

# 15 YEARS OF NEEDS-DRIVEN INNOVATION FOR ACCESS

## Key lessons, challenges, and opportunities for the future

The Drugs for Neglected Diseases *initiative* (DNDi) was created as a response to the frustration of clinicians and the desperation of patients faced with medicines that were ineffective, highly toxic, unavailable, unaffordable – or that had never been developed at all.

DNDi was launched in 2003 when the Indian Council of Medical Research (ICMR), the Oswaldo Cruz Foundation in Brazil, the Kenyan Medical Research Institute (KEMRI), the Malaysian Ministry of Health, and the Institut Pasteur of France, with the participation of the World Health Organization Special Programme on Research and Training in Tropical Diseases (WHO/TDR), teamed up with Médecins Sans Frontières (MSF), after MSF dedicated a portion of its 1999 Nobel Peace Prize award to exploring a new, alternative, not-for-profit model for developing drugs for neglected populations.

**It is hoped that the lessons described can spark debate, inform policy-making, and ultimately improve the ability of health and R&D systems to deliver necessary treatments for neglected patients.**

This is a summary of a longer report documenting the 15 years of experience that DNDi has now accumulated discovering, developing, and delivering new and improved treatments for neglected patients. It highlights both achievements and challenges, and aims to contribute to current global discussions about how to foster and sustain alternative approaches to innovation in the public interest. It is hoped that the lessons described can spark debate, inform policy-making, and ultimately improve the ability of health and research and development (R&D) systems to deliver necessary treatments for neglected patients while offering ideas for a more effective and equitable approach to biomedical innovation that may be applicable to other diseases and product types.

## Evolution of the DNDi model, 15 years on

DNDi has been an ‘experiment in innovation’, both in what it does – develop urgently needed treatments for neglected populations – and how it does so: testing an alternative virtual R&D model, based not on profit maximization but on patient needs, which aims to promote the broadest possible sharing of research knowledge and data through a collaborative approach, and which seeks to ensure both innovation and affordable access to new and improved treatments with the desire to develop drugs as public goods wherever and whenever possible.

Since 2003, eight new treatments have been discovered, developed, and delivered – reducing illness, suffering, and death for millions of people – and the pipeline for some of the world’s most neglected diseases has started to be replenished, thanks to long-term investments in drug discovery.

Photo: Xavier Vahed/DNDi



Read the full report: [www.dndi.org/15yearsreport](http://www.dndi.org/15yearsreport)

There are several distinctive features of DNDi's alternative, not-for-profit R&D model. These revolve around six central tenets:

## 1. NEEDS-DRIVEN

### Putting patients – not profits – at the heart of R&D

- Proximity to local treatment providers and close engagement with key stakeholders such as WHO, MSF, and affected communities are essential to ensure R&D efforts remain rooted in the medical needs of neglected populations and the contexts in which they live.
- Public-interest Target Product Profiles (TTPs) developed with experts and partners are critical tools to ensure that products developed are both affordable for and specifically adapted to the needs of the people affected and the health systems that serve them.
- A dynamic approach to managing an R&D portfolio can allow product developers to adapt to new, emerging, and persistent R&D needs and gaps, and respond to evolving epidemiological trends.

## 2. INDEPENDENT

### Ensuring financial and scientific independence to guarantee a needs-based approach to priority-setting and decision-making

- Scientific independence is critical to identifying target diseases, setting R&D priorities, and driving decision-making during the drug development process.
- A deliberate funding policy that safeguards independence is most effective when it ensures a balance of public and private support, maximizes unrestricted support from key donors, and guarantees that no single donor contributes more than 25% of overall funding.

## 3. COLLABORATIVE, OPEN & TRANSPARENT

### Harnessing the public, private, academic, non-profit, and philanthropic sectors to bring the best science to the most neglected and drive knowledge creation through open drug discovery, and aiming to share research data, knowledge, and costs

- Collaborative R&D organizations act as 'conductors of a virtual orchestra' and cannot function effectively without the engagement of public and private partners sharing a common vision to implement projects at all stages of the R&D process.
- More collaborative and open approaches to R&D, particularly drug discovery, can attract additional researchers to a neglected field, accelerate the R&D process by reducing duplication and generating a greater volume of hits or leads, and make R&D activities more efficient and less expensive. Innovative approaches to discovery can also contribute to unlocking capacity in low- and middle-income countries (LMICs) in particular.
- More open research collaborations could be facilitated, and duplication reduced, if public and private research funders developed clear policies to encourage openness and sharing of data, knowledge, and costs at each stage of the R&D process.
- Global health R&D actors should be encouraged to sign the WHO Joint Statement on Public Disclosure of Results from Clinical Trials and commit to registering all trials in a publicly available register, promptly reporting trial results 12 months after completion of the trial, and publishing findings in open access journals.

### R&D costs: how much does it cost DNDi to develop a drug?

DNDi's historical data on eight of its drug development projects show out-of-pocket expenses ranged from €4 to €60 million per treatment developed, up to and including registration. Adjusting these figures for average attrition costs per phase of development, DNDi estimates it can develop and register: new treatments that combine or repurpose existing drugs for €4-32 million; and a new chemical entity for €60-190 million. These figures do not include post-registration additional studies and access costs, nor in-kind contributions from pharmaceutical partners.

