OBJECTIVES: The analysis was conducted to compare trends in recommenda-
tions between the UK and Germany for orphan products, as determined by the Scottish Medicines
Consortium (SMC), the National Institute of Health and Care Excellence (NICE) and the Gemeinsamer
Bundesausschuss (G-BA), and identify disease areas that may be particularly challenging for manufacturers planning European product launch.

METHODS: SMC, NICE and G-BA adopted a common approach to mapping orphan product submissions. However, G-BA provided options for matching recommendations to NICE final guidances, and SMC positive and restricted recommendations, G-BA major, minor and consid-
erable additional benefit decisions, and ‘unable to recommend’. NICE negative
recommendations, SMC negative recommendations and non-submissions, G-BA no-benefit or unquantifiable benefit.

Analysis of products by disease area was conducted by classification into British National Formulary (BNF) categories. RESULTS: SMC, NICE and G-BA have published 1160, 147 and 100 recom-
mandations since their formation. Positive recommendations from NICE/SMC have increased in 2012–2014 (58% to 74%) but decreased from G-BA (50% to 43%).

Treatments for malignant disease and immunosuppression formed the largest categories of submissions (SMC 244, NICE 70, G-BA 34) with higher recommenda-
tion rates in Germany (65%) than the UK (50%). Significant differences in recom-
menations between the UK and Germany were found in endocrine treatments (73% vs. 24%, p=0.00003) and eye treatments (74% vs. 20%, p=0.012).

In 2011–2014, 138 orphan products were assessed by NICE, 46 by SMC and 18 by G-BA. Overall, NICE has recommended more orphan products (67%) than G-BA (63%) or SMC (49%). NICE and SMC recommendations for orphan products have increased in 2014 compared to previous years. CONCLUSIONS: This analysis indicates that the UK market may be easier to access than the German market but the scale of the challenge depends on the BNF category of the treatment. The next stage of analysis will consider trend analysis when accounting for SMCrecommendations and the view of NICE technology appraisals and multiple technology appraisals.