

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

Meriem Bouslouk-Marx, PhD, MSc  
MBM Future Health

## 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



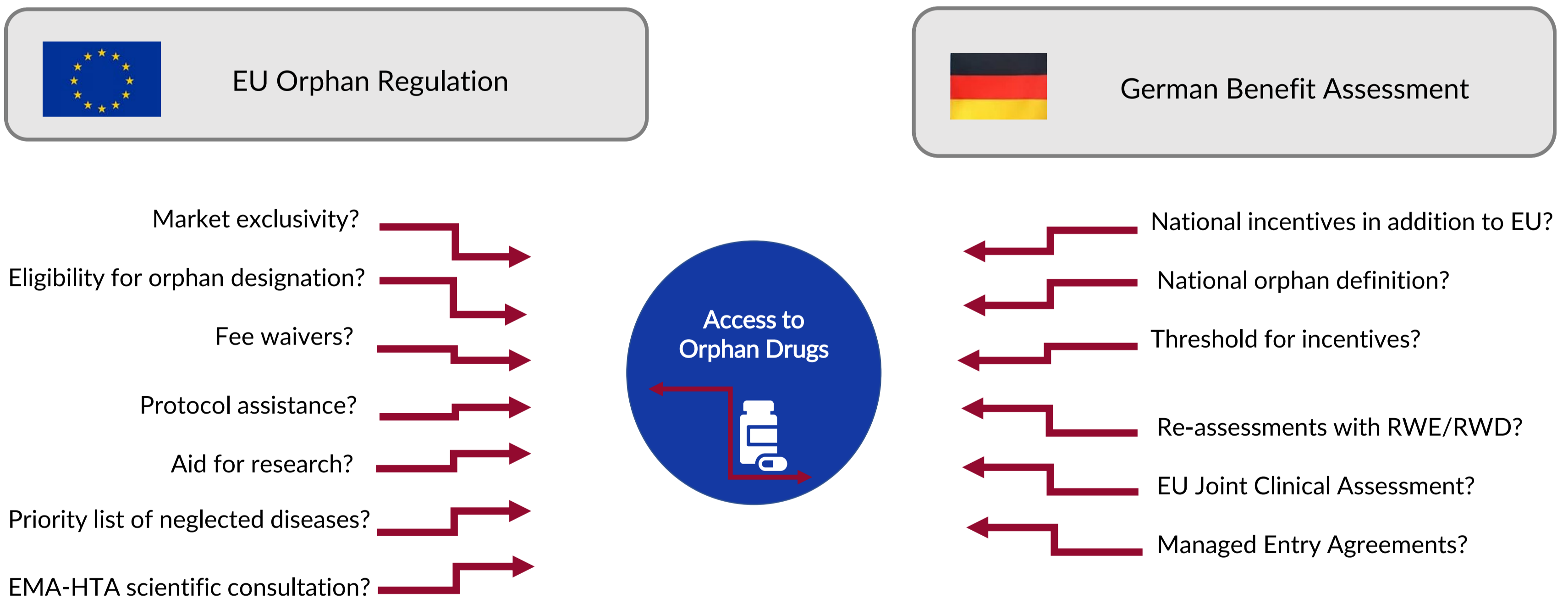
### Specific assessment at the decision-making body (G-BA)

- 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



## 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.