You can read more about DNDi's costs and costing methodology at: [www.dndi.org/costs](http://www.dndi.org/costs)



Photo: Ana Ferreira/DNDi

## 4. GLOBALLY NETWORKED

**Facilitating scientific exchange, utilising and strengthening research capacity, and nourishing innovation ecosystems and networks, particularly in LMICs**

- A virtual, collaborative R&D organization can only succeed with strong partnerships and alliances and a global network. Leadership from the public sector, particularly in LMICs, is essential to ensuring sustainable innovation ecosystems.
- Proximity to the needs of affected communities and patients is critical and can only be achieved through building trusting and equal partnerships with local clinicians, scientists, and experts, as well as patient and community/civil society groups in affected countries.
- In LMICs, innovative partnerships throughout the R&D process leverage and strengthen existing research capacity, facilitate needs definition, promote scientific exchange, and enable access. In addition, targeted investments in training and health infrastructure improvements, including in remote settings, are critical for success.

## 5. ACCESS-ORIENTED

**Making sure treatments are affordable, available, and adapted to the communities who need them most**

- Even for R&D organizations, it is critical to work with partners and treatment implementers to overcome the considerable challenges related to introducing and ensuring access and delivery of new health technologies and tools.
- Access must be prioritized from the outset of any R&D project, not only at a late stage or after regulatory approval; R&D programmes should be developed with access in mind, and TPPs should include key elements to ensure affordability, availability, and field feasibility.
- Developing robust collaborations with industrial partners is essential to securing sustainable production, supply, and distribution, and engaging key stakeholders, including affected communities, is vital to ensuring public leadership and community support from the beginning.
- Critical to success is ensuring sustainability of production; in some instances, technology transfer can be key to assuring sustained affordability and access.



Photo: Felipe Abondano/DNDi

## 6. TRANSFORMATIVE

**Piloting and incubating new approaches to innovation that promote public health-driven R&D, fostering public leadership, and engaging as an informed advocate for a more effective and equitable biomedical R&D system**

- Establishing an intellectual property policy and making it publicly available can be fundamental to achieving 'gold standard' pro-access licensing terms in contractual agreements.
- Negotiations are more complex when operating in 'competitive' fields and/or when they begin at a later stage in the development process, but this does not prevent pro-access approaches when the pharmaceutical partner has a commitment to access and when countries are prepared to make use of TRIPS flexibilities. Nevertheless, it would be helpful if access provisions were included at an earlier stage in the R&D process when public or philanthropic funds are used.
- Regulatory bottlenecks remain a challenge, but regulatory strategies should aim to secure technical and scientific review that is rigorous in terms of quality and patient safety, appropriate to the public health context, and rapid.
- Important initiatives aimed at regional regulatory harmonization for optimizing review of dossiers should be supported.
- Public leadership and public policies to address market failures – including those that guarantee a public return on public investment in R&D and that enable the setting of R&D priorities by affected countries – are critical to create a more effective, equitable, and needs-driven global biomedical R&D system.



## Conclusions

### After a period of tremendous growth in global health

financing from 2000-2010 – a ‘golden era’, during which billions of dollars were mobilized to support programmes in LMICs primarily for HIV/AIDS, tuberculosis, malaria, and maternal and child health, leading to tens of millions of people receiving treatment or vaccines, and unprecedented declines in under-five mortality, for example – **the growth trend appears to be waning.**

At the same time, the rapidly changing and volatile political environment, particularly the rise in nationalism across the globe, threatens multilateral initiatives and other investments in global health, including bilateral overseas development assistance, and further marginalizes or directly targets vulnerable populations.

Meanwhile, emerging infectious diseases and epidemic-prone diseases, non-communicable diseases, and antimicrobial resistance all loom large as massive global public health challenges. Responding to these challenges will require a redoubling of efforts to discover, develop, and deliver new health tools. But while the need to address technology gaps that hamper effective diagnosis, treatment, and prevention of diseases is gaining prominence in discussions about Universal Health Coverage and the Sustainable Development Goals, the risk of fragmentation in the absence of an overarching and sustainable framework to govern and drive public interest R&D hampers progress.

Key questions remain for DNDi and other global health R&D actors concerned about the sustainability of a more needs-driven innovation system that guarantees equitable and affordable access to new health technologies.

### What national, regional, and international mechanisms need to be put in place to steer biomedical innovation

so that it responds to priority needs? What will be the new sources of funding that will sustain needs-driven innovation, access, and delivery? What ‘safeguards’ need

to be in place to encourage collaboration, openness, and transparency, and ensure innovations of public health importance are affordable and accessible to all? What new economic models for financing R&D, including incentive mechanisms, will emerge, and will such incentives be directed at the right players at the right stage of the R&D process to ensure innovation and sustainable access? What new areas of collaboration can be explored to mutualize resources and address persistent ‘access bottlenecks’, such as manufacturing, registration, and supply, and how can existing procurement and distribution systems be better leveraged? Are new technologies that have the potential to radically transform human health and human lives being designed or implemented with equity in mind? What new opportunities exist to further develop South-South and triangular partnerships, which foster the creation of approaches to R&D and ‘knowledge hubs’ led by LMICs?

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Looking ahead to the next decade, global health R&D stakeholders will need to confront these challenges head-on. For its part, DNDi pledges to do so with a renewed commitment to addressing the needs of neglected populations, a willingness to continue to test novel approaches to R&D that can accelerate innovation in the public interest, and a steadfast commitment to sharing its experience in order to support the emergence of a more effective and equitable biomedical innovation system – one that delivers **affordable and accessible treatments and other health tools designed specifically for the people and places that need them most.**

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