DNDi’s advocates for increased public responsibility and a more enabling environment for neglected disease R&D.
A Decade of R&D for Neglected Diseases


In December 2012 in New York, Lives in the Balance: Delivering Medical Innovations for Neglected Patients and Populations, an event co-organized by MSF, DNDi, and Mount Sinai School of Medicine, brought together over 300 participants from civil society, academia, the pharmaceutical and biotechnology industry, ministries of health, and funding bodies to look at the progress and shortcomings of the last decade in medical innovations for neglected diseases. This conference took place precisely 10 years after MSF had hosted a major gathering in the same city to examine the crisis in R&D for neglected diseases, which ultimately led to the creation of DNDi in 2003.

Despite incremental progress over the past decade, the essential health needs of the vast majority of the world’s population are still largely unmet, current R&D efforts are still too fragmented, and financing is still far too fragile. The December conference focused on the urgent need for genuine therapeutic breakthroughs for patients dying from drug-resistant tuberculosis (DR-TB), Chagas disease, and vaccine-preventable illnesses. New therapies to fundamentally transform the treatment of these and other neglected diseases, notably those with the highest death rates, have yet to make their way through costly clinical trials and reach patients in need.

Furthering the reflections of 10 years of R&D for neglected diseases, DNDi, MSF, and other partners undertook their own specific analysis of the R&D pipeline for neglected diseases. The study showed that while important inroads have been made, only a small fraction of new medicines developed between 2000 and 2011 were for the treatment of neglected diseases. It concluded that the ‘fatal imbalance’

New York DNDi-MSF Event (Dec. 2012):
A Global Call For Action

“The US is the largest public funder of neglected diseases... I say this not as a kudos to us, but as almost a challenge that we absolutely need to do more, and there’s no doubt about that. And despite the constraints in resources, because it’s neglected diseases doesn’t mean that we should continue to neglect.”

Dr Anthony S. Fauci
Director, National Institute of Allergy and Infectious Diseases, US National Institutes of Health

“[T]he current model of health innovation is failing millions of the world’s poorest and most vulnerable people. [W]e must work on two fronts. We need robust research and development mechanisms, producing new technologies for the diseases that afflict poor people. This means adequate, sustainable, global research investment, as well as more open approaches to sharing research knowledge. On the other hand, we must support countries to strengthen the delivery systems that will give poor people effective access to new drugs and technologies.”

Dr Jim Yong Kim
President, World Bank Group
Between 2000 and 2011, only 3.8% of newly approved drugs (excluding vaccines) were for tropical diseases, TB, and other neglected infections, which together account for 10.5% of the global disease burden.

Much of the progress in the treatment of neglected diseases and important patient benefit during this time came about through drug reformulations and repurposing of existing drugs against these illnesses.

Only four of the 336 brand-new medicines (new chemical entities, NCEs) developed between 2000 and 2011 were for the treatment of neglected diseases.

Three of the four brand-new medicines approved for neglected diseases in the past decade were for malaria, with none for the 17 neglected tropical diseases (NTDs) defined by the World Health Organization (WHO), nor TB.

As of December 2011, only 1.4% of a total of nearly 150,000 registered clinical trials were focused on neglected diseases.

Product development partnerships (PDPs) were responsible for over 40% of neglected disease products registered between 2000 and 2011, including new TB diagnostics and malaria combination treatments.

The study was also prompted by the need to gain insights into neglected-disease R&D, given the 2012 recommendations of the WHO Consultative Expert Working Group (CEWG) on Research and Development: Financing and Coordination. The CEWG produced an analysis indicating that, indeed, at such a crucial time in the history of neglected diseases, it is vital to establish essential health needs-based R&D priorities and ensure that additional and sustainable financing is guaranteed. This was substantiated by a recommendation that all countries initiate formal negotiations towards a global framework that would strengthen coordination and financing of R&D and ensure that the cost of R&D be de-linked from the price of products in order to meet the needs of developing countries. In addition, the WHO’s essential role in setting R&D priorities would be reinforced in the process.

DNDi published in 2012 a policy brief, Why an Essential Health R&D Convention Is Needed, to relate the findings of the CEWG report to its decade of experience in drug R&D for neglected diseases. DNDi’s ‘lessons learned’ included four key components:

- New financing mechanisms are necessary to provide adequate and sustainable funding, secure new funding sources, and engage public responsibility in addressing global health needs.
- R&D strategies based on open innovation models are critical to boost innovation globally, reduce duplication and costs of R&D, and speed up delivery of new medicines to patients. Such open innovation initiatives supported by public funding should be designed to secure access for patients by delinking the costs of R&D from the price of products, delivered as public goods.
- Increased involvement of disease-endemic countries in the coordination of R&D, especially in defining priorities based on patient needs and in allocating resources to identified priorities, is essential.
- Innovative regulatory pathways are needed to ensure timely patient access to treatments, reduce total costs of delivering treatments, and ultimately support greater capacity strengthening in disease-endemic countries.

