A comparison of health technology assessment (HTA) timelines and requirements for orphan drugs in ten European countries

HTA117

Andrew Mumford[1,2], Joanne Mumford[1], Jonathan Gibson, Sarah Coyle[1], Georgia Roberts[1], Tom Edmonds[1]

1. Initiate Consultancy, London, UK. 2. Initiate Consultancy, Zug, Switzerland.

SUMMARY

OBJECTIVES

- To analyse timelines in health technology assessment (HTA) of orphan drugs across European countries.
- To analyse the degree of involvement from the Market Authorisation Holder (MAH) in orphan drug HTAs across European countries.

METHODS

- Desktop research using primary (e.g., HTA body websites/reports) and secondary sources (e.g., news articles, scientific journal articles) was used to gather evidence to estimate the average time from submission to a decision being made.
- The analysis included ten countries: Belgium, England, France, Ireland, Italy, Germany, the Netherlands, Scotland, Spain, and Switzerland.

FINDINGS

- The average duration from submission to reimbursement decision was 193 days.
- Germany had the longest HTA process of the countries studied, and Spain had the shortest.
- Belgium, England, Germany, Ireland, and Scotland required the most actions from the MAH.
- The Netherlands and Switzerland required the least involvement from the MAH.

BACKGROUND & AIMS

- Across Europe there is heterogeneity in the HTA process.
- This variation adds another layer of complexity to the already complex process of rolling out an orphan drug.
- This research aimed to evaluate the variation in HTA processes for orphan drugs in Europe, with two main objectives:
  - To analyse the timelines of orphan drug HTA processes across Europe
  - To analyse the degree of involvement from MAHs in these HTA processes

METHODS

- Secondary research was conducted in January 2023.
- Reported timelines for orphan drug HTA processes were investigated, as well as the extent of involvement from the MAH throughout each process.
- Involvement from the MAH was termed an ‘action’.
- The analysis included 10 countries: Belgium, England, France, Ireland, Italy, Germany, the Netherlands, Scotland, Spain, and Switzerland.
- Direct evidence from HTA body websites and reports was used throughout the research alongside secondary evidence from reviews, journal articles, and expert knowledge.

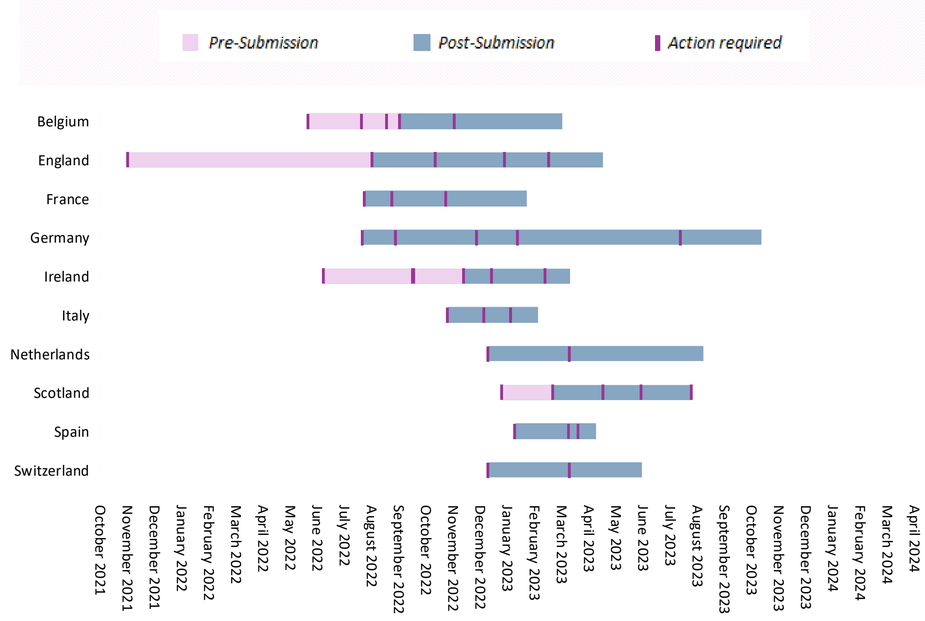
RESULTS

- Information on pre-submission activities was available for four countries: Belgium, England, Ireland, and Scotland.
- The average duration of the HTA process from submission to reimbursement decision of the selected countries is 193 days (excluding pre-submission activities).
- Germany has the longest HTA process overall (441 days) (Figure 1). This can be largely attributed to price negotiations, which can take up to 270 days after a primary decision on the clinical data is made (up to 171 days after initial submission) if no agreement is made in initial negotiations with GKV-SV.
- The country with the shortest HTA process is Spain (90 days), although in practice the Spanish process can take up to 120 days.

