RAPS 2022 Euro Convergence

Revision of the EU Orphan Drug Legislation – Implications for the German Benefit Assessment?

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INTRODUCTION

Regulation (EC) No 141/2000, EU Orphan Regulation

- Incentives at regulatory level for designated orphan drugs

German AMNOG (2011), Social Code Book V (SGB V)

- Incentives at HTA level (benefit assessment) for approved orphan drugs in accordance with the EU Orphan Regulation

Ongoing review of EU Orphan Regulation: Implications for the benefit assessment of orphan drugs in Germany?

BENEFIT ASSESSMENT IN GERMANY

AMNOG incentives for orphan drugs

- Additional benefit granted if sales at SHI (Statutory Health Insurance) expense below €50 million

Specific assessment at the decision-making body (G-BA)

- Assessment based on pivotal studies
- Appropriate comparator therapy not used for HTA
- Assessment prepared by the G-BA Office for the main part, not by IQWiG

The AMNOG incentives for orphan drugs are under discussion.

HYPOTHETICAL CHANGES

EU Orphan Regulation and Benefit Assessment of Orphan Drugs in Germany

EU Orphan Regulation

- Market exclusivity?
- Eligibility for orphan designation?
- Fee waivers?
- Protocol assistance?
- Aid for research?
- Priority list of neglected diseases?
- EMA-HTA scientific consultation?

Access to Orphan Drugs

German Benefit Assessment

- National incentives in addition to EU?
- National orphan definition?
- Threshold for incentives?
- Re-assessments with RWE/RWD?
- EU Joint Clinical Assessment?
- Managed Entry Agreements?

CONCLUSION

To improve access for patients to innovative drugs, regulatory and HTA stakeholders should

- collaborate closer to develop strategies for drug development and pricing & reimbursement in order to
- anticipate the changes in legal framework for rare diseases.

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