Response of 33 UK patients with infantileâ€onset Pomp therapy

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Citation Report

#	Article	IF	Citations
1	From Cryptic Toward Canonical Pre-mRNA Splicing in Pompe Disease: a Pipeline for the Development of Antisense Oligonucleotides. Molecular Therapy - Nucleic Acids, 2016, 5, e361.	2.3	29
2	High dose IVIG successfully reduces high rhGAA IgG antibody titers in a CRIM-negative infantile Pompe disease patient. Molecular Genetics and Metabolism, 2017, 122, 76-79.	0.5	7
3	Causally treatable, hereditary neuropathies in Fabry's disease, transthyretin-related familial amyloidosis, and Pompe's disease. Acta Neurologica Scandinavica, 2017, 136, 558-569.	1.0	10
4	Rapidly Progressive White Matter Involvement in Early Childhood: The Expanding Phenotype of Infantile Onset Pompe?. JIMD Reports, 2017, 39, 55-62.	0.7	19
5	High Sustained Antibody Titers in Patients with Classic Infantile Pompe Disease Following Immunomodulation at Start of Enzyme Replacement Therapy. Journal of Pediatrics, 2018, 195, 236-243.e3.	0.9	27
6	Classic infantile Pompe patients approaching adulthood: a cohort study on consequences for the brain. Developmental Medicine and Child Neurology, 2018, 60, 579-586.	1.1	77
7	The phenotype, genotype, and outcome of infantile-onset Pompe disease in 18 Saudi patients. Molecular Genetics and Metabolism Reports, 2018, 15, 50-54.	0.4	9
8	Long term clinical history of an Italian cohort of infantile onset Pompe disease treated with enzyme replacement therapy. Orphanet Journal of Rare Diseases, 2018, 13, 32.	1.2	65
9	Pompe disease in Austria: clinical, genetic and epidemiological aspects. Journal of Neurology, 2018, 265, 159-164.	1.8	29
10	Preventing or Eradicating Factor VIII Antibody Formation in Patients with Hemophilia A: What Can We Learn from Other Disorders?. Seminars in Thrombosis and Hemostasis, 2018, 44, 531-543.	1.5	9
11	Cardiac outcome in classic infantile Pompe disease after 13†years of treatment with recombinant human acid alpha-glucosidase. International Journal of Cardiology, 2018, 269, 104-110.	0.8	32
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15	New pharmacotherapies for genetic neuromuscular disorders: opportunities and challenges. Expert Review of Clinical Pharmacology, 2019, 12, 757-770.	1.3	12
16	Effects of immunomodulation in classic infantile Pompe patients with high antibody titers. Orphanet Journal of Rare Diseases, 2019, 14, 71.	1.2	21
17	Respiratory complications of metabolic disease in the paediatric population: A review of presentation, diagnosis and therapeutic options. Paediatric Respiratory Reviews, 2019, 32, 55-65.	1.2	5
18	Strategies for the Induction of Immune Tolerance to Enzyme Replacement Therapy in Mucopolysaccharidosis Type I. Molecular Therapy - Methods and Clinical Development, 2019, 13, 321-333.	1.8	9

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19	Treating lysosomal storage disorders: What have we learnt?. Journal of Inherited Metabolic Disease, 2020, 43, 125-132.	1.7	20
20	Earlier and higher dosing of alglucosidase alfa improve outcomes in patients with infantile-onset Pompe disease: Evidence from real-world experiences. Molecular Genetics and Metabolism Reports, 2020, 23, 100591.	0.4	23
21	Effects of higher and more frequent dosing of alglucosidase alfa and immunomodulation on longâ€ŧerm clinical outcome of classic infantile Pompe patients. Journal of Inherited Metabolic Disease, 2020, 43, 1243-1253.	1.7	22
22	Diagnosis and Care of Infants and Children with Pompe Disease. Klinische Padiatrie, 2020, 232, 55-61.	0.2	3
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24	Pulmonary Manifestations of Endocrine and Metabolic Diseases in Children. Pediatric Clinics of North America, 2021, 68, 81-102.	0.9	0
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39	Multicentric Retrospective Evaluation of Five Classic Infantile Pompe Disease Subjects Under Enzyme Replacement Therapy With Early Infratentorial Involvement. Frontiers in Neurology, 2020, 11, 569153.	1.1	6
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