



Pre-License Inspection Waiver Memorandum

From: Wei Wang, Ph.D. OCBQ/DMPQ/MRB1, CMC/Facility Reviewer

To: Administrative File: STN 125694/0

Subject: Recommendation to waive pre-license inspections of drug product release testing facilities

Sponsor: AveXis, Inc. (AveXis), (US License No. 2104); BL 125694.

Product: AVXS-101 (onasemnogene abeparvovec, ZOLGENSMA) is a gene therapy biological product, consisting of a non-replicating recombinant adeno-associated viral vector serotype 9 (AAV9) containing the human Survival Motor Neuron (SMN) gene under the control of the cytomegalovirus (CMV) enhancer/chicken- β -actin-hybrid promoter (CB). AVXS-101 is to be administered as a single dose intravenous (IV) infusion. AVXS-101 is indicated for the treatment of pediatric patients with spinal muscular atrophy (SMA) (b) (4)

Due Date: June 01, 2019

The following information provides justification to support the waiver recommendations:

1) Inspection history

Location	Activity	Most Recent Inspection
(b) (4)	(b) (4) Production Release testing Stability testing Drug Substance In-process testing Release testing Drug Product Release testing Stability testing	(b) (4) Surveillance Team Biologics Voluntary Action Indicated (VAI)
(b) (4)	Drug Substance In-process testing Release testing Stability testing Drug Product Release testing Stability testing	(b) (4) Surveillance ORA and CDER Voluntary Action Indicated (VAI)

Location	Activity	Most Recent Inspection
(b) (4)	Drug Substance Release testing Drug Product Release testing	(b) (4) Surveillance ORA Voluntary Action Indicated (VAI)
(b) (4)	Drug Substance Release testing Stability testing Drug Product Release testing Stability testing	(b) (4) Surveillance ORA Voluntary Action Indicated (VAI)

- 2) CBER waived the pre-license inspections of facilities listed in table above based on the FDA (ORA and Team Biologics) surveillance inspections of the facilities involved with drug product release tests of AVXS-101. All issues on the 483 were resolved and the inspections were all classified as voluntary action indicated (VAI).

Recommendation: Waive PLI of the product release testing facilities described in table above.

Concurrence signatures:

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