DNDi receives prestigious awards from the BBVA Foundation, the Carlos Slim Health Institute, and the Rockefeller Foundation.

In early 2013, DNDi was honoured with the BBVA Foundation Award for Development Cooperation for Delivering New Treatments for Neglected Diseases with a EUR 400,000 prize. DNDi Latin America received the 2013 Carlos Slim Award for Innovations in Neglected Disease Drug Development with USD 100,000 for 10 years of exceptional work in the region. In addition, DNDi won a public voting competition for the Rockefeller Foundation’s Next Century Innovators Award.

Three Awards in 2013!
Neglected Diseases


Human African Trypanosomiasis (Sleeping sickness)


Leishmaniasis


Chagas Disease


Malaria


DNDi experienced a successful year of fundraising in 2012, securing over EUR 33 million, up from EUR 25.8 million in 2011 (28%). This increase was directly linked to the UNITAID funding in support of the paediatric HIV mini-portfolio. Since the creation of UNITAID in 2006, this was the first time the organization allocated resources for late-stage development and treatment implementation. DNDi also received funding from the French government and the Wellcome Trust, and additional support from the UK Department for International Development, Médecins Sans Frontières, and others.

Since its inception, DNDi has recognized that the contribution of both public and private donors is essential to ensure the initiative’s independence. Every effort is made to secure diversified funding from multiple sources and minimize earmarked donations to maintain the agility required to support research opportunities and deliver quickly.

In 2012, DNDi made important strides in securing funding from endemic emerging economies, notably the government of Brazil. An agreement was signed with the Ministry of Health of Brazil, the Oswaldo Cruz Foundation (FIOCRUZ), and DNDi, uniting the three actors in a strategic partnership to collaborate on R&D for new therapies and diagnostics for neglected diseases in the region.

At year-end, donors had committed over EUR 217 million, building on the EUR 184 million in 2011, since the launch of the initiative. The overall funding goal for DNDi is to secure EUR 400 million by 2018. However, considering the financial crisis in Europe and economic constraints of a number of important donor countries, new funding mechanisms will be vital to bringing additional and sustainable resources to support DNDi and others. Much expectation resides with the Global Health Innovation Technology Fund (GHIT Fund), set up by the government of Japan together with Japanese pharmaceutical companies and the Bill & Melinda Gates Foundation, launched in 2013. Encouragingly, the European and Developing Countries Clinical Trials Partnership (EDCTP) announced in 2012 an expansion of its scope to include all phases of clinical trials and neglected infectious diseases.
Over the past four years, DNDi has experienced a slight shift toward restricted funding. Ever so minimal, the percentage of grant funding is leaning toward specific diseases or R&D projects. While the ratio is still relatively balanced, greater efforts will be exerted in the coming years to recalibrate the proportion of restricted versus unrestricted funding. Unrestricted funding has been key to DNDi’s success to date as it allowed the organization to respond quickly to research opportunities and also terminate projects that do not meet targeted goals set forth in the Business Plan. In 2012, DNDi received significant earmarked contributions from UNITAID and the Wellcome Trust, shifting the scale to restricted funding. MSF and DFID (UK) also contributed additional funding to support core activities.

The diversification of donors increased in 2012. DNDi welcomed additional donors including UNITAID, which pledged EUR 13.1 million toward paediatric HIV activities. To develop its activities and meet its objectives, DNDi seeks diversified sources of funding from public and private donors, which include financial contributions from governments, public institutions, private individuals, foundations, founding partners, and innovative funding mechanisms.

Concerted efforts are made to ensure that no one donor contributes more than 25% toward DNDi’s Business Plan, and that at maturity, half of DNDi’s budget is covered by public funds and half by private funds. In 2012, the public-private balance was maintained as per DNDi’s fundraising strategy. The ratio grew to 53% public support and 47% private support due to funding received from the EU Seventh Framework Programme Grant, the Agence Française de Développement, UNITAID, and the Swiss Agency for Development and Cooperation and DFID’s additional contributions. New grants from private donors included the Wellcome Trust and Médecins Sans Frontières.

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Challenge of a growing tendency toward restricted grants—limiting portfolio management flexibility

Evolution of restricted versus unrestricted grants between 2009 and 2012

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<thead>
<tr>
<th>Year</th>
<th>Restricted</th>
<th>Unrestricted</th>
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<tbody>
<tr>
<td>2009</td>
<td>34%</td>
<td>66%</td>
</tr>
<tr>
<td>2010</td>
<td>46%</td>
<td>54%</td>
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<tr>
<td>2011</td>
<td>54%</td>
<td>46%</td>
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<tr>
<td>2012</td>
<td>55%</td>
<td>45%</td>
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Restricted grants currently include a new category, increasingly proposed by donors: “portfolio grants”. These grants are attributed to various diseases and various projects. While still restricted, they do allow for a certain degree of risk mitigation within restricted grants overall. Portfolio grants were estimated at 18% of the 2011 total income and 22% for 2012 total income.