Moxidectin – New orphan drug approval

- On June 13, 2018, Medicines Development for Global Health (MDGH) and World Health Organization Special Programme for Research and Training in Tropical Diseases announced the FDA approval of moxidectin, for the treatment of onchocerciasis due to *Onchocerca volvulus* in patients aged 12 years and older.

    — Moxidectin does not kill adult *O. volvulus* parasites. Follow-up is advised.
    — The safety and efficacy of repeat administration of moxidectin in patients with *O. volvulus* has not been studied.

- River blindness is caused by the parasitic worm *O. volvulus*, which is transmitted from person to person by black flies that breed in fast flowing rivers in sub-Saharan Africa, Yemen and small foci in South and Central America. The millions of larvae (microfilariae) released by the infecting adult parasites invade skin and eyes where they can cause severe manifestations, including permanent blindness, itching and disfiguring skin conditions.

    — Nearly 200 million people are at risk for river blindness, and more than 99% of people infected live in sub-Saharan Africa.

- Moxidectin is a macrocyclic lactone anthelmintic medicine that selectively binds to the parasite's glutamate-gated chloride ion channels. These channels are vital to the function of invertebrate nerve and muscle cells. Moxidectin has activity against *O. volvulus* microfilariae but does not kill adult *O. volvulus* parasites.

- The efficacy and safety of moxidectin in the treatment of onchocerciasis were based on two active-controlled studies. The first study enrolled nearly 1,500 patients to moxidectin or ivermectin. The second study was a dose-ranging study that enrolled 172 patients to moxidectin or ivermectin.

    — Each study met its respective primary endpoints, showing the statistically significant superiority of moxidectin over the current standard of care, ivermectin, in suppressing the presence of the microfilariae in skin.

- Warnings and precautions of moxidectin include cutaneous, ophthalmological and/or systemic adverse reactions; symptomatic orthostatic hypotension; encephalopathy in *Loa loa* co-infected patients; and edema and worsening of onchodermatitis.

- The most common adverse reactions (> 10%) with moxidectin use were eosinophilia, pruritus, musculoskeletal pain, headache, lymphopenia, tachycardia, rash, abdominal pain, hypotension, pyrexia, leukocytosis, influenza-like illness, neutropenia, cough, lymph node pain, dizziness, diarrhea, hyponatremia and peripheral swelling.

- The recommended dosage of moxidectin is 8 mg (four 2 mg tablets) as a single oral dose, with or without food.

- Due to the low number of cases of river blindness in the U.S., moxidectin will only be available in specialists' clinics.

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- MDGH's launch plans for moxidectin are pending. Moxidectin will be available as a 2 mg tablet.