

## Rethinking prescribing in the United States

Andrew Ellner, Assistant Medical Editor  
BMJ Knowledge, London WC1H 9JR  
aellner@bmjgroup.com

*The United States wants to increase senior citizens' access to prescription drugs at a time when spending on drugs is soaring. Is a national list of essential medicines the answer?*

Many elderly people in the United States have no insurance cover for prescription drugs. Currently, one in four elderly people in the United States limit their drug use because of cost.<sup>1</sup> The US Congress recently passed a bill that partially subsidises prescriptions for elderly and disabled people while promoting their use of private health insurance plans. Previous efforts to provide drug benefits to this population have been unsuccessful, however, largely because it would be hugely expensive. One way to extend access to drugs while containing costs is to establish a limited or essential list of medicines. This article discusses the benefits of such an approach and barriers to implementation.

### Rising drug costs and spending

One of the reasons that US drug spending is rising rapidly<sup>2</sup> is shifting demographics. The group aged over 65 is one of the fastest growing parts of the population. This group tends to be more chronically ill and require more drugs. Another reason for the increase is the influx into the market of newer, more expensive drugs for many diseases.

The proliferation of direct to consumer advertising may also be contributing to the rise in drug spending.<sup>2</sup> The United States allows adverts in the broadcast media that encourage patients to talk to their doctors about trying brand name medicines. More money was spent in the year 2000 on direct to consumer advertising of the anti-arthritic drug Vioxx (rofecoxib) than was spent on advertising Pepsi, Budweiser beer, or Nike's top shoes.<sup>3</sup> But pharmaceutical company spending on consumer advertising is only a small proportion of the promotional budget, most of which is still targeted at physicians.<sup>4</sup> The industry emphasises that such adverts can have an important role in educating the public.<sup>5</sup> Overall spending may be going up partly because more people are asking for drugs that they really need.

The United States, unlike most other developed countries, has no government body that regulates the price of drugs or (as in the United Kingdom) the profits of pharmaceutical companies. International price comparisons often conclude that US citizens pay more than anyone else for drugs, although methodological issues complicate such comparisons. Other determinants that affect the price of drugs include a country's wealth, the prescription drug coverage of its citizens, cultural mores, normal medical practice, demographic differences, and the characteristics of the nation's pharmaceutical industry.<sup>6</sup> One justification for high drug prices is that they are necessary to cover the cost of innovation. But are US citizens paying more towards the discovery of new drugs than citizens of other countries or are they paying for marketing?

### Limited or essential lists of drugs

Lists of approved medicines, also called formularies, are nearly ubiquitous in the United States and internationally and have been identified by one expert committee as "an essential part of modern health care systems."<sup>7</sup> These lists are typically created by committees of physicians, pharmacists, and other experts. They include drugs that are first evaluated for clinical efficacy and safety and then often for relative cost or cost effectiveness.

Formulary systems vary in the number of drugs and medical conditions they cover and the extent to which they restrict access to unlisted medicines. With open formularies, patients are reimbursed for drugs whether or not they are listed. Other formularies may be accompanied by programmes that offer patients price incentives to choose listed drugs or that reimburse patients for only the drugs on the list. The British National Formulary lists all drugs licensed in the United Kingdom, excepting only a small group of drugs that are licensed but not recommended.

The World Health Organization has been a prominent advocate of countries establishing a list of essential medicines. It has produced a list as a model.<sup>8</sup> The list is more selective than most formularies, and in many cases contains one or a few drugs from essential pharmaceutical classes (for example, only enalapril for angiotensin converting enzyme inhibitors to treat heart failure). WHO recommends that countries select drugs from within essential classes based on availability and cost effectiveness. Decisions made about medicines on WHO's list are linked to the results of high quality clinical and cost effectiveness studies. Once identified, access to essential medicines should be guaranteed to everyone in a population who needs them through subsidies.<sup>9</sup>

### Effects on costs

Cost containment is the main reason for introducing drug lists in developed countries. Several US states, including Michigan, Vermont, and Florida, have begun implementing preferred drug lists for recipients of public health programmes. Strategies vary, but generally listed drugs are those within a therapeutically equivalent group that are the least expensive or for which the state receives the best rebate from the manufacturer.

