Changing Landscape of Orphan Drug Reimbursement: Evidence from EU4 and England

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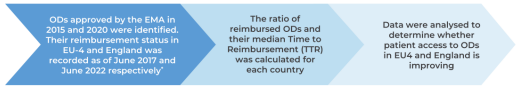
Introduction and Objective

- Orphan Drugs (ODs) are developed to treat rare, life-threatening or chronically debilitating conditions.
- While the European Medicines Agency (EMA) provides incentives to succour and accelerate the approval of Orphan Drugs (ODs) across the European Union,1 pricing and reimbursement (P&R) decisions and overall assessment timelines are subject to local country regulations. Reimbursement decisions are often driven by the outcomes of Health Technology Assessments (HTA) and pricing may be influenced by external reference pricing.
- These P&R differences can affect patient access, potentially creating significant disparities in the availability of new ODs across Europe.
- This study compares the rates of positive reimbursement decisions in Germany, England, France, Italy and Spain for ODs approved by the European Commission (EC) in 2015 and 2020 to determine whether timely patient access to Orphan Drugs (ODs) is improving in these five countries.

Methods

Figure 1: Methodology used in this research



- Time To Reimbursement (TTR) was calculated as a difference between the date of the national approval for reimbursement and the EC decision
- ODs that secured reimbursement after cut-off dates were not included in the TTR calculations to allow comparison across equal time periods
- In calculating the % reimbursement, only the drugs with their evaluation initiated before the time of performing analysis were included

Results

- 12 ODs approved by EMA in 2015 and 22 ODs approved in 2020 were identified. Their reimbursement status at the cut-off dates (June 2017 and June 2022 respectively) and median time to reimbursement in analysed countries are illustrated in Figures 2-7.

Figure 2: Median TTR for ODs approved by the EMA in 2015 and 2020

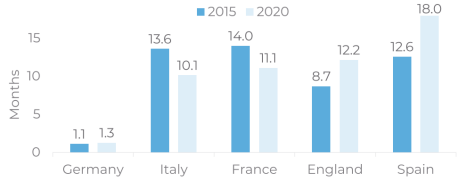
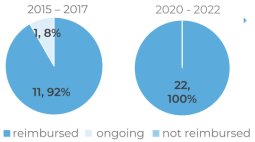
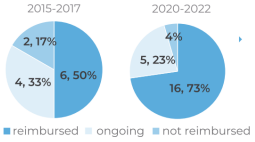


Figure 3: Reimbursement status of EMA-approved ODs in Germany



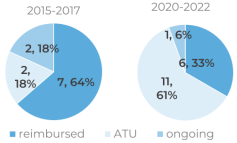
- **Germany** remained the country with the highest percentage of positive recommendations and the shortest median time to reimbursement.

Figure 4: Reimbursement status of EMA-approved ODs in Italy



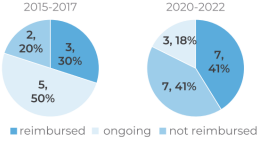
- In **Italy**, the percentage of positive reimbursement decisions increased significantly and the median TTR improved.

Figure 5: Reimbursement status of EMA-approved ODs in France



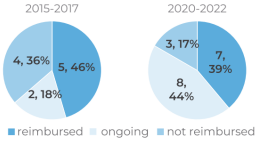
- In **France**, although a decrease in the percentage of reimbursed ODs has been noted, thanks to the implementation of the **Early Access program (ATU)**[2] patients could access respectively **82% (2017) and 94% (2022) of assessed ODs.**

Figure 6: Reimbursement status of EMA-approved ODs in England



- The percentage of positive recommendations improved in **England**, with 3 out of 7 ODs from 2020 being approved through the route for Highly Specialised Technologies (HST). However, a longer TTR than in previous years was noted.

Figure 7: Reimbursement status of EMA-approved ODs in Spain



- The percentage of positive recommendations decreased in **Spain** while the median TTR increased significantly between 2017 and 2022.

Discussion and Conclusion

- Despite the larger volume of ODs approved by EMA in 2020 than in 2015, the reimbursement ratio has increased in the majority of the countries examined, suggesting improving patient access to ODs. However, wide differences in reimbursement decisions and TTR timelines prevail.
- Germany continuously provides the most robust patient access to ODs due to the policy of automatic reimbursement of drugs with orphan designation and the strategy of not limiting access during the price negotiation process.
- The largest improvement in both ratio of approved ODs and the time to reimbursement can be seen in Italy. Progress in TTR corresponds with the legislative changes in the price negotiation process (which now imposes limitations to the clock stop) and COVID-19 mitigating strategies aimed to improve reimbursement decision-making.
- Despite the increased percentage of ODs in the ongoing price negotiation process, wide access to ODs is maintained in France owing to the implementation of the Early Access programme.
- In England, the increased percentage of positive reimbursement decisions coincides with the increased median TTR, presumably due to the higher volume of ODs in the reimbursement process.
- Analysis suggests that Spain, where the authorities frequently oppose reimbursement of medicines involving a major budget impact, remains the most challenging market.
- It is worth noting that one of the causes of the lengthier TTRs could be a delay in the manufacturer's choice to file a P&R dossier after the drug's EMA authorization, which is independent of local authorities.

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*The national reimbursement dates for each product were collected from the official websites of the national agencies: Agenzia Italiana del Farmaco (AIFA), Haute Autorité de Santé (HAS), Gemeinsamer Bundesausschuss (G-BA), The National Institute for Health and Care Excellence (NICE), and Agencia Española de Medicamentos y Productos Sanitarios (AEMPS) and Consejo General de Colegios Oficiales de Farmacéuticos (CGCOF). ATU: Autorisation Temporaire d'Utilisation; EC: European Commission; HST: Highly specialised technology; EMA: European Medicines Agency; OD: Orphan Drug; P&R: pricing and reimbursement; TTR: Time to Reimbursement

References: 1. EMA; Orphan incentives. Available at https://www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation/orphan-incentives [Accessed 25/09/22]
2. . Fontrier, A. (2022). Market access for medicines treating rare diseases: Association between specialised processes for orphan medicines and funding recommendations. Social Science & Medicine, 306, 115119. doi: 10.1016/j.socscimed.2022.115119

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