CENTER FOR GLOBAL HEALTH

Otherwise Unavailable Non-Malarial Parasitic Disease Treatment Drugs in the United States: an Update from CDC Parasitic Diseases Drug Service

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BACKGROUND

- The Centers for Disease Control and Prevention's (CDC) support to clinicians includes providing access to certain drugs that are otherwise unavailable or have limited market availability in the U.S.
- These drugs are provided by CDC under expanded access Investigational New Drug (IND) applications, authorized by the FDA, to treating physicians.
- The drugs are used to treat U.S. patients with parasitic diseases, including travelers, immigrants, and refugees from endemic countries.

METHODS

- We reviewed CDC's records of drug releases and physician adverse event reporting forms over the last 10 years to describe the number of patients treated each year and document trends over time.

RESULTS

- From 2010–2019, the annual number of patients treated ranged from 21–118 (median 99).
- Benznidazole, a treatment for Chagas disease, was the most common drug released for patients between 2012–2018, when CDC held the IND. The median number of patients treated per year was 50, ranging from 41–63% of patients treated annually.
- Requests for treatment for human African trypanosomiasis are rare. In 2019, CDC released eflornithine and nifurtimox for one patient with gambiense human African trypanosomiasis who met criteria for treatment according to the 2019 WHO recommendations.[1]
- Sodium stibogluconate (Pentostam®[2]), a treatment of certain presentations of cutaneous, visceral, and mucocutaneous leishmaniasis, accounted for 6–22% of annual releases over the last 10 years.

[1]WHO interim guidelines for treatment of gambiense HAT. Geneva: 2019

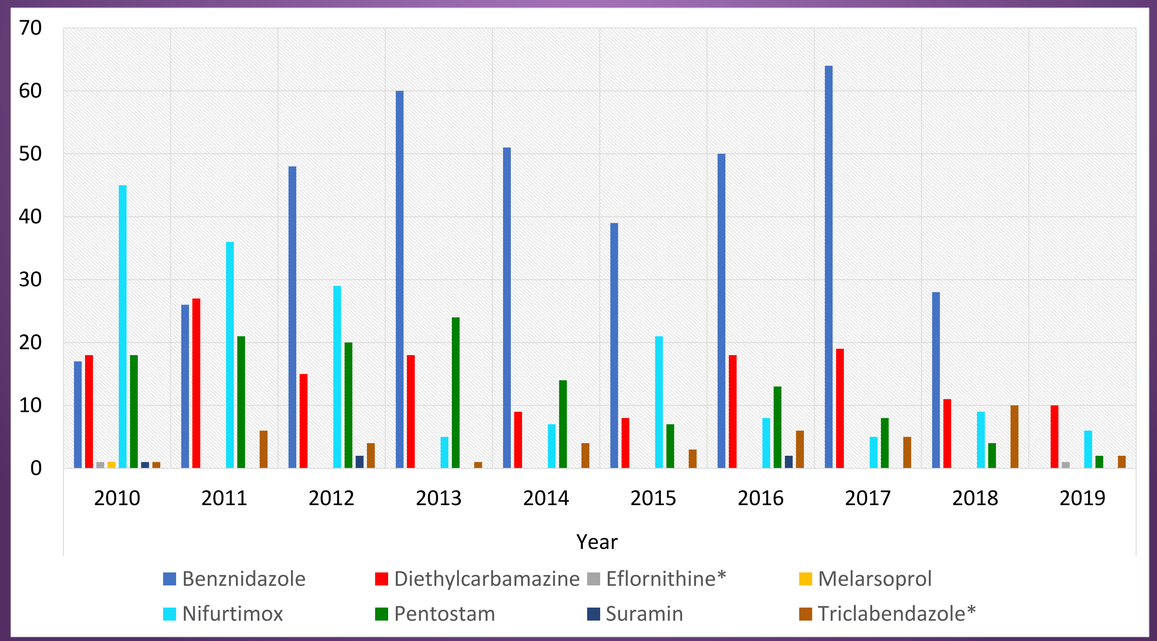
[2]Pentostam® is made by GlaxoSmithKline

DRUGS AVAILABLE FROM CDC PARASITIC DISEASES DRUG SERVICE

DRUG	SUPPLIER	MANUFACTURER
DIETHYLCARBAMAZINE (DEC)	WHO	EISAI*
EFLORNITHINE (DFMO) - ORNIDYL®	WHO	SANOFI AVENTIS
MELARSOPROL - ARSOBAL®	WHO	SANOFI AVENTIS
NIFURTIMOX - LAMPIT®	WHO	BAYER
SODIUM STIBOGLUCONATE - PENTOSTAM®	GSK	GLAXOSMITHSKLINE
SURAMIN - GERMANIN	WHO	BAYER

*manufacturer of this generic drug may vary based on WHO procurement

PATIENTS TREATED PER YEAR BY DRUG



*regard number of releases as proxy for number of patients treated; CDC does not hold the IND for these drugs, so no IND reports are available

COMMERCIALLY AVAILABLE DRUGS

- Benznidazole
- Miltefosine
- Triclabendazole

ANTICIPATED COMMERCIALLY AVAILABLE DRUGS

- Nifurtimox

CHANGES TO AVAILABLE DRUGS

- Pentostam – Intralesional Use
- Nifurtimox – Eflornithine Combination Therapy (NECT)

CONCLUSION

- Changes to release frequency of benznidazole, nifurtimox, sodium stibogluconate and triclabendazole were likely due to FDA approval and commercial availability of previously investigational drugs.
- In February 2019, triclabendazole was FDA approved for treatment of *Fasciola* infection in persons ≥6 years old and became commercially available in May 2019.
- In 2017, benznidazole was FDA approved for treatment of Chagas disease in children 2–12 years old, and in May 2018, CDC discontinued release of benznidazole under IND once the drug was commercially available.
- Miltefosine was approved by FDA in March 2014 for treatment of certain leishmaniasis infections.
- When necessary to maintain availability, CDC has successfully pursued extension of drug expiration dates with manufacturers, FDA, and other partners to ensure continued supply of treatment options.
- CDC's Parasitic Diseases Branch can be reached by telephone at 404-718-4745 or by email at parasites@cdc.gov.

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