UK health technology assessment of orphan drugs for rare oncology versus rare disease: Does orphan designation make any difference in HTA process?

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Background

Regulatory authorisation and commitments can vary for rare diseases and rare oncology. Translation of the evidence base and expectations for rigorous health technology assessment pose many challenges.

Objective

To evaluate the success of health technology appraisals for orphan drugs for rare oncology and rare disease.

Methods

Review of NICE and SMC for authorised orphan drugs on the community register to orphan medicinal products up to June 1, 2018.

Results

Of 102 ODs with 121 orphan indications

127 50 RO 77 RD
NICE TAs

136 53 RO 83 RD
SMC TAs

11 NICE Highly Specialised Techology (HST) process applied

40 SMC Patient and Clinician Engagement (PACE) process applied

Lack of TAs or no submissions identified

48% 16% RO 69% RD
NICE

36% 28% RO 41% RD
SMC

43
completed NICE TAs

39 of the completed NICE TAs were recommended with:

27 Patient Access Schemes (PAS)

9 Managed Access Agreements (MAA) with PAS

7 7 (100%) of completed HSTs were recommended
3 PAS, 2 MAA + PAS

71
completed SMC TAs

Of 71 completed SMCs, 54 were recommended/restricted with:

14 PAS

5 PACE

24 PAS + PACE

NICE and SMC agreement

24/25 RO

8/11 RD

Conclusions

The orphan drug (OD) technology appraisal (TA) rate is low with an absence of TAs in half of indications by NICE and a third by SMC. Published SMC TAs steadily increased to plateau in 2016-17, versus an exponential increase with 40% published in 2017 by NICE. NICE is less likely to appraise rare disease (RD) than SMC. As NICE is more selective, the likelihood of recommendation is higher (a quarter of SMC TAs are not recommended). Variation exists on undertaking HTAs, leading to absence of guidance in England and a higher proportion not recommended in Scotland despite PACE being introduced in May 2014. For TAs common to both NICE and SMC the level of consensus is high, but with some disparity for RD. Increased consistency on topic selection, a quicker approval process and more TAs undertaken (particularly for RD) are needed to assess the success of HTA processes for ODs.

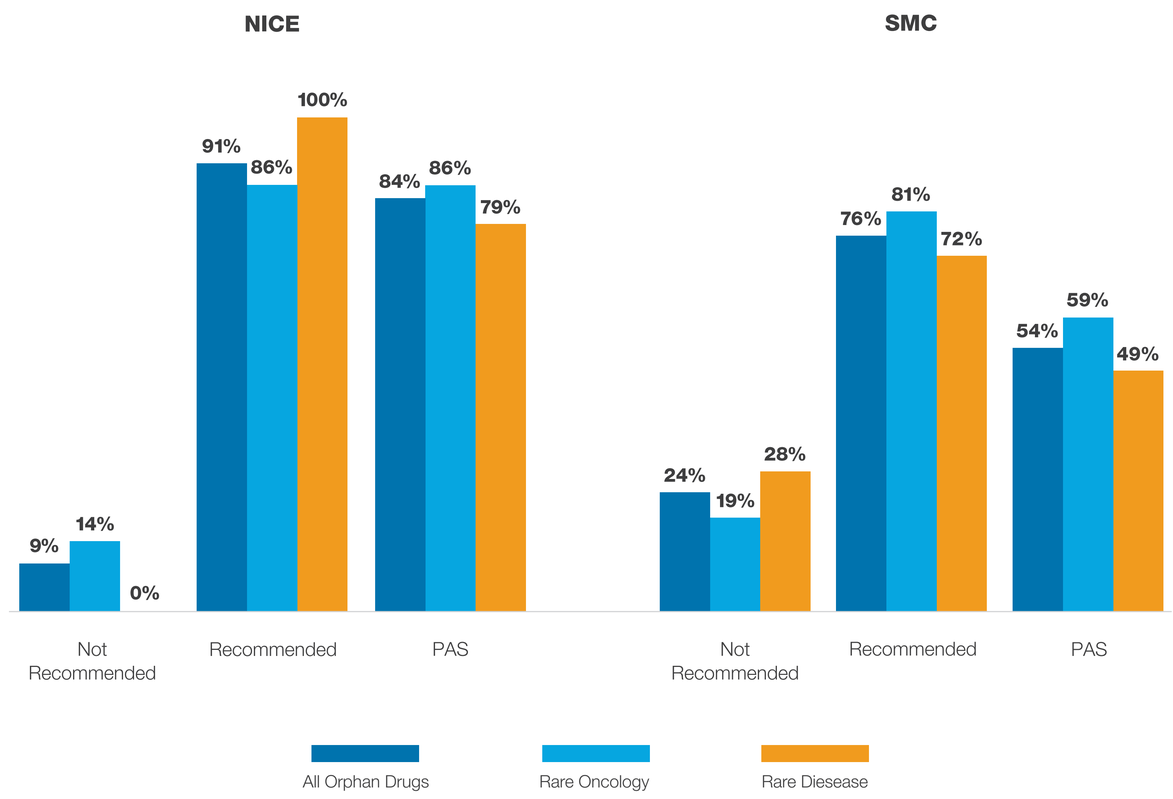
Table 1: HTA outcomes for orphan drugs on the community register

