University of Basel

African Contributions to Global Health

Video Transcript

Drug development for Neglected Tropical Diseases

Let's take a look at one of the most successful drug development stories in the field of Neglected Tropical Diseases: a treatment for Human African Trypanosomiasis, or sleeping sickness. Neglected Tropical Diseases are a set of 20 diseases that affect 1 out of 6 people worldwide. Neglected Tropical Diseases mostly affect populations in the less developed world who live in difficult-to-reach areas. Their reduced access to basic healthcare makes them vulnerable. Interest in financial investments in this field is rare because market opportunities and direct financial returns on investment are not huge. Despite all these limitations, drug and vaccine development for Neglected Tropical Diseases have to be conducted in accordance with tight clinical regulations and good clinical practices. These conditions make it challenging to address poverty-related diseases and Neglected Tropical Disease control and elimination.

Human African Trypanosomiasis, also known as sleeping sickness, is one of the Neglected Tropical Diseases which is fatal if not treated. Currently, a new era for its control and elimination is dawning. Human African Trypanosomiasis, caused by the parasite Trypanosoma brucei, is transmitted by Tsetse flies. The disease mainly affects sub-Saharan Africa, with 4 out of 5 cases in the Democratic Republic of the Congo. The treatments used in past decades were complex, risky and invasive for patients, and they required close monitoring in hospitals.

However, a novel oral lifesaving treatment against Human African Trypanosomiasis called Fexinidazole has come onto the market. It all started with the discovery of a chemical compound in the 1970s, but the drug development process was soon stopped for commercial reasons. Three decades later, the 'Drugs for Neglected Diseases initiative' was launched. On behalf of the DNDi, the Swiss Tropical and Public Health Institute conducted a search through hundreds of compounds, which finally led to the selection of Fexinidazole in 2005. The DNDi resumed the development of this compound abandoned in the 1970s. Four years later, the DNDi signed an agreement with Sanofi, a pharmaceutical company, to further develop and register the drug. The DNDi accomplished this effort for clinical development in less than 10 years. In 2018, this led to a positive response from the European Medicine Agency and the drug registration in the most affected country, the Democratic Republic of the Congo. While this successful development has been concluded, a new single-dose treatment against human African Trypanosomiasis has been discovered and developed in parallel, and it is now in the latest clinical development phases.

Such an accelerated development would have been impossible without the commitment of the National Human African Trypanosomiasis control programme in the Congo. This programme contributed to massive screening activities, which set the basis for clinical trials. In addition to finding the patients for the trials, this commitment led to a decline of cases from 2002 onwards, allowing the WHO to declare elimination of the disease by 2030 as a target. The decline corresponds to the development of Fexinidazole, its predecessor Nifurtimox-eflornithine combination therapy, and Acoziborole, the newest compound in this line.

Although clinical trials were not the main mandate of the programme, they contributed to the successful establishment of trial sites in very remote areas in the Democratic Republic of the Congo. The Phase II and III clinical trials were conducted in these sites.

The National Human African Trypanosomiasis Control Programme led the trials, with strong collaboration from institutions, the pharma industry and the World Health Organization, under the umbrella of the DNDi, a product development partnership.

Product Development Partnerships are small think tanks that strive to develop affordable vaccines and drugs. They address unmet needs while ensuring access equity to these products. For instance, the total development cost of the successful drug Fexinidazole was less than 200 million US dollars. This is a fraction of conventional industrial drug development costs, reported to be 2.6 billion dollars. Stakeholder engagement with local public institutions' commitment and the leaner drug development procedures have played a role in this cost reduction. In the Global North, the increasing drug costs have now led to a single treatment in oncology costing up to 350'000 US Dollars, which poses an increasing threat to the health system. Product Development Partnerships should be considered as one way forward in reducing this burden on the health system.

The case of Fexinidazole to treat Human African Trypanosomiasis is a great example of the power of collaboration between Product Development Partnerships, researchers, international organisations, national health programmes and pharmaceutical companies. Product Development Partnerships can therefore not only find successful treatments for Neglected Tropical Diseases; they could also improve the overall process of drug development. This would apply to both African contexts as well as high-income countries in the Global North.