The US private sector has seen the growth of companies called pharmacy benefits managers, which develop and manage drug formularies. The companies act as middlemen between patients, employers, insurance companies, public health insurance programmes, pharmaceutical manufacturers, and retail pharmacies. To keep prices down, they negotiate bulk deals for specific brands of drugs. They often have systems to encourage physicians and patients to switch to the medicines on their formularies. Among other cost cutting measures, they often favour the purchase of bioequivalent generic drugs.<sup>10</sup>

Concerns have been raised, however, about the practices of pharmacy benefit managers. Mergers with large pharmaceutical manufacturers have raised the spectre of conflict of interest.<sup>11</sup> Undisclosed pricing arrangements with manufacturers may compromise the medical appropriateness of formulary decisions.<sup>10</sup> And centralised decision making removes physicians from final drug selection.<sup>11</sup>

Debate also exists over whether formularies in general are a misguided concept. Critics are concerned that they have an adverse effect on patients' health and may only shift costs to other parts of the health service budget.<sup>12</sup> However, the studies providing the basis for this criticism have been refuted for methodological flaws and because they were supported by the pharmaceutical industry.<sup>13</sup> In addition to the policy issues, several methodological challenges would need to be overcome to produce a truly evidence based list of essential medicines (box 1).

#### **Box 1: Some methodological challenges to developing evidence based lists of essential medicines**

*Where do you start?* By identifying a list of the most common medical conditions and considering only treatments for those or by re-evaluating the contents of an existing list of medicines?

*What if there are gaps in clinical evidence?* Considerable gaps exist in evidence for treatments of even the most common conditions, particularly for the unique effects and adverse effects of drugs in older people

*Are medicines exchangeable?* Is it safe to substitute generic medicines for brand name equivalents? Is it acceptable to substitute clinically similar drugs in the same therapeutic class?

*Are cost effectiveness studies reliable enough?* Such studies involve complex models with a wide range of measures chosen by investigators. Construction of the model can lead to considerable bias. Studies are often funded by pharmaceutical companies for evaluation of their products and may be viewed by companies as marketing tools<sup>14</sup>

*What should be done about 'indication slippage'?* This is when medicines are prescribed for indications other than those for which they are placed on the list and for which they may not be cost effective

#### **Reference pricing**

A list of medicines can function only alongside a strategy for reimbursing drug costs. Reimbursement strategies vary in the extent to which they give incentives for patients to choose preferred drugs. In the US private sector, one approach is to have a three tiered benefit: patients pay the lowest amount if they purchase a generic drug, slightly more for a (listed) brand name drug, and the most for an unlisted brand name.

An alternative strategy that fits well with the goal of creating an evidence based list is a reference pricing programme. In this system, the most effective and cost effective drugs within a therapeutic class are identified as reference drugs and are fully reimbursed. Prescription of more expensive alternative drugs is not limited, but unless patients have specific indications for alternatives, they pay the difference in price. One of the advantages of such a programme is that it puts downward pressure on the price of alternative drugs that provide no clear added clinical benefit.<sup>15</sup>

Governments have used reference pricing in different ways. The public health insurance programme in the Canadian province of British Columbia uses reference pricing for drugs for common conditions when many similar drugs exist with wide variations in price—for example, non-steroidal anti-inflammatory drugs.

Australia uses reference pricing more comprehensively. It has a central Pharmaceutical Benefits Advisory Committee that recommends which drugs should be listed on the public pharmaceutical benefits scheme. The committee requires companies to submit extensive applications for new drugs that conform to strict guidelines. Among other things, the guidelines show a preference for clinical trials that compare the new drug with the existing standard of care. The committee makes recommendations to a pricing authority about the price at which new drugs are cost effective. The intent is to reward marginal improvements in efficacy with corresponding increases in price. Reimbursement is at the level of the least expensive drug within each therapeutic group.

Evidence is growing that reference pricing helps to contain costs without leading to increases in adverse outcomes or use of other health services.<sup>16, 17</sup> Savings may, however, be only short term. A system of reference pricing does not affect other drivers of increased spending on drugs, including the volume of prescriptions and the entry of new, more expensive and effective drugs.<sup>15</sup> Prescribing patterns may eventually conform towards greater use of the reference priced product, and the greater volume of prescriptions may negate savings.<sup>6</sup> Assuming that the new prescriptions are appropriate, however, this would also mean that more people were getting drugs that they needed. Critics of such programmes believe they create a

disincentive to pharmaceutical innovation, which tends to proceed through incremental advances in efficacy and tolerability, and that they place more demands on doctors' and pharmacists' time.<sup>15,18</sup>

### Political barriers

The potential resistance in the United States to a limited list of medicines and reference pricing must not be underestimated. In other countries, governments taking similar approaches have encountered appreciable opposition from prominent stakeholders, especially the pharmaceutical industry.<sup>18</sup> The pharmaceutical industry has tremendous power in the United States and has already issued legal challenges in several US states attempting to implement limited lists. Other stakeholders may also object (box 2).

