Institution: University of York

Unit of Assessment: 18, Economics and Econometrics

Title of case study: Value based pricing for new pharmaceuticals

1. Summary of the impact

The NHS spends about £11bn annually on pharmaceuticals, of which £8bn is on branded drugs, representing about 13% and 10% respectively of available NHS resources. Research at York has been central to the public and policy debate about how branded pharmaceuticals ought to be priced and has made a material contribution to the development of government policy to introduce a value based pricing (VBP) scheme for all new pharmaceuticals. VBP has significance for the prices that the NHS pays for pharmaceuticals, access to new drugs for NHS patients, and the return that manufacturers can expect from future research and development. There is also an international impact in two respects: UK prices are estimated to influence 25% of the world market and York has contributed to a wider policy debate about international pharmaceutical pricing and the potential role of value based pricing in European, North American, South American and South East Asian health care systems.

2. Underpinning research

The key insight from the underpinning research [see references 1 and 2 in Section 3] was to show how the type of economic evaluation of health technologies already undertaken for the National Institute for Health and Care Excellence (NICE) can be used to identify the maximum price the NHS can afford to pay for a new drug; signalling the collective demand for branded pharmaceuticals. The work details how a VBP scheme can be specified in a way that mirrors competitive markets in other sectors where innovation is protected by patent and protecting manufacturers from the potential exploitation of monopsony power by bodies like NICE, while ensuring that value accrues to the NHS following patent expiry and entry of generics. It demonstrates how the type of cost-effectiveness analysis conducted for NICE, can be used to align incentives for manufacturers with the objectives and resource constraints of a collectively funded health care system like the NHS.

This research makes clear that an estimate of the shadow price of the NHS budget constraint (the cost-effectiveness threshold or the health displaced by additional NHS costs), is central to any assessment of the price the NHS can afford to pay for a new drug. Underpinning research at York [3], demonstrated how national data, which identifies expenditure by broad programmes of care, can be linked to mortality outcomes and used to estimate the relationship between expenditure and health outcomes, while addressing issues of endogeneity by identifying and testing suitable instruments. This work was further developed through MRC/NIHR funding [6] to estimate expenditure and outcome equations across all 23 programmes of NHS care; capturing the effects of changes in expenditure on length and quality of life [4]. This research also identifies where (by disease area) and what types of health outcomes (mortality and quality of life effects) are likely to be lost as a consequence of additional NHS costs and for whom (by age and gender). It provides the analytic and evidential foundation to incorporate a range of values that can be attached to different types of health effects (gained or displaced), as well as the wider consumption effects associated with them. Research, commissioned by the Department of Health (DH), also demonstrates how wider consumption effects can be valued relative to constrained NHS resources and health effects [7].

Research at York [1, 5] has also shown how uncertainty and the value of additional evidence about the performance of a technology ought to influence approval and pricing decisions. This was developed in MRC/NIHR funded research, which demonstrated how NICE methods of appraisal can be extended to take account of the value of additional evidence and the commitment of irrecoverable costs on a decision to approve a technology at launch when its performance is uncertain [8]. At the request of the Department of Health this work was extended as part of the Economic Evaluation Policy Research Unit (EEPRU) [9] to inform how the VBP scheme might include these considerations.

Researchers at York : Claxton K (Professor, Oct 1989-); Culyer AJ (Professor, 1969-); Rice N (Professor; 1994-); Martin S (Senior RF; 1989-); Sculpher M (Professor, Nov 1997-); Palmer S (Professor; April 1995-); Smith PC (Professor, 1984-Sep 2009); Griffin S (Senior RF; Oct 2002-) ; Soares M (RF, 2007-); Hind S (RF, Oct 2010-); Spackman E (RF, Feb 2010-) and Walker S (RF; Oct 2006-).

3. References to the research

Peer reviewed publications:

[1] Claxton K. OFT, VBP: QED? *Health Economics* 2007, 16:545-558, DOI: 10.1002/hec.1249, (submitted to RAE 2008 where 96.6% of Departmental outputs were rated 2* or higher).