up to June 1, 2018

	All			Rare Oncology			Rare Disease		
NICE	**n**	**N**	**%**	**n**	**N**	**%**	**n**	**N**	**%**
Total HTAs	127	121	105.0%	50	44	113.6%	77	77	100.0%
HST	11	121	9.1%	0	0	0.0%	11	77	14.3%
Specialised Commissioning	6	121	5.0%	0	0	0.0%	6	77	7.8%
Ongoing TA	23	127	18.1%	13	50	26.0%	10	77	13.0%
Suspended TA	7	127	5.5%	5	50	10.0%	2	77	2.6%
No TA	54	127	42.5%	3	50	6.0%	51	77	66.2%
Complete TA	43	127	33.9%	29	50	58.0%	14	77	18.2%
Not Recommended	4	43	9.3%	4	29	13.8%	0	14	0.0%
Recommended	39	43	90.7%	25	29	86.2%	14	14	100.0%
PAS	36	39	92.3%	25	25	100.0%	11	14	78.6%
Managed Access	9	29	31.0%	6	25	24.0%	3	14	21.4%

	All			Rare Oncology			Rare Disease		
SMC	**n**	**N**	**%**	**n**	**N**	**%**	**n**	**N**	**%**
Total HTAs	136	121	112.4%	53	44	120.5%	83	77	107.8%
Ongoing TA	16	136	11.8%	6	53	11.3%	10	83	12.0%
Suspended TA	18	136	13.2%	9	53	17.0%	9	83	10.8%
No TA	31	136	22.8%	6	53	11.3%	25	83	30.1%
Complete TA	71	136	52.2%	32	53	60.4%	39	83	47.0%
Not Recommended	17	71	23.9%	6	32	18.8%	11	39	28.2%
PAS	9	17	52.9%	1	6	16.7%	8	11	72.7%
PACE	11	17	64.7%	3	6	50.0%	8	11	72.7%
Recommended	36	71	50.7%	19	32	59.4%	17	39	43.6%
Restricted	18	71	25.4%	7	32	21.9%	11	39	28.2%
PAS	38	54	70.4%	19	26	73.1%	19	28	67.9%
PACE	29	54	53.7%	18	26	69.2%	11	28	39.3%

Figure 1: HTA recommendations



Abbreviations
ODs = orphan drugs; RD = rare disease; RO = rare oncology; TAs = technology appraisals; PAS = Patient access scheme; MAA = Managed access agreement; PACE = Patient and clinician engagement

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