#### Box 2: Political challenges to essential medicines approach

Physicians may worry that such a list would restrict their clinical freedom

Physicians and pharmacists may feel that it places greater, uncompensated demands on their time

Patients may be concerned that an essential medicines process would restrict their access to the newest and best drugs

Patients may worry that a list will not meet their unique needs

Pharmaceutical companies are likely to oppose the process on the grounds that it will limit their incentive to innovate

Success of essential lists will depend on getting the right message across to the public and physicians. Public discussion needs to make it clear that a reference pricing system guides but will not restrict choice or doctors' clinical freedom and that patients will be able to get drugs outside the list subsidised when they have a clear clinical need (H Hogerzeil, personal communication). Working with the media will be important, because stories emphasising the negative opinions of health professionals or relating stories of 'victims' of new restrictions will increase hostility (D Henry, personal communication). One way of increasing acceptance may be to use the term preferred rather than essential medicines.

### Balancing innovation and access

Arguably, the central tension in creating an effective national drug plan (and thereby extending access to medicines in a sustainable fashion) is between containing costs and preserving the incentive for pharmaceutical companies to create innovative drugs. Advocates of the essential medicines approach and reference pricing say that they provide a way to reward true innovation—perhaps at an even higher price than such products would otherwise command. At the same time, there is downward pressure on the price of more expensive products that offer similar benefit to reference drugs. Evidence from Australia suggests that its programme is accomplishing such goals.<sup>19</sup>

Pharmacy benefit managers already maintain formularies and use financial incentives to influence the choice of less costly drugs in the US private sector. The potential advantage of centralising this process is that it increases the transparency and accountability of important healthcare decisions. It also allows physicians and pharmacists to have a more prominent role in decisions. A centralised programme would have considerable leverage to negotiate on prices for drugs. Depending on how selective the list was, the subsidy for preferred drugs, and therefore the benefit to older people, could be substantial.

But there are also costs to taking an essential medicines approach. As well as direct financial costs to the government and the pharmaceutical industry, indirect political costs are likely—the pharmaceutical industry has one of the most powerful and best funded political lobbies in Washington. The process may result in reduced pharmaceutical industry investment. Finally, it may be argued that the incentives to innovate are not so straightforward or easily manipulated; that the cost to society of dampening innovative drive is hard to predict; and that the risk of doing so is not worth the potential benefits.

The virtue of innovation in health care, however, is to improve the health of the public. If innovation comes at the expense of access to care for large groups of people, it may not be serving its purpose. The goal must be to strike a balance that is in the public's best interest. Arguably, by attempting to distinguish and reward only true innovation, this is what the essential medicines process does.

#### Summary points

Spending on prescription drugs is rising rapidly in the United States

The United States is exploring ways to subsidise drug costs for all senior citizens, many of whom have no insurance for prescription drugs

Limited lists of medicines are increasingly accepted as a way of controlling spending on drugs

Reference pricing may be the best way to administer drug benefits while rewarding true pharmaceutical innovation

Substantial methodological and political barriers exist to the essential medicine and reference pricing approach

**Contributors and sources:** AE has an MSc in health policy, planning, and financing. This article is based on the report *Essential Medicines in the United States*, coauthored by AE, Luisa Dillner, Beth Nash, and Fiona Godlee. To prepare the report we conducted an in-depth policy review, searching the peer reviewed literature and internet for information on essential medicines, drug lists, formularies, and related concepts. We also held semistructured interviews with experts in the fields of pharmacoconomics, pharmacology, healthcare policy, geriatrics, medicine, and biostatistics (see [bmj.com](http://bmj.com) for list of names).

**Funding:** The report was commissioned by United Health Foundation, which paid a fee to cover library costs and the work of information specialists.