[2] Claxton K, et al. Value-Based Pricing for NHS Drugs: an Opportunity Not to be Missed? *British Medical Journal* 2008, 336: 251-254. DOI: 10.1136/bmj.39434.500185.25. Citations: 68

[3] Martin S, Rice N, Smith PC. Does health care spending improve health outcomes? Evidence from English programme budgeting data. *Journal of Health Economics* 2008 27:826–42. DOI: 10.1016/j.jhealecon.2007.12.002. Citations: 33.

[4] Claxton, K. P., Martin, S., O Soares, M., Rice, N., Spackman, E., Hinde, S., Devlin, N., Smith, P. C. & Sculpher, M. Methods for the Estimation of the NICE Cost Effectiveness Threshold. 2013, *Health Technology Assessment* (accepted for publication). Working paper published as CHE Research Paper 81 (January 2013, 15162 downloads). Available on request

[5] Griffin S, Claxton K, Palmer S, Sculpher M. Dangerous omissions: the consequences of ignoring decision uncertainty. *Health Economics* 2011, 20:212-24, DOI: 10.1002/hec.1586, Citations: 22 (ISPOR Excellence Award for Methodology Excellence, 2012).

All citation counts are from Scopus, taken on 26/09/2013

Grants supporting the research:

[6] Sculpher M., Claxton K., Rice N., Martin S., Devlin N. Methods for estimation of NICE's cost-effectiveness threshold. *Medical Research Council* and *National Institute for Health Research* from 2010 to 2012. Total funding of £420,000 based at the University of York. Completed 2012.

[7] Sculpher M. Claxton K and Palmer S. Informing choices about NICE's cost perspective. *Department of Health*, 2009. Total funding of £60,000 based at the University of York. Competed 2009 and the report published as CHE Research Paper 54 (January 2010, 1106 downloads).

[8] Claxton K. Palmer S and Longworth L. Informing a decision framework for when NICE should recommend the use of health technologies only in the context of an appropriately designed programme of evidence development. *Medical Research Council*, from 2010 to 2011. Total funding of £285,000 based at the University of York. Completed 2011.

[9] Brazier J., Sculpher M., Claxton K., Dixon S., Lloyd-Jones M., Palmer S., Paisley S., Rice N., Tappenden P., Wailoo A. and Weatherley H. Department of Health Policy Research Unit: economic evaluation of health and social care interventions. *Department of Health* 2011-2016. Total funding of £5 million, £2.2 million based at York. Ongoing.

4. Details of the impact

The question of what prices ought to be paid for new pharmaceuticals is especially critical for the NHS, given current resource constraints; for pharmaceutical manufacturers, concerned that the prices paid by the NHS offer sufficient return for continued research and development; and patients groups and clinicians, concerned that they will have access to new drugs at launch. Researchers at the University of York have been central to this public and policy debate since contributing to an Office of Fair Trading (OFT) Report in 2007. They were consulted during its preparation and were invited by OFT to participate in its launch and other public debates during the consultation period following its publication, including giving evidence to the House of Commons Health Committee (HCHC) investigation of NICE. The underpinning research also informed the subsequent HCHC investigation of top-up fees [1].

Since 2008, and especially since the Government announced the introduction of a VBP scheme in 2010, this impact on public and policy debate has grown [2-3, 20-21]. This is evidenced by invited presentations at public debates, roundtable stakeholder discussions and advisory boards (e.g., Westminster Health Forum Keynote Seminars, October 2010 and 2013; House of Commons dinner debate, February 2011; PharmaTimes debate, April, 2011; and Myeloma UK Symposium, November 2011). Invitations to present and advise research consultancy organisations (e.g., Mapi Values, April 2008, IMS Health, June 2011, Heron, May 2012), manufacturers (Sanofi Aventis, February 2011, Roche Pharmaceuticals, London, February 2011, May, July and December 2012) and national bodies which represent the interest of the sector (Association of British Pharmaceutical Industries, February 2011 and February 2012) also evidence the impact of this research. The research has also had an impact on the international debate about pharmaceutical pricing [5, 6] and has influenced policy formation in global health, e.g., Global Fund, Gates Foundation, GAVI Alliance, and how prices are negotiated by HTA Agencies [4, 22]. This impact is also evidenced by invitations to present and advise institutions and policy makers in North America (CADTH, Ottawa, March 2012 and St Johns 2013), Europe (e.g., SBU, the Swedish Council on

Health Technology Assessment, Stockholm, May 2011; Portuguese Secretary of State for Health sponsored by Exigo Consultores, Lisbon October 2011; and the European Healthcare Innovation Leadership Network, London November 2011) and South America (e.g., acting Minister of Health for Chile, Santiago, March 2012).

