

References

Chapter 20

Key references/suggested reading are marked with an asterisk.*

- 1 Organisation for Economic Co-operation and Development. Health at a Glance 2011: OECD Indicators. Pharmaceutical expenditure. OECD Publishing. Available from: <http://www.oecd.org/els/health-systems/49105858.pdf> (last accessed 18 November 2015).
- 2 Malmstrom RE, Godman BB, Diogene E, Baumgartel C, Bennie M, Bishop I, et al. Dabigatran – a case history demonstrating the need for comprehensive approaches to optimize the use of new drugs. *Frontiers in Pharmacology*. 2013;**4**:39. *Key reference documenting potential models by which to optimize the utilization of new medicines.
- 3 Godman B, Malmstrom RE, Diogene E, Gray A, Jayathissa S, Timoney A, et al. Are new models needed to optimize the utilization of new medicines to sustain healthcare systems? *Expert Review of Clinical Pharmacology*. 2015;**8**(1):77–94.
- 4 Garuoliene K, Alonderis T, Marcinkevičius M. Pharmaceutical policy and the effects of the economic crisis: Lithuania. *EuroHealth*. 2011;**17**:1–4.
- 5 Experts in Chronic Myeloid Leukemia: Abboud C, Berman E, Cohen A, Cortes J, DeAngelo D, Deininger M, et al. The price of drugs for chronic myeloid leukemia (CML) is a reflection of the unsustainable prices of cancer drugs: from the perspective of a large group of CML experts. *Blood*. 2013;**121**(22):4439–42.
- 6 Kaiser J. Personalized medicine. New cystic fibrosis drug offers hope, at a price. *Science*. 2012;**335**(6069):645.
- 7 Campbell T. Expanding use of Alexion's specialty drug Soliris. Available from: <http://seekingalpha.com/article/1457331-expanding-use-of-alexions-specialty-drug-soliris-offers-upside> (last accessed 18 November 2015).
- 8 European Federation of Pharmaceutical Industries and Associations. Health & Growth: Working Together for a Healthy Europe. A Vision Towards a Life Sciences Strategy for Europe. Brussels, European Federation of Pharmaceutical Industries and Associations, 2014. Available from: http://www.efpia.eu/uploads/documents/EFPIA-health&growth_MANIFESTO_V11_pbp.pdf (last accessed 18 November 2015).
- 9 Lubloy A. Factors affecting the uptake of new medicines: a systematic literature review. *BMC Health Services Research*. 2014;**14**:469.
- 10 Light DW, Lexchin JR. Pharmaceutical research and development: what do we get for all that money? *British Medical Journal*. 2012;**345**:e4348.
- 11 Godman B, Bucsecs A, Burkhardt T, Haycox A, Seyfried H, Wieninger P. Insight into recent reforms and initiatives in Austria: implications for key stakeholders. *Expert Review of Pharmacoeconomics & Outcomes Research*. 2008;**8**(4):357–71.
- 12 Anon. New drugs and indications in 2011. France is better focused on patients' interests after the Mediator scandal, but stagnation elsewhere. *Prescrire International*. 2012;**21**(126):106–7.
- 13 Vitry AI, Shin NH, Vitre P. Assessment of the therapeutic value of new medicines marketed in Australia. *Journal of Pharmaceutical Policy and Practice*. 2013;**6**:2.
- 14 Kantarjian HM, Fojo T, Mathisen M, Zwelling LA. Cancer drugs in the United States: Justum Pretium – the just price. *Journal of Clinical Oncology*. 2013;**31**(28):3600–4.
- 15 Keegan BM. Natalizumab for multiple sclerosis: a complicated treatment. *Lancet Neurology*. 2011;**10**(8):677–8.
- 16 Kappos L, Bates D, Edan G, Eraksoy M, Garcia-Merino A, Grigoriadis N, et al. Natalizumab treatment for multiple sclerosis: updated recommendations for

- patient selection and monitoring. *Lancet Neurology*. 2011;**10**(8):745–58.
