NEW HOPE
FOR NEGLECTED PATIENTS

DNDi

Drugs for Neglected Diseases *initiative*

Acting in the public interest, the Drugs for Neglected Diseases *initiative* (DND*i*), in collaboration with the international research community, the public sector, the pharmaceutical industry and other relevant partners, discovers, develops, and makes available new therapies for patients suffering from some of the most neglected communicable diseases.

MILLIONS OF PATIENTS ARE IN NEED OF TREATMENT

Neglected diseases debilitate, disfigure, blind, or kill their victims

Despite major progress in medicine over the past 50 years, over 1 billion people, including 500 million children, continue to be affected by diseases for which adequate treatments are not available. Neglected diseases can leave patients bedridden and unproductive for weeks or months and perpetuate poverty. The poorest of the poor – particularly women and children mainly in Africa, Asia, and Latin America – living on one or two dollars a day are the hardest hit.

There is little incentive for research and development (R&D) of treatments that are better or entirely new. Existing treatments can be too expensive or not adapted to the medical needs of patients. But even worse, in some cases, adapted, safe, and effective treatments simply don't exist.

Angèle

"I had terrible headaches and chills all night long, for six months. The mobile team came to my village, and diagnosed me with sleeping sickness. I travelled two days on foot, four months pregnant, and finally reached the hospital where I was treated with NECT."

Angèle, 24 years old, mother of three and subsistence farmer, treated in Masi Manimba Hospital, Democratic Republic of the Congo.

Thanks to NECT, a treatment developed by DND*i* and its partners, patients with sleeping sickness have a safe and effective life-saving solution.

Angèle's life was saved, but others do not have the same opportunity.

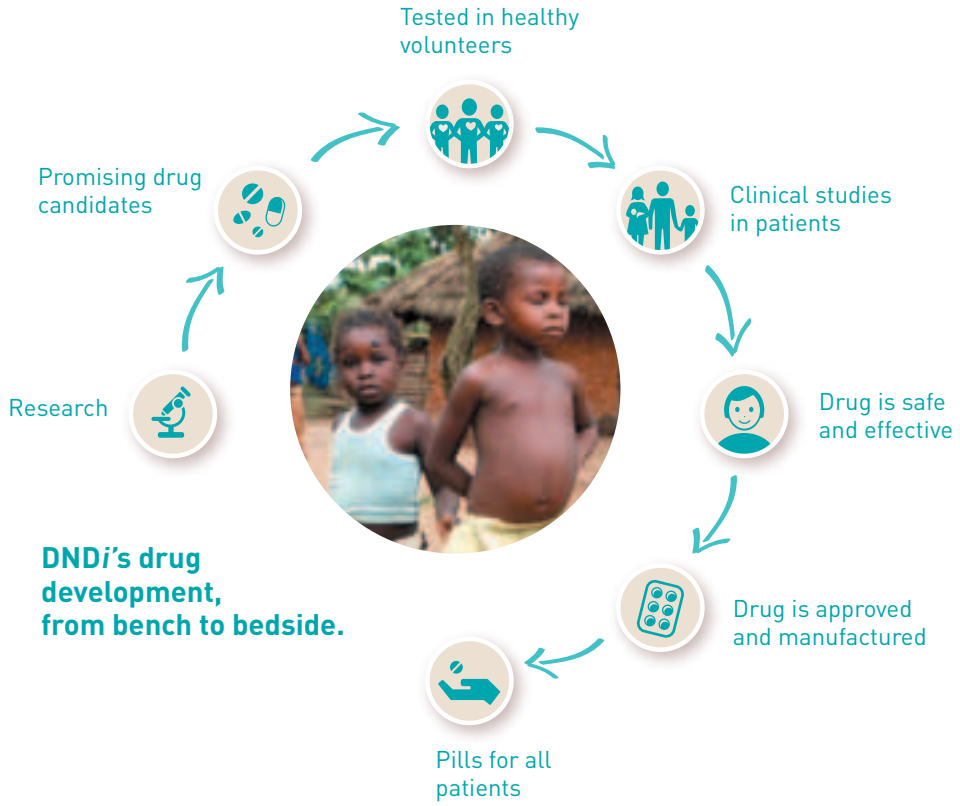
HOW DO WE FIGHT NEGLECTED DISEASES?

Working from 'A to Z' to turn a molecule in a lab into a pill in a patient's hand

Patients' needs – not financial profits – are at the core of our drug development strategy. As a 'conductor of a virtual orchestra', DND*i* brings together different partners and expertise worldwide with the objective of developing 11 to 13 new treatments by 2018.

