

R&D for **Neglected Tropical Diseases**



How new tools can transform the fight

Neglected tropical diseases (NTDs) are a group of diseases that exert a crushing burden in poor and underserved communities around the world. Named for the limited attention they have historically received, NTDs are inextricably linked to poverty and inequality. These diseases exact a heavy toll, causing chronic illness, severe disfigurement, blindness, or even death. They can also exacerbate malnutrition and delay cognitive development, as well as reduce economic productivity, trapping families and communities in poverty. Increased attention in recent years has brought new resources to the fight against NTDs and fueled research breakthroughs. Yet, very significant gaps remain in the arsenal of tools needed to control and eliminate these diseases, underscoring the need for the research and development (R&D) of new tools.

1billion+ people worldwide are affected by NTDs

179 countries

reported an NTD in 2021

21 conditions
are classified as NTDs

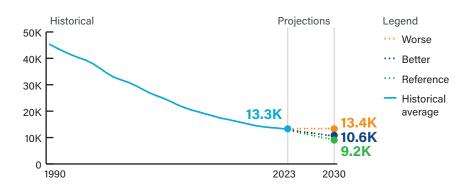
Research successes

Technologies have transformed the fight against NTDs:

- The world's first vaccine for chikungunya, a disease causing debilitating joint pain, which was developed with DoD support, was approved in 2023.
- A second dengue vaccine that is safe for use in individuals with and without prior infection was approved in 2022.
- The first all-oral cure for sleeping sickness, a fatal neurological disease, was approved in 2018. Fexinidazole cures all stages of the disease in ten days, replacing prior treatments that involved two weeks of intravenous injections at a hospital.
- The first new drug for river blindness in 20 years, moxidectrin, was approved in 2018 to treat this disfiguring and blinding disease.
- Child-friendly drugs for schistosomiasis and Chagas disease were approved in 2024 and 2011, respectively, enabling children to receive appropriate care for these painful parasitic diseases.
- New shorter, more effective combination therapies for visceral leishmaniasis—a disease causing spleen and liver damage—, introduced in the 2000s, are improving care.
- New diagnostics, including rapid tests for river blindness and elephantiasis, developed with NIH research, and a test that detects all four types of dengue, developed by CDC, have been introduced.

Continued progress is possible, not inevitable

Prevalence of 15 NTDs per 100,000 people



© Key missing tools

To eliminate NTDs, we need new and improved tools, including:

- New and improved diagnostics to rapidly detect infection at the point of care in lowresource settings and, in some cases, to distinguish between different strains or stages of a disease.
- Vaccines to prevent and treat infection. Beyond dengue and chikungunya, no vaccines exist for the remaining 20 NTDs prioritized by the World Health Organization.
- Antivenoms to treat snakebites.
- New vector and biological control tools, like space spray insecticides and Wolbachia bacteria
 that disrupt reproduction, to reduce mosquito and other vector populations.
- New and improved treatments and cures, including therapies designed for children and shorter, simplified regimens with fewer side effects. While there are low-cost, effective interventions to combat some NTDs, in many cases, these available treatments are still lengthy and burdensome with significant side effects.

Breakthroughs on the brink

- An all-oral, single-dose treatment for sleeping sickness, acoziborole, which is in late-stage development, could simplify treatment administration, laying the groundwork for the eventual elimination of this deadly disease. A pediatric version of this medicine is also in development.
- An oral treatment for dengue, developed with NIH support, showed promising results in Phase 2 clinical trials, offering hope that in the coming years, we will see the first-ever treatment for this disease.
- Researchers have created a synthetically produced antibody that can neutralize a neurotoxin found in the venom of four different deadly snake species, laying the foundation for the eventual development of a universal antivenom for snakebites.

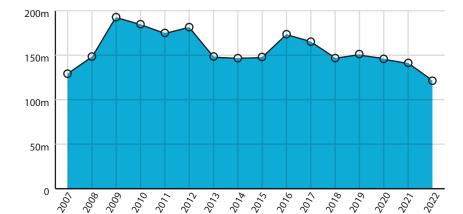


- Fosravuconazole, a potential new weekly treatment for eumycetoma, a fungal disease that can cause severe deformity of the limbs, demonstrated
 promising results in Phase 2 trials. Existing treatments for this disease are toxic, costly, only cure a fraction of patients, and require daily dosing.
- Two NIH-funded vaccine candidates against hookworm infection are in clinical development, including one in Phase 2 development. This parasitic
 infection causes gastrointestinal issues and protein deficiency and is linked to impaired learning in children.
- Several diagnostic tests for Buruli ulcer—a disease that causes painful ulcers and swelling—are in development, including an instrument-free, pointof-care test and other tools for remote health care settings. Today, diagnosis is typically done by appearance, which leads to high rates of misdiagnosis
 and delayed treatment.
- Mosquitoes infected with Wolbachia, a bacteria that impact their reproduction and reduce population size, have been released to fight dengue and
 other mosquito-borne diseases in locations ranging from Indonesia to the Florida Keys, with early results showing promise. NIH and USAID have
 funded research into this approach.



The US government is advancing R&D to control and eliminate NTDs through a whole-of-government approach:

- National Institutes of Health (NIH) conducts R&D for new treatments, vaccines, diagnostics, vector control products, and other tools to combat
- Centers for Disease Control and Prevention (CDC) advances research to develop new and improved diagnostics and interventions to strengthen NTD control and elimination efforts.
- Department of Defense (DoD) undertakes R&D to create vaccines, drugs, diagnostics, and vector control products for NTDs that threaten US service members stationed abroad.
- US Agency for International Development (USAID) has in the past supported the development of new drugs and diagnostics for a select group
 of NTDs as part of its flagship NTD Program but largely focuses on deploying existing interventions.
- Food and Drug Administration administers the Tropical Disease Priority Review Voucher Program to incentivize investment in products for NTDs



US government investment in NTD R&D (in 2022) US\$ millions



advancing innovation to save lives

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