Partnering for R&D for the Most Neglected

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This is the third part to our Product Development Partnership (PDP) mini-series. Here, Bernard Pécoul from the Drugs for Neglected Disease initiative (DNDi) discusses their experience with partnerships to develop treatments for neglected diseases.

When I was a field doctor working in Central America, Southeast Asia, and Africa in the 1980s, some of the most difficult patients to treat were those infected with sleeping sickness, also known as human African trypanosomiasis. In its late stage, this neglected disease affects the brain, causing severe neurological disturbances. If left untreated, sleeping sickness is fatal.

At the time, and even up to just ten years ago, the only treatment option for late-stage sleeping sickness was melarsoprol, an arsenic-based drug that is toxic and painful to administer—sometimes referred to as "fire in the veins"—killing 1 in 20 patients.

You do not feel very proud to be a doctor when the only option for treating your patients is a drug that may kill them. I and other clinicians in the field found this unacceptable. Our frustration with the lack of safe, effective drugs for neglected diseases like sleeping sickness led to the formation of the Drugs for Neglected Diseases *initiative* (DNDi).

Two years ago, the first new treatment for sleeping sickness in 25 years, nifurtimox-eflornithine combination therapy (NECT), became available. It was the culmination of years of dedicated field research by DND*i*, Médecins Sans Frontières/Doctors Without Borders (MSF), Epicentre, the Swiss Tropical and Public Health Institute (Swiss TPH), and the Ministries of Health of the Democratic Republic of Congo (DRC) and the Republic of Congo (RoC).

NECT is a significant therapeutic advance and has helped do away with the use of melarsoprol. However, it is not ideal: it still requires intravenous infusions, close monitoring, and hospitalization, all of which are major challenges in the resource-poor settings and conflict areas in which sleeping sickness is prevalent.

DNDi is therefore now working on researching and developing an oral drug option for sleeping sickness, which could eliminate intravenous infusions, hospitalization, and painful diagnostic procedures (lumbar punctures) to determine disease stage. Two compounds are currently in our pipeline: fexinidazole, in partnership with Sanofi and Swiss TPH; and the oxaborole compound SCYX-7158, in partnership with Anacor Pharmaceuticals, SCYNEXIS, and Pace University, among others. These product-development partnerships have helped us create a robust drug-candidate pipeline for sleeping sickness, potentially bringing new hope to patients and clinicians.

DNDi was established in 2003 by MSF, the Indian Council for Medical Research, Brazil's Oswaldo Cruz Foundation, the Kenya Medical Research Institute, the Ministry of Health of Malaysia, and the Pasteur Institute in France, with the UNICEF/UNDP/World Bank/World Health Organization's Special Programme for Research and Training in Tropical Diseases (WHO TDR) as a permanent observer. We are dedicated to developing new, field-adapted treatments for patients suffering from neglected tropical diseases, including those with the highest case-fatality rates: sleeping sickness, Chagas disease, and kala azar (visceral leishmaniasis). Malaria was also an early focus of DNDi, and new R&D programs for pediatric HIV and specific helminth infections were added this year.

Our goal is to deliver 6 to 8 new treatments by 2014—and we're halfway there, with 4 treatments delivered since 2003: NECT, two fixed-dose antimalarials (artesunate-amodiaquine [ASAQ] and artesunate-mefloquine [ASMQ]), and a combination treatment for kala azar in Africa (sodium stibogluconate and paromomycin [SSG&PM]).

DNDi exists because of the alarming lack of R&D for the most neglected diseases in the world, which affect the poorest people but do not represent lucrative "market potential" for the pharmaceutical industry. Our objective at DNDi is to address this terrible gap between neglected patient needs and R&D and to bridge the innovation-to-access divide.

But we cannot—and do not—do this alone. In fact, we can only accomplish our work through innovative and collaborative South-South and North-South partnerships. Our involvement with developing a new treatment doesn't end with drug approval or registration; we also take on the responsibility of ensuring access for the patients who need these new drugs the most. This is done by making sure the new treatments are affordable and have a robust manufacturing, distribution, and implementation network behind them, through partnerships with pharmaceutical and biotech companies, international bodies such as WHO, nongovernmental organizations like MSF, and governments.

Moreover, while our primary focus is on delivering new treatments for neglected patients, a further objective is to bolster local research and health-care capacity in endemic countries. This is accomplished through our regional disease-specific R&D platforms, including the Leishmaniasis East Africa Platform (LEAP), HAT Platform for sleeping sickness, and Chagas Clinical Research Platform in Latin America. Such capacity strengthening includes the building and renovation of hospital wards, clinics, and health posts; renovation and equipping of clinical laboratories; and training of health-service personnel in clinical trial methodology, Good Clinical Practice and Ethics, patient treatment and evaluation, accurate diagnosis and follow-up by parasitology, and safety.

And finally, we cannot accomplish our goals without also working to ensure that sound policies are in place to enable greater patient needs-driven R&D. This includes appropriate incentives and financing mechanisms, innovative regulatory pathways that will expedite access, and open innovation approaches that ensure the widest possible sharing of research knowledge and data.

Establishing partnerships and enabling policies are key elements of DNDi's start-to-finish approach—from drug discovery to treatment implementation—to tackle the urgent health needs of the most neglected patients.

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