Our ultimate goal is to develop simple, oral, safe, and effective treatments that are easy to use in areas with limited healthcare systems.

This process starts with researchers in laboratories who test hundreds of thousands of molecules, usually provided by pharmaceutical companies, to see which ones are active against each disease. Promising drug candidates are tested in healthy volunteers. Finally, patients are treated with the medicines in clinical studies where these neglected diseases are endemic. If the drug is safe and cures the disease, it is manufactured, and approved by the World Health Organization and national health authorities.



A global network of partners contributes to DND*i*'s mission

In over 40 countries, DND*i* works with a range of public and private partners, including 50 public research institutes and universities, 20 pharmaceutical and biotechnology companies, governments, several non-governmental organizations and other civil society groups, to develop non-patented treatments as public goods and ensure they are accessible and affordable.

Building research capacity in countries and communities directly affected by the diseases

DND*i* has established regional disease-specific research platforms, or networks, that **support and build local capacity to conduct clinical trials in centres close to the patients.** Infrastructure and training are provided to ensure international standards are met.

In this way, DND*i* accelerates drug development and brings down the costs. Short-term strategies aim to improve existing treatments, while long-term development strategies focus on brand-new 'breakthrough' drugs.

NEGLECTED DISEASES MAY NOT MAKE THE HEADLINES,

Focused research, concrete results, much-needed treatments

SLEEPING SICKNESS

Sleeping sickness, or human African trypanosomiasis (HAT), is transmitted by the bite of the tsetse fly. It can cause severe mental debilitation and coma. Left untreated, sleeping sickness kills.

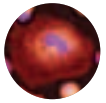
- 60 million people at risk
- 36 countries in sub-Saharan Africa, but 8 countries report 97% of all cases, over two-thirds of which in the Democratic Republic of the Congo

"Not so long ago we had to treat sleeping sickness patients with an arsenic derivative, and the stress on the staff was immense. We all experienced a patient dying from the treatment and, as doctors, it was unbearable to explain this to the families. Since NECT was developed, we experienced a first revolution in care. But an oral-only treatment would be a true transformation!"

Dr Nganzobo Pathou, Chief of Staff, Bandundu General Hospital, DRC

DNDi's current work

- Increase patient access to NECT, a treatment developed in 2009 by DND*i* and partners
- Complete clinical testing of two new oral-only drug candidates (fexinidazole, oxaborole SCYX-7158)



LEISHMANIASIS

Leishmaniasis is transmitted by the bite of a female sandfly. Visceral leishmaniasis (VL; or kala-azar) causes fever, weight loss, spleen/liver swelling, anaemia, and is fatal if untreated. Cutaneous leishmaniasis (CL) causes disfiguring skin lesions and social stigma.

- 350 million people at risk in 98 countries
- Kala-azar mainly affects children below 5 years of age, mostly in the Indian sub-continent and East Africa
- 70% of the cases are children under 12 and some 15,000 children die every year

"At first, I took herbal medicines and my health got worse. My neighbour told me about the health centre where I could get kala-azar treatment. The first morning I received my injection, I felt relief from the fever, headache, and weakness. I found out it was a new treatment that only took 17 days. This was good news. Right now everything back home is on hold."

Lemarus, 23 years old, pastoralist and farmer, East Pokot District, Kenya

DNDi's current work

- Increase patient access to SSG&PM 17-day treatment, developed in 2010 by DND*i* and partners, in East Africa and combination therapies in Asia
- Test shorter, cheaper, and safer treatments using current drugs
- Find promising new oral drug candidates

BUT THEY AFFECT OVER A BILLION PEOPLE WORLDWIDE

CHAGAS DISEASE

Chagas disease (American trypanosomiasis) is transmitted by the bite of the 'kissing bug', as well as from mother to newborn. It is the leading cause of infectious heart disease in Latin America.

- 100 million people at risk, mainly in the Americas
- 8 million people infected, leading to approximately 12,000 deaths every year
- 2-10% of mothers infected with Chagas disease in endemic regions in Argentina and Bolivia, for example, transmit the disease to their babies

My family found out that we were all infected with Chagas disease after my father passed away with heart failure from the disease. We were all treated immediately, but my 25 year-old brother suffered from drug side-effects and already has a heart condition."

Daniel, 27 years old, Cochabamba, Bolivia

DNDi's current work

- Implement use of paediatric benznidazole
- Test shorter, cheaper, and safer treatment options using current drugs
- Find promising new oral drug candidates
- Advocate for increased patient access to treatment (currently only 1%)

FILARIAL DISEASES

Filarial diseases are caused by parasitic worms transmitted by biting flies and mosquitos. They inflict blindness, swollen limbs and genitals, intense itching, and chronic pain.