- 17 Godman B, Malmstrom RE, Diogene E, Jayathissa S, McTaggart S, Cars T, et al. Dabigatran – a continuing exemplar case history demonstrating the need for comprehensive models to optimize the utilization of new drugs. *Frontiers in Pharmacology*. 2014;**5**:109.
- 18 Larock AS, Mullier F, Sennesael AL, Douxfils J, Devalet B, Chatelain C, et al. Appropriateness of prescribing dabigatran etexilate and rivaroxaban in patients with nonvalvular atrial fibrillation: a prospective study. 2014;**48**(10):1258–68.
- 19 Cohen D. Dabigatran: how the drug company withheld important analyses. *British Medical Journal*. 2014;**349**:g4670.
- 20 Phend C. Monitoring, dose adjustment for Pradaxa? Available from: <http://www.medpagetoday.com/Cardiology/Arrhythmias/46901> (last accessed 18 November 2015).
- 21 Moore TJ, Cohen MR, Mattison DR. Dabigatran, bleeding, and the regulators. *British Medical Journal*. 2014;**349**:g4517.
- 22 Eichler HG, Abadie E, Breckenridge A, Flamion B, Gustafsson LL, Leufkens H, et al. Bridging the efficacy-effectiveness gap: a regulator’s perspective on addressing variability of drug response. *Nature Reviews Drug Discovery*. 2011;**10**(7):495–506.
- 23 Friedman MA, Woodcock J, Lumpkin MM, Shuren JE, Hass AE, Thompson LJ. The safety of newly approved medicines: do recent market removals mean there is a problem? *Journal of the American Medical Association*. 1999;**281**(18):1728–34.
- 24 Ioannides-Demos LL, Piccenna L, McNeil JJ. Pharmacotherapies for obesity: past, current, and future therapies. *Journal of Obesity*. 2011;**2011**:179674.
- 25 McNaughton R, Huet G, Shakir S. An investigation into drug products withdrawn from the EU market between 2002 and 2011 for safety reasons and the evidence used to support the decision-making. *British Medical Journal Open*. 2014;**4**(1):e004221.
- 26 Griffin JP, Griffin TD. The economic implications of therapeutic conservatism. *Journal of the Royal College of Physicians of London*. 1993;**27**(2):121–6.
- 27 Richards MP. Extent and Causes of International Variations in Drug Usage. A Report for the Secretary of State for Health. Available from: https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/216249/dh_117977.pdf (last accessed 18 November 2015).
- 28 Packer C, Simpson S, Stevens A. International diffusion of new health technologies: a ten-country analysis of six health technologies. *International Journal of Technology Assessment in Health Care*. 2006;**22**(4):419–28.
- 29 Mason A. New medicines in primary care: a review of influences on general practitioner prescribing. *Journal of Clinical Pharmacy and Therapeutics*. 2008;**33**(1):1–10.
- 30 Spurling GK, Mansfield PR, Montgomery BD, Lexchin J, Doust J, Othman N, et al. Information from pharmaceutical companies and the quality, quantity, and cost of physicians’ prescribing: a systematic review. *PLoS Medicine*. 2010;**7**(10):e1000352.
- 31 Dunn AG, Braithwaite J, Gallego B, Day RO, Runciman W, Coiera E. Nation-scale adoption of new medicines by doctors: an application of the Bass diffusion model. *BMC Health Services Research*. 2012;**12**:248.
- 32 Garattini S, Bertele V, Godman B, Haycox A, Wettermark B, Gustafsson LL. Enhancing the rational use of new medicines across European health care systems. *European Journal of Clinical Pharmacology*. 2008;**64**(12):1137–8.
- 33 Chauhan D, Mason A. Factors affecting the uptake of new medicines in secondary care – a literature review. *Journal of Clinical Pharmacy and Therapeutics*. 2008;**33**(4):339–48.
