

References

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1. Mercuri E, Darras BT, Chriboga CA, et al. Nusinersen versus sham control in later-onset spinal muscular atrophy. *N Engl J Med*. 2018;378:625-35.
2. De Vivo DC, Topaloglu H, Swoboda K et al. Nusinersen in infants who initiate treatment in a presymptomatic stage of spinal muscular atrophy (SMA): Interim efficacy and safety results from the phase 2 NURTURE study. 2019; 92(15).
3. Hayes, Inc. Hayes Prognosis Overview. Zolgensma (Onasemnogene Abeparvovec) for Spinal Muscular Atrophy. Landsdale, PA: Hayes, Inc.; February 2019.
4. Mendell J, Al-Zaidy S, Shell R, [al](#). Single-dose gene-replacement therapy for spinal muscular atrophy. *N Engl J Med*. 2017;377:1713-1722.
5. Al-Zaidy S, Pickard A, Kotha K, et. al. Health outcomes in spinal muscular atrophy type 1 following AVXS-101 gene replacement therapy. *Pediatr Pulmonol*. 2019;54(2):179-185.
6. Al-Zaidy S, Kolb SJ, Lowes L, et al. AVXS-101 (onasemnogene abeparvovec) for SMA1: comparative study with a prospective natural history cohort. *J Neuromuscul Dis*. 2019a;6(3):307-317.
7. Al-Zaidy S, Mendell JR. From clinical trials to clinical practice: practical considerations for gene replacement therapy in SMA type 1. *Pediatr Neurol*. 2019;100:3-11.
8. Dabbous O, Maru B, Jansen JP, et al. Survival, motor function, and motor milestones: comparison of AVXS-101 relative to nusinersen for the treatment of infants with spinal muscular atrophy type 1. *Adv Ther*. 2019;36(5):1164-1176.
9. Lowes LP, Alfano LN, Arnold WD, et al. Impact of age and motor function in a phase 1/2a study of infants with SMA type 1 receiving single-dose gene replacement therapy. *Pediatr Neurol*. 2019;98:39-45.
10. Glascok J, Sampson J, Connolly A, et al. Revised recommendations for the treatment of infants diagnosed with spinal muscular atrophy via newborn screening who have 4 copies of SMN2. *J Neuromuscul Dis*. 2020;7(2):97-100.
11. Kirschner J, Butoianu N, Goemans N, et al. European ad-hoc consensus statement on gene replacement therapy for spinal muscular atrophy. *Eur J Paediatr Neurol*. 2020;28:38-43.
12. Mendell J, Al-Zaidy S, Shell R, et al. Five-year extension results of the phase 1 START trial of onasemnogene abeparvovec in spinal muscular atrophy. *JAMA Neurol*. 2021;78(7):834-841.
13. Day J, Finkel R, Chiriboga C, et al. Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy in patients with two

copies of SMN2 (STR1VE): An open-label, single-arm, multicentre, phase 3 trial. *Lancet Neurol.* 2021;20:284-293

14. Spinraza™ (Nusinersen) injection, for intrathecal use [package insert]. Cambridge, MA: Biogen Inc. Revised 06/2020.
15. Zolgensma® (onasemnogene abeparvovec-xioi) injection, for intravenous use [package insert]. AveXis, Inc. Bannockburn, IL. Revised 10/2021.
16. Clinical Pharmacology Compendium®. 2023. Tampa FL: Gold Standard, Inc. Nusinersen.
17. Clinical Pharmacology™ 2023. Tampa FL: Gold Standard, Inc. Onasemnogene abeparvovec.
18. Micromedex DrugDex Compendium®. 2023.
19. Micromedex DrugDex Compendium®. 2023. Onasemnogene abeparvovec.
20. Nusinersen Sodium In: AHFS Drug Information Online Electronic Medical Library. Bethesda, MD: American Society of Health-System Pharmacists. Revised January 29, 2018.