Are Patient Perceptions in Rare Diseases Consistent with Quantitative Indicators of Reimbursement and Healthcare Expenditure in the EU5?

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Objective

- To determine whether rare disease patient perceptions regarding access to treatment are consistent with quantitative indicators of reimbursement and healthcare expenditure across the EU5 countries (UK, France, Spain, Germany and Italy).

Background

- Healthcare reimbursement and expenditure data can help evaluate treatments and policy decisions aimed at improving the health and wellbeing of rare disease patients.
- Patient access to rare disease treatments will be affected by the different healthcare systems and financial circumstances of each individual country **(Table 1 and Figure 1)**.[1,2]
- A report from the UK Office of Health Economics (OHE) evaluating access to orphan medicinal products (OMPs) between 2000 and 2017 found the availability of OMPs varied between the EU5 countries. This included differences in the number of OMPs reimbursed and the wait for reimbursement following marketing authorisation.[3]
- It is important to understand whether these differences are reflected in patient perceptions and if not, to ensure the patient voice is heard when making further decisions that will impact the rare disease patient experience.
- To provide a patient perspective, the European Organisation for Rare Diseases (EURORDIS) Access Campaign developed an online Patient Access Survey, allowing European rare disease patients to report any difficulties they had experienced in accessing treatment.[4]
- The EURORDIS survey collected 1,156 responses across the EU5 between 2000 and 2014 (Germany: 343, Spain: 248, France: 247, Italy: 195 and UK: 123).[5]

Methods

- Quantitative measures of health expenditure and reimbursement indicators (OHE report, 2000–2017)[3] were compared with patient perceptions of access to rare disease treatments (EURORDIS survey, 2000–2014),[4] across the EU5 countries.
- To assess treatment affordability, the percentage of patients stating "I could not afford it (too expensive treatment and care is not well reimbursed)" in the EURORDIS survey was compared with the OHE-reported number of OMPs reimbursed.
- The wait for reimbursement was assessed by comparing the percentage of patients stating "I could not afford to pay in advance and wait for reimbursement" in the EURORDIS survey with the OHE-reported average time to OMP reimbursement.

Results

Treatment affordability is not only about reimbursement

- Of the 143 OMPs with marketing authorisation, different numbers were reimbursed per country (Germany: 133, France: 116, Italy: 84, Spain: 75, and the UK: 57).[3]
- These differences are not reflected in patient opinion; despite Germany reimbursing over twice as many OMPs than the UK, both countries had the fewest patient-reported affordability issues (12.8%), whilst Italian patients reported the most (25.3%) **(Figure 2)**.

Patients can still wait for treatments even after immediate reimbursement

- The length of time between OMP marketing authorisation and reimbursement differed greatly between countries **(Figure 3)**.
- Germany reimburses treatments immediately (0 months); the remaining reimbursement times were 19 (Italy), 20 (France), 23 (Spain) and 27 (UK) months **(Figure 3)**.
- Less than 10% of surveyed patients in each country stated that they had problems with waiting for reimbursement **(Figure 3)**.
- Although the UK had the longest time between marketing authorisation and reimbursement, they had the fewest patient-reported difficulties in wait for reimbursement (3.9%), alongside Italy (3.8%) **(Figure 3)**.
- Despite Germany's rapid reimbursement, Germany and France had the joint highest number of patient-reported difficulties in wait for reimbursement (8.1%) **(Figure 3)**.

Conclusions

- Across the EU5, healthcare expenditure and reimbursement measures were not sufficient to quantify differences in rare disease patient-reported difficulties related to treatment access.
- Further research into healthcare systems, patient engagement and cultural differences may identify why countries with more favourable reimbursement indicators did not necessarily have fewer patient-reported difficulties in accessing treatment.
- The differences identified by this research are intriguing, and should remind us that to understand if policies are really improving the wellbeing of rare disease patients, we must involve the patients themselves.

References

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Author Contributions

Substantial contributions to study conception/design, or acquisition/analysis/interpretation of data: GA, AH, KH, RLF, AG; Drafting of the publication, or revising it critically for important intellectual content: GA, AH, KH, RLF, AG; Final approval of the publication: GA, AH, KH, RLF, AG.