- 34 Bjorkhem-Bergman L, Andersen-Karlsson E, Laing R, Diogene E, Melien O, Jirlow M, et al. Interface management of pharmacotherapy. Joint hospital and primary care drug recommendations. *European Journal of Clinical Pharmacology*. 2013;**69**(Suppl. 1):73–8.
- 35 Vogler S, Zimmermann N, Habl C, Mazag J. The role of discounts and loss leaders in medicine procurement in Austrian hospitals – a primary survey of official and actual medicine prices. *Cost Effectiveness and Resource Allocation*. 2013;**11**(1):15.
- 36 Spatz I, McGee N. Health policy brief: specialty pharmaceuticals. *Health Affairs*. 25 November 2013:1–4.
- 37 Litchtenberg F. Pharmaceutical innovation and longevity growth in 30 developing and high-income countries, 2000–2009. *Health Policy and Technology*. 2014;**3**:36–58.

- 38 Ferrario A, Kanavos P. Managed entry agreements for pharmaceuticals: the European experience. Available from: <http://core.ac.uk/download/pdf/16379320.pdf> (last accessed 18 November 2015).
- 39 Cheema PK, Gavura S, Migus M, Godman B, Yeung L, Trudeau ME. International variability in the reimbursement of cancer drugs by publicly funded drug programs. *Current Oncology*. 2012;**19**(3):e165–76.
- 40 Adamski J, Godman B, Ofierska-Sujkowska G, Osinska B, Herholz H, Wendykowska K, et al. Risk sharing arrangements for pharmaceuticals: potential considerations and recommendations for European payers. *BMC Health Services Research*. 2010;**10**:153.
- 41 Klemp M, Fronsdal KB, Facey K. What principles should govern the use of managed entry agreements? *International Journal of Technology Assessment in Health Care*. 2011;**27**(1):77–83.
- 42 Jommi C. Managed market entry for drugs in Italy. *EMAUD Market Access Newsletter 2012*. June 2012.
- 43 Liner L. Recent development for CED in Sweden. *EMAUD Market Access Newsletter 2012*. June 2012.
- 44 PharmaFutures. PharmaFutures Global Conclusions. Pathways to Value: Pharma in a Changing World. Available from: <http://apps.who.int/medicinedocs/documents/s20202en/s20202en.pdf> (last accessed 18 November 2015).
- 45 Seiter A. A Practical Approach to Pharmaceutical Policy. Washington, DC, World Bank, 2010. Available from: <https://openknowledge.worldbank.org/bitstream/handle/10986/2468/552030PUB0Phar10Box349442B01PUBLIC1.pdf?sequence=4> (last accessed 18 November 2015). *Provides a good practical approach to pharmaceutical policy.
- 46 Van Minh H, Kim Phuong NT, Saksena P, James CD, Xu K. Financial burden of household out-of-pocket health expenditure in Viet Nam: findings from the National Living Standard Survey 2002–2010. *Social Science & Medicine*. 2013;**96**:258–63.
- 47 Barber SL, Huang B, Santoso B, Laing R, Paris V, Wu C. The reform of the essential medicines system in China: a comprehensive approach to universal coverage. *Journal of Global Health*. 2013;**3**(1):010303.
- 48 Meng Q, Xu L, Zhang Y, Qian J, Cai M, Xin Y, et al. Trends in access to health services and financial protection in China between 2003 and 2011: a cross-sectional study. *Lancet*. 2012;**379**(9818):805–14.
- 49 Li X, Zhang W. The impacts of health insurance on health care utilization among the older people in China. *Social Science & Medicine*. 2013;**85**:59–65.
- 50 Putrik P, Ramiro S, Kvien TK, Sokka T, Pavlova M, Uhlig T, et al. Inequities in access to biologic and synthetic DMARDs across 46 European countries. *Annals of the Rheumatic Diseases*. 2014;**73**(1):198–206. *Documents differences in the utilization of anti-TNF alpha medicines across Europe based on such issues as prescribing restrictions and patient copayments.
