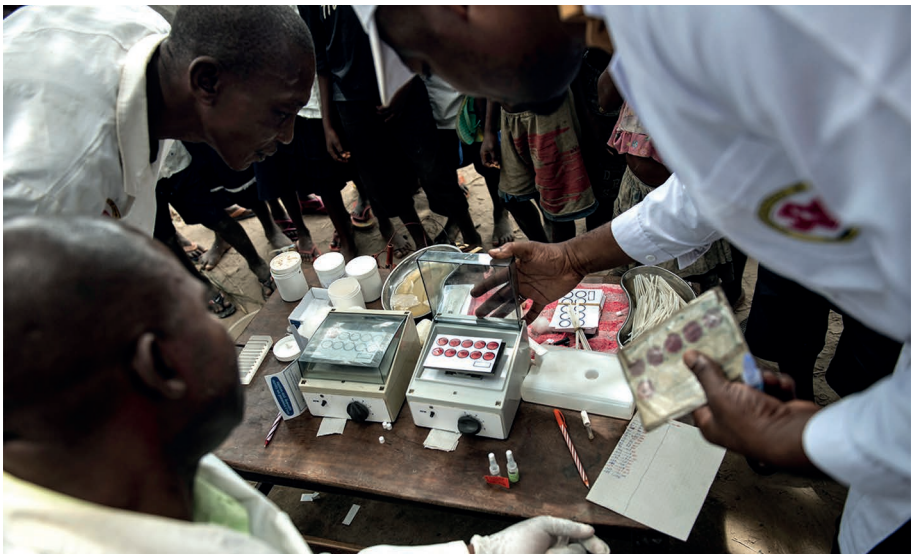
NEGLECTED DISEASES

# Pill treats sleeping sickness

*Scientists seek approval from regulators for this relatively quick and easy therapy.*

BY AMY MAXMEN



NEIL BRANDVOLD

Workers screen people for sleeping sickness in a remote village in the Democratic Republic of the Congo.

For the first time, researchers have cured the deadly neurological disease sleeping sickness using pills instead of a more complicated combination of intravenous infusions and pills. The investigators presented the results from final clinical trials on 17 October at the European Congress on Tropical Medicine and International Health in Antwerp, Belgium, providing hope that the treatment will help to eliminate the malady within a decade.

The oral therapy — called fexinidazole — cured 91% of people with severe sleeping sickness, compared with 98% who were treated with the combination therapy. It also cured 99% of people in an early stage of the disease who would typically have had a lumbar puncture, or spinal tap, to determine whether they needed infusions. The relative ease of the fexinidazole treatment means that, if approved, it might save more lives than the current option, say the investigators leading the phase 3 trial, the final step before the drug goes to regulators for approval.

Sleeping sickness is endemic to Africa and generally infects extremely poor people who live in remote regions. People often have the disease for years before seeking treatment.

"It's not just the person with sleeping sickness, it's the family that takes care of them during years of this neurological, very serious disease," says Philippe Büscher, a sleeping-sickness specialist at the Institute of Tropical Medicine in Antwerp. "Whatever money they have, they'll spend on this instead of anything else."

Büscher commends the team for conducting a quality clinical trial under extraordinary circumstances in the countries hit hardest by the disease, the Democratic Republic of the Congo and the Central African Republic. Investigators had to carry equipment to remote clinics over rugged terrain; one study site was repeatedly robbed; and early in the trial, some participants fled armed conflict.

Sleeping sickness — also known as human African trypanosomiasis — is spread through the bite of tsetse flies carrying parasites, most commonly *Trypanosoma brucei gambiense*. The organism infects the central nervous system, and patients can experience confusion, daytime sleepiness, night-time insomnia and psychiatric symptoms such as manic episodes. If left untreated, they enter a coma and die. For decades, the only treatment was an arsenic-based drug that killed 1 in 20 patients.

In 2009, researchers introduced a safer option: nifurtimox–eflornithine combination therapy, or NECT, which consists of pills and 14 intravenous infusions. For the first time in 50 years, the incidence of sleeping sickness slipped below 10,000 new cases per year; it's currently around 2,200, according to the World Health Organization. But the need for infusions, and the lumbar puncture required to qualify someone for the treatment, still present obstacles in regions where sterile equipment, electricity and doctors are in short supply.

The group that developed NECT — a non-profit research organization based in Geneva, Switzerland, called the Drugs for Neglected Diseases initiative (DNDi) — was still searching for a better therapy. In 2007, it had discovered fexinidazole, a compound shelved by Paris-based pharmaceutical company Sanofi. With the firm's agreement, the DNDi took the drug through clinical trials. It estimates that developing the therapy through to approval will cost about US$50 million — a fraction of what companies often spend on new drugs.

Sanofi will soon submit an application for drug approval through the European Medicines Agency, whose sign-off could pave the way for regulators in the Democratic Republic of the Congo. The drug might get a green light by the end of next year, says Nathalie Strub Wourgraft, the DNDi's medical director.

DNDi researchers and their colleagues are currently working on another oral treatment that they hope will cure the disease more quickly and reliably. ■


MORE ONLINE

STEPHEN LAM/REUTERS

TOP NEWS

Quantum machine goes in search of the Higgs boson go.nature.com/2h3xsa9

MORE NEWS

- The science of puppy dog eyes go.nature.com/2iyo1kz
- Shrew skulls shrink for winter survival go.nature.com/2yfigop
- DeepMind's self-taught AI is best yet at strategy game Go go.nature.com/2y0hho5

NATURE PODCAST

Undead cells; the strain of PhDs; and the traces of Antarctic instability nature.com/nature/podcast