

Appendix 3: Approval dates and references used to document annual costs of orphan drugs

Orphan drug	EMA approval date	Annual cost (£)	Reference
Adcetris	25/10/2012	42,500	[1]
Adempas	27/03/2014	53,495	[2]
Arzerra	19/04/2010	40,586	[3]
Atriance	22/08/2007	50,000	[4]
Bosulif	27/03/2013	44,799	[5]
Bronchitol	13/04/2012	6,040	[6]
Carbaglu	24/01/2003	245,000	[7]
Cayston	21/09/2009	15,399	[8]
Ceplene	07/10/2008	35,450	[9]
Cholic Acid FGK	04/04/2014	N/A	N/A
Cometriq	21/03/2014	70,664	[10]
Cystadane	15/02/2007	46,580	[11]
Dacogen	20/09/2012	58,300	[12]
Defitelio	18/10/2013	N/A	N/A
Diacomit	04/01/2007	7,600	[13]
Elaprase	08/01/2007	309,660	[14]
Esbriet	28/02/2011	26,172	[15]
Evoltra	29/05/2006	43,200	[16]
Exjade	28/08/2006	25,000	[17]
Firazyr	11/07/2008	16,740	[18]
Firdapse	23/12/2009	47,000	[19]
Gliolan	07/09/2007	2,630	[20]
Glybera	29/10/2012	216,151	[21]
Iclusig	01/07/2013	68,440	[22]
Imnovid	05/08/2013	105,600	[23]
Increlex	03/08/2007	18,000	[24]
Inovelon	16/01/2007	3,000	[25]
Jakavi	23/08/2012	43,200	[26]
Kalydeco	23/07/2012	182,625	[27]
Kuvan	02/12/2008	36,336	[28]
Litak	14/04/2004	£825 (one cycle)**	[29]
Lysodren	28/04/2004	51,900	[30]
Mepact	06/03/2009	92,000	[31]
Mozobil	31/07/2009	34,179	[32]
Myozyme	29/03/2006	230,000	[33]
Naglazyme	24/01/2006	170,000	[34]
Nexavar	19/07/2006	38,852	[35]
Nexobrid	03/09/2012	N/A	N/A
Nplate	04/02/2009	40,102	[36]
Onsenal	17/10/2003	1,800	[37]
Opsumit	20/12/2013	27,979	[38]
Orfadin	21/02/2005	24,744	[39]
Orphacol	12/09/2013	N/A	N/A
Pedea	29/07/2004	6,575	[40]
Peyona	02/07/2009	6,300	[41]

Plenadren	03/11/2011	2,920	[42]
Prialt	21/02/2005	20,000	[43]
Procysbi	06/09/2013	148,659	[44]
Revatio	28/10/2005	4,482	[45]
Revestive	30/08/2012	200,000 (US estimates)	[46]
Revlimid	14/06/2007	53,363	[47]
Rilonacept Regeneron	23/10/2009	212,000 (US estimates)	[48]
Savene	28/07/2006	6,750 (3 doses)	[49]
Signifor	24/04/2012	47,000	[50]
Siklos	29/06/2007	6,085	[51]
Sirturo	05/03/2014	N/A	N/A
Soliris	20/06/2007	378,000	[52]
Sprycel	20/11/2006	32,000	[53]
Tasigna	19/11/2007	30,000	[54]
Tepadina	15/03/2010	20,608	[55]
Thalidomide Celgene	16/04/2008	25,200	[56]
Tobi Podhaler	20/07/2011	1,540 (28 days)	[57]
Torisel	16/09/2003	36,000	[58]
Tracleer	15/05/2002	19,463	[45]
Ventavis	16/09/2003	38,000	[45]
Vidaza	17/12/2008	45,000	[59]
Volibris	21/04/2008	20,088	[60]
Votubia	02/09/2011	15,000	[61]
Vpriv	26/08/2010	146,660	[62]
Vyndaqel	16/11/2011	112,051	[63]
Wilzin	13/10/2004	726	[64]
Xagrid	16/11/2004	5,000	[65]
Xaluprine	09/03/2012	2,100	[66]
Yondelis	17/09/2007	24,898 (6 cycles)	[67]
Zavesca	20/11/2002	58,400	[68]

*N/A: Not available

**Based on correspondence from manufacturers

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