The research has also had an impact on the closely related public debate about whether decisions made by bodies like NICE sufficiently reward innovation. During the review of NICE methods undertaken by Sir Ian Kennedy in 2009 researchers at York were invited by Sir Ian to present at the associated stakeholder workshops. The NICE Decision Support Unit report on Innovation (led by Claxton) was part of the NICE submission to the Kennedy Study and informed his recommendations [7, 8]. This report drew heavily on the underpinning research.

In part this impact has been achieved by communicating key economic insights in a way that is accessible to wide audience, which has led to many reports and appearances in the media (e.g., Financial Times, Nature, PharmaTimes, BBC News 24 and Radio 4 Today Programme [9]), and through timely publication to inform the consultation on the government's proposals [10]. This publication was quoted or cited in submissions made by manufacturers as well as the Royal College of General Practitioners, Royal Pharmaceutical Society and the Stockholm Network [11-13]. Other publications have been targeted at a parliamentary and political audience, e.g., an invited article for the Bow Group magazine [14], with evidence of influence on Conservative Party policy [15]. University and Departmental support has been critical in engaging a range of stakeholders and policy makers, in part, through organising number of symposia to present the underpinning research.

The work at York has also had a direct impact on the formation of Government policy and the details of how the scheme will operate. For example, the importance of an empirical assessment of the cost-effectiveness threshold is evident in the original DH proposals [16] and the associated Impact Assessment [17]. This was reiterated in the government's response to the consultation in July 2011 [18]. The MRC/NIHR funded research provides an estimate of the 'basic threshold' described in the policy documents which is a key determinant of pharmaceutical prices. The findings of this work were submitted as written evidence to the recent HCHC investigation of NICE and members of the research team were also invited to give evidence [19]. At the request of DH, the results of this research have been presented to key civil servants in DH (March, June and July 2013), Department of Business Innovation and Skills (March 2013) and the HM Treasury (March 2013). The results, suggest that the threshold currently used by NICE should not be increased and may be lower. It also found little evidence that it had grown with NHS prices or real growth in the NHS budget. These findings, "have been discussed at the highest levels of Government and have had a direct impact on national policy making" [27] and negotiations between Government and the Association of the British Pharmaceutical Industry (ABPI).

One of the Governments objectives is to ensure that prices more fully reflect the greater value of health benefits in areas of high disease burden and where they also offer wider social benefits to carers, other public expenditure and the wider economy. York research on the 'basic' threshold identifies where (by disease area), how (in terms of mortality and quality of life) and for whom (by age and gender) health outcomes are likely to be displaced as a consequence of additional NHS costs. As part of the EEPRU project this work also provided the analytic and evidential foundation for valuing or weighting such displacements by type of health and any adverse wider social impacts (on carers, public expenditure and the wider economy). The importance of this assessment of opportunity cost ('displacement') is evident in the instructions DH gave to NICE when specifying how the scheme should be implemented. This work was presented to the NICE Working Party on VBP (July and August 2013) and reflects these social values and wider effects while taking proper account of opportunity costs when setting prices [23, 24]. The way that wider social benefits (consumption effects that fall outside the NHS budget constraint), are to be taken into account alongside health benefits and NHS resources has also been informed by work undertaken at York. Therefore, the underpinning research at York has had a direct impact on the life cycle of policy from public debate, policy formation to the details of how policy objectives are delivered.

5. Sources to corroborate the impact

[1] House of Commons Health Committee. Top-up fees. Fourth Report of Session 2008-09. Volume 1: Report, together with formal minutes, oral and written evidence. HC 194 –I. London: The Stationary Office; 2009. Cites [reference 2 from Section 3].