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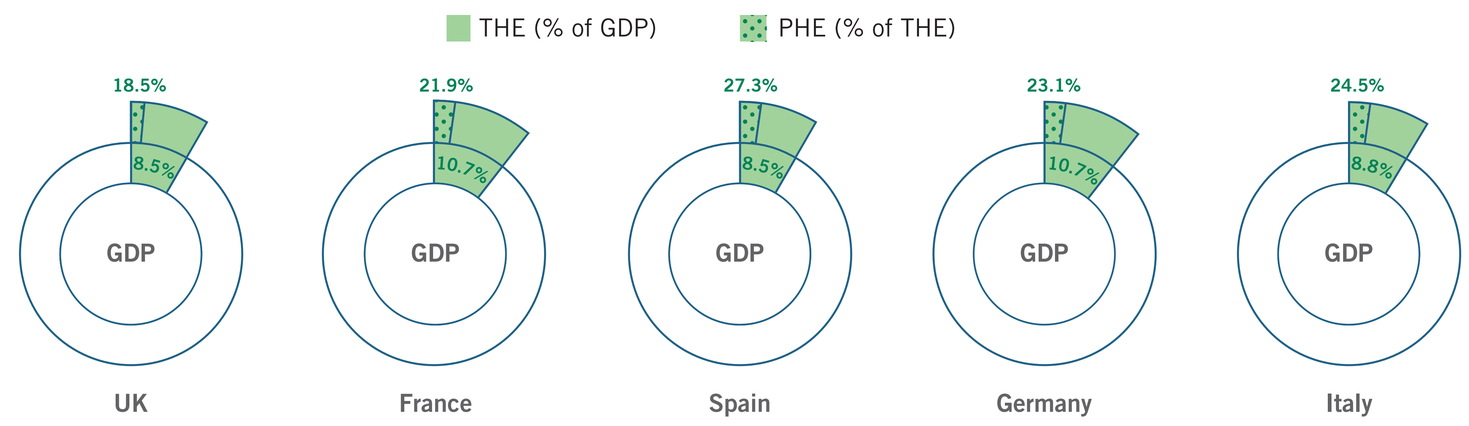
Table 1 | Funding of the EU5 healthcare systems[1,2]

Country	Main Healthcare System	Publicly Funded Health Expenditure (source)		GDP per capita†
UK	National Health Service (NHS) [public health system]	83.5% (taxation)		$38,735
France	French healthcare system [social insurance system]	77.0% (statutory health insurance)		$40,429
Spain	Sistema Nacional de Salud (SNS) [public health system]	71.0% (taxation)		$30,363
Germany	Gesetzliche Krankenversicherung (GKV) [statutory health insurance system]	57.4% (statutory health insurance)	15.5% (statutory long-term care insurance)	$41,006
Italy	Servizio Sanitario Nazionale (SSN) [public health system]	78.2% (taxation)		$36,277