- 51 Godman B, Paterson K, Malmstrom RE, Selke G, Fagot JP, Mrak J. Improving the managed entry of new medicines: sharing experiences across Europe. *Expert Review of Pharmacoeconomics & Outcomes Research*. 2012;**12**(4):439–41.
- 52 Wettermark B. The intriguing future of pharmacoepidemiology. *European Journal of Clinical Pharmacology*. 2013;**69**(Suppl. 1):43–51.
- 53 Wettermark B, Godman B, Eriksson C, van Ganse E, Garattini S, Joppi R, et al. Einführung neuer Arzneimittel in europäische Gesundheitssysteme [Introduction of new medicines into European healthcare systems]. *GGW*. 2010;**10**(3):24–34.
- 54 Wild C, Langer T. Emerging health technologies: informing and supporting health policy early. *Health Policy*. 2008;**87**(2):160–71.
- 55 Wild C, Simpson S, Douw K, Geiger-Gritsch S, Mathis S, Langer T. Information service on new and emerging health technologies: identification and prioritization processes for a European union-wide newsletter. *International Journal of Technology Assessment in Health Care*. 2009;**25**(Suppl. 2):48–55.
- 56 Packer C, Fung M, Stevens A. Analyzing 10 years of early awareness and alert activity in the United Kingdom. *International Journal of Technology Assessment in Health Care*. 2012;**28**(3):308–14.
- 57 Joppi R, Dematte L, Menti AM, Pase D, Poggiani C, Mezzalana L. The Italian Horizon Scanning Project. *European Journal of Clinical Pharmacology*. 2009;**65**(8):775–81. *Gives a good background to the development of the Italian HSP and its activities.
- 58 Wettermark B, Persson ME, Wilking N, Kalin M, Korkmaz S, Hjemdahl P, et al. Forecasting drug utilization and expenditure in a metropolitan health region. *BMC Health Services Research*. 2010;**10**:128. *Provides a good background to the development of forecasting activities within the Stockholm region of Sweden.

- 59 Nachtnebel A, Geiger-Gritsch S, Hintringer K, Wild C. Scanning the horizon: development and implementation of an early awareness system for anticancer drugs in Austria. *Health Policy*. 2012;**104**(1):1–11.
- 60 Gutiérrez-Ibarluzea I, Simpson S, Joppi R, Kearney B, Packer C, Klemp M, et al. *A Toolkit for the Identification and Assessment of New and Emerging Health Technologies*. Birmingham, EuroScan, 2009.
- 61 Martino OI, Ward DJ, Packer C, Simpson S, Stevens A. Innovation and the burden of disease: retrospective observational study of new and emerging health technologies reported by the EuroScan Network from 2000 to 2009. *Value in Health*. 2012;**15**(2):376–80.
- 62 Develop, Innivate, Advance (DIA) website: www.diahome.org.
- 63 Medscape website: www.medscape.com.
- 64 Packer C. The National Horizon Scanning Centre (NHSC): early warning for new and emerging health technologies in England. *Evidence-Based Healthcare & Public Health*. 2005;**9**:410–13.
- 65 Campbell SM, Godman B, Diogene E, Furst J, Gustafsson LL, MacBride-Stewart S, et al. Quality indicators as a tool in improving the introduction of new medicines. *Basic & Clinical Pharmacology & Toxicology*. 2015;**116**(2):146–57.
- 66 Hoffman JM, Shah ND, Vermeulen LC, Doloresco F, Grim P, Hunkler RJ, et al. Projecting future drug expenditures – 2008. *American Journal of Health System Pharmacy*. 2008;**65**(3):234–53.
- 67 Godman B, Wettermark B, Hoffmann M, Andersson K, Haycox A, Gustafsson LL. Multifaceted national and regional drug reforms and initiatives in ambulatory care in Sweden: global relevance. *Expert Review of Pharmacoeconomics & Outcomes Research*. 2009;**9**(1):65–83.
