Partnerships are the key to neglected diseases

In this second article looking at ways to stimulate R&D in neglected diseases, Mary Moran of the London School of Economics argues that public–private partnerships should be actively supported.

A recent Scrip article focused on industry's need to “fix pharma's image”, with Vincent Lawton, president of the UK pharmaceutical industry association, the ABPI, noting that, "We need to sort that out, through transparency or whatever ... ".

One of the problems with pharma's image arises from the public perception that the industry focuses single-mindedly on profit without concern for the needs of the world's poor. And beyond this perception is the more difficult reality that new drugs for developing world diseases will almost certainly need industry participation – real, not just perceived. However, industry may already be on the way to addressing both these problems, if it only knew it itself; and that "whatever" may already be a reality.

For instance, although the current view is that only 13 new drugs have been developed by the pharma industry for tropical diseases since 1975, our research shows this is no longer the whole story. In fact, there are now more than 70 drugs developed or in development for neglected diseases – the vast majority of these projects initiated since 2000.

The most interesting aspect of this finding is not the numbers themselves, but rather an understanding of what led to this burst of activity. After all, are neglected diseases not inherently non-commercial?

The answer to this question is both "yes" and "no". For multinational companies, these projects are definitely non-commercial, with expected returns falling well below the benchmark of $500 million per year peak sales. Yet multinationals now account for nearly 50% of these new neglected disease projects, conducting this R&D either alone or, predominantly and increasingly, in public–private partnerships (PPPs).

Good business sense

The prime motivator for these companies is simple good business sense, not in terms of profit maximisation, but in terms of cost–effective risk management and strategic positioning. This is particularly the case for PPP–projects. Working in a PPP allows large companies to share the cost of the R&D with their partners. By covering some or all of a company's external project costs, PPP contributions can make neglected disease projects virtually cost–neutral to the multinational company.

This allows companies to protect shareholder value, while simultaneously managing the risk of their reputation associated with accusations of company neglect for the world's poor. Additional advantages of the PPP approach are the ability to share liability and scientific risk with the public partner; company access to scientific and technical skills that might not exist in-house for these indications (eg, TB trial expertise or parasitology); and, importantly, the role of the public partner in facilitating use of the final product – no company wants to invest in a drug that is not used.

Beyond the advantages to industry, the PPP framework has very clear advantages for public health interests. Indeed, it is difficult to think of any other approach that so effectively supports industry needs while simultaneously and actively prioritising issues of vital importance to developing countries. This includes selection of projects based not just on efficacy and safety, but also on criteria such as affordability to end–users (cost of goods), appropriateness of formulations to developing country needs (heat stability, oral availability and fixed–dose combinations) and suitability for paediatric populations.

Rewards

For large companies, this public health focus can deliver a tangible reward in addressing corporate social responsibility; and responding to employees' desire to work in an ethical environment and make a real contribution to curing some of the world's major diseases. Not all large companies have come to this realisation, however, with the majority of structured neglected disease R&D still being accounted for by a handful of EU–based multinationals.
For small companies on the other hand, the answer to the question of whether these diseases are non-commercial is, "no, not necessarily". Review of orphan drug applications in the US shows that 70% come from small to medium enterprises, and that the average market they are targeting is worth only $100 million in peak annual sales. While well below the $500 million needed to motivate R&D activity for multinationals, this market size is not dissimilar to that of some neglected diseases – TB, malaria and possibly leishmaniasis. As Zentaris, the speciality firm that recently registered a new leishmaniasis drug, said: "While such a market would be negligible for a big pharmaceutical company, it has a good economic scale for us."

A key to tipping these disease markets into commercial viability may be consolidating markets (as with commercial orphans) and/or reducing costs, in particular through PPP frameworks. The latter is starting to happen, with small company-PPP projects now accounting for around 20% of neglected disease R&D.

These companies appear to welcome the up-front cash flow offered by PPP public partners (especially if venture capital is tight), as well as the ability to apply R&D findings to more commercial indications. For example, Paratek, a small US SME receiving grants from the Medicines for Malaria Venture (MMV) and the US NIH to develop tetracyclines for malaria, is simultaneously developing its tetracycline compounds for commercial Gram–positive bacterial indications.

A second area of commercial interest for small companies, in particular CROs, is as subcontractors on publicly driven projects. Projects such as MMV’s new antimalarial (synthetic peroxide) or the TB Alliance’s new tuberculosis drug (PA–824) are fully subcontracted to small companies, collectively generating tens of millions of dollars of new business for these firms.

An examination of budgets of groups like the MMV shows that 67% of their R&D budget goes directly to companies, while 30% goes to academics to commercialise basic research. Instead of starving this PPP source of industry revenue, governments might do better to support it. With sustainably well fundedPPPs, multinationals could be provided with reliable revenue streams to enable long-term strategic choices in the neglected disease area; payments to small companies could tip the balance to make these drugs commercially viable; and CROs could benefit from commercial contracts in this new niche market.

In other words, industry and public health interests could be aligned in this area, allowing pharmaceutical companies to develop new neglected disease drugs without harming their own interests – indeed, even actively protecting and improving their position.

Rethink needed
However, success will require policy makers to expand their thinking from the current focus on big-ticket future payouts to encompass a broader framework designed to support the reality of industry-neglected disease activity now. Pharma companies, small and large, are already exploiting approaches that work for them in this notoriously difficult area. Governments would do well to support and encourage these choices.

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