

CY2019 CBER Breakthrough Therapy Approvals

Data as of June 30, 2019

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
BLA 125694	ORIGINAL-1	ZOLGENSMA	Onasemnogene abeparvovec	AveXis, Inc.	24-May-2019	Indication-treatment of pediatric patients with spinal muscular atrophy (SMA) Type 1 with or without disease onset.

* Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012.