



## **Appendix 1**

**This appendix was part of the submitted manuscript and has been peer reviewed. It is posted as supplied by the authors.**

Appendix to: Alexander M, Mellor JD, McArthur G, Kee D. Ipilimumab in pretreated patients with unresectable or metastatic cutaneous, uveal and mucosal melanoma. *Med J Aust* 2014; 201: 49-53. doi: 10.5694/mja13.10448.

## Appendix 1 Summary of Published Ipilimumab Studies

Study	Design	Response Criteria	AE Criteria	Intervention	Dose Ipilimumab	# ipilimumab doses	Disease Control (CR, PR, SD)	Study Size	Incidence Grade 3-4 AE	Reason Discontinue
Wolchok et al 2010 (5)	Phase 2	mWHO & irRC	CTCAE v 3.0	Ipilimumab in pre-treated unresectable or metastatic melanoma	10mg/kg	50% received all 4 doses	29.2%	73	53.5%	41.1% PD 26.8% AE
					3mg/kg	69.4% received all 4 doses	26.4%	72	49.3%	40.3% PD 9.9% AE
					0.3mg/kg	67.1% received all 4 doses	13.7%	72	36.1%	45.8% PD 12.5% AE
Hodi et al 2010 (3)	Phase 3	mWHO	CTCAE v 3.0	Ipilimumab + GP100 in pre-treated unresectable or metastatic melanoma	3mg	60% received all 4 doses	20.1%	403	10-15%	Most frequent reason PD (unspecified %)
				Ipilimumab alone in pre-treated unresectable or metastatic melanoma	3mg	64.2% received all 4 doses	28.5%	137		
				GP100 alone in pre-treated unresectable or metastatic melanoma	NA	NA	11.0%	136	3%	
Hersh et al 2011 (6)	Phase 2	RECIST	CTCAE v 3.0	Ipilimumab in chemotherapy naive patients	3mg/kg	29.7% received all 4 doses	21.6%	37	12.8%	67.5% PD 2.5% AE
				Ipilimumab + DTIC in chemotherapy naive patients	3mg/kg	45.7 % received all 4 doses	37.1%	35	22.9%	42.9% PD 8.3% AE
Di Giacomo et al 2011 (7)	Expanded Access Program	mWHO	CTCAE v 3.0	Ipilimumab in pre-treated unresectable or metastatic melanoma	10mg/kg	78% received all 4 doses	29.6%	27	33%	11.1% PD 11.1% AE
Robert et al 2011 (4)	Phase 3	mWHO	CTCAE v 3.0	Ipilimumab + DTIC in untreated unresectable or metastatic melanoma	10mg/kg	36.8% received all 4 doses	33.2%	247	56.3%	46.2% PD 36% AE
				placebo + DTIC in untreated unresectable or metastatic melanoma	NA	NA	30.2%	251	27.5%	77.3% PD 4% AE
Ku et al 2010 (9)	Compassionate Use Program	mWHO	CTCAE v 3.0	Ipilimumab in refractory advanced stage melanoma patients	10mg/kg	-	29%	53	29%	14% AE
Margolin et al 2012 (10)	Phase 2	mWHO & irRC	CTCAE v 3.0	Ipilimumab in non-symptomatic brain metastasis	10mg/kg	Median no. doses = 3	18% overall 24% brain	51	Overall incidence not reported	54.9% PD 5.9% AE
				Ipilimumab in symptomatic brain metastasis	10mg/kg	Median no. doses = 2	5% overall 10% brain	21		52.4% PD 9.5% AE

AE – adverse event, PD – progressive disease, mWHO – modified world health organization criteria, RECIST - Response Evaluation Criteria in Solid Tumors, SAS – special access scheme, CTCAE – common terminology criteria for adverse events, CR – complete response, PR – partial response, SD – stable disease, DTIC – dacarbazine