

Developing a safe and efficacious drug to treat Chagas disease

Introduction

Of all the neglected diseases, Chagas disease is the one that receives the least investment for R&D. It is caused by the *Trypanosoma cruzi* parasite, transmitted by the bites of insects known as “kissing bugs”. A silent killer, it affects millions across the globe and takes approximately 12,000 lives every year. The disease occurs in two phases: the first phase is often asymptomatic or unrecognized due to non-specific symptoms. The second phase is a chronic one that can affect the heart and the gastrointestinal tract and, if left untreated, can lead to death.

Materials and Methods

Existing drugs, benznidazole and nifurtimox, have been used for decades, but because their efficacy against the chronic phase of the infection is poorly documented, they are of limited use in disease control strategies. In addition, long treatment periods (60-90 days) make patient compliance challenging, with increased risk of drug resistance development. In addition, until recently, there was no adapted paediatric formulation for either of the existing drugs.

Results

As part of a short term strategy to address urgent patient needs, DNDi and its partners have recently developed a paediatric formulation of benznidazole, manufactured in Brazil by LAFEPE. In the long term, new treatments that are safe, efficacious, and effective against the chronic phase of the disease – which is when most patients are diagnosed – are sorely needed to effectively fight the disease. In addition, a better understanding of biomarkers is essential to gain understanding of the disease progression and ease the development of test-of-cure diagnosis tools that support drug development.

Main conclusions

The session will explore the current state of the Chagas disease portfolio and give an overview of what is needed to provide patients with a safe, efficacious, easy-to-use and affordable treatment, with a focus on early drug discovery and assessment of treatment efficacy.