- 68 Rogers EM. Lessons for guidelines from the diffusion of innovations. *Joint Commission Journal on Quality Improvement*. 1995;**21**(7):324–8.
- 69 Joppi R, Pase D, Poggiani C. Italian Horizon Scanning Project. *Giornale Italiano di Health Technology Assessment*. 2013;**6**(1):11–21.
- 70 Joppi R, Cinconze E, Mezzalira L, Pase D, Poggiani C, Rossi E, et al. Hospitalized patients with atrial fibrillation compared to those included in recent trials on novel oral anticoagulants: a population-based study. *European Journal of Internal Medicine*. 2013;**24**(4):318–23. *Illustrates potential differences that can exist between patients enrolled into phase III studies and those treated in clinical practice.
- 71 Reilly PA, Lehr T, Haertter S, Connolly SJ, Yusuf S, Eikelboom JW, et al. The effect of dabigatran plasma concentrations and patient characteristics on the frequency of ischemic stroke and major bleeding in atrial fibrillation patients: the RE-LY Trial (Randomized Evaluation of Long-Term Anticoagulation Therapy). *Journal of the American College of Cardiology*. 2014;**63**(4):321–8.
- 72 Godman B, Campbell S, Suh HS, Finlayson A, Bennie M, Gustafsson L. Ongoing measures to enhance prescribing efficiency across Europe: implications for other countries. *Journal of Health Technology Assessment*. 2013;**1**:27–42.
- 73 Paris V, Belloni A. Value in Pharmaceutical Pricing. OECD Health Working Papers, No. 63. Washington, DC, OECD Publishing, 2013. Available from: <http://dx.doi.org/10.1787/5k43jc9v6knx-en> (last accessed 18 November 2015). *Provides a comprehensive summary of the different approaches to the pricing and reimbursement of pharmaceuticals across countries, including comparators and outcome measures.
- 74 International Society for Pharmacoeconomics and Outcomes Research. Report of the ISPOR Task Force on Good Research Practices – Budget Impact Analysis. Available from: http://www.ispor.org/workpaper/BudgetImpactAnalysis/BIA_TF0906.asp (last accessed 18 November 2015).
- 75 van de Vooren K, Duranti S, Curto A, Garattini L. A critical systematic review of budget impact analyses on drugs in the EU countries. *Applied Health Economics and Health Policy*. 2014;**12**(1):33–40.
- 76 Morel T, Arickx F, Befrits G, Siviero P, van der Meijden C, Xoxi E, et al. Reconciling uncertainty of costs and outcomes with the need for access to orphan medicinal products: a comparative study of managed entry agreements across seven European countries. *Orphanet Journal of Rare Diseases*. 2013;**8**:198.
- 77 European Medicines Agency. Guideline on Safety and Efficacy Follow-Up – Risk Management of Advanced Therapy Medicinal Products 2008. Available from: http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/10/WC500006326.pdf (last accessed 18 November 2015).
- 78 European Medicines Agency. CHMP Recommendations for the Pharmacovigilance Plan as Part of

- the Risk Management Plan to be Submitted with the Marketing Authorisation Application for a Pandemic Influenza Vaccine. Available from: http://www.ema.europa.eu/docs/en_GB/document_library/Report/2010/01/WC500051739.pdf (last accessed 18 November 2015).
- 79** Wettermark B, Godman B, Jacobsson B, Haaijer-Ruskamp FM. Soft regulations in pharmaceutical policy making: an overview of current approaches and their consequences. *Applied Health Economics and Health Policy*. 2009;**7**(3):137–47.
- 80** Ratner J, Mullins D, Buesching DP, Cantrell RA. Pragmatic clinical trials: US payers' views on their value. *American Journal of Managed Care*. 2013;**19**(5):e158–65.
- 81** VanLare JM, Wong HH, Gibbs J, Timp R, Whang S, Worrall C, et al. Comparative effectiveness research in practice: the Drug Effectiveness Review Project experience. *Journal of Comparative Effectiveness Research*. 2013;**2**(6):541–50.
