Financing R&D for neglected diseases

There has recently been a welcome increase in R&D activity to provide much-needed new drugs for neglected diseases of the developing world. This activity has resulted in an early stage pipeline of potential medicines that will require significant new funding if they are to progress to registration — a need that might be addressed by a novel type of funding body.

Providing new medicines for neglected diseases of the developing world is a key global health issue. For some diseases, such as malaria, existing medicines are encountering increasing resistance, whereas for others, such as the kinetoplast parasitic diseases, the existing drugs have unacceptable side effects, or no drugs at all are available, as is the case for dengue fever.

Responding to this need, there has been a welcome boost to R&D activity in this field in the past few years, catalysed by public–private partnerships (PPPs) between academic institutions, pharmaceutical companies, charities and governments, as well as by increased funding. These activities, which have consumed about US$500 million so far, have resulted in ~60 projects for a range of neglected diseases, most of which are at an early stage[1,2].

However, a critical concern for these projects is becoming increasingly apparent. An estimate taking into account historical attrition rates indicates that it will take a further investment of at least ~$1 billion per year in the next 10 years to generate the data required for successful compounds in this portfolio to be registered[2]. Currently, no source of funding of this magnitude exists that would invest in R&D for medicines for diseases for which no commercial returns are to be expected.

With these challenges in mind, it has recently been proposed that a new fund for R&D for neglected diseases should be established with governments of developed and developing countries, charities and other entities as donors[3]. This fund — the fund for R&D in neglected diseases (FRIND) — would allocate resources using a modification of the process applied by large pharmaceutical companies, without the aspects of high commercial returns, but within the existing patent system. It would be overseen by a board designated by the donors, and its task would be purely strategic: it would focus on ensuring accessibility to poor patients, defining fundable diseases and nominating a portfolio management team.

This team, which is crucial to maximizing output from the resources available, must include the following expertises: first, basic scientists knowledgeable in the diseases of interest, pharmacologists, molecular biologists, medicinal chemists and so on; second, development experts versed in technical, chemical and safety issues; third, medical doctors with experience in epidemiology, clinical research and in the countries where the diseases are endemic; and fourth, economists and public health experts. The team would then evaluate projects originating from academic institutions, PPPs or pharmaceutical companies at any stage during the R&D process against appropriate target product profiles, as well as competing projects.

If the project is deemed viable, the portfolio team would allocate sufficient resources, but only up to the next decision point. The project originators would then present the new data obtained with the FRIND money, and on the basis of this data the team would decide whether to continue to the next stage or not. With regard to patenting, the project originators would usually patent the molecules they propose to FRIND, but in return for the money received, they would allocate an exclusive license for the neglected disease indication to FRIND. The originators, however, would keep the ownership for composition of matter and any other indications for which a commercial return might be expected for their own development.

This model has several advantages. First, by funding projects only from one decision point to the next, waste of resources is minimized. Second, as it is expected that several entities working on the same neglected disease would at one point apply to the fund, FRIND experts could directly compare projects in the same indication and promote only the most viable ones, which is more difficult to achieve with the current fragmented state of the pipeline. Third, this proposal works within the existing global intellectual property model of the developed world, but ensures that patents are not used to prevent affordable access to patients in developing countries. Finally, in contrast to other models such as prizes, advanced marketing commitments or vouchers — in which all the failure risk remains with the originator, representing a major disincentive to invest — the FRIND proposal shifts this risk to the fund. This could encourage many entities to bring forward their solutions for neglected diseases that otherwise would not.

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1. Moran, M. *PLoS Med.* **2**, e302 (2005).
2. Dalberg Global Development Advisors. Feasibility study for a fund for R&D for neglected diseases. (2008).
3. Herrling, P. L. *Global Forum Update Res. Health* [online], <http://globalforumhealth.org/filesupld/global_update5/art/Update5_MakingDrugsAccessible_Herling.pdf> (2008).