Reimbursement of ultra-orphan medicines

A new best-practice framework?

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Background

- Since October 2018, ultra-orphan therapies are subject to a new decision-making pathway in Scotland
- Under this pathway, ultra-orphan medicines (definition: prevalence <1:50,000) would be made available for at least 3 years whilst additional evidence is collected to inform a final Scottish Medicines Consortium (SMC) appraisal
- Prior to this, the SMC had an ultra-orphan medicine evaluation process, with a broader decision-making framework versus the standard appraisal process
- This research aims to evaluate the pre-2018 SMC ultra-orphan medicine evaluation process by identifying all drugs assessed under this process and comparing their outcomes with the National Institute for Health and Care Excellence (NICE) (which has the Highly Specialized Technology [HST] program for evaluation of ultra-rare therapies)

Methods

- Publicly-available SMC guidance was screened (13/10/2014-08/04/2019) and any appraisals under the ultra-orphan process identified (alongside any corresponding NICE guidance) and key information extracted

Results

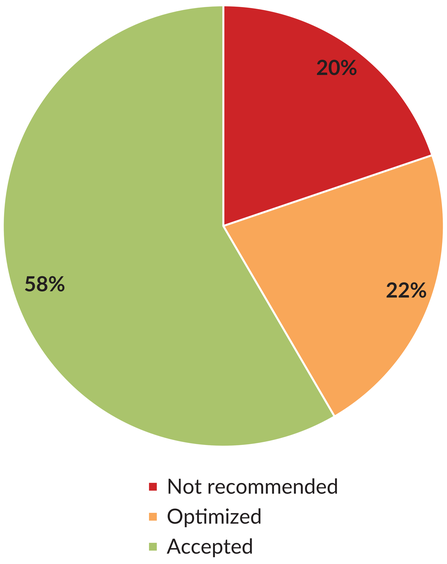
Appraisal numbers

51 SMC ultra-orphan appraisals under the pre-2018 pathway

10 per year on average

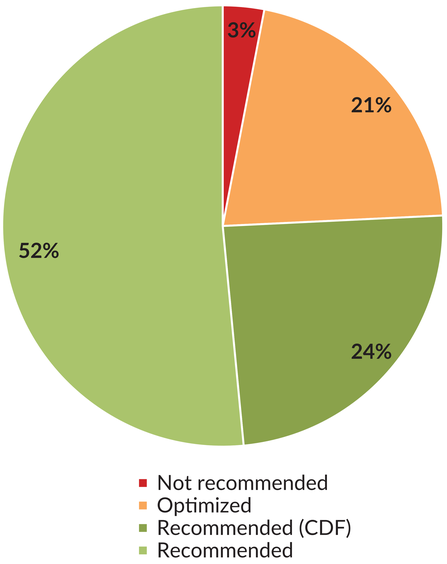
SMC appraisal outcomes

- n = 51



NICE outcomes of these appraisals

- 33/51 (65%) had also been assessed by NICE, (although only four under the HST), with another five appraisals ongoing



Conclusions

- Although recommendation rates under the pre-2018 SMC ultra-orphan process were 80%, most of these had also been assessed by NICE, almost all of which received a positive recommendation
- Further, very few of these NICE appraisals were under the HST, i.e. these high NICE recommendation rates were achieved via assessment under the standard appraisal pathway
- Further research can compare how SMC recommendations of ultra-orphan medicines will evolve according to other countries following the new decision-making pathway

CDF: Cancer Drugs Fund; HST: Highly Specialized Technology; NICE: National Institute for Health and Care Excellence; SMC: Scottish Medicines Consortium

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