

Featured Rare Disease Publications

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Modeling Publications

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An updated cost-utility model for onasemnogene abeparvovec (Zolgensma®) in spinal muscular atrophy type 1 patients and comparison with evaluation by the Institute for Clinical and Effectiveness Review (ICER)



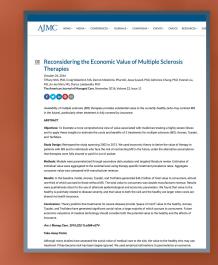
Incremental net monetary benefit of ocrelizumab relative to subcutaneous interferon b-1a.



Cost-effectiveness
analysis of using
onasemnogene
abeparvocec (AVXS-101)
in spinal muscular atrophy
type 1 patients.



Modeling the impact of patient treatment preference on health outcomes in relapsing-remitting multiple sclerosis.



Reconsidering the economic value of multiple sclerosis therapies.

JOURNAL OF MARKET ACCESS & HEALTH POLICY

2021

JME
Journal of Medical Economics

HEALTH POLICY JME James of Minister Boscomen

2020

AJMC°

2016

2017 2019

2

Evidence Synthesis Publications

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Survival, motor function, and motor milestones: Comparison of AVXS-101 relative to nusinersen for the treatment of infants with spinal muscular atrophy type 1



Crizanlizumab and comparators for adults with sickle cell disease: a systematic review and network meta-analysis.



Forced vital capacity and cross-domain late-onset
Pompe disease outcomes:
An individual patient-level data meta-analysis.



Ceftolozane/tazobactam
(Zerbaxa) for the treatment
of Pseudomonas aerginosa
(PSA) bacteremia: a
systematic literature review.

Advances in Therapy

2019 2020

BMJ Open

Journal of NEUROLOGY



2020

3

Database Publications

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Geographic variation in diagnostic ability and quality of care metrics: A case study of ankylosing spondylitis and low back pain.



Economic Burden of Bronchiolitis Obliterans Syndrome (BOS) Following Allogeneic Hematopoietic Stem Cell Transplant (alloHSCT) in Patients with Commercial Insurance in the United States.



Physical activity and health outcomes in severe hemophilia A patients on prophylaxis with conventional rFVIII vs rFVIIIFc.



Maternal and infant adverse outcomes associated with mild and severe preeclampsia during the first year after delivery in the United States.



2017





THANK YOU!

we look forward to staying in touch



a precision value & health team