6. Priority diseases and reasons for inclusion

6.9 Neglected Tropical Diseases

See Background Paper 6.9 (BP6_9NTD.pdf)

Background

Currently 17 diseases are recognized as neglected tropical diseases, including Chagas’ disease, leishmaniasis, sleeping sickness and Buruli ulcer. They affect more than one billion people, mainly in developing countries, causing great suffering and many deaths. Existing solutions are often ineffective, unaffordable and inappropriate for the needs of the resource-poor populations affected. There is an urgent need for new or improved vaccines, diagnostics and treatments for these diseases.

Developments since 2004

The 2004 Report highlighted the problem of Buruli ulcer, a painful and disabling condition that was primarily treated with wide surgical incision (see 2004 Report Background Paper 6.9). Since then considerable progress has been made in identifying effective treatments for this condition (see Background Paper 6.9, Section 2.2).

In recent years, there has been more commitment towards new R&D initiatives for neglected tropical diseases, both from the public and private sectors, resulting in a well-populated R&D pipeline. There have been efforts to use a two-pronged approach, involving preventative chemotherapy and intensified patient management. As a result, large drug donation programmes have recently been rejuvenated and specific targeted treatments for several neglected tropical diseases have now been prioritized. Both these approaches use existing tools and medicines.

Remaining challenges

There are currently several barriers to the translation of early stage scientific research into effective, affordable and accessible products for neglected tropical diseases. These include the low market value for these products, limited interest from innovators and stakeholders, and a lack of funding.

Research needs

In order to have a major impact on the high global burden of these diseases, there is a need for more R&D of medicines, vaccines and diagnostics. This will require prioritizing R&D efforts towards needs-based agendas and aligning these efforts with existing and planned treatment, control, elimination and eradication programmes. Also needed is greater involvement of the different sectors and pooling of resources, combined with adequate, sustained funding to achieve successful development of products and solutions.
Overall, a public commitment is needed to: develop a global needs-driven (as opposed to market-driven) health R&D agenda for neglected tropical diseases; and create appropriate mechanisms, incentives and monitoring to allow the effective implementation of this agenda towards sustainable, achievable solutions for neglected tropical diseases.

The public health patient-focused needs and programmatic needs (individual disease management or elimination programmes, for example) should be addressed first. A thorough search of the required health tools should be evaluated to develop priority areas for support to fill gaps for product development. Buy-in for these programmes must come from the governments of countries where neglected tropical diseases are endemic, and the regulatory systems in both high- and low-income countries should make this an area where incentives outweigh the burden of product development for these diseases. This analysis should be done for each disease, and resources used to maximize scientific, technological and programmatic opportunities, taking into account the needs of patients from among the poorest communities.

Innovative R&D aimed at radically new products and solutions for neglected tropical diseases, or adaptive R&D designed to make better use of existing medicines, vaccines, diagnostics, and technology platforms, should be supported. In all cases, translational research to transform the results of basic research into useful applications is essential.

The EU, while already supporting some excellent initiatives, should continue to address the problem of neglected tropical diseases. There is a moral and ethical obligation to address the problem of neglected tropical diseases in developing countries. The EU-African, Caribbean, and Pacific Group of States (ACP) Joint Parliamentary Assembly Resolution on poverty-related diseases and reproductive health in ACP states explicitly calls for European action for neglected diseases: “[The Assembly] Calls on the European Commission to include the most neglected diseases, such as sleeping sickness, Chagas’ disease and leishmaniasis, among its priorities and to ensure that effective, appropriate, easy-to-use medicines are developed and placed on the market in the developing countries at an affordable price”. A follow-up United Nations Assembly resolution addressed this point and has led to some efforts in reallocating EU support towards neglected tropical disease R&D (through the EU Framework Programme and government support for product development partnerships (PDPs))4. Pharmaceutical industry-driven agendas for the development of new therapeutics (such as the Innovative Medicines Initiative, IMI) have not yet addressed R&D efforts for these diseases.

Support should be increased substantially and sustained in the Horizon 2020 programme and beyond, and R&D priorities should be based on societal values and health needs and not solely be market-driven. In addition to appropriate (sustainable) funding, governments should establish incentives and obligations to encourage neglected disease R&D in both the public and private sectors. Collaborative efforts are necessary, as the sharing of complementary resources and knowledge and the building of an integrated platform for neglected tropical disease R&D is necessary to keep costs
low and impact high. Such a programme could include in-kind contributions from the (local and multinational) pharmaceutical industry, preferential funding of translational research projects (by public-private partnerships, public-driven R&D, integrated academic platforms and PDPs), risk mitigation from drivers of product development, reductions in regulatory costs and barriers and development of alternative, needs-based models for the setting of research priorities.

Organizations active in this area should be encouraged to pool their resources and work together to increase the opportunities for successful results. These include epidemiological tools and data (including drug resistance and monitoring), products developed, operational research outcomes and recommendations for implementation strategies.

Recommendations to help achieve this include:

- **Mobilize and sustain adequate funding** for neglected diseases. Committed funding over a number of years will be needed to support the implementation of a needs-based priority R&D agenda for neglected diseases;
- **Encourage translatable research** using the “3T” approach (Therapeutics, Technology and Transfer) to transform the results of basic research into useful technologies for medical applications, adapted to the needs of neglected tropical disease patients and closely linked to the interventions of existing programmes for neglected tropical diseases;
- **Establish adequate incentives for collaborative research, based on shared values**, including appropriate training, funding, and specific career incentives based on a reassessment of the way merit is evaluated in public research;
- **Mobilize the pharmaceutical/diagnostics industry by a mix of incentives and obligations** to contribute to the development of needed medical interventions and commit to donate or provide sustained access to medical interventions, **based on shared values**;
- **Engage the innovators** from emerging economies, biotechnology, pharmaceutical/diagnostic companies, small- and medium-sized enterprises (SMEs), PDPs and academic institutions through shared (societal and economic) values;
- **Monitor the performance** of PDPs, integrated academic platforms and pharmaceutical companies (including those in emerging economies) to ensure public accountability for resources spent;
- **Expand the activities of PPPs and integrated academic platforms** to include product development for medicines, vaccines, diagnostics, drug resistance platforms and control strategies for these diseases, together with efforts to strengthen health systems in the disease-endemic countries. Support integrated academic platforms, where product development and operational research is carried out by academic innovators for neglected tropical diseases;
- **Strongly encourage the expansion of the activities of the European and Developing Countries Clinical Trial Partnership (EDCTP)** to include several of the most neglected diseases as well as additional phases of clinical development.
(Phase I, Phase IV), and link this to the efforts of the pharmaceutical industry-driven non-profit organization TransCelerate; 

- **Create a centre for preclinical research** to bridge the continuing gap in the process of developing medicines and vaccines into clinical candidates for neglected tropical diseases. The centre would provide a pool of resources available for preclinical research, which should complement the activities of the EDCTP; 

- Investigate the possibility for **centralized technology platforms for adaptive R&D** (for example, adapting existing and new medicines, vaccines and diagnostics to the needs of patients in tropical countries, fixed-dose combinations and paediatric formulations. This should complement the activities of existing organizations and provide a mandate for the recently established TransCelerate.

References

1. BIO Ventures Global health, Global Health Primer. Available at [www.bvgh.org](http://www.bvgh.org)
6. TransCelerate Biopharma Inc. Available at [http://transceleratebiopharmainc.com](http://transceleratebiopharmainc.com)