**Competing interests:** None declared.

---

## References:

- <sup>1</sup> Kitchman M, Neuman T, Sandman D, Schoen C, Safran DG, Montgomery J, et al. Seniors and prescription drugs. Findings from a 2001 survey of seniors in eight states . Menlo Park, CA: Henry J Kaiser Family Foundation, Commonwealth Fund, Tufts-New England Medical Center, 2002. [www.kff.org/medicare/loader.cfm?url=/commonspot/security/getfile.cfm&PageID=14177/](http://www.kff.org/medicare/loader.cfm?url=/commonspot/security/getfile.cfm&PageID=14177/) (accessed 1 Dec 2003).
- <sup>2</sup> Steinbrook R. The prescription drug problem. *N Engl J Med* 2002;346:790.
- <sup>3</sup> National Institute for Health Care Management Foundation. Prescription drugs and mass media advertising, 2000. [www.nihcm.org/DTCbrief.pdf](http://www.nihcm.org/DTCbrief.pdf) (accessed 16 Mar 2003).
- <sup>4</sup> Rosenthal MB, Berndt ER, Donohue JM, Frank RG, Epstein AM. Promotion of prescription drugs to consumers. *N Engl J Med* 2002;346: 498-505.
- <sup>5</sup> Holmer AF. Direct to consumer advertising—strengthening our health system. *N Engl J Med* 2002;346:526-8.
- <sup>6</sup> Stuart B, Brandt N, Briesacher B, Fahlman C, Mullins D, Palumbo F, et al. Appendix A: issues on prescription drug coverage, pricing, utilization, and spending: what we know and what we need to know. In: Department of Health and Human Services. Report to the President: prescription drug coverage, spending, utilization and prices. Washington, DC, DHHS, 2000. <http://aspe.hhs.gov/health/reports/drugstudy/index.htm> (accessed 10 Feb 2003).
- <sup>7</sup> Blumenthal D, Herdman R, eds. Description and analysis of the VA national formulary . Washington, DC: National Academy Press, 2000.
- <sup>8</sup> World Health Organization. The WHO model list of essential medicines. [www.who.int/medicines/organization/par/edl/eml.shtml](http://www.who.int/medicines/organization/par/edl/eml.shtml) (accessed 1 Dec 2003).
- <sup>9</sup> World Health Organization. The selection of essential medicines. *WHO Policy Perspect Med* 2002;4. [www.who.int/medicines/organization/ood/ood6paggers.shtml](http://www.who.int/medicines/organization/ood/ood6paggers.shtml) (accessed 4 Nov 2003).
- <sup>10</sup> Goff VV. Pharmacy benefit managers: a model for Medicare? *Issue Brief Natl Health Policy Forum* 2001 Jul 9;(765):1-12.
- <sup>11</sup> American College of Physicians. Ambulatory care formularies and pharmacy benefit management by managed care organisations. Philadelphia, PA: ACP, American Society of Internal Medicine, 2001. [www.acponline.org/hpp/amb\\_care.htm](http://www.acponline.org/hpp/amb_care.htm) (accessed 1 December 2003).
- <sup>12</sup> Horn S. HMO formularies and care costs. *Lancet* 1996;348:619-20.
- <sup>13</sup> Ross-Degnan D, Soumerai SB. HMO formularies and care costs. *Lancet* 1996;348:1264.
- <sup>14</sup> Rennie D, Luft HS. Pharmaco-economic analyses: making them transparent, making them credible. *JAMA* 2000;283:2158-60.
- <sup>15</sup> Ionnides-Demos LL, Ibrahim JE, McNeil JJ. Reference-based pricing schemes: effect on pharmaceutical expenditure, resource utilisation and health outcomes. *Pharmacoconomics* 2002;20:577-91.
- <sup>16</sup> Schneeweiss S, Walker AM, Glynn RJ, Maclure M, Dormuth C, Soumerai SB. Outcomes of reference pricing for angiotensin-converting-enzyme inhibitors. *N Engl J Med* 2002;346:822-9.
- <sup>17</sup> Schneeweiss S, Soumerai SB, Glynn RJ, Maclure M, Dormuth C, Walker AM. Impact of reference-based pricing for angiotensin-converting enzyme inhibitors on drug utilization. *CMAJ* 2002;166:737-45.
- <sup>18</sup> Reference Drug Program Consultation Panel. Report to the minister of health planning, British Columbia, 2002. [www.healthplanning.gov.bc.ca/cpa/publications/rdppanel.pdf](http://www.healthplanning.gov.bc.ca/cpa/publications/rdppanel.pdf) (accessed 4 November 2003).
- <sup>19</sup> Productivity Commission. International pharmaceutical price differences. Research report, July 2001. [www.pc.gov.au/research/commres/pbsprices/finalreport/index.html.030530](http://www.pc.gov.au/research/commres/pbsprices/finalreport/index.html.030530) (accessed 21 Apr 2003).

The complete paper, “Essential Medicines in the United States” can be found at [www.unitedhealthfoundation.org](http://www.unitedhealthfoundation.org)