- 82** Berkowitz SA, Pahira JJ. Accountable care organization readiness and academic medical centers. *Academic Medicine*. 2014;**89**(9):1210–15.
- 83** Amara S, Adamson RT, Lew I, Slonim A. Accountable care organizations: impact on pharmacy. *Hospital Pharmacy*. 2014;**49**(3):253–9.
- 84** Edwards ST, Bitton A, Hong J, Landon BE. Patient-centered medical home initiatives expanded in 2009–13: providers, patients, and payment incentives increased. *Health Affairs*. 2014;**33**(10):1823–31.
- 85** Godman B, Persson M, Miranda J, Skiold P, Wettermark B, Barbui C, et al. Changes in the utilization of venlafaxine after the introduction of generics in Sweden. *Applied Health Economics and Health Policy*. 2013;**11**(4):383–93.
- 86** Leopold C, Vogler S, Mantel-Teeuwisse AK, de Joncheere K, Leufkens HG, Laing R. Differences in external price referencing in Europe: a descriptive overview. *Health Policy*. 2012;**104**(1):50–60.
- 87** Troncoso A, Diogene E. Dabigatran and rivaroxaban prescription for atrial fibrillation in Catalonia, Spain: the need to manage the introduction of new drugs. *European Journal of Clinical Pharmacology*. 2014;**70**(2):249–50.
- 88** Huisman MV, Lip GY, Diener HC, Dubner SJ, Halperin JL, Ma CS, et al. Design and rationale of Global Registry on Long-Term Oral Antithrombotic Treatment in Patients with Atrial Fibrillation: a global registry program on long-term oral antithrombotic treatment in patients with atrial fibrillation. *American Heart Journal*. 2014;**167**(3):329–34.
- 89** Piccinni C, Raschi E, Poluzzi E, Puccini A, Cars T, Wettermark B, et al. Trends in antiarrhythmic drug use after marketing authorization of dronedarone: comparison between Emilia Romagna (Italy) and Sweden. *European Journal of Clinical Pharmacology*. 2013;**69**(3):715–20.
- 90** van Vollenhoven RF, Askling J. Rheumatoid arthritis registries in Sweden. *Clinical and Experimental Rheumatology*. 2005;**23**(5 Suppl. 39):S195–200.
- 91** Raaschou P, Simard JF, Holmqvist M, Askling J. Rheumatoid arthritis, anti-tumour necrosis factor therapy, and risk of malignant melanoma: nationwide population based prospective cohort study from Sweden. *British Medical Journal*. 2013;**346**:f1939.
- 92** Forslund T, Raaschou P, Hjemdahl P, Krakau I, Wettermark B. Usage, risk, and benefit of weight-loss drugs in primary care. *Journal of Obesity*. 2011;**2011**:459263. *Study demonstrating the value of patient follow-up to provide future guidance.
- 93** Gustafsson LL, Wettermark B, Godman B, Andersen-Karlsson E, Bergman U, Hasselstrom J, et al. The 'wise list' – a comprehensive concept to select, communicate and achieve adherence to recommendations of essential drugs in ambulatory care in Stockholm. *Basic & Clinical Pharmacology & Toxicology*. 2011;**108**(4):224–33.
- 94** Martikainen JE, Saastamoinen LK, Korhonen MJ, Enlund H, Helin-Salmivaara A. Impact of restricted reimbursement on the use of statins in Finland: a register-based study. *Medical Care*. 2010;**48**(9):761–6.
- 95** Van Hoof TJ, Miller NE, Meehan TP. Do published studies of educational outreach provide documentation of potentially important characteristics? *American Journal of Medical Quality*. 2013;**28**(6):480–4.
- 96** O'Brien MA, Rogers S, Jamtvedt G, Oxman AD, Odgaard-Jensen J, Kristoffersen DT, et al. Educational outreach visits: effects on professional practice and health care outcomes. *Cochrane Database of Systematic Reviews*. 2007(4):Cd000409.