

RECORD OF TELEPHONE CONVERSATION

**Submission ID:**

BLA 125694

**Office:**

OTAT

**Product** AVXS-101-onasemnogene abeparvovec;

**Sponsor:** AveXis, Inc.

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**Telecon Date/Time:** 17- Jan-2019 10:00 AM

**Initiated by FDA?:**

Yes

**Telephone Number:** ( ) -

**Author:**

Andrew Byrnes

**Purpose:** To discuss weight restrictions on dosing of onasemnogene abeparvovec.

**FDA Participants:**

CANDACE JARVIS

ANDREW BYRNES

MIKE SINGER

LEI XU

**Sponsor Participants:**

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

Olga Santiago, MD, Chief Medical Officer, SVP

Doug Feltner, MD, VP, Head of Clinical Development

**Summary of Discussion:**

CBER requested a teleconference to discuss weight restrictions on dosing of onasemnogene abeparvovec.

CBER stated that the label should not restrict the product to patients within a certain weight range, and the dose should simply be expressed as  $1.1 \times 10^{14}$  vg/kg. The applicant agreed.

The applicant stated that they would check whether there would be any impact to logistics (shipping, secondary packaging, etc.). The applicant stated that they didn't think there would be any impact, but that they would update CBER later.

The applicant also stated that they had submitted the clinical protocol synopsis of the expanded access treatment protocol to IND 15699 and would like FDA's feedback. In the meantime, they have informed potential sponsors of single-patient expanded access INDs that these INDs should not be submitted as emergency requests.

The call ended cordially