				further					
				progression defined as 10%					
				additional					
				increase in sum					
				of target lesions					
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Brahmer et al (2015)	Randomized clinical trial	Approximately 11 months in	RECIST 1.1	Appearance of a new lesion	PD-SD/PR/CR on RECIST	NSCLC	Nivolumab	131	9 (6.9%)
(2013)	(NCT01642004	minimum		followed by	1.1				
	[CheckMate 017])			decrease from					
	-			baseline of at					
				least 10% in					
				sum of target lesions					
				■ Initial increase	1				
				from nadir ≥					
				20% in sum of					
				target lesions					
				followed by reduction from					
				baseline of at					
				least 30%					
				■ Initial increase					
				from nadir ≥					
				20% in sum of target lesions					
				followed by at					
				least 2 tumor					
				assessments					
				showing no					
				further progression					
				defined as 10%					
				additional					
				increase in sum					

				of target lesions and new lesions					
Rizvi et al (2015)	Clinical trial (NCT01721759 [CheckMate 063])	11.0 months in median	RECIST 1.1	Either a tumor burden reduction or no further progression for at least 2 tumor assessments after initial PD	PD-SD/PR/CR on RECIST 1.1	NSCLC	Nivolumab	117	4 (3.4%)
McDermott et al (2015)	Posthoc analysis of a clinical trial (NCT00730639)	45.2 months in median	RECIST 1.0	Persistent reduction in target lesions in the presence of new lesions, regression following initial PD <sup>b</sup>	PD-SD/PR/CR on RECIST 1.0	RCC	Nivolumab	34	3 (8.8%)
Hodi et al (2016)	Posthoc analysis of a clinical trial (NCT01295827 [KEYNOTE-001)	15 months in median	irRC	≥ 25% increase in tumor burden not confirmed as irRC- defined PD at next assessment	PD- irSD/PR/CR on irRC	Melanoma	Pembrolizumab	327	24 (7.3%)
George et al (2016)	Subgroup analysis of a clinical trial (NCT01354431)	NA	RECIST 1.1	≥30% decrease in the sum of diameters of target lesions and all new measurable lesions, taking as reference the baseline sum diameters, or neither sufficient shrinkage to qualify for immunerelated partial response nor sufficient increase to qualify for immune-related progressive disease, taking as reference the smallest sum diameters during study participation	PD-PR/CR on RECIST 1.1	RCC	Nivolumab	168	12 (7.1%)
Sharma et al (2016)	Clinical trial (NCT01928394 [CheckMate 032])	15.2 months in median	RECIST 1.1	Appearance of a new lesion followed by decrease from baseline of at least 10% in the	PD-SD/PR/CR on RECIST 1.1	UCC	Nivolumab	78	9 (11.5%)

				sum of the target lesions  Initial increase from nadir ≥ 20% in the sum of the target lesions followed by reduction from baseline of at least 30%  Initial increase from nadir ≥ 20% in the sum of the target lesions followed by at least two tumor assessments showing no further					
				further progression defined as a 10% additional increase in sum of target lesions and new lesions					
Rosenberg et al (2016)	Clinical trial (NCT 02108652)		RECIST 1.1	Target lesion reduction of at least 30% from their baseline scans	PD-PR/CR on RECIST 1.1	UCC	Atezolimumab	310	21 (6.8%)
Seiwert et al (2016)	Clinical trial (NCT01848834 [KEYNOTE-012)	NA	RECIST 1.1	RECIST 1.1- defined PD followed by RECIST 1.1- defined CR°	PD-PR/CR on RECIST 1.1	SqCC of head and neck	Pembrolizumab	45	1 (2.2%) <sup>d</sup>
Long et al (2017)	Posthoc analysis of two randomized clinical trials (NCT01721772 [CheckMate 066] and NCT01844505 [CheckMate 067])	14.3 months in median	RECIST 1.1	Greater than 30% tumor reduction in target lesion after progression when compared with baseline	PD-PR/CR on RECIST 1.1	Melanoma	Nivolumab	526	24 (4.6%)
Escudier et al (2017)	Subgroup analysis of a randomized clinical trial (NCT01668784 [CheckMate 025])	8.8 months in median	RECIST 1.1	≥ 30% decrease of tumor burden during treatment beyond progression	PD-PR/CR on RECIST 1.1	Renal cell carcinoma	Nivolumab	406	20 (4.9%)°
Sharma et al (2017)	Clinical trial (NCT02387996	7.0 months in median	RECIST 1.1	■ Appearance of a new lesion	PD-SD/PR/CR on RECIST	Urothelial cell	Nivolumab	265	24 (9.1%)

	[CheckMate 275])			followed by decrease from baseline of at	1.1	carcinoma			
				least 10% in the sum of the target lesions					
				■ Initial increase from nadir ≥ 20% in the sum of the	]				
				target lesions followed by					
				reduction from baseline of at least 30%					
				Initial increase from nadir ≥ 20% in the sum of the					
				target lesions followed by at least two tumor					
				assessments showing no					
				further progression defined as a 10%					
				additional increase in sum of target lesions and new lesions					
Lee et al (2018) <sup>f</sup>	Posthoc analysis of a prospective cohort	19.3 months in median	irRC	≥ 25% increase in tumor burden not confirmed as irRC- defined PD at next assessment	PD- irSD/PR/CR on irRC	Melanoma	PD-1 inhibitor (nivolumab or pembrolizumab)	125	6 (4.8%)
							PD-1 inhibitor (nivolumab or pembrolizumab) and CTLA-4 inhibitor (ipilimumab)		3 (2.4%)

Abbreviations: CR = complete response, CTLA-4 = cytotoxic T-lymphocyte-associated protein 4, irRC = immune-related response criteria, NA = not available, NCT = national clinical trial, NSCLC = non-small cell lung cancer, PD = progressive disease, PD-1 = programmed cell death protein 1, PR = partial response, PsP = pseudoprogression, RECIST = response evaluation criteria in solid tumor, RCC = renal cell carcinoma, SD = stable disease, SqCC = squamous cell carcinoma, UCC = urothelial cell carcinoma, WHO = World Health Organization.

<sup>\*</sup>All patients had locally advanced, metastatic, or recurrent tumor(s).

<sup>&</sup>lt;sup>a</sup>Include 17 patients with irSD and 5 patients with irPR.

<sup>b</sup>Further detail of persistent reduction and regression was not provided.

<sup>c</sup>There was only one patient showed pseudoprogression, and the response pattern shown by the patient is described.

<sup>d</sup>Only one patient with CR.

<sup>e</sup>All 20 patients with PR.

<sup>f</sup>Separate data extraction was available according to the agent (PD-1 inhibitor only and PD-1 inhibitor with CTLA-4 inhibitor).