Have changes to the SMC process following the Routledge Review really improved access to orphan medications and what factors influence recommendations?


Objectives

The main route to reimbursement in Scotland is through an appraisal by the Scottish Medicines Consortium (SMC). The SMC appraisal process takes between 3 and 6 months and the SMC aims to publish guidance on new products or indications within 3 months of European marketing authorization. Data published by the Office for Health Economics in 2011 showed that non-recommendation from the SMC was more likely for an orphan than a non-orphan product. Following a review of the SMC by Professor Routledge in 2012, the process for appraising end-of-life, orphan and ultra-orphan drugs was modified to facilitate greater access for patients in Scotland. We analyze if the revised SMC process has been successful in providing more positive recommendations for orphan products and which factors may have influenced the decision made by the SMC.

Methods

Analyze were based on a validated, longitudinal MAP BioPharma database of all recommendations from 2002 to 2014. Only products with a EU Orphan designation were analyzed. SMC recommendations following full submission, resubmission or abbreviated submission were reviewed and subdivided into British National Formulary (BNF) categories. In this first stage of analysis, multiple submissions per product per license were not accounted for.

Results

In 2002-2014, there were 8 abbreviated submissions to the SMC for orphan products, 68 full submissions and 25 resubmissions (Figure 1). In 2013, evidence for 7 orphan products was not submitted to the SMC within the required 3 months of marketing authorization thus received automatic negative recommendation. This figure reduced to only one non-submission in 2014. The overall positive recommendation rate (with or without restriction) from 2002-2013 was 52% (n=92); in 2014 this recommendation rate increased to 89% (n=9) (Figure 2).

Resubmissions for orphan products or indications had a higher recommendation rate than full submissions (64% vs. 47%). However, the recommendation rate for orphan products was significantly lower than non-orphan products (55% vs. 73%, p=0.0003).

Up to 2013, malignant disease and immunosuppressive treatments accounted for 43% of the published guidance of orphan products (Figure 3) with a recommendation rate of 50% (n=40); in 2014 this improved to 80% (n=5). Orphan cardiovascular treatments have a historically high recommendation rate of 91% (n=11) which has continued in recent years (Table 1) but treatments for eye (n=1), musculoskeletal and joint (n=3) diseases all received negative recommendations (Figure 3).

Conclusions

The SMC recommendation rate for orphan products improved dramatically in 2014 compared to 2002-2013 suggesting the revised SMC appraisal process may be more effective in enabling the SMC to provide positive recommendations for orphan products. In particular, orphan malignant disease and immunosuppressive products have had a substantially higher probability of positive recommendation following the 2014 changes to the SMC process (Table 1). Manufacturers are also more likely to get a positive recommendation from the SMC following a resubmission than in the original full submission.

![Image](https://www.ohe.org/news/recent-statistics-orphan-approvals-scotland-and-england)

Figure 1: Types of submission evaluated by the SMC for orphan and ultra-orphan products, 2002 to 2014

Figure 2: Recommendation rates from the SMC for orphan and ultra-orphan products, 2002 to 2014

Figure 3: Number of submissions and recommendation rates from the SMC for orphan and ultra-orphan products, 2002 to 2014 by BNF category

Table 1: SMC evaluations [number (% recommended)] for orphan and ultra-orphan products, split by BNF category, 2010 to 2014

<table>
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<tr>
<th>Year</th>
<th>Central nervous system</th>
<th>Eye</th>
<th>Infections</th>
<th>Nutrition and blood</th>
<th>Respiratory system</th>
<th>Malignant, immunosup</th>
<th>Musculoskeletal, joint</th>
<th>Cardiovascular</th>
<th>Gastro-intestinal</th>
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References:
5. SMC recommendations obtained from this SMC website: https://www.scottishmedicines.org.uk/SMCAdvice/Advance_Directory

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