Example Pombiliti®

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Challenges of the PICO Scoping Process: How does the number of involved member states impact results?

Objectives

The PICO framework (Population, Intervention, Comparator, Outcome) will be used to specify the overall assessment scope for the Joint Clinical Assessment (JCA), reflecting all EU Member States needs. Therefore, identifying the expected national PICOs will be key in the EU HTA process. This analysis aims to reproduce the findings of the EUnetHTA-21 PICO exercise, using input from pharmaceutical consultancies covering 16 EU Member States. It also investigates the impact on the results when using only a subsample of different payer archetypes as an option to reduce the workload involved in PICO Scoping.

Methods

For cipaglucosidase alfa (Pombiliti®), used in combination with miglustat for the treatment of adult patients with late-onset Pompe disease (LOPD), national PICOs were identified through a survey conducted across 16 different Member States (Germany, Denmark, France, Italy, Poland, Spain, Ireland, Cyprus, Malta, Greece, Sweden, Norway, Netherlands, Belgium, Luxembourg and Austria), using the EUnetHTA 21 deliverable 4.2 (version 1.0).
The PICOs were consolidated and compared to EUnetHTA 21 PICO exercise (10 unknown Member States) as well as to the PICO results from 8 Member States (Germany, Netherlands, France, Spain, Denmark, Poland, Norway and Sweden), representing different payer archetypes.

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Results

Consolidating the national PICOs from pharmaceutical consultancies, covering 16 EU Member States, resulted in 13 different PICOs, comprising 4 different patient populations for cipaglucosidase alfa. The EUnetHTA 21 PICO exercise resulted in a total of 9 consolidated PICOs (3 different patient populations), of which 8 PICOs could be reproduced with the Scoping Process of this analysis. However, the Scoping Process of this analysis identified 5 additional PICOs related to an additional subpopulation and further comparators, that were not discussed in the EUnetHTA 21 exercise. Reducing the number of Member States' input by considering only 8 Member States, representing different payer archetypes, resulted in only 9 PICOs and thereby ignoring several unique PICOs as well as a complete separate subpopulation from the Scoping Process used for this analysis.

	Scoping Process of this analysis (16 Member States)	EUnetHTA 21 PICO exercise (10 - unknown - Member States)	Payer archetypes (8 Member States)
Adult patients with LOPD	1. Alglucosidase alfa 2. Avalglucosidase alfa 3. Best Supportive Care 4. -	1. Alglucosidase alfa 2. Avalglucosidase alfa 3. Best Supportive Care 4. Physician's choice: – Alglucosidase alfa – Avalglucosidase alfa	1. Alglucosidase alfa 2. Avalglucosidase alfa 3. Best Supportive Care 4. -
Adult patients with LOPD – ERT-naive	5. Alglucosidase alfa 6. Avalglucosidase alfa 7. Best Supportive Care	5. Alglucosidase alfa 6. Avalglucosidase alfa 7. Best Supportive Care	5. Alglucosidase alfa 6. Avalglucosidase alfa 7. Best Supportive Care
Adult patients with LOPD – ERT-experienced	8. Alglucosidase alfa 9. Avalglucosidase alfa 10. Best Supportive Care 11. Individualized treatment taking into account previous therapies, selection of – Alglucosidase alfa – Avalglucosidase alfa	8. Alglucosidase alfa 9. Avalglucosidase alfa 10. - 11. -	8. Alglucosidase alfa 9. Avalglucosidase alfa 10. - 11. Individualized treatment taking into account previous therapies, selection of – Alglucosidase alfa – Avalglucosidase alfa
Adult patients with LOPD – 18-65 years old	12. Total population: Alglucosidase alfa 13. ERT-naive: Alglucosidase alfa 14. ERT-experienced: Alglucosidase alfa	12. - 13. - 14. -	12. - 13. - 14. -
Total number of PICOs	13	9	9

Conclusion

- As LOPD is a rare genetic disorder and only a few medicinal products are authorized in the EU, the input from 16 Member States in the Scoping Process of this analysis resulted in a higher number of PICOs compared to the EUnetHTA 21 exercise, where only 10 Member States participated.
- The input from as many Member States as possible is essential to identify all relevant PICOs for the successful EU HTA dossier preparation. However, involving 30 EU and EEA Member States causes a significant workload. Using a sample of payer archetypes has been suggested as a pragmatic shortcut. This analysis shows the potential shortcomings of such an approach – missing key PICOs and thereby incomplete preparation of the JCA dossier.

References

- EUnetHTA 21, 2023: PICO exercise III – POMBILITI https://www.eunethta.eu/wp-content/uploads/2023/09/EUnetHTA-21-PICO-3-Deliverable.pdf

Abbreviations

ACT: Appropriate comparative therapy, EEA: European Economic Area, ERT: Enzyme-replacement therapy, EU: European Union, EUnetHTA: European Network for Health Technology Assessment, EU HTA: European Health Technology Assessment, JCA: Joint Clinical Assessment, LOPD: Late-onset Pompe disease

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