[2] Houses of Parliament. Parliamentary Office of Science & Technology. Drug Pricing. Post Note, No. 364. London: 2010. Cites [ref 2].
[3] Myeloma UK. Value-based pricing position paper. Edinburgh: 2013. Cites [ref 4].
[4] Health Impact Fund and Gilberts LLP. Toward pay-for-performance: reimbursement of innovative new drugs. Health Impact Fund White Paper. New Haven: Incentives in Global Health; 2012. Cites [ref 1] and [source 10 from this Section].
[5] Paris V and Belloni A. Value in pharmaceutical pricing. OECD Health Working Paper Number 63. Paris: OECD; 2013. Cites [refs 1 and 2] and [source 10]
[6] Husereau D and Cameron CG. Value-Based Pricing of Pharmaceuticals in Canada: Opportunities to Expand the Role of Health Technology Assessment? CHSRF Series of Reports on Cost Drivers and Health System Efficiency: Paper 5. Ottawa: Canadian Health Services Research Foundation; 2011. Cites [ref 1] and [sources 6 and 10]
[7] Professor Sir Ian Kennedy. Appraising the value of innovation and other benefits: a short study for NICE. July 2009. Cites [source 6]
[8] NICE Decision Support Unit. The value of innovation: report by the Decision Support Unit. Sheffield: NICE; 2009. Cites [refs 1, 2 and 3]
[9] Nature. UK drug-price overhaul set to shake up pharmaceutical industry, Daniel Cressey. November 1, 2010 doi:10.1038/news.2010.574. Financial Times. Fears that curbing Nice will bring 'chaos', Andrew Jack. November 7, 2010. PharmaTimes. VBP through national rebates, say health economists at York, Selina McKee. February 15, 2011.
[10] Claxton K, Sculpher M, Carroll S. Value-based pricing for pharmaceuticals: its role, specification and prospects in a newly devolved NHS. University of York, CHE Research Paper 60; 2011 (2403 downloads). Cites [refs 1, 2, 3, 7 and 8]
[11] Royal College of General Practitioners. Department of Health consultation on a new value-based approach to the pricing of branded medicines. London: 2011.
[12] Royal Pharmaceutical Society. A new value-based approach to the pricing of branded medicines: Royal Pharmaceutical Society Response. London: 2011.
[13] Healy P, Pugatch M, and Disney H. A new value-based approach to the pricing of branded medicines: Stockholm Network Submission to the Department of Health Consultation. London: The Stockholm Network; 2011.
[14] Crossbow: the Bow Group Magazine, January 2011. Paying for NHS medicines – how will value-based pricing work in the "new NHS"? page 22-26
[15] Conservative Party. Improving access to new drugs: a plan to renew the National Institute of Health and Clinical Excellence (NICE). London: The Conservative Party.
[16] Department of Health, Medicines, Pharmacy & Industry Group. A new value-based approach to the pricing of branded medicines: a consultation, 16 December 2010. Gateway reference 15205. Cites [ref 6].
[17] Department of Health. Assessment of impact on equality (AIE): Value-based pricing consultation. London: 2010. Cites [refs 3 and 7]
[18] Department of Health. Update to Assessment of impact on equality (AIE): Government response to the value-based pricing consultation. London: 2011. Cites [refs 3 and 7].
[19] House of Commons Health Select Committee. National Institute for Health and Clinical Evidence. Volume II Additional written evidence. London: The Stationary Office; 2013. Cites [refs 3, 4 and 6]

Factual statements:

[20] Head of Pricing and market Access (Europe), Roche Pharmaceuticals.
[21] Chief Executive, Myeloma UK.
[22] Director, NICE International.
[23] Head of clinical and cost-effectiveness, Department of Health and Head of Medicines Analysis, Department of Health.
[24] Director of the Centre for Health Technology Evaluation at NICE; Chair of the Appraisal Committee and Chair of the Methods Working Party at NICE; and Programme Director, Technology Appraisals Centre for Health Technology Evaluation at NICE.