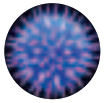
- 1.5 billion people at risk
- 25 million people worldwide infected with river blindness, the world's second leading infectious cause of blindness
- Over 120 million people are infected with elephantiasis, with about 40 million disfigured or incapacitated

Some people don't want to come near me or touch me because of my condition. I cannot take care of any of my children and send them to school because I cannot work."

Akua Nyarku, 52 years old, Ghana

DNDi's current work

- Develop a drug that kills adult worms (macrofilaricide) that can be used to shorten the duration of current mass drug administrations (MDAs), and used in individual patient treatment



PAEDIATRIC HIV/AIDS

Without treatment, half of the children infected with HIV will die before the age of two, and 80% by the age of five.

- 3.3 million children with HIV/AIDS
- 700 newly infected babies every day, about 600 die each day, mostly in sub-Saharan Africa

The current medications have a terrible taste that just makes the child want to vomit... this is where mothers struggle. What we really need is medication that is palatable for the child, user friendly for the parent, can be stored at room temperature, and ideally would have four medications in one formulation!"

Dr Els Dobbels, paediatric HIV specialist, Tygerberg Hospital, South Africa

DNDi's current work

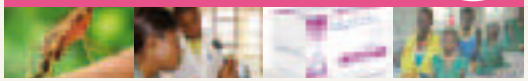
- Develop two simple, all-in-one, taste-masked antiretroviral (ARV) formulations designed for babies and young children, and that requires no refrigeration
- Develop a 'booster' formulation for HIV/TB co-infected children

SIX NEW TREATMENTS DELIVERED AND A PIPELINE OF

Easy-to-use, affordable, field-adapted, non-patented

Malaria

ASAQ 2007



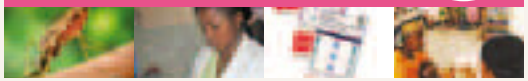
(Fixed-dose combination of artesunate + amodiaquine)

- Innovative partnership with Sanofi
- Simple regimen: 1 or 2 tablets once a day for 3 days
- Registered in 35 countries, of which 31 in Africa
- WHO prequalified
- WHO Essential Medicines List (adults and children)

320 million
treated in 31 African countries

Malaria

ASMQ 2008



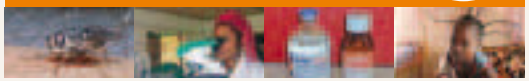
(Fixed-dose combination of artesunate + mefloquine)

- Developed by DND*i* and Farmanguinhos/Fiocruz, Brazil
- Simple and adapted regimen for children and adults
- Registered in Brazil (2008), India (2011), Malaysia and Myanmar (2012), Tanzania (2013), Vietnam and Niger (2014)
- South-South technology transfer from Farmanguinhos to Cipla, India
- WHO prequalified (Cipla)
- WHO Essential Medicines List (adults and children)

1.2 million
treated in Latin America and Asia

Sleeping Sickness

NECT 2009



(Nifurtimox-eflornithine combination therapy)

- Partnership between DND*i*, MSF, governments, pharmaceutical companies, and WHO
- Approximately 96% of all stage 2 sleeping sickness patients in endemic countries treated with NECT (2013)
- WHO Essential Medicines List (adults and children)
- On essential medicines lists of 12 African countries (covering 98% of reported cases)

13,000
treatments in Africa

Visceral Leishmaniasis

SSG&PM 2010

(Sodium stibogluconate & paromomycin combination therapy)

- Partnership between DND*i*, the Leishmaniasis East Africa Platform (LEAP), national control programmes of Kenya, Sudan, Ethiopia, and Uganda, MSF, and WHO
- Recommended by the WHO Expert Committee on the Control of Leishmaniases for East Africa (2010)
- National VL guidelines of Sudan, South Sudan, Kenya, and Ethiopia
- Paromomycin registered in Uganda (2011), in Kenya (2013), and underway in other East African countries

25,000
treated in East Africa

Visceral Leishmaniasis

NEW VL treatments in India 2011

(SD AmBisome® / PM+M / M+A®)

- Large-scale implementation programme with health authorities at state, national, and regional levels
- High efficacy and good safety profiles
- Field-adapted
- Recommended by the WHO Expert Committee on the Control of Leishmaniases (2010)

SD AmBisome® and PM+M
recommended
in revised Indian VL elimination roadmap

Chagas Disease

Benznidazole 12.5 mg 2011

(Paediatric dosage form of benznidazole)