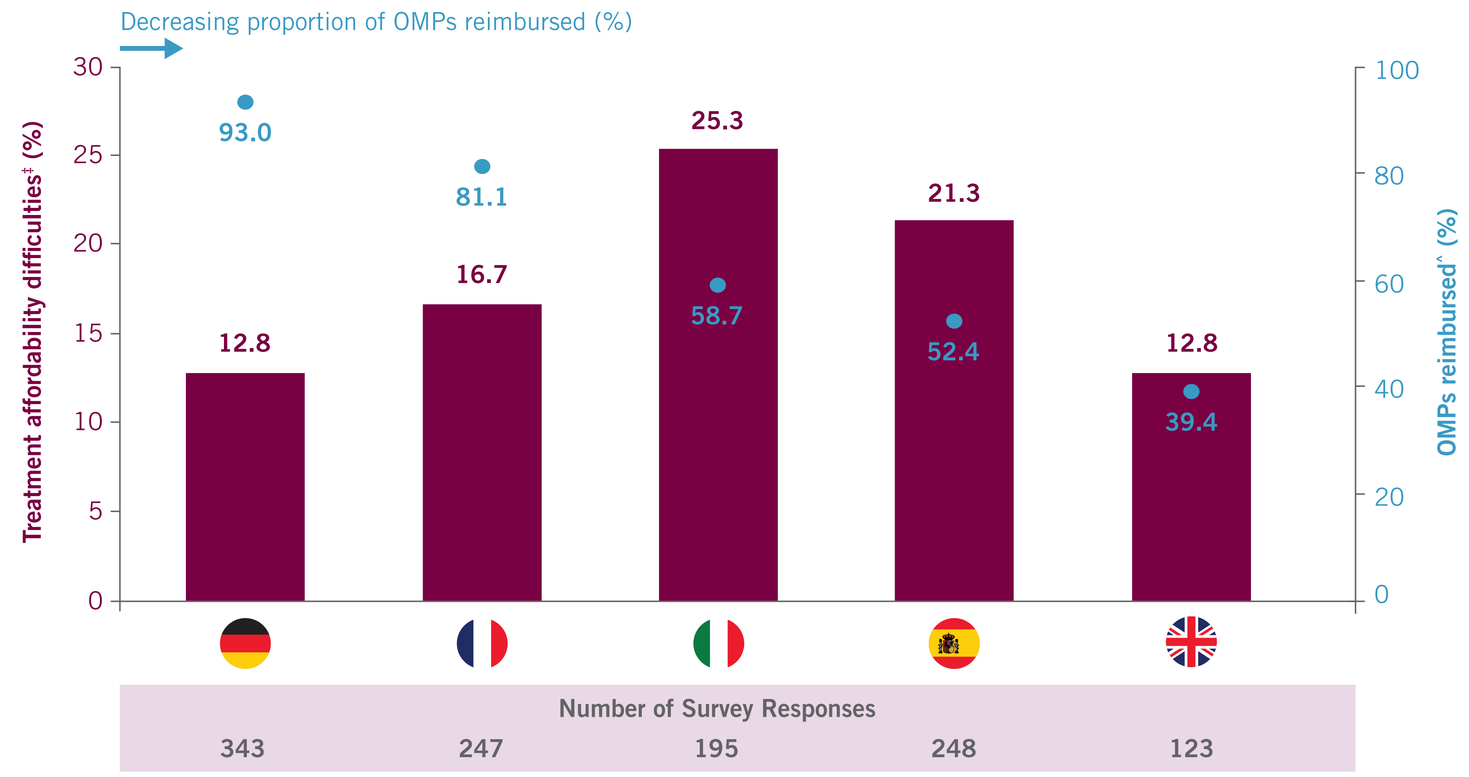
†GDP per capita reported in constant-price 2010 US$. GDP: gross domestic product.

Figure 1 | THE and PHE across the EU5 healthcare systems[2]



