

Meeting Minutes

Applicant: Avexis, Inc

Product: AVXS-101-onasemnogene abeparvovec

STN: 125694/0

Meeting Date/Time/Place: 15th November 2018/9:00 AM /WO - Bldg 71- 6100

Invitees: Andrew Byrnes, Denise Gavin, Angela Whatley, Candace Jarvis, Hyesuk Kong, James Kenney, Suzanne Carter, Maryna Eichelberger, Varsha Garnepudi

Attendees: Andrew Byrnes, Denise Gavin, Angela Whatley, Hyesuk Kong, Maryna Eichelberger, Varsha Garnepudi

Summary of Product and Proposed Indication

AVXS-101-onasemnogene abeparvovec; vector-based gene replacement therapy for Indication treatment of pediatric patients with spinal muscular atrophy (SMA) (b) (4)

Purpose/Goals:

x Clarification of DBSQC's review responsibilities x
In-Support Testing requirements x Post- licensure
testing requirements

General understanding

DBSQC should review the lot release tests and method validations only for lot release tests of drug substances and drug product to be agreed upon with the product reviewer(s)

1. Review the test methods to ensure that they are detailed enough to carry out the same tests in our lab following the sponsor's method.
2. Appropriate system suitability criteria and/or assay validity criteria are included in the procedure.
3. Review assay validation reports to ensure that the methods are appropriately validated for its intended use.

NOTE: We will not review anything regarding manufacturing, product characterization, stability studies, labeling etc.

DBSQC Review Assignments

DBSQC Reviewers assigned to 125694 are: Hyesuk Kong and Varsha Garnepudi.

Review Items and In-Support Testing from the Specification Sheets for (b) (4)**Drug Product:**

Tests from specification sheets for

1. (b) (4)
2. Drug Product

Test	(b) (4) /DP	Reviewer (DBSQC/DCGT)	In Support Testing (DP)
General:			
Appearance per (b) (4) and (b) (4)	(b) (4) /DP	DCGT	
pH per (b) (4)	(b) (4) /DP	DCGT	
Osmolality per (b) (4)	(b) (4) /DP	DCGT	
(b) (4)	DP	DCGT	
Quantity:			
(b) (4)	(b) (4)	DCGT	
(b) (4)	DP	DCGT	
Total Protein by (b) (4)	DP	DCGT	
(b) (4)	DP	DCGT	
Identity:			
(b) (4)	(b) (4) /DP	DCGT	
Identity (Protein) by (b) (4)	(b) (4) /DP	DCGT	
Identity (Protein) by (b) (4)	DP	DCGT	

Purity:			
% Total Purity by (b) (4)	(b) (4) /DP	DCGT	
% Total Impurities by (b) (4)	(b) (4) /DP	DCGT	
(b) (4)	DP	DCGT	
Impurities:			
Tests	(b) (4) /DP	Reviewer (DBSQC/DCGT)	In Support Testing (DP)
(b) (4)	(b) (4)	DCGT	
(b) (4)	(b) (4)	DCGT	
(b) (4)	(b) (4)	DCGT	
(b) (4)	(b) (4)	DCGT	
(b) (4)	(b) (4)	DCGT	
(b) (4)	(b) (4)	DCGT	
Potency:			
(b) (4)	DP	DCGT	
In (b) (4) Relative Potency by (b) (4)	DP	DCGT	
Safety:			
Bioburden per (b) (4)	(b) (4)	DBSQC	
(b) (4)	(b) (4)	DCGT	
Sterility per (b) (4)	DP	DBSQC	

Endotoxin per (b) (4)	DP	DBSQC	
Mycoplasma (b) (4)	(b) (4)	DBSQC	
Container Closure Integrity (b) (4) [REDACTED]	DP	DCGT	

Discussion

1. Specific discussion regarding review of tests.

DCGT/OTAT will perform the review of method validations for analytical assays.

DCGT/OTAT would like to DBSQC to review the validation of microbiological assays such as bioburden, mycoplasma, endotoxin, and sterility- See above table

2. In-support testing of drug product

DCGT does not see the need for in-support testing or lot release testing. Most of the release testing for product quality and purity is specific to AAV vector class, and some involve complex assay methodology (b) (4). DBSQC nor DCGT has the instrumentation to perform the (b) (4) assay.

3. Samples for in-support testing N/A

4. Post Licensure mode of release-

Lot release by protocol review only, Varsha will work with DCGT for the review of the Testing Plan.

Varsha R. 

Garnepudi -S 0.9.2342.19200300.100.1.1-0012274938cm=Va sha R. Ga nepud -S Date 2019 05 20 12 27 57 -04'00' ,