

Progress reported in global fight against diarrheal disease cryptosporidiosis

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Infectious disease scientists from Novartis, the University of Georgia and Washington State University have reported the discovery and early validation of a drug candidate for treating cryptosporidiosis, a diarrheal disease which is a major cause of child mortality in lower-income countries. Currently there are no vaccines or effective treatments.

"There's a lot of uncertainty when embarking on drug discovery for a notoriously intractable parasite such as *Cryptosporidium*, the cause of cryptosporidiosis," said Thierry Diagana, Head of the Novartis Institute for Tropical Diseases (NITD). "Thanks to the commitment of our funding collaborators and urgent action of our academic colleagues, we've made an important step toward advancing a new treatment."

Diarrheal diseases cause more than 800,000 deaths annually[1]. Epidemiological studies have highlighted the vital need for new treatment options against the protozoan parasite *Cryptosporidium*, which often infects its victims from exposure to contaminated water supplies. Nitazoxanide, the only approved <u>treatment</u> for cryptosporidiosis, has shown poor results in vulnerable infants and immune-compromised patients[2,3].

Yet there are obstacles to finding new treatments. The parasite perishes relatively quickly in labs and scientists have lacked research tools to identify drug candidates. The team developed a novel drug discovery process using transgenic parasites and novel <u>disease</u> models, leading to the identification and validation of the *Cryptosporidium* PI(4)K inhibitor



candidate KDU731. They reported the discovery and preclinical findings in a recent issue of *Nature*.

More information: A Cryptosporidium PI(4)K inhibitor is a drug candidate for cryptosporidiosis, *Nature* (2017). nature.com/articles/doi:10.1038/nature22337

Other references:

- 1. Liu, L. et al. Global, regional, and national causes of under-5 mortality in 2000-15: an updated systematic analysis with implications for the Sustainable Development Goals. *The Lancet*. 2016.
- 2. Amadi, B. et al. High dose prolonged treatment with nitazoxanide is not effective for cryptosporidiosis in HIV positive Zambian children: a randomised controlled trial. BMC infectious diseases. 2009.
- 3. Amadi, B. et al. Effect of nitazoxanide on morbidity and mortality in Zambian children with cryptosporidiosis: a randomised controlled trial. *The Lancet*. 2002.

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