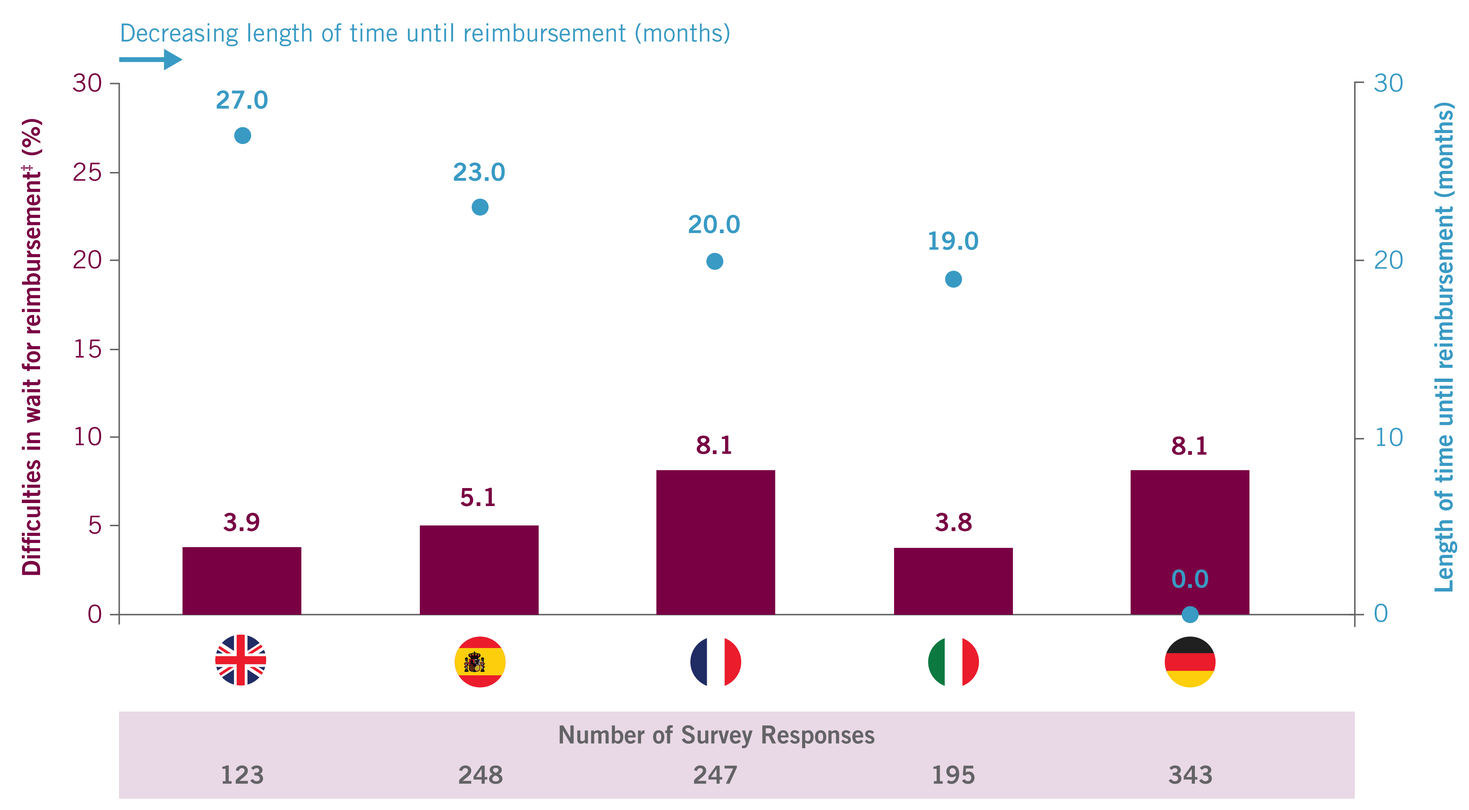
2000–2014 values reported. GDP: gross domestic product; PHE: private health expenditure; THE: total health expenditure.

Figure 2 | Variation across the EU5 in patient-reported treatment affordability and OMP reimbursement indicators



‡Rare disease patient survey responses taken from the 2000–2014 EURORDIS Access Campaign online survey.[4] ^OMPs reimbursed refers to Health Technology Assessment recommendations to use or include in reimbursement lists in respective national health systems.[3] Treatment affordability difficulties correspond to the percentage of survey responses stating "I could not afford it (too expensive treatment and care is not well reimbursed)". OMP reimbursement data sourced from the 2017 OHE "Comparing Access to Orphan Medicinal Products (OMPs) in the United Kingdom and other European Countries" consulting report.[3] Where outcomes were reported separately for England, Scotland and Wales, these were averaged to a single 'UK' value. Northern Ireland data was not reported and not included in the UK average. EURORDIS: European Organisation for Rare Diseases; OHE: Office of Health Economics; OMP: orphan medicinal product.

Figure 3 | Variation across the EU5 in patient-reported wait for reimbursement and time to OMP reimbursement



‡Rare disease patient survey responses taken from the 2000–2014 EURORDIS Access Campaign online survey.[4] Difficulties in wait for reimbursement correspond to the percentage of survey responses stating "I could not afford to pay in advance and wait for reimbursement". Length of time to OMP reimbursement data sourced from the 2017 OHE "Comparing Access to Orphan Medicinal Products (OMPs) in the United Kingdom and other European Countries" consulting report.[3] Where outcomes were reported separately for England, Scotland and Wales, these were averaged to a single 'UK' value. Northern Ireland data was not reported and not included in the UK average. EURORDIS: European Organisation for Rare Diseases; OHE: Office of Health Economics; OMP: orphan medicinal product.

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