

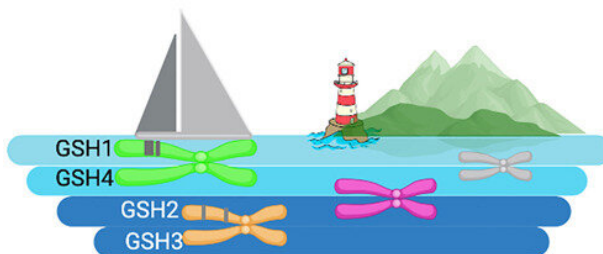
# Researchers develop model to study neglected tropical diseases

July 24 2023

## Schistosome genomic safe harbor

Genome safe harbor targeted for **insertion of transgene using CRISPR/Cas to derive transgenic schistosome**

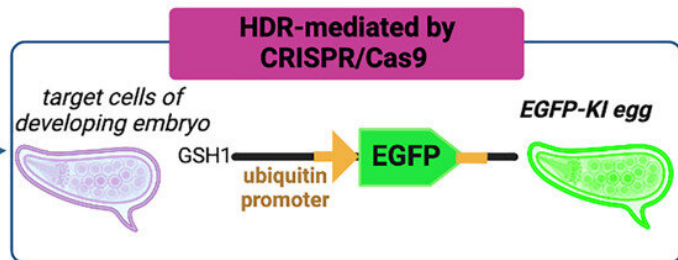
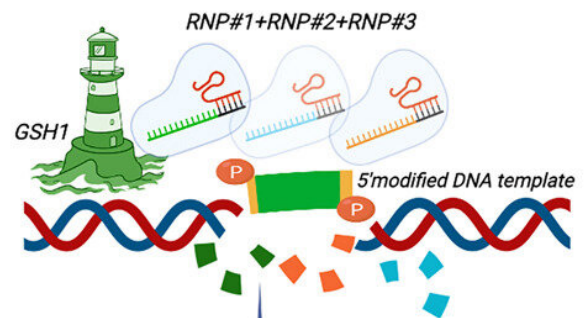
- ensure transgene expression in all parasite stages
- unique regions to avoid CRISPR off target
- located outside annotated genes



## CRISPR/Cas9

Multiple, overlapping CRISPR targets to enhance **integration of reporter transgene**

- precise integration
- highly efficient HDR
- enhanced stability of DNA donor



Credit: *Cell Reports Methods* (2023). DOI: 10.1016/j.crmeth.2023.100535

Neglected tropical diseases affect more than 1 billion people—one-sixth of the world's population. Despite their devastating health consequences, these diseases, which occur in some of the world's poorest and most isolated communities, often receive little attention from global funding agencies and pharmaceutical companies. They can also be difficult for researchers to study, given that many neglected tropical diseases are caused by parasites with large, complex genomes.

Now, in a bid to make it easier to study these neglected diseases, researchers at the George Washington University, in collaboration with colleagues in France and Germany, have developed a model organism from a genetically modified parasitic worm. The team identified four so-called genomic safe harbor sites in the chromosome of the [parasitic worm](#), *Schistosoma mansoni*. These "Goldilocks" sites are places where genes or [genetic elements](#) can be safely inserted without damaging the organism.

The article was published in the journal *Cell Reports Methods*.

Using CRISPR technology, Wannaporn Ittiprasert Tanno and Paul J. Brindley, research professors of Microbiology, Immunology, and Tropical Medicine at the George Washington University School of Medicine and Health Sciences, successfully inserted a [transgene](#) into a genome safe harbor site, that when expressed in the worm, fluoresces as bright green.

The researchers say their method creates a model that could be used for testing additional gene therapies on helminth parasites with the ultimate goal of developing new drugs and vaccines to treat and prevent

schistosomiasis and other similar diseases.

**More information:** Wannaporn Ittiprasert et al, Targeted insertion and reporter transgene activity at a gene safe harbor of the human blood fluke, *Schistosoma mansoni*, *Cell Reports Methods* (2023). [DOI: 10.1016/j.crmeth.2023.100535](https://doi.org/10.1016/j.crmeth.2023.100535)

Provided by George Washington University

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