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RARE DISEASES AND ORPHAN DRUGS

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Speeding up access to medicines for patients with unmet medical need: Integrating evidence and regulatory pathways

Patients with life-limiting diseases and few or no treatment options want early access to medicines which show potential benefits. At the same time, regulators want reassurance that the potential benefits outweigh the potential risks; that the risks can be managed effectively and that comprehensive evidence will be provided. Getting a balance between these two stakeholders needs can be difficult. This can be compounded by the needs of payers who want evidence of cost-effectiveness. In the context of a rare disease, developing comprehensive evidence for all stakeholders is even more difficult when patients may be relatively scarce. In Europe, the European Medicines Agency (EMA) developed Adaptive Pathways as a means whereby a medicine could receive an initial authorization in a niche indication with a condition that development work would continue and that real world evidence would be gathered by close monitoring of patients receiving the marketed medicine. Regulators, HTA bodies, patients and healthcare practitioners are involved in the development discussions. Access to patients beyond those with most need would be gradually expanded as more evidence became available with the expectation that a “normal” marketing authorization would be achieved. This concept of using a combination of real world evidence along with clinical trials to optimize drug development is being further developed by the Center for Biomedical Innovation, part of the Massachusetts Institute of Technology. The purpose of this presentation is to explain the Adaptive Pathways concept and the multinational discussions which led to it and how early use of real world evidence can help drug development; particularly in the rare disease field. In particular, it will explore the use of disease registries as a means of speeding up trials, providing invaluable information on the natural history of the disease and monitoring patients in the post-approval setting.

Biography

Stella Blackburn has spent over 30 years in Pharmacovigilance and Pharmacoepidemiology in both regulatory and industry environments. She was trained in Medicine at Cambridge and London Universities and has an MSc in Epidemiology from the London School of Hygiene and Tropical Medicine. At the European Medicines Agency, she was responsible for developing risk management for Europe and was the lead author of the guidelines. She is passionate about optimizing the benefit risk balance of medicines and to helping bring medicines to patients with unmet medical needs; particularly those with rare diseases. She is a Visiting Scientist at the Center for Biomedical Innovation, Massachusetts Institute of Technology where she continues to work on innovative methods for bringing medicines to market. She is a Fellow of the Royal College of Physicians of Edinburgh and a Fellow and Past President of the International Society of Pharmacoepidemiology.

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