Table 1. Reimbursement timeframe and number of actions by country

Country	Time from submission to decision (days)	Number of actions from MAH required (including pre-submission)
Belgium	180	5
England	256	5
France	180	3
Germany	441	5
Ireland	202	5
Italy	100	3
Netherlands	238	2
Scotland	210	5
Spain	90	3
Switzerland	190	2

Figure 3. Comparison of timelines and actions throughout European orphan drug HTA processes

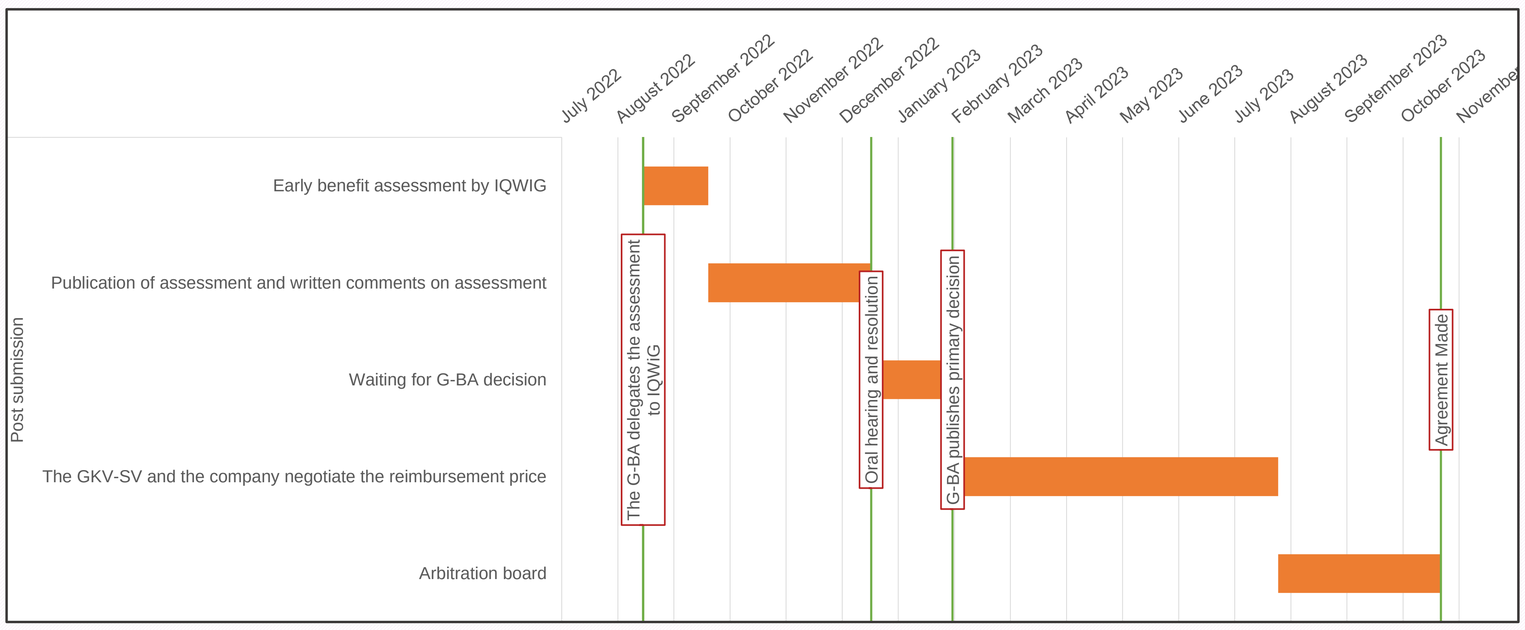


- Belgium, England, Germany, Ireland, and Scotland required the most actions from the MAH, with each country requiring five actions (including pre-submission activities) (Figure 3).
- The Netherlands and Switzerland required the least involvement from the MAH, with only two actions required each (Figure 3).

CONCLUSIONS

- This research highlights the large variations in HTA processes and timelines for orphan drugs across Europe, emphasising the importance of a streamlined market access launch plan.
- Holistic awareness of HTA processes across Europe allows for adequate allocation of resources throughout the market access launch process and can help ensure that patients get timely access to medicines in the rare disease space.

Figure 1. Germany HTA process timeline



References

1. https://www.kce.fgov.be/sites/default/files/2021-11/d20091027332.pdf#
2. www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-highly-specialised-technologies-guidance/HST-interim-methods-process-guide-may-17.pdf
3. https://www.has-sante.fr/upload/docs/application/pdf/2014-03/pricing_reimbursement_of_drugs_and_hta_policies_in_france.pdf
4. https://www.g-ba.de/english/benefitassessment/
5. https://www.ncpe.ie/submission-process/hta-guidelines/
6. https://www.aifa.gov.it/documents/20142/1283800/Linee_guida_dossier_domanda_rimborsabilita.pdf
7. https://www.zorginstituutnederland.nl/over-ons/programmas-en-samenwerkingsverbanden/horizonscan-geneesmiddelen/sluis-voor-dure-geneesmiddelen
8. https://www.scottishmedicines.org.uk/making-a-submission/
9. https://www.aemps.gob.es/medicamentos-de-uso-humano/terapias-avanzadas/?lang=en
10. https://www.bag.admin.ch/bag/de/home/versicherungen/krankenversicherung/krankenversicherung-bezeichnung-der-leistungen/antragsprozesse/AntragsprozessArzneimittel.html/

Presented at ISPOR Europe, 12th-15th November 2023

initiateconsultancy.com *hello@initiateconsultancy.com*