



BARDA INDUSTRY DAY

TPOXX® Project:

USG Collaboration Supports SIGA Smallpox Project
38th FDA Approval

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Project Overview



Candidate: TPOXX® is a novel oral therapeutic agent that inhibits ability of variola virus to spread

- Established virus-targeted mechanism
- Broad and consistent antiviral activity against orthopoxviruses
- Efficacy evaluated in two lethal animal models

- ▶ PBS Contract award: May 13, 2011
- ▶ First Entirely New Small Molecule Drug delivered to the DSNS under PBS
- ▶ Total BARDA cost-share: \$522M



Innovative USG Partnership



SIGA-BARDA Partnership



TPOXX® is FDA Approved

INDICATIONS AND USAGE

TPOXX is an inhibitor of the orthopoxvirus VP37 envelope wrapping protein and is indicated for the treatment of human smallpox disease in adults and pediatric patients weighing at least 13 kg. (1.1)

Limitations of Use:

The effectiveness of TPOXX for treatment of smallpox disease has not been determined in humans because adequate and well-controlled field trials have not been feasible, and inducing smallpox disease in humans to study the drug's efficacy is not ethical. (1.2)

TPOXX efficacy may be reduced in immunocompromised patients based on studies demonstrating reduced efficacy in immunocompromised animal models. (1.2)

- ▶ FDA approved TPOXX® for the treatment of symptomatic smallpox disease on July 13, 2018
- ▶ Approval using Segregate Orthopoxviruses under the FDA Animal Rule
- ▶ BARDA's 38th FDA Approval



"To address the risk of bioterrorism, Congress has taken steps to enable the development and approval of countermeasures to thwart pathogens that could be employed as weapons. Today's approval provides an important milestone in these efforts. This new treatment affords us an additional option should smallpox ever be used as a bioweapon."

– Scott Gottlieb, M.D., FDA Commissioner

TPOXX[®]: An Ideal Smallpox Therapeutic

EASY TO:



STORE

Small molecule with long shelf life



TRANSPORT

Stable without the need for refrigeration



ADMINISTER

Oral and (IV formulations under development)

TPOXX is one of the first new molecular entity drugs delivered to the Strategic National Stockpile under Project Bioshield