



Ensuring Innovation for Neglected Diseases

Research and Development (R&D) Workshop

9th June 2005, London

The Workshop held on 9th June provided the opportunity for around 60 participants to focus in greater detail on a selected number of the issues raised during the previous day's Conference, through breakout sessions and in depth discussions during the plenary sessions.

Disease specific breakout sessions

A breakout session on leishmaniasis and human African trypanosomiasis (HAT) identified the following needs: greater research into new drugs and diagnostics (particularly to differentiate stages 1 and 2 and to assess treatment success in HAT); better and more rational drug deployment; better pharmaco-vigilance, especially in the monitoring of drug use and of the increase of resistance (e.g. to melarsoprol); and further work into parasite differentiation and drug sensitivity (e.g. to understand the difference in drug responses between Indian and East African visceral leishmaniasis patients). The session identified possible roles for MSF in the field testing and refining of rapid diagnostic tools, in the identification of possible sites for research as well as sentinel sites for resistance monitoring, and a greater role in the dissemination of its field experience and data.

A second breakout session on HIV concluded that there was a need for advocacy for a radically different R&D agenda for HIV/AIDS. New drugs in the pipeline are not adapted to developing world needs, as they are designed for highly ARV-experienced patients in the North. Other drugs remain on the shelf and are not developed further. There is need for research on when best to switch to 2nd line drugs in resource poor settings, and on practical implementation questions prior to microbicides becoming available. There are promising developments in diagnostics: a rapid and field-adapted viral load dipstick, useful for diagnosis of children under 18 months and for viral suppression, is close to field-testing.

Tuberculosis was also the focus of a breakout session. Mark Perkins of FIND and Maria Freire of the Global Alliance for TB Drug Development shared their experiences which started the discussion. Mark Perkins raised the question around the appropriate end-cost of a product: should the development of a test be stopped if it cannot achieve the cost goal of under US\$1 or can a more expensive test still have a place, at least in middle income countries? Zafrullah Chowdury from Bangladesh argued that any new tests must be affordable for the poorest, others argued that increased donor funding is a possibility. An important challenge that was identified is the fact that considerable knowledge gaps persist in basic science that preclude the development of rapid tests for tuberculosis in the near future. As FIND does not see its role in basic research, more product orientated basic research must be called for. Maria Freire mentioned the concrete question the TB Alliance is facing if a future TB treatment should be designed for daily or once-weekly intake and which mode would encourage better compliance. It was said that there is a clear need for operational research to study the different existing variations of directly observed therapy, and to also compare these to approaches in HIV antiretroviral treatment that emphasises treatment literacy without direct observation. There was a common concern that the "TB community" is relatively conservative which could mean substantial delay and resistance to use and implement new tools once they become available. Discussions with TB programs are therefore crucial already during R&D. MSF could have an important role in promoting change once more effective tools are available.

The financial and normative environment

Issues of norms and standards for drug registration were discussed in more detail than was possible during the Conference. Marie-Helen Pinheiro from the EMEA presented on a regulation which enables EMEA, on request of the WHO, to give its scientific opinion for medical products to be marketed outside the EU. This will be based on EU criteria with regard to efficacy and safety but with appropriate risk/benefit considerations and, when EU criteria are not applicable, on WHO guidelines. There is a possibility for fast track and fees can be waived if necessary. The regulation now needs to be put to test. It will, however, not be a substitute for the critical need for capacity building and for harmonisation across developing country drug regulatory authorities.

A breakout session on R&D financing stressed the need to overcome the old R&D financing system and to move from a market-based and patent-protected system to a needs-driven priority agenda. Many people have come to the realisation over the recent years that the current system cannot help for neglected diseases R&D; nor does the system work well for diseases in the North. Several researchers are promoting alternative R&D paradigms to try to solve the neglected disease problem and/or the global failure to respond adequately to R&D needs. A better R&D paradigm would involve proper needs identification and setting of priorities; a long-term public commitment (including through funding) for R&D activities; a proper clinical assessment of drugs; and ensuring access to essential drugs for all patients.

Different proposals were discussed during the session. Intellectual property rights transfers ("patent roaming") and fast track options have been criticised from the equity side (as they represent a tax on patients in the North) and their limited ambition (as they are systems based on industry needs rather than on patients needs). Advance purchase commitments and prizes were seen as potentially interesting tools for neglected diseases. The R&D treaty proposal, although more ambitious and potentially less feasible, is clearly the most comprehensive proposal.

A final breakout session asked whether money was the only barrier preventing the benefits of science from reaching the field. Yves Champey of DNDi gave an overview on how the way research is conducted has evolved over the last 10 years: outsourcing, where R&D is contracted out to biotech companies, academia, etc is now more common; the focus is on costs; and there is more involvement of players from the South, such as India, China, or Korea.

Two different models for conducting research were presented. Both concerned for diagnostic products, a particularly promising area of research for resource-poor settings. The first, a new rapid test for malaria detecting pLDH, focussed was on the field-defined needs, not the technological possibilities. The entire process was much quicker (2.5 years) and cheaper (under €80,000) than a typical development. The second, presented by Dr Sanjeev Krishna from St Georges Hospital Medical School, concerned the identification of lab-clinic correlations. The model hopes to develop a diagnostic test by linking the very best science in the field of malaria parasite biology, and by linking this science to clinical observations to establish a correlation between what was known molecularly about the parasite and what was seen in patients.

Discussions centred on MSF's potential contribution in this field: researchers called for MSF to identify field needs, and to set the specifications because of their unique access to the patients. MSF should not limit this to the "big five" diseases, but all areas of patient care. The question of MSF's potential involvement in clinical trials was debated hotly. MSF could also be of added value in liaising with donors to ensure that appropriate technologies are being developed, and in assessing the feasibility of any tools that are developed.