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Editorial

Health Technology Assessments (HTAs) in Europe and similar assessment procedures in other regions such as comparative effectiveness in the United States, are increasingly being used to measure the clinical efficacy and cost effectiveness of health technologies. Ultimately these mechanisms are also in place to control healthcare budget expenditure.

The topic of HTAs has become in recent years increasingly debated in various international fora and it has become obvious that in order for such processes to be transparent, unbiased and carried out in a robust manner, the involvement of key stakeholders such as patient organization representatives, physicians, health economists and industry representatives amongst others is absolutely necessary.

This is particularly relevant in the case of rare diseases therapies. There are indeed many hurdles that come into play when assessing rare diseases therapies such as the unavailability of Randomised Clinical Trials (RCTs) due to the small patient populations and the seemingly high cost of such therapies.

This edition of *Pharmaceuticals Policy and Law* seeks to provide an international overview of these challenges by outlining the viewpoints of key expert stakeholders in the field.

It is hoped that these perspectives will enrich and contribute to the current discussions both at the level of EU institutions and in the United States as well as to the initiatives of platforms such as HTAi and EUnetHTA and other involved organizations. Particularly the need to involve stakeholders and look beyond medical and cost effectiveness factors to include the societal impact of health technologies in the HTA process.

We also hope that the book will be valuable to any person wishing to better understand the issues surrounding the use of HTAs and similar mechanisms in the assessment of rare diseases therapies and will contribute to a better awareness of the key factors that need be taken into account into such processes.

We are grateful to the numerous participating authors including HTA specialist theorists, health economists, patient organizations, rare diseases umbrella organizations, stakeholder platforms, physicians, HTA agency representatives and industry who accepted to present their perspectives on this important topic.

Pharmaceuticals Policy and Law wishes to thank vividly all authors that have made this monograph possible. With our special thanks to the European Commissioner for Health, Mr. John Dalli, for endorsing this important topic and for highlighting the key European health policy developments and initiatives in the Foreword to this volume.

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