- Partnership with LAFEPE, Brazil
- Age-adapted, easy-to-use, and affordable treatment
- Easily dispersible tablet for children under 2 years of age
- Registered in Brazil in 2011
- WHO Essential Medicines List
- Agreement with Mundo Sano Foundation for second source (2013)

Only child-adapted
dosage form

PROMISING NEW DRUGS

13 brand-new drug candidates
(new chemical entities)
in the pipeline

25 clinical studies undertaken
in a decade in remote, rural, resource-limited or conflict-affected areas;
33,000 patients enrolled

57 trial sites worldwide
for **13 projects** in clinical development

All studies conducted in accordance with international **ethical and quality standards**

Evalyne

"I didn't recognize my symptoms. I came to Kimalel, where I knew I would get treatment, whether it was malaria or kala-azar."

Access to life-saving treatment is a human right.

Evalyne is a 20-year old mother to a baby boy. She lives in Marigat, a small town in Kenya's Rift Valley. When she started feeling weak, nauseous, and developed a fever, she knew that she should seek treatment.

So she and her baby travelled 15 km to Kimalel Health Centre, which is supported by DND*i*. There Evalyne was diagnosed with kala-azar and given treatment with SSG&PM, developed by DND*i* and partners. She was looked after by the Kimalel staff and could continue breastfeeding her son during her stay.

Everyone can contribute – whether an individual donor, public funder, corporation, or foundation – and help support the development of new treatments by funding an entire project, or fight against a specific disease through training programmes, clinic renovation, or purchase of clinical trial equipment for local laboratories.

JOIN US IN COMBATING NEGLECTED DISEASES!

DND*i* has created an innovative R&D model by pooling expertise from around the world to address the needs of patients suffering from neglected diseases.

DND*i* has delivered six new treatments to millions of patients, but much more needs to be done to act on our promise to develop and make available simpler, more affordable treatments. From early discovery research to ensuring treatments get to patients in hard to reach places, we need your support.

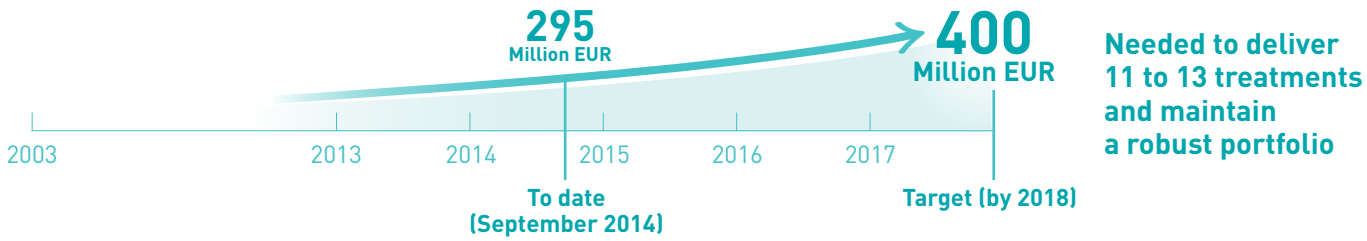
Help us change the course of neglected diseases by supporting the development of new treatments today.

Keeping a balance between public and private support

Public contributions from governments and other institutional donors

Private contributions from foundations, philanthropists, and individuals

We all have a role to play, be it big or small



Needed to deliver 11 to 13 treatments and maintain a robust portfolio

DND*i*

Drugs for Neglected Diseases *initiative*

The Drugs for Neglected Diseases *initiative* (DND*i*) is a patient-needs driven, not-for-profit research and development (R&D) organization that develops safe, effective, and affordable treatments for neglected diseases that afflict millions of the world's poorest people, notably human African trypanosomiasis (sleeping sickness), leishmaniasis, Chagas disease, paediatric HIV, filaria, and malaria.

BEST SCIENCE FOR THE MOST NEGLECTED

DND*i*'s vision

To improve the quality of life and the health of people suffering from neglected diseases by using an alternative model to develop drugs for these diseases and by ensuring equitable access to new and field-relevant health tools.

Founding Partners

- Médecins Sans Frontières (MSF) / Doctors Without Borders
- Indian Council of Medical Research, India
- Kenya Medical Research Institute, Kenya
- Ministry of Health, Malaysia
- Oswaldo Cruz Foundation, Brazil
- Institut Pasteur, France
- World Health Organization (WHO) Special Programme for Research and Training in Tropical Diseases (TDR) (permanent observer)

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