

RECORD OF TELEPHONE CONVERSATION

Submission ID:

BLA 125694

Office:

OTAT

Product AVXS-101-onasemnogene abeparvovec;

Sponsor: AveXis, Inc.

Telecon Date/Time: 24- Jan-2019 10:00 AM

Initiated by FDA?:

Yes

Telephone Number: () -

Author:

Angela Whatley

Purpose: To discuss the manufacturing process parameter classification and operating ranges are not adequately justified, and the studies provided did not vary the process or operating parameters sufficiently to justify the operating ranges.

FDA Participants:

Candace Jarvis

Andrew Byrnes

Angela Whatley

Denise Gavin

Wei Wang

Deborah Trout

Henry M. Burnell

Sponsor Participants:

James L'Italien, Ph.D., Chief Regulatory Officer, SVP, Regulatory Affairs;

Mark Roache, SVP, Quality Assurance;

Andrew Stober, SVP, Manufacturing and Supply Chain;

Robert Hodge, VP, Technical Services;

Phil Wagner

Robert Baker

Summary of Discussion:

During the T-con FDA inquired about the process validation and how AveXis determined the normal operating ranges for each parameter. FDA acknowledged the definitions of process parameters were provided and the normal operating ranges were listed but the studies and data to support the NOR were missing from the BLA. FDA asked AveXis how they determined the NOR and what data they have to support the ranges.

Additionally, FDA noted that based on the available information provided some of the supporting data needed to support the NORs may be in the small-scale studies conducted at (b) (4).

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FDA requested that the sponsor submit data to support the normal operating ranges for each parameter. FDA provided the example of the volume of (b) (4) used in the (b) (4) step. FDA stated that in 3.2.s.2.2 the critical process parameter is (b) (4) volume and the NOR is listed as (b) (4) but in other documents such as the PRO-385 the process parameter is (b) (4) concentration and the NOR is listed as (b) (4). FDA requested that the sponsor clarify the process parameter for (b) (4) and be consistent throughout the BLA. FDA also stated that supporting data for how this normal operating range was determined could not be located. FDA stated the (b) (4) is one example and the FDA asked that the sponsor provide justifications for the normal operating ranges for all critical and key process parameters.

FDA requested that the sponsor clearly link each normal operating range to the supporting data which provides the justification for each normal operating range for the all critical and key process parameters. FDA stated that this deficiency will be listed as a major deficiency in the mid-cycle communications and will be discussed at the upcoming mid-cycle communications meeting on Tuesday January 29, 2019, and FDA requested that the sponsor provide an estimate when the requested data can be provided during this meeting.

FDA also discussed inspection related questions and informed the sponsor that there will be 5 inspectors from FDA.

The call ended cordially